2023 Annual Report

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2023
OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to
Commission file number 001-38150

KALA BIO, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**27-0604595**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
1167 Massachusetts Avenue **Arlington, MA**	**02476**
(Address of principal executive offices)	(Zip Code)

(781) 996-5252
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.001 par value per share	**KALA**	**The Nasdaq Capital Market**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ Smaller reporting company ☒
Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of June 30, 2023, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the Common Stock held by non-affiliates of the registrant was approximately $34.1 million, based on the closing price of the registrant's common stock on June 30, 2023.

There were 2,816,454 shares of common stock, par value $0.001 per share, outstanding as of March 28, 2024.

DOCUMENTS INCORPORATED BY REFERENCE

Part III of this Annual Report on Form 10-K incorporates by reference information from the definitive Proxy Statement for the registrant's 2024 Annual Meeting of Stockholders, which is expected to be filed with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2023.

Table of Contents

References to KALA

Throughout this Annual Report on Form 10-K, the "Company," "KALA", "KALA BIO," "we," "us," and "our," except where the context requires otherwise, refer to KALA BIO, Inc. and its consolidated subsidiaries, and "our board of directors" refers to the board of directors of KALA BIO, Inc. On August 2, 2023, we changed our name from Kala Pharmaceuticals, Inc. to KALA BIO, Inc.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- our expectations with respect to our dependency on and potential advantages of KPI-012, our product candidate for the treatment of persistent corneal epithelial defects, or PCED;
- our expectations with respect to the potential impacts the sale of our commercial business to Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC, which we refer collectively as Alcon, will have on our business, results of operations and financial condition;
- our expectations with respect to, and the amount of, future milestone payments we may receive from Alcon in connection with the sale of our commercial business;
- our expectations with respect to, and the amount of, future milestone payments we may pay in connection with the acquisition of Combangio, Inc., or Combangio, or the Combangio Acquisition;
- our development efforts for KPI-012 and our ability to discover and develop new programs and product candidates;
- the timing, progress and results of clinical trials for KPI-012, including statements regarding the timing of initiation and completion of clinical trials, dosing of subjects and the period during which the results of the trials will become available;
- the timing, scope and likelihood of regulatory filings, including the filing of any biologics license applications for KPI-012 and any other product candidate we may develop in the future;
- our ability to obtain regulatory approvals for KPI-012;
- our commercialization, marketing and manufacturing capabilities and strategy for KPI-012, if approved;
- our estimates regarding potential future revenue from sales of KPI-012, if approved;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for KPI-012, if approved;
- the rate and degree of market acceptance and clinical utility of KPI-012 and our estimates regarding the market opportunity for KPI-012, if approved;
- plans to pursue the development of, and the timing, progress and results of preclinical studies of, KPI-012 for indications in addition to PCED, including Limbal Stem Cell Deficiency;

- our expectations with respect to our determination to cease the development of our preclinical pipeline programs that are unrelated to our mesenchymal stem cell secretome, or MSC-S, platform;
- the timing, progress and results of preclinical studies for our KPI-014 program;
- our expectations regarding our ability to fund our operating expenses, lease and debt service obligations, and capital expenditure requirements with our cash on hand;
- our expectations regarding our ability to achieve the specified milestones under our award from the California Institute for Regenerative Medicine, or CIRM, and obtain the full funding under the CIRM Award;
- our expectations regarding our ability to comply with the covenants under our loan agreement;
- our intellectual property position, including intellectual property acquired in the Combangio Acquisition;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- our estimates regarding expenses, future revenue, timing of any future revenue, capital requirements and needs for additional financing;
- the impact of government laws and regulations;
- our competitive position;
- developments relating to our competitors and our industry;
- our ability to maintain and establish collaborations or obtain additional funding;
- our business and business relationships, including with employees and suppliers; and
- the potential impact of global economic and geopolitical developments on our business, operations, strategy and goals.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in the "Risk Factors" section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K and the documents that we reference in this Annual Report on Form 10-K and have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of filing of this Annual Report on Form 10-K, and we do not assume any obligation to update any forward-looking statements except as required by applicable law.

This Annual Report on Form 10-K includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by us and third parties as well as our estimates of potential market opportunities. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunity for KPI-012 include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

Risk Factor Summary

Our business is subject to a number of risks that if realized could materially affect our business, financial condition, results of operations, cash flows and access to liquidity. These risks are discussed more fully in the "Risk Factors" section of this Annual Report on Form 10-K. Our principal risks include the following:

- We have incurred significant losses from operations and negative cash flows from operations since our inception. We expect to incur additional losses and may never achieve or maintain profitability. As of December 31, 2023, we had an accumulated deficit of $629.4 million.
- Our limited operating history and our limited experience in developing biologics may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development efforts. The milestone consideration we are eligible to receive in connection with the sale of our commercial business to Alcon is subject to various risks and uncertainties.
- Our substantial indebtedness may limit cash flow available to invest in the ongoing needs of our business, and a failure to comply with the covenants under our loan agreement, such as the requirement that our common stock continue to be listed on The Nasdaq Stock Market, could result in an event of default and acceleration of amounts due.
- We are substantially dependent on the success of KPI-012. If we are unable to successfully complete the clinical development of, and obtain marketing approval for, KPI-012 or any other product candidate we may develop in the future, or experience significant delays in doing so, or if, after obtaining marketing approvals, we fail to successfully commercialize such product candidates, our business will be materially harmed.
- If clinical trials of KPI-012 or any other biological product candidate that we develop fail to demonstrate potency, safety and purity to the satisfaction of the U.S. Food and Drug Administration, or FDA, or other regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidate. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials, and interim results of a clinical trial do not necessarily predict final results.
- If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates could be delayed or prevented, and our competitors could bring products to market before we do.
- If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.
- If serious adverse or unacceptable side effects are identified during the development or commercialization of our product candidates, we may need to abandon or limit our development and/or commercialization efforts for such product candidates.
- We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.
- KPI-012 has been evaluated in a clinical trial outside of the United States, and we may in the future conduct clinical trials for product candidates at sites outside the United States. The FDA may not accept data from trials conducted in such locations.
- Public health epidemics, including the COVID-19 pandemic, could impact the development of KPI-012 or any other product candidate we develop, and may adversely affect our business, results of operations and financial condition.

- Even if KPI-012 or any other product candidates that we may develop in the future receives marketing approval, such products may fail to achieve market acceptance by clinicians and patients, or adequate formulary coverage, pricing or reimbursement by third-party payors and others in the medical community, and the market opportunity for these products may be smaller than we estimate.
- If we are unable to establish and maintain sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, if and when necessary, we may not be successful in commercializing KPI-012 or any other product candidate that we may develop if and when they are approved.
- We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our competitors include major pharmaceutical companies with significantly greater financial resources. KPI-012 and any other product candidate we may develop, if approved, may also compete with existing branded, generic and off-label products.
- We have relied, and expect to continue to rely, on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.
- We contract with third parties for the manufacture of KPI-012 and plan to contract with third parties for preclinical, clinical and commercial supply of any other product candidates we develop. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.
- The manufacture of biologics is complex and our third-party manufacturers may encounter difficulties in production. If any of our third-party manufacturers encounter such difficulties, our ability to provide supply of product candidates for clinical trials or products for patients, if approved, could be delayed or prevented.
- Our reliance on CIRM funding for KPI-012 adds uncertainty to our research and development efforts, imposes certain compliance obligations on us and imposes requirements that may increase the costs of commercializing KPI-012.
- KPI-012 is protected by patent rights exclusively licensed from other companies or institutions. If these third parties terminate their agreements with us or fail to maintain or enforce the underlying patents, or we otherwise lose our rights to these patents, our competitive position and our market share in the markets for any of our products, if and when approved, will be harmed.
- If we fail to comply with the continued listing requirements of The Nasdaq Capital Market, our common stock may be delisted and the price of our common stock and our ability to access the capital markets could be negatively impacted. A delisting of our common stock from The Nasdaq Capital Market or a transfer of the listing of our common stock to another nationally recognized stock exchange having listing standards that are less restrictive than The Nasdaq Capital Market are each events of default under our Loan Agreement, which could adversely effect our financial condition and ability to pursue our business strategy.

Part I

Item 1. Business

Overview

We are a clinical-stage biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the front and back of the eye. Our product candidate, KPI-012, which we acquired from Combangio, Inc., or Combangio, on November 15, 2021, is a mesenchymal stem cell secretome, or MSC-S, and is currently in clinical development for the treatment of persistent corneal epithelial defects, or PCED, a rare disease of impaired corneal healing. Based on the positive results of a Phase 1b clinical safety and efficacy trial of KPI-012 in patients with PCED, along with favorable preclinical safety and efficacy results, we submitted an investigational new drug application, or IND, to the U.S. Food and Drug Administration, or FDA, which was accepted in December 2022. In February 2023, we dosed our first patient in our CHASE (**C**orneal **H**ealing **A**fter **SE**cretome therapy) Phase 2b clinical trial of KPI-012 for PCED in the United States, or the CHASE trial.

The CHASE trial is comprised of two patient cohorts. On March 27, 2023, we announced positive safety data from the first cohort of the CHASE trial, which is an open-label study to evaluate the safety of the high dose of KPI-012 ophthalmic solution (3 U/mL) dosed topically four times per day, or QID, in two patients. Both patients in the first cohort successfully completed at least one week of dosing with no safety issues observed. We have initiated the second and final patient cohort of the CHASE trial in the United States, which is a multicenter, randomized, double-masked, vehicle-controlled, parallel-group trial to evaluate the safety and tolerability of two doses of KPI-012 ophthalmic solution (3 U/mL and 1 U/mL) versus vehicle dosed topically QID for 56 days in approximately 90 patients. We plan to add trial sites in Latin America, subject to regulatory approval.

The primary endpoint of the trial is the complete healing of the PCED as measured by corneal fluorescein staining. We are targeting reporting topline safety and efficacy data from the CHASE trial by the end of 2024. If the results are positive, and subject to discussion with regulatory authorities, we believe this trial could serve as the first of two pivotal trials required to support the submission of a Biologics License Application, or BLA, for KPI-012 to the FDA.

KPI-012 has received Orphan Drug and Fast Track designations from the FDA for the treatment of PCED.

We believe the multifactorial mechanism of action of KPI-012 also makes our MSC-S a platform technology. We are evaluating the potential development of KPI-012 for additional rare front-of-the-eye diseases, such as for the treatment of Limbal Stem Cell Deficiency, or LSCD, and other rare corneal diseases that threaten vision. In addition, we have initiated preclinical studies under our KPI-014 program to evaluate the utility of our MSC-S platform for inherited retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease. In connection with the determination to focus our research and development efforts on KPI-012, in 2022, we determined to cease the development of our preclinical pipeline programs that are unrelated to our MSC-S platform. We expect to commercialize in the United States any of our product candidates that receive marketing approval.

We previously developed and commercialized two marketed products, EYSUVIS® (loteprednol etabonate ophthalmic suspension) 0.25%, for the short-term (up to two weeks) treatment of the signs and symptoms of dry eye disease, and INVELTYS® (loteprednol etabonate ophthalmic suspension) 1%, a topical twice-a-day ocular steroid for the treatment of post-operative inflammation and pain following ocular surgery. Both products applied a proprietary mucus-penetrating particle drug delivery technology, which we referred to as the AMPPLIFY® Drug Delivery Technology.

On July 8, 2022, Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC, which we refer to collectively as Alcon, purchased from us the rights to manufacture, sell, distribute, market and commercialize EYSUVIS and INVELTYS and to develop, manufacture, market and otherwise exploit the AMPPLIFY Drug Delivery Technology, which we collectively refer to as the Commercial Business. Alcon also assumed certain liabilities with respect to the Commercial Business at the closing of the transaction. Alcon paid us an upfront cash payment of $60.0 million upon the closing of the sale of the Commercial Business. We are also eligible to receive from Alcon up to four commercial-based sales milestone payments as follows: (1) $25.0 million upon the achievement of $50.0 million or more in aggregate worldwide

net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (2) $65.0 million upon the achievement of $100.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (3) $75.0 million upon the achievement of $175.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029 and (4) $160.0 million upon the achievement of $250.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029. Each milestone payment will only become payable once, if at all, upon the first time such milestone is achieved, and only one milestone payment will be paid with respect to a calendar year. In the event that more than one milestone is achieved in a calendar year, the higher milestone payment will become payable and the lower milestone payment will become payable only if the corresponding milestone is achieved again in a subsequent calendar year. To date, no milestones have been achieved, and we have not received any milestone payments from Alcon.

The following table describes the stage of each of our development programs:

Product Candidate*	Indication	Route of Administration	Pre-Clinical	Phase 1	Phase 2	Phase 3
KPI-012 for Rare Ocular Surface Disease	Persistent Corneal Epithelial Defect (PCED)	Topical				
	Limbal Stem Cell Deficiency (LSCD)	Topical				
	Other rare corneal diseases	Topical				
KPI-014 Program for Rare Inherited Retinal Disease		Intravitreal Injection				

* Product candidates are investigational and have not been approved by any regulatory authority.

We have retained worldwide commercial rights for our MSC-S platform, including KPI-012 and KPI-014. We own and/or exclusively license patents and patent applications relating to this platform, including U.S. and foreign issued patents and pending patent applications. The expiration dates of the issued U.S. patents that we control covering KPI-012 are scheduled to expire no earlier than 2040, and a portfolio of additional U.S. and ex-U.S. patent applications covering the MSC-S platform is currently in prosecution.

Strategy

Our goal is to become a leading biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the front and back of the eye. Key elements of our strategy include:

- ***Advance the clinical development of, and seek regulatory approval for, KPI-012 for the treatment of PCED.*** KPI-012 is a novel, human bone-marrow derived MSC-S currently in clinical development for the treatment of PCED. A PCED is a persistent non-healing corneal defect or wound that is refractory to conventional treatments. On March 27, 2023, we announced positive safety data from the first cohort of the CHASE trial and subsequently initiated the second and final patient cohort. We are targeting reporting topline safety and efficacy data for the CHASE trial by the end of 2024. If the results are positive, and subject to discussion with regulatory authorities, we believe this trial could serve as the first of two pivotal trials required to support the submission of a BLA for KPI-012 to the FDA. If approved, we intend to commercialize KPI-012 with a small, targeted, internal sales force in the United States. We also expect to explore commercialization of KPI-012 for the treatment of PCED in certain markets outside the United States utilizing a variety of collaboration, distribution, co-promotion and other marketing arrangements with one or more third parties.
- ***Advance KPI-012 for additional rare ocular surface disease indications and KPI-014 for rare inherited retinal degenerative diseases.*** We are also evaluating the potential of KPI-012 to treat other rare front-of-the-eye diseases, such as LSCD and other rare corneal diseases that threaten vision. In addition, we have

initiated preclinical studies of KPI-014, our preclinical program evaluating the utility of our MSC-S platform for inherited retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease.

- ***Business development through selective transactions.*** We plan to pursue value-driven business development opportunities as they arise in order to enhance our business and product pipeline, which may include opportunistically in-licensing or acquiring the rights to complementary products, product candidates and technologies, particularly for the treatment of rare ophthalmic diseases. We also plan to explore a variety of transactions to maximize the value of our assets, including out-licensing transactions, collaborations, distributions and other development and marketing arrangements with one or more third parties for our product candidates.

Our Clinical-Stage Product Candidate

KPI-012 for Persistent Corneal Epithelial Defects

Persistent Corneal Epithelial Defects Overview

PCED is a persistent non-healing corneal defect or wound that is refractory to conventional treatments. PCED is a disease of impaired corneal healing and can be the result of numerous etiologies, including (but not limited to) neurotrophic keratitis, or NK, microbial/viral keratitis, surgical epithelial debridement, corneal transplant surgery, LSCD, mechanical/thermal trauma and exposure keratopathy. Normal healing is a highly regulated multifactorial process that involves numerous biologic pathways and molecules, including growth factors, cell signaling, proliferation, migration and extracellular matrix remodeling. In PCED, the normal healing process is impaired due to an imbalance of the key biomolecules that orchestrate the normal wound healing process. We believe that effective treatment of PCED across the various etiologies requires a multifactorial mechanism of action to address the impaired healing that is responsible for the defects.

PCED is a rare disease with an estimated incidence of 100,000 cases per year in the United States and 238,000 cases per year in the United States, European Union and Japan combined. Clinical symptoms of PCED include pain, foreign body sensation, redness, photophobia and tearing. Clinical signs include non-healing epithelial defects, stromal scarring and stomal thinning. A PCED may lead to infection, corneal ulceration, corneal perforation, scarring, opacification and significant vision loss.

Limitations of Existing Treatments for Persistent Corneal Epithelial Defects

There is currently a significant unmet need for therapies to effectively treat PCED. Conventional therapies, which include bandage contact lenses, autologous serum and surgery, are usually ineffective in overcoming the dysregulation present in multiple cellular pathways that may need to be addressed to heal a PCED. Surgical procedures used in the treatment of PCED include tarsorrhaphy, corneal epithelial stem cell transplants and corneal transplants which are used to aid in restoration and maintenance of vision capabilities.

The only currently approved prescription product in the PCED space is Oxervate®, indicated for the treatment of NK, which we believe to be the primary etiology for approximately one-third of PCED cases. Oxervate contains a single growth factor – nerve growth factor (NGF) – and has been demonstrated to be effective in only the subgroup of PCED cases whose underlying etiology is neurotrophic disease. Oxervate is a topical eye drop that is administered six times per day at two-hour intervals for eight weeks. Each administration of Oxervate requires the use of a vial containing the drug product, a vial adapter, a single-use pipette and disinfectant wipes.

KPI-012 Opportunity in Persistent Corneal Epithelial Defects

KPI-012 is a novel, human bone-marrow derived MSC secretome composed of biologically active components secreted from the MSCs, such as growth factors, protease inhibitors, matrix proteins and neurotropic factors, that have been shown in preclinical studies by Combangio to facilitate corneal healing. KPI-012 is cell-free and produced from a proprietary cell bank. The drug substance for KPI-012 is produced as a chemically-defined cell-free solution followed by formulation and filling of the drug product in non-preserved single dose units. We believe that KPI-012's multi-factorial mechanism of action has the potential to normalize the impaired healing in PCED and other severe ocular surface

diseases driven by impaired healing. As such, we believe KPI-012 offers a potentially promising approach for the treatment of PCED and other ocular surface diseases across multiple etiologies. Key biological factors contained in KPI-012 and their potential wound healing functions are shown below:

Key KPI-012 Components	Ocular Surface Wound-Healing Function
Protease Inhibitors (TIMP-1, TIMP-2, Serpin E)	Inhibit destructive proteases that degrade matrix in the wound bed
Matrix Proteins (Fibronectin)	Build a molecular scaffold in the wound bed for cells to migrate and adhere to
Growth Factors (HGF)	Suppress inflammation and promote corneal epithelium repair
Neurotrophic Factors (PEDF)	Promote maintenance of neurons to support corneal health

The multifactorial mechanism of action of KPI-012 is thought to be responsible for the significant wound healing activity observed in Combangio's preclinical animal models and in the completed Phase 1b clinical trial. KPI-012 has received Orphan Drug and Fast Track designations from the FDA for the treatment of PCED.

CHASE 2b Clinical Trial of KPI-012 and Clinical Development Plan of KPI-012

We are initially developing KPI-012 for the treatment of PCED. Combangio completed a Phase 1b clinical efficacy trial in nine patients with PCED in Mexico City, Mexico. Based on the results of this Phase 1b clinical trial, we initiated a full preclinical development program and submitted an IND application to the FDA for KPI-012, which was accepted in December 2022. In February 2023, we dosed the first patient in the CHASE trial of KPI-012 in patients with PCED in the United States. The CHASE trial is comprised of two patient cohorts. On March 27, 2023, we announced positive safety data from the first cohort of the CHASE trial, which is an open-label study to evaluate the safety of the high dose of KPI-012 ophthalmic solution (3 U/mL) dosed topically QID in two patients. Both patients in the first cohort successfully completed at least one week of dosing with no safety issues observed.

We have initiated the second and final patient cohort in the CHASE trial in the United States, and we plan to add trial sites in Latin America, subject to regulatory approval. The second cohort is a multicenter, randomized, double-masked, vehicle-controlled, parallel-group trial in PCED patients with varying underlying etiologies to evaluate the safety and efficacy of two doses of KPI-012 ophthalmic solution (3 U/ml and 1 U/ml) compared to vehicle when dosed topically QID for 56 days. The trial has an 8-week treatment period with evaluations at frequent times during the dosing period and at 10 weeks and 26 weeks.

The trial is expected to enroll approximately 90 adult patients with PCED, and the primary endpoint is complete healing of the PCED at week 8 as measured by corneal fluorescein staining using a central-reading center assessment of corneal fluorescing staining photographs. We are targeting reporting topline safety and efficacy data from the CHASE trial by the end of 2024. If the results are positive, and subject to discussion with regulatory authorities, we believe this trial can serve as the first of two pivotal trials required to support the submission of a BLA for KPI-012 to the FDA.

Phase 1b Clinical Trial Results of KPI-012

Combangio conducted a Phase 1b clinical trial of KPI-012 in Mexico City, Mexico during 2020 and 2021, consisting of three subjects without active corneal disease, or the safety cohort, who were dosed twice a day (1 U/mL) for one week and nine patients with PCED, or the PCED cohort, who were dosed twice a day (1 U/mL) for up to eight weeks. Key inclusion criteria for the PCED cohort included:

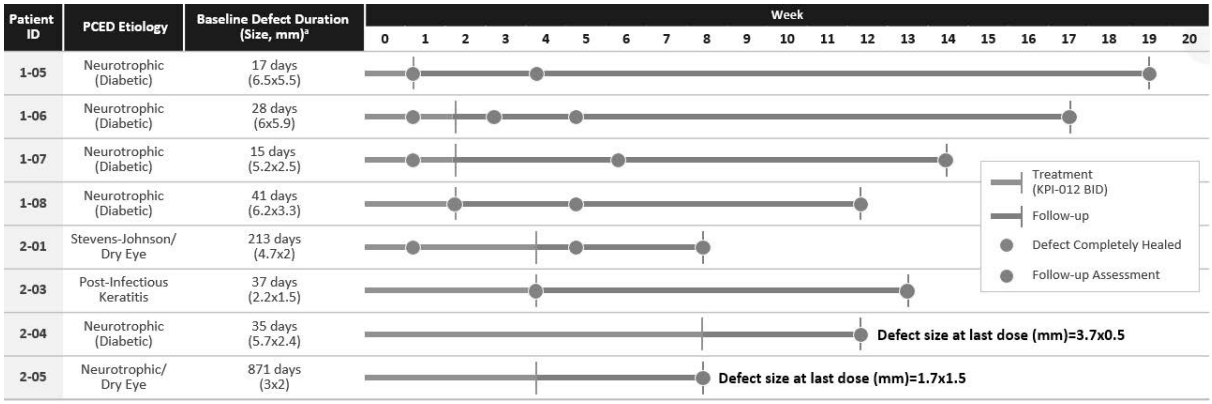
- Subjects with PCED of at least 10 days without improvement from one or more conventional non-surgical treatments in study eye due to any of the following:
  - NK, provided there was no active herpetic infection of the eye in the prior three months

- Corneal Burns (alkali, acid and thermal)
- Post-photorefractive Keratectomy
- Post-corneal Transplant Surgery
- Corneal epithelial debridement resulting from Diabetic Vitrectomy Surgery
- Trauma
- Keratoconjunctivtis sicca
- Sjögren's
- Corneal cross-linking

- Subjects with bilateral corneal burns could only have one eye entered into the clinical trial
- Any previous treatment was stopped except for the study medication

The participants in the Phase 1b trial were treated with KPI-012 topically twice a day, with the subjects in the safety cohort treated for one week and patients in the PCED cohort treated between one to eight weeks. KPI-012 was generally well tolerated in both cohorts, with only one subject experiencing treatment-related adverse events (mild and transient itching, red eye and blurred vision after study drug administration). There were no deaths or treatment-related serious adverse events during either cohort. One subject in the PCED cohort had to withdraw from the trial due to a protocol screening violation.

As depicted in Figure 1 below, six of the eight patients in the PCED cohort (75%) who completed the trial achieved complete healing of the lesion after four weeks of treatment, with the two other patients experiencing some clinical improvement but not complete healing. Four of eight patients in the PCED cohort (50%) achieved complete healing of the lesion after one week of treatment and the other two patients achieved complete healing within two to four weeks of initiation of treatment with KPI-012. All six of the patients who achieved complete healing remained healed through the follow-up period of the trial, which ranged between eight to 19 weeks. Of the two patients who did not show complete healing in the trial, clinical investigators noted some clinical improvement in both patients, but the corneal staining images did not show complete healing of the defect.



	Mean	Median
PCED Size at Baseline (mm x mm)	5.1 x 3.5	5.6 x 2.9
PCED Duration at Baseline (Days)	58	32
PCED Healing Time (Days) KPI-012, 2x/day	12	7

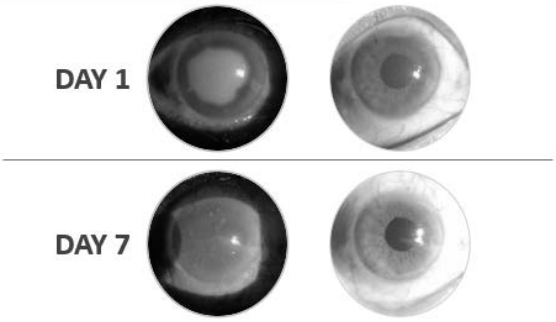


Figure 1. Summary of Phase 1b clinical trial of KPI-012 for PCED, including representative images for a healed patient study eye. The Day 1 images were taken on the first day of treatment, prior to first KPI-012 administration, with the fluorescein (green) stain demarking the corneal wound boundary of the study eye image. The Day 7 images were taken on the last day of KPI-012 treatment showing the PCED completely healed. The images on the left depict the study eye viewed under blue light to visualize the PCED with fluorescein stain.

Significant pain relief was reported by patients in the PCED cohort within one week of treatment with KPI-012, as shown in Figure 2 below. Of the six patients who reported pain at the baseline, all six patients reported a reduction in pain after one week of treatment, four patients reported a pain score of zero after one week of treatment and all six patients reported a pain score of zero after three weeks of treatment.

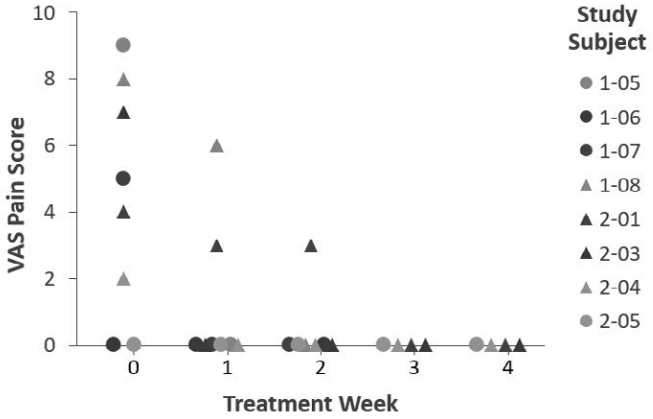


Figure 2. PCED cohort patient-reported score of pain level due to defect using a visual analogue scale, or VAS, which is a subjective rating of pain levels on a scale of 0 to 10 where a score of 0 represents no pain at all and a score of 10 represents the worst possible pain.

KPI-012 Preclinical Studies and Results

KPI-012 was evaluated by Combangio in a number of preclinical studies. In these studies, KPI-012 promoted rapid ocular re-epithelialization and mitigated scarring and neovascularization in a number of well-established animal models.

In vitro Human Corneal Epithelial Wound Closure Assay

The therapeutic mechanism of action of KPI-012 involves stimulating corneal re-epithelialization and ocular surface healing. Combangio evaluated KPI-012 in an *in vitro* wound gap assay developed using human corneal epithelial cells. In this assay, a mechanical defect (cell-free region) was introduced into a two-dimensional monolayer of epithelial cells to create a wound. The 'injured' monolayer was then treated with KPI-012 and the cell free region was monitored for wound closure as show in Figure 3 below. In this assay, KPI-012 exhibited a dose-dependent and potent wound closure response.

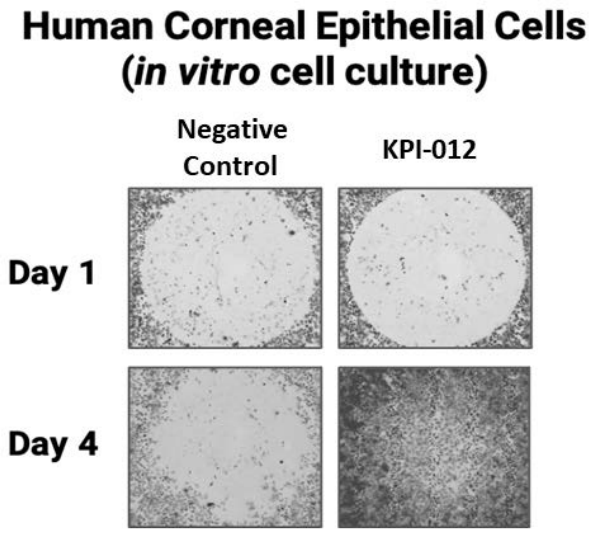


Figure 3. Representative images from an in vitro human corneal epithelial wound closure assay. A mechanical wound instilled to a corneal epithelial cell monolayer on Day 1 healed after treatment with KPI-012 (Day 4 of treatment), but not negative control (vehicle). Depicted images are wounded cell monolayers stained with Gentian Violet.

In vivo Mechanical Wound Studies of Activity

Combangio also evaluated the activity of KPI-012 in a mechanical corneal injury mouse model. In this model, a circular area on the surface of the cornea was debrided (mechanically scraped) to remove the epithelial layer and create a circular wound.

Topical formulations of vehicle or KPI-012 were administered twice daily to the wounded eyes. As shown in Figure 4 below, mice treated with KPI-012 exhibited prominent wound healing at day four of the treatment period, while the vehicle-treated wounded eyes remained largely unhealed. Further, treatment with KPI-012 resulted in reduced corneal haze and scarring relative to treatment with vehicle. Results of this mouse model suggested that at Day 4 of treatment KPI-012 promoted *in vivo* closure of cornea mechanical wounds relative to vehicle control.

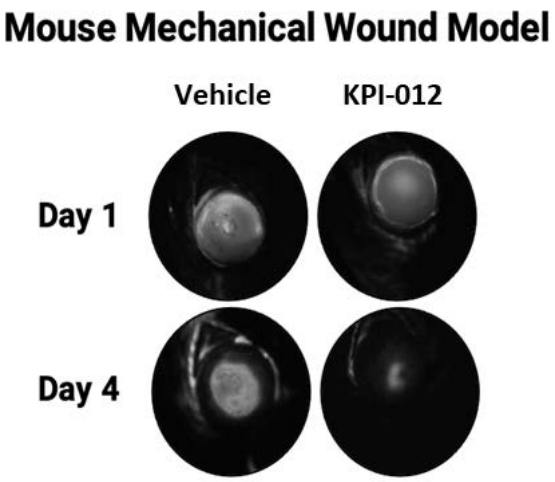


Figure 4. Representative images of wounded mouse corneas after mechanical injury (Day 1). Depicted is the fluorescein (green) stain, which demarks the corneal wound boundary. Treatment with KPI-012 rapidly healed the wound size (as indicated by the disappearance of the green stain by Day 4) relative to vehicle control-treated eyes.

A second confirmatory mechanical corneal injury mouse model study was performed according to the method described above using a different lot of KPI-012. The study yielded similar results, with KPI-012 promoting wound healing relative to vehicle as well as exhibiting dose-dependent potency dynamics. After four days of treatment, KPI-012 treated eyes exhibited more pronounced reduction in wound staining relative to vehicle-treated eyes, as shown in Figure 5A below, and after five days most KPI-012 treated eyes completely healed, as shown in Figure 5B below. Further, a KPI-012 formulation lacking key biologic factors known to mediate wound healing exhibited reduced healing capacity in the study, supporting the selection of KPI-012's critical quality attributes.

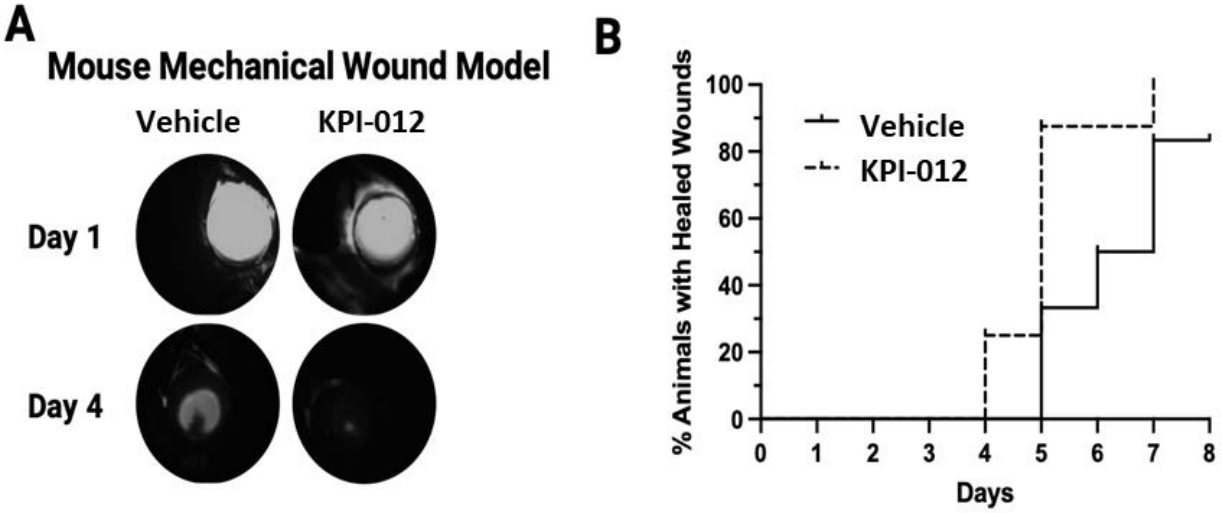


Figure 5. Summary of second mouse corneal mechanical study. (A) Representative images of wounded mouse corneas after mechanical injury (Day 1) and after four days of treatment with KPI-012 or vehicle (Day 4). Depicted is the fluorescein (green) stain, which demarks the corneal wound boundary. Treatment with KPI-012 rapidly healed the wound size (as indicated by the disappearance of the green stain by Day 4) relative to vehicle control-treated eyes; (B) Treatment with KPI-012 resulted in more rapid complete healing and a greater percentage of completely healed eyes (dashed line), relative to vehicle-treated eyes (solid line).

Other Potential Indications for KPI-012

We believe the multifactorial mechanism of action of KPI-012 also makes it a platform technology, and we are evaluating the potential development of KPI-012 for additional rare front-of-the-eye diseases, such as for the treatment of LSCD and other rare corneal diseases that threaten vision.

LSCD is an ocular surface disease characterized by the loss or deficiency of stem cells in the junction of the cornea and limbus, where they play an essential role in the generation and repopulation of corneal epithelial cells. When the limbal stem cell population is reduced or depleted, the ability of the corneal epithelium to repair and renew itself is compromised, which can result in recurrent epithelial breakdown, neovascularization, conjunctival overgrowth and other sequalae that can lead to loss of corneal clarity and vision impairment, as well as significant pain and diminished quality of life. There are currently no approved pharmaceutical products for the treatment of LSCD and there are an estimated 100,000 patients in the United States suffering from this disease. We believe these patients may be appropriate candidates for KPI-012 to maintain the integrity of the ocular surface and to avoid the vision impairment and pain associated with the disease. In addition to the effects of KPI-012 on corneal healing observed in both animal models and in PCED patients, there is data in the literature that suggest that MSC-S can restore the limbal stem cell niche, which would be of significant benefit in both partial or complete LSCD.

Potential Indications for KPI-014

We are also aiming to leverage the manufacturing and delivery expertise gained from KPI-012 development to develop a unique secretome formulation – designated as KPI-014 – specific for inherited retinal degenerative diseases. We have initiated preclinical studies under our KPI-014 program to evaluate the utility of our MSC-S platform for inherited retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease.

MSC-S therapies have shown great promise to treat inherited retinal diseases, or IRDs, with the recognition that they function through their secretome (i.e., the secretion of paracrine factors that enhance retinal cell function and survival). We believe an MSC-S engineered for intravitreal delivery may provide an improved treatment option for IRDs as compared to the traditional MSC-based approach.

IRDs are associated with mutations in over 280 different genes, where each IRD has one or more mutations that cause disease onset and results in vision loss. It is projected that over 200,000 individuals in the United States alone suffer from IRDs. While significant progress has been made with gene therapies, these are typically limited to a single gene or mutation. With over 280 different IRD-associated genes, a therapy broadly effective for most IRDs does not currently exist, leaving patients with little-to-no options to slow disease progression and vision loss. We are developing KPI-014 with the goal of providing a broad, genotype-agnostic, therapeutic benefit to reduce vision loss and improve quality of life for patients suffering from inherited retinal degenerative diseases.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technologies, knowledge, experience and scientific resources provide us with competitive advantages, we face competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Our competitors include large pharmaceutical and biotechnology companies, and specialty pharmaceutical and generic drug companies. Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. These competitors also compete with us in recruiting and retaining qualified scientific, sales and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of KPI-012 and any other product candidates that we develop are the product candidate's efficacy, safety, method of administration, convenience, price, the level of generic competition and the availability of insurance coverage and reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our products. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. In addition, our ability to compete may be affected because in many cases insurers or other third-party payors seek to encourage the use of generic products.

Competition in PCED

There is currently a significant unmet need for therapies to effectively treat PCED. Conventional therapies, which include bandage contact lenses, autologous serum and surgery, are usually ineffective in overcoming the dysregulation present in multiple cellular pathways that may need to be addressed to heal a PCED. Surgical procedures used in the treatment of PCED include tarsorrhaphy, corneal epithelial stem cell transplants and corneal transplants which are used to aid in restoration and maintenance of vision capabilities.

There is one approved prescription pharmaceutical product in the PCED space. Oxervate (cenegermin-bkbj), which was approved in August 2018 for the treatment of NK, a degenerative disease characterized by decreased corneal sensitivity and poor corneal healing, which we believe to be the primary underlying etiology of approximately one-third of all PCED cases. Oxervate is a topical eye drop that is administered six times per day at two-hour intervals for eight weeks. Each administration of Oxervate requires the use of a vial containing the drug product, a vial adapter, a single-use pipette and disinfectant wipes.

To our knowledge, there are currently only two product candidates in active clinical development for the treatment of a broad PCED population. KIO-201, a chemically modified form of the natural polymer hyaluronic acid administered as an eye drop, is currently being studied in a Phase 2 clinical trial in patients with PCED by Kiora Pharmaceuticals, Inc. Nexagon®, an antisense oligonucleotide that inhibits connexin43 being developed by Amber Ophthalmics, is currently being studied in a Phase 2/3 clinical trial in patients with PCED resulting from severe ocular chemical and/or thermal injuries. Amber Ophthalmics has also indicated that it plans to study Nexagon® in a broad PCED population.

A number of companies are pursuing development of product candidates for the treatment of NK, including ReGenTree, LLC (Timbetasin), Recordati S.p.A. (Udonitrectag) and Claris Biotherapeutics, Inc. (CSB-001).

Competition in Limbal Stem Cell Deficiency

Competitive products and product candidates in LSCD include two stem cell-based approaches. ABCB5+ limbal stem cells, which are being studied in Phase 1/2 clinical trials and are being developed by RHEACELL GmbH & Co. KG, utilize allogeneic limbal stem cells derived from human corneal rims, which are expanded ex-vivo and manufactured as an advanced-therapy medicinal product. Holoclar utilizes autologous limbal stem cells derived from the healthy portion of the patient's eye. Holoclar is approved in the European Union for treatment of LSCD caused by ocular burns and is developed by Chiesi. To our knowledge, there are no other products in development focused on LSCD.

Sales and Marketing

We have not yet established our own commercial organization or distribution capabilities specific to KPI-012. We believe that we will be able to commercialize KPI-012, if approved, for the treatment of PCED with a small, targeted, internal sales force in the United States and potentially other major markets. We expect to explore commercialization of KPI-012, if approved, in certain markets outside the United States utilizing a variety of collaboration, distribution, co-promotion, distribution and other marketing arrangements with one or more third parties.

Manufacturing and Supply

We do not own or operate, and currently have no plans to establish, any manufacturing facilities for KPI-012. We rely, and expect to continue to rely, on third parties for the manufacture of both drug substance and finished drug product for KPI-012 for preclinical and clinical testing, as well as for commercial manufacture of KPI-012 if it receives marketing approval. We utilize our substantial in-house expertise and know-how to develop and scale-up our manufacturing processes before these processes are transferred to third-party contract manufacturers, and to understand and establish controls of critical process parameters. We also have personnel with deep product development experience who actively manage the third-party contract manufacturers producing KPI-012 and plan to use such personnel to manage third-party contract manufacturers for any products that we may develop in the future.

We also rely, and expect to continue to rely, on third parties for packaging, labeling, sterilization, storage, distribution and other production logistics for KPI-012. We have only limited supply agreements in place with respect to the manufacturing of KPI-012, and these arrangements do not extend to commercial supply. We obtain supplies of drug substance and finished drug product for KPI-012 on a purchase order basis and do not have long term committed supply arrangements with respect to KPI-012.

Manufacturing biologics is complex, especially in large quantities. Biologic products must be made consistently and in compliance with a clearly defined manufacturing process. KPI-012 is a bone-marrow derived MSC-S therapeutic composed of biologically active components, including protease inhibitors and growth factors, and is produced from a proprietary cell bank. The manufacturing process for KPI-012 is comprised of three stages: (1) cultivation of MSCs from a working cell bank and production of unprocessed conditioned media (cell-free secretome), (2) production of drug substance as a chemically defined solution and (3) formulation and filling of drug product. While the drug product for Combangio's early research and Phase 1b clinical trial was cultivated using a planar culture model, we implemented a bioreactor cultivation model for our ongoing CHASE Phase 2b clinical trial of KPI-012. We also plan to utilize a bioreactor cultivation model for our planned clinical trials and for commercial supply of KPI-012. We are continuing the process of scaling up our manufacturing processes and capabilities with our third-party manufacturers to support longer term clinical development. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance.

KPI-012 drug product is manufactured from a vial of a working cell bank, which in turn was produced from a vial of master cell bank. KPI-012 master cell bank and working cell bank are stored in two separate locations. It is possible that we could lose the cell bank in both locations and have our manufacturing severely impacted by the need to replace the cell bank.

Intellectual Property

Our success depends significantly on our ability to obtain and maintain proprietary protection for our products, product candidates, technology and knowhow, to operate without infringing the proprietary rights of others and to prevent others from infringing our proprietary rights. We seek to protect our proprietary position by, among other methods, filing U.S. and certain foreign patent applications related to our proprietary technology, inventions and improvements that are important to the development of our business, where patent protection is available. We also rely on trade secrets, know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position.

As of March 25, 2024, we owned six U.S. issued patents and five U.S. patent applications, as well as twenty-nine foreign patent applications (including Patent Cooperation Treaty, or PCT, applications). We exclusively licensed one U.S. patent application, as well as two foreign patent applications including original filings, continuations and divisional applications. Our patent portfolio includes the following patents and patent applications that we own or exclusively license:

- Six U.S. patents and five U.S. patent applications relating to pharmaceutical compositions including KPI-012 for treating ocular conditions, and twenty-nine related foreign patent applications, which are owned by us, and which, if granted with respect to the patent applications, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, are expected to expire beginning in 2040;

- One U.S. patent application, related to secreted stem cell factors for tissue repairment and regeneration, and two related foreign patent applications, which are exclusively in-licensed from Stanford University, and which, if granted with respect to the patent applications, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, are expected to expire beginning in 2038;

Three U.S. patents relating to antibiotic compounds and their uses, and five related foreign patents, which are owned by us, and which, if granted with respect to the patent applications, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, are expected to expire in 2034. The term of individual patents depends upon the legal term for patents in the countries in which they are granted. In most countries, including the United States, the patent term is generally 20 years from the earliest claimed filing date of a non-provisional patent application in the applicable country. In the United States, a patent's term may, in certain cases, be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over a commonly owned patent or a patent naming a common inventor and having an earlier expiration date. The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, permits a patent term extension of up to five years beyond the expiration date of a U.S. patent as partial compensation for the length of time the drug is under regulatory review while the patent is in force. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent applicable to each regulatory review period may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. We cannot provide any assurance that any patent term extension with respect to any U.S. patent will be obtained and, if obtained, the duration of such extension.

Similar provisions are available in the European Union and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our product candidates receive approval by the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, if permitted under the applicable laws, regulations, and rules and depending upon the length of the clinical trials for each drug and other factors. The expiration dates referred to above are without regard to potential patent term extension or other market exclusivity that may be available to us. However, we cannot provide any assurances that any such patent term extension of any patent will be obtained and, if obtained, the duration of such extension.

Trade Secrets

In addition to patents, we may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, and obtain and maintain ownership of certain technologies, in part, by confidentiality and invention assignment agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems.

Licensing and Other Arrangements

Stanford University License Agreement

As part of our acquisition of Combangio, we acquired Combangio's exclusively in-licensed patent portfolio from Stanford University. In October 2019, Combangio entered into a license agreement with The Board of Trustees of The Leland Stanford Junior University, or Stanford, which was amended in February 2020. Pursuant to the license agreement with Stanford, or the Stanford Agreement, we hold a worldwide, exclusive, sublicensable license under certain patent rights, or licensed patents, directed to methods to promote eye wound healing, to make, have made, use, import, offer to sell and sell products that are covered by the licensed patents, or licensed products, for use in all fields.

Financial Terms

In consideration for that license, Combangio paid Stanford an upfront fee of $15,000. Under the Stanford Agreement, we are obligated to pay Stanford annual license maintenance fees in the low-to-mid five figures which are creditable against earned royalties owed to Stanford for the same year, an aggregate of up to $1.1 million for the achievement of specified development and regulatory milestones, and an aggregate of up to $1.1 million for the achievement of specified sales milestones. Stanford is also entitled to receive tiered royalties from us in a low single digit percentage range of our, our affiliates' and our sublicensees' net sales of licensed products that are covered by a valid claim of a licensed patent. Our obligation to pay royalties will continue, on a country-by-country and licensed product-by-licensed product basis, until the last-to-expire valid claim of a licensed patent covering such licensed product in the country of manufacture and sale. Additionally, we are required to pay Stanford a low double-digit percentage of certain consideration we receive as a result of granting sublicenses to the licensed patents. In connection with our acquisition of Combangio, we paid Stanford a one-time change of control fee of $100,000. Stanford retains the right, on behalf of itself, Stanford Health Care, Lucile Packard Children's Hospital at Stanford, and all other non-profit research institutions, to practice the licensed patents for any non-profit purpose. In addition, the United States government retains nonexclusive rights under the licensed patents to practice or have practiced the licensed patents by or on behalf of the United States government or on behalf of any foreign government or international organization pursuant to treaty or agreement.

Diligence Obligations

Under the Stanford Agreement, we are obligated to diligently develop, manufacture and sell licensed product, diligently develop markets for licensed product, and use commercially reasonable efforts to achieve certain funding and development milestones by specified dates.

Term and Termination

Unless earlier terminated, our exclusive license under the Stanford Agreement will continue until the expiration of the licensed patents. We may terminate the Stanford Agreement at any time for any reason by providing at least 30 days' written notice to Stanford. Stanford may terminate the agreement if we breach certain provisions of the agreement and fail to remedy such breach within 60 days after written notice of such breach by Stanford.

Combangio Merger Agreement

Pursuant to the Agreement and Plan of Merger, or the Merger Agreement, entered into with Combangio in connection with the acquisition of Combangio in November 2021, the former Combangio stockholders or other equityholders, or the Combangio Equityholders, are entitled to receive from us up to $105.0 million in payments that are contingent upon the achievement of specified development, regulatory and commercialization milestones, or the Contingent Consideration.

Upon dosing of the first patient in our CHASE trial for PCED in the United States in February 2023, we paid the former Combangio Equityholders an aggregate of $2.5 million in cash and $2.4 million in shares of our common stock (representing an aggregate of 105,038 shares of our common stock) in March 2023. The remaining amount of $0.1 million was paid in January 2024. Any Contingent Consideration payable under the Merger Agreement in the future will be paid only in cash as follows:

- (i) $5.0 million payable upon the first patient dosed with any product candidate whose active ingredient comprises one or more biological factors secreted by MSCs or their progenitors, including KPI-012, or the Product Candidate, in a pivotal clinical trial, (ii) $12.5 million payable upon regulatory approval by the FDA of marketing and sale of a Product Candidate in the United States, subject to certain specified reductions; (iii) $17.5 million payable upon the first commercial sale of a Product Candidate in the United States, subject to certain specified reductions and (iv) an aggregate of up to $65.0 million payable upon the achievement of specified sales milestones;
- tiered cash royalties at percentage rates in the mid-to-high single digits payable on annual net sales of all Product Candidates; and

- a cash payment at a percentage rate in the high single digits of all income, including earnout payments, received by us or any of our affiliates from a product license granted by us to a third party to sell or otherwise commercialize the Product Candidate in countries where neither we nor our affiliates conduct sales of such Product Candidate, subject to certain exceptions set forth in the Merger Agreement.

If the aggregate amount of Contingent Consideration payable in any calendar year exceeds $2.5 million, or the Excess Cash Cap, such excess portion, or the Carry Forward Contingent Cash Consideration, will be carried forward and, subject to application of the Excess Cash Cap in the following calendar year, become payable on the first business day of the following calendar year. Any Carry Forward Contingent Cash Consideration outstanding on June 1, 2026 is payable in full on June 1, 2026.

CIRM Award

Our development of KPI-012 is currently being funded, in part, by an award from the California Institute for Regenerative Medicine, or CIRM. On August 2, 2023, Combangio entered into an award agreement with CIRM for a $15.0 million grant, or the CIRM Award, to support the KPI-012 program for the treatment of PCED as well as product and process characterization and analytical development for the program. The CIRM Award is subject to a co-funding requirement under which Combangio is obligated to spend a specified minimum amount on the development of KPI-012 to obtain the full award amount and a significant portion of the award is payable to Combangio upon the achievement of specified milestones that are primarily related to Combangio's progress in conducting the CHASE clinical trial. Combangio has received an initial $5.9 million disbursement from CIRM, and the balance of $9.1 million is payable to Combangio upon the achievement of specified milestones. If we fail to satisfy the co-funding requirement under the CIRM Award or fail to achieve the milestones within the timeframe required by the CIRM Award, we may not receive full funding under the CIRM Award. CIRM may permanently cease disbursements under the CIRM Award if the milestones are not met within four months of their scheduled completion dates. Additionally, if CIRM determines, in its sole discretion, that Combangio has not complied with the terms and conditions of the CIRM Award, CIRM may suspend or permanently cease disbursements. Moreover, disbursements under the CIRM Award are contingent upon the availability of funds in the state of California's Stem Cell Research and Cures Fund.

The CIRM Award also imposes financial conditions that may increase the costs of commercializing KPI-012, if approved. Under the terms of the CIRM Award, Combangio is obligated to pay a royalty on net sales of any product, service or approved drug resulting in whole or in part from the CIRM Award in the amount of 0.1% per $1.0 million of funds utilized by us until the earlier of 10 years from the date of first commercial sale of such product, service or approved drug and such time as nine times the amount of funds awarded by CIRM has been paid in royalties, or the Base Royalty. In addition, following the satisfaction of the Base Royalty, Combangio is obligated to pay a 1.0% royalty on net sales of any CIRM-funded invention in excess of $500 million per year until the last to expire patent covering such invention expires.

Additionally, there are significant compliance requirements associated with the CIRM Award, such as reporting, notification, recordkeeping and audit requirements, for which internal and external resources may be needed and which may increase our costs of doing business.

Securities Purchase Agreement for 2022 Private Placement

On November 28, 2022, we entered into a Securities Purchase Agreement, or the 2022 Securities Purchase Agreement, with certain institutional investors named therein, or the Purchasers, pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of our common stock and shares of our Series E Convertible Non-Redeemable Preferred Stock, or Series E Preferred Stock, in two tranches for aggregate gross proceeds of up to $31.0 million, which we refer to collectively as the Private Placement.

Pursuant to the 2022 Securities Purchase Agreement, if at any time during the four-year period following the date of the first tranche closing, or the Participation Period, we propose to offer and sell new equity securities in an offering that is conducted pursuant to an exemption from registration under the Securities Act of 1933, as amended, or the Securities Act, or in an offering that is registered under the Securities Act that is not conducted as a firm-commitment underwritten offering, then, subject to compliance with securities laws and regulations, we have agreed to offer each Purchaser the right to purchase its pro rata share of the total amount of the new equity securities, subject to certain conditions and limitations. In addition, if during the Participation Period, we propose to offer and sell new equity

securities in a firm-commitment underwritten offering registered under the Securities Act, then subject to compliance with securities laws and regulations, we have agreed to use our commercially reasonable efforts to cause the managing underwriters of such offering to contact the Purchasers about potentially participating in such offering and to provide to each Purchaser the opportunity to purchase its pro rata share of such new equity securities, subject to certain conditions and limitations. The participation rights will terminate if the Purchasers are offered the opportunity to participate in an offering pursuant to the participation rights and do not purchase at least 50% of their aggregate pro rata share of the new equity securities offered for sale in such offering.

Pursuant to the 2022 Securities Purchase Agreement, the Purchasers have the right to have up to two non-voting observers attend and participate in all Board and committee meetings and, subject to the Purchasers owning directly specified minimum amounts of our common stock, the right to have the Board nominate and recommend for election by the stockholders up to three Purchaser designees to the Board (one designee at 9.9%, two designees at 15.0% and three designees at 25.0%) designated by the Purchasers, provided that at such time as the Purchasers have designated three Board designees, at least one such designee must qualify as an "independent" director under Nasdaq rules and be acceptable to the members of the Board who are not Purchaser designees.

The Purchasers' participation rights, observer rights and Board designation rights also will terminate at such time as the Purchasers and their affiliates cease to own, in the aggregate, specified minimum amounts of the shares purchased in the Private Placement.

Pursuant to the 2022 Securities Purchase Agreement, we agreed that we will not without the prior approval of the requisite Purchasers (i) issue or authorize the issuance of any equity security that is senior or *pari passu* to the Series E Preferred Stock with respect to liquidation preference, (ii) incur any additional indebtedness for borrowed money in excess of $1,000,000, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of our existing indebtedness or (iii) pay or declare any dividend or make any distribution on, any shares of our capital stock, subject to specified exceptions. We have filed a registration statement covering the resale of the shares of common stock acquired in the Private Placement and the shares of common stock issuable upon conversion of the shares of Series E Preferred Stock acquired in the Private Placement.

Securities Purchase Agreement for 2023 Private Placement

On December 21, 2023, we entered into a Securities Purchase Agreement, or the 2023 Securities Purchase Agreement, with certain institutional investors named therein, or the Series F Purchasers, pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of our Series F Convertible Non-Redeemable Preferred Stock, or Series F Preferred Stock, for aggregate gross proceeds of approximately $2.0 million. We agreed that we will not without the prior approval of the requisite Series F Purchasers (i) issue or authorize the issuance of any equity security that is senior or *pari passu* to the Series F Preferred Stock with respect to liquidation preference, (ii) incur any additional indebtedness for borrowed money in excess of $1,000,000, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of our existing indebtedness or (iii) pay or declare any dividend or make any distribution on, any shares of our capital stock, subject to specified exceptions. We have agreed to register for resale the shares of common stock issuable upon conversion of the Series F Preferred Stock, upon demand by the Series F Purchasers.

Securities Purchase Agreement for 2024 Private Placement

On March 25, 2024, we entered into a Securities Purchase Agreement, or the 2024 Securities Purchase Agreement, with certain institutional investors named therein, or the Series G Purchasers, pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of our Series G Convertible Non-Redeemable Preferred Stock, or Series G Preferred Stock, for aggregate gross proceeds of approximately $8.6 million. We agreed that we will not without the prior approval of the requisite Series G Purchasers (i) issue or authorize the issuance of any equity security that is senior or *pari passu* to the Series G Preferred Stock with respect to liquidation preference, (ii) incur any additional indebtedness for borrowed money in excess of $1,000,000, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of our existing indebtedness or (iii) pay or declare any dividend or make any distribution on, any shares of our capital stock, subject to specified exceptions. We have agreed to register for resale the shares of common stock issuable upon conversion of the Series G Preferred Stock, upon demand by the Series G Purchasers.

Government Regulation and Product Approvals

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, pricing, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of biopharmaceutical products. The processes for obtaining marketing approvals in the United States and in foreign countries and jurisdictions, along with compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

U.S. Government Regulation of Biological Products

In the United States, biological products, or biologics, are licensed for marketing by the FDA under the Public Health Service Act, or the PHSA, and regulated by the FDA under the Food, Drug and Cosmetic Act, or FDCA. A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products, and for their regulatory approval, is typically referred to as a sponsor. A sponsor seeking approval to market and distribute a new biologic in the United States must satisfactorily complete each of the following steps:

- completion of preclinical laboratory tests, animal studies and formulation studies according to good laboratory practices, or GLP, regulations or other applicable regulations;
- design of a clinical protocol and submission to the FDA of an IND, which must become effective before human clinical trials may begin and must be updated when certain changes are made;
- approval by an independent institutional review board, or IRB, or ethics committee representing each clinical trial site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with applicable IND regulations, good clinical practices, or GCPs, and other clinical-trial related regulations to evaluate the safety and efficacy of the investigational product for each proposed indication;
- preparation and submission to the FDA of a BLA, demonstrating the safety, purity and potency of the proposed product and requesting marketing approval for one or more proposed indications, including payment of application user fees;
- review of the BLA by an FDA advisory committee, where applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the biologic is produced to assess compliance with current good manufacturing practice, or cGMP, requirements to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of any FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data submitted in support of the BLA; and
- FDA review and approval of the BLA, which may be subject to additional post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and any post-approval studies required by the FDA.

Preclinical Studies

Before a sponsor begins testing a product candidate with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry,

formulation and stability, as well as other studies to evaluate, among other things, the toxicity of the product candidate. These studies are typically referred to as IND-enabling studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements, including GLP regulations and standards and the United States Department of Agriculture's Animal Welfare Act, if applicable. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, and long-term toxicity studies, may continue after the IND is submitted.

The IND and IRB Processes

An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. Such authorization must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved BLA. In addition to reviewing an IND to ensure the safety and rights of patients, the FDA also focuses on the quality of the investigation and whether it will be adequate to permit an evaluation of the drug's safety and efficacy. In support of a request for an IND, sponsors must submit a protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, must be submitted to the FDA as part of an IND. The FDA requires a 30-day waiting period after the filing of each IND before clinical trials may begin. This waiting period is designed to allow the FDA to review the IND to determine whether human research subjects will be exposed to unreasonable health risks. At any time during this 30-day period, or thereafter, the FDA may raise concerns or questions about the conduct of the trials as outlined in the IND and impose a clinical hold or partial clinical hold. In this case, the IND sponsor and the FDA must resolve any outstanding concerns before clinical trials can begin.

Following commencement of a clinical trial under an IND, the FDA may also place a clinical hold or partial clinical hold on that trial. A clinical hold is an order issued by the FDA to the sponsor to delay a proposed clinical investigation or to suspend an ongoing investigation. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND. For example, a specific protocol or part of a protocol is not allowed to proceed, while other protocols may do so. No more than 30 days after imposition of a clinical hold or partial clinical hold, the FDA will provide the sponsor a written explanation of the basis for the hold. Following issuance of a clinical hold or partial clinical hold, an investigation may only resume after the FDA has notified the sponsor that the investigation may proceed. The FDA will base that determination on information provided by the sponsor correcting the deficiencies previously cited or otherwise satisfying the FDA that the investigation can proceed.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all FDA IND requirements must be met unless waived. When a foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee, or DSMB. This group provides recommendations as to whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Suspension or termination of development during any phase of clinical trials can occur if it is determined that the participants or patients are being exposed to an unacceptable health risk.

Reporting Clinical Trial Results

Under the PHSA, sponsors of clinical trials of certain FDA-regulated products, including biologics, are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the U.S. National Institutes of Health, or NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. Although sponsors are also obligated to disclose the results of their clinical trials after completion,

disclosure of the results can be delayed in some cases for up to two years after the date of completion of the trial. The NIH's final rule on registration and reporting requirements for clinical trials became effective in 2017. With the issuance of pre-notices for voluntary corrective action and several notices of non-compliance during the past two years, the FDA has signaled the government's willingness to enforce these requirements against non-compliant clinical trial sponsors. While these notices of non-compliance did not result in civil monetary penalties, the failure to submit clinical trial information to clinicaltrials.gov, as required, is a prohibited act under the FDCA with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational new drug products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational drugs for patients who may benefit from investigational therapies. FDA regulations allow access to investigational drugs under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the drug under a treatment protocol or Treatment IND Application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its investigational products available for expanded access. Sponsors are required, however, to make their expanded access policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 study; or 15 days after the drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act, but the manufacturer must develop an internal policy and respond to patient requests according to that policy.

Human Clinical Trials in Support of a BLA

Clinical trials involve the administration of the investigational product candidate to human subjects under the supervision of a qualified investigator in accordance with GCP requirements which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written clinical trial protocols detailing, among other things, the objectives of the study, inclusion and exclusion criteria, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated.

Human clinical trials are typically conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may also be required after approval.

Phase 1 clinical trials are initially conducted in a limited population to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics in healthy humans or in patients. During Phase 1 clinical trials, information about the investigational product's

pharmacokinetics and pharmacological effects may be obtained to permit the design of well-controlled and scientifically valid Phase 2 clinical trials.

Phase 2 clinical trials are generally conducted in a limited patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials. Phase 2 clinical trials are well controlled, closely monitored and conducted in a limited patient population.

Phase 3 clinical trials proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are undertaken within an expanded patient population to further evaluate dosage, provide substantial evidence of clinical efficacy and further test for safety in an expanded and diverse patient population at multiple, geographically dispersed clinical trial sites. A well-controlled, statistically robust Phase 3 clinical trial may be designed to deliver the data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a drug or biologic: such Phase 3 studies are referred to as "pivotal." A company's designation of the phase of a trial is not necessarily indicative that the trial will be sufficient to satisfy the FDA requirements of that phase.

In some cases, the FDA may approve a BLA for a product candidate but require the sponsor to conduct additional clinical trials to further assess the product candidate's safety and effectiveness after approval. Such post-approval trials are typically referred to as Phase 4 clinical trials. These studies are used to gain additional experience from the treatment of a larger number of patients in the intended treatment group and to further document a clinical benefit in the case of products approved under accelerated approval regulations. Failure to exhibit due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

In December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, actions plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In addition to these requirements, the legislation directs the FDA to issue new guidance on diversity action plans.

In June 2023, the FDA issued draft guidance with updated recommendations for GCPs aimed at modernizing the design and conduct of clinical trials. The updates are intended to help pave the way for more efficient clinical trials to facilitate the development of medical products. The draft guidance is adopted from the International Council for Harmonisation's recently updated E6(R3) draft guideline that was developed to enable the incorporation of rapidly developing technological and methodological innovations into the clinical trial enterprise. In addition, the FDA issued draft guidance outlining recommendations for the implementation of decentralized clinical trials.

Interactions with FDA During the Clinical Development Program

Following the clearance of an IND and the commencement of clinical trials, the sponsor will continue to have interactions with the FDA. Progress reports detailing the results of clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the product; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or completed at all. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

In addition, sponsors are given opportunities to meet with the FDA at certain points in the clinical development program. There are five types of meetings that occur between sponsors and the FDA. Type A meetings are those that are necessary for an otherwise stalled product development program to proceed or to address an important safety issue. Type B meetings include meetings prior to the submission of an IND and meetings prior to the submission of a BLA, as well as end of phase meetings such as end of Phase 2 meetings. A Type C meeting is any meeting other than a Type A or

Type B meeting regarding the development and review of a product, including for example, meetings to facilitate early consultations on the use of a biomarker as a new surrogate endpoint that has never been previously used as the primary basis for product approval in the proposed context of use. A type D meeting is focused on a narrow set of issues (typically limited to no more than two focused topics) and should not require input from more than three disciplines or divisions. Finally, INTERACT meetings are intended for novel products and development programs that present unique challenges in the early development of an investigational product.

These meetings provide an opportunity for the sponsor to share information about the data gathered to date with the FDA and for the FDA to provide advice on the next phase of development. Such meetings may be conducted in person, via teleconference/videoconference or written response only with minutes reflecting the questions that the sponsor posed to the FDA and the FDA's responses. The FDA has indicated that its responses, as conveyed in meeting minutes and advice letters, only constitute mere recommendations and/or advice made to a sponsor and, as such, sponsors are not bound by such recommendations and/or advice. Nonetheless, from a practical perspective, a sponsor's failure to follow the FDA's recommendations for design of a clinical program may put the program at significant risk of failure. In September 2023, the FDA issued draft guidance outlining the terms of such meetings in more detail.

Clinical Trials Outside the United States in Support of FDA Approval

In connection with our clinical development program, we may have trial sites outside the United States. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. Specifically, the studies must be conducted in accordance with GCP, including undergoing review and receiving approval by an independent ethics committee and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies are conducted in a manner comparable to that required for IND studies.

The acceptance by the FDA of study data from clinical trials conducted outside the United States in support of U.S. approval may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

Where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted.

Manufacturing and Other Regulatory Requirements

Concurrent with clinical trials, sponsors usually complete additional animal safety studies, develop additional information about the chemistry and physical characteristics of the product candidate and finalize a process for manufacturing commercial quantities of the product candidate in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other criteria, the sponsor must develop methods for testing the identity, strength, quality, and purity of the finished product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

Specifically, the FDA's regulations require that pharmaceutical products be manufactured in specific approved facilities and in accordance with cGMPs. The cGMP regulations include requirements relating to organization of

personnel, buildings and facilities, equipment, control of components and product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports and returned or salvaged products. Manufacturers and other entities involved in the manufacture and distribution of approved pharmaceuticals are required to register their establishments with the FDA and some state agencies. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation, compounding or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States.

Manufacturing facilities are subject to periodic unannounced inspections by the FDA for compliance with cGMPs and other requirements. Inspections must follow a "risk-based schedule" that may result in certain establishments being inspected more frequently. Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated. Changes to the manufacturing process, specifications or container closure system for an approved product are strictly regulated and often require prior FDA approval before being implemented. The FDA's regulations also require, among other things, the investigation and correction of any deviations from cGMP and the imposition of reporting and documentation requirements upon the sponsor and any third-party manufacturers involved in producing the approved product.

Pediatric Studies

Under the Pediatric Research Equity Act of 2003, or PREA, an application or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the sponsor plans to conduct, including study objectives and design, any deferral or waiver requests and other information required by regulation. The sponsor, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The law requires the FDA to send a PREA Non-Compliance letter to sponsors who have failed to submit their pediatric assessments required under PREA, have failed to seek or obtain a deferral or deferral extension or have failed to request approval for a required pediatric formulation. It further requires the FDA to publicly post the PREA Non-Compliance letter and sponsor's response.

Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation, although FDA has taken steps to limit what it considers abuse of this statutory exemption in PREA by announcing that it does not intend to grant any additional orphan drug designations for rare pediatric subpopulations of what is otherwise a common disease. The FDA also maintains a list of diseases that are exempt from PREA requirements due to low prevalence of disease in the pediatric population. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under the PREA.

Expedited Review Programs

The FDA is authorized to expedite the review of applications in several ways. None of these expedited programs, however, changes the standards for approval but they may help expedite the development or approval process of product candidates.

- *Fast Track designation*: Product candidates are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to

have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track application before the application is complete, a process known as rolling review.

- *Breakthrough therapy designation.* To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.

- *Priority review.* A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.

- *Accelerated approval.* Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

  With the passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed); and use expedited procedures to withdraw accelerated approval of a BLA after the confirmatory trial fails to verify the product's clinical benefit. Further, FDORA requires the FDA to publish on its website the rationale for why a post-approval study is not appropriate or necessary, whenever it decides not to require such a study upon granting accelerated approval. In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval.

- *Regenerative advanced therapy.* With passage of the 21st Century Cures Act, in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

Acceptance and Review of BLAs

Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, along with information relating to the product's chemistry, manufacturing, controls, safety updates, patent information, abuse information and proposed labeling, are submitted to the FDA as part of an application requesting approval to market the product candidate for one or more indications. Data may come from company-sponsored clinical trials or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety, potency and purity of the biological product to the satisfaction of the FDA.

The fee required for the submission and review of an application under the Prescription Drug User Fee Act, or PDUFA, is substantial (for example, for fiscal year 2024 this application fee is approximately $4.05 million), and the sponsor of an approved application is also subject to an annual program fee, currently $416,734 per eligible prescription product. These fees are typically adjusted annually, and exemptions and waivers may be available under certain circumstances, such as where a waiver is necessary to protect the public health, where the fee would present a significant barrier to innovation, or where the sponsor is a small business submitting its first human therapeutic application for review.

The FDA conducts a preliminary review of all applications within 60 calendar days of receipt and must inform the sponsor at that time or before whether an application is sufficiently complete to permit substantive review. In pertinent part, FDA's regulations state that an application "shall not be considered as filed until all pertinent information and data have been received" by the FDA. In the event that FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. Typically, an RTF will be based on administrative incompleteness, such as clear omission of information or sections of required information; scientific incompleteness, such as omission of critical data, information or analyses needed to evaluate safety and efficacy or provide adequate directions for use; or inadequate content, presentation, or organization of information such that substantive and meaningful review is precluded. The FDA may request additional information rather than accept an application for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing.

After the submission is accepted for filing, the FDA begins an in-depth substantive review of the application. The FDA reviews the application to determine, among other things, whether the proposed product is safe and effective for its intended use, whether it has an acceptable purity profile and whether the product is being manufactured in accordance with cGMP. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has 10 months from the filing date in which to complete its initial review of a standard application that is a new molecular entity, and six months from the filing date for an application with "priority review". The review process may be extended by the FDA for three additional months to consider new information or in the case of a clarification provided by the sponsor to address an outstanding deficiency identified by the FDA following the original submission. Despite these review goals, it is not uncommon for FDA review of an application to extend beyond the PDUFA goal date.

In connection with its review of an application, the FDA will typically submit information requests to the sponsor and set deadlines for responses thereto. The FDA will also conduct a pre-approval inspection of the manufacturing facilities for the new product to determine whether the manufacturing processes and facilities comply with cGMPs. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications.

The FDA also may inspect the sponsor and one or more clinical trial sites to assure compliance with IND and GCP requirements and the integrity of the clinical data submitted to the FDA. With passage of FDORA, Congress clarified FDA's authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to FDA as well as other persons holding study records or involved in the study process. To ensure cGMP and GCP compliance by its employees and third-party contractors, a sponsor may incur significant expenditure of time, money and effort in the areas of training, record keeping, production and quality control.

Additionally, the FDA may refer an application, including applications for novel product candidates which present difficult questions of safety or efficacy, to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations when making final decisions on approval. Data from clinical trials are not always conclusive, and the FDA or its advisory committee may interpret data differently than the sponsor interprets the same data. The FDA may also re-analyze the clinical trial data, which could result in extensive discussions between the FDA and the sponsor during the review process.

The FDA also may require submission of a REMS, if it determines that a REMS is necessary to ensure that the benefits of the product outweigh its risks and to assure the safe use of the product. The REMS could include medication guides, physician communication plans, assessment plans and/or elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools. The FDA determines the requirement for a REMS, as well as the specific REMS provisions, on a case-by-case basis. If the FDA concludes a REMS is needed, the sponsor of the application must submit a proposed REMS and the FDA will not approve the application without a REMS.

Decisions on BLAs

On the basis of the FDA's evaluation of the application and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter, or CRL. To reach a determination of approval, the FDA must determine that the biologic is safe, pure and potent and that its expected benefits outweigh its potential risks to patients. This "benefit-risk" assessment is informed by the extensive body of evidence about the product in the BLA. This assessment is also informed by other factors, including: the severity of the underlying condition and how well patients' medical needs are addressed by currently available therapies; uncertainty about how the premarket clinical trial evidence will extrapolate to real-world use of the product in the post-market setting; and whether risk management tools are necessary to manage specific risks. In connection with this assessment, the FDA review team will assemble all individual reviews and other documents into an "action package," which becomes the record for FDA review. The review team then issues a recommendation and a senior FDA official makes a decision.

With respect to the evidentiary standard for making this determination, the FDA typically requires a robust safety database and two adequate and well-controlled clinical investigations to establish effectiveness of a new product. Under certain circumstances, however, the FDA has indicated that a single trial with certain characteristics and additional information may satisfy this standard. This approach was subsequently endorsed by Congress in 1998 with legislation providing, in pertinent part, that "If [FDA] determines, based on relevant science, that data from one adequate and well-controlled clinical investigation and confirmatory evidence (obtained prior to or after such investigation) are sufficient to establish effectiveness, the FDA may consider such data and evidence to constitute substantial evidence." This modification to the law recognized the potential for the FDA to find that one adequate and well controlled clinical investigation with confirmatory evidence, including supportive data outside of a controlled trial, is sufficient to establish effectiveness. In December 2019, the FDA issued draft guidance further explaining the studies that are needed to establish substantial evidence of effectiveness. The FDA has not yet finalized that guidance, but it did issue draft guidance in September 2023 that outlines considerations for relying on confirmatory evidence in lieu of a second clinical trial to demonstrate efficacy.

A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The CRL may require additional clinical or other data, additional pivotal Phase 3 clinical trial(s) and/or other significant and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. If a CRL is issued, the sponsor will have one year to respond to the deficiencies identified by the FDA, at which time the FDA can deem the application withdrawn or, in its discretion, grant the sponsor an additional six month extension to respond. The FDA has committed to reviewing resubmissions in response to an issued CRL in either two or six months depending on the type of information included. Even with the submission of this additional information, however, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. The FDA has taken the position that a CRL is not final agency action making the determination subject to judicial review.

An approval letter, on the other hand, authorizes commercial marketing of the product with specific prescribing information for specific indications. That is, the approval will be limited to the conditions of use (e.g., patient population, indication) described in the FDA-approved labeling. Further, depending on the specific risk(s) to be addressed, the FDA may require that contraindications, warnings or precautions be included in the product labeling, require that post-approval trials, including Phase 4 clinical trials, be conducted to further assess a product's safety after approval, require testing and surveillance programs to monitor the product after commercialization or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a REMS which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing trials or surveillance programs. After approval, some types of changes

to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Under the Ensuring Innovation Act, which was signed into law in April 2021, the FDA must publish action packages summarizing its decisions to approve new biologics within 30 days of approval of such products. To date, CRLs are not publicly available documents.

Post-Approval Regulation

If regulatory approval for marketing of a new product or new indication for an existing product is obtained, the sponsor will be required to comply with all regular post-approval regulatory requirements as well as any post-approval requirements that the FDA may have imposed as part of the approval process. The sponsor will be required to report, among other things, certain adverse reactions and manufacturing problems to the FDA, provide updated safety and efficacy information and comply with requirements concerning advertising and promotional labeling requirements. Manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMP regulations, which impose certain procedural and documentation requirements upon manufacturers. Accordingly, the sponsor and its third-party manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMP regulations and other regulatory requirements.

A product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release, the manufacturer must submit samples of each lot, together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot, to the FDA. The FDA may in addition perform certain confirmatory tests on lots of some products before releasing the lots for distribution. Finally, the FDA will conduct laboratory research related to the safety, purity, potency and effectiveness of pharmaceutical products.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription products placed on the market. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet and social media. Promotional claims about a product's safety or effectiveness are prohibited before the product is approved. After approval, a product generally may not be promoted for uses that are not approved by the FDA, as reflected in the product's prescribing information. In September 2021, the FDA published final regulations which describe the types of evidence that the FDA will consider in determining the intended use of a biologic. In addition, in October 2023, the FDA published draft guidance outlining the FDA's non-

binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use.

It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information, such as distributing scientific or medical journal information. Further, with passage of the Pre-Approval Information Exchange Act in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products.

If a company is found to have promoted off-label uses, it may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the Department of Justice, or the DOJ, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion, and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, and its implementing regulations, as well as the Drug Supply Chain Security Act, or DSCA, which regulate the distribution and tracing of prescription drug samples at the federal level, and set minimum standards for the regulation of distributors by the states. The PDMA, its implementing regulations and state laws limit the distribution of prescription pharmaceutical product samples, and the DSCA imposes requirements to ensure accountability in distribution and to identify and remove counterfeit and other illegitimate products from the market.

Regulatory Exclusivity Governing Biologics

When a biological product is licensed for marketing by FDA with approval of a BLA, the product may be entitled to certain types of market and data exclusivity barring FDA from approving competing products for certain periods of time. In March 2010, the Patient Protection and Affordable Care Act was enacted in the United States and included the Biologics Price Competition and Innovation Act of 2009, or the BPCIA. The BPCIA amended the PHSA to create an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. To date, the FDA has approved a number of biosimilar products and interchangeable biosimilar products.

Under the BPCIA, a manufacturer may submit an application for a product that is "biosimilar to" a previously approved biological product, which the statute refers to as a "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and the proposed biosimilar product in terms of safety, purity and potency. The biosimilar sponsor may demonstrate that its product is biosimilar to the reference product on the basis of data from analytical studies, animal studies and one or more clinical studies to demonstrate safety, purity and potency in one or more appropriate conditions of use for which the reference product is approved. In addition, the sponsor must show that the biosimilar and reference products have the same mechanism of action for the conditions of use on the label, route of administration, dosage and strength, and the production facility must meet standards designed to assure product safety, purity and potency.

For the FDA to approve a biosimilar product as interchangeable with a reference product, the FDA must find not only that the product is biosimilar to the reference product but also that it can be expected to produce the same clinical results as the reference product such that the two products may be switched without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. Upon licensure by the FDA, an interchangeable biosimilar may be substituted for the reference product without the intervention of the health care provider who prescribed the reference product. Following approval of the interchangeable biosimilar product, the FDA

may not grant interchangeability status for any second biosimilar until one year after the first commercial marketing of the first interchangeable biosimilar product. In December 2022, Congress clarified through FDORA that FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

A reference biological product is granted 12 years of exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. Even if a product is considered to be a reference product eligible for exclusivity, however, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of their product. There have been recent government proposals to reduce the 12-year reference product exclusivity period, but none has been enacted to date. At the same time, since passage of the BPCIA, many states have passed laws or amendments to laws, which address pharmacy practices involving biosimilar products.

Pediatric Exclusivity

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of exclusivity. For biologic products, the six month period may be attached to any existing regulatory exclusivities but not to any patent terms. The conditions for pediatric exclusivity include the FDA's determination that information relating to the use of a new product in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric clinical trials, and the sponsor agreeing to perform, and reporting on, the requested clinical trials within the statutory timeframe. This six-month exclusivity may be granted if a sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patents that cover the product are extended by six months. Although this is not a patent term extension, it effectively extends the regulatory period during which the FDA cannot approve another application.

Orphan Drug Designation and Exclusivity

Orphan drug designation in the United States is designed to encourage sponsors to develop products intended for treatment of rare diseases or conditions. In the United States, a rare disease or condition is statutorily defined as a condition that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available the product for the disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation qualifies a company for tax credits and potentially market exclusivity for seven years following the date of the product's approval if granted by the FDA. An application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. A product becomes an orphan when it receives orphan drug designation from the Office of Orphan Products Development at the FDA based on acceptable confidential requests. The product must then go through the review and approval process like any other product.

A sponsor may request orphan drug designation of a previously unapproved product or new orphan indication for an already marketed product. In addition, a sponsor of a product that is otherwise the same product as an already approved orphan drug may seek and obtain orphan drug designation for the subsequent product for the same rare disease or condition if it can present a plausible hypothesis that its product may be clinically superior to the first approved product. More than one sponsor may receive orphan drug designation for the same product for the same rare disease or condition, but each sponsor seeking orphan drug designation must file a complete request for designation.

If a product with orphan designation receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan drug exclusivity. Orphan drug exclusivity means that the FDA may not approve

another sponsor's marketing application for the same product for the same disease or condition for seven years, except in certain limited circumstances. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity.

The period of market exclusivity begins on the date that the marketing application is approved by the FDA and applies only to the disease or condition for which the product has been designated. Orphan drug exclusivity will not bar approval of another product under certain circumstances, including if the company with orphan drug exclusivity is not able to meet market demand or the subsequent product is shown to be clinically superior to the approved product on the basis of greater efficacy or safety, or providing a major contribution to patient care. This is the case despite an earlier court opinion holding that the Orphan Drug Act unambiguously required the FDA to recognize orphan drug exclusivity regardless of a showing of clinical superiority. Under Omnibus legislation passed in December 2020, the requirement for a product to show clinical superiority applies to drug products that received orphan drug designation before enactment of amendments to the FDCA in 2017 but have not yet been approved by FDA.

In September 2021, the Court of Appeals for the 11th Circuit held that, for the purpose of determining the scope of market exclusivity, the term "same disease or condition" in the statute means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, FDA announced that, in matters beyond the scope of that court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved.

Patent Term Restoration and Extension

In the United States, a patent claiming a new product, its method of use or its method of manufacture may be eligible for a limited patent term extension under the Hatch-Waxman Act, which permits a patent extension of up to five years for patent term lost during product development and FDA regulatory review. Assuming grant of the patent for which the extension is sought, the restoration period for a patent covering a product is typically one-half the time between the effective date of the IND involving human beings is begun and the submission date of the BLA, plus the time between the submission date of the application and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date in the United States. Only one patent applicable to an approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. The U.S. Patent and Trademark Office reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

Companion Diagnostics

In August 2014, the FDA issued final guidance clarifying the requirements that will apply to approval of therapeutic products and in vitro companion diagnostics. According to the guidance, for novel biologics, a companion diagnostic device and its corresponding therapeutic should be approved or cleared contemporaneously by the FDA for the use indicated in the therapeutic product's labeling.

Approval or clearance of the companion diagnostic device will ensure that the device has been adequately evaluated and has adequate performance characteristics in the intended population. In July 2016, the FDA issued a draft guidance intended to assist sponsors of the therapeutic product and *in vitro* companion diagnostic device on issues related to co-development of the products.

The 2014 guidance also explains that a companion diagnostic device used to make treatment decisions in clinical trials of a biologic product candidate generally will be considered an investigational device, unless it is employed for an intended use for which the device is already approved or cleared. If used to make critical treatment decisions, such as patient selection, the diagnostic device generally will be considered a significant risk device under the FDA's Investigational Device Exemption, or IDE, regulations. Thus, the sponsor of the diagnostic device will be required to comply with the IDE regulations. According to the guidance, if a diagnostic device and a product are to be studied together to support their respective approvals, both products can be studied in the same investigational study, if

the study meets both the requirements of the IDE regulations and the IND regulations. The guidance provides that depending on the details of the study plan and subjects, a sponsor may seek to submit an IND application alone, or both an IND and IDE application.

In April 2020, the FDA issued additional guidance which describes considerations for the development and labeling of companion diagnostic devices to support the indicated uses of multiple drug or biological oncology products, when appropriate. This guidance builds upon existing policy regarding the labeling of companion diagnostics. In its 2014 guidance, the FDA stated that if evidence is sufficient to conclude that the companion diagnostic is appropriate for use with a specific group of therapeutic products, the companion diagnostic's intended use/indications for use should name the specific group of therapeutic products, rather than specific products. The 2020 guidance expands on the policy statement in the 2014 guidance by recommending that companion diagnostic developers consider a number of factors when determining whether their test could be developed, or the labeling for approved companion diagnostics could be revised through a supplement, to support a broader labeling claim such as use with a specific group of oncology therapeutic products (rather than listing an individual therapeutic product(s)). Under the FDCA, in vitro diagnostics, including companion diagnostics, are regulated as medical devices. In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import, and post-market surveillance. Unless an exemption applies, diagnostic tests require pre-notification marketing clearance or approval from the FDA prior to commercial distribution.

The FDA previously has required in vitro companion diagnostics intended to select the patients who will respond to the product candidate to obtain pre-market approval, or PMA, simultaneously with approval of the therapeutic product candidate. The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, can take several years or longer. It involves a rigorous premarket review during which the sponsor must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA applications are subject to an application fee.

After a device is placed on the market, it remains subject to significant regulatory requirements. Medical devices may be marketed only for the uses and indications for which they are cleared or approved. Device manufacturers must also establish registration and device listings with the FDA. A medical device manufacturer's manufacturing processes and those of its suppliers are required to comply with the applicable portions of the Quality System Regulation, which covers the methods and documentation of the design, testing, production, processes, controls, quality assurance, labeling, packaging, and shipping of medical devices. Domestic facility records and manufacturing processes are subject to periodic unscheduled inspections by the FDA. The FDA also may inspect foreign facilities that export products to the United States.

Healthcare Law and Regulation

Health care providers and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, patient privacy laws and regulations and other health care laws and regulations that may constrain business and/or financial arrangements. Restrictions under applicable federal and state health care laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal health care program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or

fraudulent or knowingly making, using or causing to made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government.

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any health care benefit program or making false statements relating to health care matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing ·or covering up a material fact or making any materially false statement in connection with the delivery of or payment for health care benefits, items or services;
- the Foreign Corrupt Practices Act, or FCPA, which prohibits companies and their intermediaries from making, or offering or promising to make improper payments to non-U.S. officials for the purpose of obtaining or retaining business or otherwise seeking favorable treatment;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or the Affordable Care Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the United States Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians, other healthcare providers and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to health care items or services that are reimbursed by non-government third-party payors, including private insurers.

Further, some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. Additionally, some state and local laws require the registration of pharmaceutical sales representatives in the jurisdiction. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Healthcare Reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of drug and biologic products, limiting coverage and reimbursement for medical products and other changes to the healthcare system in the United States.

In March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, which, among other things, includes changes to the coverage and payment for pharmaceutical products under government healthcare programs. Other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to

2% per fiscal year, which went into effect in April 2013 and will remain in effect through the first half of 2032. Under current legislation, the actual reductions in Medicare payments may vary up to 4%.

The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010 sequester for two years, through the end of 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act's health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into 2032 and lowers the payment reduction percentages in years 2030 and 2031.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed into law on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. In June 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden rescinded those orders and issued a new executive order that directs federal agencies to reconsider rules and other policies that limit access to healthcare, and consider actions that will protect and strengthen that access. Under this order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and under the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

Pharmaceutical Prices

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In addition, in October 2020, the Department of Human and Health Services, or HHS, and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states (Colorado, Florida, Maine, New Hampshire, New Mexico, North Dakota, Texas, Vermont and Wisconsin) have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. On January 5, 2024, the FDA approved Florida’s plan for Canadian drug importation.

Further, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would also eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1, 2022, but with passage of the Inflation Reduction Act of 2022, or IRA, has been delayed by Congress to January 1, 2032.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028 and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated “maximum fair price” under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

The IRA includes a provision exempting orphan drugs from Medicare price negotiation but this exclusion has been interpreted by CMS in final guidance issued in July 2023 to apply only to those orphan drugs with an approved indication (or indications) for a single rare disease or condition. The final guidance clarifies that CMS will consider only

active designations/approvals when evaluating a drug for the exclusion, such that designations/indications withdrawn before the selected drug publication date will not be considered. CMS also clarified that, if a drug loses its orphan drug exclusion status, the agency will use the earliest date of approval/licensure to determine whether the product is a qualifying single source drug subject to price negotiations.

In June 2023, Merck & Co. filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including the U.S. Chamber of Commerce and pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. A number of states, for example, require drug manufacturers and other entities in the drug supply chain, including health carriers, pharmacy benefit managers, wholesale distributors, to disclose information about pricing of pharmaceuticals. In addition, regional healthcare organizations and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription pharmaceutical and other healthcare programs.

Federal and State Data Privacy Laws

There are multiple privacy and data security laws that may impact our business activities, in the United States and in other countries where we conduct trials or where we may do business in the future. These laws are evolving and may increase both our obligations and our regulatory risks in the future. In the health care industry generally, under HIPAA, HHS has issued regulations to protect the privacy and security of protected health information used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. HIPAA also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. HIPAA may apply to us in certain circumstances and may also apply to our business partners in ways that may impact our relationships with them. Our clinical trials are regulated by the Common Rule, which also includes specific privacy-related provisions. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that may be applicable to our business. In addition to possible federal civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state attorneys general (along with private plaintiffs) have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. State attorneys general also have authority to enforce state privacy and security laws. New laws and regulations governing privacy and security may be adopted in the future as well.

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, in Europe, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the

California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities. In addition to California, a number of other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data (which includes health data in some cases). Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering privacy laws that will go into effect in 2025 and beyond. Other states will be considering these laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, Washington state passed a health privacy law in 2023 that will regulate the collection and sharing of health information, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data, and more states are considering such legislation in 2024. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our product candidates, if approved.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil and administrative penalties, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Review and Approval of Medical Products in the European Union

In order to market any product outside of the United States, a sponsor must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, a sponsor will need to obtain the necessary approvals by the comparable non-U.S. regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The process governing approval of medicinal products in the European Union, or EU, generally follows the same lines as in the United States. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the EU.

Clinical Trial Approval

On January 31, 2022, the new Clinical Trials Regulation (EU) No 536/2014, or the Clinical Trials Regulation, became effective in the European Union and replaced the prior Clinical Trials Directive 2001/20/EC, or the Clinical Trials Directive. The new regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the European Union. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one Member State of the European Union, or EU Member State, will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a new clinical trials portal overseen by the European Medicines Agency, or EMA, and available to clinical trial sponsors, competent authorities of the EU Member States and the public.

All ongoing clinical trials in the European Union approved under the prior Clinical Trials Directive must be transitioned to the Clinical Trials Information System by January 31, 2025. This date marks the end of a three-year

transition period that began when the Clinical Trials Regulation became applicable in the European Union on January 31, 2022. Clinical trials that were started under the Clinical Trials Directive and subject to transition to the Clinical Trials Regulation will, by January 31, 2025, have to comply with the obligations of the Clinical Trials Regulation even if these are not included in the previous study protocol, such as (i) obligations of notification via the Clinical Trials Information System; (ii) safety reporting rules; (iii) archiving requirement; and (iv) transparency requirements. The failure to transition ongoing clinical trials to the Clinical Trials Regulation by January 31, 2025 can result in corrective measures under Article 77 of the Clinical Trials Regulation, including revocation of the authorization of the clinical trial or suspension of the clinical trial as well as criminal sanctions and fines under national law of EU Member States.

Beyond streamlining the process, the Clinical Trials Regulation includes a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (EU Member States concerned). Part II is assessed separately by each EU Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the Clinical Trials Regulation.

The Clinical Trials Regulation did not change the preexisting requirement that a sponsor must obtain prior approval from the competent national authority of the EU Member State in which the clinical trial is to be conducted. If the clinical trial is conducted in different EU Member States, the competent authorities in each of these EU Member States must provide their approval for the conduct of the clinical trial. Furthermore, the sponsor may only start a clinical trial at a specific study site after the applicable ethics committee has issued a favorable opinion.

Parties conducting certain clinical trials must, as in the United States, post clinical trial information in the EU at the EU Clinical Trials Register.

PRIME Designation in the EU

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises, or SMEs, may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, a dedicated Agency contact and rapporteur from the Committee for Medicinal Products for Human Use, or CHMP, or Committee for Advanced Therapies are appointed early in PRIME scheme facilitating increased understanding of the product at EMA's Committee level.

Pediatric Studies

Companies developing a new medicinal product must agree upon a Pediatric Investigation Plan, or PIP, with the EMA's pediatric committee, or PDCO, and must conduct pediatric clinical trials in accordance with that PIP, unless a waiver applies (e.g., because the relevant disease or condition occurs only in adults). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted by the PDCO of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults, in which case the pediatric clinical trials must be completed at a later date.

Marketing Authorization

Marketing authorization applications, or MAAs, can be filed either under the so-called centralized or national authorization procedures, albeit through the Mutual Recognition or Decentralized procedure for a product to be authorized in more than one EU member state.

The centralized procedure provides for the grant of a single marketing authorization following a favorable opinion by the EMA that is valid in all EU Member States, as well as Iceland, Liechtenstein and Norway, which are part of the European Economic Area, or EEA. The centralized procedure is compulsory for medicines produced by specified biotechnological processes, products designated as orphan medicinal products, advanced-therapy medicines (such as gene-therapy, somatic cell-therapy or tissue-engineered medicines) and products with a new active substance indicated for the treatment of specified diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions and viral diseases. The centralized procedure is optional for products that represent a significant therapeutic, scientific or technical innovation, or whose authorization would be in the interest of public health. Under the centralized procedure, the maximum timeframe for the evaluation of an MAA by the EMA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the sponsor in response to questions asked by the CHMP. Accelerated assessment might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. The timeframe for the evaluation of an MAA under the accelerated assessment procedure is 150 days, excluding stop-clocks.

There are also two other possible routes to authorize medicinal products in several EU countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:

- *Decentralized procedure*. Using the decentralized procedure, a sponsor may apply for simultaneous authorization in more than one EU country of medicinal products that have not yet been authorized in any EU country and that do not fall within the mandatory scope of the centralized procedure. The sponsor may choose a member state as the reference member State to lead the scientific evaluation of the application.

- *Mutual recognition procedure*. In the mutual recognition procedure, a medicine is first authorized in one EU Member State (which acts as the reference member state), in accordance with the national procedures of that country. Following this, further marketing authorizations can be progressively sought from other EU countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization produced by the reference member state.

Under the above-described procedures, before granting the marketing authorization, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

Conditional Approval

In particular circumstances, E.U. legislation (Article 14–a Regulation (EC) No 726/2004 (as amended by Regulation (EU) 2019/5 and Regulation (EC) No 507/2006 on Conditional Marketing Authorizations for Medicinal Products for Human Use) enables sponsors to obtain a "conditional marketing authorization" prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for product candidates (including medicines designated as orphan medicinal products), if (1) the product candidate is intended for the treatment, prevention or medical diagnosis of seriously debilitating or life-threatening disease; (2) the product candidate is intended to meet unmet medical needs of the patients; (3) a marketing authorization may be granted prior to submission of comprehensive clinical data provided that the benefit of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required; (4) the risk-benefit balance of the product candidate is positive, and (5) it is likely that the sponsor will be in a position to provide the required comprehensive clinical trial data. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data. Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions or specific obligations. The timelines

for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization, but sponsors can also request EMA to conduct an accelerated assessment, for instance, in cases of unmet medical needs.

Periods of Authorization and Renewals

A marketing authorization has an initial validity for five years in principle. The marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the EU Member State. To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. The European Commission or the competent authorities of the EU Member States may decide, on justified grounds relating to pharmacovigilance, to proceed with one further five-year period of marketing authorization. Once subsequently definitively renewed, the marketing authorization shall be valid for an unlimited period. Any authorization which is not followed by the actual placing of the medicinal product on the EU n market (in case of centralized procedure) or on the market of the authorizing EU Member State within three years after authorization ceases to be valid (the so-called sunset clause).

Regulatory Requirements after a Marketing Authorization has been Obtained

In case an authorization for a medicinal product in the EU is obtained, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include:

- Compliance with the EU's stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.
- The manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with EU cGMP standards when manufacturing medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU.
- The marketing and promotion of authorized drugs, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the EU notably under Directive 2001/83EC, as amended, and EU Member State laws. Direct-to-consumer advertising of prescription medicines is prohibited across the EU.

Regulatory Data Protection in the EU

In the EU, innovative medicinal products approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Directive 2001/83/EC. Regulation (EC) No 726/2004 repeats this entitlement for medicinal products authorized in accordance the centralized authorization procedure. Data exclusivity prevents sponsors for authorization of generics of these innovative products from referencing the innovator's data to assess a generic (abridged) application for a period of eight years. During an additional two-year period of market exclusivity, a generic marketing authorization application can be submitted and authorized, and the innovator's data may be referenced, but no generic medicinal product can be placed on the EU market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company obtained marketing

authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Pediatric Exclusivity

If a sponsor obtains a marketing authorization in all EU Member States, or a marketing authorization granted in the centralized procedure by the European Commission, and the study results for the pediatric population are included in the product information, even when negative, the medicine is then eligible for an additional six-month period of qualifying patent protection through extension of the term of the Supplementary Protection Certificate, or SPC, or alternatively a one year extension of the regulatory market exclusivity from ten to eleven years, as selected by the marketing authorization holder.

Orphan Drug Designation and Exclusivity

Regulation (EC) No. 141/2000, as implemented by Regulation (EC) No. 847/2000 provides that a drug can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the EU when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives it is unlikely that the marketing of the drug in the EU would generate sufficient return to justify the necessary investment. For either of these conditions, the sponsor must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Once authorized, orphan medicinal products are entitled to 10 years of market exclusivity in all EU Member States and in addition a range of other benefits during the development and regulatory review process including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure covering all member countries and a reduction or elimination of registration and marketing authorization fees. However, marketing authorization may be granted to a similar medicinal product with the same orphan indication during the 10-year period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if it can be demonstrated on the basis of available evidence that the original orphan medicinal product is sufficiently profitable not to justify maintenance of market exclusivity.

Patent Term Extensions

The European Union also provides for patent term extension through Supplementary Protection Certificates, or SPCs. The rules and requirements for obtaining a SPC are similar to those in the United States. An SPC may extend the term of a patent for up to five years after its originally scheduled expiration date and can provide up to a maximum of fifteen years of marketing exclusivity for a drug. In certain circumstances, these periods may be extended for six additional months if pediatric exclusivity is obtained. Although SPCs are available throughout the European Union, sponsors must apply on a country-by-country basis. Similar patent term extension rights exist in certain other foreign jurisdictions outside the European Union.

Reimbursement and Pricing of Prescription Pharmaceuticals

In the EU, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, EU Member States have the option to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. EU Member States may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products, but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the EU have increased the amount of discounts required on pharmaceuticals and these efforts could

continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the EU. The downward pressure on health care costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced EU Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

General Data Protection Regulation

Many countries outside of the United States maintain rigorous laws governing the privacy and security of personal information. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, and it imposes heightened requirements on companies that process health and other sensitive data, such as requiring in many situations that a company obtain the consent of the individuals to whom the sensitive personal data relate before processing such data. Examples of obligations imposed by the GDPR on companies processing personal data that fall within the scope of the GDPR include providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, appointing a data protection officer, providing notification of data breaches, and taking certain measures when engaging third-party processors.

The GDPR also imposes strict rules on the transfer of personal data to countries outside the EEA, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR is a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or the Court of Justice of the European Union, or CJEU, invalidated the EU-U.S. Privacy Shield framework, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the United States. This CJEU decision may lead to increased scrutiny on data transfers from the EU to the United States generally and increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which would serve as a replacement to the EU-U.S. Privacy Shield. The EU initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the EU to the U.S. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business.

As with other issues related to withdrawal of the United Kingdom from the EU, there are open questions about how personal data will be protected in the United Kingdom and whether personal information can transfer from the EU to the United Kingdom. Following the withdrawal of the United Kingdom from the EU, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by the GDPR. While the Data Protection Act 2018 in the United Kingdom that "implements" and complements the GDPR has achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the EEA to the United Kingdom will remain lawful under the GDPR. The UK government has already determined that it considers all European Union and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the United Kingdom to the European Union/EEA remain unaffected. In addition, a recent decision from the European Commission appears to deem the United Kingdom as being "essentially adequate" for purposes of data transfer from the EU to the United Kingdom, although this decision may be re-evaluated in the future.

Beyond the GDPR, there are privacy and data security laws in a growing number of countries around the world, including Latin American countries where we plan to conduct clinical trials. While many loosely follow the GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and any eventual sale and distribution of commercial products.

Human Capital

Our ability to sustain and grow our business requires us to hire, retain and develop a highly skilled workforce. As of December 31, 2023, we had a total of 43 full time employees. We continually evaluate our business needs and opportunities and balance in-house expertise and capacity with outsourced expertise and capacity.

Recruiting, motivating and retaining qualified employees is critical to our success. We monitor our compensation programs and aim to provide our employees a competitive mix of cash compensation and medical insurance benefits, as well as the opportunity to participate in our equity programs. We believe that our philosophy of providing competitive compensation, along with opportunities for career growth and development, encourages a high level of corporate employee tenure and low level of voluntary turnover. A large majority of our employees have obtained advanced degrees in their professions. Our employees are supported with training and development opportunities to pursue their careers and to ensure compliance with our policies. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

We value the health, safety and wellbeing of our employees and their families. In response to the COVID-19 pandemic, we have implemented significant changes that we determined were in the best interest of our employees, as well as the communities in which we operate, and which comply with government regulations. This includes allowing our corporate employees to work remotely.

Our Corporate Information

We were incorporated under the laws of the State of Delaware in July 2009. Our website address is www.kalarx.com. The information on our website is not incorporated by reference into this Annual Report on Form 10-K and should not be considered to be a part of this Annual Report on Form 10-K. Our website address is included in this Annual Report on Form 10-K as an inactive technical reference only.

Available Information

Through our website, we make available free of charge our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, or the Exchange Act. We make these reports available through our website as soon as reasonably practicable after we electronically file such reports with, or furnish such reports to, the Securities and Exchange Commission, or the SEC. You can review our electronically filed reports and other information that we file with the SEC on the SEC's web site at http://www.sec.gov. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. In addition, we regularly use our website to post information regarding our business, product development programs and governance, and we encourage investors to use our website, particularly the information in the section entitled "Investors," as a source of information about us.

The information on our website is not incorporated by reference into this Annual Report on Form 10-K and should not be considered to be a part of this Annual Report on Form 10-K. Our website address is included in this Annual Report on Form 10-K as an inactive technical reference only.

Item 1A Risk Factors

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Annual Report on Form 10-K, including our financial statements and the related notes appearing at the end of this Annual Report on Form 10-K, before deciding to invest in our common stock. These risks, some of which have occurred and any of which may occur in the future, can have a material adverse effect on our business, prospects, operating results and financial condition. In such event, the trading price of our common stock could decline and you might lose all or part of your investment. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business, prospects, operating results and financial condition.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses from operations and negative cash flows from operations since our inception. We expect to incur additional losses and may never achieve or maintain profitability.

Since inception, we have incurred significant losses from operations and negative cash flows from operations. Our net losses were $42.2 million and $44.8 million for the years ended December 31, 2023 and 2022, respectively. As of December 31, 2023, we had an accumulated deficit of $629.4 million. Prior to the sale of the rights to manufacture, sell, distribute, market and commercialize EYSUVIS and INVELTYS and to develop, manufacture, market and otherwise exploit the AMPPLIFY Drug Delivery Technology, which we collectively refer to as the Commercial Business, to Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC, or collectively Alcon, in July 2022, we generated only limited revenues from sales of EYSUVIS and INVELTYS. We have financed our operations primarily through proceeds from the sale of our Commercial Business to Alcon in July 2022, our initial public offering, follow-on public offerings of common stock and sales under our at-the-market offering facilities, private placements of common stock and/or preferred stock, borrowings under credit facilities and the Loan and Security Agreement with Oxford Finance LLC, or the Loan Agreement, convertible promissory notes and warrants. Upon entry into the CIRM award in August 2023, Combangio received an initial $5.9 million disbursement from CIRM, and the balance of the $15.0 million award is payable to Combangio upon the achievement of specified milestones. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials, and prior to the sale of our Commercial Business to Alcon in July 2022, engaging in activities to launch and commercialize EYSUVIS and INVELTYS. As a result of the acquisition of Combangio in November 2021 and the sale of our Commercial Business to Alcon, we are devoting substantial financial resources to the research and development and potential commercialization of KPI-012, our product candidate in clinical development for the treatment of persistent corneal epithelial defects, or PCED, and any other indications we determine to pursue, including Limbal Stem Cell Deficiency. We have no revenue-generating commercial products, our cash flows have diminished as a result of the sale of our Commercial Business to Alcon and, as a result of our acquisition of Combangio, we may be required to pay certain milestones and royalty payments to former equityholders of Combangio. Although we are eligible to receive up to $325.0 million in payments from Alcon based upon the achievement of specified commercial sales-based milestones with respect to EYSUVIS and INVELTYS, there can be no assurance as to when we may receive such milestone payments or of the amount of milestone payments we may receive, if any. We also cannot assure you that we will achieve milestones within the timeframe required by the CIRM award, or at all, and as such we may never receive the remaining $9.1 million under the award. We expect to continue to incur significant expenses and operating losses for the foreseeable future, including in connection with our continued development, regulatory approval efforts and commercialization, if any, of KPI-012. We may never achieve or maintain profitability. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year.

We anticipate that our research and development expenses will increase substantially in the future as compared to prior periods as we advance the clinical development of KPI-012. Our research and development expenses will also increase in the future as we conduct any necessary preclinical studies and clinical trials and other development activities for any other product candidates we may develop in the future, including our planned preclinical studies under our KPI-014 program, which is a mesenchymal secretome formulation that is in preclinical development for the treatment of inherited retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease. If we obtain marketing approval for KPI-012 or any product candidates we may develop, we expect that our selling, general and administrative expenses will increase substantially if and as we incur commercialization expenses related to product marketing, sales and distribution.

Our expenses will also increase if and as we:

- continue the clinical development of KPI-012 for PCED;
- initiate and continue the research and development of KPI-012 for additional indications, such as Limbal Stem Cell Deficiency, including initiating and conducting preclinical studies and clinical trials;
- scale up our manufacturing processes and capabilities to manufacture the clinical supply of KPI-012;
- seek regulatory approval for KPI-012 for PCED in the United States and other jurisdictions;
- seek regulatory approval for KPI-012 for additional indications;
- grow our sales, marketing and distribution capabilities in connection with the commercialization of any product candidates for which we may submit for and obtain marketing approval;
- initiate and progress any preclinical development programs under our mesenchymal stem cell secretome, or MSC-S platform, including from our KPI-014 program;
- conduct clinical trials and other development activities and/or seek marketing approval for any product candidates we may develop in the future;
- in-license or acquire the rights to other products, product candidates or technologies;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, quality control, scientific, manufacturing, commercial and management personnel to support our operations;
- expand our operational, financial and management systems; and
- increase our product liability insurance coverage if we initiate commercialization efforts for our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Our expenses will increase from what we anticipate if:

- we elect or are required by the U.S. Food and Drug Administration, or FDA, or non-U.S. regulatory agencies to perform clinical trials or studies in addition to those expected;
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates;
- we in-license or acquire rights to other products, product candidates or technologies; or
- there are any third-party challenges to our intellectual property portfolio, or the need arises to defend against intellectual property-related claims or enforce our intellectual property rights.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate revenue from KPI-012 or any other product candidate we may develop for the foreseeable future, if at all. Achieving and maintaining profitability will require us to be successful in a range of challenging activities, including:

- completing the clinical development of KPI-012 for PCED and any other indications we determine to pursue, including Limbal Stem Cell Deficiency;
- subject to obtaining favorable results from our ongoing and planned clinical trials of KPI-012, applying for and obtaining marketing approval of KPI-012;
- successfully commercializing KPI-012, if approved;
- discovering, developing and successfully seeking marketing approval and commercialization of any additional product candidates we may develop in the future, including under our KPI-014 program;
- hiring and building a full commercial organization required for marketing, selling and distributing those products for which we obtain marketing approval;
- manufacturing at commercial scale, marketing, selling and distributing those products for which we obtain marketing approval;
- achieving an adequate level of market acceptance, and obtaining and maintaining coverage and adequate reimbursement from third-party payors for any products we commercialize; and
- obtaining, maintaining and protecting our intellectual property rights.

As a company, we have limited experience commercializing products, and we may not be able to commercialize a product successfully in the future. There are numerous examples of unsuccessful product launches and failures to meet expectations of market potential, including by pharmaceutical companies with more experience and resources than us.

We may never succeed in the foregoing activities and we may never generate revenue that is sufficient to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Our limited operating history and our limited experience in developing biologics may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to organizing and staffing our company, acquiring rights to intellectual property, business planning, raising capital, conducting research and development activities, and prior to the sale of our Commercial Business to Alcon in July 2022, developing and commercially launching EYSUVIS and INVELTYS. While we have had experience with obtaining marketing approval for and commercially launching two commercial products, we no longer have any commercial products following the sale of our Commercial Business to Alcon, we have only one product candidate in clinical development and we cannot be certain that we will be able to develop, obtain marketing approval for and commercialize a product in the future. If we are successful in developing and obtaining marketing approval for KPI-012 or any product candidate we may develop in the future, we will again have to transition from a company with a research and development focus to a company capable of supporting commercial activity. We may not be successful in such a transition. In addition, prior to our acquisition of KPI-012 in November 2021, we had no prior experience developing biological product candidates. As such, we may encounter delays or difficulties in our efforts to develop and commercialize KPI-012.

Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had prior experience developing biological product candidates or a longer operating and commercialization history.

We expect our financial condition and operating results to fluctuate significantly from quarter-to-quarter and year-to-year due to a variety of factors, many of which are beyond our control. Accordingly, you should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development efforts.

We expect to devote substantial financial resources to our ongoing and planned activities, particularly as we conduct research and development activities, and initiate clinical trials of, and seek regulatory approval for, KPI-012 and any other product candidate that we develop in the future. If we do obtain regulatory approval for KPI-012 or any other product candidate that we develop, we expect to incur commercialization expenses related to product sales, marketing, distribution and manufacturing capabilities. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

Our future capital requirements will depend on many factors, including:

- the timing and amount of milestone payments we ultimately receive from Alcon under the asset purchase agreement;
- the timing and amount of our future milestone payments to Combangio equityholders under the merger agreement;
- the timing and amount of milestone payments we ultimately receive from CIRM in connection with the CIRM Award;
- the progress, costs and results of our ongoing and planned clinical trials of KPI-012;
- the costs and timing of process development and manufacturing scale-up activities associated with KPI-012 for PCED and any other indications we determine to pursue;
- the costs, timing and outcome of regulatory review of KPI-012;
- the costs and timing of commercialization activities for KPI-012, if approved, including establishing and/or expanding product sales, marketing, medical affairs, distribution and outsourced manufacturing capabilities;
- our ability to successfully commercialize KPI-012, if approved, in the United States and other jurisdictions and the amount of revenue received from commercial sales;
- our ability to establish and maintain strategic collaborations, licensing or other agreements and the financial terms of such agreements;
- the scope, progress, results and costs of research and development of any other product candidates that we may develop, including under our KPI-014 program;
- the extent to which we successfully advance and/or in-license or acquire rights to other products, product candidates or technologies; and
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against any intellectual property-related claims.

We expect to continue to incur significant expenses and operating losses. Net losses may fluctuate significantly from quarter-to-quarter and year-to-year. We expect that our cash and cash equivalents of $50.9 million as of December 31, 2023, together with the $8.6 million of gross proceeds we received from the sale of shares of our preferred stock in a private placement in March 2024 and the $9.1 million of remaining funding anticipated under the CIRM Award, will enable us to fund our operations, lease and debt service obligations and capital expenditure requirements into the third quarter of 2025. We expect that our existing cash resources will be sufficient to enable us to obtain safety and efficacy data from our ongoing CHASE Phase 2b clinical trial of KPI-012 in PCED. However, we do not expect that our existing cash resources will be sufficient to enable us to complete the clinical development of KPI-012 for PCED or for any other indication. We have based our estimates on assumptions that may prove to be wrong, and our operating plan may change

as a result of many factors currently unknown to us. For example, we may not receive all of the funds awarded under the CIRM Award. As a result, we could deplete our available capital resources sooner than we currently expect.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete. Completion dates and completion costs can vary significantly for each product candidate and are difficult to predict. We may never generate the necessary data or results required to obtain marketing approval and achieve product sales from KPI-012 or any other product candidate we develop. Also, even if we successfully develop KPI-012 or any other product candidate and one or more of those are approved, we may not achieve commercial success with them. Accordingly, we will require additional financing to achieve our business objectives. In addition, we may opportunistically raise additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. Adequate additional financing may not be available to us on acceptable terms, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce or terminate preclinical studies, clinical trials or other development activities for one or more of our product candidates or delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize any product candidate for which we obtain approval.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements, royalty agreements, and marketing and distribution arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other rights and preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include pledging of assets as collateral and covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

For example, our pledge of our assets as collateral to secure our obligations under our Loan Agreement may limit our ability to obtain additional debt financing. Under the Loan Agreement, we are also restricted from paying dividends on our common stock, granting liens, making investments, making acquisitions, making certain restricted payments, selling assets and making certain other uses of our cash without the lenders' consent, subject in each case to certain exceptions. In addition, under our securities purchase agreements for our 2022, 2023 and 2024 private placements, we have agreed that we will not, without the prior approval of the requisite purchasers: (1) issue or authorize the issuance of any equity security that is senior or *pari passu* to the Series E Convertible Non-Redeemable Preferred Stock, the Series F Convertible Non-Redeemable Preferred Stock or the Series G Convertible Non-Redeemable Preferred Stock with respect to liquidation preference, (2) incur any additional indebtedness for borrowed money in excess of $1.0 million, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of its existing indebtedness or (3) pay or declare any dividend or make any distribution on, any of our shares of capital stock, subject to specified exceptions.

In addition, if we raise additional funds through collaborations, strategic alliances, licensing arrangements, royalty agreements, or marketing and distribution arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or current or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Our substantial indebtedness may limit cash flow available to invest in the ongoing needs of our business and a failure to comply with the covenants under our Loan Agreement, such as the requirement that our common stock continue to be listed on The Nasdaq Stock Market, could result in an event of default and acceleration of amounts due.

We have a substantial amount of indebtedness. As of December 31, 2023, we had $34.0 million of outstanding borrowings under the tranche A term loan under the Loan Agreement, which through June 30, 2023 bore interest at a

floating rate equal to the greater of 30-day LIBOR and 0.11%, plus 7.89%. Effective July 1, 2023, the term loan bears interest at a floating rate equal to the greater of (i) 8.00% and (ii) the sum of (a) the 1-Month CME Term Secured Overnight Financing Rate, or SOFR, (b) 0.10% and (c) 7.89%. Fluctuations in interest rates could materially affect the interest expense on our Loan Agreement. The start date for amortization payments under the Loan Agreement is January 1, 2025, at which time the aggregate principal balance of the term loan then outstanding under the Loan Agreement is required to be repaid in monthly installments through May 1, 2026. Pursuant to the Loan Agreement, we may also make partial prepayments of the term loan to the lender, subject to specified conditions, including the payment of applicable fees and accrued and unpaid interest on the principal amount of the term loan being repaid. Our obligations under the Loan Agreement are secured by substantially all of our assets.

Our debt combined with our other financial obligations and contractual commitments could have significant adverse consequences, including:

- requiring us to dedicate a substantial portion of cash flow from operations or cash on hand to the payment of interest on, and principal of, our debt, which will reduce the amounts available to fund working capital, capital expenditures, product development efforts and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to acquire other businesses for cash, take certain other corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

We may not have sufficient funds or may be unable to arrange for additional financing to pay the amounts due under our existing debt, particularly if we are in default under our Loan Agreement and all of our indebtedness under the Loan Agreement is due, and funds from external sources may not be available on a timely basis or acceptable terms, if at all. In addition, a failure to comply with the covenants under our Loan Agreement could result in an event of default and acceleration of amounts due. In particular, a delisting of our common stock from The Nasdaq Capital Market or a transfer of the listing of our common stock to another nationally recognized stock exchange having listing standards that are less restrictive than The Nasdaq Capital Market, in each case after a specified cure period, are events of default under our Loan Agreement. In such event, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness. Acceleration of the repayment of the outstanding indebtedness would raise substantial doubt about our ability to continue as a going concern, shorten the period for which we will be able to fund our operations and capital expenditure requirements and would adversely effect our financial condition and ability to pursue our business strategy.

The milestone consideration we are eligible to receive in connection with the sale of our Commercial Business to Alcon is subject to various risks and uncertainties.

The milestone consideration we are eligible to receive for the sale of our Commercial Business to Alcon is subject to various risks and uncertainties. In addition to the upfront payment of $60.0 million we received from Alcon at closing, we are eligible to receive up to four commercial-based sales milestone payments as follows: (1) $25.0 million upon the achievement of $50.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (2) $65.0 million upon the achievement of $100.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (3) $75.0 million upon the achievement of $175.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029 and (4) $160.0 million upon the achievement of $250.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029.

We cannot predict what success, if any, Alcon and its affiliates may have with respect to sales of EYSUVIS and INVELTYS and, therefore, it is uncertain as to when we may receive the milestone payments, which milestone payments we may receive and if we will receive any milestone payments at all. If we do not receive some or all of the milestone payments, our business will be harmed.

If our estimates or judgments relating to our critical accounting policies, or any of our projections, prove to be inaccurate or financial reporting standards or interpretations change, our results of operations could be adversely affected.

The preparation of financial statements in conformity with generally accepted accounting principles in the United States requires management to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. The preparation of our financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities and expenses. Such estimates and judgments include the present value of lease liabilities and the corresponding right-of-use assets, the fair value of warrants, stock-based compensation, accrued expenses, contingent consideration, grant income and deferred grant income, the valuation of embedded derivatives and the recoverability of our net deferred tax assets and related valuation allowance. We base our estimates and judgments on historical experience, expected future experience and on various other assumptions that we believe to be reasonable under the circumstances. In addition, from time to time, we may rely on projections regarding our expected future performance that represent our management's then-current estimates. However, any of these estimates, judgments or projections, or the assumptions underlying them, may change over time or may otherwise prove to be inaccurate. In particular, to report historical product revenue, we estimated the amount of our products that may be returned and presented this amount as a reduction of revenue in the period the related product revenue was recognized, in addition to establishing a liability. If our product return estimates are lower than the actual amount of product returns we experience, our existing reserves will be insufficient to cover future returns. Our results of operations may be adversely affected if our estimates, assumptions or projections change or if actual circumstances differ from those in our estimates or assumptions, which could cause our results of operations to fall below the expectations of securities analysts and investors, resulting in a decline in the trading price of our common stock.

Additionally, we regularly monitor our compliance with applicable financial reporting standards and review new pronouncements and drafts thereof that are relevant to us. As a result of new standards, changes to existing standards and changes in their interpretation, we might be required to change our accounting policies, alter our operational policies and implement new or enhance existing systems so that they reflect new or amended financial reporting standards, or we may be required to restate our published financial statements. Such changes to existing standards or changes in their interpretation may have an adverse effect on our reputation, business, financial position and results of operations.

Risks Related to Product Development

We are substantially dependent on the success of our product candidate, KPI-012. If we are unable to successfully complete the clinical development of, and obtain marketing approval for, KPI-012 or any other product candidate we may develop in the future, or experience significant delays in doing so, or if, after obtaining marketing approvals, we fail to successfully commercialize such product candidates, our business will be materially harmed.

We are substantially dependent on the success of KPI-012 and any other product candidate we may develop in the future. As a result, we intend to devote a substantial portion of our research and development resources and business efforts to the development of KPI-012.

The success of KPI-012 and any other product candidates we may develop in the future will depend on many factors, including the following:

- completing and obtaining favorable results from our ongoing and planned clinical trials of KPI-012 and any other product candidate we develop;
- clearance of any investigational new drug application, or IND, submission for any other product candidates we develop;
- applying for and receiving marketing approvals from the FDA and any other regulatory authorities for KPI-012 and any other product candidate we develop;
- if approved, successfully launching and commercializing KPI-012 or any other product candidate we develop in the United States, including establishing and maintaining sales, marketing, manufacturing and distribution capabilities for KPI-012 or any other product candidate we develop;

- if approved, obtaining acceptance of KPI-012 and any other product candidate we develop by patients, the medical community and third-party payors;
- obtaining and maintaining coverage, adequate pricing, and adequate reimbursement from third-party payors, including government payors, for our product candidates;
- obtaining and maintaining regulatory approval of our manufacturing processes and our third-party manufacturers' facilities from applicable regulatory authorities and obtaining and maintaining adequate supply of any such approved products;
- maintaining a workforce of experienced scientists and others with experience in eye diseases and biologics to continue to develop our product candidates;
- effectively competing with other therapies;
- maintaining an acceptable potency, purity and safety profile of our products following approval;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- protecting our rights in our intellectual property portfolio; and
- not infringing, misappropriating or otherwise violating others' intellectual property rights.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize KPI-012 or any other product candidate we may develop in the future, which would materially harm our business. We may never generate the necessary data or results required to obtain regulatory approval of KPI-012 or any other product candidate we develop and the commercialization of KPI-012 or any other product candidate we develop may never occur.

If clinical trials of KPI-012 or any other biological product candidate that we develop fail to demonstrate potency, safety and purity to the satisfaction of the FDA or other regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of such product candidate.

The risk of failure in developing product candidates is high. It is impossible to predict when or if any product candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the potency, purity and safety for a biologic product in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later stage clinical trials, and interim results of a clinical trial do not necessarily predict final results. For example, the results of Combangio's Phase 1b clinical trial of KPI-012 in twelve patients, including nine with PCED, may not be indicative of future results in later stage clinical trials, including in our ongoing CHASE Phase 2b clinical trial of KPI-012 in patients with PCED. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their product candidates. Furthermore, the failure of any product candidates to demonstrate potency, safety and purity in any clinical trial could negatively impact the perception of our other product candidates and/or cause the FDA or other regulatory authorities to require additional testing before approving any of our product candidates. For example, in our STRIDE 2 Phase 3 clinical trial evaluating the safety and efficacy of EYSUVIS versus placebo in patients with dry eye disease, we did not achieve statistical significance for the primary symptom endpoint of ocular discomfort severity, and subsequently we received a complete response letter from the FDA indicating that positive efficacy data from an additional clinical trial was needed to support a new drug application for EYSUVIS.

If we are required to conduct additional clinical trials or other testing of KPI-012 or any other product candidate we develop beyond those that we currently expect, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates could be delayed or prevented, and our competitors could bring products to market before we do.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize KPI-012 or any other product candidate that we may develop, including:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may recommend or require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their obligations to us in a timely manner, or at all;
- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may decide, or regulators or institutional review boards may require us, to suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- we may be subject to additional post-marketing testing requirements to maintain regulatory approval;
- regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate or may be delayed;
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate trials;
- restrictions resulting from health epidemics, including COVID-19, and their collateral consequences may result in internal and external operational delays and limitations; and

- regulatory authorities may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a modified Risk Evaluation and Mitigation Strategy, or REMS.

Our product development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors, such as those developing treatments for PCED, to bring products to market before we do and impair our ability to successfully commercialize our product candidates.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for KPI-012 or any other product candidate we may develop if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States.

Patient enrollment is affected by a variety of factors, including:

- the prevalence and severity of the disease or condition under investigation;
- the patient eligibility criteria for the trial in question;
- the perceived risks and benefits of the product candidate under study;
- the existence of existing treatments for the indications for which we are conducting clinical trials;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of clinicians;
- the ability to monitor patients adequately during and after treatment;
- the proximity and availability of clinical trial sites for prospective patients;
- the conducting of clinical trials by competitors for product candidates that treat the same indications as our product candidates;
- the impact of public health epidemics, such as COVID-19; and
- the lack of adequate compensation for prospective patients.

We are developing KPI-012 for PCED, which is a rare condition with an estimated incidence in the United States of 100,000 cases per year, and, as such, we may have difficulty identifying and enrolling a sufficient number of patients in our ongoing and planned clinical trials of KPI-012 given the limited number of patients with PCED. Our inability to locate and enroll a sufficient number of patients for our clinical trials could result in significant delays, could require us to abandon one or more clinical trials altogether and could delay or prevent our receipt of necessary regulatory approvals. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

If serious adverse or unacceptable side effects are identified during the development or commercialization of our product candidates, we may need to abandon or limit our development and/or commercialization efforts for such product candidates.

If KPI-012 or any other product candidate we develop are associated with serious adverse events or undesirable side effects in clinical trials or following approval and/or commercialization, or if any of our product candidates have characteristics that are unexpected, we may need to abandon their development or limit development or marketing to narrower uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. While KPI-012 was generally well-tolerated in Combangio's Phase 1b clinical trial, it was only administered in 12 subjects. Compounds that initially show promise in clinical or earlier stage testing for treating eye disease or other diseases may later be found to cause side effects that prevent further development and commercialization of the compound. In addition, adverse events which had initially been considered unrelated to the study treatment may later, even following approval and/or commercialization, be found to be caused by the study treatment. Moreover, incorrect or improper use of a product by patients could result in additional unexpected side effects or adverse events. There can be no assurance that any product we may develop will be used correctly, and if used incorrectly, such misuse could hamper commercial adoption or market acceptance of such products or product candidates, if approved, at the rate we currently expect.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. In July 2022, we sold our Commercial Business, including EYSUVIS and INVELTYS, to Alcon and we made a strategic determination to cease the development of our preclinical pipeline programs that are unrelated to our MSC-S platform and to focus our research and development efforts solely on this platform.

We may never realize the anticipated benefits of these decisions and, as a result, we may be required to forego or delay other opportunities. In addition, our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and KPI-012 for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

KPI-012 has been evaluated in a clinical trial outside of the United States and we may in the future conduct clinical trials for product candidates at sites outside the United States. The FDA may not accept data from trials conducted in such locations.

Combangio has in the past chosen, and we may in the future choose, to conduct one or more of our clinical trials outside the United States, including adding sites in Latin America for the CHASE Phase 2b clinical trial, subject to regulatory approval. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, where data from foreign clinical trials are not intended to serve as the sole basis for approval in the United States, the FDA will not accept the data as support for a marketing application unless the clinical trial was well designed and conducted in accordance with GCP requirements. The FDA must also be able to validate the data from the trial through an onsite inspection, if necessary. In addition, these clinical trials are subject to the applicable local laws of the jurisdictions where the trials are conducted. There can be no assurance that the FDA will accept data from trials conducted outside of the United States.

If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates.

In addition, conducting clinical trials outside the United States could have a significant adverse impact on us. Risks inherent in conducting international clinical trials include: clinical practice patterns and standards of care that vary

widely among countries; non-U.S. regulatory authority requirements that could restrict or limit our ability to conduct our clinical trials; compliance with foreign manufacturing, customs, shipment and storage requirements; administrative burdens of conducting clinical trials under multiple non-U.S. regulatory authority schema; foreign exchange fluctuations; diminished protection of intellectual property in some countries; and interruptions or delays resulting from geopolitical events, such as wars.

In 2020 and 2021, Combangio conducted a Phase 1b clinical trial of KPI-012 in nine patients with PCED in Mexico. Based on the results of the Phase 1b clinical trial conducted in Mexico, we initiated a full preclinical development program and submitted an IND application to the FDA for KPI-012 which was approved in December 2022, and in February 2023, we dosed our first patient in the CHASE Phase 2b clinical trial of KPI-012 for PCED in the United States. We are also planning to add clinical trial sites in Latin America to our CHASE Phase 2b trial, subject to regulatory approval. If the FDA does not accept the data from any trial that we conduct outside the United States, it could delay or permanently halt our development of the applicable product candidates.

Public health epidemics, including the COVID-19 pandemic, could impact the development of KPI-012 or any other product candidate we may develop, and may adversely affect our business, results of operations and financial condition.

Public health epidemics, including the COVID-19 pandemic, may affect our ability to initiate and complete preclinical studies and clinical trials for KPI-012 and any other product candidates we develop, including disruptions in procuring supplies that are essential for our research and development activities, manufacturing disruptions, disruptions in our ability to obtain necessary trial site approvals, as well as delays in or difficulties with enrollment and other delays at clinical trial sites. The public health emergency declarations related to COVID-19 ended on May 11, 2023. The FDA ended certain COVID-19 related policies and retained others. As a result of these and other measures, we may in the future face disruptions to our business. We do not know the extent to which public health epidemics, including the COVID-19 pandemic, will impact our development of KPI-012, including our ongoing CHASE Phase 2b clinical trial, or any other product candidates that we develop. Additionally, while we currently are not experiencing interruptions in our manufacturing of KPI-012, any reinstatement of quarantines, travel restrictions and other measures related to a public health emergency may significantly impact the ability of employees of our third-party suppliers to get to their places of work to manufacture and deliver future supplies if and when needed.

Public health epidemics may cause disruptions in financial markets, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. Moreover, the impact of COVID-19 on economies worldwide could result in adverse effects on our business and operations.

While the public health emergency declared for the COVID-19 pandemic has terminated, we cannot be certain what the overall impact of the COVID-19 pandemic or any other public health emergencies or pandemics will be on our business in the future and a continuation of the pandemic has the potential to adversely affect our business, financial condition, results of operations and prospects.

Risks Related to the Commercialization of our Product Candidates

Even if KPI-012 or any other product candidates that we may develop in the future receives marketing approval, such products may fail to achieve market acceptance by clinicians and patients, or adequate formulary coverage, pricing or reimbursement by third-party payors and others in the medical community, and the market opportunity for these products may be smaller than we estimate.

If KPI-012 or any other product candidate that we develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by clinicians, patients, third-party payors and others in the medical community. We are developing KPI-012 for PCED, which is a rare disease. Our understanding of both the number of people who have a PCED, as well as the subset of people with PCED diseases who have the potential to benefit from treatment with KPI-012, are based on estimates. These estimates may prove to be incorrect. The number of patients with PCED may turn out to be lower than expected, may not be otherwise amenable to treatment with KPI-012 or may become increasingly difficult to identify and access, all of which would adversely affect our business, financial condition, results of operations and prospects.

Biosimilar and generic versions of any products that compete with KPI-012 or any other product candidates we may develop would likely be offered at a substantially lower price than we expect to offer for our product candidates, if approved. As a result, clinicians, patients and third-party payors may choose to rely on such products rather than our product candidates.

Our assessment of the potential market opportunity for KPI-012 is based on industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data. The potential market opportunity for the treatment of PCED is difficult to precisely estimate. Our estimates of the potential market opportunities for KPI-012 include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions. If any of our assumptions or estimates, or these publications, research, surveys or studies prove to be inaccurate, then the actual market for KPI-012 for PCED may be smaller than we expect, and as a result our future product revenue may be limited and it may be more difficult for us to achieve or maintain profitability.

If KPI-012 or any other product candidate for which we may obtain marketing approval does not achieve adequate levels of acceptance by physicians and patients, formulary coverage, pricing or reimbursement, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of KPI-012 or any other product candidate for which we may obtain marketing approval, will depend on a number of factors, including:

- the efficacy and potential advantages of our product candidates compared to alternative treatments, including the existing standard of care;
- our ability to offer our products for sale at competitive prices, particularly in light of the lower cost of alternative treatments;
- the availability of third-party formulary coverage and adequate reimbursement;
- the clinical indications for which the product is licensed or approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of clinicians to prescribe these therapies;
- the strength of our marketing and distribution support;
- the timing of market introduction of competitive products;

- the prevalence and severity of any side effects; and
- any restrictions on the use of our products together with other medications.

Even if we are able to successfully commercialize KPI-012 or any other product candidate that we may develop, if and when they are approved, the products may become subject to unfavorable pricing regulations, third-party coverage or reimbursement practices or healthcare reform initiatives, which could harm our business.

Our ability to successfully commercialize KPI-012 or any other product candidate that we may develop if and when they are approved will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government healthcare programs, private health insurers, managed care plans and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Coverage and reimbursement may not be available for KPI-012 or any other product candidate that we may commercialize and, even if they are available, the level of reimbursement may be limited or not satisfactory.

Inadequate reimbursement may adversely affect the demand for, or the price of KPI-012 or any other product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize KPI-012 or any other product candidate if and when they are approved.

There may be significant delays in obtaining coverage and reimbursement for newly approved products and coverage may be more limited than the indications for which the product is approved by the FDA or similar regulatory authorities outside the United States. Reimbursement agencies in Europe may be more conservative than the Centers for Medicare & Medical Services, or CMS, in the United States. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost products and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies.

Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop would compromise our ability to generate revenues and become profitable.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a product before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies.

Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Even if a product candidate we develop is approved for sale in the United States or in other countries, there can be no assurance that such product candidate will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payors, or that coverage and an adequate level of reimbursement will be available or that third-party payors' reimbursement policies will not adversely affect our ability to sell such product candidate profitably.

If we are unable to establish and maintain sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, if and when necessary, we may not be successful in commercializing KPI-012 or any other product candidate that we may develop if and when they are approved.

We established a sales, marketing and distribution infrastructure for the commercial launch of EYSUVIS and INVELTYS, and, as a company, we have limited experience in the sales, marketing and distribution of therapeutic products. Following the sale of our Commercial Business to Alcon in July 2022 and our determination to focus our research and development efforts on KPI-012 and our MSC-S platform, we terminated our entire commercial sales force and certain employees in our commercial, scientific, manufacturing, finance and administrative functions. To achieve commercial success for any product for which we obtain marketing approval in the future, we will again need to establish sales, marketing and distribution capabilities, either ourselves or through collaborations or other arrangements with third parties.

There are risks involved with establishing, maintaining and expanding, if and when necessary, our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming, may divert our management and business development resources and could delay any future product launch. Establishing and maintaining a sales force would require us to continue to implement and improve our managerial, operational and financial systems, which we may not do effectively. Any inability to manage growth, when necessary, could delay the execution of our business plans or disrupt our operations. Further, we may overestimate or underestimate the size of the sales force required for a successful product launch.

We have not yet established our own commercial organization or distribution capabilities specific to KPI-012. While we believe that we will be able to commercialize KPI-012, if approved, for the treatment of PCED with a small, targeted, internal sales force in the United States and potentially other major markets, our assumptions may prove inaccurate. In the future, we may need a larger sales force and at a higher cost than previously anticipated. If the commercial launch of any product candidate for which we establish a commercial infrastructure is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition any such sales, marketing and distribution personnel.

Factors that may inhibit our efforts to commercialize on our own KPI-012 or any other product candidate we develop, if and when approved, include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to obtain and maintain coverage, adequate pricing and adequate reimbursement from third-party payors, including government payors;
- the inability of sales personnel to obtain access to clinicians or persuade adequate numbers of clinicians to prescribe our products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with establishing, maintaining and expanding, if and when necessary, an independent sales, marketing and distribution organization.

While we cannot be certain when, if ever, we will seek and/or receive marketing approval to commercialize any of our product candidates outside the United States, we may seek marketing approval and explore commercialization of

KPI-012 in certain markets outside the United States utilizing a variety of collaboration, distribution, co-promotion and other marketing arrangements with one or more third parties. Our product revenues and our profitability, if any, under any such third-party collaboration, distribution or other marketing arrangements are likely to be lower than if we were to market, sell and distribute KPI-012 ourselves. We may also consider seeking marketing approval outside the United States for other product candidates we may develop in the future. If we decide to seek regulatory approval for any of our product candidates outside the United States, we may need to seek additional patent approvals, seek licenses to patents held by third parties and/or face claims of infringing third-party patent rights.

In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute KPI-012 or any other product candidate we may develop or we may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market effectively any product candidate for which we obtain marketing approval. If we do not establish and maintain our sales, marketing and distribution capabilities successfully, when needed, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidate for which we obtain marketing approval.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. Our competitors include major pharmaceutical companies with significantly greater financial resources. KPI-012 and any other product candidate we may develop, if approved, will also compete with existing branded, generic and off-label products.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our product candidate, KPI-012, and we will face competition with respect to any other product candidate that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

If approved, we expect KPI-012 to compete with Oxervate®, which is the only approved prescription pharmaceutical product in the PCED space. Oxervate (cenegermin-bkbj) was approved in August 2018 for the treatment of neurotrophic keratitis, or NK, a degenerative disease characterized by decreased corneal sensitivity and poor corneal healing, which we believe to represent approximately one-third of all PCED cases. Oxervate is a topical eye drop that is administered six times per day at two-hour intervals for eight weeks. Each administration of Oxervate requires the use of a vial containing the drug product, a vial adapter, a single-use pipette and disinfectant wipes. To our knowledge, there are currently only two product candidates in active clinical development for the treatment of a broad PCED population. KIO-201, a chemically modified form of the natural polymer hyaluronic acid administered as an eye drop, is currently being studied in a Phase 2 clinical trial in patients with PCED by Kiora Pharmaceuticals, Inc. Nexagon®, an antisense oligonucleotide that inhibits connexin43 being developed by Amber Ophthalmics, is currently being studied in a Phase 2/3 clinical trial in patients with PCED resulting from severe ocular chemical and/or thermal injuries. Amber Ophthalmics has also indicated that it plans to study Nexagon ® in a broad PCED population. A number of companies are pursuing development of product candidates for the treatment of NK, including ReGenTree, LLC (Timbetasin), Recordati S.p.A. (Udonitrectag) and Claris Biotherapeutics, Inc. (CSB-001).

We are also aware of potential competitors for KPI-012 for Limbal Stem Cell Deficiency, or LSCD. Competitive products and product candidates in LSCD include two stem cell-based approaches. ABCB5+ limbal stem cells, which are being studied in Phase 1/2 clinical trials and are being developed by RHEACELL GmbH & Co. KG, utilize allogeneic limbal stem cells derived from human corneal rims, which are expanded ex-vivo and manufactured as an advanced-therapy medicinal product. Holoclar utilizes autologous limbal stem cells derived from the healthy portion of the patient's eye. Holoclar is approved in the European Union for treatment of LSCD caused by ocular burns and is developed by Chiesi.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our products. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Our competitors may develop products that are available on a generic basis, and our product candidates may not demonstrate sufficient additional clinical benefits to clinicians, patients or payors to justify a

higher price compared to generic products. In many cases, insurers or other third-party payors, particularly Medicare, seek to encourage the use of biosimilar and generic products.

Many of the companies against which we are competing or which we may compete against in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Product liability lawsuits against us could divert our resources and could cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the use of our product candidates that we develop in human clinical trials, including KPI-012. We face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced time and attention of our management to pursue our business strategy; and
- the inability to successfully commercialize any products that we may develop.

We currently hold $10 million in product liability insurance coverage in the aggregate, with a per incident limit of $10 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage if we expand our ongoing and planned clinical trials for KPI-012. We will need to further increase our insurance coverage when and if we begin commercialization of KPI-012 or any other product candidate for which we obtain marketing approval. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Dependence on Third Parties

We have relied, and expect to continue to rely, on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We have relied on third parties, such as clinical research organizations, clinical data management organizations, medical institutions and clinical investigators, in conducting our clinical trials and expect to continue to rely on such parties to conduct clinical trials of any product candidate that we develop. We or these third parties may terminate their engagements with us at any time for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, that could delay our product development activities.

Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA

requires us to comply with GCP standards for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors.

We also have relied, and expect to continue to rely, on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of products, producing additional losses and depriving us of potential product revenue.

We contract with third parties for the manufacture of KPI-012 and plan to contract with third parties for preclinical, clinical and commercial supply of any other product candidates we develop. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not own or operate manufacturing facilities for the production of preclinical and clinical quantities of any product candidates. We do not own or operate, and currently have no plans to establish, any manufacturing facilities for KPI-012. We rely, and expect to continue to rely, on third parties for the manufacture of both drug substance and finished product for KPI-012 for preclinical and clinical testing, as well as for commercial manufacture of KPI-012 if it receives marketing approval. We also rely, and expect to continue to rely, on third parties for packaging, labeling, sterilization, storage, distribution and other production logistics for KPI-012. We have only limited supply agreements in place with respect to KPI-012, and these arrangements do not extend to commercial supply. We obtain supplies of drug substance and finished product for KPI-012 on a purchase order basis and do not have long term committed supply arrangements with respect to KPI-012. We may be unable to maintain our current arrangements for KPI-012 or enter into agreements for commercial supply of KPI-012 on acceptable terms or at all. We also expect to rely on third-party manufacturers to manufacture preclinical, clinical and commercial supplies of any other product candidates we develop, as well as for packaging, serialization, storage, distribution and other production logistics.

We are subject to risks related to our reliance on third-party manufacturers for the manufacture of the drug substance and product of KPI-012, a biological product candidate. Manufacturing biologics is complex, especially in large quantities. Biologic products must be made consistently and in compliance with a clearly defined manufacturing process. KPI-012 is a bone-marrow derived MSC-S therapeutic composed of biologically active components, including protease inhibitors and growth factors, and is produced from a proprietary cell bank. The manufacturing process for KPI-012 is comprised of three stages: (1) cultivation of mesenchymal stem cells from a working cell bank and production of unprocessed conditioned media (cell-free secretome), (2) production of drug substance as a chemically defined solution and (3) formulation and filling of drug product. While the drug product for Combangio's early research and Phase 1b clinical trial was cultivated using a planar culture model, we implemented a bioreactor cultivation model for our ongoing CHASE Phase 2b clinical trial of KPI-012. We also plan to utilize a bioreactor cultivation model for our planned clinical trials and for commercial supply of KPI-012. We are continuing the process of scaling up our manufacturing processes and capabilities with our third-party manufacturers to support longer term clinical development. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. In addition, KPI-012 drug product is manufactured from a vial of a working cell bank, which in turn was produced from a vial of master cell bank. KPI-012 master cell bank and working cell bank is stored in two separate locations. It is possible that we could lose the cell bank in both locations and have our manufacturing severely impacted by the need to replace the cell bank.

Our third party manufacturers may encounter shortages in the raw materials necessary to produce our product candidates in the quantities needed for our clinical trials, or our product candidates, if approved, in sufficient quantities for commercialization or to meet an increase in demand, as a result of capacity constraints or delays or disruptions in the market for the raw materials, including shortages caused by the purchase of such raw materials by our competitors or others and shortages related to epidemics or pandemics, such as the COVID-19 pandemic. The failure of us or our third

party manufacturers to obtain the raw materials necessary to manufacture sufficient quantities of KPI-012 or any other product candidates we may develop, may have a material adverse effect on our business.

The FDA maintains strict requirements governing the manufacturing process and third-party manufacturers are subject to inspection and approval by the FDA before a company can commence the manufacture and sale of any of its products or product candidates, and thereafter subject to FDA inspection from time to time. Failure by third-party manufacturers to pass such inspections and otherwise satisfactorily complete the FDA approval regimen with respect to products or product candidates may result in regulatory actions such as the issuance of FDA Form 483 notices of observations, warning letters or injunctions or the loss of operating licenses. Depending on the severity of any potential regulatory action, our clinical or commercial supply could be interrupted or limited, which could have a material adverse effect on our business. When a manufacturer seeks to modify or make even seemingly minor changes to the manufacturing process, the FDA may require the applicant to conduct a comparability study that evaluates the potential differences in the product resulting from the change in the manufacturing process. In connection with any application for approval to market product candidates in the United States, we may be required to conduct a comparability study if the product we intend to market is supplied by a manufacturer different from the one who supplied the product evaluated in our clinical studies. Delays in designing and completing this study to the satisfaction of the FDA could delay or preclude our development and commercialization plans and thereby limit our revenues and growth.

Reliance on third-party manufacturers entails additional risks, including reliance on the third-party for regulatory compliance and quality assurance, the possible breach of the manufacturing agreement by the third-party, the possible misappropriation of our proprietary information, including our trade secrets and know-how, and the possible termination or nonrenewal of the agreement by the third-party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates and harm our business and results of operations.

KPI-012 and any other product candidate that we may develop may compete with other product candidates and products for access to a limited number of suitable manufacturing facilities that operate under cGMP regulations. For example, we were previously required to change our third-party manufacturer when the manufacturer was purchased by a third-party and exited the contract manufacturing business. The process of changing manufacturers can cause substantial time delays, and if we are required to change our manufacturer again in the future, it may delay our ongoing and planned clinical trials or development timeline.

Our current and anticipated future dependence upon others for the manufacture of KPI-012 or any other product candidate we develop may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

The manufacture of biologics is complex and our third-party manufacturers may encounter difficulties in production. If any of our third-party manufacturers encounter such difficulties, our ability to provide supply of product candidates for clinical trials or products for patients, if approved, could be delayed or prevented.

Manufacturing biologics, especially in large quantities, is often complex and may require the use of innovative technologies to handle living cells. Each lot of an approved biologic must undergo thorough testing for identity, strength, quality, purity and potency. Manufacturing biologics requires facilities specifically designed for and validated for this purpose, and sophisticated quality assurance and quality control procedures are necessary. Slight deviations anywhere in the manufacturing process, including filling, labeling, packaging, storage and shipping and quality control and testing, may result in lot failures, product recalls or spoilage. When changes are made to the manufacturing process, we may be required to provide preclinical and clinical data showing the comparable identity, strength, quality, purity or potency of the products before and after such changes. If microbial, viral or other contaminations are discovered at the facilities of our manufacturers, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials and adversely harm our business.

In addition, there are risks associated with large scale manufacturing for clinical trials or commercial scale including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, compliance with cGMPs, lot consistency and timely availability of raw materials. Even if we obtain regulatory approval for KPI-012 or any product candidates we may develop in the future, there is no assurance that our manufacturers will be able to manufacture the approved product to specifications acceptable to the FDA or other comparable foreign regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential commercial launch of the product or to meet potential future demand. If our manufacturers are unable to produce sufficient quantities for clinical trials or for commercialization, our development and commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and growth prospects.

Our reliance on CIRM funding for KPI-012 adds uncertainty to our research and development efforts, imposes certain compliance obligations on us and imposes requirements that may increase the costs of commercializing KPI-012.

Our development of KPI-012 is currently being funded, in part, by an award from the California Institute for Regenerative Medicine, or CIRM. On August 2, 2023, our wholly-owned subsidiary, Combangio, entered into an award agreement with CIRM for a $15.0 million grant, or the CIRM Award, to support the ongoing KPI-012 program for the treatment of PCED as well as product and process characterization and analytical development for the program. The CIRM Award is subject to a co-funding requirement under which Combangio is obligated to spend a specified minimum amount on the development of KPI-012 to obtain the full award amount and the remaining $9.1 million under the award is payable to Combangio only upon the achievement of specified milestones that are primarily related to Combangio's progress in conducting the CHASE clinical trial. If we fail to satisfy the co-funding requirement under the CIRM Award or fail to achieve the milestones within the timeframe required by the CIRM Award, we may not receive full funding under the CIRM Award. CIRM may permanently cease disbursements under the CIRM Award if the milestones are not met within four months of their scheduled completion dates. We cannot be certain that we will achieve such milestones within the timeframe required by the CIRM Award, or at all, and as such we may never receive the remaining $9.1 million under the award. Additionally, if CIRM determines, in its sole discretion, that Combangio has not complied with the terms and conditions of the CIRM Award, CIRM may suspend or permanently cease disbursements. Moreover, disbursements under the CIRM Award are contingent upon the availability of funds in the state of California's Stem Cell Research and Cures Fund, which is outside of our control.

The CIRM Award also imposes financial conditions that may increase the costs of commercializing KPI-012, if approved. Under the terms of the CIRM Award, Combangio is obligated to pay a royalty on net sales of any product, service or approved drug resulting in whole or in part from the CIRM Award in the amount of 0.1% per $1.0 million of funds utilized by us until the earlier of 10 years from the date of first commercial sale of such product, service or approved drug and such time as nine times the amount of funds awarded by CIRM has been paid in royalties, or the Base Royalty. In addition, following the satisfaction of the Base Royalty, Combangio is obligated to pay a 1.0% royalty on net sales of any CIRM-funded invention in excess of $500 million per year until the last to expire patent covering such invention expires.

Additionally, there are significant compliance requirements associated with the CIRM Award, such as reporting, notification, recordkeeping and audit requirements, for which internal and external resources may be needed and which may increase our costs of doing business.

Noncompliance with the requirements of the CIRM Award may cause a default under our Loan Agreement with Oxford Finance. It is an event of default under our Loan Agreement if we receive funding under the CIRM Award and are required to return such funds to CIRM in an amount in excess of $500,000 due to our or Combangio's failure to comply with the requirements of the CIRM Award, or if we are required to return funds to CIRM in excess of $1.0 million due to non-utilization of such funds or because CIRM exercises its rights to recover such funds for any reason. Such an event of default could result in the acceleration of amounts due under our Loan Agreement. In such event, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness. Acceleration of the repayment of the outstanding indebtedness would raise substantial doubt about our ability to continue as a going concern, shorten the period for which we will be able to fund our operations and capital expenditure requirements and would adversely affect our financial condition and ability to pursue our business strategy.

In addition, as a result of the CIRM Award, we may not have the right to prohibit the State of California from using certain technologies developed by us. Under the CIRM Award, the California government can exercise march-in rights, which may include granting a third party nonexclusive, partially exclusive, or exclusive rights to CIRM-funded technology in any territory and field of use, if it determines that such action is necessary, if Combangio fails to make reasonable efforts to achieve practical application of a CIRM-funded technology, fails to comply with agreed to access and pricing requirements, or because action is necessary to address a public health emergency declared by the governor of California.

We may enter into collaborations with third parties for the development or commercialization of our product candidates. If our collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We expect to utilize a variety of types of collaboration, distribution and other marketing arrangements with third parties to develop and commercialize KPI-012 or any other product candidate we develop and for which we seek or obtain marketing approval in markets outside the United States. We also may enter into arrangements with third parties to perform these services in the United States if we do not establish our own sales, marketing and distribution capabilities in the United States for our product candidates or if we determine that such third-party arrangements are otherwise beneficial. We also may seek third-party collaborators for development and commercialization of our product candidates. For example, we may consider potential collaborative partnership opportunities prior to initiating IND-enabling studies on product candidates we may develop. Our likely collaborators for any sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. We are not currently party to any such arrangement. However, if we do enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Collaborations that we enter into may pose a number of risks, including the following:

- collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development of our product candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may not pursue commercialization of our product candidates that receive marketing approval or may elect not to continue or renew commercialization programs based on changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own products or product candidates, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;

- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would divert management attention and resources, be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates or products in the most efficient manner, or at all. If any collaborations that we enter into do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed, and we may need additional resources to develop our product candidates. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our collaborators.

Additionally, subject to its contractual obligations to us, if a collaborator of ours were to be involved in a business combination, it might de-emphasize or terminate the development or commercialization of any product or product candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be harmed.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans and our business could be adversely affected.

For some of our product candidates, we may decide to collaborate with pharmaceutical or biotechnology companies for the development of our product candidates or the potential commercialization of our product candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under future license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a product candidate, reduce or delay its development program or one or more of our other development programs, delay the potential commercialization of a product candidate or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization

activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market or continue to develop our product platform.

Risks Related to Our Intellectual Property

We may be unable to obtain and maintain patent protection for our technology or product candidates, or the scope of the patent protection obtained may not be sufficiently broad or enforceable, such that our competitors could develop and commercialize technology, products and product candidates similar or identical to ours, and our ability to successfully commercialize our technology and product candidates may be impaired.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and product candidates, including KPI-012. We have sought to protect our proprietary position by filing in the United States and in certain foreign jurisdictions patent applications related to our proprietary technologies and product candidates.

The patent prosecution process is expensive and time-consuming, and we may not have filed, maintained, or prosecuted and may not be able to file, maintain and prosecute all necessary or desirable patents or patent applications at a reasonable cost or in a timely manner. We may also fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of pharmaceutical, biotechnology, and medical device companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may fail to result in issued patents in the United States or in other foreign countries which protect our technology or product candidates, or which effectively prevent others from commercializing competitive technologies and products. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and the standards applied by the U.S. Patent and Trademark Office and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, unlike patent law in the United States, European patent law precludes the patentability of methods of treatment of the human body and imposes substantial restrictions on the scope of claims it will grant if broader than specifically disclosed embodiments. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain whether we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. Databases for patents and publications, and methods for searching them, are inherently limited so we may not know the full scope of all issued and pending patent applications. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies, products and product candidates. In particular, during prosecution of any patent application, the issuance of any patents based on the application may depend upon our ability to generate additional preclinical or clinical data that support the patentability of our proposed claims. We may not be able to generate sufficient additional data on a timely basis, or at all. Moreover, changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection for our proprietary technology and product candidates, prevent competitors from competing with us, or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies, products or product candidates in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, ownership, scope, validity, or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable,

in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology, products or product candidates, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing, and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we are not able to obtain patent term extension in the United States under the Hatch-Waxman Act and in foreign countries under similar legislation, thereby potentially extending the term of our marketing exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration, and specifics of FDA marketing approval of our product candidates, one of the U.S. patents covering each of such product candidates or the use thereof may be eligible for up to five years of patent term extension under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA approved product as compensation for the patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only those claims covering such approved drug product, a method for using it or a method for manufacturing it may be extended. Also, the regulatory review period of an FDA-approved product may not serve as a basis for a patent term extension if the active ingredient of such product was subject to regulatory review and approval in an earlier product approved by the FDA. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates. Nevertheless, we may not be able to seek or be granted patent term extension either in the United States or in any foreign country because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request.

If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product may be shortened and our competitors may obtain approval of competing products following our patent expiration sooner, and our revenue could be reduced, possibly materially.

It is possible that we will not obtain patent term extension under the Hatch-Waxman Act for a U.S. patent covering our product candidates even where that patent is eligible for patent term extension, or if we obtain such an extension, it may be for a shorter period than we had sought. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the U.S. Patent and Trademark Office, a petition for patent term extension under the Hatch-Waxman Act. Thus, if one of our licensed patents is eligible for patent term extension under the Hatch-Waxman Act, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the U.S. Patent and Trademark Office.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property rights, which could be expensive, time-consuming and unsuccessful.

Competitors and other third parties may infringe, misappropriate or otherwise violate our owned and licensed patents, trade secrets, or other intellectual property rights. As a result, to counter infringement, misappropriation or unauthorized use, we may be required to file infringement or misappropriation claims or other intellectual property related proceedings, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents or that our asserted patents are invalid. In addition, in a patent infringement or other intellectual property related proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly, and could put any of our patent applications at risk of not yielding an issued patent. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information or trade secrets could be compromised by disclosure during this type of litigation.

We may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in other contested proceedings such as opposition, derivation, reexamination, inter partes review, post-grant review, or interference proceedings in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

In the United States, the FDA does not prohibit clinicians from prescribing an approved product for uses that are not described in the product's labeling. Although use of a product directed by off-label prescriptions may infringe our method-of-treatment patents, the practice is common across medical specialties, particularly in the United States, and such infringement is difficult to detect, prevent, or prosecute and may have negative impacts on our business, operating results and financial condition.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability to develop, manufacture, market, and sell KPI-012 and any other product candidate we may develop in the future and to use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property and other proprietary rights of third parties. There is a considerable amount of intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, infringement litigation claims regarding our product candidates and technology, including claims from competitors or from non-practicing entities that have no relevant product revenue and against whom our own patent portfolio may have no deterrent effect. Moreover, we may become party to future adversarial proceedings or litigation regarding our patent portfolio or the patents of third parties. Such proceedings could also include contested post-grant proceedings such as oppositions, inter partes review, reexamination, interference, or derivation proceedings before the U.S. Patent and Trademark Office or foreign patent offices.

The legal threshold for initiating litigation or contested proceedings is low, so that even lawsuits or proceedings with a low probability of success might be initiated and require significant resources to defend. Litigation and contested proceedings can also be expensive and time-consuming, and our adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we can. The risks of being involved in such litigation and proceedings may increase if our product candidates commence commercialization. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. We may not be aware of all such intellectual property rights potentially relating to our product candidates and their uses. Thus, we do not know with certainty that any of our product candidates or our development and commercialization thereof, do not and will not infringe or otherwise violate any third-party's intellectual property.

If we are found to infringe, misappropriate or otherwise violate a third-party's intellectual property rights, we could be required to obtain a license from such third-party to continue developing, manufacturing, marketing and selling any products, if and when approved, product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us and could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease commercializing the infringing technology, products or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent and could be forced to indemnify our customers or collaborators. A finding of infringement could also result in an injunction that prevents us from commercializing our product candidates or forces us to cease some of our business operations, which could materially harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance, renewal and annuity fees on any issued patent must be paid to the U.S. Patent and Trademark Office and foreign patent agencies in several stages or annually over the lifetime of our owned and licensed patents and patent applications. The U.S. Patent and Trademark Office and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In certain circumstances, we may rely on our licensing partners to pay these fees to, or comply with the procedural and documentary rules of, the relevant patent agency. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it would have a material adverse effect on our business.

KPI-012 is protected by patent rights exclusively licensed from other companies or institutions. If these third parties terminate their agreements with us or fail to maintain or enforce the underlying patents, or we otherwise lose our rights to these patents, our competitive position and our market share in the markets for any of our products, if any when approved, will be harmed.

A portion of our patent portfolio is in-licensed. As such, we are a party to license agreements and certain aspects of our business depend on patents and/or patent applications owned by other companies or institutions. In particular, we hold an exclusive license for a patent family relating to KPI-012. We rely on a license from Stanford University for certain patent rights related to KPI-012. The license agreement between Combangio and Stanford University, or Stanford University License Agreement, imposes specified diligence, milestone payment, royalty and other obligations on us and requires that we meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the license. Our rights with respect to in-licensed patents and patent applications may be lost if the applicable license agreement expires or is terminated or if we fail to satisfy the obligations under the Stanford University License Agreement. We are likely to enter into additional license agreements to in-license patents and patent applications as part of the development of our business in the future, under which we may not retain control of the preparation, filing, prosecution, maintenance, enforcement and defense of such patents. If we are unable to maintain these patent rights for any reason, our ability to develop and commercialize our product candidates could be materially harmed.

Our licensors may not successfully prosecute certain patent applications, the prosecution of which they control, under which we are licensed and on which our business depends. Even if patents issue from these applications, our licensors may fail to maintain these patents, may decide not to pursue litigation against third-party infringers, may fail to prove infringement, or may fail to defend against counterclaims of patent invalidity or unenforceability.

Risks with respect to parties from whom we have obtained intellectual property rights may also arise out of circumstances beyond our control. In spite of our best efforts, our licensors might conclude that we have materially breached our intellectual property agreements and might therefore terminate the intellectual property agreements, thereby removing our ability to market products covered by these intellectual property agreements. If our intellectual property agreements are terminated, or if the underlying patents fail to provide the intended market exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products similar or identical to ours. Moreover, if our intellectual property agreements are terminated, our former licensors and/or assignors may be able to prevent us from utilizing the technology covered by the licensed or assigned patents and patent applications. This could have a material adverse effect on our competitive business position and our financial condition, results of operations and our business prospects.

Some intellectual property which we own or have licensed may have been discovered through government funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for United States industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements, and limit our ability to contract with non-U.S. manufacturers.

Some of the intellectual property rights we own or have licensed have been generated through the use of United States government funding and may therefore be subject to certain federal regulations. For example, certain aspects of KPI-012 were developed using United States government funds. As a result, the United States government may have certain rights to intellectual property embodied in KPI-012 pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole. These United States government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the United States government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third-party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The United States government also has the right to take title to these inventions if we fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. In addition, the United States government may acquire title to these inventions in any country in which a patent application is not filed within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the United States government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for United States manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. Further, to the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of Bayh-Dole may similarly apply. Accordingly, any exercise by the government of any of the foregoing rights could harm our competitive position, business, financial condition, results of operations and prospects.

Moreover, in December 2023, the National Institute of Standards and Technology, or NIST, released for public comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights, or the Draft Framework. The Draft Framework sets forth the factors that an agency may consider when deciding whether to exercise march-in rights pursuant to Bayh-Dole, and includes a first-ever specification that price can be a factor in determining that a drug or other taxpayer-funded invention is not accessible to the public. NIST is currently seeking public comments on the proposed Draft Framework. The potential inclusion of price as a factor in a march-in determination and the exercise of "march-in" rights by the federal government could result in decreased demand for our future products, which could have a material adverse effect on our results of operations and financial condition. In addition, any failure to comply with applicable laws or regulations could harm our business and divert our management's attention.

If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

Our Stanford University License Agreement, under which we license certain patent rights related to KPI-012, imposes royalty and other financial obligations on us and other substantial performance obligations. We also may enter into additional licensing and funding arrangements with third parties that may impose diligence, development and commercialization timelines and milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under current or future license and collaboration agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market any product or product candidate that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could diminish the value of any product or product candidate. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

In addition, it is possible that Stanford may conclude that we have materially breached the Stanford University License Agreement and might therefore terminate the agreement, thereby removing our ability to market products covered by our license agreement with Stanford. If the Stanford University License Agreement is terminated, or if the underlying patents fail to provide the intended market exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products similar or identical to ours. Moreover, if our Stanford University License Agreement is terminated, Stanford and/or its assignors may be able to prevent us from utilizing the technology covered by the licensed or assigned patents and patent applications. If we breach the agreement (including by failing to meet our payment obligations) and do not adequately cure such breach, the rights in the technology licensed to us under the Stanford University License Agreement will revert to Stanford at no cost to Stanford. This could have a material adverse effect on our competitive business position, our financial condition, our results of operations and our business prospects.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize any affected product or product candidate, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

We may not be able to protect our intellectual property and proprietary rights throughout the world.

Filing, prosecuting, and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection or licenses, but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Beginning June 1, 2023, European patent applications and patents may be subjected to the jurisdiction of the Unified Patent Court, or UPC. Under the unitary patent system, European applications will have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the UPC. As the UPC is a new court system, there is minimal precedent for the court, increasing the uncertainty of any litigation. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially

diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations, and prospects may be adversely affected.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our and our licensors' employees and contractors were previously employed at other biotechnology, medical device or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees and contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Furthermore, we are unable to control whether our licensors have obtained similar assignment agreements from their own employees and contractors. Our and their assignment agreements may not be self-executing or may be breached, and we or our licensors may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we or our licensors fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel which could have a material adverse effect on our competitive business position and prospects. Such intellectual property rights could be awarded to a third-party, and we could be required to obtain a license from such third-party to commercialize our technology or products, which may not be available on commercially reasonable terms or at all. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation or other legal proceedings relating to intellectual property could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and may also have an advantage in such proceedings due to their more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have an adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for our technology and our product candidates, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Detecting the disclosure or misappropriation of a trade secret and enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling

to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

Risks Related to Regulatory Approval of Our Product Candidates and Other Legal Compliance Matters

If we are not able to obtain required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate significant revenue will be materially impaired. The marketing approval process is expensive, time-consuming and uncertain. As a result, we cannot predict when or if we, or any collaborators we may have in the future, will obtain marketing approval to commercialize KPI-012 or any product candidates we may develop in the future.

KPI-012 and any other future product candidate and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, potency, purity, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate.

Other than EYSUVIS and INVELTYS, which we sold to Alcon in July 2022, we have not received approval to market any product candidate from regulatory authorities in any jurisdiction. We may never generate the necessary data or results required to obtain regulatory approval of KPI-012 or any other product candidate we may develop with the market potential sufficient to enable us to achieve profitability. We have only limited experience in submitting and supporting the applications necessary to gain marketing approvals and have relied on, and expect to continue to rely on, third-party consultants and vendors to assist us in this process. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish a biologic product candidate's purity, safety and potency. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. The FDA or other regulatory authorities may determine that KPI-012 or any other product candidate that we develop does not satisfy these standards or has undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Further, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA for certain biological products must contain data to assess the safety, potency and purity of the biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe, potent and pure, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety, potency and purity data need to be collected before the pediatric trials begin. The applicable legislation in the EU also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the European Medicines Agency, or EMA, or to obtain a waiver or deferral from the conduct of these studies by this Committee. For any of our product candidates for which we are seeking regulatory approval in the United States or the European Union, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action.

In addition, disruptions at the FDA and other agencies may prolong the time necessary for new biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. The ability of the FDA to review and approve new biologics can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA have fluctuated in recent years. Over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, including as a result of Congress failing to timely raise the U.S. debt ceiling, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Further, our ability to develop and market new products may be impacted by ongoing litigation challenging the FDA's approval of mifepristone. Specifically, in April 2023, the U.S. District Court for the Northern District of Texas stayed the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various conditions adopted under a REMS. In reaching that decision, the district court made a number of findings that may negatively impact the development, approval and distribution of drug products in the United States. In April 2023, the district court decision was stayed, in part, by the U.S. Court of Appeals for the Fifth Circuit. Thereafter, the U.S. Supreme Court entered a stay of the district court's decision, in its entirety, pending disposition of the appeal of the district court decision in the Court of Appeals for the Fifth Circuit and the disposition of any petition for a writ of certiorari to or the Supreme Court. In August 2023, the Court of Appeals declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the Appeals Court did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious. In December 2023, the Supreme Court granted these petitions for writ of certiorari for the appeals court decision.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidate that we develop, the commercial prospects for such product candidate may be harmed and our ability to generate revenues will be materially impaired.

Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad. We may be subject to additional risks in commercializing any of our product candidates that receive marketing approval in foreign jurisdictions.

In order to market and sell KPI-012 or any other product candidate we may develop in the European Union and many other jurisdictions outside of the United States, we or our potential third-party collaborators, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. Clinical trials of any product candidate in the United States may not be sufficient to support an application for marketing approval outside the United States.

The time required to obtain approval outside of the United States may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be sold in that country. We or our potential collaborators may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in other countries. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market, which could significantly and materially harm our business.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the European Union pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (including potentially reducing the duration of regulatory data protection and revising the eligibility for expedited pathways) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European

Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may however have a significant impact on the pharmaceutical industry and our business in the long term.

Even if our product candidates receive regulatory approval, they will be subject to significant post-marketing regulatory requirements and oversight.

Any regulatory approvals that we may receive for our product candidates will require the submission of reports to regulatory authorities and ongoing surveillance to monitor the safety and efficacy of the product candidate, may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements and regulatory inspection. For example, the FDA may require a REMS in order to approve our product candidates, which could entail requirements for a medication guide, physician training and communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA, EMA or foreign regulatory authorities approve our product candidates, the manufacturing processes, labelling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as ongoing compliance with cGMPs and GCPs for any clinical trials that we conduct post-approval.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA, EMA and other regulatory authorities for compliance with cGMP regulations and standards. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a drug or biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States. If we or a regulatory authority discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facilities where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. In addition, failure to comply with FDA, EMA and other comparable foreign regulatory requirements may subject our company to administrative or judicially imposed sanctions, including:

- delays in or the rejection of product approvals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on the products, manufacturers or manufacturing process;
- warning or untitled letters;
- civil and criminal penalties;
- injunctions;
- suspension or withdrawal of regulatory approvals;
- product seizures, detentions or import bans;
- voluntary or mandatory product recalls and publicity requirements;
- total or partial suspension of production;
- imposition of restrictions on operations, including costly new manufacturing requirements;
- revisions to the labelling, including limitation on approved uses or the addition of additional warnings, contraindications or other safety information, including boxed warnings;
- imposition of a REMS, which may include distribution or use restrictions; and
- requirements to conduct additional post-market clinical trials to assess the safety of the product.

The FDA, EMA and other regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses, and if we are found to have improperly promoted such off-label uses, we may become subject to significant liability.

If any of our product candidates are approved and we are found to have improperly promoted off-label uses of those products, we may become subject to significant liability, which would materially adversely affect our business and financial condition. The FDA, EMA and other regulatory authorities strictly regulate the promotional claims that may be made about prescription products. In particular, a product may not be promoted in the United States for uses that are not approved by the FDA as reflected in the product's approved labelling, or in other jurisdictions for uses that differ from the labelling or uses approved by the applicable regulatory authorities. While physicians may prescribe products for off-label uses, the FDA, EMA and other regulatory authorities actively enforce laws and regulations that prohibit the promotion of off-label uses by companies, including promotional communications made by companies' sales force with respect to off-label uses that are not consistent with the approved labelling. The U.S. federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific communications concerning their products in certain circumstances. For example, in October 2023, the FDA published draft guidance outlining the agency's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. In addition, under some relatively recent guidance from the FDA and the Pre-Approval Information Exchange Act signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices.

We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

We may not be able to obtain orphan drug exclusivity for one or more of our product candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products. Additionally, if another company with a competing product candidate were to obtain orphan drug exclusivity for its competing product candidate before we do, we may be barred from marketing our product candidate for the same indication as the competing product candidate during the exclusivity period.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. KPI-012 has received orphan drug designation from the FDA for the treatment of PCED.

Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified. If a competing product candidate with an orphan designation for PCED were to obtain regulatory approval before we are able to obtain approval of KPI-012 for PCED, we could be barred from marketing KPI-012 for PCED in the United States during the seven-year orphan exclusivity period, which would have a severe adverse effect on our business.

In order for the FDA to grant orphan drug exclusivity to one of our products, the FDA must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which orphan drug exclusivity is sought does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition.

In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

The FDA Reauthorization Act of 2017, or FDARA, requires that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. FDARA reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the Court of Appeals concluded that orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, FDA announced that, in matters beyond the scope of that court order, the FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

We may seek certain designations for our product candidates, including Breakthrough Therapy, Fast Track and Priority Review designations in the United States, and PRIME Designation in the European Union, but we might not receive such designations, and even if we do, such designations may not lead to a faster development or regulatory review or approval process.

We may seek certain designations for one or more of our product candidates that could expedite review and approval by the FDA. A Breakthrough Therapy product is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as Breakthrough Therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens.

The FDA may also designate a product for Fast Track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For Fast Track review products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a Fast Track product's application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a Fast Track review product may be effective. In April 2023, the FDA designated KPI-012 for the treatment of PCED for Fast Track review.

We may also seek a priority review designation for one or more of our product candidates. If the FDA determines that a product candidate offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal is for the FDA to review an application for marketing approval in six months, rather than the standard review period of 10 months.

These designations are within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for these designations, the FDA may disagree and instead determine not to make such designation. Further, even if we receive a designation, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to product candidates considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualifies for these designations, such as the Fast Track designation for KPI-012 for the treatment of PCED, the FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

In the European Union, we may seek PRIME designation for some of our product candidates in the future. The PRIME program focuses on product candidates that target conditions for which there exists no satisfactory method of treatment in the European Union, or even if such a method exists, the product candidate may offer a major therapeutic advantage over existing treatments. To be accepted for PRIME designation, a product candidate must meet the eligibility criteria in respect of its major public health interest and therapeutic innovation based on information that is capable of substantiating the claims. The benefits of a PRIME designation include the appointment of a rapporteur of the Committee for Medicinal Products for Human Use to provide continued support and help to build knowledge ahead of a marketing authorization application, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review, meaning reduction in the review time for an opinion on approvability to be issued earlier in the application process. PRIME designation enables an applicant to request parallel EMA scientific advice and health technology assessment advice to facilitate timely market access. Even if we receive PRIME designation for any of our product candidates, the designation may not result in a materially faster development process, review or approval compared to conventional EMA procedures. Further, obtaining PRIME designation does not assure or increase the likelihood of EMA's grant of a marketing authorization.

If approved, our products regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biologic products that are biosimilar to or interchangeable with an FDA-licensed reference biologic product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of regulatory exclusivity, another company may still market a competing version of the reference product if the FDA approves a BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity, and potency of the other company's product.

In December 2022, Congress clarified through the Food and Drug Omnibus Reform Act, that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the same first day on which such a product is approved as interchangeable with the reference product and the exclusivity period may be shared amongst multiple first interchangeable products. More recently, in October 2023, the FDA issued its first interchangeable exclusivity determination under the BPCIA.

To date, we have not had a product candidate approved as a biologic product. We believe that any of our product candidates that may be approved as a biologic product under a BLA should qualify for the 12-year period of exclusivity. Nonetheless, the approval of biosimilar products referencing any of our product candidates would have a material adverse impact on our business due to increased competition and pricing pressures. Moreover, there is a risk that any exclusivity we do receive could be shortened due to congressional action or otherwise, or that the FDA will not consider our products to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. The extent to which a biosimilar, once licensed, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biologic products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing. If competitors are able to obtain regulatory approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

The ultimate impact, implementation, and meaning of the BPCIA are subject to uncertainty, and any new regulations, guidance, policies or processes adopted by the FDA to implement the law could have a material adverse effect on the future commercial prospects for our biological product candidates.

Our relationships with customers and third-party payors may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, transparency, health information privacy and security, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings.

Healthcare providers, clinicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription and use of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it in order to have committed a violation;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti- Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, and their respective implementing regulations, which imposes obligations, including mandatory contractual terms, on covered healthcare providers, health plans and healthcare clearinghouses, as well as their business associates, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or transfers of value made to physicians, other healthcare providers and teaching hospitals, as well as information regarding ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers, state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to clinicians and other healthcare providers or marketing expenditures, and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations are found to be in violation of any of the laws described above or any governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, individual imprisonment, integrity obligations, and the curtailment or restructuring of our operations. Any penalties, damages, fines, individual imprisonment, integrity obligations, exclusion from funded healthcare programs, or curtailment or restructuring of our operations could adversely affect our financial results. Our corporate compliance program is designed to ensure that we will develop, market and sell our products and product candidates in compliance with all applicable laws and regulations, but we cannot guarantee that this program will protect us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the clinicians or other healthcare providers or entities with whom we do or expect to do business is found to be not in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government funded healthcare programs.

Existing and future legislation may affect our ability to commercialize our products, if and when approved, and increase the difficulty and cost for us to obtain reimbursement for our products, if and when approved.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could affect our ability to profitably sell or commercialize any product candidate for which we obtain marketing approval. The pharmaceutical industry has been a particular focus of these efforts and have been significantly affected by legislative initiatives. Current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any FDA approved product.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the ACA. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. For example, in August 2021, the Budget Control Act of 2011, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year which went into effect in 2013 and will remain in effect through the first half of 2032.

The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any product candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory PAYGO sequester for two years, through the end of calendar year 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act's health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into fiscal year 2032 and lowers the payment reduction percentages in fiscal years 2030 and 2031.

We expect that additional healthcare reforms may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any product which receives regulatory approval and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or

administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

Since enactment of the ACA, there have been and continue to be numerous legal challenges and Congressional actions to repeal and replace provisions of the law and litigation and legislation over the ACA is likely to continue with unpredictable and uncertain results. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the 2017 Tax Act, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which required most Americans to carry a minimal level of health insurance, became effective in 2019.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, but those were rescinded by the Biden Administration. President Biden issued an executive order which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this executive order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents.

We expect that additional healthcare reforms may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any product which receives regulatory approval and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

Current and future legislation designed to reduce prescription drug costs may affect the prices we and any collaborators may obtain for our product candidates.

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In 2020, President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

In October 2020, Health Insurance Portability and Accountability Act of 1996, or HHS, and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by PhRMA but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. At least nine states have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. On January 5, 2024, the FDA approved Florida's plan for Canadian drug importation.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers. Pursuant to court order, the removal and addition of the aforementioned safe

harbors were delayed and recent legislation imposed a moratorium on implementation of the rule until January 1, 2026. The Inflation Reduction Act of 2022, or IRA, further delayed implementation of this rule to January 1, 2032.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028 and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be at risk of government action if our products are the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on our products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $4,000 a year in 2024 and, thereafter beginning in 2025, at 2,000 a year.

Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition. For example, based on current guidance from CMS concerning the application of the IRA's drug pricing provisions to orphan drugs, we may be eligible for reduced reimbursement if and when, if ever, KPI-012 is approved as an orphan drug for PCED and a different rare disease or condition.

On June 6, 2023, Merck & Co. filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, other parties, including the U.S. Chamber of Commerce and certain pharmaceutical companies have also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results.

Further, in December 2023, NIST released for public comment a Draft Interagency Guidance Framework for Considering the Exercise of March-In Rights, or the "Draft Framework. The Draft Framework sets forth the factors that an agency may consider when deciding whether to exercise march-in rights pursuant to Bayh-Dole, and includes a first-ever specification that price can be a factor in determining that a drug or other taxpayer-funded invention is not accessible to the public. NIST is currently seeking public comments on the proposed Draft Framework. The potential inclusion of price as a factor in a march-in determination and the exercise of "march-in" rights by the federal government could result in decreased demand for our future products, which could have a material adverse effect on our results of operations and financial condition. In addition, any failure to comply with applicable laws or regulations could harm our business and divert our management's attention.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

If we or any third-party manufacturers we engage or may engage in the future fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur significant costs.

We and any third-party manufacturers we engage or may engage in the future are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous materials, including chemicals and biological materials, and produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain general liability insurance as well as workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Further, with respect to the operations of any future third-party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, be precluded from developing, manufacturing and selling certain products outside the United States or be required to develop and implement costly compliance programs, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.S. Foreign Corrupt Practices Act, or FCPA, the U.K. Bribery Act 2010, or Bribery Act, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The FCPA, Bribery Act and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. Compliance with the FCPA, in particular, is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

We may in the future operate in jurisdictions that pose a high risk of potential FCPA or Bribery Act violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to

liability under the FCPA, Bribery Act or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted. If we expand our operations outside of the United States, we will need to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the FCPA, the Bribery Act or other legal requirements, including Trade Control laws. If we are not in compliance with the FCPA, Bribery Act and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Any investigation of any potential violations of the FCPA, the Bribery Act, other anti-corruption laws or Trade Control laws by U.S., U.K. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the U.S., EU and U.K. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be

interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

In addition to potential enforcement by HHS, we are also potentially subject to privacy enforcement from the Federal Trade Commission, or the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the Federal Trade Commission Act, as well as the types of activities it views to trigger the Health Breach Notification Rule, which the FTC also has the authority to enforce. The FTC is also in the process of developing rules related to commercial surveillance and data security that may impact our business. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate our risk for a potential enforcement action, which may be costly. If we are subject to a potential FTC enforcement action, we may be subject to a settlement order that requires us to adhere to very specific privacy and data security practices, which may impact our business. We may also be required to pay fines as part of a settlement, depending on the nature of the alleged violations. If we violate any consent order that we reach with the FTC, we may be subject to additional fines and compliance requirements.

States are also active in creating specific rules relating to the processing of personal information. In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, described below, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. The California Privacy Rights Act, or the CPRA, went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA, and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, a number of other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering or have already passed comprehensive privacy laws during the 2024 legislative sessions. Other states will be considering these laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Similar to the laws in the United States, there are significant privacy and data security laws that apply in Europe, Latin America and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the European Economic Area, or EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

The GDPR places restrictions on the cross-border transfer of personal data from the EU to countries that have not been found to offer adequate data protection legislation, such as the United States. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the United States. The CJEU decision has resulted in increased scrutiny on data transfers generally and may increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The European Commission adopted the adequacy decision in July 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business.

Beyond GDPR and similar laws in the United States, there are privacy and data security laws in a growing number of countries around the world, including countries in Latin America where we are planning to open trial sites in the CHASE Phase 2b clinical trial. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws may impact our ability to conduct our business activities.

While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken by data protection authorities in the EEA and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant. Similarly, failure to comply with federal and state laws in the United States regarding privacy and security of personal information could expose us to penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.

As of December 31, 2023, we had federal net operating loss, or NOL, carryforwards of $369.3 million, which may be available to offset future federal tax liabilities and expire at various dates beginning in 2030. As of December 31, 2023, we also had state NOL carryforwards of $413.7 million, which may be available to offset future state income tax liabilities and expire at various dates beginning in 2024. As of December 31, 2023, we had $1,154 federal and state research and development credit carryforwards. Our NOL carryforwards could expire unused and be unavailable to offset our future income tax liabilities.

In general, under Sections 382 and 383 of the Code, the amount of benefits from our NOL and research and development tax credit carryforwards, respectively, may be impaired or limited if we incur an "ownership change," generally defined as a greater than 50% change (by value) in our equity ownership by certain stockholders, over a three-year period. We previously completed an analysis and determined that an ownership change has materially limited our net operating loss carryforwards and research and development tax credits available to offset future tax liabilities. During December 2022, an additional ownership change occurred as a result of our entry into the securities purchase agreement for the private placement transaction. As a result of this ownership change, the utilization of our net operating loss carryforwards is subject to an annual limitation of $0.2 million. We may be further limited by any changes that may have occurred or may occur subsequent to December 31, 2022. Any such limitations may result in greater tax liabilities than we would incur in the absence of such limitations and increased liabilities could adversely affect our business,

results of operations, financial position and cash flows. If our ability to use our historical NOL and research and development tax credit carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs and research and development tax credit carryforwards could expire or otherwise become unavailable to offset future income tax liabilities. As described below in "Changes in tax laws or in their implementation or interpretation could adversely affect our business and financial condition," the 2017 Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that have significantly impacted our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we will likely be unable to use a material portion of our NOLs and other tax attributes.

Risks Related to Employee Matters

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the research and development, clinical, business development and commercialization expertise of Mark Iwicki, our Chief Executive Officer, Todd Bazemore, our President and Chief Operating Officer, Mary Reumuth, our Chief Financial Officer, Kim Brazzell, Ph.D., our Head of Research and Development and Chief Medical Officer, Darius Kharabi, our Chief Business Officer, and Eric L. Trachtenberg, our Chief Legal Officer, Chief Compliance Officer and Corporate Secretary, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment agreements with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. In addition, we are highly dependent on the employees who joined us in connection with the Combangio Acquisition and their expertise developing biologics.

Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and other personnel will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. Our decision to sell our Commercial Business to Alcon, our determination to solely focus our research and development efforts on our MSC-S platform, including KPI-012, and our workforce reduction completed during the second half of 2022 could harm our ability to attract and retain qualified personnel who are critical to our business. In addition, we rely on consultants and advisors, including scientific, clinical and regulatory advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to successfully develop and commercialize KPI-012 and any other product candidate we may develop in the future will be harmed.

Our internal computer systems, or those of our vendors, contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite the implementation of security measures, our information technology systems and those of our current and any future vendors, contractors or consultants, including any collaborator, are vulnerable to damage from cyber-attacks, computer viruses, worms and other destructive or disruptive software, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Cyber incidents or attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, unauthorized access to or deletion of files, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information.

Cyber incidents also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient. System failures, accidents, cyberattacks or security breaches could cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential, personal or proprietary information, we could incur liability, including civil fines and penalties under the GDPR, HIPAA and other relevant state and federal privacy laws in the United States and abroad, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed. In addition, we may not have adequate insurance coverage to provide compensation for any losses associated with such events.

While we have not experienced any material losses relating to cyber-attacks, we have been the subject of a successful phishing attempt. We could be subject to risks caused by misappropriation, misuse, leakage, falsification or intentional or accidental release or loss of information maintained in the information systems and networks of our company, including personal information of our employees. In addition, outside parties may attempt to penetrate our systems or those of our vendors, contractors or consultants or fraudulently induce our employees or employees of our vendors, contractors or consultants to disclose sensitive information in order to gain access to our data. Like other companies, we may experience threats to our data and systems, including malicious codes and viruses, and other cyber-attacks. The number and complexity of these threats continue to increase over time. If a material breach of our security or that of our vendors, contractors or consultants occurs, the market perception of the effectiveness of our security measures could be harmed, we could lose business and our reputation and credibility could be damaged. We could be required to expend significant amounts of money and other resources to repair or replace information systems or networks. Although we develop and maintain systems and controls designed to prevent these events from occurring, and we have a process to identify and mitigate threats, the development and maintenance of these systems, controls and processes is costly and requires ongoing monitoring and updating as technologies change and efforts to overcome security measures become more sophisticated. Moreover, despite our efforts, the possibility of these events occurring cannot be eliminated entirely.

A partially or fully remote workplace could negatively impact our business.

We terminated our lease for office and laboratory space at our former corporate headquarters in Watertown, Massachusetts, effective January 11, 2022. While we have retained a nominal amount of office space on a short-term basis to conduct in-person meetings from time-to-time in Arlington, Massachusetts and lease office and laboratory space in Menlo Park, California, the vast majority of our employees no longer have individual offices. As a result, our management team and the vast majority of our employees will work remotely and without dedicated office space, until such time as we determine to obtain a new operating lease. By migrating to a remote workforce, our employees are accessing our servers remotely through home or other networks to perform their job responsibilities, which may be less secure. The risk of cyber incidents or other privacy or data security incidents may be heightened as a result of our remote work environment. Remote working arrangements could also impact employee productivity and morale, impede employee training, strain our technology resources and introduce operational risks, all of which could negatively impact our business. Furthermore, our transition to a largely remote workplace will increase our reliance on third parties to conduct a significant portion of our research and development activities. We have limited ability to control the amount or timing of resources that any such third party will devote to our research and development activities, and such third parties may terminate their engagements with us at any time. We also expect to have to negotiate budgets and contracts with such third parties, and we may not be able to do so on favorable terms, which may result in delays to our development timelines and increased costs.

Risks Related to Our Common Stock

If we fail to comply with the continued listing requirements of Nasdaq, our common stock may be delisted and the price of our common stock and our ability to access the capital markets could be negatively impacted. If our common stock is delisted from Nasdaq, we will be in default under our Loan Agreement.

Our common stock is currently listed on The Nasdaq Capital Market. We must satisfy Nasdaq's continued listing requirements, including, among other things, a minimum closing bid price of $1.00 per share and either a minimum stockholders' equity of $2,500,000, or a minimum market value of our common stock of at least $35,000,000, or risk delisting, which would have a material adverse effect on our business. There are many factors that may adversely affect our ability comply with the requirements for continued listing on The Nasdaq Capital Market, including those described throughout this "Risk Factors" section. Many of these factors are outside of our control. As a result, we cannot assure you that we will continue to comply with the requirements for continued listing on The Nasdaq Capital Market, including the minimum stockholders' equity requirement.

A delisting of our common stock from Nasdaq could materially reduce the liquidity of our common stock and result in a corresponding material reduction in the price of our common stock. In addition, delisting could harm our ability to raise capital through alternative financing sources on terms acceptable to us, or at all, and may result in the potential loss of confidence by investors and employees and fewer business development opportunities. In addition, any potential delisting of our common stock from Nasdaq would also make it more difficult for our stockholders to sell their shares in the public market.

We have a history of receiving deficiency letters from Nasdaq. During 2022, we received multiple deficiency letters from Nasdaq notifying us of our noncompliance with various listing standards for continued inclusion on The Nasdaq Global Select Market. On each of March 2, 2022 and May 24, 2022, we received a deficiency letter from Nasdaq notifying us that, for 30 consecutive business days, the bid price of our common stock had closed below the $1.00 per share minimum bid price requirement for continued inclusion on The Nasdaq Global Select Market pursuant to Nasdaq Listing Rule 5450(a)(1), or the Bid Price Requirement. We were provided a period of 180 calendar days to regain compliance with the Bid Price Requirement, and in each case, we regained compliance within the cure period, including in the second instance by implementing a reverse stock split of our common stock.

On July 6, 2022, we received another deficiency letter from Nasdaq notifying us that we were not in compliance with Nasdaq Listing Rule 5450(b)(2)(A), or the Minimum MVLS Requirement, for continued listing on The Nasdaq Global Select Market, as the market value of our common stock was less than $50,000,000 for the previous 30 consecutive business days. Nasdaq also noted that we were not in compliance with Nasdaq Listing Rule 5450(b)(1)(A), as our stockholders' equity was less than $10,000,000 and Nasdaq Listing Rule 5450(b)(3)(A), as our total assets and total revenue for the most recently completed fiscal year or for two of the three most recently completed fiscal years were less than $50,000,000. A company that has its primary equity security listed on The Nasdaq Global Select Market must satisfy at least one of the standards in Nasdaq Listing Rule 5450(b).

On December 5, 2022, we received another deficiency letter from Nasdaq notifying us that we were not in compliance with Nasdaq Listing Rule 5450(b)(2)(C), or the Minimum MVPHS Requirement, for continued listing on The Nasdaq Global Select Market, as the market value of our publicly held shares was less than $15,000,000 for each of the previous 30 consecutive business days.

In accordance with Nasdaq Listing Rule 5810(c)(3), we were provided until January 2, 2023 to regain compliance with the Minimum MVLS Requirement and until June 5, 2023 to regain compliance with the Minimum MVPHS Requirement. Alternatively, if we did not regain compliance with the Minimum MVLS Requirement or the Minimum MVPHS Requirement by the applicable compliance date, we were eligible to transfer the listing of our common stock to The Nasdaq Capital Market, provided that we met the applicable requirements for continued listing on The Nasdaq Capital Market.

Following the receipt of the proceeds from the second tranche of a private placement in December 2022 and after amending our Loan Agreement to permit a transfer, we applied to transfer the listing of our common stock to The Nasdaq Capital Market. The transfer was approved effective January 11, 2023 following Nasdaq's determination that we

met the applicable requirements for continued listing on The Nasdaq Capital Market, including Nasdaq Listing Rule 5550(b)(1), the minimum stockholders equity requirement for continued listing on The Nasdaq Capital Market. In addition, Nasdaq advised us that, upon the transfer of our listing to The Nasdaq Capital Market, we would be in compliance with Nasdaq Listing Rule 5550(a)(5), the market value of publicly held shares requirement for continued listing on The Nasdaq Capital Market.

Any delisting of our common stock from The Nasdaq Capital Market or a transfer of the listing of our common stock to another nationally recognized stock exchange having listing standards that are less restrictive than The Nasdaq Capital Market, in each case after a specified cure period, are events of default under our Loan Agreement, which could adversely effect our financial condition and ability to pursue our business strategy.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors are responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- provide for a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- provide for advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three-years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

An active trading market for our common stock may not be sustained.

From July 20, 2017 through January 10, 2023, our common stock traded on The Nasdaq Global Select Market. On January 11, 2023, our common stock began trading on The Nasdaq Capital Market. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price for our common stock and thereby affect your ability to sell your shares. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for purchasers of our common stock.

Our stock price is volatile and fluctuates substantially. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the price you paid for such common stock. The market price for our common stock may be influenced by many factors, including:

- whether we receive, and the amount of, any future milestone payments from Alcon in connection with the sale of our Commercial Business;
- our strategic decision to focus our research and development efforts on our MSC-S platform, including KPI-012;
- results of preclinical studies and clinical trials of KPI-012 or any other product candidates we may develop;
- our ability to receive marketing approval for and to successfully commercialize KPI-012 or any other product candidate we may develop;
- results of clinical trials of product candidates of our competitors;
- changes in the structure of healthcare payment systems;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key scientific, commercial or management personnel;
- the level of expenses related to the development of KPI-012 and any other product candidate we develop;
- the results of our efforts to discover, develop, acquire or in-license additional products, product candidates or technologies for the treatment of diseases or conditions, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- sales of common stock by us, our executive officers, directors or principal stockholders, or others, or the anticipation of such sales;
- variations in our financial results or those of companies that are perceived to be similar to us;
- market conditions in the pharmaceutical and biotechnology sectors;
- the societal and economic impact of public health epidemics, such as the COVID-19 pandemic;
- general economic, industry and market conditions;
- political instability in the United States and Europe, including as a result of Congress failing to timely raise the U.S. debt ceiling; or
- the other factors described in this “Risk Factors” section.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation has often been instituted against that company. We also may face securities class-action litigation if we cannot obtain regulatory approval for or fail to successfully commercialize KPI-012 or any other product candidate we develop. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management’s attention and resources.

Sale of a substantial number of shares of our common stock could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of March 28, 2024, we had outstanding 2,816,454 shares of common stock.

Shares of our common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended, or the Securities Act, or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates of ours. If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline. In addition, we have filed or intend to file registration statements registering all shares of common stock that we may issue under our equity compensation plans or pursuant to equity awards made to newly hired employees outside of equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates.

In December 2022, we sold to certain institutional investors shares of our common stock and shares of our Series E Convertible Non-Redeemable Preferred Stock in a private placement. We have filed a registration statement on Form S-3 covering the resale of the common stock held by such investors in the private placement and the common stock issuable upon conversion of the Series E Preferred Stock issued in the private placement, and we have agreed to keep such registration statement effective until the date the shares covered by it have been sold or can be resold without restriction under Rule 144 of the Securities Act. In December 2023 and in March 2024, we also sold to certain institutional investors in private placements shares of our Series F Convertible Non-Redeemable Preferred Stock and shares of our Series G Convertible Non-Redeemable Preferred Stock, respectively. We have agreed to register for resale the shares of common stock issuable upon conversion of such preferred stock, upon demand by the investors.

The sale or resale of these shares in the public market, or the market's expectation of such sales, may result in an immediate and substantial decline in our stock price. Such a decline will adversely affect our investors and also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Our existing stockholders will experience dilution upon any future conversion of the outstanding shares of our preferred stock into shares of our common stock.

Each outstanding share of Series E Convertible Non-Redeemable Preferred Stock, Series F Convertible Non-Redeemable Preferred Stock and Series G Convertible Non-Redeemable Preferred Stock is initially convertible into 100 shares of our common stock at any time at the option of the holder, subject to certain beneficial ownership limitations. Our existing stockholders will experience dilution upon any future conversion of the outstanding shares of our Series E Convertible Non-Redeemable Preferred Stock, Series F Convertible Non-Redeemable Preferred Stock or Series G Convertible Non-Redeemable Preferred Stock into shares of our common stock.

Our largest stockholder may have the ability to exercise significant influence over certain of our business decisions and could influence matters submitted to stockholders for approval.

Entities affiliated with our largest stockholder owned, in the aggregate, shares of common stock representing approximately 9.47% of our outstanding common stock as of March 28, 2024. Such stockholder also holds all of the outstanding shares of our Series E Convertible Non-Redeemable Preferred Stock, the Series F Convertible Non-Redeemable Preferred Stock and the Series G Convertible Non-Redeemable Preferred Stock.

Pursuant to the terms of the certificates of designation governing our outstanding shares of preferred stock, such stockholder can elect to convert its shares of preferred stock into shares of common stock at any time, provided that it would not own, following such conversion, in excess of 9.99% of the outstanding shares of our common stock. Such stockholder can also elect for its beneficial ownership limitation to be increased up to 19.99% upon 61 days' notice. If such stockholder elects to convert its shares of preferred stock into common stock and/or increase its beneficial ownership limitations to up to 19.99%, it would hold a significant percentage of our outstanding shares of common stock and could exercise significant influence matters submitted to our stockholders for approval.

In addition, pursuant to the terms of our securities purchase agreements for the private placement transactions, we have agreed that we will not, without the prior approval of such stockholder (1) issue or authorize the issuance of any equity security that is senior or pari passu to the Series E Convertible Non-Redeemable Preferred Stock, the Series F Convertible Non-Redeemable Preferred Stock or the Series G Convertible Non-Redeemable Preferred Stock with respect to liquidation preference, (2) incur any additional indebtedness for borrowed money in excess of $1.0 million, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of our existing indebtedness or (3) pay or declare any dividend or make any distribution on, any of our shares of capital stock, subject to specified exceptions. As a holder of our Series E Preferred Stock, the stockholder has the right to have our board of directors nominate and recommend for election by the stockholders up to three designees to our board of directors, subject to certain requirements and exceptions. In addition, as a holder of our Series E Preferred Stock, such stockholder has certain rights to participate in our future equity offerings, which rights are more fully described in Item 1, "Business" of our Annual Report on Form 10-K for the year ended December 31, 2023.

As a result of the foregoing, our largest stockholder may have the ability to exercise significant influence over certain matters affecting our business. Such stockholder may have interests that differ from your interests, and it may vote as a stockholder or act in a way with which you disagree and that may be adverse to your interests. The concentration of ownership of our capital stock may have the effect of delaying, preventing or deterring a change of control of our company, could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company and may adversely affect the market price of our common stock.

We are a "smaller reporting company", and the reduced disclosure requirements applicable to smaller reporting companies may make our common stock less attractive to investors.

We are a "smaller reporting company," as defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended. We would cease to be a smaller reporting company if we have a public float in excess of $250 million or have annual revenues in excess of $100 million and a public float in excess of $700 million, determined on an annual basis.

As a smaller reporting company, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not smaller reporting companies. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- reduced disclosure obligations regarding executive compensation;
- being permitted to provide only two years of audited financial statements in our annual report on Form 10-K, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure; and
- not being required to furnish a stock performance graph in our annual report.

We cannot predict whether investors will find our common stock less attractive as a result of our reliance on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We have incurred and will continue to incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives and corporate governance practices.

As a public company, and particularly since we ceased being an "emerging growth company", we incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Capital Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs relative to prior years and will make some activities more time-consuming and costly.

For as long as we remain a smaller reporting company, we may take advantage of certain exemptions from various reporting requirements as described in the preceding risk factor.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain a non-accelerated filer and a smaller reporting company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of our Loan Agreement and our securities purchase agreements entered into with certain institutional investors for our 2022, 2023 and 2024 private placements restrict us from paying dividends. Any future debt agreements that we may enter into may preclude us from paying dividends without the lenders' consent or at all. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Our certificate of incorporation designates the state courts in the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors, officers and employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws or as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware, or any action asserting a claim against us governed by the internal affairs doctrine. We do not expect this choice of forum provision will apply to suits brought to enforce a duty or liability created by the Securities Act, the Exchange Act, or any other claim for which federal courts have exclusive jurisdiction.

This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur

additional costs associated with resolving such action in other jurisdictions, which could materially adversely affect our business, financial condition and operating results.

General Risk Factors

Changes in tax laws or in their implementation or interpretation could adversely affect our business and financial condition.

Changes in tax law may adversely affect our business or financial condition. The 2017 Tax Act, as amended by the CARES Act, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21% and the limitation of the deduction for NOLs to 80% of current year taxable income for losses arising in taxable years beginning after December 31, 2017 (though any such NOLs may be carried forward indefinitely). In addition, beginning in 2022, the 2017 Tax Act eliminates the option to deduct research and development expenditures currently and requires corporations to capitalize and amortize them over five years or 15 years for expenditures attributable to foreign research.

In addition to the CARES Act, as part of Congress's response to the COVID-19 pandemic, economic relief legislation was enacted in 2020 and 2021 containing tax provisions. The Inflation Reduction Act, or IRA, was also signed into law in August 2022. The IRA introduced new tax provisions, including a one percent excise tax imposed on certain stock repurchases by publicly traded companies. The one percent excise tax generally applies to any acquisition of stock by the publicly traded company (or certain of its affiliates) from a stockholder of the company in exchange for money or other property (other than stock of the company itself), subject to a de minimis exception. Thus, the excise tax could apply to certain transactions that are not traditional stock repurchases.

Regulatory guidance under the 2017 Tax Act, the IRA, and such additional legislation is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. In addition, it is uncertain if and to what extent various states will conform to the 2017 Tax Act, the IRA and such additional legislation.

Patent reform legislation under Leahy-Smith America Invents Act could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

On September 16, 2011, Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent Office has been developing new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. The first to file provisions limit the rights of an inventor to patent an invention if not the first to file an application for patenting that invention, even if such invention was the first invention. Although it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, which could have a material adverse effect on our business, financial condition, results of operations and prospects. For example, the Leahy-Smith Act provides a new administrative tribunal known as the Patent Trial and Appeals Board, or PTAB, that provides a venue for companies to challenge the validity of competitor patents at a cost that is much lower than district court litigation and on timelines that are much faster. Although it is not clear what, if any, long term impact the PTAB proceedings will have on the operation of our business, the initial results of patent challenge proceedings before the PTAB since its inception in 2013 have resulted in the invalidation of many U.S. patent claims. The availability of the PTAB as a lower-cost, faster and potentially more potent tribunal for challenging patents could therefore increase the likelihood that our own patents will be challenged, thereby increasing the uncertainties and costs of maintaining, defending and enforcing them.

Item 1B. Unresolved Staff Comments

None.

Item 1C. Cybersecurity

We have certain processes for assessing, identifying and managing cybersecurity risks, which are designed to help protect our information assets and operations from internal and external cyber threats, as well as secure our networks and systems. Such processes, which are effected principally through an outside information technology management/cybersecurity consultant and a computer security firm that we have engaged, include procedural and technical safeguards, response plans, incident simulations and routine review of our policies and procedures to identify risks and refine our practices. Our computer security firm serves as our managed security services provider, and its services include managed detection and response, incident management, managed security awareness and a quarterly risk assessment. Our information technology management/cybersecurity consultant has responsibility for managing detection and incident response in consultation with out our managed security services provider. We considered the internal risk oversight programs of our information technology management/cybersecurity consultant and our managed security services provider before engaging them. As part of our overall risk mitigation strategy, we also maintain cyber insurance coverage; however, such insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related breaches.

While we have not experienced any material losses relating to cyber-attacks, in 2019 we were the subject of a successful phishing attempt. We do not believe that there are currently any known risks from cybersecurity threats that are reasonably likely to materially affect us or our business strategy, results of operations or financial condition.

The Audit Committee of our Board of Directors, or the Audit Committee, provides direct oversight over cybersecurity risk. Our Audit Committee and Board of Directors receive periodic updates from our Chief Legal Officer and Chief Compliance Officer, together with our outside information technology management/cybersecurity consultant, and the Audit Committee and Board of Directors is notified between such updates regarding significant new cybersecurity threats or incidents.

Our Chief Legal Officer and Chief Compliance Officer is responsible for the management oversight of company-wide cybersecurity strategy, policy, standards and processes and works across relevant departments to assess and help prepare us to address cybersecurity risks. Our Chief Legal Officer and Chief Compliance Officer has many years of experience overseeing company-wide legal and compliance risks, including at multiple publicly-traded companies. Our Chief Legal Officer and Chief Compliance Officer is supported by our outside information technology management/cybersecurity consultant and our managed security services provider.

We have also established a cross-functional Cybersecurity Committee led by our Chief Legal Officer and Chief Compliance Officer serving as the chair and consisting of senior leaders within our organization. The Cybersecurity Committee, with assistance from our outside information technology management/cybersecurity consultant, oversees our cybersecurity policy, which includes risk assessment, investments in cybersecurity technologies, cybersecurity insurance and review of relevant information technology policies.

In an effort to deter and detect cyber threats, we provide all employees, including part-time and temporary employees, with periodic cybersecurity training. This training program covers timely and relevant topics, including social engineering, phishing, password protection, confidential data protection, asset use and mobile security, and educates employees on the importance of reporting all cybersecurity incidents immediately. We also use technology-based tools to mitigate cybersecurity risks and to bolster our employee-based cybersecurity programs.

Item 2. Properties

We currently lease a limited amount of office space in Arlington, Massachusetts, which serves as our corporate headquarters.

Combangio, our wholly-owned subsidiary as a result of the Combangio Acquisition, entered into a space sharing agreement with Lagunita, LLC on October 11, 2019, pursuant to which it subleased 1,550 square feet of shared office and lab space. The term of the space-sharing agreement expired on June 30, 2023.

In April 2023, Combangio entered into a lease agreement with Menlo Prepi I, LLC, pursuant to which Combangio leases approximately 6,135 square feet of office, laboratory and research and development space in Menlo

Park, California. The term of the lease commenced on July 1, 2023. The initial term of the lease is for 62 months, unless earlier terminated. The lease provides Combangio with an option to extend the lease for an additional five-year term.

Item 3. Legal Proceedings

We are not currently subject to any material legal proceedings.

Item 4. Mine Safety Disclosures

None.

Part II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer's Purchases of Equity Securities

Our common stock has been publicly traded on The Nasdaq Stock Market under the symbol "KALA" since July 20, 2017 in connection with our initial public offering, or IPO. From July 20, 2017 through January 10, 2023 our common stock traded on The Nasdaq Global Select Market. On January 11, 2023, our common stock began trading on The Nasdaq Capital Market. Prior to our IPO, there was no public market for our common stock.

Holders

As of March 28, 2024, there were approximately 22 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

Dividend Policy

We have not declared or paid any cash dividends on our common stock since our inception. We intend to retain all available funds and any future earnings to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future. In addition, our ability to pay cash dividends is currently restricted by the terms of our Loan and Security Agreement with Oxford Finance LLC and our Securities Purchase Agreements relating to our 2022, 2023 and 2024 private placements (which securities purchase agreements are more fully described in Item 1., Business and Item 7., Management's Discussion and Analysis of Financial Condition and Results of Operations). Future debt financing arrangements also may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock. Any future determination to declare and pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our results of operations, financial condition, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

Information About our Equity Compensation Plans

The information required by this item will be set forth in our Proxy Statement for the 2024 Annual Meeting of Stockholders and is incorporated herein by reference.

Recent Sales of Unregistered Securities

We did not sell any shares of our common stock, shares of our preferred stock or warrants to purchase shares of our stock, or grant any stock options, restricted stock units or restricted stock awards, during the year ended December 31, 2023 that were not registered under the Securities Act of 1933, as amended, or the Securities Act, and that have not otherwise been described in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Item 6. [Reserved]

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes thereto appearing at the end of this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. See "Special Note Regarding Forward-Looking Statements and Industry Data." Because of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a clinical-stage biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the front and back of the eye. Our product candidate, KPI-012, which we acquired from Combangio, Inc., or Combangio, on November 15, 2021, is a mesenchymal stem cell secretome, or MSC-S, and is currently in clinical development for the treatment of persistent corneal epithelial defects, or PCED, a rare disease of impaired corneal healing. Based on the positive results of a Phase 1b clinical safety and efficacy trial of KPI-012 in patients with PCED, along with favorable preclinical safety and efficacy results, we submitted an investigational new drug application, or IND, to the U.S. Food and Drug Administration, or FDA, which was accepted in December 2022. In February 2023, we dosed our first patient in our CHASE (**C**orneal **H**ealing **A**fter **SE**cretome therapy) Phase 2b clinical trial of KPI-012 for PCED in the United States, or the CHASE trial.

The CHASE trial is comprised of two patient cohorts. On March 27, 2023, we announced positive safety data from the first cohort of the CHASE trial, which is an open-label study to evaluate the safety of the high dose of KPI-012 ophthalmic solution (3 U/mL) dosed topically four times per day, or QID, in two patients. Both patients in the first cohort successfully completed at least one week of dosing with no safety issues observed. We have initiated the second and final patient cohort of the CHASE trial in the United States, which is a multicenter, randomized, double-masked, vehicle-controlled, parallel-group trial to evaluate the safety and tolerability of two doses of KPI-012 ophthalmic solution (3 U/mL and 1 U/mL) versus vehicle dosed topically QID for 56 days in approximately 90 patients. We plan to add trial sites in Latin America, subject to regulatory approval.

The primary endpoint of the trial is the complete healing of the PCED as measured by corneal fluorescein staining. We are targeting reporting topline safety and efficacy data from the CHASE trial by the end of 2024. If the results are positive, and subject to discussion with regulatory authorities, we believe this trial could serve as the first of two pivotal trials required to support the submission of a Biologics License Application, or BLA, for KPI-012 to the FDA.

KPI-012 has received Orphan Drug and Fast Track designations from the FDA for the treatment of PCED.

We believe the multifactorial mechanism of action of KPI-012 also makes our MSC-S a platform technology. We are evaluating the potential development of KPI-012 for additional rare front-of-the-eye diseases, such as for the treatment of Limbal Stem Cell Deficiency, or LSCD, and other rare corneal diseases that threaten vision. In addition, we have initiated preclinical studies under our KPI-014 program to evaluate the utility of our MSC-S platform for inherited retinal degenerative diseases, such as Retinitis Pigmentosa and Stargardt Disease. In connection with the determination to focus our research and development efforts on KPI-012, in 2022, we determined to cease the development of our preclinical pipeline programs that are unrelated to our MSC-S platform. We expect to commercialize in the United States any of our product candidates that receive marketing approval. For a further description of our acquisition of Combangio, or Combangio Acquisition, see Item 1, "Business," "Liquidity and Capital Resources" below and Note 3, "Acquisitions and Divestitures" of our consolidated financial statements.

We previously developed and commercialized two marketed products, EYSUVIS® (loteprednol etabonate ophthalmic suspension) 0.25%, for the short-term (up to two weeks) treatment of the signs and symptoms of dry eye disease, and INVELTYS® (loteprednol etabonate ophthalmic suspension) 1%, a topical twice-a-day ocular steroid for the

treatment of post-operative inflammation and pain following ocular surgery. Both products applied a proprietary mucus-penetrating particle drug delivery technology, which we referred to as the AMPPLIFY® Drug Delivery Technology.

On July 8, 2022, Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC, which we refer to collectively as Alcon, purchased from us the rights to manufacture, sell, distribute, market and commercialize EYSUVIS and INVELTYS and to develop, manufacture, market and otherwise exploit the AMPPLIFY Drug Delivery Technology, which we collectively refer to as the Commercial Business. We refer to this transaction as the Alcon Transaction. Alcon also assumed certain liabilities with respect to the Commercial Business at the closing of the Alcon Transaction. For a further description of the Alcon Transaction, see Item 1, "Business," "Liquidity and Capital Resources" below and Note 3, "Acquisitions and Divestitures" of our consolidated financial statements.

During 2022, we terminated our entire commercial sales force and certain employees in our commercial, scientific, manufacturing, finance and administrative functions. The determination to proceed with the workforce reduction was made in the context of the closing of the Alcon Transaction and the changes to the scope of our research and development activities of KPI-012 as more fully described above.

Since inception, we have incurred significant losses from operations and negative cash flows from operations. Our net losses were $42.2 million for the year ended December 31, 2023 and $44.8 million for the year ended December 31, 2022. As of December 31, 2023, we had an accumulated deficit of $629.4 million. We generated only limited revenues from product sales of EYSUVIS and INVELTYS prior to the sale of the Commercial Business to Alcon in July 2022. We have financed our operations primarily through proceeds from the sale of our Commercial Business to Alcon, our initial public offering, or IPO, follow-on public common stock offerings and sales of our common stock under our sales agreement with Jefferies, LLC, or Jefferies, in at-the-market offerings, private placements of common stock and/or preferred stock (including our private placement of preferred stock for gross proceeds of approximately $2.0 million in December 2023, or our 2023 Private Placement, and $8.6 million in March 2024, or our 2024 Private Placement), borrowings under credit facilities and our Loan Agreement with Oxford Finance, or the Loan Agreement, a grant from California Institute for Regenerative Medicine, or CIRM, convertible promissory notes and warrants.

We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials and, prior to the sale of our Commercial Business to Alcon in July 2022, engaging in activities to launch and commercialize EYSUVIS and INVELTYS. As a result of our acquisition of Combangio and the sale of our Commercial Business to Alcon, we are devoting substantial financial resources to the research and development and potential commercialization of KPI-012 for PCED and any other indications we determine to pursue, including Limbal Stem Cell Deficiency. We have no revenue-generating commercial products and, as a result of our acquisition of Combangio, we may be required to pay certain milestones and royalty payments to former equityholders of Combangio, which are more fully described in the "Liquidity and Capital Resources" section. Although we are eligible to receive up to $325.0 million in payments from Alcon based upon the achievement of specified commercial sales-based milestones with respect to EYSUVIS and INVELTYS, there can be no assurance when we may receive such milestone payments or of the amount of milestone payments we may receive, if any. We cannot be certain that we will achieve the milestones within the timeframe required by the CIRM award, or at all, and as such we may never receive the remaining $9.1 million under the award. We expect to continue to incur significant expenses and operating losses for the foreseeable future, including in connection with our continued development, regulatory approval efforts and commercialization, if any, of KPI-012. We may never achieve or maintain profitability. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year.

Financial Operations Overview

Product Revenues, Net

On July 8, 2022, we sold our Commercial Business, including EYSUVIS and INVELTYS, to Alcon and ceased recording gross revenue on sales of EYSUVIS and INVELTYS. Our product revenues for the periods presented herein are recorded net of provisions relating to estimates for (i) trade discounts and allowances, such as discounts for prompt payment and other discounts and distributor fees, (ii) estimated rebates, chargebacks and co-pay assistance programs, and (iii) reserves for expected product returns. These estimates reflect current contractual and statutory requirements, known market events and trends, industry data and forecasted customer buying and payment patterns. Actual amounts

may ultimately differ from these estimates. If actual results vary, estimates may be adjusted in the period such change in estimate becomes known, which could have an impact on earnings in the period of adjustment.

We currently have no commercial products in our portfolio. Moreover, we only recently commenced the CHASE trial of KPI-012 for PCED in the United States and, accordingly, we do not expect to generate revenue from KPI-012 or any other product candidate we may develop for the foreseeable future, if at all.

Cost of Product Revenues

Cost of product revenues consisted primarily of materials, third-party manufacturing costs, freight and distribution costs, royalty expense, allocation of labor, quality control and assurance, reserves for defective inventory, reserves for excess and obsolete inventory, losses on inventory purchase commitments, and other manufacturing overhead costs. Prior to the sale of our Commercial Business in July 2022, write-downs of inventory were recorded as a cost of product revenues in the consolidated statements of operations and comprehensive loss. Following the sale of our Commercial Business, any adjustments to the remaining EYSUVIS and INVELTYS inventory, or the Remaining Inventory, were recorded within other expense in the consolidated statements of operations and comprehensive loss. Following the sale of the Commercial Business, the only customer for our Remaining Inventory was Alcon. The Remaining Inventory balance, net of the deferred gain on sale of Commercial Business, was written off during the year ended December 31, 2023, and is recorded in other (expense) income, net in the consolidated statements of operations and comprehensive loss. As a result of the sale of our Commercial Business to Alcon, we do not expect to generate cost of product revenues unless we commercialize another product candidate.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries, benefits, commissions, stock-based compensation and travel expenses related to our commercial infrastructure and our executive, finance, human resources, legal, compliance, information technology and business development functions. Selling, general and administrative expenses also include external selling and marketing costs related to EYSUVIS and INVELTYS prior to the sale of the Commercial Business to Alcon, costs to manufacture sample units and professional fees for auditing, tax, information technology, consultants, legal services and allocated facility-related costs not otherwise included in research and development expenses.

We expect that our selling, general and administrative expenses for 2024 will be comparable to such expenses for the year ended December 31, 2023. We anticipate that our selling, general and administrative expenses will stabilize at 2023 expense levels for the next several years. If we obtain marketing approval for KPI-012 or any product candidates we may develop, we expect that our selling, general and administrative expenses will increase substantially if and as we incur commercialization expenses related to product marketing, sales and distribution.

Research and Development Expenses

Research and development expenses consist of costs associated with our research activities, including compensation and benefits for full-time research and development employees, an allocation of facilities expenses, overhead expenses and certain outside expenses. Our research and development expenses include:

- employee-related expenses, including salaries, related benefits, travel and stock-based compensation;
- expenses incurred for the preclinical and clinical development of our product candidates and under agreements with contract research organizations, including costs of manufacturing product candidates prior to the determination that FDA approval of a drug candidate is probable and before the future economic benefit of the drug is expected to be realized; and
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities and supplies.

We expense research and development costs as they are incurred. We expense costs relating to the production of inventory for our product candidates, as research and development expenses within our consolidated statements of operations and comprehensive loss in the period incurred, unless we believe regulatory approval and subsequent

commercialization of the product candidate is probable and we expect the future economic benefit from sales of the drug to be realized. Research and development costs that are paid in advance of performance are capitalized as a prepaid expense until incurred. We track outsourced development costs by development program but do not allocate personnel costs, payments made under license agreements or other costs to specific product candidates or development programs. These costs are included in employee-related costs and other research and development costs in the line items in the tables under "Results of Operations".

We expect that our research and development costs will increase in 2024 as compared to such expenses for the year ended December 31, 2023 as we advance the clinical development of KPI-012 and as we conduct any necessary preclinical studies and clinical trials and other development activities for any other product candidate we may develop in the future, including our planned preclinical studies under our KPI-014 program. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time-consuming. We may never succeed in obtaining marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability.

KPI-012 is in Phase 2b clinical development and all of our other research and development programs are in preclinical development. Successful development and completion of preclinical studies and clinical trials is uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each product candidate and future product candidate and are difficult to predict. We will continue to make determinations as to which product candidates to pursue and how much funding to direct to each product candidate on an ongoing basis in response to the scientific and clinical success of each product candidate as well as ongoing assessments as to the commercial potential of product candidates and our ability to enter into collaborations with respect to each product candidate. We will need to raise additional capital and may seek collaborations in the future to advance KPI-012 and any product candidate we may develop. Additional private or public financings may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy.

(Gain) Loss on Fair Value Remeasurement of Deferred Purchase Consideration

In connection with the closing of the Combangio Acquisition on November 15, 2021, we agreed to issue an aggregate of 155,664 shares, or the Deferred Purchase Consideration, of our common stock to former Combangio stockholders and other equityholders, or the Combangio Equityholders, consisting of (i) an aggregate of 136,314 shares of common stock which were issued on January 3, 2022 and (ii) an aggregate of 19,350 shares of common stock that were held back by us as partial security for the satisfaction of indemnification obligations and other payment obligations of the Combangio Equityholders which were issued on March 10, 2023. We recorded an obligation for such Deferred Purchase Consideration at fair value on the acquisition date. We then revalued our Deferred Purchase Consideration obligations each reporting period. Changes in the fair value of our Deferred Purchase Consideration obligations, other than changes due to issuance, are recognized as a gain or loss on fair value remeasurement of Deferred Purchase Consideration in our consolidated statements of operations and comprehensive loss.

Loss (Gain) on Fair Value Remeasurement of Contingent Consideration

In addition to the Deferred Purchase Consideration, consideration payable to the Combangio Equityholders includes potential payments of up to $105.0 million that are contingent upon the achievement of specified development, regulatory and commercialization milestones. As of December 31, 2023, of the up to $105.0 million in contingent milestone payments, we paid to the Combangio Equityholders an aggregate of $2.5 million in cash and $2.4 million in shares of common stock (representing an aggregate of 105,038 shares of our common stock) as a result of our dosing the first patient in our CHASE trial in February 2023, or the First Dosing Milestone. The remaining amount of $0.1 million due in connection with the First Dosing Milestone was paid in January 2024. All potential milestone payments to the Combangio Equityholders are payable in cash going forward. We recorded an obligation for such contingent consideration at fair value on the acquisition date. We then revalue our contingent consideration obligations each reporting period. Changes in the fair value of our contingent consideration obligations, other than changes due to issuance, are recognized as a gain or loss on fair value remeasurement of contingent consideration in our consolidated statements of operations and comprehensive loss.

The potential payments and milestones are more fully described in Item 1, "Business" and in "Liquidity and Capital Resources" below and Note 3, "Acquisitions and Divestitures" of our consolidated financial statements.

Interest Income

Interest income consists of interest earned on our cash, cash equivalents and short-term investments, if any.

Interest Expense

Interest expense primarily consists of contractual coupon interest, amortization of debt discounts and debt issuance costs and accretion of the final payment fee recognized on our debt arrangements.

Grant Income

On April 28, 2023, CIRM awarded Combangio a $15.0 million grant, or the CIRM Award, subject to entering into a final award agreement, to support Combangio's ongoing KPI-012 program for the treatment of PCED as well as product and process characterization and analytical development for the program. On August 2, 2023, Combangio entered into the CIRM Award and became entitled to receive an initial $5.9 million disbursement from CIRM.

The CIRM Award is subject to a co-funding requirement under which Combangio is obligated to spend a specified minimum amount on the development of KPI-012 to obtain the full award amount. The remaining $9.1 million available under the award is payable to Combangio only upon the achievement of specified milestones that are primarily related to Combangio's progress in conducting the CHASE clinical trial. CIRM may permanently cease disbursements if the milestones are not met within four months of the scheduled completion dates. Additionally, if CIRM determines, in its sole discretion, that Combangio has not complied with the terms and conditions of the CIRM Award, CIRM may suspend or permanently cease disbursements. Under the terms of the CIRM Award, Combangio is obligated to pay a royalty on net sales of any product, service or approved drug resulting in whole or in part from the CIRM Award in the amount of 0.1% per $1.0 million of funds utilized by us until the earlier of ten years from the date of first commercial sale of such product, service or approved drug and such time as nine times the amount of funds awarded by CIRM has been paid in royalties, or the Base Royalty. In addition, following the satisfaction of the Base Royalty, Combangio is obligated to pay a 1.0% royalty on net sales of any CIRM-funded invention in excess of $500 million per year until the last to expire patent covering such invention expires.

The CIRM Award is not in the scope of the contracts with customers accounting guidance as the government entity is not a customer under the agreement. Rather, the CIRM Award is accounted for as a contract to perform research and development activities. As a result, grant income is recognized as the related research and development expenses are incurred.

Loss on Extinguishment of Debt

Loss on extinguishment of debt primarily consists of unamortized debt discount and issuance costs, a prepayment premium and unaccreted final payment fees paid upon extinguishment of a debt agreement. There was no loss on extinguishment of debt for the year ended December 31, 2023. For the year ended December 31, 2022, the loss on extinguishment of debt related to the partial extinguishment of debt under the Loan Agreement with Oxford Finance on July 8, 2022 in connection with the closing of the Alcon Transaction.

Gain on Sale of Commercial Business

Gain on sale of Commercial Business represents the gain recognized as a result of the sale of our Commercial Business to Alcon on July 8, 2022.

Other (Expense) Income, Net

Other (expense) income, net consists of expenses recorded to assets held for sale for the write-off of the remaining inventory balance and the write-off of the deferred gain related to the Alcon Transaction, as well as an

adjustment for the returns reserve associated with our former commercial products, partially offset by reimbursable transition-related services we provided to Alcon following the sale of the Commercial Business.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. GAAP. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates—which also would have been reasonable—could have been used. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing at the end of this Annual Report on Form 10-K, we believe that the following critical accounting estimates are those most critical to the judgments and estimates used in the preparation of our financial statements and that involve a significant level of estimation uncertainty.

Grant Income

We account for grants received to perform research and development activities in accordance with Accounting Standards Codification Topic 730-20, *Research and Development Arrangements*, which requires an assessment, at the inception of the grant, of whether the grant is a liability or a contract to perform research and development activities. If we are obligated to repay the grant funds to the grantor regardless of the outcome of the research and development activities, then we are required to estimate and recognize that liability. Alternatively, if we are not required to repay, or if we were required to repay the grant funds only if the research and development activities are successful, then the grant agreement is accounted for as a contract to perform research and development activities, in which case, grant income is recognized as the related research and development expenses are incurred. Costs of grant income are recorded as a component of research and development expenses in our statements of operations and comprehensive loss.

Revenue

Following the sale of our Commercial Business to Alcon in July 2022, we no longer have any commercial products in our portfolio. We accounted for revenue in accordance with Accounting Standards Codification, or ASC, Topic 606, *Revenue from Contracts with Customers*. Under ASC Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to be entitled in exchange for those goods or services. We performed the following five steps to recognize revenue under ASC Topic 606: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only recognized revenue when it was probable that we would collect the consideration to which we were entitled in exchange for the goods or services that would be transferred to the customer.

Product revenues, net

We sold EYSUVIS and INVELTYS primarily to wholesalers in the United States, or Customers. These Customers subsequently resold our products to specialty and other retail pharmacies. In addition to agreements with Customers, we entered into arrangements with third-party payors that provide for government-mandated and/or privately-negotiated rebates, chargebacks and discounts for the purchase of our products.

The goods promised in our product sales contracts represented a single performance obligation. We recognized revenue from product sales at the point the Customer obtained control of the product, which occurred upon delivery. The transaction price ("net sales price") that was recognized as revenue for product sales included the selling price to the

Customer and an estimate of variable consideration. Components of variable consideration included prompt pay and other discounts, product returns, government rebates, third-party payor rebates, coverage gap rebates, incentives such as patient co-pay assistance, and other fees paid to Customers and other third-party payors where a distinct good or service was not received. Variable consideration was recorded on the consolidated balance sheet as either a reduction of accounts receivable, if payable to a Customer, or as a current liability, if payable to a third-party other than a Customer. We considered all relevant information when estimating variable consideration such as assessment of our then current and anticipated sales and demand forecasts, actual payment history, information from third parties regarding the payor mix for products, information from third parties regarding the units remaining in the distribution channel, specific known market events and trends, industry data and current contractual and statutory requirements that were reasonably available. We included estimated amounts for variable consideration in the net sales price to the extent it was determined probable that a significant reversal of cumulative revenue recognized would not occur when the uncertainty associated with the variable consideration was resolved.

Payment terms with Customers did not exceed one year and, therefore, we did not account for a significant financing component in our arrangements. We expensed the incremental cost of obtaining a contract with a Customer when incurred as the period of benefit was generally less than one year.

Reserves for Variable Consideration:

Trade Discounts and Allowances

We provided our Customers with certain trade discounts and allowances including discounts for prompt payments and other discounts and fees paid for distribution, data and administrative services. These discounts and fees were based on contractually-determined percentages and were recorded as a reduction of revenue and accounts receivable in the period in which the related product revenue was recognized.

Chargebacks

Chargebacks for fees and discounts to providers represent the estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices charged to Customers who directly purchased the product from us. Customers charged us for the difference between what they paid for the product and the ultimate selling price to the qualified healthcare providers. These components of variable consideration were established in the same period that the related revenue was recognized, resulting in a reduction of product revenue and accounts receivable. Reserves for chargebacks consisted of credits we expected to issue for units that remained in the distribution channel at the end of each reporting period and that we expected would be sold to qualified healthcare providers, as well as chargebacks that Customers had claimed, but for which we had not yet issued a credit.

Product Returns

Consistent with industry practice, we had a product returns policy that provides Customers right of return for product purchased within a specified period prior to and subsequent to the product's expiration date. We estimated the amount of our products that may be returned and presented this amount as a reduction of revenue in the period the related product revenue was recognized, in addition to establishing a liability. Our estimates for product returns were based upon available industry data and our own sales information, including our visibility into the inventory remaining in the distribution channel as well as historical returns, which developed over time.

Commercial Payor and Medicare Part D Rebates

We contracted with certain third-party payors, primarily pharmacy benefit managers, or PBMs, and health plans, or Plans, for the payment of rebates with respect to utilization of our product. These rebates were based on contractual percentages applied to the amount of product prescribed to patients who were covered by the PBMs or the Plans with which it contracted. We estimated the rebates for commercial and Medicare Part D payors based on the contractual discount percentage, the various payor mix for EYSUVIS and INVELTYS as well as future rebates that would be made for product that had been recognized as revenue but remained in the distribution channel at the end of each reporting period. We also estimated the number of patients in the prescription drug coverage gap for whom we would owe an additional liability under the Medicare Part D program. Such estimates were recorded in the same period

the related revenue was recognized, resulting in a reduction of product revenue and the establishment of a current liability.

Government Rebates

We were subject to discount obligations under Medicaid and other government programs. For Medicaid, reserves were based on actual payment history, and estimates of future Medicaid beneficiary utilization applied to the Medicaid unit rebate formula established by the Centers for Medicaid and Medicare Services. Our liability for these rebates consisted of estimates of claims for the current period and estimated future claims that would be made for product that had been recognized as revenue but remained in the distribution channel at the end of each reporting period. These reserves were recorded in the same period the related revenue was recognized, resulting in a reduction of product revenue and the establishment of a current liability.

Co-pay Assistance Programs

We offered co-pay assistance programs (the "co-pay programs"), which were intended to provide financial assistance to patients who may or may not be covered by commercial insurance or, with respect to INVELTYS, who opt out of Medicare Part D programs. The calculation of accruals for the co-pay programs were based on actual claims processed during the period as well as an estimate of the number and cost per claim that we expected to receive associated with product that had been recognized as revenue but remained in the distribution channel at the end of each reporting period. Allowances for estimated co-pay claims are recorded in the same period the related revenue was recognized, resulting in a reduction of product revenue and the establishment of a current liability.

Acquisition Accounting

We are required to make significant judgments and estimates to determine whether an acquisition constitutes an acquisition of a business or assets. For asset acquisitions, this includes whether substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or a group of similar identifiable assets. We are also required to make several significant judgments and estimates in order to determine the total consideration transferred for the asset acquisition and then allocate it to the assets that we have acquired and the liabilities that we have assumed on a relative fair value basis. If the asset related to acquired in-process research and development expenses has no alternative future use, it is expensed immediately upon the completion of the transaction.

In addition to upfront consideration, our asset acquisitions may also include contingent consideration payments to be made for future milestone events or royalties on net sales of future products. We assess whether such contingent consideration is required to be recorded at fair value on the date of the acquisition and subsequently remeasured to fair value at each reporting date. Contingent consideration payments in an asset acquisition not required to be recorded at fair value are recognized when the contingency is resolved, and the consideration is paid or becomes payable. Changes in the fair value of the contingent milestone payments can result from changes to one or more inputs, including adjustments to the probability of achievement, timing of the contingent milestone payments and changes to the applicable discount rates. Significant judgment is used in determining these assumptions and estimates during each reporting period. Reasonable changes in these assumptions can cause material changes to the fair value of our contingent consideration liability. Any changes in the fair value of these contingent consideration liabilities are included in loss from operations in the consolidated statements of operations and comprehensive loss. For information related to the unobservable inputs related to the contingent consideration, see Note 5, "Fair Value of Financial Instruments", of our consolidated financial statements.

Results of Operations

Comparison of the Years ended December 31, 2023 and 2022

The following table summarizes the results of our operations for the years ended December 31, 2023 and 2022:

	Year Ended December 31,		
	2023	2022	Change
	(in thousands)		
Product revenues, net	$ —	$ 3,892	$ (3,892)
Costs and expenses:			
Cost of product revenues	—	2,560	(2,560)
Selling, general and administrative	20,567	65,035	(44,468)
Research and development	18,586	17,653	933
(Gain) loss on fair value remeasurement of Deferred Purchase Consideration	(230)	638	(868)
Loss (gain) on fair value remeasurement of contingent consideration	740	(288)	1,028
Total operating expenses	39,663	85,598	(45,935)
Loss from operations	(39,663)	(81,706)	42,043
Other income (expense)			
Interest income	2,711	664	2,047
Interest expense	(5,814)	(7,266)	1,452
Grant income	4,825	—	4,825
Loss on extinguishment of debt	—	(2,583)	2,583
Gain on sale of Commercial Business	—	46,995	(46,995)
Other (expense) income, net	(4,258)	(926)	(3,332)
Net loss	$ (42,199)	$ (44,822)	$ 2,623

Product revenues, net

We did not have any product revenues during the year ended December 31, 2023 due to the sale of our Commercial Business to Alcon in July 2022. Product revenues, net was $3.9 million for the year ended December 31, 2022, consisting of $2.3 million from EYSUVIS sales and $1.6 million from INVELTYS sales. As a result of the sale of our Commercial Business, we no longer have any commercial products in our portfolio.

Cost of product revenues

We did not have any cost of product revenues during the year ended December 31, 2023 due to the sale of our Commercial Business to Alcon in July 2022. Cost of product revenues was $2.6 million for the year ended December 31, 2022.

Selling, general and administrative expenses

Selling, general and administrative expenses were $20.6 million for the year ended December 31, 2023, compared to $65.0 million for the year ended December 31, 2022, which was a decrease of $44.5 million. The decrease in selling, general and administrative expenses for the year ended December 31, 2023 was primarily due to the sale of our Commercial Business to Alcon and our related workforce reduction completed during the second half of 2022 and includes a $22.5 million decrease in employee-related expenses and a $17.9 million decrease in external sales and marketing costs. Also contributing to the decrease as compared to the year ended December 31, 2022, was a $3.3 million decrease in administrative and professional service fees and $0.8 million of transaction costs related to the Alcon Transaction which were not incurred in the year ended December 31, 2023.

Research and development expenses

The following table summarizes the research and development expenses incurred during the years ended December 31, 2023 and 2022:

	Year Ended December 31,		
	2023	2022	Change
	(in thousands)		
KPI-012 development costs	$ 7,678	$ 5,803	$ 1,875
Employee-related costs	9,710	9,256	454
Other research and development costs	1,198	2,594	(1,396)
Total research and development	$ 18,586	$ 17,653	$ 933

Research and development expenses were $18.6 million for the year ended December 31, 2023 compared to $17.7 million for the year ended December 31, 2022, an increase of $0.9 million. The increase was primarily related to a $2.3 million increase in employee-related costs and KPI-012 development costs, as we advance the clinical development of KPI-012, partially offset by a decrease of $1.4 million of other research and development costs, which primarily included preclinical studies related to our former pipeline programs.

(Gain) loss on fair value remeasurement of Deferred Purchase Consideration

The gain on fair value remeasurement of Deferred Purchase Consideration for the year ended December 31, 2023 was $0.2 million and the loss on fair value remeasurement of Deferred Purchase Consideration for the year ended December 31, 2022 was $0.6 million. The amounts were primarily due to a change in the fair value of our stock price.

Loss (gain) on fair value remeasurement of contingent consideration

Loss on fair value remeasurement of contingent consideration for the year ended December 31, 2023 was $0.7 million, primarily due to changes in discount rates, partially offset by changes in the expected timing and probability of payment. Gain on fair value remeasurement of contingent consideration for the year ended December 31, 2022 was $0.3 million and was primarily due to changes in discount rates, partially offset by the passage of time.

Interest income

Interest income was $2.7 million for the year ended December 31, 2023, compared to $0.7 million for the year ended December 31, 2022, an increase of $2.0 million. Interest income consists of interest earned on our cash, cash equivalents and short-term investments, if any. The increase was attributable to higher interest rates during the year ended December 31, 2023 as well as the mix and quantity of investments during the year ended December 31, 2023.

Interest expense

Interest expense was $5.8 million for the year ended December 31, 2023, compared to $7.3 million for the year ended December 31, 2022, a decrease of $1.5 million. Interest expense for the years ended December 31, 2023 and 2022 was comprised of the contractual coupon interest expense, the amortization of the debt discount and the accretion of the final payment fee associated with our Loan Agreement with Oxford Finance. During the year ended December 31, 2023, $43.3 million of indebtedness was outstanding under our Loan Agreement until $9.3 million was repaid on January 25, 2023 resulting in an outstanding indebtedness of $34.0 million as of December 31, 2023. During the year ended December 31, 2022, $80.0 million of indebtedness was outstanding under our Loan Agreement until $36.7 million was repaid on July 8, 2022 resulting in an outstanding indebtedness of $43.3 million as of December 31, 2022. While interest expense decreased during the year ended December 31, 2023 due to the lower outstanding principal balance, this decrease was partially offset by the variable rate on the debt and the rising interest rates.

Grant income

Grant income for the year ended December 31, 2023 was $4.8 million related to the CIRM Award. There was no grant income recognized during the year ended December 31, 2022.

Loss on extinguishment of debt

The was no loss on extinguishment of debt for the year ended December 31, 2023. The loss on extinguishment of debt was $2.6 million for the year ended December 31, 2022. Upon the partial repayment of $36.7 million of indebtedness under our Loan Agreement in July 2022, the prepayment premium, unaccreted amount of the final payment fee due and a pro-rata portion of the debt discount were recorded as loss on extinguishment of debt for the year ended December 31, 2022.

Gain on sale of Commercial Business

There was no gain on sale of Commercial Business for the year ended December 31, 2023. The gain on sale of Commercial Business was $47.0 million for the year ended December 31, 2022, which was comprised of the $65.0 million in cash consideration received from Alcon at the closing less $4.2 million of deferred gain on sale of Commercial Business, $11.7 million net book value of assets transferred and $2.1 million of transaction costs.

Other income (expense), net

Other income and expense was a net expense of $4.3 million for the year ended December 31, 2023 consisting of a $7.6 million expense recorded to assets held for sale to write-off the remaining inventory balance and $1.1 million related to an adjustment for the returns reserve associated with our former commercial products, partially offset by the $4.2 million write-off related to the deferred gain recorded on the sale of the Commercial Business and $0.2 million of reimbursable transition related services we provided to Alcon following the sale of the Commercial Business.

Other income and expense was a net expense of $0.9 million for the year ended December 31, 2022, which primarily represents a $4.2 million expense recorded to assets held for sale for expiring inventory, partially offset by $3.6 million of reimbursable transition related services we provided to Alcon following the sale of the Commercial Business.

Liquidity and Capital Resources

Since our inception, we have incurred significant operating losses. We only generated limited revenues from product sales of EYSUVIS and INVELTYS prior to the sale of our Commercial Business to Alcon in July 2022. We have financed our operations primarily through proceeds from the sale of our Commercial Business to Alcon in July 2022, our IPO, follow-on public common stock offerings and sales of our common stock under our at-the-market equity offerings, private placements of common stock and/or preferred stock, borrowings under credit facilities and our Loan and Security Agreement, or the Loan Agreement, with Oxford Finance LLC, or Oxford Finance, a grant from CIRM, convertible promissory notes and warrants.

Sale of Commercial Business

In July 2022, we sold our Commercial Business to Alcon. In addition to the upfront cash payment of $60.0 million we received from Alcon, we are also eligible to receive from Alcon up to four commercial-based sales milestone payments as follows: (1) $25.0 million upon the achievement of $50.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (2) $65.0 million upon the achievement of $100.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (3) $75.0 million upon the achievement of $175.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029 and (4) $160.0 million upon the achievement of $250.0 million or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029. Each milestone payment will only become payable once, if at all, upon the first time such milestone is achieved, and only one milestone payment will be paid with respect to a calendar year. In the event that more than one milestone is achieved in a calendar year, the higher milestone payment will become payable and the lower milestone payment will become payable

only if the corresponding milestone is achieved again in a subsequent calendar year. To date, we have not received any such milestone payments. We now have no revenue-generating commercial products, and although we are eligible to receive up to $325.0 million in milestone-based payments from Alcon, there can be no assurance as to when we may receive such milestone payments or the amount of milestone payments we may receive, if any.

Offerings under Registration Statements

In connection with the filing of a registration statement on Form S-3 with the SEC, or the 2020 Shelf Registration, we entered into an amended and restated sales agreement with Jefferies, or the Amended and Restated Sales Agreement, pursuant to which we could issue and sell, from time to time, up to an aggregate of $75.0 million of our common stock under our at-the-market offering. During the year ended December 31, 2022, we sold an aggregate of 148,461 shares of our common stock under the Amended and Restated Sales Agreement, resulting in net proceeds of $1.0 million. From January 1, 2023 to January 10, 2023, we sold 245,887 shares of our common stock under the Amended and Restated Sales Agreement, resulting in net proceeds of $10.0 million. On January 10, 2023, the Amended and Restated Sales Agreement terminated in accordance with its terms when we completed the sale of $75.0 million of our shares of common stock thereunder. As of the date of termination of the Amended and Restated Sales Agreement, we had sold an aggregate of 565,974 shares of our common stock under such agreement for aggregate gross proceeds of $75.0 million.

On January 19, 2023, we entered into a new sales agreement with Jefferies, or the Open Market Sale Agreement, pursuant to which we may issue and sell, from time to time, shares of our common stock through Jefferies under our at-the-market offering. We filed a prospectus supplement relating to the Open Market Sale Agreement under our 2020 Shelf Registration, or the 2020 Shelf ATM Prospectus Supplement, pursuant to which we could offer and sell shares of common stock having an aggregate offering price of up to $40.0 million under the Open Market Sale Agreement. From January 19, 2023 to May 11, 2023, we sold 229,378 shares of our common stock under our at-the-market offering pursuant to the Open Market Sale Agreement under the 2020 Shelf Registration, resulting in net proceeds of $4.9 million.

On March 3, 2023, we filed a shelf registration statement on Form S-3 with the SEC, or the 2023 Shelf Registration, which was declared effective on May 11, 2023. Under the 2023 Shelf Registration we may offer and sell up to $350.0 million of a variety of securities including common stock, preferred stock, warrants, depositary shares, debt securities, subscription rights or units. In accordance with the terms of the Open Market Sale Agreement, we may issue and sell, from time to time, up to an aggregate of $40.0 million of our common stock in an at-the-market equity offering through Jefferies. Upon effectiveness of the 2023 Shelf Registration, we ceased any further offers or sales of our common stock pursuant to the 2020 Shelf ATM Prospectus Supplement and the 2020 Shelf Registration. During the year ended December 31, 2023, we sold 256,256 shares of our common stock under our at-the-market offering pursuant to the 2023 Shelf Registration for total net proceeds of $3.6 million.

During the year ended December 31, 2023, we sold an aggregate of 731,521 shares of our common stock pursuant to (1) our Amended and Restated Sales Agreement and our Open Market Sale Agreement under the 2020 Shelf Registration and (2) the Open Market Sale Agreement under the 2023 Shelf Registration, for total net proceeds of $18.5 million.

Loan Agreement

On May 4, 2021, we entered into the Loan Agreement with Oxford Finance, in its capacity as lender, or the Lender, and in its capacity as collateral agent, or Agent, pursuant to which a term loan of up to an aggregate principal amount of $125.0 million became available to us, consisting of a tranche A term loan that was disbursed on the closing date of the Loan Agreement in the aggregate principal amount of $80.0 million and additional tranches that are no longer available to us. Through June 30, 2023, the term loan bore interest at a floating rate equal to the greater of 30-day LIBOR and 0.11%, plus 7.89%. Effective July 1, 2023, the term loan bears interest at a floating rate equal to the greater of (a) 8.00% and (b) the sum of (i) the 1-Month CME Term Secured Overnight Financing Rate, or SOFR, (ii) 0.10% and (iii) 7.89%. Certain of the customary negative covenants limit our and certain of our subsidiaries' ability, among other things, to incur future debt, grant liens, make investments, make acquisitions, distribute dividends, make certain restricted payments and sell assets, subject in each case to certain exceptions. In connection with our entry into the purchase agreement for the sale of our Commercial Business to Alcon, on May 21, 2022, we entered into an amendment

to the Loan Agreement, or the Second Loan Amendment, pursuant to which the Lender and Agent consented to the entry by us into the asset purchase agreement and the sale of the Commercial Business to Alcon and agreed to release its liens on the Commercial Business in consideration for the payment by us at the closing of the Alcon Transaction of an aggregate amount of $40.0 million, or the Second Amendment Prepayment, to the Lender and Agent. The Second Amendment Prepayment, which represented a partial prepayment of principal in the amount of $36.7 million of the $80.0 million principal amount outstanding under the term loan advanced by the Lender under the Loan Agreement, plus a prepayment fee of $0.7 million and a final payment fee of $2.6 million, was paid on July 8, 2022 in connection with the closing of the Alcon Transaction.

On December 27, 2022, we entered into an amendment to the Loan Agreement with Combangio and Oxford Finance, or the Third Loan Amendment, pursuant to which Oxford Finance agreed to amend certain provisions of the Loan Agreement to permit the transfer of the listing of our common stock from The Nasdaq Global Select Market to The Nasdaq Capital Market. Pursuant to the Third Loan Amendment, we agreed (A) to make partial prepayments of the principal amount of the term loan outstanding under the Loan Agreement as follows, or the Third Amendment Prepayments: (1) a payment of $5.0 million on or before June 30, 2023, representing a partial prepayment of principal in the amount of $4.7 million, plus a final payment fee of $0.3 million and (2) a payment of $5.0 million on or before January 31, 2024, representing a partial prepayment of principal in the amount of $4.7 million, plus a final payment fee of $0.3 million and (B) the start date for us to make amortization payments under the Loan Agreement was changed from January 1, 2026 to January 1, 2025, or the Amortization Date. On January 25, 2023, we paid the Third Amendment Prepayments and the principal loan balance under the Loan Agreement following such prepayments was $34.0 million.

Pursuant to the Third Loan Amendment, in addition to the Third Amendment Prepayments, if we make an additional prepayment under the Loan Agreement equal to $5.0 million (inclusive of the final payment fee) on or prior to December 31, 2024, or the First Extension Prepayment, the Amortization Date will be automatically changed to July 1, 2025, and the maturity date of the Loan Agreement will be automatically changed from May 1, 2026 to November 1, 2026. If, in addition to the Third Amendment Prepayments and the First Extension Prepayment, we make an additional prepayment under the Loan Agreement equal to $2.5 million (inclusive of the final payment fee) on or prior to June 30, 2025, or the Second Extension Prepayment, the Amortization Date will be automatically changed to January 1, 2026, and the maturity date of the Loan Agreement will be automatically changed to May 1, 2027.

Under the Third Loan Amendment, the Oxford Finance also agreed to waive the prepayment fees for the Third Amendment Prepayments, the First Extension Prepayment, the Second Extension Prepayment and any other prepayments under the Loan Agreement. Pursuant to the Loan Agreement, we also will be required to pay all accrued and unpaid interest on the principal amounts of the term loan being repaid at the time of repayment.

We will be required to make a final payment fee of 7.00% of the original principal amount of any funded term loan payable on the earlier of (i) the prepayment of the term loan in full or (ii) the maturity date. At our option, we may elect to make partial repayments of the term loan to the Lender, subject to specified conditions, including the payment of applicable fees and accrued and unpaid interest on the principal amount of the term loan being repaid. For further information about the Loan Agreement, see Note 11, “Debt” of our consolidated financial statements.

On August 1, 2023, we entered into a fourth amendment to the Loan Agreement pursuant to which certain provisions of the Loan Agreement were amended in connection with the change in our corporate name and the cessation of the U.S. Dollar LIBOR rate. On August 2, 2023, we entered into a fifth amendment to the Loan Agreement pursuant to which Oxford Finance consented to our entry into the CIRM Award and certain provisions of the Loan Agreement were amended in connection therewith.

Private Placements

On November 28, 2022, we entered into a Securities Purchase Agreement with certain institutional investors named therein, or the Series E Purchasers, pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of our common stock and shares of our Series E Convertible Non-Redeemable Preferred Stock, or the Series E Preferred Stock, in two tranches for aggregate gross proceeds of up to $31.0 million, which we refer collectively as the 2022 Private Placement. At the first closing of the 2022 Private Placement on December 1, 2022, we issued and sold to the Series E Purchasers (i) 76,813 shares of common stock, at a price per share equal to $5.75 and (ii) 9,666 shares of Series E Preferred Stock, at a price per share of Series E Preferred Stock equal to

$575.00, for aggregate gross proceeds of approximately $6.0 million. On December 27, 2022, following the certification by our Chief Executive Officer that the FDA accepted our IND application for KPI-012, we issued and sold to the Series E Purchasers at a second closing of the 2022 Private Placement a total of 43,478 shares of Series E Preferred Stock, at a price per share of Series E Preferred Stock equal to $575.00, for aggregate gross proceeds of approximately $25.0 million.

On December 21, 2023, we entered into a securities purchase agreement with certain institutional investors named therein pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, 2,928 shares of our Series F Convertible Non-Redeemable Preferred Stock, or the Series F Preferred Stock, at a price per share of $683.00, for aggregate gross proceeds of approximately $2.0 million.

On March 25, 2024, we entered into a securities purchase agreement with certain institutional investors named therein pursuant to which we agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, 10,901 shares of our Series G Convertible Non-Redeemable Preferred Stock, or the Series G Preferred Stock, at a price per share of $788.90, for aggregate gross proceeds of approximately $8.6 million.

CIRM Award

On April 28, 2023, CIRM awarded Combangio a $15 million grant, subject to entering into a final award agreement, to support its ongoing KPI-012 program for the treatment of PCED as well as product and process characterization and analytical development for the program. On August 2, 2023, Combangio entered into the CIRM Award and became entitled to receive $5.9 million. For a further description of the CIRM Award and the potential milestone payments we may receive, see "Financial Operations Overview – Grant Income" above.

Combangio Acquisition

As a result of the acquisition of Combangio, we may be required to pay additional contingent consideration to the former Combangio Equityholders. Former Combangio Equityholders are entitled to receive from us, subject to the terms and conditions of the Merger Agreement, contingent consideration, which would become payable upon our achievement of various development, regulatory and sales milestones and as a result of certain cash royalty payment obligations which are in the mid-to-high single digits. The total potential maximum payout for the milestone payments which are contingent upon the achievement of specified development, regulatory and commercialization milestones is $40.0 million and the total potential maximum payout for future sales-based milestone payments is an additional $65.0 million. To date, of the $40.0 million of contingent consideration payable upon achievement of specified development, regulatory and commercialization milestones, in March 2023 we paid to the former Combangio Equityholders an aggregate of $2.5 million in cash and $2.4 million in shares of our common stock (representing an aggregate of 105,038 shares of our common stock) following dosing of the first patient in our CHASE trial in February 2023. The remaining amount of $0.1 million for this milestone was paid in cash in January 2024. For a full description of the consideration payable as a result of the Combangio Acquisition, see Note 3, "Acquisitions and Divestitures" of our consolidated financial statements.

Other Contractual Obligations

Our other material cash requirements from known contractual and other obligations as of December 31, 2023 primarily related to our licensing agreement with Stanford University and our operating lease. For information related to our future commitments relating to our licensing agreement, see Note 17, "Commitments and Contingencies" of our consolidated financial statements. For information related to our future commitments for our lease related obligations, see Note 10, "Lease" of our consolidated financial statements.

Cash Flows

As of December 31, 2023 and 2022, we had $50.9 million and $70.5 million in cash and cash equivalents, respectively. As of December 31, 2023 and 2022, we had $34.0 million and $43.3 million in indebtedness, respectively, which represented the aggregate principal amount that was outstanding under the Loan Agreement with Oxford Finance.

The following table summarizes our sources and uses of cash for each of the periods presented:

	Year Ended December 31,		
	2023	2022	Change
	(in thousands)		
Net cash used in operating activities	$ (27,927)	$ (78,908)	$ 50,981
Net cash (used in) provided by investing activities	(429)	62,717	(63,146)
Net cash provided by (used in) financing activities	8,506	(7,942)	16,448
Decrease in cash and restricted cash	$ (19,850)	$ (24,133)	$ 4,283

Operating Activities

Net cash used in operating activities for the year ended December 31, 2023 was $27.9 million compared to $78.9 million for the year ended December 31, 2022, a decrease of $51.0 million, primarily due to a $42.6 million decrease in the net loss adjusted for non-cash charges and an $8.4 million decrease due to the timing of working capital fluctuations. Notable working capital fluctuations included a decrease in accounts payable, accrued expenses and other current liabilities during the year ended December 31, 2023 of $4.3 million, as compared to a decrease in accounts payable, accrued expenses and other current liabilities in the year ended December 31, 2022 of $14.0 million. Prepaid expenses and other current assets decreased by $5.7 million during the year ended December 31, 2023, as compared to an increase of $2.0 million during the year ended December 31, 2022, as a result of the collection of receivables due from Alcon and third parties in connection with transition related services. Inventory and assets held for sale decreased by $7.5 million during the year ended December 31, 2023, as a result of the expense recorded to assets held for sale to write-off the remaining inventory balance, as compared to a decrease of $1.7 million during the year ended December 31, 2022. These changes in working capital from the year ended December 31, 2022 to the year ended December 31, 2023 were partially offset by a $0.1 million decrease in accounts receivable in the year ended December 31, 2023 as compared to a $15.1 million decrease in the year ended December 31, 2022 as a result of the sale of our Commercial Business in 2022.

Investing Activities

Net cash used in investing activities for the year ended December 31, 2023 was $0.4 million compared to net cash provided of $62.7 million for the year ended December 31, 2022, a decrease of $63.1 million. Net cash used in investing activities for the year ended December 31, 2023 related to purchases of short-term investments of $9.9 million and purchases of property and equipment and other assets of $0.6 million, partially offset by proceeds from the sale or maturities of short-term investments of $10.0 million.

Net cash provided by investing activities for the year ended December 31, 2022 related to proceeds from the disposition of the Commercial Business, net of transaction costs, of $62.9 million, proceeds from the sales or maturities of short-term investments of $5.0 million and proceeds from the sale of property and equipment of $0.1 million, partially offset by the purchases of short-term investments of $5.0 million and purchases of property and equipment and other assets of $0.3 million.

Financing Activities

Net cash provided by financing activities for the year ended December 31, 2023 was $8.5 million, a change of $16.4 million compared to net cash used in financing activities of $7.9 million in the year ended December 31, 2022. Net cash provided by financing activities for the year ended December 31, 2023 largely consisted of $18.5 million of net proceeds from the sale of shares of our common stock through Jefferies under our at-the market offering, and $2.0 million of proceeds from the sale of Series F Preferred Stock in our 2023 Private Placement, partially offset by $10.0

million of repayment of principal and final payment fee on our Loan Agreement and a $2.0 million payment for the First Dosing Milestone reflected in financing activities.

Net cash used in financing activities for the year ended December 31, 2022 largely consisted of $40.0 million of repayment of principal, prepayment premium and final payment fee on our Loan Agreement, partially offset by net proceeds of $30.8 million from the sale of common stock and Series E Preferred Stock in our 2022 Private Placement, $1.0 million of net proceeds from the sale of shares of our common stock through Jefferies under our at-the-market offering, and $0.3 million of proceeds from the exercise of stock options and the issuance of common stock under our employee stock purchase plan.

Funding Requirements

We anticipate that our research and development expenses will increase substantially in the future as compared to prior periods as we advance the clinical development of KPI-012. Our research and development expenses will also increase in the future as we conduct any necessary preclinical studies and clinical trials and other development activities for any other product candidates we may develop in the future, including our planned preclinical studies under our KPI-014 program. If we obtain marketing approval for KPI-012 or any product candidates we may develop, we expect that our selling, general and administrative expenses will increase substantially if and as we incur commercialization expenses related to product marketing, sales and distribution.

Our expenses will also increase if and as we:

- continue the clinical development of KPI-012 for PCED;
- initiate and continue the research and development of KPI-012 for additional indications, such as Limbal Stem Cell Deficiency, including initiating and conducting preclinical studies and clinical trials;
- scale up our manufacturing processes and capabilities to manufacture the clinical supply of KPI-012;
- seek regulatory approval for KPI-012 for PCED in the United States and other jurisdictions;
- seek regulatory approval for KPI-012 for additional indications;
- grow our sales, marketing and distribution capabilities in connection with the commercialization of any product candidates for which we may submit for and obtain marketing approval;
- initiate and progress any preclinical development programs under our MSC-S platform, including from our KPI-014 program;
- conduct clinical trials and other development activities and/or seek marketing approval for any product candidates we may develop in the future;
- in-license or acquire the rights to other products, product candidates or technologies;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, quality control, scientific, manufacturing, commercial and management personnel to support our operations;
- expand our operational, financial and management systems; and
- increase our product liability insurance coverage if we initiate commercialization efforts for our product candidates.

We expect to continue to incur significant expenses and operating losses. Net losses may fluctuate significantly from quarter-to-quarter and year-to-year. We anticipate that our cash and cash equivalents as of December 31, 2023, together with the $8.6 million of gross proceeds we received from the sale of shares of our preferred stock in a private placement in March 2024 and the $9.1 million of remaining funding anticipated under the CIRM Award, will enable us to fund our operations, lease and debt service obligations, and capital expenditure requirements into the third quarter of 2025. We expect that our existing cash resources will be sufficient to enable us to obtain safety and efficacy data from our ongoing CHASE trial of KPI-012 in PCED. However, we do not expect that our existing cash resources will be

sufficient to enable us to complete the clinical development of KPI-012 for PCED or for any other indication. We have based our estimates on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. For example, we may not receive all of the funds awarded under the CIRM Award. As a result, we could deplete our available capital resources sooner or later than we currently expect.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Our expenses will increase from what we anticipate if:

- we elect or are required by the FDA or non-U.S. regulatory agencies to perform clinical trials or studies in addition to those expected;
- there are any delays in enrollment of patients in or completing our clinical trials or the development of our product candidates;
- we in-license or acquire rights to other products, product candidates or technologies; or
- there are any third-party challenges to our intellectual property portfolio, or the need arises to defend against intellectual property-related claims or enforce our intellectual property rights.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate revenue from KPI-012 or any other product candidate we may develop for the foreseeable future, if at all. Achieving and maintaining profitability will require us to be successful in a range of challenging activities, including:

- completing the clinical development of KPI-012 for PCED and any other indications we determine to pursue, including Limbal Stem Cell Deficiency;
- subject to obtaining favorable results from our ongoing and planned clinical trials of KPI-012, applying for and obtaining marketing approval of KPI-012;
- successfully commercializing KPI-012, if approved;
- discovering, developing and successfully seeking marketing approval and commercialization of any additional product candidates we may develop in the future, including under our KPI-014 program;
- hiring and building a full commercial organization required for marketing, selling and distributing those products for which we obtain marketing approval;
- manufacturing at commercial scale, marketing, selling and distributing those products for which we obtain marketing approval;
- achieving an adequate level of market acceptance, and obtaining and maintaining coverage and adequate reimbursement from third-party payors for any products we commercialize; and
- obtaining, maintaining and protecting our intellectual property rights.

As a company, we have limited experience commercializing products, and we may not be able to commercialize a product successfully in the future. There are numerous examples of unsuccessful product launches and failures to meet expectations of market potential, including by pharmaceutical companies with more experience and resources than us. We may never succeed in the foregoing activities and we may never generate revenue that is sufficient to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements, royalty agreements, and marketing and distribution arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing

and preferred equity financing, if available, may involve agreements that include pledging of assets as collateral, covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Our pledge of our assets as collateral to secure our obligations under our Loan Agreement may limit our ability to obtain additional debt financing. Under our Loan Agreement, we are also restricted from incurring future debt, granting liens, making investments, making acquisitions, distributing dividends on our common stock, making certain restricted payments and selling assets and making certain other uses of our cash, without the lenders' consent, subject in each case to certain exceptions. In addition, under the securities purchase agreements for our 2022, 2023 and 2024 Private Placements, we also agreed that we will not, without the prior approval of the requisite purchasers, (i) issue or authorize the issuance of any equity security that is senior or *pari passu* to the Series E Preferred Stock, the Series F Preferred Stock or the Series G Preferred Stock with respect to liquidation preference, (ii) incur any additional indebtedness for borrowed money in excess of $1.0 million, in the aggregate, outside the ordinary course of business, subject to specified exceptions, including the refinancing of our existing indebtedness or (iii) pay or declare any dividend or make any distribution on, any shares of our capital stock, subject to specified exceptions.

We will need to raise additional capital in the future to advance our business. Additional private or public financings may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy. If we raise additional funds through collaborations, strategic alliances, licensing arrangements, royalty agreements, or marketing and distribution arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or current or future commercialization efforts or grant rights to develop and market products or product candidates that we would otherwise prefer to develop and market ourselves.

Recently Issued Accounting Pronouncements

From time to time the Financial Accounting Standards Board or other standard-setting bodies, issue new accounting pronouncements. Where applicable, we adopt these new standards according to the specified effective dates. Unless otherwise disclosed in Note 2, "Summary of Significant Accounting Policies" to the consolidated financial statements appearing at the end of this Annual Report on Form 10-K, we believe that the impact of any recently issued accounting pronouncements that are not yet effective will not have a material impact on our financial position or results of operation upon adoption.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

Our financial instruments as of December 31, 2023 consisted primarily of cash equivalents which consisted of money market accounts and U.S. treasury securities that have contractual maturities of less than 90 days from the date of acquisition. Due to the short-term maturities of our cash equivalents, and the fixed income nature of these investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our cash equivalents.

As of December 31, 2023 and 2022, the aggregate principal amount outstanding under the Loan Agreement was $34.0 million and $43.3 million, respectively. The aggregate principal amount outstanding under the Loan Agreement bore interest through June 30, 2023 at a floating rate equal to the greater of (i) 30-day LIBOR and (ii) 0.11%, plus 7.89%. Effective July 1, 2023, the aggregate principal amount outstanding under the Loan Agreement bears interest at a floating rate equal to the greater of (i) 8.00% and (ii) the sum of (a) the 1-Month CME Term SOFR, (b) 0.10% and (c) 7.89% per annum. An immediate 10% change in the 1-Month CME Term SOFR rate would not have a material impact on our operating results or cash flows.

Item 8. Financial Statements and Supplementary Data

Our financial statements, together with the report of our independent registered public accounting firm, appear on pages F-1 through F-41 of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of disclosure controls and procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2023. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of December 31, 2023, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's annual report on internal control over financial reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for the company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company's principal executive and principal financial officers and effected by the company's board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles and includes those policies and procedures that:

- Pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the company;
- Provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and
- Provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management assessed the effectiveness of our internal control over financial reporting as of December 31, 2023. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control—Integrated Framework (2013). Based on that assessment, our management concluded that, as of December 31, 2023, our internal control over financial reporting was effective.

As a non-accelerated filer and a "smaller reporting company", as defined in Rule 12-b-2 under the Exchange Act, our independent registered public accounting firm is not required to issue an attestation report on the internal control over financial reporting.

Changes in internal control over financial reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the fourth quarter ended December 31, 2023 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

(b) None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the three months ended December 31, 2023.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not applicable.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item 10 is incorporated by reference from the information that will be contained in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates pursuant to General Instruction G(3) of Form 10-K.

Item 11. Executive Compensation

The information required by this Item 11 is incorporated by reference from the information that will be contained in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates pursuant to General Instruction G(3) of Form 10-K.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this Item 12 is incorporated by reference from the information that will be contained in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates pursuant to General Instruction G(3) of Form 10-K.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this Item 13 is incorporated by reference from the information that will be contained in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates pursuant to General Instruction G(3) of Form 10-K.

Item 14. Principal Accountant Fees and Services

The information required by this Item 14 is incorporated by reference from the information that will be contained in our Proxy Statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of the fiscal year to which this Annual Report on Form 10-K relates pursuant to General Instruction G(3) of Form 10-K.

Part IV

Item 15. Exhibits and Financial Statement Schedules

(1) **Financial Statements.**

The following documents are included beginning on page F-1 attached hereto and are filed as part of this Annual Report on Form 10-K.

KALA BIO, INC.
INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

(2) **Financial Statement Schedules.**

No financial statement schedules have been filed as part of this Annual Report on Form 10-K because they are not applicable, not required or because the information is otherwise included in our financial statements or notes thereto.

(3) **Exhibits**.

The following is a list of exhibits filed or furnished as part of this Annual Report on Form 10-K.

Exhibit Number	Description of Exhibit
2.1#	Agreement and Plan of Merger, dated as of November 15, 2021, by and among the Registrant, Ceres Merger Sub, Inc., Combangio, Inc. and, solely in its capacity as Combangio Equityholder Representative, Fortis Advisors LLC. (incorporated by reference to Exhibit 2.1 of the Registrant's current report on Form 8-K (File No. 001-38150) filed on November 15, 2021)
2.2#	Asset Purchase Agreement, by and between the Registrant, Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC (incorporated by reference to Exhibit 2.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on May 23, 2022)
3.1*	Restated Certificate of Incorporation of the Registrant, as amended as of November 28, 2022, including Certificate of Designation of the Series D Preferred Stock of Registrant, Certificate of Elimination of Number of Shares of Preferred Stock Designated as Series D Preferred Stock of Registrant, Certificate of Designations, Preferences and Rights of Series E Convertible Non-Redeemable Preferred Stock of Registrant, Certificate of Designations, Preferences and Rights of Series F Convertible Non-Redeemable Preferred Stock of Registrant and Certificate of Designations, Preferences and Rights of Series G Convertible Non-Redeemable Preferred Stock of Registrant
3.2	Third Amended and Restated By-Laws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on August 2, 2023)
4.1	Specimen Stock Certificate evidencing the shares of common stock (incorporated by reference to Exhibit 4.1 to the Registrant's annual report on Form 10-K (File No. 001-38150) filed on March 3, 2023)
4.2	Third Amended and Restated Registration Rights Agreement of the Registrant dated April 6, 2016, as amended by Amendment No. 1 dated December 13, 2017, of the Registrant (incorporated by reference to Exhibit 4.2 to the Registrant's annual report on Form 10-K (File No. 001-38150) filed on February 25, 2021)
4.3*	Description of the Registrant's Securities Registered under Section 12 of the Exchange Act

Exhibit Number	Description of Exhibit
4.4	Form of Series E Preferred Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on November 28, 2022)
4.5	Form of Series F Preferred Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on December 22, 2023)
4.6	Form of Series G Preferred Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on March 26, 2024)
4.7	Registration Rights Agreement, dated March 2, 2023, by and among the Registrant and the persons party thereto (incorporated by reference to Exhibit 4.5 to the Registrant's annual report on Form 10-K (File No. 001-38150) filed on March 3, 2023)
10.1+	2009 Employee, Director and Consultant Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registrant's registration statement on Form S-1 (File No. 333-218936) filed on June 23, 2017)
10.2+	Form of Stock Option Agreement under the 2009 Employee, Director and Consultant Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to the Registrant's registration statement on Form S-1 (File No. 333-218936) filed on June 23, 2017)
10.3+	Amended and Restated 2017 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.1 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on May 9, 2019)
10.4+	2017 Equity Incentive Plan, as amended (incorporated by reference to Exhibit 99.1 to the Registrant's Current Report on Form 8-K (File No. 001-38150) filed on June 26, 2020)
10.5+	Amended and Restated 2017 Equity Incentive Plan of the Registrant (incorporated by reference to Exhibit 99.1 to the Registrant's registration statement on Form S-8 (File No. 333-272834) filed on June 22, 2023)
10.6+	Form of Incentive Stock Option Agreement under 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.5 to the Registrant's registration statement on Form S-1/A (File No. 333-218936) filed on July 10, 2017)
10.7+	Forms of Non-Qualified Option Agreement under 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registrant's registration statement on Form S-1/A (File No. 333-218936) filed on July 10, 2017)
10.8+	Form of Non-Employee Director Restricted Stock Unit Award under 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on May 7, 2020)
10.9+	Form of Non-Employee Director Deferred Restricted Stock Unit Award under 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on May 7, 2020)
10.10+	Form of Employee Restricted Stock Unit Award under 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 6, 2020)
10.11+	Form of Incentive Stock Option Agreement under Amended and Restated 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.2 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.12+	Forms of Non-Qualified Option Agreement under Amended and Restated 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.3 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.13+	Form of Non-Employee Director Restricted Stock Unit Award under Amended and Restated 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.14+	Form of Non-Employee Director Deferred Restricted Stock Unit Award under Amended and Restated 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.5 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.15+	Form of Employee Restricted Stock Unit Award under Amended and Restated 2017 Equity Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.16+	Form of Inducement Stock Option Agreement (incorporated by reference to Exhibit 10.1 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on November 8, 2018)
10.17+	Form of Inducement Restricted Stock Unit Agreement (incorporated by reference to Exhibit 10.7 to the Registrant's quarterly report on Form 10-Q (File No. 333-218936) filed on August 4, 2023)

Exhibit Number	Description of Exhibit
10.18#	Exclusive License Agreement, dated October 11, 2019, by and between Combangio, Inc. and The Board of Trustees of the Leland Stanford Junior University (incorporated by reference to Exhibit 10.18 to the Registrant's annual report on Form 10-K (File No. 001-38150) filed on March 29, 2022)
10.19+	Letter Agreement, dated March 25, 2018, by and between the Registrant and Eric L. Trachtenberg (incorporated by reference to Exhibit 10.3 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 9, 2018)
10.20+	Amended and Restated Letter Agreement, dated September 10, 2015, by and between the Registrant and Mark Iwicki, as amended by the First Amendment, dated September 28, 2017 (incorporated by reference to Exhibit 10.1 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on November 7, 2017)
10.21+	Letter Agreement, dated November 6, 2017, by and between the Registrant and Todd Bazemore (incorporated by reference to Exhibit 10.12 of the Registrant's annual report on Form 10-K (File No. 001-38150) filed on April 2, 2018)
10.22+	Amended and Restated Letter Agreement, dated May 10, 2016, by and between the Registrant and Kim Brazzell (incorporated by reference to Exhibit 10.13 to the Registrant's registration statement on Form S-1 (File No. 333-218936) filed on June 23, 2017)
10.23+	Form of Amendment to Offer Letters (incorporated by reference to Exhibit 10.30 to the Registrant's annual report on Form 10-K (File No. 001-38150) filed on March 12, 2019)
10.24+	Form of Indemnification Agreement between the Registrant and each of its Executive Officers and Directors (incorporated by reference to Exhibit 10.14 to the Registrant's registration statement on Form S-1/A (File No. 333-218936) filed on July 10, 2017)
10.25	Common Stock Purchase Warrant (incorporated by reference to Exhibit 10.4 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on October 2, 2018)
10.26#	Loan and Security Agreement, dated May 4, 2021, by and among the Registrant and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-38150) filed on May 5, 2021)
10.27#	First Amendment to Loan and Security Agreement, dated November 15, 2021, by and among the Registrant, Combangio, Inc. and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.1 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 11, 2022)
10.28	Second Amendment to Loan and Security Agreement, dated May 21, 2022, by and among the Registrant, Combangio, Inc. and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.2 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 11, 2022)
10.29	Third Amendment to Loan and Security Agreement, dated December 27, 2022, by and among the Registrant, Combangio, Inc. and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on December 27, 2022)
10.30	Fourth Amendment to Loan and Security Agreement, dated August 1, 2023, by and among the Registrant, Combangio, Inc. and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.8 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.31	Fifth Amendment to Loan and Security Agreement, dated August 2, 2023, by and among the Registrant, Combangio, Inc. and Oxford Finance LLC, as collateral agent and lender (incorporated by reference to Exhibit 10.9 to the Registrant's quarterly report on Form 10-Q (File No. 001-38150) filed on August 4, 2023)
10.32	Securities Purchase Agreement, dated November 28, 2022, by and among the Registrant and the purchasers party thereto (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K (File No. 001-38150) filed on November 28, 2022)
10.33	Securities Purchase Agreement, dated December 21, 2023, by and among the Registrant and the purchasers party thereto (incorporated by reference to Exhibit 10.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on December 22, 2023)
10.34	Securities Purchase Agreement, dated March 25, 2024, by and among the Registrant and the purchasers party thereto (incorporated by reference to Exhibit 10.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on March 26, 2024)

Exhibit Number	Description of Exhibit
10.35	Open Market Sale Agreement℠, dated as of January 19, 2023, by and between Registrant and Jefferies LLC (incorporated by reference to Exhibit 1.1 to the Registrant's current report on Form 8-K (File No. 001-38150) filed on January 19, 2023)
21.1*	Subsidiaries of the Registrant
23.1*	Consent of Deloitte & Touche LLP
31.1*	Rule 13a-14(a) Certification of Principal Executive Officer
31.2*	Rule 13a-14(a) Certification of Principal Financial Officer
32.1**	Certification of Principal Executive Officer pursuant to 18 U.S.C. §1350
32.2**	Certification of Principal Financial Officer pursuant to 18 U.S.C. §1350
97.1*+	Dodd-Frank Compensation Recovery Policy
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101)

* Filed herewith.

** Furnished herewith.

Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

\+ Management contract or compensatory plan or arrangement filed in response to Item 15(a)(3) of the Instructions to the Annual Report on Form 10-K.

Item 16. Form 10-K Summary

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

KALA BIO, INC.

Dated: March 29, 2024

By: /s/ Mark Iwicki
Mark Iwicki
Chief Executive Officer and
Chairman of the Board of Directors

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

/s/ MARK IWICKI Mark Iwicki	Chief Executive Officer and Chairman of the Board of Directors (Principal Executive Officer)	March 29, 2024
/s/ MARY REUMUTH Mary Reumuth	Chief Financial Officer (Principal Financial and Accounting Officer)	March 29, 2024
/s/ MARK S. BLUMENKRANZ Mark S. Blumenkranz, M.D.	Director	March 29, 2024
/s/ MARJAN FARID Marjan Farid, M.D.	Director	March 29, 2024
/s/ ANDREW I. KOVEN Andrew I. Koven	Director	March 29, 2024
/S/ C. DANIEL MYERS C. Daniel Myers	Director	March 29, 2024
/s/ GREGORY PERRY Gregory Perry	Director	March 29, 2024
/s/ HOWARD B. ROSEN Howard B. Rosen	Director	March 29, 2024

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the stockholders and the Board of Directors of KALA BIO, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of KALA BIO, Inc. and subsidiaries (formerly Kala Pharmaceuticals, Inc.) (the "Company") as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, changes in mezzanine equity and stockholders' equity, and cash flows, for the years then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and 2022, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current-period audit of the financial statements that was communicated or required to be communicated to the audit committee and that (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Grant Income - Refer to Notes 2 and 6 to the financial statements

Critical Audit Matter Description

On August 2, 2023, Combangio, a wholly owned subsidiary of the Company, entered into an award agreement with the California Institute for Regenerative Medicine (“CIRM”) for a $15 million grant (the “CIRM Award”) to support Combangio’s KPI-012 program for the treatment of persistent corneal epithelial defects (“PCED”). The award includes funding for the Company’s CHASE Phase 2b clinical trial of KPI-012 for PCED, as well as product and process characterization and analytical development for the program. The CIRM Award is subject to a co-funding requirement

under which Combangio is obligated to spend a specified minimum amount on the development of KPI-012 to obtain the full award amount. Upon entry into the CIRM Award, Combangio received an initial disbursement from CIRM, and the balance of the award is payable to Combangio upon the achievement of specified milestones that are primarily related to Combangio's progress in conducting the CHASE Phase 2b clinical trial.

We have identified the Company's accounting for the CIRM Award, including the conclusion as to the appropriate accounting standards under which to record the funding received, as our critical audit matter given the increased extent of effort and high degree of auditor judgment.

How the Critical Audit Matter Was Addressed in the Audit

Our audit procedures related to the accounting for the CIRM Award, including the recording of grant income, included the following, among others:

- We obtained and read the CIRM Award agreement, along with the Company's accounting position paper, to evaluate the reasonableness of the accounting conclusions and methodology management used to record the transaction.
- We evaluated the reasonableness of management's conclusion as to the appropriateness of the accounting standards followed.
- We tested the expenses recorded by the Company relating to work performed under the CIRM Award, which included corroboration of the work performed under the CIRM Award with those outside of finance.
- We tested the mathematical accuracy of management's calculations and the related recording of grant income.
- We evaluated the accuracy and completeness of management's disclosure for the CIRM Award.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
March 29, 2024

We have served as the Company's auditor since 2013.

KALA BIO, INC.

CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 50,895	$ 70,495
Prepaid expenses and other current assets (Note 7)	1,975	7,852
Current assets held for sale (Note 4)	—	7,595
Total current assets	52,870	85,942
Non-current assets:		
Property and equipment, net	753	400
Right-of-use assets	2,025	16
Restricted cash and other long-term assets	301	462
Total assets	$ 55,949	$ 86,820
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 919	$ 2,832
Accrued expenses and other current liabilities	6,018	8,910
Deferred gain on sale of commercial business	—	4,189
Deferred grant income	1,075	—
Current portion of lease liabilities	334	13
Current portion of long-term debt	—	5,000
Current portion of contingent consideration	—	4,146
Current portion of deferred purchase consideration	—	595
Total current liabilities	8,346	25,685
Long-term liabilities:		
Long-term lease liabilities	1,799	—
Long-term debt	34,190	37,937
Long-term contingent consideration	4,110	4,224
Total long-term liabilities	40,099	42,161
Total liabilities	48,445	67,846
Commitments and Contingencies (Note 17)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 5,000,000 shares authorized as of December 31, 2023 and 2022; 51,246 and 53,144 shares of Series E Convertible Non-Redeemable Preferred Stock issued and outstanding as of December 31, 2023 and 2022, respectively, and 2,928 and 0 shares of Series F Convertible Non-Redeemable Preferred Stock issued and outstanding as of December 31, 2023 and 2022, respectively	—	—
Common stock, $0.001 par value; 120,000,000 shares authorized as of December 31, 2023 and 2022; 2,759,372 and 1,706,971 shares issued and outstanding as of December 31, 2023 and 2022, respectively	3	2
Additional paid-in capital	636,910	606,182
Accumulated deficit	(629,409)	(587,210)
Total stockholders' equity	7,504	18,974
Total liabilities and stockholders' equity	$ 55,949	$ 86,820

The accompanying notes are an integral part of these consolidated financial statements.

KALA BIO, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2023	**2022**
Product revenues, net	$ —	$ 3,892
Costs and expenses:		
Cost of product revenues	—	2,560
Selling, general and administrative	20,567	65,035
Research and development	18,586	17,653
(Gain) loss on fair value remeasurement of deferred purchase consideration	(230)	638
Loss (gain) on fair value remeasurement of contingent consideration	740	(288)
Total costs and expenses	39,663	85,598
Loss from operations	(39,663)	(81,706)
Other income (expense):		
Interest income	2,711	664
Interest expense	(5,814)	(7,266)
Grant income	4,825	—
Loss on extinguishment of debt	—	(2,583)
Gain on sale of commercial business	—	46,995
Other (expense) income, net	(4,258)	(926)
Total other (expense) income	(2,536)	36,884
Net loss	$ (42,199)	$ (44,822)
Net loss per share attributable to common stockholders—basic and diluted	$ (17.35)	$ (29.48)
Weighted average shares outstanding—basic and diluted	2,432,008	1,520,611
Net loss	$ (42,199)	$ (44,822)
Other comprehensive income:		
Change in unrealized gain on investments	—	—
Total other comprehensive income	—	—
Total comprehensive loss	$ (42,199)	$ (44,822)

The accompanying notes are an integral part of these consolidated financial statements.

KALA BIO, INC.

CONSOLIDATED STATEMENTS OF CHANGES IN MEZZANINE EQUITY AND STOCKHOLDERS' EQUITY

(In thousands, except share amounts)

	Mezzanine Equity		Stockholders' Equity									
	Series D Preferred Stock $0.001 Par Value		Series E Preferred Stock $0.001 Par Value		Series F Preferred Stock $0.001 Par Value		Common Stock $0.001 Par Value		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2021	—	$ —	—	$ —	—	$ —	1,322,464	$ 1	$ 559,191	$ —	$ (542,388)	$ 16,804
At the market offering, net of offering costs $29	—	—	—	—	—	—	148,461	1	1,036	—	—	1,037
Exercise of stock options	—	—	—	—	—	—	102	—	3	—	—	3
Issuance of common stock for vested restricted stock units	—	—	—	—	—	—	9,026	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	—	—	—	—	—	—	13,791	—	298	—	—	298
Issuance of common stock to satisfy deferred purchase consideration	—	—	—	—	—	—	136,314	—	7,936	—	—	7,936
Issuance of redeemable Series D preferred stock	73,208	—	—	—	—	—	—	—	—	—	—	—
Redemption of redeemable Series D preferred stock	(73,208)	—	—	—	—	—	—	—	—	—	—	—
Issuance of common stock, net of issuance cost of $3	—	—	—	—	—	—	76,813	—	438	—	—	438
Issuance of convertible Series E preferred stock and Second Closing Right, net of issuance costs of $43	—	—	9,666	—	—	—	—	—	5,515	—	—	5,515
Issuance of convertible Series E preferred stock upon settlement of Second Closing Right, net of issuance costs of $194	—	—	43,478	—	—	—	—	—	24,807	—	—	24,807
Stock-based compensation expense	—	—	—	—	—	—	—	—	6,958	—	—	6,958
Net loss	—	—	—	—	—	—	—	—	—	—	(44,822)	(44,822)
Balance as of December 31, 2022	—	$ —	53,144	$ —	—	$ —	1,706,971	$ 2	606,182	$ —	$ (587,210)	$ 18,974
At the market offering, net of offering costs of $458	—	—	—	—	—	—	731,521	1	18,535	—	—	18,536
Issuance of common stock for vested restricted stock units	—	—	—	—	—	—	3,002	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	—	—	—	—	—	—	3,690	—	46	—	—	46
Issuance of common stock to satisfy deferred purchase consideration	—	—	—	—	—	—	19,350	—	365	—	—	365
Issuance of common stock to satisfy contingent consideration	—	—	—	—	—	—	105,038	—	2,354	—	—	2,354
Issuance of common stock upon conversion of Series E Preferred Stock	—	—	(1,898)	—	—	—	189,800	—	—	—	—	—
Issuance of convertible Series F preferred stock, net of issuance cost of $35	—	—	—	—	2,928	—			1,965			1,965
Stock-based compensation expense	—	—	—	—	—	—	—	—	7,463	—	—	7,463
Net loss	—	—	—	—	—	—	—	—	—	—	(42,199)	(42,199)
Balance as of December 31, 2023	—	$ —	51,246	$ —	2,928	$ —	2,759,372	$ 3	$ 636,910	$ —	$ (629,409)	$ 7,504

The accompanying notes are an integral part of these consolidated financial statements.

KALA BIO, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,	
	2023	**2022**
Cash flows from operating activities:		
Net loss	$ (42,199)	$ (44,822)
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	303	537
Non-cash operating lease cost	171	439
Gain on sale of commercial business	—	(46,995)
Loss on extinguishment of debt	—	2,583
(Gain) loss on fair value remeasurement of deferred purchase consideration	(230)	638
Loss (gain) on fair value remeasurement of contingent consideration	740	(288)
Amortization of debt discount and other non-cash interest	1,253	1,425
Stock-based compensation	7,463	7,008
Other non-cash (gains) losses, net	(4,323)	76
Change in operating assets and liabilities:		
Accounts receivable	78	15,138
Prepaid expenses and other current assets	5,756	(2,009)
Inventory and assets held for sale	7,544	1,708
Other long-term assets	(144)	—
Accounts payable	(1,904)	(2,076)
Accrued expenses and other current liabilities	(2,422)	(11,926)
Lease liabilities and other long-term liabilities	(13)	(344)
Net cash used in operating activities	(27,927)	(78,908)
Cash flows from investing activities:		
Proceeds from sale of commercial business, net of transaction costs	—	62,908
Purchases of property and equipment and other assets	(610)	(313)
Proceeds from sale of property and equipment	47	114
Purchases of short-term investments	(9,866)	(4,992)
Proceeds from sales or maturities of short-term investments	10,000	5,000
Net cash (used in) provided by investing activities	(429)	62,717
Cash flows from financing activities:		
Payment of principal, prepayment premium and final payment fee on debt	(10,000)	(40,000)
Proceeds from issuance of common stock and Series E preferred stock, net of issuance costs of $240	—	30,760
Proceeds from issuance of Series F preferred stock, net of issuance costs of $35	1,965	—
Proceeds from common stock offerings, net of offering costs	18,536	1,036
Contingent consideration related to Combangio acquisition	(2,041)	—
Payment of principal on finance lease	—	(39)
Proceeds from exercise of stock options and issuance of common stock under employee stock purchase plan	46	301
Net cash provided by (used in) financing activities	8,506	(7,942)
Net decrease in cash, cash equivalents and restricted cash:	(19,850)	(24,133)
Cash, cash equivalents and restricted cash at beginning of period	70,745	94,878
Cash, cash equivalents and restricted cash at end of period	$ 50,895	$ 70,745
Reconciliation of cash, cash equivalents and restricted cash:		
Cash, cash equivalents, and restricted cash at end of period	$ 50,895	$ 70,745
Less restricted cash	—	(250)
Cash and cash equivalents at end of period	$ 50,895	$ 70,495
Non-cash investing and financing activities:		
Purchases of property and equipment in accounts payable and accrued expenses	$ —	$ 9
Issuance of common stock to satisfy deferred purchase consideration in additional paid-in capital	365	7,936
Issuance of common stock to satisfy contingent consideration in additional paid-in capital	2,354	—
Supplemental disclosure:		
Cash paid for interest	$ 4,620	$ 5,958
Right-of-use assets obtained in exchange of operating lease obligations	2,180	424

The accompanying notes are an integral part of these consolidated financial statements.

Note 1: Nature of Business

Nature of Business— KALA BIO, Inc. (the "Company") was incorporated on July 7, 2009, and is a clinical-stage biopharmaceutical company dedicated to the research, development and commercialization of innovative therapies for rare and severe diseases of the front and back of the eye. On August 2, 2023, the Company changed its name from Kala Pharmaceuticals, Inc. to KALA BIO, Inc.

On November 15, 2021, the Company and its newly formed, direct wholly owned subsidiary, Ceres Merger Sub, Inc. (the "Merger Subsidiary"), entered into an Agreement and Plan of Merger (the "Merger Agreement") with Combangio, Inc. ("Combangio") and Fortis Advisors LLC, solely in its capacity as Combangio Equityholder Representative in connection with the Merger Agreement, pursuant to which on November 15, 2021, the Merger Subsidiary merged with and into Combangio with Combangio surviving such merger and becoming a direct wholly owned subsidiary of the Company (the "Combangio Acquisition"). In connection with the Combangio Acquisition, the Company acquired Combangio's mesenchymal stem cell secretomes ("MSC-S") platform, including its lead product candidate for the treatment of persistent corneal epithelial defects ("PCED"), which the Company refers to as KPI-012. PCED is a rare disease of impaired corneal healing. The Company submitted an investigational new drug application, ("IND") to the U.S. Food and Drug Administration, ("FDA"), which was accepted in December 2022. In February 2023, the Company dosed its first patient in the CHASE ("**C**orneal **H**ealing **A**fter **SE**cretome therapy") Phase 2b clinical trial of KPI-012 for PCED in the United States. KPI-012 has received both Orphan Drug and Fast Track designations from the FDA for the treatment of PCED. The Company expects to commercialize in the United States any of its product candidates that receive marketing approval. In connection with the determination to focus its research and development efforts on KPI-012, in 2022, the Company ceased the development of its preclinical pipeline programs that are unrelated to its MSC-S platform.

The Company previously developed and commercialized two marketed products, EYSUVIS® (loteprednol etabonate ophthalmic suspension) 0.25%, for the short-term (up to two weeks) treatment of the signs and symptoms of dry eye disease, and INVELTYS® (loteprednol etabonate ophthalmic suspension) 1%, a topical twice-a-day ocular steroid for the treatment of post-operative inflammation and pain following ocular surgery. Both products applied a proprietary mucus-penetrating particle drug delivery technology, which the Company referred to as the AMPPLIFY® Drug Delivery Technology. On July 8, 2022, the Company closed the transaction (the "Alcon Transaction"), contemplated by the asset purchase agreement, dated as of May 21, 2022 (the "Asset Purchase Agreement"), by and between the Company, Alcon Pharmaceuticals Ltd. and Alcon Vision, LLC (together referred to as "Alcon"), pursuant to which Alcon purchased the rights to manufacture, sell, distribute, market and commercialize EYSUVIS and INVELTYS and to develop, manufacture, market and otherwise exploit the Company's AMPPLIFY Drug Delivery Technology (collectively, the "Commercial Business"). Alcon also assumed certain liabilities with respect to the Commercial Business at the closing of the Alcon Transaction. See Note 3, "Acquisitions and Divestitures" for additional information about the Alcon Transaction and the Combangio Acquisition.

The Company's success is dependent upon its ability to develop, obtain regulatory approval for and commercialize KPI-012 and any other product candidate it may develop in the future, the success of its research and development efforts, whether it receives any commercial-based sales milestone payments from Alcon, its ability to raise additional capital when needed and, ultimately, attain profitable operations.

Reverse Stock Split— On October 20, 2022, the Company effected a 1-for-50 reverse stock split of the Company's shares of common stock either issued and outstanding or held by the Company as treasury stock (the "Reverse Stock Split"). As a result of the Reverse Stock Split, every 50 shares of issued and outstanding common stock were automatically combined into one issued and outstanding share of common stock, without any change in the par value per share. No fractional shares were issued as a result of the Reverse Stock Split. Any fractional shares that would otherwise have resulted from the Reverse Stock Split were rounded up to the next whole number. The number of authorized shares of common stock under the Company's Restated Certificate of Incorporation, as amended, remained

unchanged at 120,000,000 shares. All historical share and per share amounts reflected throughout these financial statements have been adjusted to reflect the Reverse Stock Split. Proportionate adjustments were made to the per share exercise price and the number of shares of common stock that may be purchased upon exercise of outstanding stock options and warrants, and the number of shares of common stock reserved for future issuance under the Company's 2017 Equity Incentive Plan and Employee Stock Purchase Plan.

Recent Equity Financings— On May 7, 2020, the Company filed a shelf registration statement on Form S-3 with the SEC, which was declared effective on May 19, 2020 (the "2020 Shelf Registration"). Under the 2020 Shelf Registration, the Company may offer and sell up to $350,000 of a variety of securities including common stock, preferred stock, warrants, depositary shares, debt securities or units during the three-year period that commenced upon the 2020 Shelf Registration becoming effective. In connection with the filing of the 2020 Shelf Registration, the Company entered into an amended and restated sales agreement (the "Amended and Restated Sales Agreement") with Jefferies LLC ("Jefferies") pursuant to which the Company could issue and sell, from time to time, up to an aggregate of $75,000 of its common stock in an at-the-market equity offering through Jefferies, as a sales agent. During the year ended December 31, 2022, the Company issued and sold an additional 148,461 shares of its common stock under its at-the-market offering pursuant to the terms of the Amended and Restated Sales Agreement, resulting in net proceeds of $1,036. From January 1, 2023 to January 10, 2023, the Company sold 245,887 shares of its common stock pursuant to the terms of the Amended and Restated Sales Agreement, resulting in net proceeds of $9,994. On January 10, 2023, the Amended and Restated Sales Agreement terminated in accordance with its terms when the Company completed the sale of $75,000 of its shares of common stock thereunder. As of the date of termination of the Amended and Restated Sales Agreement, the Company had sold an aggregate of 565,974 shares of its common stock under such agreement for aggregate gross proceeds of $75,000.

On January 19, 2023, the Company entered into an Open Market Sale Agreement with Jefferies (the "Open Market Sale Agreement"), pursuant to which the Company may issue and sell, from time to time, shares its common stock under an at-the-market equity offering. The Company filed a prospectus supplement relating to the Open Market Sale Agreement under its 2020 Shelf Registration (the "2020 Shelf ATM Prospectus Supplement"), pursuant to which the Company could offer and sell shares of common stock having an aggregate offering price of up to $40,000 under the Open Market Sale Agreement. From January 19, 2023 to May 11, 2023, the Company sold 229,378 shares of its common stock under its at-the-market offering pursuant to the Open Market Sale Agreement under the 2020 Shelf ATM Prospectus Supplement, resulting in net proceeds of $4,899.

On March 3, 2023, the Company filed a shelf registration statement on Form S-3 with the SEC, which was declared effective on May 11, 2023 (the "2023 Shelf Registration"). Under the 2023 Shelf Registration, the Company may offer and sell up to $350,000 of a variety of securities including common stock, preferred stock, warrants, depositary shares, debt securities, subscription rights or units after such time as the shelf registration statement is declared effective by the SEC. In accordance with the terms of the Open Market Sale Agreement, the Company may issue and sell, from time to time, up to $40,000 of its common stock in an at-the-market equity offering through Jefferies, as sales agent. Upon effectiveness of the 2023 Shelf Registration, the Company ceased any further offers or sales of its common stock pursuant to the 2020 Shelf ATM Prospectus Supplement and the 2020 Shelf Registration. During the year ended December 31, 2023, the Company sold 256,256 shares of its common stock under its at-the-market offering pursuant to the 2023 Shelf Registration for total net proceeds of $3,642.

During the year ended December 31, 2023, the Company sold an aggregate of 731,521 shares of its common stock pursuant to (1) the Amended and Restated Sales Agreement and the Open Market Sale Agreement under the 2020 Shelf Registration and (2) the Open Market Sale Agreement under the 2023 Shelf Registration, for total net proceeds of $18,536.

On November 28, 2022, the Company entered into a Securities Purchase Agreement (the "2022 Securities Purchase Agreement") with certain institutional investors named therein, pursuant to which the Company agreed to issue

and sell, in a private placement priced at-the-market under Nasdaq rules, shares of common stock and shares of Series E Convertible Non-Redeemable Preferred Stock, par value $0.001 per share, of the Company (the "Series E Preferred Stock"), in two tranches for aggregate gross proceeds of up to $31,000 (collectively, the "2022 Private Placement"). Pursuant to the 2022 Securities Purchase Agreement, on December 1, 2022, the Company issued and sold to the purchasers at the first closing of the 2022 Private Placement, (i) 76,813 shares of common stock, at a price per common share equal to $5.75 and (ii) 9,666 shares of Series E Preferred Stock, at a price per share equal to $575.00, for aggregate gross proceeds of approximately $6,000. On December 27, 2022, following the certification by the Chief Executive Officer of the Company that the FDA accepted the Company's IND for KPI-012, the Company issued and sold to the purchasers at a second closing of the 2022 Private Placement a total of 43,478 shares of Series E Preferred Stock, at a price per share equal to $575.00, for aggregate gross proceeds of approximately $25,000. Costs incurred in connection with the 2022 Private Placement were $240, which were recorded as a reduction to additional paid-in capital.

On December 21, 2023, the Company entered into a Securities Purchase Agreement (the "2023 Securities Purchase Agreement") with certain institutional investors named therein, pursuant to which the Company agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of Series F Convertible Non-Redeemable Preferred Stock, par value $0.001 per share, of the Company (the "Series F Preferred Stock"), for aggregate gross proceeds of approximately $2,000 (the "2023 Private Placement"). Pursuant to the 2023 Securities Purchase Agreement, the Company issued and sold to the purchasers at the closing of the 2023 Private Placement, 2,928 shares of Series F Preferred Stock, at a price per preferred share equal to $683.00. Costs incurred in connection with the 2023 Private Placement were $35, which were recorded as a reduction to additional paid-in capital.

On March 25, 2024, the Company entered into a Securities Purchase Agreement (the "2024 Securities Purchase Agreement") with certain institutional investors named therein, pursuant to which the Company agreed to issue and sell, in a private placement priced at-the-market under Nasdaq rules, shares of Series G Convertible Non-Redeemable Preferred Stock, par value $0.001 per share, of the Company (the "Series G Preferred Stock"), for aggregate gross proceeds of approximately $8,600 (the "2024 Private Placement"). Pursuant to the 2024 Securities Purchase Agreement, the Company issued and sold to the purchasers at the closing of the 2024 Private Placement, 10,901 shares of Series G Preferred Stock, at a price per preferred share equal to $788.90.

Refer to Note 11, "Debt" for a discussion of debt financing activity.

Note 2: Summary of Significant Accounting Policies

Principles of Consolidation—The accompanying consolidated financial statements include the accounts of KALA BIO, Inc. and its wholly owned subsidiaries, Kala Pharmaceuticals Security Corporation, which is a Massachusetts subsidiary created to buy, sell and hold securities, and Combangio, Inc. All intercompany transactions and balances have been eliminated.

Basis of Presentation—The accompanying consolidated financial statements have been prepared on a going concern basis which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. The Company generated only limited revenues from product sales prior to the sale of the Commercial Business to Alcon in July 2022 and has incurred recurring losses and negative cash flows from operations, including a net loss of $42,199 and $44,822, for the years ended December 31, 2023 and 2022, respectively, and used cash in operations of $27,927 and $78,908, in the years ended December 31, 2023 and 2022, respectively. The Company has financed its operations to date primarily through proceeds from the sale of the Commercial Business to Alcon, its initial public offering of common stock, follow-on public offerings of common stock and sales of its common stock under its at-the-market offering facility, private placements of common stock and preferred stock (including the Company's 2022 Private Placement, 2023 Private Placement and 2024 Private Placement), borrowings under credit facilities and the Loan and Security Agreement with Oxford Finance LLC (the "Loan Agreement"), convertible promissory notes and warrants. In August 2023, following entry into the award agreement with the California Institute for Regenerative Medicine

("CIRM"), Combangio received an initial $5,900 disbursement from CIRM, and the balance of the total $15,000 award is payable to Combangio upon the achievement of specified milestones (see Note 6, "Grant Income" for further information about the CIRM Award (as defined below)). The Company has devoted substantially all of its financial resources and efforts to research and development, including preclinical studies and clinical trials, and, prior to the sale of the Commercial Business to Alcon in July 2022, engaging in activities to launch and commercialize EYSUVIS and INVELTYS. As a result of the Combangio Acquisition and the sale of the Commercial Business to Alcon, the Company is devoting substantial financial resources to the research and development and potential commercialization of KPI-012 for PCED and any other indications the Company determines to pursue, including Limbal Stem Cell Deficiency. The Company has no revenue-generating commercial products and, as a result of the Combangio Acquisition, may be required to pay certain milestones and royalty payments to former equityholders of Combangio. Although the Company is eligible to receive up to $325,000 in payments from Alcon based upon the achievement of specified commercial sales-based milestones with respect to EYSUVIS and INVELTYS, there can be no assurance when the Company may receive such milestone payments or of the amount of milestone payments the Company may receive, if any. The Company cannot be certain that it will achieve the milestones within the timeframe required by the CIRM Award, or at all, and as such the Company may never receive the remaining $9,100 under the award. The Company expects to continue to incur significant expenses and operating losses for the foreseeable future, including in connection with its continued development, regulatory approval efforts and commercialization, if any, of KPI-012. The Company may never achieve or maintain profitability. Net losses may fluctuate significantly from quarter-to-quarter and year-to-year.

The Company expects that its cash and cash equivalents as of December 31, 2023, together with the $8,600 of gross proceeds received from the sale of shares of Series G Preferred Stock in March 2024, will enable it to fund its operating expenses, lease and debt service obligations and capital expenditure requirements for at least 12 months from the date these consolidated financial statements were issued. This evaluation is based on relevant conditions and events that are known and reasonably knowable at the date that the consolidated financial statements are issued. To the extent these conditions or events change, the Company could deplete its available capital resources sooner than it currently expects.

Use of Estimates— The preparation of consolidated financial statements in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP") requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenue, expense, and related disclosures. The Company bases estimates and assumptions on historical experience when available and on various factors that it believes to be reasonable under the circumstances. The Company evaluates its estimates and assumptions on an ongoing basis. Estimates and assumptions relied upon in preparing these consolidated financial statements relate to, but are not limited to, the present value of lease liabilities and the corresponding right-of-use assets, the fair value of warrants, stock-based compensation, accrued expenses, contingent consideration, grant income and deferred grant income, the valuation of embedded derivatives and the recoverability of the Company's net deferred tax assets and related valuation allowance. Actual results may differ from these estimates under different assumptions or conditions.

Grant Income— Grant income consists of amounts earned from incurring costs to support the CHASE Phase 2b clinical trial of KPI-012 for PCED, as well as product process characterization and analytical development from the program due to the receipt of the CIRM Award. The grant between the Company and CIRM is subject to a co-funding requirement and generally provides for the Company to meet certain milestones in order for funds to be provided. The Company accounts for grants received to perform research and development activities in accordance with Accounting Standards Codification ("ASC") Topic 730-20, *Research and Development Arrangements*, which requires an assessment, at the inception of the grant, of whether the grant is a liability or a contract to perform research and development activities. If the Company is obligated to repay the grant funds to the grantor regardless of the outcome of the research and development activities, then the Company is required to estimate and recognize that liability. Alternatively, if the Company is not required to repay, or if it is required to repay the grant funds to the grantor only if the research and development activities are successful, then the grant agreement is accounted for as a contract to perform research and development activities, in which case, grant income is recognized as the related research and development

expenses are incurred. Costs of grant income are recorded as a component of research and development expenses in the Company's consolidated statements of operations and comprehensive loss as opposed to grant revenue.

Grant funds received in advance are recorded as deferred grant income on the consolidated balance sheets. Management has determined that the Company is the principal participant under the Company's CIRM Award, and accordingly, the Company records amounts earned under this arrangement as grant income on the consolidated statements of operations and comprehensive loss.

Product Revenues, Net— Following the sale of its Commercial Business to Alcon in July 2022, the Company no longer has any commercial products in its portfolio. The Company sold EYSUVIS for the short-term (up to two weeks) treatment of the signs and symptoms of dry eye disease, and INVELTYS, its topical twice-a-day ocular steroid for the treatment of post-operative inflammation and pain following ocular surgery, primarily to wholesalers in the United States ("Customers"). These Customers subsequently resold the Company's products to specialty and other retail pharmacies. In addition to agreements with Customers, the Company entered into arrangements with third-party payors that provided for government-mandated and/or privately-negotiated rebates, chargebacks and discounts for the purchase of its products.

The Company accounted for revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers*. Under ASC Topic 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to be entitled in exchange for those goods or services. The Company performed the following five steps to recognize revenue under ASC Topic 606: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only recognized revenue when it was probable that it would collect the consideration to which it was entitled in exchange for the goods or services that would be transferred to the customer.

Performance Obligations

The Company determined that performance obligations were satisfied and revenue was recognized when a customer took control of the Company's products, which occurred at a point in time. This generally occurred upon delivery of the products to customers, at which point the Company recognized revenue and recorded accounts receivable. Payment was typically received 70 to 90 days after satisfaction of the Company's performance obligations.

Transaction Price and Variable Consideration

Revenue was measured as the amount of consideration the Company expected to receive in exchange for transferring products to a customer ("transaction price"). The transaction price for product sales included variable consideration related to chargebacks, rebates, sales incentives and allowances, distribution service fees, and returns. The Company estimated the amount of variable consideration that should have been included in the transaction price. These estimates took into consideration a range of possible outcomes that were probability-weighted for relevant factors such as the Company's historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. These provisions reflected the Company's best estimates of the amount of consideration to which it was entitled based on the terms of the contract. The amount of variable consideration that was included in the transaction price may be constrained and was included in net sales only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized would not occur in a future period. In general, performance obligations did not include any estimated amounts of variable consideration that were constrained. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

(In thousands, except share and per share amounts)

The following table summarizes activity in each of the Company's product revenue provision and allowance categories for the years ended December 31, 2023 and 2022:

	Trade Discounts, Allowances and Chargebacks (1)	Product Returns (2)	Rebates and Incentives (3)
Balance as of January 1, 2022	$ 2,672	$ 1,140	$ 11,280
Provision related to current period sales	5,005	291	28,915
Changes in estimate related to prior period sales	(47)	(24)	(200)
Credit/payments made	(7,619)	(889)	(39,223)
Balance as of December 31, 2022	$ 11	$ 518	$ 772
Changes in estimate related to prior period sales	(9)	1,355	(240)
Credit/payments made	(2)	(224)	(522)
Balance as of December 31, 2023	$ —	$ 1,649	$ 10

(1) Trade discounts, allowances and chargebacks included fees for distribution service fees, prompt pay and other discounts, and chargebacks. Estimated trade discounts, allowances and chargebacks were deducted from gross revenue at the time revenues were recognized and were recorded as a reduction to accounts receivable on the Company's consolidated balance sheets.

(2) Estimated provisions for product returns were generally deducted from gross revenues at the time revenues were recognized and are included in accrued expenses and other current liabilities on the Company's consolidated balance sheets.

(3) Rebates and incentives included managed care rebates, government rebates, co-pay program incentives, and sales incentives and allowances. Estimated provisions for rebates and discounts were deducted from gross revenues at the time revenues were recognized and are included in accrued expenses and other current liabilities on the Company's consolidated balance sheets.

As of December 31, 2023 and 2022, the Company did not have any transaction price allocated to remaining performance obligations and any costs to obtain contracts with customers, including pre-contract costs and set up costs, were immaterial.

Accounts Receivable, net—Accounts receivable are reported on the consolidated balance sheets at outstanding amounts due from customers for product sales. The Company deducts sales discounts for prompt payments and other discounts, contractual fees for service arrangements, and chargebacks from accounts receivable. The Company evaluates the collectability of accounts receivable on a regular basis, by reviewing the financial condition and payment history of customers, an overall review of collections experience on other accounts, and economic factors or events expected to affect future collections experience. An allowance for doubtful accounts is recorded when a receivable is deemed to be uncollectible.

The Company recorded no allowance for doubtful accounts as of December 31, 2023 and 2022. The Company recorded an allowance of $11 for expected sales discounts, related to prompt pay discounts and other discounts, contractual fee for service arrangements and chargebacks, to wholesalers and distributors as of December 31, 2022 and did not record any allowance for expected sales discounts as of December 31, 2023.

Contingent Consideration—In addition to upfront consideration and Deferred Purchase Consideration (as defined below) (see Note 3), the Company's asset acquisitions may also include contingent consideration payments to be made for future milestone events. The Company assesses whether such contingent consideration is required to be recorded at fair value on the date of the acquisition and subsequently remeasured to fair value at each reporting date. Contingent consideration payments in an asset acquisition not required to be accounted for at fair value are recognized when the contingency is resolved, and the consideration is paid or becomes payable. Changes to contingent consideration obligations can result from changes to discount rates, accretion of the liability due to the passage of time,

changes in the Company's estimates of the likelihood or timing of achieving certain milestones. Any changes in the fair value of these contingent consideration liabilities are included in loss from operations in the consolidated statements of operations and comprehensive loss.

Derivative Instruments—ASC 815, *Derivatives and Hedging*, ("ASC 815") requires companies to bifurcate certain conversion options and redemption features from their host instruments and account for them as free-standing derivative financial instruments should certain criteria be met. The Company evaluates its financial instruments, including its debt arrangements, to determine whether such instruments are derivatives or contain features that qualify as embedded derivatives. Embedded derivatives must be separately measured from the host contract if all the requirements for bifurcation are met. The assessment of the conditions surrounding the bifurcation of embedded derivatives depends on the nature of the host contract and the features of the derivatives. Bifurcated embedded derivatives are recognized at fair value, with changes in fair value recognized in the consolidated statement of operations and comprehensive income each period. Bifurcated embedded derivatives are classified with the related host contract in the Company's consolidated balance sheet.

Cost of Product Revenues—The cost of product revenues consists primarily of materials, third-party manufacturing costs, freight and distribution costs, royalty expense, allocation of labor, quality control and assurance, reserves for defective inventory as well as excess or obsolete inventory, and other manufacturing overhead costs. The Company recorded the cost of product revenues related to INVELTYS as research and development expenses prior to regulatory approval and recorded the cost of product revenues related to EYSUVIS as research and development expenses prior to the determination that FDA approval was probable and before the future economic benefit of the drug was expected to be realized.

Cash and Concentration of Credit Risk—Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents, short-term investments, if any, and accounts receivable. Periodically, the Company maintains cash, cash equivalents, short-term investments in accredited financial institutions in excess of federally insured limits. The Company deposits its cash, cash equivalents, short-term investments, if any, in financial institutions that it believes have high credit quality and has not experienced any losses on such accounts and does not believe it is exposed to any unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company's accounts receivable balance as of December 31, 2023 and 2022 were *de minimis*. The Company had no revenue during the year ended December 31, 2023. Three customers comprised 10% or more of the Company's revenue during the year ended December 31, 2022. These Customers comprised 47%, 28% and 21% of revenue, respectively. The Company has no financial instruments with off-balance sheet risk of loss.

Cash Equivalents—The Company considers all short-term, highly liquid investments with original maturities of 90 days or less at acquisition date to be cash equivalents.

Restricted Cash— As of December 31, 2023, the Company had no long-term restricted cash. As of December 31, 2022, the Company had long-term restricted cash of $250.

Investments—The Company determines the appropriate classification of its investments at the time of purchase. The Company's investments are classified as available-for-sale in accordance with ASC Topic 320, *Investments—Debt and Equity Securities*. The Company classifies investments available to fund current operations as current assets on its consolidated balance sheets. Investments are classified as long-term assets on the consolidated balance sheets if (i) the Company has the intent and ability to hold the investments for a period of at least one year and (ii) the contractual maturity date of the investments is greater than one year.

Available-for-sale investments are recorded at fair value, with unrealized gains or losses included in comprehensive loss on the consolidated statements of operations and comprehensive loss and in accumulated other comprehensive income or loss on the consolidated balance sheets. Realized gains and losses, interest income earned on the Company's cash, cash equivalents and investments, and amortization or accretion of discounts and premiums on investments are included within other income (expense).

The Company reviews investments for other-than-temporary impairment whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. The Company did not record any such impairments during the years ended December 31, 2023 or 2022.

Assets Held for Sale—The Company classifies its long-lived assets to be sold as held for sale, as specified by ASC 360, *Property, Plant, and Equipment*, in the period (i) it has approved and committed to a plan to sell the asset, (ii) the asset is available for immediate sale in its present condition, (iii) an active program to locate a buyer and other actions required to sell the asset have been initiated, (iv) the sale of the asset is probable, (v) the asset is being actively marketed for sale at a price that is reasonable in relation to its current fair value and (vi) it is unlikely that significant changes to the plan will be made or that the plan will be withdrawn. The Company initially measures a long-lived asset that is classified as held for sale at the lower of its carrying value or fair value less any costs to sell. Any loss resulting from this measurement is recognized in the period in which the held for sale criteria are met. Conversely, gains are not recognized on the sale of a long-lived asset until the date of sale. Upon designation as an asset held for sale, the Company stops recording depreciation and amortization expense on long-lived assets. The Company assesses the fair value of a long-lived asset less any costs to sell at each reporting period and until the asset is no longer classified as held for sale.

There were no assets held for sale as of December 31, 2023. As of December 31, 2022, certain assets, including EYSUVIS and INVELTYS inventory, met the criteria to be classified as held for sale. Fair value was determined based on the estimated proceeds from the sale of the assets. The Company reclassified the inventory and property and equipment, which had a combined net realizable value of $7,595, to current assets held for sale on the consolidated balance sheet as of December 31, 2022. See Note 4, "Assets Held for Sale", for additional information.

Leases—At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease. Most leases with a term greater than one year are recognized on the balance sheet as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. The Company has elected not to recognize on the balance sheet leases with terms of one-year or less. Lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected lease term. The interest rate implicit in lease contracts is typically not readily determinable. As such, the Company utilizes the appropriate incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment. Certain adjustments to the right-of-use asset may be required for items such as initial direct costs paid or incentives received.

The components of a lease should be split into three categories: lease components (e.g., land, building, etc.), non-lease components (e.g., common area maintenance, maintenance, consumables, etc.), and non-components (e.g., property taxes, insurance, etc.). Then the fixed and in-substance fixed contract consideration (including any related to

non-components) must be allocated based on fair values to the lease components and non-lease components. Although separation of lease and non-lease components is required, certain practical expedients are available to entities. Entities electing the practical expedient would not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. The Company's facilities operating leases had lease and non-lease components which the Company has elected to use the practical expedient and account for each lease component and related non-lease component as one single component. The lease component resulted in a right-of-use asset being recorded on the consolidated balance sheets and amortized as lease expense on a straight-line basis to the consolidated statements of operations and comprehensive loss.

Property and Equipment, net—Property and equipment are recorded at cost. Depreciation is provided using the straight-line method over the estimated useful lives of the related assets. Depreciation expense is included in loss from operations on the consolidated statements of operations and comprehensive loss. Laboratory equipment and office and computer equipment is depreciated over three to five years. Leasehold improvements are depreciated over the shorter of their useful life or the life of the lease. Major additions and upgrades are capitalized; maintenance and repairs, which do not improve or extend the life of the respective assets, are expensed as incurred. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in loss from operations on the consolidated statements of operations and comprehensive loss.

Patent Costs—Costs to secure and defend patents are expensed as incurred and are classified as selling, general and administrative expenses in the Company's consolidated statements of operations and comprehensive loss.

Advertising Costs—Advertising costs are expensed as incurred. The Company incurred no advertising costs for the year ended December 31, 2023 and incurred $11,249 of advertising costs for the year ended December 31, 2022, which were included in selling, general and administrative expenses in the accompanying consolidated statement of operations and comprehensive loss.

Impairment of Long-Lived Assets—Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If the undiscounted cash flows are insufficient to recover the carrying value, the assets are recorded at the lesser of the carrying value or fair value. The Company recorded no impairment charges for the year ended December 31, 2023 and impairment charges recorded for the year ended December 31, 2022 were *de minimis*.

Segment Information—Operating segments are identified as components of an enterprise about which separate discrete financial information is made available for evaluation by the chief operating decision maker ("CODM") in making decisions regarding resource allocation and assessing performance. The CODM is the Company's Chief Executive Officer. The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on the development and commercialization of innovative therapies for diseases of the eye. All of the Company's tangible assets are held in the United States. To date, all of the Company's revenue has been generated in the United States.

Research and Development Costs—Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for full-time research and development employees, an allocation of facilities expenses, overhead expenses and other outside expenses. Research and development costs are expensed as incurred. The Company expenses costs relating to the production of inventory for its product candidates as research and development expenses within its consolidated statements of operations and comprehensive loss in the period incurred, until the point the Company believes regulatory approval and subsequent commercialization of the product candidate is probable and it expects the future economic benefit from sales of the drug to be realized. Research and development costs that are paid in advance of performance, including nonrefundable prepayments for goods or services, are deferred and capitalized as a prepaid expense. Such amounts are recognized as an expense as the goods are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

Accrued Expenses— The Company accrues for variable consideration related to rebates, sales incentives and allowances, and returns. Such estimates are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of the accrued expense. The Company also accrues expenses related to development activities performed by third parties based on an evaluation of services received and efforts expended pursuant to the terms of the contractual arrangements. Payments under some of these contracts depend on clinical trial milestones. There may be instances in which payments made to the Company's vendors will exceed the level of services provided and result in a prepayment of expenses. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual or prepaid expense accordingly.

Stock-Based Compensation—The Company accounts for all stock-based awards granted as compensation expense at fair value. The Company generally issues stock-based awards with the measurement date for awards as the date of grant. Stock-based compensation costs are recognized as expense over the employees' requisite service period, which is the vesting period, on a straight-line basis. For performance awards whose vesting is contingent upon a specified event, the Company recognizes stock-based compensation expense over the derived service period, based on the probability of achievement of the specified event. The Company recognizes compensation expense for the portion of awards that have vested. Forfeitures are recorded as they occur. Stock-based compensation is classified in the accompanying consolidated statements of operations and comprehensive loss based on the function to which the related services are provided, or capitalized with inventory until related expense is recognized.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The assumptions used in calculating the fair value of stock-based payment awards represent management's best estimates. The Company previously lacked sufficient company-specific historical and implied volatility information. Therefore, it estimated its expected stock volatility based on the historical volatility of a publicly traded set of peer companies in addition to its own historical volatility. Beginning in the second half of 2023, the Company determined it had adequate historical data regarding the volatility of its own traded stock price and began exclusively using its own historical volatility. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future. The fair value of restricted stock units ("RSUs") and performance stock units ("PSUs") are equal to the closing sale price of the Company's common stock on the date of grant.

Income Taxes—Deferred tax assets and liabilities are recognized for the expected future tax consequences of events that have been included in the Company's consolidated financial statements and tax returns. Deferred tax assets and liabilities are determined based upon the differences between the consolidated financial statement carrying amounts and the tax basis of existing assets and liabilities and for loss and credit carryforwards using enacted tax rates expected to be in effect in the years in which the differences are expected to reverse. Deferred tax assets are reduced by a valuation allowance if it is more likely than not that some portion or all of the deferred tax asset will not be realized.

The Company provides reserves for potential payments of tax to various tax authorities related to uncertain tax positions and other issues. The Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not that the position will be sustained on examination by the taxing authorities based on the technical merits of the position. The tax benefits recognized in the consolidated financial statements from such a position is measured based on the largest benefit that has a greater than 50% likelihood of being realized upon ultimate settlement. As a result, reserves are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies present.

Net Loss per Share Attributable to Common Stockholders—The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The two-class method is not applicable during periods with a net loss, as the holders of the convertible preferred stock have no contractual obligation to share in losses. For all periods presented, the two-class method was not applicable.

Basic net loss per share attributable to common stockholders is computed using the weighted-average number of common shares outstanding during the period. Diluted net loss per share attributable to common stockholders is computed using the weighted average number of common shares outstanding during the period and, if dilutive, the weighted average number of potential shares of common stock, including the assumed exercise of stock options and warrants, the issuance of unvested RSUs and PSUs and convertible preferred stock using the if-converted method.

The weighted average number of common shares included in the computation of diluted net loss gives effect to all potentially dilutive common equivalent shares, including outstanding stock options, warrants, unvested RSUs and PSUs and convertible preferred stock using the if-converted method. Common stock equivalent shares are excluded from the computation of diluted net loss per share attributable to common stockholders if their effect is antidilutive. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common stockholders for the years ended December 31, 2023 and 2022. (See Note 15).

Recent Accounting Pronouncements

Management has considered all recent accounting pronouncements issued since the last audit of our consolidated financial statements. The Company's management believes that these recent pronouncements will not have a material effect on our company's consolidated financial statements.

(In thousands, except share and per share amounts)

Note 3: Acquisitions and Divestitures

Sale of Commercial Business to Alcon

On July 8, 2022, the Company closed the Alcon Transaction contemplated by the Asset Purchase Agreement, pursuant to which Alcon purchased the Commercial Business and assumed certain liabilities with respect to the Commercial Business. Alcon paid to the Company an upfront cash payment of $60,000 upon the closing of the Alcon Transaction. In addition, pursuant to the Asset Purchase Agreement, the Company is eligible to receive from Alcon up to four commercial-based sales milestone payments as follows: (1) $25,000 upon the achievement of $50,000 or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (2) $65,000 upon the achievement of $100,000 or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2028, (3) $75,000 upon the achievement of $175,000 or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029 and (4) $160,000 upon the achievement of $250,000 or more in aggregate worldwide net sales of EYSUVIS and INVELTYS in a calendar year from 2023 to 2029. Each milestone payment will only become payable once, if at all, upon the first time such milestone is achieved, and only one milestone payment will be paid with respect to a calendar year. In the event that more than one milestone is achieved in a calendar year, the higher milestone payment will become payable and the lower milestone payment will become payable only if the corresponding milestone is achieved again in a subsequent calendar year.

Pursuant to the Asset Purchase Agreement, on July 8, 2022, the Company entered into supply and commercial agreements under which the Company agreed to supply EYSUVIS and INVELTYS to Alcon and distribute EYSUVIS and INVELTYS to third-party customers of the Commercial Business on behalf of Alcon for a period of six months following the closing of the Alcon Transaction. In addition, the Company entered into a transition services agreement under which the Company provided certain transition services to Alcon on a cost-plus pricing arrangement for six months following the closing of the Alcon Transaction. Pursuant to the supply agreement, Alcon purchased from the Company, at the closing of the Alcon Transaction, $5,027 of EYSUVIS and INVELTYS inventory on-hand at the Company. Together, the supply, commercial and transition services agreements are referred to herein as the "Transition Agreement."

The Company has determined that the disposition of these assets does not qualify for reporting as a discontinued operation as it was not considered a component of an entity that comprises operations and cash flows that can be clearly distinguished, operationally and for financial reporting purposes, from the rest of the Company. During the year ended December 31, 2022, the Company recognized a net gain on the sale of the Commercial Business as follows:

Gross consideration from the sale of the Commercial Business	$ 65,027
Closing and transaction costs	2,119
Net proceeds from the sale of the Commercial Business	62,908
Book value of assets transferred	
Inventories	8,915
Prepaid expenses and other current assets	556
Property and equipment, net	1,819
Other long-term assets	434
Total book value of assets transferred	11,724
Gain on sale of Commercial Business	51,184
Deferred gain on sale of Commercial Business	4,189
Net gain on sale of Commercial Business	$ 46,995

The Company deferred a portion of the gross consideration related to the discounted pricing on any remaining inventory owned by the Company (the "Remaining Inventory") that Alcon could have purchased. The deferred gain on the sale of the Commercial Business of $4,189 was recorded on the consolidated balance sheet as of the transaction date as deferred gain on sale of Commercial Business. The Remaining Inventory and deferred gain on the sale of the Commercial Business were written off during the year ended December 31, 2023, and the net impact of $3,355 is recorded in the other (expense) income, net line item in the consolidated statements of operations and comprehensive loss.

The Company collected cash on behalf of Alcon for revenue generated by sales of EYSUVIS and INVELTYS from July 8, 2022 through the transition period and the Company transferred all cash generated by such sales to Alcon as of December 31, 2022.

The Company recorded income from the Transition Agreement of $157, which is presented in other income (expense), net on the consolidated statement of operations and comprehensive loss for the year ended December 31, 2023 and which offset operating expenses related to the Transition Agreement captured within loss from operations. Other than noted above, there was no other income from the Transition Agreement recorded in the year ended December 31, 2023. There were no payables due to third parties related to amounts the Company is obligated to pay on Alcon's behalf included on the Company's consolidated balance sheet as of December 31, 2023 and there were no receivables due from Alcon or third parties in connection with the Transition Agreement.

As of December 31, 2022, the Company had total receivables due from Alcon and third parties of $5,394 and $26, respectively, and total payables to third parties related to the Transition Agreement of $3,981 of which $1,737 was included in accounts payable and $2,244 within accrued expenses and other current liabilities related to invoices the Company was obligated to pay on Alcon's behalf. As of December 31, 2022, the Company had a net receivable due from Alcon and third parties in connection with the Transition Agreement of $1,439.

Acquisition of Combangio, Inc.

In connection with the closing of the Combangio Acquisition on November 15, 2021 (the "Closing"), the Company made an upfront payment of an aggregate of $5,000 in cash to former Combangio stockholders and other equityholders (the "Combangio Equityholders"), subject to customary adjustments, and agreed to issue an aggregate of 155,664 shares (the "Deferred Purchase Consideration") of the Company's common stock to the Combangio Equityholders with an aggregate value of approximately $16,123, consisting of (i) an aggregate of 136,314 shares of common stock which were issued on January 3, 2022 (the "Upfront Shares") and (ii) an aggregate of 19,350 shares of common stock that were initially held back as partial security for the satisfaction of indemnification obligations and other payment obligations of the Combangio Equityholders (the "Holdback Shares") and that were issued in March 2023 upon escrow release. The aggregate value of the Deferred Purchase Consideration was calculated using the closing price of the Company's common stock on The Nasdaq Global Select Market on November 12, 2021, the last trading day prior to the Closing.

In addition, pursuant to the Merger Agreement, the Combangio Equityholders are entitled to receive from the Company up to $105,000 in payments that are contingent upon the achievement of specified development, regulatory and commercialization milestones (the "Contingent Consideration") and are payable in cash and shares of the Company's common stock, subject to the Share Cap (as defined below). If the issuance of the Deferred Purchase Consideration or any contingent consideration payable in shares of the Company's common stock (the "Contingent Stock Consideration") would result in the aggregate number of shares of common stock issued under the Merger Agreement equaling or exceeding 19.9% of the total number of shares of the Company's common stock issued and outstanding immediately prior to the closing (the "Share Cap"), then the Company will be required to pay the portion of such consideration in excess of the Share Cap in cash. The portion of any payment of Contingent Consideration payable in cash is referred to as "Contingent Cash Consideration".

Upon dosing of the first patient in the CHASE Phase 2b clinical trial of KPI-012 for PCED in the United States in February 2023 (the "First Dosing Milestone"), in March 2023, the Company paid the former Combangio Equityholders an aggregate of $2,500 in cash and $2,354 in shares of our common stock (representing an aggregate of 105,038 shares of the Company's common stock). The remaining amount due for the First Dosing Milestone of $146 was paid in cash in January 2024. Upon payment of the First Dosing Milestone, the Company reached the Share Cap and any Contingent Consideration payable under the Merger Agreement in the future will be paid only in cash.

Subject to the terms and conditions of the Merger Agreement, the former Combangio Equityholders, are entitled to receive from the Company the following remaining Contingent Consideration in cash:

- (i) $5,000 payable upon the first patient dosed with any product candidate whose active ingredient comprises one or more biological factors secreted by MSCs or their progenitors, including KPI-012 (the "Product Candidate") in a pivotal clinical trial, (ii) $12,500 payable upon regulatory approval by the FDA of marketing and sale of a Product Candidate in the United States, subject to certain specified reductions; (iii) $17,500 payable upon the first commercial sale of a Product Candidate in the United States, subject to certain specified reductions, and (iv) an aggregate of up to $65,000 payable upon the achievement of specified sales milestones;
- tiered cash royalties at percentage rates in the mid-to-high single digits payable on annual net sales of all Product Candidates; and
- a cash payment at a percentage rate in the high single digits of all income, including earnout payments, received by the Company or any of its affiliates from a product license granted by the Company to a third party to sell or otherwise commercialize the Product Candidate in countries where neither the Company nor its affiliates conduct sales of such Product Candidate, subject to certain exceptions set forth in the Merger Agreement.

If the aggregate amount of Contingent Cash Consideration payable in any calendar year (after giving effect to the Share Cap) exceeds $2,500 (the "Excess Cash Cap"), such excess portion ("Carry Forward Contingent Cash Consideration") will be carried forward and, subject to application of the Excess Cash Cap in the following calendar year, become payable on the first business day of the following calendar year. Any Carry Forward Contingent Cash Consideration outstanding on June 1, 2026 is payable in full on June 1, 2026.

For accounting purposes, the transaction was accounted for as an asset acquisition, as substantially all of the fair value of the gross assets acquired was concentrated in a single asset, KPI-012.

Note 4: Assets Held for Sale

No assets held for sale remained on the consolidated balance sheet as of December 31, 2023. The Remaining Inventory and deferred gain on the sale of the Commercial Business were written off and recognized during the year ended December 31, 2023 and included in other (expense) income, net on the consolidated statements of operations and comprehensive loss. As of December 31, 2022, the Company presented assets to be disposed of that met the criteria as held for sale as a single asset in its consolidated financial statements. The EYSUVIS and INVELTYS product inventory classified as held for sale represented the net realizable value of the Remaining Inventory which Alcon, and solely Alcon, had the right to purchase. The Company deferred a portion of the gain on the sale of the Commercial Business related to the discounted pricing on the Remaining Inventory of $4,189.

(In thousands, except share and per share amounts)

The following is a summary of the major categories of assets that were reclassified to held for sale on the consolidated balance sheet as of December 31, 2022:

	December 31, 2022
Inventories	$ 7,544
Property and equipment, net	51
Current assets held for sale	$ 7,595

See Note 3, "Acquisitions and Divestitures", for further information on the sale of the Commercial Business.

Note 5: Fair Value of Financial Instruments

ASC 820, *Fair Value Measurements and Disclosures*, establishes a fair value hierarchy for those instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and its own assumptions (unobservable inputs). The hierarchy consists of three levels:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's financial instruments as of December 31, 2023 and 2022 consisted primarily of cash equivalents and contingent consideration. Cash equivalents and contingent consideration are reported at their respective fair values on the Company's consolidated balance sheets.

As discussed in Note 3, "Acquisitions and Divestitures", the Company acquired Combangio in November 2021 and in connection with the closing of the Combangio Acquisition, the Company agreed to issue Deferred Purchase Consideration for which the Company established liabilities for these considerations. The Deferred Purchase Consideration related to the Combangio Acquisition was measured at fair value each reporting period using Level 3 unobservable inputs. The fair value of the Deferred Purchase Consideration was based on the fair value of the underlying stock and a discount for lack of marketability. Any change in the fair value of the Deferred Purchase Consideration was included in loss from operations in the consolidated statements of operations and comprehensive loss. During the year ended December 31, 2022, the Company settled $7,935 of the liability upon issuance of the Upfront Shares and during the year ended December 31, 2023, the Company settled the remaining liability of $365 upon the issuance of the Holdback Shares. During the years ended December 31, 2023 and 2022, the change in the fair value of the Deferred Purchase Consideration was a gain of $230 and a loss of $638, respectively, primarily due to the change in the fair value of the underlying stock price and was recognized as the (gain) loss on fair value remeasurement of deferred purchase consideration in the consolidated statements of operations and comprehensive loss for the years ended December 31, 2023 and 2022.

Additionally, the purchase price in connection with the Combangio Acquisition included potential future payments of up to $105,000 that are contingent upon the achievement of specified development, regulatory and commercialization milestones and are required to be recorded at fair value. To date, of the $105,000 in contingent milestone payments, the Company has paid to the Combangio Equityholders an aggregate of $2,500 in cash and $2,354 in shares of the Company's common stock (representing an aggregate of 105,038 shares of the Company's common stock) upon achieving the First Dosing Milestone in February 2023 and paid the remaining amount due in connection

(In thousands, except share and per share amounts)

with the First Dosing Milestone of $146 in cash in January 2024, which was included within accrued expenses and other current liabilities on the consolidated balance sheet as of December 31, 2023. Contingent consideration liabilities related to acquisitions are measured at fair value each reporting period using Level 3 unobservable inputs. The fair values of the contingent consideration liabilities were based on a probability-adjusted discounted cash flow calculation using Level 3 fair value measurements. Changes in these estimates and assumptions could have a significant impact on the fair value of the contingent consideration liabilities. Any changes in the fair value of these contingent consideration liabilities are included in loss from operations in the consolidated statements of operations and comprehensive loss. During the year ended December 31, 2023, the change in the fair value of the contingent consideration liabilities was a loss of $740, primarily due to changes in discount rates, as well as changes in the expected timing and probability of payment, partially offset by the passage of time, and was recognized as a loss on fair value remeasurement of contingent consideration in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2023. During the year ended December 31, 2022, the change in the fair value of the contingent consideration liabilities was a gain of $288, primarily due to changes in discount rates, partially offset by the passage of time, and was recognized as a gain on fair value remeasurement of contingent consideration in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2022.

The following tables set forth the fair value of the Company's financial instruments by level within the fair value hierarchy as of December 31, 2023 and 2022:

	December 31, 2023			
	Fair Value	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 44,639	$ 44,639	$ —	$ —
Total Assets	$ 44,639	$ 44,639	$ —	$ —
Liabilities:				
Contingent consideration	$ 4,110	$ —	$ —	$ 4,110
Total Liabilities	$ 4,110	$ —	$ —	$ 4,110

	December 31, 2022			
	Fair Value	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 31,587	$ 31,587	$ —	$ —
Total Assets	$ 31,587	$ 31,587	$ —	$ —
Liabilities:				
Deferred purchase consideration	$ 595	$ —	$ —	$ 595
Contingent consideration	8,370	—	—	8,370
Total Liabilities	$ 8,965	$ —	$ —	$ 8,965

(In thousands, except share and per share amounts)

The following tables summarize quantitative information and assumptions pertaining to the fair value measurement of the Level 3 inputs as of December 31, 2023 and 2022:

Financial Instrument	Fair Value at December 31, 2023	Valuation Technique	Unobservable Input	Range (Average)
Contingent consideration	$ 4,110	Probability-adjusted discounted cash flow model	Period of expected milestone achievement	2025 - 2028 (2027)
			Probabilities of achievement	16.6% - 35.5% (23.4%)
			Discount rate	16.3%

Financial Instrument	Fair Value at December 31, 2022	Valuation Technique	Unobservable Input	Range (Average)
Deferred purchase consideration	$ 595	Option pricing model	Discount for lack of marketability	20%
Contingent consideration	$ 8,370	Probability-adjusted discounted cash flow model	Period of expected milestone achievement	2023 - 2027 (2025)
			Probabilities of achievement	19.9% - 95.0% (44.9%)
			Discount rate	19.0%
			Discount for lack of marketability	20.0%

The following table summarizes the changes in the Deferred Purchase Consideration and contingent consideration liabilities measured at fair value using Level 3 inputs for the year ended December 31, 2023:

Deferred purchase consideration	
Balance at January 1, 2022	$ 7,892
Fair value adjustments	638
Settlements	(7,935)
Balance at January 1, 2023	$ 595
Fair value adjustments	(230)
Settlements	(365)
Balance at December 31, 2023	$ —
Contingent consideration	
Balance at January 1, 2022	$ 8,658
Fair value adjustments	(288)
Balance at January 1, 2023	$ 8,370
Fair value adjustments	740
Settlements	(4,854)
Reclassification to accrued expenses and other current liabilities	(146)
Balance at December 31, 2023	$ 4,110

During the years ended December 31, 2023 and 2022, there were no transfers between Level 1, Level 2, and Level 3.

The carrying value reported on the accompanying consolidated balance sheets of cash, restricted cash, accounts receivable, accounts payable and accrued expenses approximate their fair value due to their short-term nature. Management believes that the Company's long-term debt (see Note 11) bears interest at the prevailing market rate for instruments with similar characteristics and, accordingly, the carrying value of long-term debt, also approximates its fair value.

(In thousands, except share and per share amounts)

Note 6: Grant Income

CIRM Award

On August 2, 2023, Combangio, a wholly owned subsidiary of the Company, entered into an award agreement with CIRM for a $15,000 grant (the "CIRM Award") to support Combangio's KPI-012 program for the treatment of PCED. The award includes funding for the CHASE Phase 2b clinical trial of KPI-012 for PCED, as well as product and process characterization and analytical development for the program. The CIRM Award is subject to a co-funding requirement under which Combangio is obligated to spend a specified minimum amount on the development of KPI-012 to obtain the full award amount. Upon entry into the CIRM Award, Combangio received an initial $5,900 disbursement from CIRM, and the balance of the award is payable to Combangio upon the achievement of specified milestones that are primarily related to Combangio's progress in conducting the CHASE Phase 2b clinical trial. CIRM may permanently cease disbursements if the milestones are not met within four months of the scheduled completion dates. Additionally, if CIRM determines, in its sole discretion, that Combangio has not complied with the terms and conditions of the CIRM Award, CIRM may suspend or permanently cease disbursements. Under the terms of the CIRM Award, Combangio is obligated to pay a royalty on net sales of any product, service or approved drug resulting in whole or in part from the CIRM Award in the amount of 0.1% per $1,000 of funds utilized by the Company until the earlier of ten years from the date of first commercial sale of such product, service or approved drug and such time as nine times the amount of funds awarded by CIRM has been paid in royalties (the "Base Royalty"). In addition, following the satisfaction of the Base Royalty, Combangio is obligated to pay a 1.0% royalty on net sales of any CIRM-funded invention in excess of $500,000 per year until the last to expire patent covering such invention expires.

During the year ended December 31, 2023, the Company recognized $4,825 of grant income related to the CIRM Award on its consolidated statement of operations. As of December 31, 2023, the Company had deferred grant income of $1,075 on its consolidated balance sheet.

Note 7: Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets, consists of the following:

	December 31, 2023	December 31, 2022
Insurance	$ 675	$ 698
Prepaid research and development	555	—
Non-trade receivables	119	908
Trade receivables, net	117	195
Due from Alcon	—	5,394
Other	509	657
Prepaid expenses and other current assets	$ 1,975	$ 7,852

(In thousands, except share and per share amounts)

Note 8: Property and Equipment, Net

Property and equipment, net, consists of the following:

	December 31, 2023	December 31, 2022
Equipment	$ 894	$ 391
Computer hardware and software	869	1,204
Furniture and office equipment	—	29
Construction in progress	100	—
Property and equipment—at cost	1,863	1,624
Less: Accumulated depreciation	(1,110)	(1,224)
Property and equipment—net	$ 753	$ 400

Depreciation expense for the years ended December 31, 2023 and 2022 was $248 and $421, respectively.

Note 9: Accrued Expenses

Accrued expenses and other current liabilities consist of the following:

	December 31, 2023	December 31, 2022
Compensation and benefits	$ 2,616	$ 3,334
Accrued revenue reserves (1)	1,659	807
Development costs	837	446
Professional services	515	948
Commercial costs	33	271
Contract manufacturing	11	453
Due to third parties in connection with Transition Agreement (2)	—	2,244
Other	347	407
Accrued expenses and other current liabilities	$ 6,018	$ 8,910

(1) There were additional revenue reserves included in accounts payable of $483 as of December 31, 2022. There were no such amounts included in accounts payable as of December 31, 2023.
(2) There were additional amounts due to third parties in connection with the Transition Agreement included in accounts payable of $1,737 as of December 31, 2022. There were no such amounts included in accounts payable as of December 31, 2023.

Note 10: Lease

Operating leases

Menlo Park, California Office Lease

In April 2023, Combangio entered into a lease agreement with Menlo Prepi I, LLC, pursuant to which Combangio leases approximately 6,135 square feet of office, laboratory and research and development space in Menlo Park, California. The Company entered into a guaranty of lease agreement guarantying the obligations of Combangio under the lease agreement. The initial term of the lease is for 62 months which commenced on the lease commencement date of July 1, 2023, unless earlier terminated pursuant to the terms of the lease. The lease provides Combangio with an

(In thousands, except share and per share amounts)

option to extend the lease for an additional five-year term. Combangio was required to make a payment in the amount of $144, as a security deposit pursuant to the lease during the year ended December 31, 2023, which is included in other long-term assets on the consolidated balance sheet as of December 31, 2023. Upon the lease commencement, the Company recorded a right-of-use asset of $2.2 million and corresponding $2.1 million of lease liability.

As of December 31, 2023, the Company recognized $2.0 million of right-of-use asset and a corresponding $2.1 million of lease liability (current and non-current) by calculating the present value of lease payments, discounted at 13.1%, the Company's estimated incremental borrowing rate, over the expected term of the lease. As of December 31, 2023, the remaining lease term on the lease was 4.7 years. Variable lease expense for the lease, includes real estate taxes, common area maintenance, and management fees.

Terminated Vehicle Fleet Lease

During the year ended December 31, 2019, the Company entered into a master fleet lease agreement (the "Vehicle Fleet Lease"), pursuant to which it leased vehicles. The Vehicle Fleet Lease commenced upon the delivery of the initial vehicles in March 2019 and had been subject to modifications as the number of leased vehicles had increased or decreased. During the year ended December 31, 2022, in connection with the closing of the Alcon Transaction, the Company terminated the Vehicle Fleet Lease and, as of December 31, 2022, there was no remaining right-of-use asset or corresponding lease liability. In connection with the Vehicle Fleet Lease, the Company issued a letter of credit for $450 which was released in September 2022. As of December 31, 2022, the Company had a receivable of $775 due from the vendor for the sale of used vehicles following the lease termination, which was included within prepaid expenses and other current assets on the consolidated balance sheet. The remaining receivable from the vendor as of December 31, 2023 is *de minimis.*

The components of lease expense and related cash flows were as follows:

	Year Ended December 31,	
	2023	2022
Lease cost		
Operating lease cost	$ 314	$ 414
Short-term lease cost	161	173
Variable lease cost	98	758
Total lease cost	$ 573	$ 1,345
Operating cash outflows from operating leases	$ 203	$ 1,318

The weighted average remaining lease term and weighted average discount rate of operating leases are as follows:

	December 31, 2023	December 31, 2022
Weighted average remaining lease term	4.7 years	0.5 years
Weighted average discount rate	13.1%	10.4%

(In thousands, except share and per share amounts)

As of December 31, 2023, the Company's future minimum lease payments will become due and payable as follows:

Years Ending December 31,		
2024	$	581
2025		601
2026		622
2027		644
2028		440
Less: interest		(755)
Total	$	2,133

Note 11: Debt

Loan and Security Agreement

On May 4, 2021, the Company entered into the Loan Agreement with Oxford Finance, in its capacity as lender (in such capacity, the "Lender"), and in its capacity as collateral agent (in such capacity, the "Agent"), pursuant to which a term loan of up to an aggregate principal amount of $125,000 was available to the Company, consisting of a tranche A term loan that was disbursed on the closing date in the aggregate principal amount of $80,000 and additional tranches that are no longer available to the Company. The Company utilized substantially all of the proceeds from the tranche A term loan to repay a prior credit facility.

Through June 30, 2023, the term loan bore interest at a floating rate equal to the greater of (i) 30-day LIBOR and (ii) 0.11%, plus 7.89%. Effective July 1, 2023, the term loan bears interest at a floating rate equal to the greater of (i) 8.00% and (ii) the sum of (a) the 1-Month CME Term Secured Overnight Financing Rate, (b) 0.10% and (c) 7.89%. The Loan Agreement, prior to the Second Loan Amendment and Third Loan Amendment (as defined below), provided for interest-only payments until December 1, 2024 if neither the tranche B term loan nor the tranche C term loan are made, and until June 1, 2025 if either the tranche B term loan or the tranche C term loan is made (the "Amortization Date"). The aggregate outstanding principal balance of the term loans were required to be repaid in monthly installments starting on the Amortization Date based on a repayment schedule equal to (i) 18 months if neither the tranche B term loan nor the tranche C term loan is made and (ii) 12 months if either the tranche B term loan or the tranche C term loan is made. All unpaid principal and accrued and unpaid interest with respect to each term loan was due and payable in full on May 1, 2026 (the "Maturity Date").

The Company paid a facility fee of $400 on the closing date of the Loan Agreement and agreed to pay a facility fee of $100 upon closing of the tranche B term loan and a $125 facility fee upon the closing of the tranche C term loan. The Company will be required to make a final payment fee of 7.00% of the original principal amount of any funded term loan payable on the earlier of (i) the prepayment of the term loan in full or (ii) the Maturity Date. At the Company's option, the Company may elect to make partial repayments of the term loan to the Lender, subject to specified conditions, including the payment of applicable fees and accrued and unpaid interest on the principal amount of the term loan being repaid.

In connection with its entry into the Loan Agreement, the Company granted the Agent a security interest in substantially all of the Company's personal property owned or later acquired, including intellectual property and the Commercial Business. The Loan Agreement also contains customary representations and warranties and affirmative and negative covenants, as well as customary events of default. Certain of the customary negative covenants limit the ability of the Company and certain of its subsidiaries, among other things, to incur future debt, grant liens, make investments, make acquisitions, distribute dividends, make certain restricted payments and sell assets, subject in each case to certain exceptions.

The Loan Agreement includes features requiring (i) additional interest rate upon an event of default accrued at an additional 5%, and (ii) the Lender's right to declare all outstanding principal and interest immediately payable upon an event of default. These two features were analyzed and determined to be embedded derivatives to be valued as separate financial instruments. These embedded derivatives were bundled and valued as one compound derivative in accordance with the applicable accounting guidance for derivatives and hedging transactions. The Company determined that, due to the unlikely event of default, the embedded derivatives have a *de minimis* value as of December 31, 2023. The derivative liability will be remeasured at fair value at each reporting date, with changes in fair value being recorded as other income (expense) in the consolidated statements of operations and comprehensive loss.

On May 21, 2022, in connection with its entry into the Asset Purchase Agreement with Alcon, the Company entered into an amendment to the Loan Agreement (the "Second Loan Amendment"). Pursuant to the Second Loan Amendment, the Lender and Agent consented to the entry by the Company into the Asset Purchase Agreement and the sale of the Commercial Business to Alcon and agreed to release its liens on the Commercial Business in consideration for the payment by the Company at the closing of the Alcon Transaction of an aggregate amount of $40,000 (the "Second Amendment Prepayment") to the Lender and Agent, representing a partial prepayment of principal in the amount of $36,697 of the $80,000 principal amount outstanding under the term loan advanced by the Lender under the Loan Agreement, plus a prepayment fee of $734 and a final payment fee of $2,569. In addition, the Company was required to pay all accrued and unpaid interest on the principal amount of the term loan being repaid.

In addition, under the Second Loan Amendment, the Lender and Agent agreed that, following the closing of the Alcon Transaction and the Second Amendment Prepayment, the Amortization Date would be extended from December 1, 2024 to January 1, 2026, at which time the aggregate principal balance of the term loan then outstanding under the Loan Agreement is required to be repaid in five monthly installments. Pursuant to the Second Loan Amendment, the Company may also make partial prepayments of the term loan to the Lender, subject to specified conditions, including the payment of applicable fees and accrued and unpaid interest on the principal amount of the term loan being repaid.

On July 8, 2022, the Second Amendment Prepayment was paid in connection with the closing of Alcon Transaction, and as such, the Amortization Date was extended to January 1, 2026. The transaction resulted in a loss on extinguishment of debt of $2,583 for the year ended December 31, 2022, consisting of the prepayment premium, a pro-rata portion of the unamortized debt discount and issuance costs and the unaccreted exit fee due upon the Second Amendment Prepayment.

On December 27, 2022, the Company entered into an amendment to the Loan Agreement (the "Third Loan Amendment"). Pursuant to the Third Loan Amendment, the Lender and Agent agreed to amend certain provisions of the Loan Agreement to permit the transfer of the listing of the Company's common stock from The Nasdaq Global Select Market to The Nasdaq Capital Market. Pursuant to the Third Loan Amendment, the Company agreed (A) to make partial prepayments of the principal amount of the term loan outstanding under the Loan Agreement as follows (the "Third Amendment Prepayments"): (1) a payment of $5,000 on or before June 30, 2023, representing a partial prepayment of principal in the amount of $4,673, plus a final payment fee of $327 and (2) a payment of $5,000 on or before January 31, 2024, representing a partial prepayment of principal in the amount of $4,673, plus a final payment fee of $327 and (B) that the Amortization Date under the Loan Agreement shall be changed from January 1, 2026 to January 1, 2025.

Pursuant to the Third Loan Amendment, in addition to the Third Amendment Prepayments, if the Company makes an additional prepayment under the Loan Agreement equal to $5,000 (inclusive of the final payment fee) on or prior to December 31, 2024 (the "First Extension Prepayment"), the Amortization Date will be automatically changed to July 1, 2025, and the maturity date of the Loan Agreement will be automatically changed from May 1, 2026 to November 1, 2026. If, in addition to the Third Amendment Prepayments and the First Extension Prepayment, the Company makes an additional prepayment under the Loan Agreement equal to $2,500 (inclusive of the final payment

fee) on or prior to June 30, 2025 (the "Second Extension Prepayment"), the Amortization Date will be automatically changed to January 1, 2026, and the maturity date of the Loan Agreement will be automatically changed to May 1, 2027.

Under the Third Loan Amendment, the Lender and Agent also agreed to waive the prepayment fees for the Third Amendment Prepayments, the First Extension Prepayment, the Second Extension Prepayment and any other prepayments under the Loan Agreement. Pursuant to the Loan Agreement, the Company also will be required to pay all accrued and unpaid interest on the principal amounts of the term loan being repaid at the time of repayment. The Company paid the Third Amendment Prepayments on January 25, 2023, following which the Company became required to repay the Loan Agreement in monthly installments from January 1, 2025 through May 1, 2026. The principal loan balance under the Loan Agreement following the Third Amendment Prepayments was $33,957.

On August 1, 2023, the Company entered into an amendment to the Loan Agreement with Combangio and Oxford Finance (the "Fourth Loan Amendment"). Pursuant to the Fourth Loan Amendment, certain provisions of the Loan Agreement were amended in connection with the change of the Company's name and the cessation of the U.S. Dollar LIBOR rate. On August 2, 2023, the Company entered into an amendment to the Loan Agreement with Combangio and Oxford Finance (the "Fifth Loan Amendment"). Pursuant to the Fifth Loan Amendment, Oxford Finance consented to the Company's entry into the CIRM Award and certain provisions of the Loan Agreement were amended in connection therewith.

In addition, in connection with the Loan Agreement, the Company paid certain fees to the Lender and other third-party service providers. The fees paid to the Lender were recorded as a debt discount while the fees paid to other third-party service providers were recorded as debt issuance cost. These costs are being amortized using the effective interest method over the term of the Loan Agreement. The amortization of debt discount and debt issuance cost is included in interest expense within the consolidated statements of operations and comprehensive loss. As of December 31, 2023, the effective interest rate was 17.22%, which takes into consideration the non-cash accretion of the exit fee and the amortization of the debt discount and issuance costs.

During the year ended December 31, 2023, the Company recognized interest expense of $5,814 for the Loan Agreement. This consisted of amortization of debt discount of $274, accretion of the final payment fee of $979 and the contractual coupon interest expense of $4,561. During the year ended December 31, 2022, the Company recognized interest expense of $7,280 for the Loan Agreement. This consisted of amortization of debt discount of $342, accretion of the final payment fee of $1,083 and the contractual coupon interest expense of $5,855.

(In thousands, except share and per share amounts)

The components of the carrying value of the debt as of December 31, 2023 and 2022 are detailed below:

	December 31, 2023	December 31, 2022
Principal loan balance	$ 33,957	$ 43,303
Unamortized debt discount and issuance cost	(532)	(806)
Cumulative accretion of exit fee	765	440
Total debt	$ 34,190	$ 42,937
Less: current portion of long-term debt	—	(5,000)
Long-term debt, net	$ 34,190	$ 37,937

The annual principal payments due under the Loan Agreement as of December 31, 2023 were as follows:

Years Ending December 31,	
2024	$ —
2025	23,970
2026	9,987
2027	—
Total	$ 33,957

Note 12: Warrants

The Company has issued warrants in connection with debt transactions that were completed in 2018 and prior.

The following table summarizes the common stock warrants outstanding as of December 31, 2023 and 2022, each exercisable into the number of shares of common stock set forth below as of the specified dates:

Issued	Exercise Price Per Share	Expiration Date	Exercisable From	Shares Exercisable at December 31, 2023	Shares Exercisable at December 31, 2022
2014	$ 375.00	November 2024	July 2017	320	320
2016	$ 413.50	October 2026	September 2017	290	290
2018	$ 609.23	October 2025	October 2018	3,693	3,693
				4,303	4,303

Note 13: Common and Preferred Stock

Preferred Stock

The Company was authorized to issue up to 5,000,000 shares of preferred stock as of December 31, 2023 and 2022. As of December 31, 2023, the Company designated 2,928 shares of preferred stock as Series F Preferred Stock, all of which were outstanding as of December 31, 2023. As of December 31, 2022, the Company designated 54,000 shares of preferred stock as Series E Preferred Stock of which 51,246 and 53,144 shares were outstanding as of December 31, 2023 and 2022, respectively.

Series D Convertible Redeemable Preferred Stock

On August 18, 2022, the Board of Directors (the "Board") declared a dividend of one one-thousandth of a share of the Company's Series D Preferred Stock ("Series D Preferred Stock"), for each outstanding share of the Company's common stock held of record as of 5:00 p.m. Eastern Time on August 29, 2022. The Certificate of Designation of Series D Preferred Stock was filed with the Delaware Secretary of State and became effective on August 19, 2022. The dividend was based on the number of outstanding shares of common stock prior to the Reverse Stock Split. The outstanding shares of Series D Preferred Stock were entitled to vote together with the outstanding shares of common stock, as a single class, exclusively with respect to a proposal giving the Board the authority, as it determined appropriate, to implement a reverse stock split within twelve months following the approval of such proposal by the Company's stockholders (the "Reverse Stock Split Proposal"), as well as any proposal to adjourn any meeting of stockholders called for the purpose of voting on the Reverse Stock Split Proposal (the "Adjournment Proposal").

The Company held a special meeting of stockholders on October 19, 2022 (the "Special Meeting") for the purpose of voting on the Reverse Stock Split Proposal and an Adjournment Proposal. All shares of Series D Preferred Stock that were not present in person or by proxy at the Special Meeting were automatically redeemed by the Company immediately prior to the opening of the polls at Special Meeting (the "Initial Redemption"). All shares that were not redeemed pursuant to the Initial Redemption were redeemed automatically upon the approval by the Company's stockholders of the Reverse Stock Split Proposal at the Special Meeting (the "Subsequent Redemption" and, together with the Initial Redemption, the "Redemption"). Each share of Series D Preferred Stock was entitled to receive $0.10 in cash for each 100 whole shares of Series D Preferred Stock immediately prior to the Redemption. As of September 30, 2022, there were 73,208 shares of Series D Preferred Stock issued and outstanding. As of December 31, 2022, both the Initial Redemption and the Subsequent Redemption have occurred. As a result, no shares of Series D Preferred Stock remain outstanding.

On November 28, 2022, the Company filed a Certificate of Elimination of Number of Shares of Preferred Stock Designated as Series D Preferred Stock with the Secretary of State of the State of Delaware which, effective upon filing, eliminated all matters set forth in the Certificate of Designation of Series D Preferred Stock previously filed by the Company and all shares of Preferred Stock previously designated as Series D Preferred Stock resumed their status as undesignated shares of preferred stock of the Company.

Series E and Series F Convertible Non-Redeemable Preferred Stock

Pursuant to the Company's Certificate of Designations, Preferences and Rights of Series E Convertible Non-Redeemable Preferred Stock and the Company's Certificate of Designations, Preferences and Rights of Series F Convertible Non-Redeemable Preferred Stock, each filed with the Secretary of State of the State of Delaware, the Company designated 54,000 and 2,928 shares of its authorized and unissued preferred stock as Series E Preferred Stock and Series F Preferred Stock, respectively, and established the rights, preferences and privileges of the Series E Preferred Stock and Series F Preferred Stock. As discussed more fully in Note 1, "Nature of Business," on December 21, 2023, the Company entered into the 2023 Securities Purchase Agreement pursuant to which it issued 2,928 shares of Series F

Preferred Stock at a per share price of $683.00. In December 2022, the Company entered into the 2022 Securities Purchase Agreement pursuant to which it issued 53,144 shares of Series E Preferred Stock at a per share price of $575.00. Each share of Series E Preferred Stock and Series F Preferred Stock has a par value of $0.001.

Series E Preferred Stock and Series F Preferred Stock

Conversion

Each share of Series E Preferred Stock and Series F Preferred Stock is initially convertible into 100 shares of common stock (subject to adjustment as provided in the applicable Certificate of Designations) at any time at the option of the holder, provided that the holder will be prohibited, subject to certain exceptions, from converting its shares of Series E Preferred Stock and/or Series F Preferred Stock for shares of common stock to the extent that immediately prior to or following such conversion, the holder, together with its affiliates and other attribution parties, would own in excess of 9.99% of the total number of shares of common stock of the Company then issued and outstanding after giving effect to such conversion, which percentage may be changed at the holder's election to a lower percentage at any time or to a higher percentage not to exceed 19.99% upon 61 days' notice to the Company (collectively, the "Beneficial Ownership Limitation").

Voting

Shares of Series E Preferred Stock and shares of Series F Preferred Stock will generally have no voting rights, except to the extent provided by applicable law, and except that (i) the consent of the holders of a majority of the outstanding Series E Preferred Stock will be required to waive any provisions of the Series E Certificate of Designations and (ii) the consent of the holders of a majority of the outstanding Series F Preferred Stock will be required to waive any provisions of the Series F Certificate of Designations.

Dividends

Shares of Series E Preferred Stock and Series F Preferred Stock will be entitled to receive dividends equal to (on an as-if-converted-to-common stock basis), and in the same form and manner as, dividends actually paid on shares of common stock.

Liquidation Rights

Upon any dissolution, liquidation or winding up of the Company, whether voluntary or involuntary ("Dissolution"), subject to any superior rights of holders of senior securities, if any, holders of Series E Preferred Stock and holders of Series F Preferred Stock will be entitled to receive, on a *pari passu* basis, as applicable (A) an amount per share of Series E Preferred Stock equal to the greater of (i) $575.00 (as adjusted for stock splits, combinations, reorganizations and the like) plus any dividends declared but unpaid thereon or (ii) such amount per share as would have been payable had all shares of Series E Preferred Stock been converted into common stock (without regard to any restrictions on conversion, including the Beneficial Ownership Limitation) immediately prior to such Dissolution and (B) an amount per share of Series F Preferred Stock equal to the greater of (i) $683.00 (as adjusted for stock splits, combinations, reorganizations and the like) plus any dividends declared but unpaid thereon or (ii) such amount per share as would have been payable had all shares of Series F Preferred Stock been converted into common stock (without regard to any restrictions on conversion, including the Beneficial Ownership Limitation) immediately prior to such Dissolution, in each case, before any distributions shall be made to holders of common stock or any series of preferred stock ranked junior to the Series E Preferred Stock and the Series F Preferred Stock. If, upon any such Dissolution, the assets of the Company are insufficient to pay the holders of shares of the Series E Preferred Stock and the holders of shares of Series F Preferred Stock the full amount required under the preceding sentence, the holders of shares of Series E Preferred Stock and the holders of shares of Series F Preferred Stock will share in any distribution of the assets

(In thousands, except share and per share amounts)

available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares of Series E Preferred Stock and the Series F Preferred Stock held by them upon such distribution if all amounts payable on or with respect to such shares of were paid in full.

Common Stock

The Company was authorized to issue up to 120,000,000 shares of common stock with a $0.001 par value per share as of December 31, 2023 and 2022. The Company had 2,759,372 and 1,706,971 shares of common stock issued and outstanding as of December 31, 2023 and 2022, respectively.

Holders of the Company's common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Each election of directors by the Company's stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by the Company's Board of Directors, subject to any preferential dividend rights of any preferred stock that the Company may issue in the future.

In the event of the Company's Dissolution, whether voluntary or involuntary, the holders of its common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of the Series F Preferred Stock, the Series E Preferred Stock and any preferred stock that the Company may issue in the future. Holders of the Company's common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of the Company's common stock are subject to and may be adversely affected by the rights of the holders of Series F Preferred Stock, the Series E Preferred Stock and shares of any series of its preferred stock that the Company may designate and issue in the future.

Reserved Shares

As of December 31, 2023 and 2022, the Company has reserved the following shares of common stock for issuance upon exercise of rights under warrants, under the Amended and Restated 2017 Employee Stock Purchase Plan (the "ESPP"), upon the exercise of stock options, upon the vesting of RSUs and PSUs, upon the issuance of the Deferred Purchase Consideration in connection with the Combangio Acquisition (see Note 5), upon conversion of the Series E Preferred Stock and upon conversion of the Series F Preferred Stock:

	December 31, 2023	December 31, 2022
Warrant rights to acquire common stock	4,303	4,303
ESPP	28,927	15,548
Outstanding inducement stock option awards	14,180	11,080
2009 Plan	26	32,642
2017 Plan	1,596,113	248,221
Deferred Purchase Consideration	—	19,350
Series E Preferred Stock (as converted to common shares)	5,124,600	5,314,400
Series F Preferred Stock (as converted to common shares)	292,800	—
Total	7,060,949	5,645,544

Second Closing Right

The Company determined that the common stock and the Series E Preferred Stock issued on December 1, 2022 and the Series E Preferred Stock issued on December 27, 2022 (the "Second Closing Right") pursuant to the 2022 Securities Purchase Agreement, each represented separate freestanding financial instruments and were not within the scope of ASC 480, *Distinguishing Liabilities from Equity*, ("ASC 480"). The instruments did not contain any embedded derivatives required to be bifurcated from the Series E Preferred Stock and the instruments were each equity classified within permanent equity. The Company determined that the relative fair value of the Second Closing Right was *de minimis*.

The Series F Preferred Stock issued on December 22, 2023 pursuant to the 2023 Securities Purchase Agreement was not within the scope of ASC 480, the instrument did not contain any embedded derivatives required to be bifurcated from the Series F Preferred Stock and the instrument was equity classified within permanent equity.

Note 14: Stock-based Compensation

Stock Incentive Plans

On June 22, 2023, the Company's stockholders approved the Company's Amended and Restated 2017 Equity Incentive Plan, which amended and restated the Company's 2017 Equity Incentive Plan, as amended (the "2017 Plan"), to (i) increase the number of shares of common stock authorized for issuance thereunder by 1,250,000 shares; (ii) limit the number of incentive stock options that can be granted under the plan to 7,738,761 shares of common stock; (iii) add an annual limit on non-employee director compensation, including cash and the value of equity awards, of $750,000 for incumbent directors and $1,000,000 in a director's first year of service; and (iv) extend the term of the plan (including the duration of the evergreen) to 10 years from June 22, 2023, the date that stockholders approved the plan. As of December 31, 2023, there were 112,597 shares of common stock available for grant under the Amended and Restated 2017 Equity Incentive Plan. In addition, the Amended and Restated 2017 Equity Plan provides for an annual increase to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2024 and continuing for each fiscal year until, and including, the fiscal year ending December 31, 2033, equal to the lower of (i) 4% of the sum of (I) the number of outstanding shares of common stock on such date and (II) the number of shares of common stock issuable upon conversion of any outstanding shares of convertible preferred stock of the Company on such date (without giving effect to any restrictions or limitations on conversion) and (ii) an amount determined by the Company's board of directors. On January 1, 2024, 327,070 shares of common stock were added and were available for grant under the Amended and Restated 2017 Equity Incentive Plan.

Under the plans, the Board determines the number of shares of common stock to be granted pursuant to the awards, as well as the exercise price and terms of such awards. The exercise price of incentive stock options cannot be less than the fair value of the common stock on the date of grant. Stock options awarded under the plans expire 10 years after the grant date, unless the Board sets a shorter term. Options granted under the plans generally vest over a four-year period. A portion of the unvested stock options will vest upon the sale of all or substantially all of the stock or assets of the Company.

Stock Option Awards

On May 1, 2023, the Company commenced a one-time stock option exchange program (the "Option Exchange Program"), under which the Company's eligible executive officers, other employees and non-employee directors (collectively, "Eligible Holders") were given the opportunity to exchange outstanding options to purchase shares of the Company's common stock held by them for an equal number of RSUs that are subject to vesting conditions. The Option Exchange Program expired on May 30, 2023. A total of 36 Eligible Holders participated in the Option Exchange Program. Pursuant to the terms and conditions of the Option Exchange Program, the Company accepted for exchange

options to purchase a total of 182,251 shares of the Company's common stock. All surrendered options were cancelled effective as of the expiration of the Option Exchange Program, and immediately thereafter, in exchange therefor, the Company granted a total of 182,251 RSUs pursuant to the terms of the Option Exchange Program and the 2017 Plan. A de minimis number of eligible options were not surrendered for exchange and remain outstanding. Based upon the modification guidance under ASC 718, the Company is required to record an incremental compensation expense of $1,210, which will be recorded, along with the unrecognized compensation cost as of the date of the modification, over the remaining service period of the modified awards.

During the year ended December 31, 2023, the Company granted options for the purchase of 666,962 shares of common stock, including options to directors and inducement grant options to purchase 13,920 shares of common stock to new employees made outside of the 2017 Plan in accordance with Nasdaq Listing Rule 5635(c)(4). During the year ended December 31, 2022, the Company granted options for the purchase of 83,221 shares of common stock, including options with performance criteria as described below, options to directors and inducement grant options to purchase 2,800 shares of common stock to new employees made outside of the 2017 Plan in accordance with Nasdaq Listing Rule 5635(c)(4).

A summary of option activity is as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of January 1, 2023	206,221	$ 260.00	6.2	$ 203
Granted	666,962	14.57		
Exercised	—	—		
Forfeited	(203,119)	259.25		
Outstanding as of December 31, 2023	670,064	$ 15.93	9.4	$ —
Vested or expected to vest as of December 31, 2023	670,064	$ 15.93	9.4	$ —
Options exercisable as of December 31, 2023	2,892	$ 329.92	3.3	$ —

The Company records stock-based compensation related to stock options granted at fair value. The Company utilizes the Black-Scholes option-pricing model to estimate the fair value of stock option grants and to determine the related compensation expense. The assumptions used in calculating the fair value of stock-based payment awards represent management's best estimates. The assumptions used in determining fair value of the stock options granted during the years ended December 31, 2023 and 2022 are as follows:

	Year Ended December 31,					
	2023			2022		
Expected volatility	108.4%	–	123.1%	72.9%	–	87.0%
Risk-free interest rate	3.55%	–	4.43%	1.43%	–	4.19%
Expected dividend yield	0%			0%		
Expected term (in years)	5.50	–	6.10	5.50	–	6.10

The Company derived the risk-free interest rate assumption from the U.S. Treasury rates for U.S. Treasury zero-coupon bonds with maturities similar to those of the expected term of the awards being valued. The Company based the expected dividend yield on its expectation of not paying dividends in the foreseeable future. The Company calculated the expected term of options using the simplified method, as the Company lacks relevant historical data due to the Company's limited operating experience. The expected volatility is based upon the historical volatility of the Company as well as the volatility of comparable companies with publicly available share prices. The impact of forfeitures on compensation expense is recorded as they occur.

(In thousands, except share and per share amounts)

The weighted average grant-date fair value of options granted during the years ended December 31, 2023 and 2022, was $12.82 and $40.40, respectively. The fair value is being expensed over the vesting period of the options on a straight-line basis as the services are being provided. As of December 31, 2023, there was $7,346 of unrecognized compensation cost related to the stock options granted, which is expected to be expensed over a weighted-average period of 3.34 years. Stock-based compensation expense was classified in the consolidated statements of operations and comprehensive loss as follows:

	Year Ended December 31,	
	2023	2022
Cost of product revenues	$ —	$ 166
Research and development	2,110	1,292
Selling, general and administrative	5,353	5,550
Total	$ 7,463	$ 7,008

There were no stock-based compensation costs capitalized into inventory in the year ended December 31, 2023. Stock-based compensation costs capitalized into inventory totaled $190 for the year ended December 31, 2022. Capitalized stock-based compensation was recognized as an expense in cost of product revenues when the related product was sold or in selling, general and administrative expense when the related product was designated as a sample.

There were no stock options exercised during the year ended December 31, 2023. Cash proceeds received from the exercise of stock options were *de minimis* during the year ended December 31, 2022. The total intrinsic value of options exercised for the year ended December 31, 2022, was *de minimis*.

In January 2022, the Company granted stock options to purchase up to 14,850 shares of common stock to certain executives tied to certain performance criteria. On March 14, 2023, the Compensation Committee of the Company's Board determined that certain of the performance conditions were achieved at specific levels of achievement, resulting in vesting of options to purchase an aggregate of 3,960 shares of common stock. All outstanding stock options tied to performance criteria were surrendered in the Option Exchange Program and as such, there were none outstanding as of December 31, 2023.

Restricted Stock Units and Performance-Based Restricted Stock Units—In June 2020, the Company issued 13,854 PSUs to certain executives and other employees tied to certain performance criteria, which vested as to 50% of the PSUs in October 2021 on the first anniversary of satisfying the performance criteria and the remaining 50% vested in October 2022 upon the second anniversary of satisfying the performance criteria.

During the years ended December 31, 2023 and 2022, the Company issued 824,190 RSUs (which includes the RSUs issued in connection with the Option Exchange Program) and 6,910 RSUs, respectively, to certain executives and other employees which will vest no sooner than one-third per year over three years on the anniversary of the date of grant.

As of December 31, 2023, a total of 827,658 RSUs were outstanding, consisting of 824,998 unvested shares and 2,660 vested and deferred shares by directors. This results in unrecognized stock-based compensation of $13,343 to be recognized as stock-based compensation expense over the remaining weighted-average vesting period of 2.16 years.

(In thousands, except share and per share amounts)

A summary of activity for RSUs for the year ended December 31, 2023 is as follows:

	Shares	Weighted Average Grant Date Fair Value
Unvested and outstanding balance as of January 1, 2023	4,887	$ 230.29
Changes during the period:		
Granted	824,190	21.73
Vested	(2,202)	248.11
Forfeited	(1,877)	43.75
Unvested and outstanding balance as of December 31, 2023	824,998	$ 22.31
Vested and deferred balance as of December 31, 2023	2,660	

Employee Stock Purchase Plan—In 2017, the Company approved the 2017 Employee Stock Purchase Plan, which was amended and restated in December 2018 (as amended, the "ESPP"). The ESPP reserved an aggregate of 4,466 shares of common stock and provides for an annual increase on the first day of each fiscal year, beginning on January 1, 2019 and ending on December 31, 2029, in an amount equal to the lowest of: (1) 17,868 shares of the Company's common stock; (2) 1% of the total number of shares of the Company's common stock outstanding on the first day of the applicable fiscal year; and (3) an amount determined by the Company's board of directors. On January 1, 2024, 27,593 shares of common stock were added and were available for grant under the ESPP.

The ESPP provides for two six-month offering periods each year: the first offering period begins on the first trading day on or after each January 1 and the second offering period begins on the first trading day on or after each July 1. Under the ESPP, participating employees can authorize the Company to withhold a portion of their base pay during consecutive six-month payment periods for the purchase of shares of the Company's common stock. At the conclusion of the period, participating employees can purchase shares of the Company's common stock at 85% of the lesser of the closing price of the common stock on (i) the first business day of the plan period or (ii) the exercise date. The fair value of the purchase rights granted under the ESPP was estimated on the date of grant, using the Black-Scholes option-pricing model. During the years ended December 31, 2023 and 2022, employees of the Company purchased an aggregate of 3,690 and 13,791 shares, respectively under the ESPP.

(In thousands, except share and per share amounts)

Note 15: Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows for the years ended December 31, 2023 and 2022:

	Year Ended December 31,	
	2023	2022
Numerator:		
Net loss attributable to common stockholders	$ (42,199)	$ (44,822)
Denominator:		
Weighted-average common shares outstanding, basic and diluted(1)	2,432,008	1,520,611
Net loss per share attributable to common stockholders, basic and diluted	$ (17.35)	$ (29.48)

(1) Included in the weighted-average common shares outstanding, basic and diluted for the year ended December 31, 2022 is an aggregate of 19,350 shares of common stock that were held back by the Company as partial security for the satisfaction of indemnification obligations and other payment obligations of the Combangio Equityholders and were issued in March 2023.

The following potential common stock equivalents were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect. The share amounts presented below represent the average of the quarters' incremental shares:

	Year Ended December 31,	
	2023	2022
Options to purchase shares of common stock	549,952	233,231
Unvested RSUs and PSUs	654,907	9,718
Unexercised warrants	4,303	4,303
Convertible preferred stock (as converted to common stock)	5,282,850	1,328,600
	6,492,012	1,575,852

Note 16: Income Taxes

The Company has had no income tax expense due to operating losses incurred for the years ended December 31, 2023 and 2022. The Company has also not recorded any income tax benefits for the net operating losses incurred in each period due to its uncertainty of realizing a benefit from those items. All of the Company's losses before income taxes were generated in the United States.

(In thousands, except share and per share amounts)

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,	
	2023	2022
Federal statutory income tax rate	21.0 %	21.0 %
Effect of:		
State income taxes, net of federal benefit	7.6	5.4
Research and development tax credits	2.4	—
Stock-based compensation	(15.0)	(4.6)
Change in valuation allowance	(15.3)	7.5
Losses and Credits Limited by Section 382 & Section 383	—	(28.8)
Other	(0.7)	(0.5)
Effective income tax rate	— %	— %

Net deferred tax assets as of December 31, 2023 and 2022 consisted of the following:

	December 31,	
	2023	2022
Deferred tax assets:		
Net operating loss carryforwards	$ 108,919	$ 102,565
Capitalized research and development and start-up expenditures	12,153	8,230
Stock-based compensation	1,195	7,243
Research and development tax credit carryforwards	1,154	—
Lease liabilities	672	4
Rebates, incentives, trade discounts and allowances	522	223
Deferred gain on sale of Commercial Business	—	1,145
Other	4,754	2,000
Total deferred tax assets	$ 129,369	$ 121,410
Deferred tax liabilities:		
Right-of-use assets	(638)	(4)
Total deferred tax liabilities	$ (638)	$ (4)
Valuation allowance	$ (128,731)	$ (121,406)
Net deferred tax assets	$ —	$ —

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2023 and 2022. The valuation allowance increased by $7,325 and decreased by $2,727 during the years ended December 31, 2023 and 2022, respectively. The current year increase is primarily the result of an increase in federal net operating losses and the generation of federal and state research and development tax credits. The prior year decrease is primarily the result of a material reduction to the net operating loss carryforward and the research and development tax credits deferred tax assets caused by limitations under Section 382 and Section 383 of the Internal Revenue Code of 1986, thus lowering the valuation allowance required. Management reevaluates the positive and negative evidence at each reporting period.

As of December 31, 2023 and 2022, the Company had federal net operating loss carryforwards of $369,337 and $349,378, respectively, which may be available to offset future federal tax liabilities and expire at various dates beginning in 2030. As of December 31, 2023 and 2022, the Company had state net operating loss carryforwards of $413,711 and $390,607, respectively, which may be available to offset future state income tax liabilities and expire at various dates beginning in 2024. As of December 31, 2023, the Company had $1,154 federal and state research and

development credit carryforwards and as of December 31, 2022, the Company had no federal and state research and development credit carryforwards.

Realization of the future tax benefits is dependent on many factors, including the Company's ability to generate taxable income within the net operating loss carryforward period. Under the provisions of Section 382 of the Internal Revenue Code of 1986, certain substantial changes in the Company's ownership, including a sale of the Company, or significant changes in ownership due to sales of equity, may have limited, or may limit in the future, the amount of net operating loss carryforwards, which could be used annually to offset future taxable income. The Company completed an analysis as of December 31, 2022 and determined that an ownership change occurred during December 2022 as a result of the Company's entry into the 2022 Securities Purchase Agreement which materially limited the net operating loss carryforwards and research and development tax credits. As a result of this most recent ownership change, the utilization of the Company's net operating loss carryforwards is subject to an annual limitation of $222, which is reflected in the numbers presented above. The Company has not completed an analysis as of December 31, 2023 but does not expect any change would further limit the net operating loss carryforwards.

The Company files its corporate income tax returns in the United States and various states. All tax years since the date of incorporation remain open to examination by the major taxing jurisdictions (state and federal) to which the Company is subject, as carryforward attributes generated in years past may still be adjusted upon examination by the Internal Revenue Service (''IRS'') or other authorities if they have or will be used in a future period. The Company is not currently under examination by the IRS or any other jurisdictions for any tax year.

As of December 31, 2023 and 2022 the Company had no uncertain tax positions. The Company's policy is to recognize interest and penalties related to income tax matters as a component of income tax expense, of which no interest or penalties were recorded for the years ended December 31, 2023 and 2022.

Note 17: Commitments and Contingencies

Stanford License Agreement — In October 2019, Combangio entered into a license agreement with The Board of Trustees of The Leland Stanford Junior University ("Stanford"), which was amended in February 2020 and subsequently transferred to the Company by operation of law upon the Combangio Acquisition. Pursuant to the license agreement with Stanford (the "Stanford Agreement"), the Company has a worldwide, exclusive, sublicensable license under certain patent rights ("licensed patents"), directed to methods to promote eye wound healing, to make, have made, use, import, offer to sell and sell products ("licensed products") that are covered by the licensed patents for use in all fields. Under the Stanford Agreement, the Company is required to pay Stanford annual license maintenance fees and milestone payments upon the achievement of specified development, regulatory and sales milestones, as well as tiered royalties on net sales of licensed products that are covered by a valid claim of a licensed patent. During the year ended December 31, 2023, the Company paid Stanford a $175 milestone payment which was triggered by the commencement of the CHASE Phase 2b clinical trial of KPI-012 for PCED in the United States. Additional amounts paid to Stanford in the years ended December 31, 2023 and 2022 were *de minimis*.

(In thousands, except share and per share amounts)

The Company's minimum obligations due under the Stanford License Agreement as of December 31, 2023, are as follows:

Years Ending December 31,		
2024	$	30
2025		65
2026		65
2027		65
2028		65
Thereafter		325
Total minimum license payments	$	615

Contingencies related to the Merger Agreement— In connection with the Combangio Acquisition, the Company agreed to make additional payments based on the achievement of certain milestone events related to KPI-012. The Company recognized certain contingent consideration liabilities at fair value on the acquisition date, and revalues the remaining obligations each reporting period. The total potential maximum payout for the milestone payments, which have been recorded as liabilities at fair value, is $40,000 and the milestone payments are contingent upon the achievement of specified development, regulatory and commercialization milestones. Following the achievement of the First Dosing Milestone in February 2023, the Company paid an aggregate of $2,500 in cash and $2,354 in shares of the Company's common stock (representing an aggregate of 105,038 shares of the Company's common stock) to the former Combangio Equityholders in March 2023. The Company paid the remaining amount due in connection with the First Dosing Milestone of $146 in cash in January 2024. Additionally, pursuant to the Merger Agreement, the Company could trigger potential future sales-based milestone payments of up to $65,000. Because the achievement of these sales-based milestones related to KPI-012 was not considered probable as of December 31, 2023 or December 31, 2022, such contingencies have not been recorded in the Company's consolidated financial statements. Amounts related to contingent milestone payments are not considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory or commercial milestones.

Litigation—The Company is not currently subject to any material legal proceedings.

Guarantees and Indemnifications—The Company's Certificate of Incorporation authorizes the Company to indemnify and advance expenses to its officers and directors and agents to the fullest extent permitted by law.

The Company's equity agreements and certain other arrangements include standard indemnifications against claims, actions, or other matters that may arise in connection with these arrangements.

As of December 31, 2023 and 2022, the Company had not experienced any losses related to these indemnification obligations, and no claims with respect thereto were outstanding. The Company does not expect significant claims related to these indemnification obligations and has no amount accrued related to these contingencies. The Company does not expect these indemnifications to have a material adverse effect on these consolidated financial statements.

Note 18: Defined Contribution Plan

The Company has a 401(k) defined contribution plan (the "401(k) Plan") for substantially all of its employees. Eligible employees may make pretax contributions to the 401(k) Plan up to statutory limits.

The Company made discretionary matching contributions of $142 and $396 to the 401(k) Plan during the years ended December 31, 2023 and 2022, respectively.

Directors and Executive Officers of KALA BIO, Inc. (as of April 29, 2024)

Board of Directors

Mark Iwicki
Chief Executive Officer and Chair of Board of Directors of KALA BIO, Inc.

Mark S. Blumenkranz, M.D.
HJ Smead Professor Emeritus in the Department of Ophthalmology at Stanford University; Managing Partner of Lagunita Biosciences LLC and Garland Investments, Inc.

Marjan Farid
Professor of Clinical Ophthalmology, Director of Cornea, Refractive & Cataract Surgery, and Vice Chair of Ophthalmic Faculty at the Gavin Herbert Eye Institute, University of California Irvine

Andrew I. Koven
Board of Directors, NeuroBo Pharmaceuticals, Inc.

C. Daniel Myers
Chairman Emeritus, Alimera Sciences, Inc.

Gregory Perry
Chief Financial Officer, Merus N.V.

Howard B. Rosen
Board of Directors, Firecycte Therapeutics, Inc., Hammerton, Inc., Hopewell Therapeutics, Inc. and Entrega, Inc.

Executive Officers

Mark Iwicki
Chief Executive Officer and Chair of Board of Directors of KALA BIO, Inc.

Todd Bazemore
President and Chief Operating Officer

Kim Brazzell, Ph.D.
Head of Research and Development and Chief Medical Officer

Darius Kharabi
Chief Business Officer

Mary Reumuth, C.P.A.
Chief Financial Officer, Treasurer and Secretary

KALA BIO, Inc.

1167 Massachusetts Avenue

Arlington, MA 02476

https://www.kalarx.com/