

Engineering medicines  
to improve patient care.

**2022**  
Annual Report





## ABOUT VIRIDIAN THERAPEUTICS

Viridian Therapeutics is a biopharmaceutical company focused on engineering and developing potential best-in-class medicines for patients with serious and rare diseases. Viridian's expertise in antibody discovery and engineering enables it to develop differentiated therapeutic candidates for previously validated drug targets in commercially established disease areas.

Viridian is advancing multiple candidates in the clinic for the treatment of patients with thyroid eye disease (TED). The Company recently initiated its first global Phase 3 trial called 'THRIVE' to evaluate the safety and efficacy of VRDN-001 in patients with active TED. Viridian is also evaluating VRDN-001 in a Phase 2 proof-of-concept trial in patients with chronic TED. In addition to its program for intravenously administered VRDN-001, the Company is advancing three candidates for its subcutaneous strategy with the goal of providing a more conveniently administered therapy to patients with TED. Viridian is also developing multiple preclinical assets in autoimmune and rare diseases.

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2022

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 001-36483



**VIRIDIAN THERAPEUTICS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**

**47-1187261**

(State or other jurisdiction of incorporation or organization)

(I.R.S. Employer Identification No.)

**221 Crescent Street, Suite 401, Waltham, MA 02453**

(Address of principal executive offices)

Registrant's telephone number, including area code: **(617) 272-4600**

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.01 par value per share	VRDN	The Nasdaq Stock Market LLC

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.

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

The aggregate market value of the voting and non-voting common stock held by non-affiliates of the registrant, based upon the closing sale price of the registrant's common stock on June 30, 2022, as reported on The Nasdaq Capital Market, was \$329.3 million. Shares of common stock held by each executive officer and director and by each person who owns 10% or more of the outstanding common stock have been excluded in that such persons may be deemed to be affiliates. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 3, 2023, there were 42,897,951 shares of the registrant's common stock outstanding.

#### DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement for the 2023 annual meeting of shareholders (the "2023 Proxy Statement") are incorporated herein by reference in Part III of this Annual Report on Form 10-K where indicated. The 2023 Proxy Statement will be filed with the U.S. Securities and Exchange Commission within 120 days of the registrant's fiscal year ended December 31, 2022.

**VIRIDIAN THERAPEUTICS, INC.**  
**INDEX**

		Page No.
<b>PART I</b>		
ITEM 1.	BUSINESS	5
ITEM 1A.	RISK FACTORS	28
ITEM 1B.	UNRESOLVED STAFF COMMENTS	58
ITEM 2.	PROPERTIES	58
ITEM 3.	LEGAL PROCEEDINGS	59
ITEM 4.	MINE SAFETY DISCLOSURES	59
<b>PART II</b>		
ITEM 5.	MARKET FOR REGISTRANT’S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES	60
ITEM 6.	RESERVED	60
ITEM 7.	MANAGEMENT’S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS	60
ITEM 7A.	QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK	67
ITEM 8.	FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA	67
ITEM 9.	CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE	67
ITEM 9A.	CONTROLS AND PROCEDURES	67
ITEM 9B.	OTHER INFORMATION	68
ITEM 9C.	DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTION	68
<b>PART III</b>		
ITEM 10.	DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE	68
ITEM 11.	EXECUTIVE COMPENSATION	69
ITEM 12.	SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS	69
ITEM 13.	CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE	69
ITEM 14.	PRINCIPAL ACCOUNTING FEES AND SERVICES	69
<b>PART IV</b>		
ITEM 15.	EXHIBITS, FINANCIAL STATEMENT SCHEDULES	70
ITEM 16.	FORM 10-K SUMMARY	72
SIGNATURES		

## CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K (“Annual Report”) contains forward-looking statements that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “plan,” “expect,” “preliminary,” “predict,” “potential,” “opportunity,” “goals,” or “should,” and similar expressions are intended to identify forward-looking statements. All statements contained in this Annual Report, other than statements of historical fact are forward-looking statements. You should not unduly rely on forward-looking statements because they involve known and unknown risks, uncertainties and other factors, some of which are beyond our control. These risks, uncertainties and other factors may cause our actual results, performance or achievements to be materially different from the anticipated future results, performance or achievements expressed or implied by the forward-looking statements.

Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation, statements relating to:

- our future research and development activities, including clinical testing and the costs and timing thereof;
- our strategy, including clinical development of VRDN-001, VRDN-002, VRDN-003 and other product candidates, and the clinical and commercial potential of our product candidates, if approved;
- the sufficiency of our cash resources;
- our ability to raise additional funding when needed;
- any statements concerning anticipated regulatory activities or licensing or collaborative arrangements;
- business interruptions resulting from pandemics or other public health crises, which could cause a disruption in the development of our product candidates and adversely impact our business;
- our research and development and other expenses;
- our operations and legal risks;
- developments relating to our competitors and our industry, including competing product candidates and therapies; and
- any statement of assumptions underlying any of the foregoing.

We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors. These forward-looking statements are subject to a number of risks, uncertainties, and assumptions, as described in greater detail in Part I, Item 1A, “Risk Factors” in this Annual Report, and under a similar heading in any other periodic or current report we may file with the Securities and Exchange Commission (“SEC”) in the future. You are advised to consult any further disclosures we make on related subjects in our Annual Reports, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and our website. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties, and assumptions, the future events and trends discussed in this Annual Report, may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

We undertake no obligation to revise or publicly release the results of any revision to these forward-looking statements, except as required by law. Given these risks and uncertainties, readers are cautioned not to place undue reliance on such forward-looking statements. All forward-looking statements are qualified in their entirety by this cautionary statement.

Unless otherwise mentioned or unless the context requires otherwise, all references in this Annual Report, to “Viridian,” “Viridian Therapeutics,” the “Company,” “we,” “us,” and “our” or similar references refer to Viridian Therapeutics, Inc., and our consolidated subsidiaries.



## PART I

### ITEM 1. BUSINESS

#### Company Overview

We are a biopharmaceutical company focused on discovering and developing potential best-in-class medicines for serious and rare diseases. We target under-competitive disease areas where marketed therapies often leave room for improvements in efficacy, safety, and/or dosing convenience. We believe that first-generation medicines rarely represent optimal solutions, especially in rare disease areas, and that there is potential to develop differentiated, best-in-class medicines that could lead to improved patient outcomes, reduced side effects, improved quality of life, expanded market access, and augmented market competition. Our business model is designed to identify and evaluate product opportunities in disease areas where trial data establishes proof-of-concept for a drug target in the clinic, but the competitive evolution of the product life cycle management and number of entrants appears incomplete. We intend to prioritize indications where a fast-follower and a potentially differentiated drug candidate, or overall product profile, could create significant medical benefit for patients. We are engineering medicines to address unmet medical needs for patients and further advance drug innovation.

Our goal is to identify and evaluate product concepts leveraging clinically validated molecular targets using established therapeutic modalities. We prioritize product concepts that are aligned with clinical and commercial hypotheses, which we expect will provide an attractive balance of risk and opportunity, thereby representing a compelling allocation of our resources. We focus on advancing therapeutic antibodies that we either in-license or discover internally, incorporating proprietary monoclonal antibody discovery and optimization platforms to advance clinical candidates with unique characteristics. We have built relevant expertise in monoclonal antibody discovery and engineering, biologics manufacturing, and nonclinical and clinical development for thyroid eye disease (“TED”) and other undisclosed target indications in rare and autoimmune diseases.

Our approach to rapidly discovering and developing novel therapeutics relies on our scientific expertise in evaluating pre-existing clinical proof-of-concept data for the drug targets we are pursuing, and opportunities to improve upon existing investigational and/or approved therapies. This approach informs how we design, select, and develop our product candidates, including in critical areas such as pharmacokinetics, pharmacodynamics, clinical trial design, trial endpoints, and the selection and recruitment of patients. We believe this strategy reduces the risks associated with discovering and developing novel therapeutics.

We have initially prioritized the development of therapies for the treatment of TED, a serious and debilitating rare autoimmune disease that causes inflammation within the orbit of the eye that can cause bulging of the eyes, redness and swelling, double vision, pain, and potential blindness. TED significantly impacts quality of life, imposing a high burden on activities of daily living and mental health for patients suffering from the disease. TED is a progressive disease consisting of an initial active phase, followed by a transition to a secondary chronic phase. The only medicine approved by the U.S. Food and Drug Administration (“FDA”) for TED is Tepezza® (teprotumumab), which is an intravenously administered monoclonal antibody that targets insulin-like growth factor 1 receptor (“IGF-1R”). Tepezza® is marketed in the United States by Horizon Therapeutics plc.

The results from clinical trials of teprotumumab conducted by Horizon provide strong clinical validation linking the targeting of IGF-1R to clinical benefit in patients with TED. However, clinical trials evaluating teprotumumab in patients with TED reported to date used a single dosing regimen, providing little guidance as to the optimal dosing required for clinical activity in TED. We believe that there are multiple opportunities to develop fast-follower therapeutics that improve on teprotumumab’s features, including dosing schedule and route of administration.

We are developing three product candidates, VRDN-001, VRDN-002, and VRDN-003, that are being developed for intravenous (“IV”) or subcutaneous administration to treat patients who suffer from TED.

Our most advanced program, VRDN-001, is a differentiated humanized monoclonal antibody targeting IGF-1R intravenously administered for the treatment for TED. In October 2022, we presented preclinical data from *in-vitro* studies demonstrating that VRDN-001 is a potentially differentiated full antagonist of IGF-1R, compared to teprotumumab’s incomplete antagonism of IGF-1R. In addition, the Phase 1/2 clinical data reported from all three dose cohorts of VRDN-001 in patients with active TED (n=21), showed significant and rapid improvement in both signs and symptoms of TED after two infusions of VRDN-001. Across all VRDN-001 treated patients in the trial, 71% were proptosis responders, 67% were overall responders, 62% achieved a clinical activity score (“CAS”) of 0 or 1, and 54% had complete resolution of their diplopia. VRDN-001 had a favorable safety profile and was well-tolerated by all patients treated in the three dose cohorts. We believe the data further validate the differentiated and potentially best-in-class clinical activity of VRDN-001.

In December 2022, we announced that we enrolled our first patient in our ongoing THRIVE Phase 3 trial (“THRIVE”) evaluating the safety and efficacy of VRDN-001 in patients with active TED. THRIVE is a double-blind, placebo-controlled, randomized study enrolling approximately 120 patients with active TED. The study participants will be randomized 1:1:1 across three arms: (1) VRDN-001 10 mg/kg administered once every three weeks for eight cycles, (2) a shortened course of VRDN-001 10 mg/kg administered once every three weeks for five cycles, and (3) a placebo arm. THRIVE will be conducted in approximately 50 centers across North America and Europe. We expect to report topline results for the THRIVE trial in the middle of 2024.

Data from the proof-of-concept Phase 1/2 trial evaluating the safety and efficacy of VRDN-001 in patients with chronic TED is expected in the second quarter of 2023. Following the release of the data, we are planning to initiate a global Phase 3 trial in patients with chronic TED called THRIVE-2. We expect that the THRIVE and THRIVE-2 Phase 3 trials will support global health authority registration for marketing approval in both active and chronic TED, respectively.

The low-dose data of VRDN-001 in patients with active TED support the potential for VRDN-001 to also be administered as a convenient, low volume, subcutaneous pen. We plan to initiate a Phase 1 trial of subcutaneously administered VRDN-001 in healthy volunteers, with results expected in the fourth quarter of 2023.

VRDN-002, a distinct IGF-1R antibody that incorporates half-life extension technology, is designed to support administration as a convenient, low volume, subcutaneous pen injection to treat patients who suffer from TED. Data reported from the Phase 1 trial in healthy volunteers, showed that VRDN-002 achieved an extended half-life of up to 43 days. VRDN-002 had a favorable safety profile and was well-tolerated. We are currently planning a Phase 2 trial to evaluate the safety and efficacy of a low volume subcutaneous injection of VRDN-002 in patients with active TED.

VRDN-003 is an anti-IGF-1R monoclonal antibody with the same amino acid sequence as VRDN-001, except for the addition of the same half-life extension technology that has been incorporated into VRDN-002. We plan to submit an investigational new drug (“IND”) application for VRDN-003 with the FDA in the second quarter of 2023. Following FDA acceptance of the IND, we plan to initiate a Phase 1 trial of VRDN-003 in healthy volunteers, with results expected in the fourth quarter of 2023.

Our most recent pharmacokinetic (“PK”) modeling supports the feasibility of ongoing development of a self-administered pen for subcutaneous administration, and a planned dosing interval of up to once-monthly for VRDN-001, VRDN-002 and VRDN-003. Following clinical results expected by year-end 2023, we expect to select VRDN-001, VRDN-002 or VRDN-003 as our lead subcutaneous program to advance to a pivotal Phase 3 trial in the middle of 2024.

In addition to developing therapies for TED, we are executing a similar strategic approach to identify opportunities to develop more convenient, better performing therapeutic candidates in other rare and/or serious disease indications. We are currently advancing three preclinical programs, VRDN-004, VRDN-005, and VRDN-006. We plan to disclose additional details around at least one of these preclinical programs in 2023.

## **Our Strategy**

Our mission is to create and advance new biologic medicines for patients suffering from serious and rare diseases that are underserved by today’s therapies. Key elements of our business strategy are to:

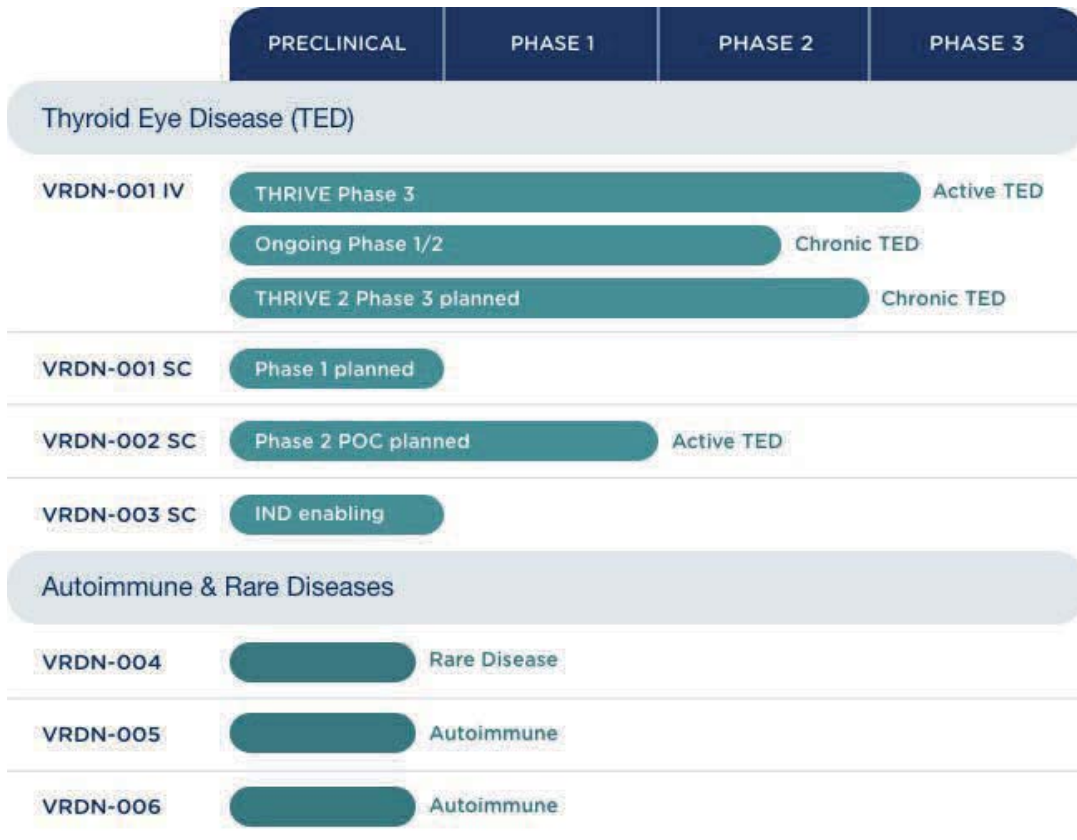
- **Evaluate opportunities to identify, engineer, and develop potential best-in-class monoclonal antibodies that optimize patient care.** Our pipeline of therapeutic programs represents a patient-centric model of innovation that leverages proven biology and antibody technology to reduce research and development risk, while striving to address strategic gaps related to access, delivery, quality of life, efficacy, and/or safety and tolerability in targeted therapeutic areas. Our multidisciplinary search process evaluates scientific and clinical validation of therapeutic targets, market potential, and feasibility of efficiently developing a competitive product.
- **Initial clinical development and commercial focus on thyroid eye disease (TED):**
  - **Rapidly advance VRDN-001 clinical development to enter the TED market quickly.** Our ongoing THRIVE Phase 3 clinical trial is a double-blind, placebo-controlled, randomized study evaluating the safety and efficacy of VRDN-001 in patients with active TED. We expect data from the ongoing Phase 1/2 proof-of-concept trial evaluating VRDN-001 in patients with chronic TED in the second quarter of 2023. Following the release of the data, we are planning to initiate a global Phase 3 trial in patients with chronic TED called



THRIVE-2. We expect that the THRIVE and THRIVE-2 Phase 3 trials will support global health authority registration for marketing approval in active and chronic TED, respectively.

- **Offer patients suffering from TED improved convenience and broader settings of care.** Our next generation IGF-1R antibodies, VRDN-002 and VRDN-003, are humanized monoclonal antibodies that incorporate half-life extension technology and are designed as a self-administered low-volume, subcutaneous pen injection for the treatment of TED. Low-dose data of VRDN-001 administered intravenously support its potential as a subcutaneous candidate. Our most recent PK modeling for VRDN-001, VRDN-002, and VRDN-003 supports a planned dosing interval of up to once-monthly. Following data expected by the end of 2023 from the ongoing clinical trials of VRDN-001, VRDN-002 and VRDN-003, we expect to select one of the programs as our lead subcutaneous product candidate by year-end 2023. We plan to advance our selected lead subcutaneous program into a pivotal Phase 3 trial in the middle of 2024.
- **Be a trusted partner in the care of TED patients.** We plan to cultivate a network across TED stakeholders to inform our patient-centric approach, including with patients and advocacy groups, key opinion leaders, research institutions, healthcare professionals and payers.
- **Prepare for commercialization of the intravenously administered VRDN-001 and one of our subcutaneously administered candidates, VRDN-001, VRDN-002, or VRDN-003, for the treatment of patients with TED.** We hold worldwide commercialization rights, excluding the greater area of China, to VRDN-001, VRDN-002, and VRDN-003. As a result, we have the flexibility to develop and potentially commercialize products ourselves, or alternatively to enter collaborations with industry partners.
- **Expand our portfolio beyond TED with strategically aligned opportunities in rare and autoimmune diseases.** We believe there are significant opportunities to provide more convenient, better performing products compared to therapeutics approved or in development for several diseases. To that end, we continue to invest in portfolio expansion and have dedicated resources to seek additional opportunities to develop best-in-class therapeutics for newly validated targets. VRDN-004, VRDN-005, and VRDN-006 are preclinical-stage therapeutic antibody programs for undisclosed rare and autoimmune diseases.
- **Leverage our therapeutic antibody and multi-disciplinary search expertise to continue discovering and developing novel, best-in class product candidates.** We plan to continue to identify and advance novel product candidates and technologies to generate potential best-in-class antibody therapeutics, either internally or through in-licensing. We continue to identify and evaluate several disease targets and therapeutic candidates with the aim of advancing a steady pipeline of best-in-class product candidates from discovery and preclinical research into clinical trials.

## Our Pipeline



**Thyroid Eye Disease (TED)**

TED, commonly associated with Graves’ Disease, and in some cases referred to as Graves’ orbitopathy, is a serious and rare autoimmune disorder affecting the eye and its adjacent tissue. It is characterized by inflammation within the orbit of the eye that can cause bulging of the eyes, redness and swelling, double vision, pain, and potential blindness. TED is a progressive disease consisting of an initial active phase, followed by a transition to a secondary chronic phase. In the active phase of TED, patients present with several inflammatory signs and symptoms such as pain, redness, swelling, proptosis, diplopia, and eyelid retraction. The active phase is generally defined as the first 18 to 24 months of disease onset, and is typically when inflammatory signs and symptoms reach their peak levels. The second, chronic phase of TED is characterized by a reduction in some of the inflammatory signs, such as redness and swelling in the area surrounding the eye, compared to the active phase of the disease. However, proptosis, pain, diplopia, and eyelid retraction often persist throughout life for patients with chronic TED. Patients with active and chronic TED experience significant impairment to their quality of life, including difficulties activities of daily living, trouble functioning in social situations, and decreased psychological well-being. About one in every three patients experience anxiety and depression.

**Pathologies Leading to the Development of TED**

TED develops in parallel with Graves’ Disease, an autoimmune disease in which antibodies form against the thyroid-stimulating hormone receptor (“TSHR”), which is present in the thyroid and other cells such as adipocytes and fibroblasts. A close temporal relationship exists between the onset of Graves’ Disease and the onset of TED. Regardless of which condition occurs first, the other condition develops within 18 months in 80% of patients. In addition to antibodies against TSHR, patients with TED also develop antibodies against IGF-1R.

Insulin-like growth factor 1 (“IGF-1”) is a hormone similar in molecular structure to insulin with higher growth-promoting activity. IGF-1R, the receptor for IGF-1, is highly expressed in fibrocytes, cells that are derived from the bone marrow and that have the potential to differentiate into either myofibroblasts or fat cells. IGF-1R and TSHR function in concert to regulate the proliferation and differentiation of fibrocytes in the orbital socket.

One potential cause of TED is autoimmune antibodies against IGF-1R that lead to the activation of IGF-1R, resulting in increased proliferation, secretion of extracellular complex carbohydrates, and differentiation into fat cells. These antibodies, and

autoimmune antibodies to TSHR, can elicit an immune attack against the fibrocytes that surround the eye triggering the development of TED. Inflammation associated with this attack combined with activation of IGF-1R leads to the wide spectrum of pathologies seen with this disease.

Exposure to other inflammatory agents, such as cigarette smoke, leads to exacerbation of the disease resulting in more severe symptoms.

### ***Current Treatments for TED***

Prior to 2020, moderate to severe cases of TED were treated off-label with steroids as daily doses of oral prednisone, or in more severe cases, weekly doses of IV methylprednisolone. Treatment with steroids is associated with a wide range of serious complications including high blood pressure, diabetes, psychological effects, personality change, insomnia, skin thinning, immunosuppression, hyperglycemia, and increased risks of infections. Systemic steroids showed limited efficacy for most of the signs and symptoms of TED and are not a sustainable long-range intervention given the side effects. If steroid treatment proved to be inadequate, or could not be tolerated, the only remaining options for patients were orbital radiation or surgery to reduce swelling, decompress orbital contents, and protect the vision. Again, each of these therapies was incomplete and inadequate from the perspective of both patient and treating physician.

In January 2020, teprotumumab, an antibody that blocks the activation of IGF-1R, was approved by the FDA for the treatment of TED. In two randomized, double-blind placebo-controlled trials, infusions of teprotumumab every three weeks, for a total of eight doses, led to a greater than 2 mm decrease in proptosis in 71% and 83% of patients, respectively, compared to 20% and 10% with placebo. Treatment with teprotumumab also led to a 53% decrease in diplopia compared to a 25% decrease when patients were treated with placebo control. Thus, the defined target and its successful blockade has been de-risked and shown to provide a clinically meaningful improvement in the quality of life for these patients, allowing them to return to the workforce and to avoid radiation therapy or orbital decompressive surgeries.

### ***Market Potential***

TED has an annual incidence of approximately 19 in 100,000 people, which corresponds to over 60,000 patients in the United States. Of these, it is estimated that more than 20,000 patients in the United States have moderate to severe active disease that requires IV treatment, either with teprotumumab or steroids. In the secondary chronic phase of the disease, it is estimated that more than 75,000 patients in the United States are living with moderate to severe disease. At launch, Horizon Therapeutics plc announced a price of approximately \$16,300 per vial of Tepezza® which translates to a list price of approximately \$375,000 based on patient weight for a six-month course of therapy. In the first three years following launch, Horizon Therapeutics plc reported full-year 2020, 2021, and 2022 net sales for Tepezza® of \$820.0 million, \$1.66 billion, and \$2.0 billion, respectively, in the first three years of launch. This demonstrates that TED is a multi-billion-dollar market in the United States alone, with the potential for additional revenue in the United States with indication expansion to chronic TED and more convenient routes of administration. There is also potential for additional revenue following regulatory approvals and commercial expansion outside of the United States, which will accommodate multiple entrants.

### ***Our Product Candidates***

#### **VRDN-001, a potential best-in-class intravenously administered IGF-1R antibody**

VRDN-001 is a monoclonal antibody that binds to and is believed to act as a full antagonist of IGF-1R signaling pathway. This mechanism of action is clinically and commercially validated by the only FDA product approved for the treatment of TED, Tepezza®. Based on our ongoing THRIVE trial, our goal is for VRDN-001 to be second to market in this class of medicine, with the opportunity to offer a differentiated IV product.

We have an exclusive license to the worldwide rights to develop and commercialize VRDN-001 for all non-oncology indications that do not use radiopharmaceuticals, including the treatment of patients with TED, from ImmunoGen. The antibody sequence that we are developing as VRDN-001 in TED had previously been developed in oncology as AVE-1642 and studied in over 100 patients. However, development in oncology was stopped in 2009 due to its failure to meet the primary efficacy endpoints in multiple myeloma. As described below, we have sublicensed the right to develop, manufacture, and commercialize certain IGF-1R directed antibody products for non-oncology indications in the greater area of China to Zenas BioPharma (Cayman) Limited.

#### **Clinical Trials for VRDN-001**

### *Phase 1/2 Trial of VRDN-001 in Patients with Active TED*

In the second half of 2022 and early 2023, we announced data from our Phase 1/2 clinical trial evaluating the safety and efficacy of VRDN-001 in patients with active TED. The proof-of-concept portion of this double-blind, placebo-controlled Phase 1/2 trial evaluated two infusions of VRDN-001 administered intravenously, three weeks apart, with efficacy measured six weeks after the first dose. VRDN-001 was evaluated at doses of 3, 10, and 20 mg/kg, with each cohort designed to include six patients randomized to drug, and two patients randomized to placebo. We previously announced positive results from the first two dose cohorts, which demonstrated a favorable safety profile. In January 2023, we announced results from the third cohort, which evaluated a VRDN-001 dose of 3 mg/kg with 6-week data. In the 3 mg/kg dose cohort, nine patients were randomized to receive VRDN-001 to enable all consented patients who were eligible following screening to participate in the trial, and two patients were randomized to receive placebo.

The following activity was observed across all three dose groups (n=21) at week six:

#### Proptosis

- 71% proptosis responder rate, defined as a  $\geq 2$ -millimeter (mm) reduction in proptosis from baseline as measured by exophthalmometry
- 2.3 mm mean reduction in proptosis from baseline as measured by exophthalmometry
- 2.76 mm mean reduction in proptosis from baseline as measured by blinded, centrally reviewed magnetic resonance imaging (“MRI”, preliminary data available for 16 of the 21 patients)

#### Clinical Activity Score (“CAS”)

- 4.1 point mean reduction in CAS from baseline on a 7-point measure of signs and symptoms of TED
- 62% maximal or near-maximal therapeutic effect on CAS, defined as reaching a CAS of 0 or 1 on the 7-point composite measure of signs and symptoms of TED

#### Overall response

- 67% overall responder rate, defined as a  $\geq 2$  mm reduction in proptosis and a  $\geq 2$  point reduction in CAS

#### Diplopia

- 54% complete resolution of diplopia, defined as patients with baseline diplopia who achieved a score of 0 on the Gorman subjective diplopia scale (13 patients with diplopia at baseline)

VRDN-001 had a favorable safety profile and was well-tolerated by all patients treated in the three dose cohorts. There were no reported serious adverse events (“SAEs”), no discontinuations, and no infusion reactions in patients treated with VRDN-001 as of December 19, 2022, the most recent cut-off date for follow-up observation.

### *Phase 3 Trial (THRIVE) of VRDN-001 in Patients with Active TED*

In December 2022, we enrolled our first patient in our ongoing Phase 3 THRIVE trial evaluating the safety and efficacy of VRDN-001 in patients with active TED. The THRIVE trial is a double-blind, placebo-controlled, randomized trial enrolling approximately 120 patients with active TED. The trial participants will be randomized 1:1:1 across three arms: (1) VRDN-001 10 mg/kg administered once every three weeks for eight cycles, (2) a shortened course of VRDN-001 10 mg/kg administered once every three weeks for five cycles, and (3) a placebo arm. The global THRIVE trial is expected to be conducted in approximately 50 centers across North America and Europe. We expect to report topline results for the THRIVE trial in the middle of 2024.

### *Phase 1/2 Trial of VRDN-001 in Patients with Chronic TED*

Data from the Phase 1/2 proof-of-concept trial evaluating the safety and efficacy of VRDN-001 in patients with chronic TED is expected in the second quarter of 2023.

### *Phase 3 Trial (THRIVE-2) of VRDN-001 in Patients with Chronic TED*

Following the release of the data from our proof-of-concept trial in chronic TED, we plan to initiate a global Phase 3 trial in patients with chronic TED called THRIVE-2. We expect that the THRIVE and THRIVE-2 Phase 3 trials will support global health authority registration for marketing approval in active and chronic TED, respectively.

### **VRDN-001, VRDN-002 and VRDN-003, potential best-in-class subcutaneously administered IGF-1R antibodies**

VRDN-002 and VRDN-003 are next generation IGF-1R humanized monoclonal antibodies targeting IGF-1R and incorporating half-life extension technology, designed to support administration as a self-administered low-volume, subcutaneous pen injection for the treatment of TED. The low-dose data of VRDN-001 in patients with active TED support the potential for VRDN-001 to also be administered as a convenient, low volume, subcutaneous pen. We plan to initiate a Phase 1 trial of subcutaneously administered VRDN-001 in healthy volunteers, with results expected in the fourth quarter of 2023.

VRDN-002 is a distinct antibody that acts as a partial antagonist of the IGF-1R. In 2022, we reported data from the Phase 1 trial in healthy volunteers, showing that VRDN-002 achieved a half-life of up to 43 days compared to the half-life of 10 to 11 days achieved by VRDN-001 and Tepezza in separate trials. VRDN-002 had a favorable safety profile and was well tolerated. We are currently planning to evaluate the safety and efficacy of a low-volume subcutaneous injection of VRDN-002 in patients with active TED. Results from the trial are expected by year-end 2023.

VRDN-003 is an anti-IGF-1R monoclonal antibody with the same amino acid sequence as VRDN-001, except for the addition of the half-life extension technology that is incorporated in VRDN-002. We plan to submit the IND with the FDA in the second quarter of 2023. Following FDA acceptance of the IND, we plan to initiate a Phase 1 trial of VRDN-003 in healthy volunteers, with results expected in the fourth quarter of 2023.

Our most recent PK modeling supports the feasibility of ongoing development of a self-administered pen for subcutaneous administration, and a planned dosing interval of up to once-monthly for VRDN-001, VRDN-002, and VRDN-003. We expect to select VRDN-001, VRDN-002, or VRDN-003 as our lead subcutaneous program by year-end 2023. We plan to advance our selected lead subcutaneous program into a pivotal Phase 3 trial in the middle of 2024.

### **Intellectual Property**

As of December 31, 2022, with regard to our VRDN-001, VRDN-002 and VRDN-003 product candidates, we have five pending U.S. non-provisional patent applications, three PCT applications, two foreign applications, and one provisional application directed to compositions and directed to methods of using VRDN-001, VRDN-002 and/or VRDN-003 for the treatment of TED. We wholly own one issued patent, U.S. Patent No. 11,548,951, which issued on January 10, 2023. The patent claims, among other things, are directed to methods of treating thyroid eye disease by inhibiting IGF-1R activity in a subject in need thereof with VRDN-001 or VRDN-003. The '951 patent is expected to expire no earlier than 2041, subject to any disclaimers or extensions. Any patents to issue from any pending non-provisional applications would be expected to expire no earlier than 2041, without taking potential patent term extensions or disclaimers into account.

### **Competition**

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. Our product candidates may address multiple markets. Ultimately, the diseases our product candidates target, and for which product candidates we may receive marketing authorization, will determine our competition. We believe that for most or all of our product development programs, there will be one or more competing programs under development by other companies. Any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future. We face potential competition from many different sources, including larger and better-funded biotechnology and pharmaceutical companies. In many cases, the companies with competing programs will have access to greater resources and expertise than we do and may be more advanced in those programs. Horizon Therapeutics plc's Tepezza® is the only FDA-approved medication for TED. Other therapies, such as corticosteroids, have been used on an off-label basis to alleviate some of the symptoms of TED. Other companies that are advancing therapies for the treatment of TED in clinical development include ACELYRIN, Inc., argenx, Harbour BioMed, Immunovant, Inc., and Sling Therapeutics, Inc.

- ACELYRIN, INC. is developing Lonigutamab (VB-421), a subcutaneously delivered anti-IGF-1R currently being evaluated in a Phase 1 study for TED.



- argenx is developing efgartigimod (Vyvgart®), an antibody fragment to target the neonatal Fc receptor (FcRn) expected to be evaluated in a registrational Phase 3 trial in patients with TED.
- Immunovant and Harbour BioMed are developing batoclimab (IMVT-1401/HBM9161), a monoclonal antibody targeting FcRn currently being evaluated in an ongoing Phase 3 trial in patients with TED.
- Sling Therapeutics, Inc. is developing linsitinib, a small molecule IGF-1R inhibitor currently be evaluated in an ongoing Phase 2b LIDS clinical trial in patients with active, moderate-to-severe TED.

## **License Agreements**

### ***License Agreement with Zenas BioPharma***

In October 2020, Viridian Therapeutics, Inc. (“Private Viridian”) entered a license agreement with Zenas BioPharma (Cayman) Limited (“Zenas BioPharma”) to license technology comprising certain materials, patent rights, and know-how to Zenas BioPharma. On October 27, 2020, in connection with the closing of the Private Viridian acquisition, we became party to the license agreement with Zenas BioPharma. Since February 2021, we have entered into several letter agreements with Zenas BioPharma in which we agreed to provide assistance to Zenas BioPharma with certain development activities, including manufacturing. The license agreement, as amended, and letter agreements (collectively, the “Zenas Agreements”) were negotiated with a single commercial objective and are treated as a combined contract for accounting purposes. Under the terms of the Zenas Agreements, we granted Zenas BioPharma an exclusive license to develop, manufacture, and commercialize certain IGF-1R directed antibody products for non-oncology indications in the greater area of China.

As consideration for the Zenas Agreements, the transaction price included upfront non-cash consideration and variable consideration in the form of payment for our goods and services provided and milestone payments due upon the achievement of specified events. Under the Zenas Agreements, we are eligible to receive non-refundable milestone payments upon achieving specific milestone events during the contract term. Additionally, we are eligible to receive royalty payments based on a percentage of the annual net sales of any licensed products sold on a country-by-country basis in the greater area of China. The royalty percentage may vary based on different tiers of annual net sales of the licensed products made. Zenas BioPharma is obligated to make royalty payments to us for the royalty term in the Zenas Agreements.

### ***License Agreement with ImmunoGen, Inc.***

On October 12, 2020, Private Viridian entered into a license agreement with ImmunoGen (the “ImmunoGen License Agreement”), under which we obtained rights to an exclusive, sublicensable, worldwide license to certain patents and other intellectual property rights to develop, manufacture, and commercialize certain products for non-oncology and non-radiopharmaceutical indications. In consideration for rights granted by ImmunoGen, we are obligated to make certain development milestone payments of up to \$48.0 million. Additionally, if we successfully commercialize any product candidate subject to the ImmunoGen License Agreement, we are responsible for royalty payments equal to a percentage in the mid-single digits of net sales and commercial milestone payments of up to \$95.0 million. We assumed the ImmunoGen License Agreement in connection with the merger with Private Viridian in 2020.

### ***License Agreements with Xencor, Inc.***

In December 2020, we entered into a license agreement with Xencor, Inc. (“Xencor”) (the “Xencor License Agreement”), under which Xencor granted us rights to an exclusive, worldwide, sublicensable, non-transferable, royalty-bearing license to use specified Xencor technology for the research, development, manufacturing, and commercialization of therapeutic antibodies targeting IGF-1R. In consideration for rights granted by Xencor, we issued 322,407 shares of our common stock to Xencor in December 2020. The shares were valued at \$6.0 million and recorded as research and development expense in 2020. Under the terms of the Xencor License Agreement, we are obligated to make future development milestone payments of up to \$30.0 million. Additionally, if we successfully commercialize any product candidate subject to the Xencor License Agreement, we are responsible for royalty payments equal to a percentage in the mid-single digits of net sales and commercial milestone payments of up to \$25.0 million.

In December 2021, we entered into a subsequent technology license agreement with Xencor (the “2021 Xencor License Agreement”) for a non-exclusive license to certain antibody libraries developed by Xencor. Under the 2021 Xencor License Agreement, we received a one-year research license to review the antibodies and the right to select up to three antibodies for further development. In consideration for rights granted by Xencor, we issued 394,737 shares of our common stock to Xencor in December 2021. The shares were valued at \$7.5 million and recorded as research and development expense during the year



ended December 31, 2021. Under the terms of the 2021 Xencor License Agreement, for each licensed product, we are obligated to make future milestone payments of up to \$27.75 million, which includes development and regulatory milestone payments of up to \$4.75 million, special milestone payments of up to \$3.0 million, and commercial milestone payments of up to \$20.0 million. Additionally, for each licensed product that we successfully commercialize, we are responsible for royalty payments equal to a percentage in the mid-single digits of net sales.

### ***Antibody and Discovery Option Agreement with Paragon Therapeutics, Inc.***

In January 2022, we entered into an antibody and discovery option agreement with Therapeutics, Inc. (“Paragon”) (the “Paragon Agreement”) under which we and Paragon will cooperate to develop one or more antibodies. Under the terms of the Paragon Agreement, Paragon will perform certain development activities in accordance with an agreed upon research plan, and we will pay Paragon agreed upon development fees in exchange for Paragon’s commitment of the necessary personnel and resources to perform these activities. The Paragon Agreement stipulates a final deliverable to us comprising of a report summarizing the experiments and processes performed under the research plan (the “Final Deliverable”).

Additionally, Paragon agreed to grant us an option for an exclusive license to all of Paragon’s right, title and interest in and to certain antibody technology and the Final Deliverable, and a non-exclusive license to certain background intellectual property owned by Paragon solely to research, develop, make, use, sell, offer for sale and import of the licensed intellectual property and resulting products worldwide (each, an “Option” and together, the “Options”). Paragon also granted us a limited, exclusive, royalty-free license, without the right to sublicense, to certain antibody technology and the Final Deliverable, and a non-exclusive, royalty-free license without the right to sublicense, under certain background intellectual property owned by Paragon, solely to evaluate the antibody technology and Option and for the purpose of allowing us to determine whether to exercise the Option with respect to certain programs. We may, at our sole discretion, exercise the Option with respect to specified programs at any time until the date that is 90 days after the Company’s receipt of the Final Deliverable the applicable program, or such longer period as agreed upon by the parties (“Option Period”) by delivering written notice of such exercise to Paragon. If we fail to exercise an Option prior to expiration of the applicable Option Period, such Option for such Program will terminate. In consideration for Paragon’s grant of the Options to us, we paid to Paragon a non-refundable, non-creditable one-time fee of \$2.5 million, which was recorded as research and development expense during the three months ended March 31, 2022. In December 2022, we and Paragon entered into a first amendment to the Paragon Agreement, under which we obtained an additional limited license for the purpose of conducting certain activities. In consideration for the rights and licenses obtained under the first amendment, Viridian paid Paragon a non-refundable fee of \$2.3 million (the “First Amendment Payment”), which was recorded as research and development expense during the three months ended December 31, 2022. The non-refundable upfront fee and the First Amendment Payment are separate from any development costs or cost advance paid or owing with respect to the specified program. During the year ended December 31, 2022, the Company recorded \$5.6 million in research and development costs related to the Paragon Agreement.

### **Government Regulation**

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of biologics such as those we are developing. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

### ***U.S. Biologics Regulation***

In the United States, biological products are subject to regulation under the Federal Food, Drug, and Cosmetic Act (“FDCA”), the Public Health Service Act (“PHSA”) and other federal, state, local, and foreign statutes and regulations. The process required by the FDA before biologic product candidates may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and animal studies performed in accordance with the FDA’s current Good Laboratory Practices (“GLP”) regulation;
- submission to the FDA of an IND, which must become effective before clinical trials may begin and must be updated annually or when significant changes are made;
- approval by an independent institutional review board (“IRB”) or ethics committee at each clinical site before the trial is commenced;

- manufacture of the proposed biologic candidate in accordance with cGMPs;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice (“GCP”) requirements to establish the safety, purity and potency of the proposed biologic product candidate for its intended purpose;
- preparation of and submission to the FDA of a biologics license application (“BLA”) after completion of all pivotal clinical trials;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of a BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMPs, and to assure that the facilities, methods and controls are adequate to preserve the biological product’s continued safety, purity and potency, and of selected clinical investigation sites to assess compliance with GCPs; and
- FDA review and approval of a BLA to permit commercial marketing of the product for particular indications for use in the United States.

### ***Preclinical and Clinical Development***

Prior to beginning the first clinical trial with a product candidate, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

In addition to the IND submission process, supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee (“IBC”), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment and such review may result in some delay before initiation of a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries.

For purposes of BLA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- Phase 1. The investigational product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- Phase 2. The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- Phase 3. The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the BLA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the biological characteristics of the product candidate and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product, or for biologics, the safety, purity and potency. 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.

#### ***BLA Submission and Review***

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of a BLA requesting approval to market the product for one or more indications. The BLA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of the product, or from a number of alternative sources, including studies initiated and sponsored by investigators. The submission of a BLA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies.

In addition, under the Pediatric Research Equity Act ("PREA"), a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product candidate 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. The Food and Drug Administration Safety and Innovation Act requires that a sponsor who is planning to submit a marketing application for a biological product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial pediatric study plan ("PSA"), within sixty days after an end-of-Phase 2 meeting or as may be agreed between the sponsor and FDA. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. Once a BLA has been accepted for filing, the FDA's goal is to review standard applications within ten months after the filing date, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process may also be extended by FDA requests for additional information or clarification. The FDA reviews a BLA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving a BLA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates a BLA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be produced, the FDA may issue an approval letter or a Complete Response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response letter will describe all of the deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response letter, the FDA may recommend actions that the applicant might take to place the BLA in condition for approval, including requests for additional information or clarification. The FDA may delay or refuse approval of a BLA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the BLA with a Risk Evaluation and Mitigation Strategy (“REMS”) to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product’s safety and effectiveness after commercialization and may limit further marketing of the product based on the results of these post-marketing studies.

### ***Orphan Drug Designation***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a New Drug Application (“NDA”) or BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full BLA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the marketing application fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

### ***Expedited Development and Review Programs***



The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a fast track product has opportunities for more frequent interactions with the review team during product development and, once a BLA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA.

A product intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers.

Any marketing application for a biologic submitted to the FDA for approval, including a product with a fast track designation and/or breakthrough therapy designation, may be eligible for other types of FDA programs intended to expedite the FDA review and approval process, such as priority review and accelerated approval. A product is eligible for priority review if it has the potential to provide a significant improvement in the treatment, diagnosis or prevention of a serious disease or condition. For original BLAs, priority review designation means the FDA's goal is to take action on the marketing application within six months of the 60-day filing date (as compared to ten months under standard review).

Additionally, products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. Products receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to conduct the required post-marketing studies or if such studies fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

In 2017, the FDA established a new regenerative medicine advanced therapy ("RMAT") designation as part of its implementation of the 21st Century Cures Act. The RMAT designation program is intended to fulfill the 21st Century Cures Act requirement that the FDA facilitate an efficient development program for, and expedite review of, any drug that meets the following criteria: (i) the drug qualifies as a RMAT, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (ii) the drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and (iii) preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such a disease or condition. RMAT designation provides all the benefits of breakthrough therapy designation, including more frequent meetings with the FDA to discuss the development plan for the product candidate and eligibility for rolling review and priority review. Products granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or reliance upon data obtained from a meaningful number of sites, including through expansion to additional sites. Once approved, when appropriate, the FDA can permit fulfillment of post-approval requirements under accelerated approval through: the submission of clinical evidence, preclinical studies, clinical trials, patient registries or other sources of real world evidence such as electronic health records; the collection of larger confirmatory datasets; or post-approval monitoring of all patients treated with the therapy prior to approval.

Fast track designation, breakthrough therapy designation, priority review and RMAT designation do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. In May 2018, the Right to Try Act established a new regulatory

pathway to increase access to unapproved, investigational treatments for patients diagnosed with life-threatening diseases or conditions who have exhausted approved treatment options and who are unable to participate in a clinical trial.

The Consolidated Appropriations Act, 2023 strengthens the FDA's authority to require and regulate post-approval studies of accelerated approval drugs and to expedite the rescission of accelerated approval based on these post-approval studies.

### ***Post-Approval Requirements***

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved BLA. Biologic manufacturers and 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 cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards 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 studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of biologics. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

### ***Biosimilars and Reference Product Exclusivity***



The Affordable Care Act (“ACA”) includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (“BPCIA”), which created an abbreviated approval pathway for biological products that are highly similar, or “biosimilar,” to or interchangeable with an FDA-approved reference biological product. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars.

Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, is generally shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product in any given patient and, for products that are administered multiple times to an individual, the biologic and the reference biologic may be alternated or switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. A product shown to be biosimilar or interchangeable with an FDA-approved reference biological product may rely in part on the FDA’s previous determination of safety and effectiveness for the reference product for approval, which can potentially reduce the cost and time required to obtain approval to market the product. Complexities associated with the larger, and often more complex, structures of biological products, as well as the processes by which such products are manufactured, pose significant hurdles to implementation of the abbreviated approval pathway that are still being worked out by the FDA. In September 2021, the FDA issued two guidance documents intended to inform prospective applicants and facilitate the development of proposed biosimilars and interchangeable biosimilars, as well as to describe the FDA’s interpretation of certain statutory requirements added by the BPCIA.

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 exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing that applicant’s own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of its product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed “interchangeable” by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

A biological product can also obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued “Written Request” for such a study.

The BPCIA is complex and continues to be interpreted and implemented by the FDA. In July 2018, the FDA announced an action plan to encourage the development and efficient review of biosimilars, including the establishment of a new office within the agency that will focus on therapeutic biologics and biosimilars. On December 20, 2020, Congress amended the PHSA as part of the COVID-19 relief bill to further simplify the biosimilar review process by making it optional to show that conditions of use proposed in labeling have been previously approved for the reference product, which used to be a requirement of the application. In addition, government proposals have sought to reduce the 12-year reference product exclusivity period. As of March 2020, certain products previously approved as drugs under the FDCA, such as insulin and human growth hormone, are now deemed to be biologics under the PHSA, which means they may face competition through the biosimilars pathway and are not be eligible for the twelve-year period of exclusivity granted to new BLAs. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. As a result, the ultimate impact, implementation, and impact of the BPCIA is subject to significant uncertainty.

As discussed below, the Inflation Reduction Act of 2022 (“IRA”) is a significant new law that intends to foster generic and biosimilar competition and to lower drug and biologic costs.

## **Foreign Regulation**

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing, among other things, research and development, clinical trials, testing, manufacturing, safety, efficacy, quality control, labeling, packaging, storage, record keeping, distribution, reporting, export and import, advertising, marketing and other promotional practices involving biological products as well as authorization, approval as well as post-approval monitoring and reporting of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials.

The requirements and process governing the conduct of clinical trials, including requirements to conduct additional clinical trials, product licensing, safety reporting, post-authorization requirements, marketing and promotion, interactions with healthcare professionals, pricing and reimbursement may vary widely from country to country. No action can be taken to market any product in a country until an appropriate approval application has been approved by the regulatory authorities in that country. The current approval process varies from country to country, and the time spent in gaining approval varies from that required for FDA approval. In certain countries, the sales price of a product must also be approved. The pricing review period often begins after market approval is granted. Even if a product is approved by a regulatory authority, satisfactory prices may not be approved for such product, which would make launch of such products commercially unfeasible in such countries.

## **Regulation in the European Union**

### ***European Data Laws***

The collection and use of personal health data and other personal data in the European Union (“EU”) is governed by the provisions of the European General Data Protection Regulation 2016/679 (“GDPR”), which came into force in May 2018, and related data protection laws in individual EU Member States. The GDPR imposes a number of strict obligations and restrictions on the ability to process, including collecting, analyzing and transferring, personal data of individuals, in particular with respect to health data from clinical trials and adverse event reporting. The GDPR includes requirements relating to the legal basis of the processing (such as consent of the individuals to whom the personal data relates), the information provided to the individuals prior to processing their personal data, the notification obligations to the national data protection authorities, and the security and confidentiality of the personal data. EU Member States may also impose additional requirements in relation to health, genetic and biometric data through their national legislation.

In addition, the GDPR imposes specific restrictions on the transfer of personal data to countries outside of the EU/European Economic Area (“EEA”) that are not considered by the European Commission (“EC”) to provide an adequate level of data protection (including the United States). Appropriate safeguards are required to enable such transfers. Among the appropriate safeguards that can be used, the data exporter may use the standard contractual clauses (“SCCs”). On March 25, 2022, the EC and the United States announced that they have agreed in principle on a new Trans-Atlantic Data Privacy Framework. Following this statement, on October 7, 2022, President Biden signed an Executive Order on ‘Enhancing Safeguards for United States Signals Intelligence Activities’, which implemented the agreement in principle. On that basis, the EC prepared a draft adequacy decision and launched its adoption procedure. While this new EU-U.S. privacy framework is expected to enter into force in 2023, there is still some uncertainty around the new framework.

Failure to comply with the requirements of the GDPR and the related national data protection laws of the EU Member States may result in significant monetary fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater, other administrative penalties and a number of criminal offenses (punishable by uncapped fines) for organizations and, in certain cases, their directors and officers, as well as civil liability claims from individuals whose personal data was processed. Data protection authorities from the different EU Member States may still implement certain variations, enforce the GDPR and national data protection laws differently, and introduce additional national regulations and guidelines, which adds to the complexity of processing personal data in the EU. Guidance developed at both the EU level and at the national level in individual EU Member States concerning implementation and compliance practices are often updated or otherwise revised.

Furthermore, there is a growing trend towards the required public disclosure of clinical trial data in the EU, which adds to the complexity of obligations relating to processing health data from clinical trials. Such public disclosure obligations are provided in the new EU Clinical Trials Regulation No.536/2014 (“CTR”), European Medicines Agency (“EMA”) disclosure initiatives and voluntary commitments by industry. Failure to comply with these obligations could lead to government enforcement actions and significant penalties against us, harm to our reputation, and adversely impact our business and operating results. The uncertainty regarding the interplay between different regulatory frameworks, such as the CTR and the GDPR, further adds to the complexity that we face with regard to data protection regulation.

With regard to the transfer of data from the EU to the United Kingdom, personal data may now freely flow from the EU to the UK since the UK is deemed to have an adequate data protection level. However, the adequacy decisions include a ‘sunset clause’ which entails that the decisions will automatically expire four years after their entry into force. Additionally, following

the UK's withdrawal from the EU and the EEA, companies also have to comply with the UK's data protection laws (including the GDPR, as incorporated into UK national law), the latter regime having the ability to separately fine up to the greater of £17.5 million or 4% of global turnover.

### ***Drug and Biologic Development Process***

Regardless of where they are conducted, all clinical trials included in applications for marketing authorization for human medicines in the European EU / EEA must have been carried out in accordance with EU regulations. This means that clinical trials conducted in the EU / EEA have to comply with EU clinical trial legislation but also that clinical trials conducted outside the EU / EEA have to comply with ethical principles equivalent to those set out in the EEA, including adhering to international good clinical practice and the Declaration of Helsinki. The conduct of clinical trials in the EU is governed by the CTR, which entered into force on January 31, 2022. The CTR replaced the Clinical Trials Directive 2001/20/EC, (Clinical Trials Directive) and introduced a complete overhaul of the existing regulation of clinical trials for medicinal products in the EU.

Under the former regime, which will expire after a transition period of one or three years, respectively, as outlined below in more detail, before a clinical trial can be initiated it must be approved in each EU member state where there is a site at which the clinical trial is to be conducted. The approval must be obtained from two separate entities: the National Competent Authority (“NCA”) and one or more Ethics Committees. The NCA of the EU Member States in which the clinical trial will be conducted must authorize the conduct of the trial, and the independent Ethics Committee must grant a positive opinion in relation to the conduct of the clinical trial in the relevant EU member state before the commencement of the trial. Any substantial changes to the trial protocol or other information submitted with the clinical trial applications must be submitted to or approved by the relevant NCA and Ethics Committees. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the NCA and to the Ethics Committees of the EU member state where they occur.

A more unified procedure will apply under the new CTR. A sponsor will be able to submit a single application for approval of a clinical trial through a centralized EU clinical trials portal. One national regulatory authority (the reporting EU member state proposed by the applicant) will take the lead in validating and evaluating the application consult and coordinate with the other concerned EU Member States. If an application is rejected, it may be amended and resubmitted through the EU clinical trials portal. If an approval is issued, the sponsor may start the clinical trial in all concerned EU Member States. However, a concerned EU member state may in limited circumstances declare an “opt-out” from an approval and prevent the clinical trial from being conducted in such member state. The CTR also aims to streamline and simplify the rules on safety reporting, and introduces enhanced transparency requirements such as mandatory submission of a summary of the clinical trial results to the EU Database. The CTR foresees a three-year transition period. EU Member States will work in CTIS immediately after the system has gone live. On January 31, 2023, submission of initial clinical trial applications via CTIS became mandatory, and by January 31, 2025, all ongoing trials approved under the former Clinical Trials Directive will need to comply with the CTR and have to be transitioned to CTIS.

Under both the former regime and the new CTR, national laws, regulations, and the applicable Good Clinical Practice, or GCP, and Good Laboratory Practice standards must also be respected during the conduct of the trials, including the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use, or ICH, guidelines on Good Clinical Practice and the ethical principles that have their origin in the Declaration of Helsinki.

During the development of a medicinal product, the European Medical Agency, or EMA and national regulators within the EU provide the opportunity for dialogue and guidance on the development program. At the EMA level, this is usually done in the form of scientific advice, which is given by the Committee for Medicinal Products for Human Use (“CHMP”) on the recommendation of the Scientific Advice Working Party (“SAWP”). A fee is incurred with each scientific advice procedure, but is significantly reduced for designated orphan medicines. Advice from the EMA is typically provided based on questions concerning, for example, quality (chemistry, manufacturing and controls testing), nonclinical testing and clinical studies, and pharmacovigilance plans and risk-management programs. Advice is not legally binding with regard to any future Marketing Authorization Application (“MAA”) of the product concerned.

### ***Drug Marketing Authorization***

In the European Union, medicinal products, including advanced therapy medicinal products (“ATMPs”) are subject to extensive pre- and post-market regulation by regulatory authorities at both the European Union and national levels. ATMPs comprise gene therapy products, somatic cell therapy products and tissue engineered products, which are genes, cells or tissues that have undergone substantial manipulation and that are administered to human beings in order to cure, diagnose or prevent diseases or regenerate, repair or replace a human tissue. Pursuant to the ATMP Regulation, the Committee on Advanced Therapies

(“CAT”) is responsible in conjunction with the CHMP for the evaluation of ATMPs. The CHMP and CAT are also responsible for providing guidelines on ATMPs. These guidelines provide additional guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs and include, among other things, the preclinical studies required to characterize ATMPs manufacturing and control information that should be submitted in a In the EU and EEA, after completion of all required clinical testing, pharmaceutical products may only be placed on the market after obtaining a Marketing Authorization (“MA”). To obtain an MA of a drug under European Union regulatory systems, an applicant can submit an MAA, through, amongst others, a centralized or decentralized procedure.

### ***Centralized Authorization Procedure***

The centralized procedure provides for the grant of a single MA that is issued by the EC, following the scientific assessment of the application by the EMA that is valid for all EU Member States as well as in the three additional EEA Member States. The centralized procedure is compulsory for specific medicinal products, including for medicines developed by means of certain biotechnological processes, products designated as orphan medicinal products, ATMP, and medicinal products with a new active substance indicated for the treatment of certain diseases (AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases). For medicinal products containing a new active substance not yet authorized in the EEA before May 20, 2004 and indicated for the treatment of other diseases, medicinal products that constitute significant therapeutic, scientific or technical innovations or for which the grant of a MA through the centralized procedure would be in the interest of public health at EU level, an applicant may voluntarily submit an application for a marketing authorization through the centralized procedure.

Under the centralized procedure, the Committee for Medicinal Products for Human Use established at the EMA, is responsible for conducting the initial assessment of a drug. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure, the timeframe for the evaluation of an MAA by the EMA’s CHMP is, in principle, 210 days from receipt of a valid MAA. However, this timeline excludes clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP, so the overall process typically takes a year or more, unless the application is eligible for an accelerated assessment. Accelerated evaluation 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. Upon request, the CHMP can reduce the time frame to 150 days if the applicant provides sufficient justification for an accelerated assessment. The CHMP will provide a positive opinion regarding the application only if it meets certain quality, safety and efficacy requirements. This opinion is then transmitted to the EC, which has the ultimate authority for granting MA within 67 days after receipt of the CHMP opinion.

### ***Decentralized Authorization Procedure***

Medicines that fall outside the mandatory scope of the centralized procedure have three routes to authorization: (i) they can be authorized under the centralized procedure if they concern a significant therapeutic, scientific or technical innovation, or if their authorization would be in the interest of public health; (ii) they can be authorized under a decentralized procedure where an applicant applies for simultaneous authorization in more than one EU member state; or (iii) they can be authorized in an EU member state in accordance with that state’s national procedures and then be authorized in other EU countries by a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization (mutual recognition procedure).

The decentralized procedure permits companies to file identical MA applications for a medicinal product to the competent authorities in various EU Member States simultaneously if such medicinal product has not received marketing approval in any EU Member State before. This procedure is available for pharmaceutical products not falling within the mandatory scope of the centralized procedure. The competent authority of a single EU Member State, the reference member state, is appointed to review the application and provide an assessment report. The competent authorities of the other EU Member States, the concerned member states, are subsequently required to grant a marketing authorization for their territories on the basis of this assessment. The only exception to this is where the competent authority of an EU Member State considers that there are concerns of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the EC, whose decision is binding for all EU Member States.

### ***Risk Management Plan***

All new MAAs must include a Risk Management Plan (“RMP”) describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. RMPs are continually modified and updated throughout the lifetime of the medicine as new information becomes available. An updated RMP must be



submitted: (i) at the request of EMA or a national competent authority, or (ii) whenever the risk-management system is modified, especially as the result of new information being received that may lead to a significant change to the benefit-risk profile or as a result of an important pharmacovigilance or risk-minimization milestone being reached. The regulatory authorities may also impose specific obligations as a condition of the MA. RMPs and Periodic Safety Update Reports (“PSURs”) are routinely available to third parties requesting access, subject to limited redactions.

### ***MA Validity Period***

Marketing Authorizations have an initial duration of five years. After these five years, the authorization may subsequently be renewed on the basis of a reevaluation of the risk-benefit balance. Once renewed, the MA is valid for an unlimited period unless the EC or the national competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with only one additional five-year renewal. Applications for renewal must be made to the EMA at least nine months before the five-year period expires.

Additionally, the holder of a MA for an ATMP must put in place and maintain a system to ensure that each individual product and its starting and raw materials, including all substances coming into contact with the cells or tissues it may contain, can be traced through the sourcing, manufacturing, packaging, storage, transport and delivery to the relevant healthcare institution where the product is used.

### ***Exceptional Circumstances/Conditional Approval***

Similar to accelerated approval regulations in the United States, conditional MAs can be granted in the EU in exceptional circumstances. A conditional MA can be granted for medicinal products where, although comprehensive clinical data referring to the safety and efficacy of the medicinal product have not been supplied, a number of criteria are fulfilled: (i) the benefit/risk balance of the product is positive, (ii) it is likely that the applicant will be in a position to provide the comprehensive clinical data, (iii) unmet medical needs will be fulfilled by the grant of the MA and (iv) the benefit to public health 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. A conditional MA must be renewed annually.

### ***Orphan Designation and Exclusivity***

The criteria for designating an orphan medicinal product in the European Union are similar in principle to those in the United States. The EMA grants orphan drug designation if the medicinal product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (prevalence criterion). In addition, Orphan Drug Designation can be granted if, for economic reasons, the medicinal product would be unlikely to be developed without incentives and if there is no other satisfactory method approved in the European Union of diagnosing, preventing, or treating the condition, or if such a method exists, the proposed medicinal product is a significant benefit to patients affected by the condition. An application for orphan drug designation (which is not a marketing authorization, as not all orphan-designated medicines reach the authorization application stage) must be submitted first before an application for marketing authorization of the medicinal product is submitted. The applicant will receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted, and sponsors must submit an annual report to EMA summarizing the status of development of the medicine. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Designated orphan medicines are eligible for conditional marketing authorization.

The EMA’s Committee for Orphan Medicinal Products reassesses the orphan drug designation of a product in parallel with the review for a marketing authorization; for a product to benefit from market exclusivity it must maintain its orphan drug designation at the time of marketing authorization review by the EMA and approval by the EC. Additionally, any marketing authorization granted for an orphan medicinal product must only cover the therapeutic indication(s) that are covered by the orphan drug designation. Upon the grant of a marketing authorization, orphan drug designation provides up to ten years of market exclusivity in the orphan indication.

During the 10-year period of market exclusivity, with a limited number of exceptions, the regulatory authorities of the EU Member States and the EMA may not accept applications for marketing authorization, accept an application to extend an existing marketing authorization or grant marketing authorization for other similar medicinal products for the same therapeutic indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. An orphan medicinal product can also obtain an additional two years of market exclusivity for an orphan-designated condition

when the results of specific studies are reflected in the Summary of Product Characteristics (“SmPC”), addressing the pediatric population and completed in accordance with a fully compliant Pediatric Investigation Plan (“PIP”). No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, i.e. the condition prevalence or financial returns criteria under Article 3 of Regulation (EC) No. 141/2000 on orphan medicinal products. When the period of orphan market exclusivity for an indication ends, the orphan drug designation for that indication expires as well. Orphan exclusivity runs in parallel with normal rules on data exclusivity and market protection. Additionally, a marketing authorization may be granted to a similar medicinal product (orphan or not) for the same or overlapping indication subject to certain requirements.

### ***PRIME Designation***

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 (“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 may qualify for earlier entry into the PRIME scheme than larger companies on the basis of compelling non-clinical data and tolerability data from initial clinical trials. 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 potentially accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, once a candidate medicine has been selected for the PRIME scheme, a dedicated contact point and rapporteur from the CHMP or from CAT are appointed facilitating increased understanding of the product at EMA’s Committee level. A kick-off meeting with the CHMP/CAT rapporteur initiates these relationships and includes a team of multidisciplinary experts to provide guidance on the overall development plan and regulatory strategy. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval.

### **Other Regulations**

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation: the federal Anti-Kickback Statute (“AKS”); the federal False Claims Act (“FCA”); the Health Insurance Portability and Accountability Act of 1996 (“HIPAA”) and similar foreign, federal and state fraud, abuse and transparency laws.

The AKS prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value. The AKS has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand, and prescribers and purchasers on the other. The government often takes the position that to violate the AKS, only one purpose of the remuneration need be to induce referrals, even if there are other legitimate purposes for the remuneration. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from AKS prosecution, but they are drawn narrowly and practices that involve remuneration, such as consulting agreements, that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the AKS. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

Civil and criminal false claims laws, including the FCA, and civil monetary penalty laws, which can be enforced through civil whistleblower or qui tam actions, prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment of federal government funds, including in federal healthcare programs, that are false or fraudulent. Pharmaceutical and other healthcare companies have been prosecuted under these laws for engaging in a variety of different types of conduct that caused the submission of false claims to federal healthcare programs. Under the AKS, for example, a claim resulting from a violation of the AKS is deemed to be a false or fraudulent claim for purposes of the FCA. The FCA imposes mandatory treble damages and per-violation civil penalties up to approximately \$25,000.



HIPAA created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program, including private third-party payors, and making false statements relating to healthcare matters. A person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate the statute in order to have committed a violation.

The FDCA addresses, among other things, the design, production, labeling, promotion, manufacturing, and testing of drugs, biologics and medical devices, and prohibits such acts as the introduction into interstate commerce of adulterated or misbranded drugs or devices. The PHSa also prohibits the introduction into interstate commerce of unlicensed or mislabeled biological products.

The U.S. 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 annually report to CMS information related to payments or other transfers of value made to various healthcare professionals including physicians, physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, certified nurse-midwives, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Beginning on January 1, 2023, California Assembly Bill 1278 requires California physicians and surgeons to notify patients of Open Payments.

We are also subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

## **Data Privacy and Security**

Numerous state, federal, and foreign laws govern the collection, dissemination, use, access to, confidentiality, and security of personal information, including health-related information. In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws and regulations, govern the collection, use, disclosure, and protection of health-related and other personal information could apply to our operations or the operations of our partners. For example, HIPAA, as amended by Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations imposes privacy, security, and breach notification obligations on certain health care providers, health plans, and health care clearinghouses, known as covered entities, as well as their business associates that perform certain services that involve using, disclosing, creating, receiving, maintaining, or transmitting individually identifiable health information for or on behalf of such covered entities. Entities that are found to be in violation of HIPAA may be subject to significant civil, criminal, and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance. Further, entities that knowingly obtain, use, or disclose individually identifiable health information maintained by a HIPAA covered entity in a manner that is not authorized or permitted by HIPAA may be subject to criminal penalties.

Even when HIPAA does not apply, according to the FTC, violating consumers' privacy rights or failing to take appropriate steps to keep consumers' personal information secure may constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act.

In addition, state laws govern the privacy and security of personal information, including health-related information, in certain circumstances. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. For example, the CCPA, which went into effect on January 1, 2020, creates new data privacy obligations for covered companies and provides new privacy rights to California residents.

## **Health Reform**

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state legislative initiatives, including those designed to

limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

The ACA, which was enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future.

Other legislative changes have been proposed and adopted since the ACA was enacted, including automatic aggregate reductions of Medicare payments to providers of 2% per fiscal year as part of the federal budget sequestration under the Budget Control Act of 2011. These reductions went into effect in April 2013 and, due to subsequent legislative amendments, will remain in effect through 2030 with the exception of a temporary suspension from May 1, 2020 through December 31, 2020, unless additional action is taken by Congress. In addition, the Bipartisan Budget Act of 2018, among other things, amended the Medicare Act (as amended by the ACA) to increase the point-of-sale discounts that manufacturers must agree to offer under the Medicare Part D coverage discount program from 50% to 70% off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs being covered under Medicare Part D.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state measures designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, in May 2019, CMS adopted a final rule allowing Medicare Advantage Plans the option to use step therapy for Part B drugs, permitting Medicare Part D plans to apply certain utilization controls to new starts of five of the six protected class drugs, and requiring the Explanation of Benefits for Part D beneficiaries to disclose drug price increases and lower cost therapeutic alternatives, which went into effect on January 1, 2021.

Notwithstanding the IRA, continued legislative and enforcement interest exists in the United States with respect to specialty drug pricing practices. Specifically, we expect regulators to continue pushing for transparency to drug pricing, reducing the cost of prescription drugs under Medicare, reviewing the relationship between pricing and manufacturer patient programs, and reforming government program reimbursement methodologies for drugs.

### **Coverage and Reimbursement**

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third-party not to cover a product could reduce physician usage and patient demand for the product.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. The IRA provides CMS with significant new authorities intended to curb drug costs and to encourage market competition. For the first time, CMS will be able to directly negotiate prescription drug prices and to cap out-of-pocket costs. Each year, CMS will select and negotiate a preset number of high-spend drugs and biologics that are covered under Medicare Part B and Part D that do not have generic or biosimilar competition. These price negotiations will begin in 2023. The IRA also provides a new “inflation rebate” covering Medicare patients that will take effect in 2023 and is intended to counter certain price increases in prescriptions drugs. The inflation rebate provision will require drug manufacturers to pay a rebate to the federal government if the price for a drug or biologic under Medicare Part B and Part D increases faster than the rate of inflation. To support biosimilar competition, beginning in October 2022, qualifying biosimilars may receive a Medicare Part B payment increase for a period of five years. Separately, if a biologic drug for which no biosimilar exists delays a biosimilar’s market entry beyond two years, CMS will be authorized to subject the biologics manufacturer to price negotiations intended to ensure fair competition. Notwithstanding these provisions, the IRA’s impact on commercialization and competition remains largely uncertain.

## **Manufacturing**

We do not own or operate clinical or commercial manufacturing facilities for the production of our product candidates that we develop, nor do we have plans to develop our own manufacturing operations in the foreseeable future. We currently depend on third-party contract manufacturers for all of our required raw materials, active pharmaceutical ingredients, and finished product candidates for our clinical trials. We do not have any current contractual arrangements for the manufacture of commercial supplies of our product candidates that we develop. We currently employ internal resources and third-party consultants to manage our manufacturing contractors.

Historically, we have relied on third-party contract development and manufacturing organizations (“CDMOs”), to manufacture and supply our preclinical and clinical materials used during the development of our product candidates. We initially pursued three separate manufacturing paths to supply investigational product for our planned clinical trials in order to mitigate delays and uncertainties. We currently rely on a single multi-site CDMO for such manufacturing, although other avenues remain available if our current manufacturer were to be negatively impacted. We maintain a long-term master services agreement with our CDMO pursuant to which the CDMO provides biologics development and manufacturing services on a per-project basis and a related cell line license. We may terminate the master services agreement at any time for convenience in accordance with the terms of the agreement. We may also terminate the master services agreement in the event that the CDMO does not obtain or maintain any material governmental license or approval in accordance with the terms of the agreement. The agreement includes confidentiality and intellectual property provisions to protect our proprietary rights related to our product candidates. We do not currently have arrangements in place for redundant supply. While any reduction or halt in supply from the CDMO could limit our ability to develop our product candidates until a replacement CDMO is found and qualified, we believe that we have sufficient supply to support our current clinical trial programs. Any reduction or halt in supply from the CDMO could limit our ability to develop our product candidates until a replacement CDMO is found and qualified, although we believe that we have supply on hand that can partially support our current clinical trial programs until a replacement CDMO is secured.

## **Sales and Marketing**

We have not yet defined our sales, marketing, or product distribution strategy for our product candidates because our product candidates are still in preclinical or early-stage clinical development. Our commercial strategy may include the use of strategic partners, distributors, a contract sale force, or the establishment of our own commercial and specialty sales force. We plan to further evaluate these alternatives as we continue to advance into later stages of development for each one of our product candidates.

## **Human Capital Management**

As of December 31, 2022, we employed 86 full-time employees located in the United States. During 2022, we expanded our capabilities in the organization by hiring 49 new employees. These employees were hired to support our clinical development,

preclinical research and development, and efforts associated with operating as a public company. We consider our relationship with our employees to be good. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective bargaining arrangements. We consider our employee relations to be good. We track and report internally on key talent metrics including workforce demographics, diversity data and the status of open positions. We are committed to equality, inclusion and diversity in the workplace. As of December 31, 2022, nearly 35% of our workforce identify as members of underrepresented ethnic communities and 55% identify as female. We strive to interview diverse candidates for our open positions.

Attracting, developing and retaining talented employees to support the growth of our business is an integral part of our human capital strategy and critical to our long-term success. We continue to seek additions to our staff, although the competition in our industry and in the Greater Boston area, where our headquarters is located, is significant. The principal purpose of our equity incentive and annual bonus programs is to attract, retain and motivate personnel through the granting of stock-based compensation awards and cash-based performance bonus awards. As a biopharmaceutical company, we recognize the importance of access to high quality healthcare and as such we cover 100% of our employees' monthly healthcare premiums. We offer a package of competitive employee benefits, including 401(k) plan matching contributions and an employee stock purchase plan.

We have a performance development review process in which managers provide regular feedback to assist with the development of our employees, including the use of individual plans to assist with career development. We also invest in the growth and development of our employees through various training and development programs that help build and strengthen our employees' leadership and professional skills.

We believe our management team has the experience necessary to effectively execute our strategy and advance our product and technology leadership. A large majority of our employees have obtained advanced degrees in their professions. We support our employees' further development with individualized development plans, mentoring, coaching, group training and conference attendance.

## **Our Corporate Information**

We were initially founded as a Delaware limited liability company in January 2010 and subsequently incorporated as a Delaware corporation in June 2014. On January 20, 2021, pursuant to a merger agreement under which miRagen Therapeutics, Inc. acquired Viridian Therapeutics, Inc., we changed our name from Miragen Therapeutics, Inc. to Viridian Therapeutics, Inc. Our common stock currently trades on The Nasdaq Capital Market under the ticker symbol "VRDN." Our principal executive office is located at 221 Crescent Street, Suite 401, Waltham, MA 02453, and our telephone number is (617) 272-4600. Our website address is [www.viridiantherapeutics.com](http://www.viridiantherapeutics.com). The information contained on, or that can be accessed through, our website is not part of this Annual Report. We have included our website in this Annual Report solely as an inactive textual reference.

This Annual Report contains references to our trademarks and trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this Annual Report, including logos, artwork, and other visual displays, may appear without the ® or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other company.

## **Available Information**

Our Annual Reports, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and amendments to reports filed pursuant to Sections 13(a) and 15(d) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") are available free of charge on our website located at [www.viridiantherapeutics.com](http://www.viridiantherapeutics.com) as soon as reasonably practicable after they are filed with the SEC. The reports are also available at the SEC's internet website at [www.sec.gov](http://www.sec.gov). A copy of our Corporate Governance Guidelines, Code of Business Conduct and Ethics, and the charters of the Audit Committee, Compensation Committee, and Nominative and Corporate Governance Committee are posted on our website, [www.viridiantherapeutics.com](http://www.viridiantherapeutics.com), under "Corporate Governance."

## **ITEM 1A. RISK FACTORS**

*Our business, financial condition, and operating results may be affected by a number of factors, whether currently known or unknown, including but not limited to those described below. Any one or more of such factors could directly or indirectly cause*

*our actual results of operations and financial condition to vary materially from past or anticipated future results of operations and financial condition. Any of these factors, in whole or in part, could materially and adversely affect our business, financial condition, results of operations, and stock price. The following information should be read in conjunction with Part II, Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the consolidated financial statements and related notes in Part II, Item 8, "Financial Statements and Supplementary Data" of this Annual Report.*

## **Risk Factor Summary**

*Investing in our common stock involves a high degree of risk because our business is subject to numerous risks and uncertainties, as fully described below. The principal factors and uncertainties that make investing in our common stock risky include, among others:*

- We will need to raise additional capital, and if we are unable to do so when needed, we will not be able to continue as a going concern.
- We have historically incurred losses, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.
- We have never generated any revenue from product sales and may never be profitable.
- Raising additional capital may cause dilution to our stockholders, restrict our operations, or require us to relinquish rights.
- Clinical trials are costly, time consuming, and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.
- We are heavily dependent on the success of our product candidates, which are in the early stages of clinical development. Some of our product candidates have produced results only in non-clinical settings, or for other indications than those for which we contemplate conducting development and seeking FDA approval, and we cannot give any assurance that we will generate data for any of our product candidates sufficiently supportive to receive regulatory approval in our planned indications, which will be required before they can be commercialized.
- Product development involves a lengthy and expensive process with an uncertain outcome, and results of earlier preclinical studies and clinical trials may not be predictive of future clinical trial results.

## **Risks Related to Our Financial Condition and Capital Requirements**

***We will need to raise additional capital, and if we are unable to do so when needed, we will not be able to continue as a going concern.***

As of December 31, 2022, we had \$424.6 million of cash, cash equivalents, and short-term investments. We believe that our current cash, cash equivalents and short-term investments, including the Term Loan, will be sufficient to fund our operations, including our clinical development plan described elsewhere in this Annual Report, into the second half of 2025. We will need to raise additional capital to continue to fund our operations and service our obligations in the future. If we are unable to raise additional capital when needed, we will not be able to continue as a going concern.

Developing our product candidates requires a substantial amount of capital. We expect our research and development expenses to increase in connection with our ongoing activities, particularly as we advance our product candidates through clinical trials. We will need to raise additional capital to fund our operations and such funding may not be available to us on acceptable terms, or at all.

We do not currently have any products approved for sale and do not generate any revenue from product sales. Accordingly, we expect to rely primarily on equity and/or debt financings to fund our continued operations. Our ability to raise additional funds will depend, in part, on the success of our preclinical studies and clinical trials and other product development activities, regulatory events, our ability to identify and enter into licensing or other strategic arrangements, and other events or conditions



that may affect our value or prospects, as well as factors related to financial, economic and market conditions, many of which are beyond our control. There can be no assurances that sufficient funds will be available to us when required or on acceptable terms, if at all.

If we are unable to raise additional capital when required or on acceptable terms, we may be required to:

- significantly delay, scale back, or discontinue the development or commercialization of our product candidates;
- seek strategic alliances, or amend existing alliances, for research and development programs at an earlier stage than otherwise would be desirable or that we otherwise would have sought to develop independently, or on terms that are less favorable than might otherwise be available in the future;
- dispose of technology assets, or relinquish or license on unfavorable terms, our rights to technologies or any of our product candidates that we otherwise would seek to develop or commercialize ourselves;
- pursue the sale of our company to a third party at a price that may result in a loss on investment for our stockholders; or
- file for bankruptcy or cease operations altogether.

Any of these events could have a material adverse effect on our business, operating results, and prospects.

***We have historically incurred losses, have a limited operating history on which to assess our business, and anticipate that we will continue to incur significant losses for the foreseeable future.***

We are a biopharmaceutical company with a limited operating history. We have historically incurred net losses. During the years ended December 31, 2022 and 2021, net loss was \$129.9 million and \$79.4 million, respectively. As of December 31, 2022, we had an accumulated deficit of \$488.2 million and cash, cash equivalents, and short-term investments of \$424.6 million.

We believe that our current cash, cash equivalents and short-term investments, including the Term Loan, will be sufficient to fund our operations, including our clinical development plan described elsewhere in this Annual Report, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2025. We will need to raise substantial additional capital to continue to fund our operations in the future. The amount and timing of our future funding requirements will depend on many factors, including the pace, results and costs of our clinical development efforts and macroeconomic conditions affecting our business and industry.

Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including conducting clinical trials and providing general and administrative support for our operations. To date, we have financed our operations primarily through the sale of equity securities and convertible promissory notes and the Hercules Loan and Security Agreement. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect our losses to increase as our product candidates enter more advanced clinical trials. It may be several years, if ever, before we complete pivotal clinical trials or have a product candidate approved for commercialization. We expect to invest significant funds into the research and development of our current product candidates to determine the potential to advance these product candidates to regulatory approval.

If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, coverage, and adequate reimbursement from third-party payors, and adequate market share for our product candidates in those markets.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future and our expenses will increase substantially if and as we:

- continue the development of our product candidates;
- continue efforts to discover and develop new product candidates;
- continue the manufacturing of our product candidates or increase volumes manufactured by third parties;
- advance our programs into large expensive clinical trials;
- initiate additional preclinical studies or clinical trials for our product candidates;
- seek regulatory and marketing approvals and reimbursement for our product candidates;
- establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval and market for ourselves;
- seek to identify, assess, acquire, and/or develop other product candidates;
- make milestone, royalty, or other payments under third-party license agreements;
- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel; and
- experience any delays or encounter issues with the development and potential for regulatory approval of our clinical and product candidates such as safety issues, manufacturing delays, clinical trial accrual delays, longer follow-up for planned studies or trials, additional major studies or trials, or supportive trials necessary to support marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

***We have never generated any revenue from product sales and may never be profitable.***

We have no products approved for commercialization and have never generated any revenue from product sales. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaborators, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and development of our product candidates;
- obtaining regulatory and marketing approvals for our product candidates;
- manufacturing product candidates and establishing and maintaining supply and manufacturing relationships with third parties that are commercially feasible, meet regulatory requirements and our supply needs in sufficient quantities to meet market demand for our product candidates, if approved;
- marketing, launching, and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- gaining market acceptance of our product candidates as treatment options;
- addressing any competing products;
- protecting and enforcing our intellectual property rights, including patents, trade secrets, and know-how;

- negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;
- obtaining coverage and adequate reimbursement from third-party payors and maintaining pricing for our product candidates that supports profitability; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Portions of our current pipeline of product candidates have been in-licensed from third parties, which make the commercial sale of such in-licensed products potentially subject to additional royalty and milestone payments to such third parties. We will also have to develop or acquire manufacturing capabilities or continue to contract with contract manufacturers in order to continue development and potential commercialization of our product candidates. For instance, if the costs of manufacturing our drug product are not commercially feasible, we will need to develop or procure our drug product in a commercially feasible manner in order to successfully commercialize a future approved product, if any. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

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

Until such time, if ever, as we can generate substantial revenue from the sale of our product candidates, we expect to finance our cash needs through a combination of equity offerings, debt financings, and license and development agreements. To the extent that we raise additional capital through the sale of equity securities or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution, or licensing arrangements with third parties, we may be required to relinquish valuable rights to our 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 or other arrangements with third parties when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to third parties to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

To the extent that we raise additional capital through the sale of equity, including pursuant to any sales under the Open Market Sale Agreement<sup>SM</sup> entered into in September 2022 (the “September 2022 ATM Agreement”) with Jefferies LLC (“Jefferies”), convertible debt, or other securities convertible into equity, the ownership interest of our stockholders will be diluted, and the terms of these new securities may include liquidation or other preferences that adversely affect the rights of our stockholders. In December 2019, the Company entered into a common stock purchase agreement (the “Aspire Stock Purchase Agreement”) with Aspire Capital, LLC (“Aspire Capital”). Any additional sales of our common stock under the September 2022 ATM Agreement and the Aspire Common Stock Purchase Agreement will dilute the ownership interest of our stockholders and may cause the price per share of our common stock to decrease. In addition, any exercise of outstanding warrants will dilute the ownership interest of our stockholders and may cause the price per share of our common stock to decrease.

Debt financing, including under our Hercules Loan and Security Agreement, may include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, making additional product acquisitions, or declaring dividends. If we raise additional funds through strategic collaborations or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates or future revenue streams or grant licenses on terms that are not favorable to us. We cannot be assured that we will be able to obtain additional funding if and when necessary to fund our entire portfolio of product candidates to meet our projected plans. If we are unable to obtain funding on a timely basis, we may be required to delay or discontinue one or more of our development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on potential business opportunities, which could materially harm our business, financial condition, and results of operations.

***Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.***

Our operations, and those of our third-party research institution collaborators, contract research organizations (“CROs”), contract manufacturing operations (“CMOs”), and other contractors and consultants, could be subject to acts of war, earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical pandemics or epidemics, such as the novel coronavirus, and other natural or man-made disasters or business interruptions, for which we are partly uninsured. In addition, we rely on our third-party research institution collaborators for conducting research and development of our product candidates, and they may be affected by government shutdowns or withdrawn funding. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses.

### **Risks Related to the Discovery and Development of Our Product Candidates**

***Clinical trials are costly, time consuming, and inherently risky, and we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.***

Clinical development is expensive, time consuming, and involves significant risk. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of development. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate satisfactory preclinical, toxicology, or other in vivo or in vitro data or diagnostics to support the initiation or continuation of clinical trials;
- delays in reaching agreement on acceptable terms with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs, clinical trial sites, and in countries or regions where our trials are conducted;
- delays in obtaining required approvals from institutional review boards or independent ethics committees at each clinical trial site;
- failure to permit the conduct of a clinical trial by regulatory authorities;
- delays in recruiting eligible patients and/or subjects in our clinical trials;
- failure by clinical sites, CROs, or other third parties to adhere to clinical trial requirements;
- failure by our clinical sites, CROs, or other third parties to perform in accordance with the good clinical practices requirements of the FDA or applicable foreign regulatory guidelines;
- patients and/or subjects dropping out of our clinical trials;
- adverse events or tolerability or animal toxicology issues significant enough for the FDA or other regulatory agencies to put any or all clinical trials on hold;
- occurrence of adverse events associated with our product candidates;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- significant costs of clinical trials of our product candidates, including manufacturing activities;
- negative or inconclusive results from our clinical trials, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development programs in other ongoing or planned indications for a product candidate; and
- delays in reaching agreement on acceptable terms with third-party manufacturers and the time to manufacture sufficient quantities of our product candidates acceptable for use in clinical trials.

The FDA may withdraw approval if compliance with regulatory requirements and standards 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 studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

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

Any inability to successfully complete clinical development and obtain regulatory approval for our product candidates could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional nonclinical studies and the results obtained from studying such new formulation may not be consistent with previous results obtained. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow competitors to develop and bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

***Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial viability of an approved label, or result in significant negative consequences following marketing approval, if any.***

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or terminate clinical trials. They additionally may result in a delay of regulatory approval by the FDA or comparable foreign authorities, or, even in the instance that an affected product candidate is approved, may result in a restrictive drug label.

Even if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such products;
- regulatory authorities may require additional warnings on the drug label;
- we may be required to create a REMS, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients or subjects; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of a product candidate, even if approved, and could significantly harm our business, results of operations, and prospects.

***Our product development program may not uncover all possible adverse events that patients or subjects who take our product candidates may experience. The number of patients or subjects exposed to our product candidates and the average exposure time in the clinical development program may be inadequate to detect rare adverse events that may only be detected once the product is administered to more patients or subjects and for greater periods of time.***

Clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, we cannot be fully assured that rare and severe side effects of our product candidates will be uncovered. Such rare and severe side effects may only be uncovered with a significantly larger number of patients or subjects exposed to the drug. If such safety problems occur or are identified after our product candidates reach the market, the FDA may require that we amend the labeling of the product or recall the product or may even withdraw approval for the product.



***We are heavily dependent on the success of our product candidates and we cannot give any assurance that we will generate data for any of our product candidates sufficiently supportive to receive regulatory approval in our planned indications, which will be required before they can be commercialized.***

We have invested substantially all of our effort and financial resources to identify, acquire, and develop our portfolio of product candidates. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for and commercialize one or more product candidates. We currently generate no revenue from sales of any products, and we may never be able to develop or commercialize a product candidate. We continue to evaluate and pursue additional opportunities to expand our product pipeline, either by discovering novel antibodies internally, or by acquiring rights to existing antibodies or antibody sequences. Our goal is to build a sustainable portfolio of investigational monoclonal antibody therapies.

We currently have a limited number of product candidates. There can be no assurance that the data that we may or may not develop for our product candidates in our planned indications will be sufficiently supportive to obtain regulatory approval.

We are not permitted to market or promote any of our product candidates before they receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. We cannot be certain that any of our product candidates will be successful in clinical trials or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical trials. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

***Product development involves a lengthy and expensive process with an uncertain outcome, and results of earlier preclinical studies and clinical trials may not be predictive of future clinical trial results.***

Clinical testing is expensive and generally takes many years to complete, and the outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of larger, later-stage controlled clinical trials. Product candidates that have shown promising results in early-stage clinical trials may still suffer significant setbacks in subsequent clinical trials. We will have to conduct well-controlled trials in our proposed indications to support any regulatory submissions for further clinical development. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles despite promising results in earlier, smaller clinical trials. Moreover, clinical data are often susceptible to varying interpretations and analyses. We do not know whether any clinical trials we may conduct will demonstrate consistent or adequate efficacy and safety of our product candidates, with respect to the proposed indication for use, sufficient to receive regulatory approval to market our drug candidates.

***We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and human resources, we may forgo or delay pursuit of opportunities with some programs or product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or more profitable market opportunities. Our spending on current and future research and development programs and future product candidates for specific indications may not yield any commercially viable products. We may also enter into additional strategic collaboration agreements to develop and commercialize some of our programs and potential product candidates in indications with potentially large commercial markets. 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 strategic collaborations, 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, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a collaboration arrangement.

***We may find it difficult to enroll and maintain patients or subjects in our clinical trials, in part due to the limited number of patients or subjects who have the diseases for which our product candidates are being studied. We cannot predict if we will have difficulty enrolling and maintaining patients or subjects in our future clinical trials. Difficulty in enrolling and maintaining patients or subjects could delay or prevent clinical trials of our product candidates.***

Identifying and qualifying patients or subjects to participate in clinical trials of our product candidates is essential to our success. The timing of our clinical trials depends in part on the rate at which we can recruit patients or subjects to participate in clinical trials of our product candidates, and we may experience delays in our clinical trials if we encounter difficulties in

enrollment. In addition, our enrollment has been and may in the future be delayed due to supply chain delays and difficulties in site activation.

The eligibility criteria of our clinical trials may further limit the available eligible trial participants as we expect to require that patients or subjects have specific characteristics that we can measure or meet the criteria to assure their conditions are appropriate for inclusion in our clinical trials. Accordingly, we may not be able to identify, recruit, enroll, and maintain a sufficient number of patients or subjects to complete our future clinical trials in a timely manner because of the perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical trials, the option for patients to choose alternate existing approved therapies, and the willingness of physicians to participate in our planned clinical trials. Our ability to enroll patients in our planned clinical trials may be impacted by the COVID-19 pandemic, or any future disease pandemic. If patients or subjects are unwilling or unable to participate in our clinical trials for any reason, the timeline for conducting trials and obtaining regulatory approval of our product candidates may be delayed.

If we experience delays in the completion of, or termination of, any clinical trials of our product candidates, the commercial prospects of our product candidates could be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical trials would likely increase our overall costs, impair product candidate development, and jeopardize our ability to obtain regulatory approval relative to our current plans. Any of these occurrences may harm our business, financial condition, and prospects significantly.

***We may face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use or misuse of our approved products, if any, or product candidates harm patients or subjects, or is perceived to harm patients or subjects even when such harm is unrelated to our approved products, if any, or product candidates, our regulatory approvals, if any, could be revoked or otherwise negatively impacted, and we could be subject to costly and damaging product liability claims. If we are unable to obtain adequate insurance or are required to pay for liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage, a material liability claim could adversely affect our financial condition.***

The use or misuse of our product candidates in clinical trials and the sale of any products for which we may obtain marketing approval exposes us to the risk of potential product liability claims. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. Patients with the diseases targeted by our product candidates may already be in severe and advanced stages of disease and have both known and unknown significant preexisting and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may or may not be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact, or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which an adverse event is unrelated to our product candidates, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may delay our regulatory approval process or impact and limit the type of regulatory approvals our product candidates receive or maintain.

As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition, or results of operations.

Although we have product liability insurance, which covers our historical clinical trials in the United States, for up to \$5.0 million per occurrence, up to an aggregate limit of \$5.0 million, our insurance may be insufficient to reimburse us for any expenses or losses we may suffer. We will also likely be required to increase our product liability insurance coverage for any future clinical trials that we may initiate. If we obtain marketing approval for any of our product candidates, we will need to expand our insurance coverage to include the sale of commercial products. There is no way to know if we will be able to continue to obtain product liability coverage and obtain expanded coverage, if we require it, in sufficient amounts to protect us against losses due to liability, on acceptable terms, or at all. We may not have sufficient resources to pay for any liabilities resulting from a claim excluded from, or beyond the limits of, our insurance coverage. Where we have provided indemnities in favor of third parties under our agreements with them, there is also a risk that these third parties could incur liability and bring a claim under such indemnities. An individual may bring a product liability claim against us alleging that one of our product candidates causes, or is claimed to have caused, an injury or is found to be unsuitable for consumer use. Any such product liability claims may include allegations of defects in manufacturing, defects in design, failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. Any product liability claim brought against us, with or without merit, could result in:

- withdrawal of clinical trial volunteers, investigators, patients or subjects, or trial sites, or limitations on approved indications;

- the inability to commercialize, or if commercialized, decreased demand for, our product candidates;
- if commercialized, product recalls, labeling, marketing or promotional restrictions, or the need for product modification;
- initiation of investigations by regulators;
- loss of revenue;
- substantial costs of litigation, including monetary awards to patients or other claimants;
- liabilities that substantially exceed our product liability insurance, which we would then be required to pay ourselves;
- an increase in our product liability insurance rates or the inability to maintain insurance coverage in the future on acceptable terms, if at all;
- the diversion of management’s attention from our business; and
- damage to our reputation and the reputation of our products and our technology.

Product liability claims may subject us to the foregoing and other risks, which could have a material adverse effect on our business, financial condition, or results of operations.

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

*We expect the product candidates we develop will be regulated as biologics, and therefore they may be subject to competition sooner than anticipated.*

The Biologics Price Competition and Innovation Act of 2009 (“BPCIA”) was enacted as part of the Affordable Care Act to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as “interchangeable” based on its similarity to an approved biologic. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the reference product was approved under a Biologics License Application (“BLA”). The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when processes intended to implement BPCIA may be fully adopted by the FDA, any of these processes could have a material adverse effect on the future commercial prospects for our biological products.

We believe that any of the product candidates we develop that is approved in the United States as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject product candidates to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

In addition, the approval of a biologic product biosimilar to one of our product candidates could have a material adverse impact on our business as it may be significantly less costly to bring to market and may be priced significantly lower than our product candidates.

*We are seeking Orphan drug designation for VRDN-001 from the FDA and may seek Orphan drug designation for future product candidates, but we might not receive such designation.*

In September 2022, we filed an amended application for Orphan drug designation for VRDN-001 based on clinical data. In response, the FDA has indicated that further review of the application is suspended pending receipt of additional information. There is no guarantee that upon submission of this additional data information the FDA will grant us Orphan drug designation. See “Business—Government Regulation and Product Approvals—Orphan Drug Designation.”

***We may seek Breakthrough Therapy designation for one or more of our product candidates from the FDA, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.***

We may seek a breakthrough therapy designation from the FDA for some of our product candidates. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one of our product candidates is designated as a breakthrough therapy, the FDA may later decide that the product candidate no longer meets the conditions for designation and the designation may be rescinded. See “Business—Government Regulation and Product Approvals—Expedited Development and Review Programs.”

***We may seek Fast Track designation for one or more of our product candidates, but we might not receive such designation, and even if we do, such designation may not actually lead to a faster development or regulatory review or approval process.***

If a product candidate is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA Fast Track designation. If we seek Fast Track designation for a product candidate, we may not receive it from the FDA. However, even if we receive Fast Track designation, Fast Track designation does not ensure that we will receive marketing approval in any particular timeframe or at all. We may not experience a faster development or regulatory review or approval process with Fast Track designation compared to conventional FDA procedures. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast Track designation alone does not guarantee qualification for the FDA’s priority review procedures. See “Business—Government Regulation and Product Approvals—Expedited Development and Review Programs.”

***We may attempt to obtain accelerated approval of our product candidates. If we are unable to obtain accelerated approval, we may be required to conduct clinical trials beyond those that we contemplate, or the size and duration of our pivotal clinical trials could be greater than currently planned, which could increase the expense of obtaining, reduce the likelihood of obtaining, and/or delay the timing of obtaining necessary marketing approvals. Even if we receive accelerated approval from the FDA, the FDA may require that we conduct confirmatory trials to verify clinical benefit. If our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-approval requirements, the FDA may seek to withdraw accelerated approval.***

We may seek accelerated approval for our product candidates. The FDA may grant accelerated approval to a product designed to treat a serious or life-threatening condition that provides meaningful therapeutic advantage over available therapies and demonstrates an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease. If granted, accelerated approval may be contingent on the sponsor’s agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug’s predicted effect on irreversible morbidity or mortality or other clinical benefit. The FDA may require that any such confirmatory study be initiated or substantially underway prior to the submission of an application for accelerated approval. If such post-approval studies fail to confirm the drug’s clinical benefits relative to its risks, the FDA may withdraw its approval of the drug. If we choose to pursue accelerated approval, there can be no assurance that the FDA will agree that our proposed primary endpoint is an appropriate surrogate endpoint. Similarly, there can be no assurance that after subsequent FDA feedback that we will continue to pursue accelerated approval or any other form of expedited development, review, or approval, even if we initially decide to do so. Furthermore, if we submit an application for accelerated approval, there can be no assurance that such application will be accepted or that approval will be granted on a timely basis, or at all. The FDA also could require us to conduct further studies or trials prior to considering our application or granting approval of any type. We might not be able to fulfill the FDA’s requirements in a timely manner, which would cause delays, or approval might not be granted because our submission is deemed incomplete by the FDA.

Even if we receive accelerated approval from the FDA, we will be subject to rigorous post-approval requirements, including submission to the FDA of all promotional materials prior to their dissemination. The FDA may require us to conduct a confirmatory study to verify the predicted clinical benefit. The FDA could withdraw accelerated approval for multiple reasons, including our failure to conduct any required post-approval study with due diligence, or the inability of such study to confirm the predicted clinical benefit. A failure to obtain accelerated approval or any other form of expedited review or approval for a



product candidate could result in a longer time period prior to commercializing such product candidate, increase the cost of development of such product candidate, and harm our competitive position in the marketplace.

***Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory requirements.***

If any of our product candidates are approved, we will be subject to ongoing regulatory requirements with respect to manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing clinical trials, and submission of safety, efficacy, and other post-approval information, including both federal and state requirements in the United States, and requirements of comparable foreign regulatory authorities. See “Business—Government Regulation and Product Approvals—Expedited Development and Review Programs.”

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the marketed product. We will be required to report adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation could result in delays in product development or commercialization, or increased costs to assure compliance. If our original marketing approval for a product candidate was granted accelerated approval by the FDA, we could be required to conduct a successful post-marketing clinical trial in order to confirm the clinical benefit of our products. An unsuccessful post-marketing clinical trial or failure to complete such a trial could result in the withdrawal of marketing approval. Any government investigation of alleged violations of law would be expected to require us to expend significant time and resources in response and could generate adverse publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to develop and commercialize our products, and the value of the company and our operating results would be adversely affected. In addition, if we were able to obtain accelerated approval of any of our drug candidates, the FDA may require us to conduct a confirmatory study to verify the predicted clinical benefit. Other regulatory authorities outside of the United States may have similar requirements. The results from the confirmatory study may not support the clinical benefit, which could result in the approval being withdrawn. While operating under accelerated approval, we will be subject to certain restrictions that we would not be subject to upon receiving regular approval.

***Healthcare legislative reform measures may have a material adverse effect on our business, financial condition, or results of operations, and current and future legislation may increase the difficulty and cost for us, and any collaborators, to obtain marketing approval of and commercialize our drug candidates and affect the prices we, or they, may obtain.***

In the United States, there have been and continues to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Affordable Care Act was passed, which was intended to substantially change the way healthcare is financed by both governmental and private insurers, and significantly impact the U.S. pharmaceutical industry. More recently, on August 16, 2022, President Biden signed into law the Inflation Reduction Act of 2022 (“IRA”), which, among other provisions, included several measures intended to lower the cost of prescription drugs and related healthcare reforms. See “Business—Health Reform.”

Heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products has resulted in several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. We expect that additional state and federal healthcare reform measures will be adopted in the future, particularly in light of the new presidential administration, any of which could limit the amounts that federal and state governments will pay for healthcare therapies, which could result in reduced demand for our product candidates or additional pricing pressures. We cannot be sure whether additional legislation or rulemaking related to the IRA will be issued or enacted, or what impact, if any, such changes will have on the profitability of any of our drug candidates, if approved for commercial use, in the future.

***We may be subject, directly or indirectly, to foreign, federal, and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties, sanctions, or other liability.***

Our operations may be subject to various foreign, federal, and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and Physician Payments Sunshine Act, the EU’s GDPR, and other regulations. These laws may impact, among other things, our relationships with healthcare professionals and our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. See “Business—Other Regulations.”



If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including significant civil, criminal, and administrative penalties, disgorgement, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of noncompliance 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.

***If we fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on our business, financial condition, or results of operations.***

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts, and business operations, and cause environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by us and our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources, and state or federal or other applicable authorities may curtail our use of specified materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

***Failure to comply with existing or future laws and regulations related to privacy or data security could lead to government enforcement actions (which could include civil or criminal fines or penalties), private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such laws could increase the costs of our products and services, could limit their use or adoption, and could otherwise negatively affect our operating results and business.***

Regulation of personal data or personal information processing is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of such data. We, our collaborators, and our service providers may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations (e.g., laws and regulations that address data privacy and data security, including, without limitation, health data). These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. These and other requirements could require us or our collaborators to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our collaborators' ability to process or use data in order to support the provision of our products or services, affect our or our collaborators' ability to offer our products and services or operate in certain locations, cause regulators to reject, limit, or disrupt our clinical trial activities, result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or collaborators. See "Business—Other Regulations."

Failure to comply with U.S. and foreign data protection laws and regulations could result in government investigations and enforcement actions (which could include civil or criminal penalties, fines, or sanctions), private litigation, and/or adverse publicity and could negatively affect our operating results and business. Moreover, patients or subjects about whom we or our collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy notices even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Any failure by our third-party collaborators, service providers, contractors, or consultants to comply with applicable law, regulations, or contractual obligations related to data privacy or security could result in proceedings against us by governmental entities or others.

We may publish privacy policies and other documentation regarding our collection, processing, use, and disclosure of personal information and/or other confidential information. Although we endeavor to comply with our published policies and other

documentation, we may at times fail to do so or may be perceived to have failed to do so. Moreover, despite our efforts, we may not be successful in achieving compliance if our employees or vendors fail to comply with our published policies and documentation. Such failures can subject us to potential foreign, local, state, and federal action if they are found to be deceptive, unfair, or misrepresentative of our actual practices. Moreover, subjects about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy notices even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business. Any of these matters could materially adversely affect our business, financial condition, or operational results.

### **Risks Related to Our Reliance on Third Parties**

***We rely on third parties to conduct our preclinical development activities and clinical trials, manufacture our product candidates, and perform other services. If these third parties do not successfully perform and comply with regulatory requirements, we may not be able to successfully complete clinical development, obtain regulatory approval, or commercialize our product candidates and our business could be substantially harmed.***

We have relied upon and plan to continue to rely upon third-party CROs to conduct, monitor, and manage preclinical and clinical programs. We rely on these parties for execution of clinical trials, and we manage and control only some aspects of their activities. We remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with all applicable laws, regulations, and guidelines, including those required by the FDA and comparable foreign regulatory authorities for all of our product candidates in clinical development. If we or any of our CROs or vendors fail to comply with applicable laws, regulations, and guidelines, the results generated in our clinical trials may be deemed unreliable, and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot be assured that our CROs and other vendors will meet these requirements, or that upon inspection by any regulatory authority, such regulatory authority will determine that efforts, including any of our clinical trials, comply with applicable requirements. Our failure to comply with these laws, regulations, and guidelines may require us to repeat clinical trials, which would be costly and delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs in a timely manner or do so on commercially reasonable terms. In addition, our CROs may not prioritize our clinical trials relative to those of other customers, and any turnover in personnel or delays in the allocation of CRO employees by the CRO may negatively affect our clinical trials. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, our clinical trials may be delayed or terminated, and we may not be able to meet our current plans with respect to our product candidates. Additionally, regional disruptions, including natural disasters or health emergencies (such as novel viruses or pandemics), could significantly disrupt the timing of clinical trials. CROs may also involve higher costs than anticipated, which could negatively affect our financial condition and operations.

Shortages and governmental restrictions resulting from the COVID-19 pandemic may disrupt the ability of or increase the cost for our clinical trial sites and other CROs to procure items that are essential for our research and development activities, including animals that are used for preclinical studies. For example, there have been shortages of various animals used in research studies, such as several types of monkeys, which are typically sourced from China, due to the COVID-19 pandemic and disruptions to the global supply chain.

In addition, we do not currently have, nor do we currently plan to establish, the capability to manufacture product candidates for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale without the use of third-party manufacturers. We plan to rely on third-party manufacturers and their responsibilities will include purchasing from third-party suppliers the materials necessary to produce our product candidates for our clinical trials and regulatory approval. There are expected to be a limited number of suppliers for the active ingredients and other materials that we expect to use to manufacture our product candidates, and we may not be able to identify alternative suppliers to prevent a possible disruption of the manufacture of our product candidates for our clinical trials, and, if approved, ultimately for commercial sale. Although we generally do not expect to begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the trial, any significant delay or discontinuity in the supply of a product candidate, or the active ingredient or other material components in the manufacture of the product candidate, could delay completion of our clinical trials and potential timing for regulatory approval of our product candidates, which would harm our business and results of operations.

***Our manufacturing process is complex and we may encounter difficulties in production, which would delay or prevent our ability to provide a sufficient supply of our product candidates for future clinical trials or commercialization, if approved.***

The process of manufacturing our biologic product candidates is complex, highly regulated, variable, and subject to numerous risks. Our manufacturing process is susceptible to product loss or failure, or product variation that may negatively impact patient outcomes, due to logistical issues associated with preparing the product for administration, infusing the patient with the product, manufacturing issues, or different product characteristics resulting from the inherent differences in starting materials, variations between reagent lots, interruptions in the manufacturing process, contamination, equipment or reagent failure, improper installation or operation of equipment and/or programs, vendor or operator error and variability in product characteristics.

Even minor variations in starting reagents and materials, or deviations from normal manufacturing processes could result in reduced production yields, product defects, manufacturing failure, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in any of the manufacturing facilities in which products or other materials are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. Any failure in the foregoing processes could render a batch of product unusable, could affect the regulatory approval of such product candidate, could cause us to incur fines or penalties, or could harm our reputation and that of our product candidates.

We may make changes to our manufacturing process for various reasons, such as to control costs, increase yield or dose, achieve scale, decrease processing time, increase manufacturing success rate, or for other reasons. Changes to our process made during the course of clinical development could require us to show the comparability of the product used in earlier clinical phases or at earlier portions of a trial to the product used in later clinical phases or later portions of the trial. Other changes to our manufacturing process made before or after commercialization could require us to show the comparability of the resulting product to the product candidate used in the clinical trials using earlier processes. Such showings could require us to collect additional nonclinical or clinical data from any modified process prior to obtaining marketing approval for the product candidate produced with such modified process. If such data are not ultimately comparable to that seen in the earlier trials or earlier in the same trial in terms of safety or efficacy, we may be required to make further changes to our process and/or undertake additional clinical testing, either of which could significantly delay the clinical development or commercialization of the associated product candidate, which would materially adversely affect our business, financial condition, results of operations and growth prospects.

***We rely and expect to continue to rely on third parties to manufacture our clinical product supplies, and we intend to rely on third parties to produce and process our product candidates, if approved, and our commercialization of any of our product candidates could be stopped, delayed, or made less profitable if those third parties fail to obtain approval of government regulators, fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.***

We do not currently have, nor do we currently plan to develop, the infrastructure or capability internally to manufacture our clinical supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We currently rely on outside vendors to manufacture our clinical supplies of our product candidates and plan to continue relying on third parties to manufacture our product candidates on a commercial scale, if approved.

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing of our product candidates and our current cost to manufacture our drug products may not be commercially feasible. Additionally, the actual cost to manufacture our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product.

In addition, our reliance on third-party manufacturers exposes us to the following additional risks:

- We may be unable to identify manufacturers of our product candidates on acceptable terms or at all.
- Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- Our future third-party manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products.

- Manufacturers are subject to ongoing periodic unannounced inspection by the FDA and some state agencies to ensure strict compliance with cGMPs and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our product candidates.
- Our third-party manufacturers could breach or terminate their agreement with us.
- We may experience labor disputes or shortages, including from the effects of health emergencies (such as novel viruses or pandemics) and natural disasters.
- Our third-party manufacturers may be impacted by global conflicts, including any potential conflict involving China and Taiwan, and any resulting trade sanctions.

Each of these risks could delay our clinical trials, as well as the approval, if any, of our product candidates by the FDA, or the commercialization of our product candidates, or could result in higher costs, or could deprive us of potential product revenue. In addition, we rely on third parties to perform release testing on our product candidates prior to delivery to patients. If these tests are not appropriately conducted and test data are not reliable, patients could be put at risk of serious harm, and this could result in product liability suits.

The manufacture of medical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of medical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. Furthermore, if contaminants are discovered in our supply of our product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. We cannot be assured that any stability issue or other issues relating to the manufacture of our product candidates will not occur in the future. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes, shortages, including from the effects of health emergencies (such as novel viruses or pandemics) and natural disasters, or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidates to patients or subjects in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

***We may be unable to realize the potential benefits of any collaboration.***

Even if we are successful in entering into additional future collaborations with respect to the development and/or commercialization of one or more product candidates, there is no guarantee that the collaboration will be successful. Collaborations may pose a number of risks, including:

- collaborators often have significant discretion in determining the efforts and resources that they will apply to the collaboration and may not commit sufficient resources to the development, marketing, or commercialization of the product or products that are subject to the collaboration;
- collaborators may not perform their obligations as expected;
- any such collaboration may significantly limit our share of potential future profits from the associated program and may require us to relinquish potentially valuable rights to our current product candidates, potential products, proprietary technologies, or grant licenses on terms that are not favorable to us;
- collaborators may cease to devote resources to the development or commercialization of our product candidates if the collaborators view our product candidates as competitive with their own products or product candidates;

- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation, or the course of development, might cause delays or termination of the development or commercialization of product candidates, and might result in legal proceedings, which would be time consuming, distracting, and expensive;
- collaborators may be impacted by changes in their strategic focus or available funding, or business combinations involving them, which could cause them to divert resources away from the collaboration;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the collaborations may not result in us achieving revenue to justify such transactions; and
- collaborations may be terminated and, if terminated, may result in a need for us to raise additional capital to pursue further development or commercialization of the applicable product candidate.

As a result, a collaboration may not result in the successful development or commercialization of our product candidates.

***We enter into various contracts in the normal course of our business in which we indemnify the other party to the contract. In the event we have to perform under these indemnification provisions, we could have a material adverse effect on our business, financial condition, and results of operations.***

In the normal course of business, we periodically enter into commercial, service, licensing, consulting, and other agreements that contain indemnification provisions. With respect to our research agreements, we typically indemnify the party and related parties from losses arising from claims relating to the products, processes, or services made, used, sold, or performed pursuant to the agreements for which we have secured licenses, and from claims arising from our or our sublicensees' exercise of rights under the agreement. With respect to future collaboration agreements, we may indemnify our collaborators from any third-party product liability claims that could result from the production, use, or consumption of the product, as well as for alleged infringements of any patent or other intellectual property right by a third party. With respect to consultants, we indemnify them from claims arising from the good faith performance of their services.

Should our obligation under an indemnification provision exceed applicable insurance coverage or if we were denied insurance coverage, our business, financial condition, and results of operations could be adversely affected. Similarly, if we are relying on a collaborator to indemnify us and the collaborator is denied insurance coverage or the indemnification obligation exceeds the applicable insurance coverage, and if the collaborator does not have other assets available to indemnify us, our business, financial condition, and results of operations could be adversely affected.

### **Risks Related to Our Intellectual Property**

***We intend to rely on patent rights, trade secret protections, and confidentiality agreements to protect the intellectual property related to our product candidates and any future product candidates. If we are unable to obtain or maintain exclusivity from the combination of these approaches, we may not be able to compete effectively in our markets.***

We rely or will rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. Our success depends in large part on our and our licensors' ability to obtain regulatory exclusivity and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technologies and product candidates.

We have sought to protect our proprietary position by filing and licensing the rights to patent applications in the United States and abroad related to our technologies and product candidates that are important to our business. This process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unresolved. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may



result in such patents being narrowed, found unenforceable, or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We, independently or together with our licensors, have filed patent applications covering various aspects of our product candidates and their methods of use. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent, or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

If we cannot obtain and maintain effective protection of exclusivity from our regulatory efforts and intellectual property rights, including patent protection or data exclusivity, for our product candidates, we may not be able to compete effectively, and our business and results of operations would be harmed.

***We may not have sufficient patent term protections for our product candidates to effectively protect our business.***

Patents have a limited term. In the United States, the statutory expiration of a patent is generally 20 years after it is filed. Additional patent terms may be available through a patent term adjustment (“PTA”) process, resulting from the United States Patent and Trademark Office (“USPTO”) delays during prosecution. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product candidate, we may be open to competition from generic medications.

Patent term extensions (“PTEs”) under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent or data exclusivity terms of our product candidates. We will likely rely on patent term extensions, and we cannot provide any assurances that any such patent term extensions will be obtained and, if so, for how long. As a result, we may not be able to maintain exclusivity for our product candidates for an extended period after regulatory approval, if any, which would negatively impact our business, financial condition, results of operations, and prospects. If we do not have sufficient patent terms or regulatory exclusivity to protect our product candidates, our business and results of operations will be adversely affected.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products, and recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.***

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore costly, time-consuming, and inherently uncertain. In addition, in 2011 the United States enacted the Leahy-Smith America Invents Act (the “Leahy-Smith Act”) and is still currently implementing wide-ranging patent reform legislation. Recent rulings from the U.S. Supreme Court and the Court of Appeals for the Federal Circuit have narrowed the scope of patent protection available in specified circumstances and weakened the rights of patent owners in specified situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

The USPTO has issued subject matter eligibility guidance instructing USPTO examiners on the ramifications of the Supreme Court rulings in *Mayo Collaborative Services v. Prometheus Laboratories, Inc.* and *Association for Molecular Pathology v. Myriad Genetics, Inc.*, and applied the Myriad ruling to natural products and principles including all naturally occurring molecules. In addition, the USPTO continues to provide updates to its guidance continues to be a developing area. The USPTO guidance may make it impossible for us to obtain similar patent claims in future patent applications. Currently, our patent portfolio contains claims of various types and scope, including methods of medical treatment. The presence of varying types of claims in our patent portfolio significantly reduces, but may not eliminate, our exposure to potential validity challenges.

For our U.S. patent applications, which contain claims entitled to priority after March 16, 2013, there is a greater level of uncertainty due to the the Leahy-Smith Act mentioned above. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent

litigation. The USPTO has promulgated regulations and developed 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, did not come into effect until March 16, 2013. Accordingly, it is not yet clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, 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, all of which could have a material adverse effect on our business, financial condition, or results of operations.

An important change introduced by the Leahy-Smith Act is that, as of March 16, 2013, the United States transitioned to a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we were the first to either: (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the Leahy-Smith Act are changes that limit where a patentee may file a patent infringement suit and new procedures providing opportunities for third parties to challenge any issued patent in the USPTO. Included in these new procedures is a process known as Inter Partes Review (“IPR”), which has been generally used by many third parties since the enactment of the Leahy-Smith Act to invalidate patents. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Additionally, the rights of review and appeal for IPR decisions is an area of law that is still developing.

***If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our proposed markets.***

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, such as processes for which patents are difficult to enforce other elements of our product candidate discovery and/or development processes that involve proprietary know-how, information, or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality 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. While we have confidence in these individuals, organizations, and systems, agreements or security measures may be breached, and we may not have adequate remedies for such a breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed, or that our trade secrets and other confidential proprietary information will not be disclosed, or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business, financial condition, or results of operations. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

***Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.***

Our commercial success depends in part on our ability to develop, manufacture, market, and sell our product candidates and use our proprietary technology without infringing the patent rights of third parties. Numerous third-party U.S. and non-U.S. issued patents and pending applications exist in the area of our product candidates. From time to time, we may also monitor these patents and patent applications. We may in the future pursue available proceedings in the U.S. and foreign patent offices to challenge the validity of these patents and patent applications. In addition, or alternatively, we may consider whether to seek to negotiate a license of rights to technology covered by one or more of such third-party patents and patent applications. If any

patents or patent applications cover our product candidates or technologies, we may not be free to manufacture or market our product candidates as planned, absent such a license, which may not be available to us on commercially reasonable terms, or at all.

It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 remain confidential until patents issue, and applications filed after that date that will not be filed outside the United States can elect to remain confidential until patents issue.

Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates and technologies because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale, or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable, or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to specified limitations, be later amended in a manner that could cover our technologies, our product candidates, or the use of our product candidates.

There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits in federal courts, and interferences, oppositions, inter partes reviews, post-grant reviews, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign-issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products, or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

***We may not be successful in meeting our obligations under our existing license agreements necessary to maintain our product candidate licenses in effect. In addition, if required in order to commercialize our product candidates, we may be unsuccessful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.***

We currently have rights to the intellectual property, through licenses from third parties and under patents that we do not own, to develop and commercialize our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to maintain in effect these proprietary rights. Any termination of license agreements with third parties with respect to our product candidates would be expected to negatively impact our business prospects.

We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license their patent rights to us. Even if we are able to license or acquire third-party intellectual property rights that are necessary for our product candidates, there can be no assurance that they will be available on favorable terms.

If we are unable to successfully obtain and maintain rights to required third-party intellectual property, we may have to abandon development of that product candidate or pay additional amounts to the third party, and our business and financial condition could suffer.

***The patent protection and patent prosecution for some of our product candidates are dependent on third parties.***

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors. If any of our licensors fail to appropriately follow our instructions with regard to the prosecution and maintenance of patent protection for patents covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected, and we may not be able to prevent competitors from making, using, importing, and selling competing products. In addition, even where we now have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors in effect from actions prior to us assuming control over patent prosecution.

***If we fail to comply with obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

We are a party to intellectual property licenses and supply agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing agreements impose, and we expect that future license agreements will impose, various diligence, milestone payments, royalties, purchasing, and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, our agreements may be subject to termination by the licensor, in which event we would not be able to develop, manufacture, or market products covered by the license or subject to supply commitments.

***We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.***

Competitors may infringe our patents or the patents of our licensors. If we, or one of our licensing partners, were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description, clarity, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld material information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to us from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

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 could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

***We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we have written agreements and make every effort to ensure that our employees, consultants, and independent contractors do not use the proprietary information or intellectual property rights of others in their work for us, we may in the future be subject to any claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.



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

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop our own products and may also export infringing products to territories where we have patent protection, 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 some countries, particularly some developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology and therapeutic products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, 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 and 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 rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

#### **Risks Related to Commercialization of Our Product Candidates**

***If we are unable to establish commercial manufacturing, sales and marketing capabilities or enter into agreements with third parties to commercially manufacture, market and sell our product candidates, we may be unable to generate any revenue.***

Although some of our employees may have been employed at companies that have launched pharmaceutical products in the past, we have no experience establishing commercial manufacturing relationships for or selling and marketing our product candidates and we currently have no commercial manufacturing relationships or marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to find one or more collaborators to commercialize our products or invest in and develop these capabilities, either on our own or with others, which would be expensive, difficult, and time consuming. Any failure or delay in entering into agreements with third parties to market or sell our product candidates or in the timely development of our internal commercialization capabilities could adversely impact the potential for the launch and success of our products.

If commercialization collaborators do not commit sufficient resources to commercialize our future products and we are unable to develop the necessary marketing and sales capabilities on our own, we will be unable to generate sufficient product revenue to sustain or grow our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations, particularly in the markets our product candidates are intended to address. Without appropriate capabilities, whether directly or through third-party collaborators, we may be unable to compete successfully against these more established companies.

***We may attempt to form collaborations in the future with respect to our product candidates, but we may not be able to do so, which may cause us to alter our development and commercialization plans.***

We may attempt to form strategic collaborations, create joint ventures, or enter into licensing arrangements with third parties with respect to our programs that we believe will complement or augment our existing business. We may face significant competition in seeking appropriate strategic collaborators, and the negotiation process to secure appropriate terms is time consuming and complex. We may not be successful in our efforts to establish such a strategic collaboration for any product candidates and programs on terms that are acceptable to us, or at all. This may be because our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort, our research and development pipeline may be viewed as insufficient, the competitive or intellectual property landscape may be viewed as too intense or risky, and/or third parties may not view our product candidates and programs as having sufficient potential for commercialization, including the likelihood of an adequate safety and efficacy profile.



Even if we are able to successfully enter into a collaboration regarding the development or commercialization of our product candidates, we cannot guarantee that such a collaboration will be successful. Any delays in identifying suitable collaborators and entering into agreements to develop and/or commercialize our product candidates could delay the development or commercialization of our product candidates, which may reduce their competitiveness even if they reach the market. Absent a strategic collaborator, we would need to undertake development and/or commercialization activities at our own expense. If we elect to fund and undertake development and/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 are unable to do so, we may not be able to develop our product candidates or bring them to market and our business may be materially and adversely affected.

***We face substantial competition and our competitors may discover, develop, or commercialize products faster or more successfully than us.***

The development and commercialization of new drug products is highly competitive. We face competition from major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, universities, and other research institutions worldwide with respect to our product candidates that we may seek to develop or commercialize in the future. We are aware that the following companies have therapeutics marketed or in development for TED: Horizon Therapeutics plc and Immunovant, Inc., Harbour BioMed, Valenza Bio, Inc. and Sling Therapeutics, Inc. If approved, VRDN-001 will also compete against generic medications, such as corticosteroids, that are prescribed for and surgical procedures for the treatment of TED.

Our competitors may succeed in developing, acquiring, or licensing technologies and drug products that are more effective or less costly than our product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive. Our competitors may also adopt a similar licensing and development strategy as ours with regard to the development of an existing anti-IGF-1R monoclonal antibody for the treatment of TED. If any competitor was able to effect this strategy in a more efficient manner, there may be less demand for our product candidates if any are approved.

Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Third-party payors, including governmental and private insurers, may also encourage the use of generic products. For example, if VRDN-001 is approved, it may be priced at a significant premium over other competitive products. This may make it difficult for VRDN-001 or any other future products to compete with these products.

If our competitors obtain marketing approval from the FDA or comparable foreign regulatory authorities for their product candidates more rapidly than us, it could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have materially greater name recognition and financial, manufacturing, marketing, research, and drug development resources than we do. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. For example, in December 2022, Amgen Inc. announced that it intends to acquire Horizon Therapeutics plc, which could have a significant impact on the competitive landscape for therapeutics for TED. Large pharmaceutical companies in particular have extensive expertise in preclinical and clinical testing and in obtaining regulatory approvals for drugs. In addition, academic institutions, government agencies, and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies. These organizations may also establish exclusive collaborative or licensing relationships with our competitors. Failure of VRDN-001, VRDN-002 or our other product candidates to effectively compete against established treatment options or in the future with new products currently in development would harm our business, financial condition, results of operations, and prospects.

The commercial success of any of our current or future product candidates will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our products will depend in part on the healthcare providers, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, and third-party payors. The degree of market acceptance of any of our products will depend on a number of factors, including but not limited to:

- the efficacy of the product as demonstrated in clinical trials and potential advantages over competing treatments;

- the prevalence and severity of the disease and any side effects;
- the clinical indications for which approval is granted, including any limitations or warnings contained in a product's approved labeling;
- the convenience and ease of administration;
- the cost of treatment;
- the willingness of the patients and physicians to accept these therapies;
- the perceived ratio of risk and benefit of these therapies by physicians and the willingness of physicians to recommend these therapies to patients based on such risks and benefits;
- the marketing, sales, and distribution support for the product;
- the publicity concerning our products or competing products and treatments; and
- the pricing and availability of third-party payor coverage and adequate reimbursement.

Even if a product displays a favorable efficacy and safety profile upon approval, market acceptance of the product remains uncertain. Efforts to educate the medical community and third-party payors on the benefits of the products may require significant investment and resources and may never be successful. If our products fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and other healthcare providers, we will not be able to generate sufficient revenue to become or remain profitable.

***We may not be successful in any efforts to identify, license, discover, develop, or commercialize additional product candidates.***

Although a substantial amount of our effort will focus on clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business is also expected to depend in part upon our ability to identify, license, discover, develop, or commercialize additional product candidates. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development and commercialization for a number of reasons, including but not limited to the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
- we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in preclinical or clinical testing;
- our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business, financial condition, or results of operations and could potentially cause us to cease operations.

***Failure to obtain or maintain adequate reimbursement or insurance coverage for our products, if any, could limit our ability to market those products and decrease our ability to generate revenue.***

The pricing, as well as the coverage, and reimbursement of our approved products, if any, must be sufficient to support our commercial efforts and other development programs, and the availability of coverage and adequacy of reimbursement by third-party payors, including government healthcare programs, health maintenance organizations, private insurers, and other healthcare management organizations, are essential for most patients to be able to afford expensive treatments. Sales of our approved products, if any, will depend substantially, both domestically and abroad, on the extent to which the costs of our approved products, if any, will be paid for or reimbursed by third-party payors. If coverage and reimbursement are not available, or are available only in limited amounts, we may have to subsidize or provide products for free, or we may not be able to successfully commercialize our products. See “Business—Coverage and Reimbursement.”

Outside the United States, international operations are generally subject to extensive governmental price controls and other price-restrictive regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of products. In many countries, the prices of products are subject to varying price control mechanisms as part of national health systems. Price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our products, if any. Accordingly, in markets outside the United States, the potential revenue may be insufficient to generate commercially reasonable revenue and profits.

We expect to experience pricing pressures in connection with products due to the increasing trend toward managed healthcare, including the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs, has increased and is expected to continue to increase in the future. As a result, profitability of our products, if any, may be more difficult to achieve even if they receive regulatory approval.

## **Risks Related to Our Business Operations**

***Our future success depends in part on our ability to attract, retain, and motivate qualified personnel.***

Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, are critical to our success. There is currently a shortage of highly qualified personnel in our industry, which is likely to continue. As a result, competition for personnel is intense, and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in development and commercialization of our product candidates may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel may impede the progress of our research, development, and commercialization objectives and would negatively impact our ability to succeed in our product development strategy.

***We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.***

As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of our attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

***Unstable market and economic conditions, inflation, increases in interest rates, natural disasters, public health crises such as the COVID-19 pandemic, political crises, geopolitical events, such as the crisis in Ukraine, or other macroeconomic conditions, may have serious adverse consequences on our business and financial condition.***

The global economy, including credit and financial markets, have experienced extreme volatility and disruptions at various points over the last few decades, including, among other things, diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates, and uncertainty about economic stability. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. The Federal Reserve has raised interest rates multiple times in response to concerns about inflation and it may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets, may increase economic uncertainty and affect consumer spending. Similarly, the ongoing military conflict between Russia and Ukraine and the rising tensions between China and Taiwan have created extreme volatility in the global capital markets and may have further global economic consequences, including disruptions of the global supply chain. Any such volatility and disruptions may adversely affect our business or the third parties on whom we rely. If the equity and credit markets deteriorate, including as a result of political unrest or war, it may make any necessary debt or equity financing more difficult to complete, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and share price and could require us to delay or abandon development or commercialization plans. In addition, there is a risk that one or more of our service providers, manufacturers or other partners would not survive or be able to meet their commitments to us under such circumstances, which could directly affect our ability to attain our operating goals on schedule and on budget.

We have experienced and may in the future experience disruptions as a result of such macroeconomic conditions, including delays or difficulties in initiating or expanding clinical trials and manufacturing sufficient quantities of materials. Any one or a combination of these events could have a material and adverse effect on our results of operations and financial condition.

***The Hercules Loan and Security Agreement contains certain covenants that could adversely affect our operations and, if an event of default were to occur, we could be forced to repay any outstanding indebtedness sooner than planned and possibly at a time when we do not have sufficient capital to meet this obligation.***

Pursuant to the Hercules Loan and Security Agreement, we have pledged substantially all of our assets, other than our intellectual property rights. Additionally, the Hercules Loan and Security Agreement contains certain affirmative and negative covenants that could prevent us from taking certain actions without the consent of our lenders. These covenants may limit our flexibility in operating our business and our ability to take actions that might be advantageous to us and our stockholders. The Hercules Loan and Security Agreement also contains customary affirmative and negative covenants that, among other things, limit our ability, subject to certain exceptions, to incur indebtedness, grant liens, enter into a merger or consolidation, enter into transactions with affiliates, or sell all or a portion of our property, business or assets. The Hercules Loan and Security Agreement contains customary events of default. Upon the occurrence and continuation of an event of default, all amounts due under the Hercules Loan and Security Agreement become (in the case of an insolvency or bankruptcy event), or may become (in the case of all other events of default and at the option of Hercules), immediately due and payable. If an event of default under the Hercules Loan and Security Agreement should occur, we could be required to immediately repay any outstanding indebtedness. If we are unable to repay such debt, the lenders would be able to foreclose on the secured collateral, including our cash accounts, and take other remedies permitted under the Hercules Loan and Security Agreement. Even if we are able to repay any indebtedness on an event of default, the repayment of these sums may significantly reduce our working capital and impair our ability to operate as planned.

***Failure in our information technology and storage systems or those of third parties upon whom we rely could significantly disrupt the operation of our business and adversely impact our financial condition.***

Our ability to execute our business plan and maintain operations depends on the continued and uninterrupted performance of our information technology (“IT”) systems or those of third parties upon whom we rely. IT systems are vulnerable to risks and damages from a variety of sources, including telecommunications or network failures, malicious human acts, and natural disasters (such as a tornado, an earthquake, or a fire). Moreover, despite network security and back-up measures, some of our and our vendors’ servers are potentially vulnerable to physical or electronic break-ins, including cyber-attacks, computer viruses, and similar disruptive problems. The techniques used by criminal elements to attack computer systems are sophisticated, change frequently, and may originate from less regulated and remote areas of the world. As a result, we may not be able to address these techniques proactively or implement adequate preventative measures. If the IT systems are compromised, we could be subject to fines, damages, litigation, and enforcement actions, and we could lose trade secrets, the occurrence of which could harm our business. Despite precautionary measures designed to prevent unanticipated problems that could affect the IT systems, sustained or repeated system failures that interrupt our ability to generate and maintain data could adversely affect our ability to operate our business. In addition, the failure of our systems, maintenance problems, upgrading or transitioning to new platforms, or a breach in security could result in delays and reduce efficiency in our operations. Remediation of such problems could result in significant, unplanned capital investments.

Furthermore, parties in our supply chain may be operating from single sites, increasing their vulnerability to natural disasters or other sudden, unforeseen, and severe adverse events. If such an event were to affect our supply chain, it could have a material adverse effect on our business.

***A network or data security incident may allow unauthorized access to our network or data, which could result in a material disruption of our clinical trials, harm our reputation, harm our business, create additional liability and adversely impact our financial results or operational results.***

Increasingly, we are subject to a wide variety of threats on our information networks, and systems and those of our service providers or collaborators. In addition to threats from natural disasters, telecommunications and electrical failures, traditional computer hackers, malicious code (such as malware, viruses, worms, and ransomware), employee error, theft or misuse, password spraying, phishing, and distributed denial-of-service (“DDOS”) attacks, we now also face threats from sophisticated nation-state and nation-state supported actors who engage in attacks (including advanced persistent threat intrusions) that add to the risks to our internal networks, our third-party service providers, our collaborators and the information that they store and process. Despite significant efforts to create security barriers to safeguard against such threats, it is virtually impossible for us to entirely mitigate these risks. The security measures we have integrated into our internal networks and systems, which are designed to detect unauthorized activity and prevent or minimize security incidents or breaches, may not function as expected or may not be sufficient to protect our internal networks and platform against certain threats. In addition, techniques used to obtain unauthorized access to networks in which data is stored or through which data is transmitted change frequently and generally are not recognized until launched against a target. As a result, we may be unable to anticipate these techniques or implement adequate preventative measures to prevent an electronic intrusion.

In addition, security incidents or breaches or those of our current or future collaborators or third-party service providers could result in a risk of loss or unauthorized access to or disclosure of the information we process. This, in turn, could require notification under applicable data privacy regulations or contracts, and could lead to litigation, governmental audits, investigations, fines, penalties, and other possible liability, damage our relationships with our collaborators, trigger indemnification and other contractual obligations, cause us to incur investigation, mitigation and remediation expenses, and have a negative impact on our ability to conduct clinical trials. For example, the loss of clinical trial data for our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

We may not have adequate insurance coverage for security incidents or breaches or information system failures. The successful assertion of one or more large claims against us that exceeds our available insurance coverage or results in changes to our insurance policies (including premium increases or the imposition of large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that any existing insurance coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

Any failure or perceived failure by us or any collaborators, service providers, or others to comply with our privacy, confidentiality, data security or similar obligations to third parties, or any data security incidents or other security breaches that result in the unauthorized access, acquisition, or disclosure of sensitive information (including, without limitation personally identifiable information), may result in governmental investigations, enforcement actions, regulatory fines, litigation, or public statements against us, could cause third parties to lose trust in us or result in claims against us. Any of these events could cause harm to our reputation, business, financial condition, or operational results.

***Our ability to use net operating loss carryforwards and certain other tax attributes to offset future taxable income or taxes may be limited.***

Our net operating loss (“NOL”) carryforwards could expire unused and be unavailable to offset future income tax liabilities because of their limited duration or because of restrictions under U.S. tax law. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act, our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs generated in tax years beginning after December 31, 2017 is limited. It is uncertain if and to what extent various states will conform to the Tax Act.

In addition, under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the “Code”), and corresponding provisions of state law, if a corporation undergoes an “ownership change,” which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOL carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. Our most recent



analysis of possible ownership changes was completed for certain tax periods ending through December 31, 2022. It is possible that we have in the past undergone and may in the future undergo, additional ownership changes that could result in additional limitations on our NOL and tax credit carryforwards. In addition, at the state level, there may be periods during which the use of net operating losses is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our NOL carryforwards and certain other tax attributes, which could have a material adverse effect on cash flow and results of operations.

***Changes in tax laws or regulations that are applied adversely to us or our customers may have a material adverse effect on our business, cash flow, financial condition, or results of operations.***

New income, sales, use, or other tax laws, statutes, rules, regulations, or ordinances could be enacted at any time, which could adversely affect our business operations and financial performance. Further, existing tax laws, statutes, rules, regulations, or ordinances could be interpreted, changed, modified, or applied adversely to us. For example, the Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. In addition, it is uncertain if and to what extent various states will conform to the Tax Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

***Our effective tax rate may fluctuate, and we may incur obligations in tax jurisdictions in excess of accrued amounts.***

We are subject to taxation in numerous U.S. states and territories and non-U.S. jurisdictions. As a result, our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate. In preparing our financial statements, we estimate the amount of tax that will become payable in each of such places. Nevertheless, our effective tax rate may be different than experienced in the past due to numerous factors including the results of examinations and audits of our tax filings, our inability to secure or sustain acceptable agreements with tax authorities, changes in accounting for income taxes, and changes in tax laws. Any of these factors could cause us to experience an effective tax rate significantly different from previous periods or our current expectations and may result in tax obligations in excess of amounts accrued in our financial statements.

## **Risks Related to Ownership of our Common Stock**

***Anti-takeover provisions in our charter documents and under Delaware law and the terms of some of our contracts could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our management.***

Provisions in our Certificate of Incorporation and Bylaws may delay or prevent an acquisition or a change in management. These provisions include a prohibition on actions by written consent of our stockholders and the ability of our board of directors to issue Preferred Stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which prohibits stockholders owning in excess of 15% of our outstanding voting stock from merging or combining with us. Although we believe these provisions collectively will provide for an opportunity to receive higher bids by requiring potential acquirers to negotiate with our board of directors, they would apply even if the offer may be considered beneficial by some stockholders. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove then current management by making it more difficult for stockholders to replace members of the board of directors, which is responsible for appointing the members of management.

In addition, the Certificate of Designation of our Series A Preferred Stock and the provisions of our warrants issued in 2020 may delay or prevent a change in control of our company. At any time while at least 30% of the originally issued Series A Preferred Stock remains issued and outstanding, we may not consummate a Fundamental Transaction (as defined in the Certificate of Designation of the Series A Preferred Stock) or any merger or consolidation of the Company with or into another entity or any stock sale to, or other business combination in which the stockholders of the Company immediately before such transaction do not hold at least a majority of the capital stock of the Company immediately after such transaction, without the affirmative vote of the holders of a majority of the then outstanding shares of the Series A Preferred Stock. As of December 31, 2022, a majority of the then outstanding shares of Series A Preferred Stock was held by entities affiliated with one stockholder. This provision of the Certificate of Designation may make it more difficult for us to enter into any of the aforementioned transactions. In addition, pursuant to such warrants, under certain circumstances each warrant holder has the right to demand that we redeem the warrant for a cash amount equal to the Black-Scholes value of a portion of the warrant upon the occurrence of specified events, including a merger, an asset sale or certain other change of control transactions. A takeover of us may trigger the requirement

that we redeem the warrants, which could make it more costly for a potential acquirer to engage in a business combination transaction with us.

***Our Bylaws provide that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or other employees.***

Our Bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a breach of fiduciary duty owed by any of our directors, officers, or other employees to us or our stockholders, any action asserting a claim against us arising pursuant to any provisions of the Delaware General Corporation Law, our certificate of incorporation or our bylaws, or any action asserting a claim against us that is governed by the internal affairs doctrine. While these choice of forum provisions do not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended (the "Securities Act"), the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction, the choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage such lawsuits against our and our directors, officers, and other employees. If a court were to find the choice of forum provision contained in the bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

***We do not anticipate that we will pay any cash dividends in the foreseeable future.***

The current expectation is that we will retain our future earnings, if any, to fund the development and growth of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future.

***Future sales of shares by existing stockholders could cause our stock price to decline.***

If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale lapse, the trading price of our common stock could decline. In addition, shares of our common stock that are subject to our outstanding options will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements and Rules 144 and 701 under the Securities Act.

***Future sales and issuances of equity and debt could result in additional dilution to our stockholders.***

We expect that we will need significant additional capital to fund our current and future operations, including to complete potential clinical trials for our product candidates. To raise capital, we may sell common stock, convertible securities, or other equity securities in one or more transactions at prices and in a manner we determine from time to time. As a result, our stockholders may experience additional dilution, which could cause our stock price to fall.

In addition, pursuant to our Equity Incentive Plans, we may grant equity awards and issue additional shares of our common stock to our employees, directors, and consultants, and the number of shares of our common stock reserved for future issuance under certain of these plans will be subject to automatic annual increases in accordance with the terms of the plans. To the extent that new options are granted and exercised, or we issue additional shares of common stock in the future, our stockholders may experience additional dilution, which could cause our stock price to fall.

***Our principal stockholders own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

Our directors, officers, 5% stockholders, and their affiliates currently beneficially own a substantial portion of our outstanding voting stock. Therefore, these stockholders have the ability and may continue to have the ability to influence us through this ownership position. These stockholders may be able to determine some or all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

## **General Risk Factors**

***The market price of our common stock has historically been volatile, and the market price of our common stock may drop in the future.***

The market price of our common stock has been, and may continue to be, subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology, and other life sciences companies have historically been particularly volatile. In addition to the factors described elsewhere in this “Risk Factors,” some of the factors that may cause the market price of our common stock to fluctuate include:

- failure to meet or exceed financial and development projections we may provide to the public and the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures, or capital commitments by us or our competitors;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our business and stock;
- changes in the market valuations of similar companies;
- announcements by commercial partners or competitors of new commercial products, clinical progress or the lack thereof, significant contracts, commercial relationships, or capital commitments;
- the introduction of technological innovations or new therapies that compete with our potential products;
- changes in the structure of health care payment systems; and
- period-to-period fluctuations in our financial results.

Moreover, the capital markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies, including volatility resulting from the COVID-19 pandemic and general global macroeconomic conditions. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company’s securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our profitability and reputation.

***We incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.***

We incur significant legal, accounting, and other expenses associated with public company reporting requirements. We also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the SEC and The Nasdaq Stock Market LLC (“Nasdaq”). These rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly. These rules and regulations may also make it difficult and expensive for us to obtain directors’ and officers’ liability insurance. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence and could cause our business or stock price to suffer.

***If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business, or our market, our stock price and trading volume could decline.***

The trading market for our common stock is influenced by the research and reports that equity research analysts publish about us and our business. Equity research analysts may elect not to provide research coverage of our common stock and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

*If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our common stock may be negatively affected.*

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, and the rules and regulations of Nasdaq. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our annual report filing for that year, as required by Section 404 of the Sarbanes-Oxley Act. This requires that we incur substantial professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. We may experience difficulty in meeting these reporting requirements in a timely manner for each period.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal controls, it could result in a material misstatement of our financial statements that would not be prevented or detected on a timely basis, which could require a restatement, cause us to be subject to sanctions or investigations by Nasdaq, the SEC, or other regulatory authorities, cause investors to lose confidence in our financial information, or cause our stock price to decline.

As a public company, we incur significant legal, accounting, insurance, and other expenses, and our management and other personnel have and will need to continue to devote a substantial amount of time to compliance initiatives resulting from operating as a public company. We also anticipate that these costs and compliance initiatives will continue to increase as a result of ceasing to be an "emerging growth company," as defined in the in the Jumpstart Our Business Startups Act of 2012.

## **ITEM 1B. UNRESOLVED STAFF COMMENTS**

None.

## **ITEM 2. PROPERTIES**

We are party to a multi-year, non-cancelable lease agreement for our office space in Waltham, Massachusetts (the "Original Lease"). In July 2021, we entered into an amendment to the Original Lease to increase our Massachusetts-based office space (the "First Amendment"). In April 2022, we entered into a second amendment to the Original Lease (the "Second Amendment"). The Second Amendment made certain modifications to both the Original Lease and First Amendment, including (i) the addition of 2,432 square feet of office space in the same building (the "April 2022 Expansion Premises"), (ii) the termination of the 1,087 square feet of leased space under the Original Lease seven days after the delivery of the April 2022 Expansion Premises, which occurred in the fourth quarter of 2022, and (iii) the extension of the expiration date of the 3,284 square feet of leased space under the First Amendment from October 2024 through November 2026. Under the Second Amendment, we have the option to extend the lease term for an additional period of three years upon notice to the landlord. In July 2022, we entered into a third amendment to the Original Lease (the "Third Amendment"). The Third Amendment makes certain modifications to the Original Lease, including (i) the addition of 5,240 square feet of office space in the same building (the "July 2022 Expansion Premises"), (ii) the termination of the 1,087 square feet of leased space under the Original Lease, and (iii) the extension of the expiration date of the 3,284 square feet of leased space under the First Amendment to four years from the delivery of the July 2022 Expansion Premises. Under the Third Amendment, we have the option to extend the lease term for an additional period of three years upon notice to the landlord.

Additionally, we lease approximately 27,128 sq. ft. of office and laboratory space in Boulder, Colorado under a lease that expires in December 2024, subject to two three-year renewal options prior to the expiration, and that includes rent escalation clauses through the lease term.

**ITEM 3. LEGAL PROCEEDINGS**

From time to time, we may be involved in legal proceedings in the ordinary course of business. We are currently not a party to any legal proceedings that we believe would have a material adverse effect on our business, financial condition, or results of operations.

**ITEM 4. MINE SAFETY DISCLOSURES**

Not applicable.



## PART II

### ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Our common stock is traded on The Nasdaq Capital Market under the symbol "VRDN."

#### *Holder*s

As of March 3, 2023, we had 20 registered holders of record of our common stock. A substantially greater number of holders of our common stock are in "street name" or beneficial holders, whose shares of record are held by banks, brokers, other financial institutions, and registered clearing agencies.

#### *Dividend Policy*

We historically have not paid, and do not anticipate in the future paying, dividends on our common stock. We currently intend to retain all of our future earnings, as applicable, to finance the growth and development of our business. In addition to legal restrictions under applicable law, we are subject to certain dividend-related limitations under the Hercules Loan and Security Agreement. Subject to these limitations, any future determination as to the payment of cash dividends on our common stock will be at our board of directors' discretion and will depend on our financial condition, operating results, capital requirements, and other factors that our board of directors considers to be relevant.

#### *Unregistered Sales of Equity Securities*

None.

### ITEM 6. RESERVED

### ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*The following discussion and analysis should be read together with our consolidated financial statements and the related notes thereto included elsewhere in this Annual Report. This discussion and other parts of this report contain forward-looking statements reflecting our current expectations that involve risks and uncertainties, such as our plans, objectives, expectations, intentions, and beliefs. See "Forward-Looking Statements" for a discussion of the uncertainties, risks, and assumptions associated with these statements. Actual results and the timing of events could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section entitled "Risk Factors" included elsewhere in this Annual Report.*

#### **Overview**

We are a biopharmaceutical company focused on discovering and developing potential best-in-class medicines for serious and rare diseases. We target under-competitive disease areas where marketed therapies often leave room for improvements in efficacy, safety, and/or dosing convenience. We believe that first-generation medicines rarely represent optimal solutions, especially in rare disease areas, and that there is potential to develop differentiated, best-in-class medicines that could lead to improved patient outcomes, reduced side effects, improved quality of life, expanded market access, and augmented market competition. Our business model is designed to identify and evaluate product opportunities in disease areas where trial data establishes proof-of-concept for a drug target in the clinic, but the competitive evolution of the product life cycle management and number of entrants appears incomplete. We intend to prioritize indications where a fast-follower and a potentially differentiated drug candidate, or overall product profile, could create significant medical benefit for patients. We are engineering medicines to address unmet medical needs for patients and further advance drug innovation.

We are developing three product candidates, VRDN-001, VRDN-002 and VRDN-003, that are being developed for intravenous or subcutaneous administration to treat patients who suffer from thyroid eye disease ("TED"). Our most advanced program, VRDN-001, is a differentiated humanized monoclonal antibody targeting IGF-1R intravenously administered for the treatment of TED.

## **Global Economic Considerations**

The global macroeconomic environment is uncertain, and could be negatively affected by, among other things, increased U.S. trade tariffs and trade disputes with other countries, instability in the global capital and credit markets, supply chain weaknesses, and instability in the geopolitical environment, including as a result of the Russian invasion of Ukraine, the rising tensions between China and Taiwan and other political tensions, and lingering effects of the COVID-19 pandemic. Such challenges have caused, and may continue to cause, recession fears, concerns regarding potential sanctions, rising interest rates, foreign exchange volatility and inflationary pressures. At this time, we are unable to quantify the potential effects of this economic instability on our future operations.

## **Financial Operations Overview**

### ***Revenue***

Our revenue has historically consisted primarily of up-front payments for licenses, milestone payments, and payments for other research and development services earned under license and collaboration agreements as well as for amounts earned under certain grants we have been awarded.

In October 2020, we became party to a license agreement with Zenas BioPharma. Since February 2021, we have entered into several letter agreements with Zenas BioPharma in which we agreed to provide assistance to Zenas BioPharma with certain development activities, including manufacturing (collectively with the license agreement, the “Zenas Agreements”). Under the terms of the Zenas Agreements, we granted Zenas BioPharma an exclusive license to develop, manufacture, and commercialize certain IGF-1R directed antibody products for non-oncology indications in the greater area of China in exchange for upfront non-cash consideration and non-refundable milestone payments upon achieving specific milestone events during the contract term. Zenas BioPharma announced that it had obtained IND approval in China in July 2022. Under the license agreement, we received a \$1.0 million milestone payment from Zenas BioPharma. Additionally, we are eligible to receive royalty payments based on a percentage of the annual net sales of any licensed products sold on a country-by-country basis in the greater area of China. The royalty percentage may vary based on different tiers of annual net sales of the licensed products made. Zenas BioPharma is obligated to make royalty payments to us for the royalty term in the Zenas Agreements. In May 2022, we entered into a Manufacturing Development and Supply Agreement with Zenas BioPharma to manufacture and supply, or have manufactured and supplied, clinical drug product for development purposes.

In the future, we expect to continue to generate revenue from a combination of license fees and other up-front payments, payments for research and development services, milestone payments, product sales, and royalties in connection with strategic alliances. We expect that any revenue we generate could fluctuate from quarter to quarter as a result of the timing of our achievement of development and commercial milestones, the timing and amount of payments relating to such milestones, and the extent to which any of our product candidates are approved and successfully commercialized by us or our strategic alliance collaborators, if any. If we or our strategic alliance collaborators, if any, fail to develop product candidates in a timely manner or to obtain regulatory approval for them, then our ability to generate future revenue, and our results of operations and financial position would be adversely affected.

### ***Research and Development Expenses***

Research and development expenses consist of costs incurred for the research and development of our therapeutic programs and product candidates, which include:

- employee-related expenses, including salaries, severance, retention, benefits, insurance, and share-based compensation expense;
- expenses incurred under agreements with clinical research organizations (“CROs”), investigative sites that conduct our clinical trials, and other clinical trial-related vendors, and consultants;
- the costs of acquiring, developing, and manufacturing and testing clinical and preclinical materials, including costs incurred under agreements with contract manufacturing organizations (“CMOs”);
- costs associated with non-clinical activities and regulatory operations;

- license fees and milestone payments related to the acquisition and retention of certain licensed technology and intellectual property rights; and
- facilities, depreciation, market research, and other expenses, which include allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory supplies.

We make non-refundable advance payments for goods and services that will be used in future research and development activities. These payments are recorded as expense in the period in which we receive or take ownership of the goods or when the services are performed.

We record up-front and milestone payments to acquire and retain contractual rights to in-licensed technology and intellectual property rights as research and development expenses when incurred if there is uncertainty in our receiving future economic benefit from the acquired contractual rights. We consider future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved by the U.S. Food and Drug Administration (“FDA”) or when other significant risk factors are abated.

We expect that our research and development expenses will increase as we expand our clinical development programs and initiate new clinical trials. The process of conducting clinical trials and preclinical studies necessary to obtain regulatory approval is costly and time consuming. We, or our strategic alliance collaborators, if any, may never succeed in achieving marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including clinical data, preclinical data, competition, manufacturability, and commercial viability of our product candidates.

Successful development of future product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each future product candidate and are difficult to predict. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to our ability to maintain or enter into new strategic alliances with respect to each program or potential product candidate, the scientific and clinical success of each future product candidate, and ongoing assessments as to each future product candidate’s commercial potential. For example, upon receipt of topline data from our planned proof of concept trial of VRDN-002 by the end of 2023, we expect to determine which of VRDN-002 or VRDN-003 to advance into a pivotal Phase 3 trial in the first half of 2024. We will need to raise additional capital and may seek additional strategic alliances in the future in order to advance the various clinical trials that are part of our clinical development program described above.

### ***General and Administrative Expenses***

General and administrative expenses consist primarily of salaries and related benefits, including share-based compensation, and severance and retention benefits related to our finance, accounting, human resources, legal, business development, and other support functions, professional fees for auditing, tax, and legal services, as well as insurance, board of director compensation, consulting, and other administrative expenses.

### ***Other Income, net***

Other income, net consists primarily of interest income, net of fees, and various income items of a non-recurring nature. Interest expense consists of cash and non-cash interest expense on our long-term debt. We earn interest income from interest-bearing accounts, money market funds, and short-term investments.

### **Critical Accounting Policies and Estimates**

This discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles (“U.S. GAAP”). The preparation of financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policy discussed below is critical to understanding our historical and future performance, as this policy relates to the more significant areas involving our judgments and estimates.

## ***Clinical Trial and Preclinical Study Accruals***

We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on certain facts and circumstances at that time. Our accrued expenses for preclinical studies and clinical trials are based on estimates of costs incurred for services provided by external service providers and for other trial-related activities. The timing and amount of expenses we incur through our external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, we obtain information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to our research and development expenses may be necessary in future periods as our estimates change.

## **Results of Operations**

### **Comparison of the Years Ended December 31, 2022 and 2021**

	Year Ended December 31,		Increase (Decrease)
	2022	2021	
	(in thousands)		
Revenue	\$ 1,772	\$ 2,963	\$ (1,191)
Research and development expenses	100,894	56,886	44,008
General and administrative expenses	35,182	25,805	9,377
Other income (expense), net	4,430	315	4,115
Net loss	\$ (129,874)	\$ (79,413)	\$ (50,461)

### ***Revenue***

Revenue was \$1.8 million for the year ended December 31, 2022, as compared to \$3.0 million for the year ended December 31, 2021. Revenue for both periods was attributable to our collaboration agreement with Zenas BioPharma. The \$1.2 million decrease in revenue is due to the timing of activities performed under the collaboration agreement. During the year ended December 31, 2022, the Company recognized \$1.0 million upon Zenas BioPharma's achievement of a certain development milestone.

### ***Research and Development Expenses***

Research and development expenses were \$100.9 million during the year ended December 31, 2022, compared to \$56.9 million during the year ended December 31, 2021. The \$44.0 million increase in research and development expenses is primarily attributable to an increase of \$8.9 million in clinical trial expenses related to our lead product candidates, VRDN-001 and VRDN-002; an increase of \$8.7 million in costs associated with our preclinical programs; an increase of \$7.8 million in personnel related costs, including share-based compensation, due to an increase in headcount; an increase of \$7.6 million in milestone, license and option fees due to milestone and upfront payments to ImmunoGen and Paragon Therapeutics, Inc.; an increase of \$6.5 million in chemistry, manufacturing and controls costs; and an increase of \$2.4 million in consulting expenses.

We expect our research and development expenses to increase as we work to progress our clinical and preclinical programs.

### ***General and Administrative Expenses***

General and administrative expenses were \$35.2 million during the year ended December 31, 2022, compared to \$25.8 million during the year ended December 31, 2021. The \$9.4 million increase in general and administrative expenses is due primarily to an increase of \$6.0 million in personnel costs, including share based compensation, due to an increase in headcount, as well as an increase of \$3.1 million in consulting, professional and license fees.

### ***Other Income, net***

Other income, net was \$4.4 million during the year ended December 31, 2022 compared to \$0.3 million during the year ended December 31, 2021. Other income, net for the year ended December 31, 2022, is comprised of \$4.9 million of interest income earned on short-term investments as well as sub-lease income, offset by \$0.5 million in interest expense related to our Hercules

Loan and Security Agreement. Other income, net for the year ended December 31, 2021, is comprised of interest income earned on short-term investments as well as sub-lease income.

## **Liquidity and Capital Resources**

We have funded our operations to date principally through proceeds received from the sale of our common stock, our Series A Preferred Stock, our Series B Preferred Stock and other equity securities, debt financings, license fees, and reimbursements received under collaboration agreements. As of December 31, 2022, we had \$424.6 million in cash, cash equivalents, and short-term investments. We expect that our current resources will enable us to fund our planned operations into the second half of 2025.

We have no products approved for commercial sale and have not generated any revenue from product sales. Since our inception and through December 31, 2022, we have generated an accumulated deficit of \$488.2 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We will continue to require substantial additional capital to continue the development of our product candidates, and potential commercialization activities, and to fund our ongoing operations, including our clinical development plan described above. The amount and timing of future funding requirements will depend on many factors, including the pace and results of our clinical development efforts, equity financings, securing additional license and collaboration agreements, and issuing debt or other financing vehicles. Our ability to secure capital is dependent upon a number of factors, including success in developing our technology and product candidates. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop our product candidates. Changing circumstances, such as changes in the scope and timing of our clinical studies, may cause us to consume capital significantly faster or slower than we currently anticipate. If we are unable to acquire additional capital or resources, we will be required to modify our operational plans to complete future milestones. We have based these estimates on assumptions that may prove to be wrong, and we could exhaust our available financial resources sooner than we currently anticipate. We may be forced to reduce our operating expenses and raise additional funds to meet our working capital needs, principally through the additional sales of our securities or debt financings or entering into strategic collaborations.

Our material cash requirements include obligations as of December 31, 2022, as well as resources required to fulfill our research and development activities and the effects that such obligations and activities are expected to have on our liquidity and cash flows in future periods. We expect that our operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of our development activities and efforts to achieve regulatory approval.

If we raise additional funds through the issuance of debt, the obligations related to such debt could be senior to rights of holders of our capital stock and could contain covenants that may restrict our operations. Should additional capital not be available to us in the near term, or not be available on acceptable terms, we may be unable to realize value from our assets and discharge our liabilities in the normal course of business, which may, among other alternatives, cause us to further delay, substantially reduce, or discontinue operational activities to conserve our cash resources.

### ***Loan and Security Agreement with Hercules Capital, Inc.***

On April 1, 2022, we entered into a loan and security agreement (the “Hercules Loan and Security Agreement”) among the Company, certain of our subsidiaries from time to time party thereto (together with the Company, collectively, the “Borrower”), Hercules Capital, Inc. (“Hercules”) and certain other lenders party thereto (the “Lenders”). Under the Hercules Loan and Security Agreement, the Lenders provided us with access to a term loan with an aggregate principal amount of up to \$75.0 million, in four tranches (collectively the “Term Loan”), consisting of: (1) an initial tranche of \$25.0 million, available through June 15, 2023; (2) a second tranche of \$10.0 million, subject to the achievement of certain regulatory milestones, available through June 15, 2023; (3) a third tranche of \$15.0 million, subject to the achievement of certain regulatory milestones, available through March 15, 2024; and (4) a fourth tranche of \$25.0 million, subject to approval by the Lenders’ investment committee(s), available through December 15, 2024. The first tranche of \$25.0 million will be available to us through June 15, 2023. Upon signing we drew an initial principal amount of \$5.0 million.

The Term Loan bears interest at a floating per annum rate equal to the greater of (1) 7.45% and (2) 4.2% above the Prime Rate, provided that the Term Loan interest rate shall not exceed a per annum rate of 8.95%. Interest is payable monthly in arrears on the first day of each month. Per the terms of the Hercules Loan and Security Agreement, we were originally obligated to make interest-only payments through April 1, 2024. However, upon the achievement of a development milestone the interest-only



period was extended to October 1, 2024. If additional development milestones are met, the interest-only period will be extended to April 1, 2025 pursuant to a second extension. We are required to repay the Term Loan amount in equal monthly installments of the principal amount and interest between the end of the interest-only period and the maturity date of October 1, 2026. In addition, we are required to pay an end-of-term fee equal to 6% of the principal amount of funded Term Loan Advances at maturity, which are being accreted as additional interest expense over the term of the loan.

### ***ATM Agreements***

In September 2022, we entered into an Open Market Sale Agreement<sup>SM</sup> (the “September 2022 ATM Agreement”) with Jefferies, LLC (“Jefferies”) pursuant to which we may offer and sell shares of our common stock having an aggregate offering price of up to \$175.0 million from time to time at prices and on terms to be determined by market conditions at the time of offering, with Jefferies acting as the sales agent. Jefferies will receive a commission of 3.0% of the gross proceeds of any shares of common stock sold under the September 2022 ATM Agreement. As of December 31, 2022, 964,357 shares were sold under the September 2022 ATM Agreement at a weighted average price of \$26.01 per share, for aggregate net proceeds of approximately \$24.2 million, including commissions to Jefferies as a sales agent.

In November 2021, we entered into an Open Market Sale Agreement<sup>SM</sup> (the “November 2021 ATM Agreement”) with Jefferies, pursuant to which we could offer and sell shares of our common stock having an aggregate offering price of up to \$75.0 million from time to time at prices and on terms to be determined by market conditions at the time of offering, with Jefferies acting as sales agent. The November 2021 ATM Agreement was terminated in connection with the September 2022 ATM Agreement. No shares were sold under the November 2021 ATM Agreement prior to its termination.

In April 2021, we entered into an Open Market Sale Agreement<sup>SM</sup> (the “April 2021 ATM Agreement”) with Jefferies pursuant to which we could offer and sell shares of our common stock having an aggregate offering price of up to \$50.0 million at prices and on terms to be determined by market conditions at the time of offering, with Jefferies acting as sales agent. Jefferies received a commission equal to 3.0% of the gross sales proceeds of any common stock sold through Jefferies under the April 2021 ATM Agreement. The April 2021 ATM Agreement was terminated in connection with the November 2021 ATM Agreement. An aggregate of 2,551,269 shares of common stock were sold pursuant to the April 2021 ATM Agreement prior to its termination, at a volume weighted-average price of \$13.13 per share, for aggregate net proceeds of approximately \$32.4 million, including commissions to Jefferies as sales agent.

### ***Underwritten Public Offerings***

In August 2022, we entered into an underwriting agreement (the “2022 Underwriting Agreement”) with Jefferies, SVB Securities LLC and Evercore Group LLC relating to the offer and sale (the “2022 Offering”) of 11,352,640 shares of our common stock, which includes 1,725,000 shares of common stock issued in connection with the exercise in full by the underwriters of their option to purchase additional shares at a public offering price of \$23.50 per share, and 28,084 shares of the Company’s Series B Non-Voting Convertible Preferred Stock, at a public offering price of \$1,566.745 per share (collectively, the “2022 Public Offering”). The aggregate gross proceeds to us from the 2022 Public Offering, including the exercise of the option, were approximately \$311.0 million, before deducting underwriting discounts and commissions and other offering expenses payable by us.

In September 2021, we entered into an underwriting agreement (the “2021 Underwriting Agreement”) with Jefferies, SVB Leerink LLC and Evercore Group, LLC for the sale and issuance of 7,344,543 shares of common stock, which include 1,159,089 shares of common stock issued in connection with the exercise in full by the underwriters of their option to purchase additional shares at a public offering price of \$11.00 per share, and 23,126 shares of Series B Non-Voting Convertible Preferred Stock at a public offering price of \$733.37 per share (the “2021 Public Offering”). Our aggregate gross proceeds from the 2021 Public Offering, including the exercise of the option, were approximately \$97.7 million, before deducting underwriting discounts and commissions and estimated offering expenses payable by us.

### ***Purchase Agreements***

In October 2020, we entered into a securities purchase agreement (the “Purchase Agreement”) with the purchasers named therein (the “Investors”). Pursuant to the Purchase Agreement, we agreed to sell an aggregate of approximately 195,290 shares of Series A Preferred Stock for an aggregate purchase price of approximately \$91.0 million. Each share of Series A Preferred Stock is convertible into 66.67 shares of our common stock, subject to specified conditions. The powers, preferences, rights, qualifications, limitations, and restrictions applicable to the Series A Preferred Stock are set forth in the applicable certificate of designations. During the year ended December 31, 2021, 138,050 shares of Series A Preferred Stock were converted into 9,203,732 shares of common stock.

In December 2019, we entered into a common stock purchase agreement (the “Aspire Stock Purchase Agreement”) with Aspire Capital Fund, LLC (“Aspire Capital”), which provides that, subject to the terms, conditions, and limitations set forth therein, Aspire Capital is committed to purchase up to an aggregate of \$20.0 million of shares of our common stock over the 30-month term of the Aspire Stock Purchase Agreement. Upon execution of the Aspire Stock Purchase Agreement, we sold to Aspire Capital 106,564 shares of common stock at \$9.38 per share for proceeds of \$1.0 million as the Initial Purchase Shares (as defined in the Aspire Stock Purchase Agreement). During the year ended December 31, 2020, we sold to Aspire Capital 412,187 shares of our common stock at a weighted-average price of \$21.35 per share for aggregate net proceeds of \$8.8 million. As of December 31, 2022, we have the ability to sell an additional \$10.2 million of shares of our common stock to Aspire Capital.

Summarized cash flows for the year ended December 31, 2022 and 2021 are as follows:

	Year Ended December 31,	
	2022	2021
(in thousands)		
Net cash provided by (used in):		
Operating activities	\$ (93,838)	\$ (54,581)
Investing activities	(115,126)	(74,292)
Financing activities	322,244	125,275
Total	<u>\$ 113,280</u>	<u>\$ (3,598)</u>

### ***Operating Activities***

Net cash used in operating activities was \$93.8 million for the year ended December 31, 2022, and primarily consisted of a net loss of \$129.9 million, adjusted for non-cash items of \$20.1 million (primarily share-based compensation of \$19.8 million) and working capital adjustments of \$16.0 million.

Net cash used in operating activities was \$54.6 million for the year ended December 31, 2021, and primarily consisted of a net loss of \$79.4 million, adjusted for non-cash items of \$23.1 million (primarily share-based compensation of \$14.5 million and a \$7.5 million non-cash charge related to the issuance of common stock as payment for licensing fees to Xencor, Inc.) and working capital adjustments of \$1.7 million.

### ***Investing Activities***

Net cash used in investing activities was \$115.1 million during the year ended December 31, 2022. Net cash used in investing activities in 2022 primarily consisted of net purchases of investments of \$114.3 million and property and equipment purchases of \$0.8 million.

Net cash used in investing activities was \$74.3 million during the year ended December 31, 2021. Net cash used in investing activities in 2021 primarily consisted of net purchases of investments of \$74.0 million and property and equipment purchases of \$0.3 million.

## ***Financing Activities***

Net cash provided by financing activities was \$322.2 million for the year ended December 31, 2022. Net cash provided by financing activities in 2022 was primarily driven by net proceeds of \$313.8 million in net proceeds from the 2022 Public Offering and the September 2022 ATM Agreement, \$4.6 million in net proceeds from the Hercules Loan and Security Agreement, as well as \$2.8 million in proceeds from the exercise of stock options, \$0.9 million in proceeds from the exercise of warrants and \$0.2 million in proceeds from the issuance of common stock under our employee stock purchase plan.

Net cash provided by financing activities was \$125.3 million for the year ended December 31, 2021. Net cash provided by financing activities in 2021 was primarily driven by net proceeds of \$107.3 million from the sale of common stock in 2021 through the 2021 Public Offering and the April 2021 ATM Agreement, \$15.7 million in net proceeds from the sale of our Series B Preferred Stock, \$1.3 million from the exercise of common stock warrants, and \$1.0 million in proceeds from employee stock option exercises.

## **ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

We are a smaller reporting company, as defined by Rule 12b-2 under the Exchange Act and in Item 10(f)(1) of Regulation S-K, and are not required to provide the information under this item.

## **ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA**

The financial statements and supplemental data required by this item are set forth on the pages indicated in Part IV, Item 15(a)(1) of this Annual Report.

## **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**

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file under the Exchange Act, is recorded, processed, summarized, and reported within the time periods specified in the rules and forms of the SEC, and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosures. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Under the supervision and with the participation of our principal executive officer, principal financial officer, and other senior management personnel, we evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Annual Report. Based on this evaluation, our principal executive officer and principal financial officer have concluded that our disclosure controls and procedures were effective as of the end of the period covered by this Annual Report.

### **Management's Report on Internal Control Over Financial Reporting**

Internal control over financial reporting refers to the process designed by, or under the supervision of, our principal officer and principal financial officer, and effected by our 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 U.S. GAAP, and includes those policies and procedures that: (1) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of our assets, (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with U.S. GAAP, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors, and (3) 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.

Internal control over financial reporting cannot provide absolute assurance of achieving financial reporting objectives because of its inherent limitations. Internal control over financial reporting is a process that involves human diligence and compliance and is subject to lapses in judgment and breakdowns resulting from human failures. Internal control over financial reporting also can be circumvented by collusion or improper management override. Because of such limitations, there is a risk that material misstatements may not be prevented or detected on a timely basis by internal control over financial reporting. However, these inherent limitations are known features of the financial reporting process. Therefore, it is possible to design into the process safeguards to reduce, though not eliminate, this risk.

Management is responsible for establishing and maintaining adequate internal control over our financial reporting, as such term is defined in Rule 13a-15(f) under the Exchange Act. Under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting as of the end of the period covered by this Annual Report. Management used the framework set forth in the report entitled “Internal Control — Integrated Framework (2013 Framework)” published by the Committee of Sponsoring Organizations of the Treadway Commission to evaluate the effectiveness of our internal control over financial reporting. Based on its evaluation, management concluded that our internal control over financial reporting was effective at a reasonable level of assurance as of December 31, 2022, the end of our most recent fiscal year.

### **Changes in Internal Control Over Financial Reporting**

There have been no changes in our internal control over financial reporting that occurred during our most recent fiscal quarter that have materially affected, or are reasonably likely to materially effect, our internal control over financial reporting.

### **ITEM 9B. OTHER INFORMATION**

None.

### **ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTION**

Not applicable.

### **ITEM 10. DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE**

The information required by this Item is incorporated by reference to our 2023 Proxy Statement to be filed with the SEC within 120 days after December 31, 2022.

Our board of directors has adopted a written code of business conduct and ethics that applies to our directors, officers, and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on our website, which is located at [www.viridiantherapeutics.com](http://www.viridiantherapeutics.com). If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a Current Report on Form 8-K.

## **PART III**

### **ITEM 11. EXECUTIVE COMPENSATION**

The information required by this Item 11 is incorporated herein by reference to our 2023 Proxy Statement, including under headings “Executive Compensation” and “Directors, Executive Officers and Corporate Governance.”

### **ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS**

The information required by this Item 12 is incorporated herein by reference to our 2023 Proxy Statement, including under headings “Security Ownership of Certain Beneficial Owners and Management” and “Securities Authorized for Issuance Under Equity Compensation Plans.”

### **ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE**

The information required by this Item 13 is incorporated herein by reference to our 2023 Proxy Statement, including under headings “Directors, Executive Officers and Corporate Governance” and “Transactions with Related Persons.”

### **ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES**

The information required by this Item 14 is incorporated herein by reference to our 2023 Proxy Statement, including under the heading “Ratification of Selection of Independent Registered Accounting Firm.”



## PART IV

### ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

#### (a)(1) Financial Statements

The financial statements required by this item are submitted in a separate section beginning on page F-1 of this Annual Report.

#### (a)(2) Financial Statement Schedules

Financial statement schedules have been omitted because they are either not required, not applicable, or the information is otherwise included.

#### (a)(3) Exhibits

See Exhibit Index, which is incorporated herein by reference.

### EXHIBIT INDEX

The exhibits listed in the Exhibit Index are required by Item 601 of Regulation S-K. The SEC file number for all items incorporated by reference herein from reports on Forms 10-K, 10-Q, and 8-K is 001-36483.

Exhibit No.	Description of Exhibit	Incorporated by Reference		
		Form	Filing Date	Number
2.1 <sup>^</sup>	Agreement and Plan of Merger, dated October 27, 2020, by and among the Registrant, Oculus Merger Sub I, Inc., Oculus Merger Sub II, LLC, and Viridian Therapeutics, Inc.	8-K	10/28/2020	2.1
3.1	Second Restated Certificate of Incorporation of the Registrant, effective as of March 9, 2022	10-K	03/11/2022	3.1
3.2	Amended and Restated Bylaws of the Registrant, effective as of May 11, 2022.	10-Q	05/13/2022	3.2
3.3	Certificate of Designation of Series A Non-Voting Convertible Preferred Stock.	8-K	10/28/2020	3.1
3.4	Certificate of Designation of Series B Non-Voting Convertible Preferred Stock.	8-K	09/23/2021	3.1
4.1	Specimen Common Stock Certificate.	S-1	03/19/2014	4.1
4.2	Warrant to Purchase Stock between Miragen Therapeutics, Inc. and Silicon Valley Bank, dated April 30, 2015.	10-K	03/14/2019	4.2
4.3	Warrant to Purchase Stock between Miragen Therapeutics, Inc. and Silicon Valley Bank, dated November 14, 2017.	8-K	11/15/2017	10.2
4.4	Form of Warrant to Purchase Common Stock.	8-K	02/07/2020	4.1
4.5	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934.			x
10.1 <sup>^</sup>	License Agreement, by and between the Registrant and ImmunoGen, dated as of October 12, 2020.	8-K	12/09/2020	10.1
10.2 <sup>^</sup>	Technology License Agreement, by and between the Registrant and Xencor, Inc., dated as of December 16, 2020.	10-K	03/26/2021	10.2
10.3	Subscription Agreement, by and between the Registrant and Xencor, Inc., dated as of December 16, 2020.	10-K	03/26/2021	10.3
10.4	Subscription Agreement, by and between the Registrant and Xencor, Inc., dated as of December 2, 2021.	10-K	03/11/2022	10.4
10.5+	Form of Indemnity Agreement between the Registrant and each of its directors and executive officers.	S-4	12/02/2016	10.32
10.6+	Barrett Katz, M.D. Employment Agreement, dated January 18, 2021.	10-K	03/26/2021	10.14
10.7+	Kristian Humer Employment Agreement, dated June 9, 2021	8-K	07/26/2021	10.1
10.8+	Carrie Melvin Employment Agreement, dated May 25, 2022.	8-K	06/23/2022	10.1

10.9+	Scott Myers Employment Agreement, dated December 29, 2022	8-K	02/06/2023	10.1
10.10+	Jonathan Violin General Release and Separation and Consulting Agreement, dated February 6, 2023	8-K	02/06/2023	10.2
10.11+	Viridian Therapeutics, Inc. Amended & Restated 2016 Equity Incentive Plan.	10-Q	08/15/2022	10.3
10.12+	Form of Stock Option Grant Notice and Stock Option Agreement under 2016 Equity Incentive Plan.	S-4	12/02/2016	10.38
10.13+	Form of Restricted Stock Award Agreement under the 2016 Equity Incentive Plan.	10-Q	05/11/2017	10.12
10.14+	2016 Employee Stock Purchase Plan.	S-4	12/02/2016	10.39
10.15+	Viridian Therapeutics, Inc. 2020 Stock Incentive Plan.	S-8	11/24/2020	99.1
10.16+	Form of 2020 Incentive Stock Option Grant Notice under Viridian Therapeutics, Inc. 2020 Stock Incentive Plan.	10-K	03/26/2021	10.23
10.17+	Form of 2008 Equity Incentive Plan.	S-4	12/02/2016	10.48
10.18+	Form of Stock Option Grant Notice and Stock Option Agreement under the Registrant 2008 Equity Incentive Plan.	S-4	12/02/2016	10.49
10.19	Lease by and between Registrant and Crestview, LLC, dated as of December 16, 2010.	S-4	12/02/2016	10.40
10.20	First Addendum to Lease by and between Registrant and Crestview, LLC, dated as of February 18, 2015.	S-4	12/02/2016	10.40.1
10.21	Second Addendum to Lease by and between Registrant and Crestview, LLC, dated as of October 23, 2015.	S-4	12/02/2016	10.40.2
10.22	Third Addendum to Lease by and between Registrant and Crestview, LLC, dated as of January 17, 2020.	10-K	03/13/2020	10.12.3
10.23	Fourth Addendum to Lease by and between Registrant and Crestview, LLC, dated as of April 7, 2020.	10-Q	05/08/2020	10.2
10.24	Fifth Addendum to Lease by and between Registrant and Crestview LLC, dated as of March 26, 2021.	10-Q	08/12/2021	10.4
10.25	Waltham Lease between Registrant and Watch City Ventures MT, LLC dated as of January 13, 2020.	10-Q	11/05/2021	10.1
10.26	First Amendment to Waltham Lease between Registrant and Watch City Ventures MT, LLC dated as of July 6, 2021.	10-Q	11/05/2021	10.4
10.27	Second Amendment to Lease by and between the Registrant and Watch City Ventures MT, LLC dated as of April 13, 2022.	10-Q	08/15/2022	10.2
10.28	Third Amendment to Lease by and between Registrant and Watch City Ventures MT, LLC dated as of July 29, 2022.	10-Q	11/14/2022	10.1
10.29	Registration Rights Agreement, dated December 11, 2019, by and between the Registrant and Aspire Capital Fund, LLC.	8-K	12/11/2019	4.1
10.30^	Securities Purchase Agreement, dated as of October 27, 2020, by and among the Registrant and each purchaser identified on Annex A thereto.	8-K	10/28/2020	10.1
10.31^	Registration Rights Agreement, dated as of October 30, 2020, by and among the Registrant and certain purchasers.	10-Q	11/12/2020	10.8
10.32	Open Market Sale Agreement <sup>SM</sup> , dated as of November 8, 2021, by and among the Registrant and Jefferies LLC	S-3	11/08/2021	1.2
10.33	Open Market Sale Agreement <sup>SM</sup> , dated as of September 9, 2022 by and between the Registrant and Jefferies LLC.	S-3	09/09/2022	1.2
10.34^	Loan and Security Agreement, dated as of April 1, 2022, among the Viridian Therapeutics, Inc., certain of its subsidiaries from time to time party thereto, the Lenders from time to time party thereto and Hercules Capital, Inc., as Agent.	8-K	04/05/2022	10.1
21.1	Subsidiaries of the Registrant.			x
23.1	Consent of Independent Registered Public Accounting Firm.			x
24.1	Power of Attorney (included on signature page hereto).			x
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.			x

31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) and Rule 15d-14(a) of the Securities and Exchange Act, as amended.	x
32.1*	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	x
101.INS	XBRL Instance Document	x
101.SCH	XBRL Taxonomy Extension Schema Document	x
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document	x
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document	x
101.LAB	XBRL Taxonomy Extension Label Linkbase Document	x
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document	x
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)	x

^ Schedules have been omitted from this filing pursuant to Item 601(b)(2) of Regulation S-K. Viridian agrees to furnish supplementally a copy of any omitted schedule to the SEC upon its request; provided, however, that Viridian may request confidential treatment pursuant to Rule 24b-2 of the Exchange Act for any schedule so furnished. Certain portions of the exhibit, identified by the mark, “[\*\*\*],” may have been omitted because such portions contained information that is both (i) not material and (ii) would likely cause competitive harm if publicly disclosed.

+ Indicates management contract or compensatory plan

\* This certification is being furnished pursuant to 18 U.S.C. Section 1350 and is not being filed for purposes of Section 18 of the Exchange Act and is not to be incorporated by reference into any filing of the Registrant, whether made before or after the date hereof.

\* In accordance with Rule 406T of Regulation S-T, the Interactive Data Files in Exhibit 101 are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act and are deemed not filed for purposes of Section 18 of the Exchange Act and otherwise are not subject to liability under these sections.

x Filed/furnished herewith.

## ITEM 16. FORM 10-K SUMMARY

None.

**VIRIDIAN THERAPEUTICS, INC.**  
**INDEX TO CONSOLIDATED FINANCIAL STATEMENTS**

Reports of Independent Registered Public Accounting Firm (KPMG LLP, Boulder CO, Auditor Firm ID: 185 )	2
Consolidated Balance Sheets	4
Consolidated Statements of Operations and Comprehensive Loss	5
Consolidated Statements of Changes in Stockholders' Equity	6
Consolidated Statements of Cash Flows	7
Notes to Consolidated Financial Statements	9

## Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors  
Viridian Therapeutics, Inc.:

### *Opinion on the Consolidated Financial Statements*

We have audited the accompanying consolidated balance sheets of Viridian Therapeutics, Inc. and subsidiaries (the Company) as of December 31, 2022 and 2021, the related consolidated statements of operations and comprehensive loss, changes in stockholders' equity, and cash flows for the years then ended, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2022 and 2021, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

### *Basis for Opinion*

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated 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 consolidated 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 consolidated 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 consolidated 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 consolidated 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 consolidated 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 consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of a critical audit matter does not alter in any way our opinion on the consolidated 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.

### *Accrued outsourced clinical trials and preclinical studies*

As discussed in Notes 2 and 5 to the consolidated financial statements, accrued expenses for clinical trials and preclinical studies are based on estimates of costs incurred for services provided by clinical research organizations, manufacturing organizations, and other providers. In accruing for these activities, the Company obtains information from various sources and estimates the level of effort or expense allocated to each period. The estimates consider a number of factors such as site initiation, patient screening, enrollment, delivery of reports, and other events. Accrued liabilities for outsourced clinical trials and preclinical studies were \$12.6 million as of December 31, 2022.

We identified the evaluation of accrued outsourced clinical trials and preclinical studies as a critical audit matter. Specifically, evaluating the sufficiency of audit evidence obtained over the estimates of costs incurred by third



parties, including the factors described above, required subjective auditor judgment due to the nature of available evidence. Such evidence included communications from third parties regarding tasks completed, invoices received from third parties, and management's analysis of expenses incurred against budgeted and contractual amounts.

The following are the primary procedures we performed to address this critical audit matter. For a selection of accrued liabilities for outsourced clinical trials and preclinical studies, we compared the relevant factors used by management to estimate the accrued expenses to contracts, invoices and third-party confirmations of contractual milestones and project status. We compared the Company's estimate of costs accrued as of year-end to a selection of third-party invoices received after year-end, but prior to the issuance of the Company's financial statements. We assessed the sufficiency of audit evidence obtained related to accrued outsourced clinical trials and preclinical studies by assessing the cumulative results of the audit procedures.

/s/ KPMG LLP

We have served as the Company's auditor since 2009.

Boulder, Colorado  
March 9, 2023

**VIRIDIAN THERAPEUTICS, INC.**  
**CONSOLIDATED BALANCE SHEETS**  
(in thousands, except share and per share data)

	December 31,	
	2022	2021
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 155,579	\$ 42,299
Short-term investments	268,971	154,666
Prepaid expenses and other current assets	6,521	2,747
Unbilled revenue - related party	102	451
Total current assets	431,173	200,163
Property and equipment, net	1,326	375
Operating lease right-of-use asset	1,610	1,680
Other assets	982	1,491
Total assets	<u>\$ 435,091</u>	<u>\$ 203,709</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 14,234	\$ 2,329
Accrued liabilities	18,827	11,018
Current portion of deferred revenue - related party	288	289
Total current liabilities	33,349	13,636
Long-term debt, net	4,645	—
Deferred revenue - related party	861	1,149
Other liabilities	1,172	1,208
Total liabilities	40,027	15,993
Commitments and contingencies		
Stockholders' equity:		
Preferred stock, series A non-voting convertible preferred stock, \$0.01 par value; 435,000 shares authorized; 188,381 and 260,437 shares issued and outstanding as of December 31, 2022 and 2021, respectively	85,470	118,164
Preferred stock, series B non-voting convertible preferred stock, \$0.01 par value; 500,000 shares authorized; 51,210 and 23,126 shares issued and outstanding as of December 31, 2022 and 2021, respectively	56,677	15,669
Common stock, \$0.01 par value; 200,000,000 shares authorized; 41,305,947 and 23,924,004 shares issued and outstanding as of December 31, 2022 and 2021, respectively	414	239
Additional paid-in capital	741,067	412,101
Accumulated other comprehensive loss	(390)	(157)
Accumulated deficit	(488,174)	(358,300)
Total stockholders' equity	395,064	187,716
Total liabilities and stockholders' equity	<u>\$ 435,091</u>	<u>\$ 203,709</u>

*See accompanying notes to these consolidated financial statements.*

**VIRIDIAN THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
(in thousands, except share and per share data)

	Year Ended December 31,	
	2022	2021
Revenue:		
Collaboration revenue - related party	\$ 1,772	\$ 2,963
Operating expenses:		
Research and development (related party - \$5,619 and \$— at December 31, 2022 and 2021, respectively)	100,894	56,886
General and administrative	35,182	25,805
Total operating expenses	<u>136,076</u>	<u>82,691</u>
Loss from operations	<u>(134,304)</u>	<u>(79,728)</u>
Other income (expense):		
Interest and other income	4,916	318
Interest and other expense	<u>(486)</u>	<u>(3)</u>
Other income, net	4,430	315
Net loss	<u>\$ (129,874)</u>	<u>\$ (79,413)</u>
Net loss per share, basic and diluted	\$ (4.05)	\$ (6.66)
Weighted-average shares used to compute basic and diluted net loss per share	32,087,293	11,918,712
Comprehensive loss:		
Net loss	\$ (129,874)	\$ (79,413)
Other comprehensive loss:		
Change in unrealized loss on investments	<u>(233)</u>	<u>(149)</u>
Total other comprehensive loss	<u>(233)</u>	<u>(149)</u>
Total comprehensive loss	<u>\$ (130,107)</u>	<u>\$ (79,562)</u>

*See accompanying notes to these consolidated financial statements.*

**VIRIDIAN THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY**  
(in thousands, except share data)

	Preferred Stock		Series B Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Series A Convertible Preferred Stock		Series B Convertible Preferred Stock		Common Stock					
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance as of December 31, 2020	398,487	\$ 180,801	—	\$ —	4,231,135	\$ 42	\$ 218,089	\$ (8)	\$ (278,887)	\$ 120,037
Issuance of common stock upon the conversion of convertible preferred stock	(138,050)	(62,637)	—	—	9,203,732	92	62,545	—	—	—
Issuance of Series B preferred stock and common stock in the 2021 Public Offering, net of issuance costs of \$1,291 and \$5,983, respectively	—	—	23,126	15,669	7,344,543	73	74,734	—	—	90,476
Issuance of common stock upon exercises of warrants	—	—	—	—	77,871	1	1,284	—	—	1,285
Issuance of common stock for exercises of stock options	—	—	—	—	106,831	1	1,026	—	—	1,027
Issuance of common stock upon the vesting of restricted stock units	—	—	—	—	10,574	—	—	—	—	—
Issuance of common stock, 2021 ATM, net of issuance costs of \$1,004	—	—	—	—	2,551,269	26	32,423	—	—	32,449
Issuance of common stock under license agreement	—	—	—	—	394,737	4	7,496	—	—	7,500
Issuance of common stock for cash under employee stock purchase plan	—	—	—	—	3,312	—	39	—	—	39
Share-based compensation expense	—	—	—	—	—	—	14,465	—	—	14,465
Change in unrealized loss on investments	—	—	—	—	—	—	—	(149)	—	(149)
Net loss	—	—	—	—	—	—	—	—	(79,413)	(79,413)
Balance as of December 31, 2021	260,437	118,164	23,126	15,669	23,924,004	239	412,101	\$ (157)	(358,300)	187,716
Issuance of common stock upon the conversion of convertible preferred stock	(72,056)	(32,694)	—	—	4,803,965	48	32,646	—	—	—
Issuance of Series B preferred stock and common stock, 2022 Public Offering, net of issuance costs of \$2,992 and \$18,146, respectively	—	—	28,084	41,008	11,352,640	114	248,528	—	—	289,650
Issuance of common stock, September 2022 ATM, net of issuance costs of \$926	—	—	—	—	964,357	10	24,150	—	—	24,160
Issuance of common stock upon exercises of warrants	—	—	—	—	56,666	1	934	—	—	935
Issuance of common stock for exercises of stock options	—	—	—	—	191,291	2	2,760	—	—	2,762
Issuance of common stock for cash under employee stock purchase plan	—	—	—	—	13,024	—	183	—	—	183
Share-based compensation expense	—	—	—	—	—	—	19,765	—	—	19,765
Change in unrealized loss on investments	—	—	—	—	—	—	—	(233)	—	(233)
Net loss	—	—	—	—	—	—	—	—	(129,874)	(129,874)
Balance as of December 31, 2022	188,381	\$ 85,470	51,210	\$ 56,677	41,305,947	414	\$ 741,067	\$ (390)	\$ (488,174)	\$ 395,064

See accompanying notes to these consolidated financial statements.

**VIRIDIAN THERAPEUTICS, INC.**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(in thousands)**

	Year Ended December 31,	
	2022	2021
Cash flows from operating activities:		
Net loss	\$ (129,874)	\$ (79,413)
Adjustments to reconcile net loss to net cash (used in) operating activities:		
Issuance of common stock under license agreement	—	7,500
Share-based compensation expense	19,765	14,465
Non-cash interest expense and amortization of debt issuance costs	228	87
Depreciation and amortization	255	120
Accretion and amortization of premiums and discounts on available-for-sale securities	(210)	965
Realized gain on investments	—	(4)
Loss on sale of equipment	—	77
Non-cash lease expenses	36	(102)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(3,339)	(1,496)
Accounts payable	11,524	1,655
Accrued and other liabilities	7,716	1,380
Unbilled revenue	349	(451)
Deferred revenue	(288)	636
Net cash used in operating activities	<u>(93,838)</u>	<u>(54,581)</u>
Cash flows from investing activities:		
Purchases of short-term investments	(223,264)	(188,431)
Proceeds from sales and maturities of short-term investments	108,935	114,398
Proceeds from sale of property and equipment	—	79
Purchases of property and equipment	(797)	(338)
Net cash used in investing activities	<u>(115,126)</u>	<u>(74,292)</u>
Cash flows from financing activities:		
Proceeds from the issuance of common stock, pursuant to 2022 Public Offering and September 2022 ATM Agreement	291,874	—
Proceeds from the issuance of common stock, pursuant to 2021 Public Offering and April 2021 ATM Agreement	—	114,242
Payments of issuance costs associated with the sale of common stock	(19,072)	(6,987)
Proceeds from the issuance of Series B preferred stock	44,000	16,960
Payment of issuance costs associated with the sale of preferred stock	(2,992)	(1,291)
Proceeds from the exercise of warrants	935	1,285
Proceeds from issuance of long-term debt	5,000	—
Payment of debt issuance costs	(446)	—
Proceeds from issuance of common stock upon the exercise of stock options	2,762	1,027
Proceeds from the issuance of common stock for cash under employee stock purchase plan	183	39
Net cash provided by financing activities	<u>322,244</u>	<u>125,275</u>
Net increase (decrease) in cash and cash equivalents	<u>113,280</u>	<u>(3,598)</u>
Cash and cash equivalents at beginning of period	42,299	45,897
Cash and cash equivalents at end of period	<u>\$ 155,579</u>	<u>\$ 42,299</u>
Supplemental disclosure of cash flow information		
Interest paid	\$ 293	\$ —
Supplemental disclosure of non-cash investing and financing activities		



Purchase of property and equipment in accounts payable and accrued liabilities	\$	380	\$	4
Amortization of public offering costs	\$	75	\$	87

*See accompanying notes to these consolidated financial statements.*

**VIRIDIAN THERAPEUTICS, INC.**  
**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS**

**1. DESCRIPTION OF BUSINESS**

Viridian Therapeutics, Inc., a Delaware corporation (the “Company” or “Viridian”), is a biopharmaceutical company advancing new treatments for patients suffering from serious diseases that are underserved by today’s therapies. The Company’s most advanced program, VRDN-001, is a differentiated monoclonal antibody targeting insulin-like growth factor-1 receptor (“IGF-1R”), a clinically and commercially validated target for the treatment of thyroid eye disease (“TED”). The Company’s second product candidate, VRDN-002, is a distinct anti-IGF-1R antibody that incorporates half-life extension technology. VRDN-003 is an extended half-life version of VRDN-001. Both VRDN-002 and VRDN-003 are designed for administration as convenient, low-volume, subcutaneous pen injections. TED is a debilitating autoimmune disease that causes inflammation and fibrosis within the orbit of the eye which can cause double vision, pain, and potential blindness. Patients with severe disease often require multiple remedial surgeries to the orbit, eye muscles and eyelids. In addition to developing therapies for TED, the Company is executing a similar strategic approach to identify opportunities in other rare and/or serious disease indications.

***Liquidity***

The accompanying consolidated financial statements have been prepared on a basis that assumes the Company is a going concern and do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classifications of liabilities that may result from any uncertainty related to its ability to continue as a going concern. The Company has funded its operations to date principally through proceeds received from the sale of the Company’s common stock, its Series A Preferred Stock, Series B Preferred Stock, and other equity securities, debt financings, license fees, and reimbursements received under collaboration agreements. Since its inception and through December 31, 2022, the Company has generated an accumulated deficit of \$488.2 million. The Company expects to continue to generate operating losses for the foreseeable future.

The Company has no products approved for commercial sale, has not generated any revenue from product sales, and cannot guarantee when or if it will generate any revenue from product sales. Substantially all of the Company’s operating losses resulted from expenses incurred in connection with its research and development programs and from general and administrative costs associated with its operations. The Company expects to incur significant expenses and operating losses for at least the next several years as it continues the development of, and seeks regulatory approval for, its product candidates. It is expected that operating losses will fluctuate significantly from quarter to quarter and year to year due to timing of development programs and efforts to achieve regulatory approval.

As of December 31, 2022, the Company had approximately \$424.6 million in cash, cash equivalents, and short-term investments. As of the issuance date of these consolidated financial statements, the Company expects that its current resources will be sufficient to fund its operating expenses and capital expenditure requirements for at least the next twelve months from the issuance date of these financial statements.

The Company will continue to require additional capital in order to continue to finance its operations. The amount and timing of future funding requirements will depend on many factors, including the pace and results of the Company’s clinical development efforts, equity financings, entering into license and collaboration agreements, and issuing debt or other financing vehicles. The Company’s ability to secure additional capital is dependent upon a number of factors, some of which are outside of the Company’s control, including success in developing its technology and drug product candidates, operational performance, and market conditions, including those resulting from the current inflationary and broader macroeconomic environment.

Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on the Company’s financial condition and its ability to develop its product candidates. Changing circumstances may cause the Company to consume capital significantly faster or slower than currently anticipated. If the Company is unable to acquire additional capital or resources, it will be required to modify its operational plans. The estimates included herein are based on assumptions that may prove to be wrong, and the Company could exhaust its available financial resources sooner than currently anticipated.

## 2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

### ***Basis of Presentation***

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, Viridian Therapeutics Europe Limited and Viridian Therapeutics S.à.r.l., both of which were formed for the sole purpose of submitting regulatory filings in Europe, and Viridian Securities Corporation, which was formed in July 2021. The Company's subsidiaries have no employees or operations.

The consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position, results of operations, and cash flows for the periods presented. All significant intercompany balances have been eliminated in consolidation. The Company's management performed an evaluation of its activities through the date of filing of these consolidated financial statements and concluded that there are no subsequent events requiring disclosure, other than as disclosed.

### ***Global Economic Considerations***

The global macroeconomic environment is uncertain, and could be negatively affected by, among other things, increased U.S. trade tariffs and trade disputes with other countries, instability in the global capital and credit markets, supply chain weaknesses, and instability in the geopolitical environment, including as a result of the Russian invasion of Ukraine, the rising tensions between China and Taiwan and other political tensions, and lingering effects of the COVID-19 pandemic. Such challenges have caused, and may continue to cause, recession fears, concerns regarding potential sanctions, rising interest rates, foreign exchange volatility and inflationary pressures. At this time, the Company is unable to quantify the potential effects of this economic instability on its future operations.

### ***Going Concern***

At each reporting period, the Company evaluates whether there are conditions or events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued. The Company is required to make certain additional disclosures if it concludes substantial doubt exists and it is not alleviated by the Company's plans or when its plans alleviate substantial doubt about the Company's ability to continue as a going concern.

The Company's evaluation entails, among other things, analyzing the results of the Company's clinical development efforts, license and collaboration agreements as well as the entity's current financial condition including conditional and unconditional obligations anticipated within a year, and related liquidity sources at the date the financial statements are issued. This is reflected in the Company's prospective operating budgets and forecasts and compared to the current cash, cash equivalents and short-term investments balance.

### ***Use of Estimates***

The Company's consolidated financial statements are prepared in accordance with U.S. GAAP, which requires it to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and contingent liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual for clinical trial costs and other outsourced research and development expenses and the valuation of share-based awards. Although these estimates are based on the Company's knowledge of current events and actions it may take in the future, actual results may ultimately differ from these estimates and assumptions.

### ***Revenue Recognition***

The Company accounts for revenue in accordance with ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606").

The Company enters into collaboration agreements and certain other agreements that are within the scope of ASC 606, under which the Company licenses, may license, or grants an option to license rights to certain of the Company's product candidates and performs research and development services in connection with such agreements. The terms of these agreements typically include payment of one or more of the following: non-refundable, up-front fees; reimbursement of research and development costs; developmental, clinical, regulatory, and commercial sales milestone payments; and royalties on net sales of licensed products.

In accordance with ASC 606, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services.

To determine the appropriate amount of revenue to be recognized, for agreements within the scope of ASC 606, the Company performs the following five steps: (i) identification of the goods or services within the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct within the terms of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the identified performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the Company will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer.

The promised goods or services in the Company's agreements typically consist of a license, or option to license, rights to the Company's intellectual property or research and development services. Performance obligations are promises in a contract to transfer a distinct good or service to the customer and are considered distinct when (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on its own or whether the required expertise is readily available, and whether the goods or services are integral or dependent to other goods or services in the contract.

The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each agreement that includes variable consideration, the Company evaluates the amount of potential payment and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected value method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration that is included in the transaction price may be constrained and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

The Company's contracts often include development and regulatory milestone payments that are assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within the Company's control or the licensee's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of such development and clinical milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration and other research and development revenue in the period of adjustment.

For agreements that include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of the Company's collaboration or strategic alliance agreements.

The Company allocates the transaction price based on the estimated standalone selling price. The Company must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. The Company utilizes key assumptions to determine the stand-alone selling price, which may include other comparable transactions, pricing considered in negotiating the transaction, and the estimated costs. Variable consideration is allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts the Company would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

The Company receives payments from its customers based on billing schedules established in each contract. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional.

### ***Research and Development***

Research and development costs are expensed as incurred in performing research and development activities. The costs include employee-related expense including salaries, benefits, share-based compensation, restructuring charges, fees for acquiring and maintaining licenses under third-party license agreements, consulting fees, costs of research and development activities conducted by third parties on the Company's behalf, costs to manufacture or have manufactured clinical trial materials, laboratory supplies, depreciation, and facilities and overhead costs. The Company records research and development expense in the period in which the Company receives or takes ownership of the applicable goods or when the applicable services are performed. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense.

The Company records up-front and milestone payments to acquire and retain contractual rights to licensed technology as research and development expenses when incurred if there is uncertainty in the Company receiving future economic benefit from the acquired contractual rights. Such expenses are included within operating activities in the consolidated statements of cash flows. The Company considers future economic benefits from acquired contractual rights to licensed technology to be uncertain until such a drug candidate is approved for sale by the U.S. Food and Drug Administration ("FDA") or when other significant risk factors are abated.

### ***Clinical Trial and Preclinical Study Accruals***

The Company makes estimates of accrued expenses as of each balance sheet date in its consolidated financial statements based on certain facts and circumstances at that time. The Company's accrued expenses for clinical trials and preclinical studies are based on estimates of costs incurred for services provided by clinical research organizations, manufacturing organizations, and other providers. Payments under the Company's agreements with external service providers depend on a number of factors, such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, the Company obtains information from various sources and estimates the level of effort or expense allocated to each period. Adjustments to the Company's research and development expenses may be necessary in future periods as its estimates change.

### ***Share-Based Compensation***

The Company accounts for share-based compensation expense to employees and non-employees based on the fair value of each stock option or award on the date of the grant. The Company uses the Black-Scholes option pricing model to determine the fair value of stock options. The use of the Black-Scholes option-pricing model requires the Company to make assumptions with respect to the expected term of the option, the expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates and expected dividend yields of the common stock. The Company recognizes share-based compensation expense for awards with service-based conditions using the straight-line method over the requisite service period, net of any actual forfeitures.

### ***Cash and Cash Equivalents***

All highly-liquid investments that have maturities of 90 days or less at the date of purchase are classified as cash equivalents. Cash equivalents are reported at cost, which approximates fair value due to the short maturities of these instruments.

### ***Investments***

The Company's investments consist of highly-rated corporate and U.S. Treasury securities and have been classified as available-for-sale securities. Accordingly, these investments are recorded at their respective fair values, as determined based on quoted market prices. The Company may hold securities with stated maturities greater than one year. All available-for-sale securities are considered available to support current operations, and thus investments with maturities beyond one year are generally classified as current assets.

Unrealized gains and losses are reported as a component of stockholders' equity until their disposition. Realized gains and losses are included as a component of other income (expense), net based on the specific identification method. The securities are subject to a periodic impairment review. An impairment charge would occur when a decline in the fair value of the investments below the cost basis is judged to be other-than-temporary.



### ***Fair Value Measurements***

Certain assets and liabilities are carried at fair value under U.S. GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities.
- Level 2 inputs utilizes observable inputs other than Level 1 prices, such as quoted prices, for similar assets or liabilities, quoted market prices in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities.
- Level 3 inputs are unobservable data points for the asset or liability and include situations where there is little, if any, market activity for the asset or liability.

Certain of the Company's financial instruments are not measured at fair value on a recurring basis but are recorded at amounts that approximate their fair value due to the short-term nature of their maturities, such as cash and cash equivalents, accounts receivable, accounts payable and accrued expenses.

### ***Concentrations of Credit Risk***

Financial instruments that potentially subject the Company to concentrations of credit risk consist primarily of cash equivalents, which include short-term investments that have maturities of less than three months. The Company maintains deposits in federally insured financial institutions in excess of federally insured limits. The Company has not experienced any losses in such accounts. The Company invests its excess cash primarily in deposits and money market funds held with one financial institution.

### ***Property and Equipment***

The Company carries its property and equipment at cost, less accumulated depreciation and amortization. Depreciation is computed using the straight-line method over the estimated useful lives of the assets, generally three to five years. Leasehold improvements are amortized over the shorter of the life of the lease (including any renewal periods that are deemed to be reasonably assured) or the estimated useful life of the assets. Construction in progress is not depreciated until placed in service. Repairs and maintenance costs are expensed as incurred and expenditures for major improvements are capitalized.

### ***Operating Lease Right-of-Use Assets and Liabilities***

The Company determines if an arrangement is, or contains, a lease at contract inception and during modifications or renewal of existing leases. Operating lease assets represent the Company's right to use an underlying asset for the lease term and operating lease liabilities represent the Company's obligation to make lease payments arising from the lease. The Company has recorded operating lease assets and liabilities pursuant to the guidance in Accounting Standards Update ("ASU") No. 2016-02, *Leases (Topic 842)*, and subsequent amendments to the initial guidance: ASU No. 2017-13, ASU No. 2018-10, and ASU No. 2018-11 (collectively, "ASC 842"). These operating lease assets and liabilities are recognized at the commencement date of the lease based upon the present value of lease payments over the lease term. The lease payments used to determine the Company's operating lease assets may include lease incentives, stated rent increases, and escalation clauses and are recognized in the Company's operating lease assets in the Company's consolidated balance sheets. The Company's operating leases are reflected in operating lease right-of-use asset and operating lease liability within accrued and other liabilities in the Company's consolidated balance sheets. Lease expense for minimum lease payments is recognized on a straight-line basis over the lease term. Short-term leases, defined as leases that have a lease term of 12 months or less at the commencement date, are excluded from this treatment and are recognized on a straight-line basis over the term of the lease. Refer to Note 8. *Commitments and Contingencies - Lease Obligations* for additional information related to the Company's operating leases.

### ***Debt and Debt Issuance Costs***

Debt issuance costs and expenses paid by the Company to its lenders are presented on the consolidated balance sheet as a direct deduction from the related debt liability rather than capitalized as an asset in accordance with ASU No. 2015-03, *Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs*. Debt issuance costs represent

legal and other direct costs incurred in connection with the Company's Term Loan (as defined in Note 6. *Debt*). These costs are amortized as a non-cash component of interest expense using the effective interest method over the term of the loan.

### ***Convertible Preferred Stock***

The Company records shares of non-voting convertible preferred stock at their respective fair values on the dates of issuance, net of issuance costs. The Company has applied the guidance in ASC 480-10-S99-3A, *SEC Staff Announcement: Classification and Measurement of Redeemable Securities*, and at issuance classified the Series A Preferred Stock outside of stockholders' equity because, if convertibility of Series A Preferred Stock into common stock was not approved by the stockholders, the Series A Preferred Stock would be redeemable at the option of the holders for cash equal to the closing price of the common stock on last trading day prior to the holder's redemption request. On December 31, 2020, the stockholders approved the convertibility of the Series A Preferred Stock into common stock and as such, the Company reclassified the Series A Preferred Stock to permanent equity. In September 2021, the Company issued Series B Preferred Stock with conversion rights which the Company has classified as permanent equity in its consolidated balance sheets.

### ***Impairment of Long-Lived Assets***

The Company assesses the carrying amount of its property and equipment whenever events or changes in circumstances indicate the carrying amount of such assets may not be recoverable. No impairment charges were recorded during the years ended December 31, 2022 and 2021.

### ***Net Loss per Share***

Basic net loss per share is calculated by dividing the net loss by the weighted average number of shares of common stock outstanding during the period without consideration of common stock equivalents. Since the Company was in a loss position for all periods presented, diluted net loss per share is the same as basic net loss per share for all periods, as the inclusion of all potential common shares outstanding is antidilutive.

### ***Comprehensive Loss***

Comprehensive loss is comprised of net loss and adjustments for the change in unrealized gains and losses on investments. Unrealized accumulated comprehensive gains or losses are reflected as a separate component in the consolidated statements of changes in stockholders' equity. The Company had unrealized losses on investments of \$0.2 million and \$0.1 million during the years ended December 31, 2022 and 2021, respectively.

### ***Income Taxes***

The Company accounts for income taxes by using an asset and liability method of accounting for deferred income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. A valuation allowance is recorded to the extent it is more likely than not that a deferred tax asset will not be realized. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in operations in the period that includes the enactment date.

The Company's significant deferred tax assets are for net operating loss carryforwards, tax credits, accruals and reserves, and capitalized start-up costs. The Company has provided a valuation allowance for its entire net deferred tax assets since inception as, due to its history of operating losses, the Company has concluded that it is more likely than not that its deferred tax assets will not be realized.

The Company has no unrecognized tax benefits. The Company classifies interest and penalties arising from the underpayment of income taxes in the consolidated statements of operations and comprehensive loss as general and administrative expenses. No such expenses have been recognized during the years ended December 31, 2022 and 2021.

### ***Warrants***

Upon the issuance of warrants to purchase shares of common stock, the Company evaluates the terms of the warrant issue to determine the appropriate accounting and classification of the warrant issue pursuant to FASB ASC Topic 480, *Distinguishing Liabilities from Equity*, FASB ASC Topic 505, *Equity*, FASB ASC 815, *Derivatives and Hedging*, and ASC 718,

*Compensation - Stock Compensation*, and classifies warrants for common stock as liabilities or equity. Warrants are classified as liabilities when the Company may be required to settle a warrant exercise in cash and classified as equity when the Company settles a warrant exercise in shares of its common stock.

### ***Segment Information***

The Company operates in one operating segment and, accordingly, no segment disclosures have been presented herein. All equipment, leasehold improvements, and other fixed assets are physically located within the United States and all agreements with the Company's partners are denominated in U.S. dollars, except where noted.

### ***Accounting Pronouncements – To Be Adopted***

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that the Company adopts as of the specified effective date. The Company does not believe that the adoption of recently issued standards have or may have a material impact on the Company's consolidated financial statements or disclosures.

## **3. INVESTMENTS AND FAIR VALUE MEASUREMENTS**

### ***Investments***

The Company's investments consisted of the following as of December 31, 2022 and December 31, 2021:

<b>(in thousands)</b>	<b>Amortized Cost</b>	<b>Gross Unrealized Gains</b>	<b>Gross Unrealized Losses</b>	<b>Fair Value</b>
<b>December 31, 2022</b>				
Money market funds	\$ 137,903	\$ —	\$ —	\$ 137,903
U.S. treasury securities	85,578	123	(96)	85,605
U.S. corporate paper and bonds	199,350	1	(385)	198,966
International corporate bond holdings	2,009	—	(33)	1,976
<b>Total</b>	<b>\$ 424,840</b>	<b>\$ 124</b>	<b>\$ (514)</b>	<b>\$ 424,450</b>
<b>December 31, 2021</b>				
Money market funds	\$ 42,199	\$ —	\$ —	\$ 42,199
U.S. treasury securities	22,215	—	(54)	22,161
U.S. corporate paper and bonds	128,005	6	(94)	127,917
International corporate bond holdings	4,603	—	(15)	4,588
<b>Total</b>	<b>\$ 197,022</b>	<b>\$ 6</b>	<b>\$ (163)</b>	<b>\$ 196,865</b>

The Company considers the unrealized losses in its investment portfolio to be temporary in nature and not due to credit losses. The Company has the intent and ability to hold such investments until their recovery at fair value. The Company had realized gains of zero and \$4 thousand in its available for sale securities for the years ended December 31, 2022 and 2021, respectively. The contractual maturity dates of all of the Company's investments are all less than 24 months.

### ***Fair Value Measurements***

The following tables summarize the Company's assets and liabilities that are measured at fair value on a recurring basis:

(in thousands)	Quoted Prices in Active Markets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
<b>December 31, 2022</b>				
Assets:				
<b>Cash equivalents:</b>				
Money market funds	\$ 137,903	\$ —	\$ —	\$ 137,903
U.S. corporate paper and bonds	—	17,576	—	17,576
<b>Short-term investments:</b>				
U.S. treasury securities	—	85,605	—	85,605
U.S. corporate paper and bonds	—	181,390	—	181,390
International corporate bond holdings	—	1,976	—	1,976
Total cash equivalents and short-term investments	<u>\$ 137,903</u>	<u>\$ 286,547</u>	<u>\$ —</u>	<u>\$ 424,450</u>
<b>December 31, 2021</b>				
Assets:				
<b>Cash equivalents:</b>				
Money market funds	\$ 42,199	\$ —	\$ —	\$ 42,199
<b>Short-term investments:</b>				
U.S. treasury securities	—	22,161	—	22,161
U.S. corporate paper and bonds	—	127,917	—	127,917
International corporate bond holdings	—	4,588	—	4,588
Total cash equivalents and short-term investments	<u>\$ 42,199</u>	<u>\$ 154,666</u>	<u>\$ —</u>	<u>\$ 196,865</u>

#### 4. PROPERTY AND EQUIPMENT

Property and equipment, net, consisted of the following:

	December 31,	
	2022	2021
	(in thousands)	
Lab equipment	\$ 1,160	\$ 775
Leasehold improvements	1,113	749
Computer hardware and software	725	430
Furniture and fixtures	332	197
Property and equipment, gross	3,330	2,151
Less: accumulated depreciation and amortization	(2,004)	(1,776)
Property and equipment, net	<u>\$ 1,326</u>	<u>\$ 375</u>

During the year ended December 31, 2021, certain lab equipment met the criteria to be classified and were reclassified as held for sale and included in prepaid expenses and other current assets. The assets held for sale totaled \$0.1 million, which was the net book value on the date of transfer. During the year ended December 31, 2021, the Company sold the equipment, received proceeds of approximately \$0.1 million and recorded a loss of approximately \$0.1 million.

During the years ended December 31, 2022 and 2021, depreciation and amortization expense was \$0.3 million and \$0.1 million, respectively.

## 5. ACCRUED LIABILITIES

Accrued liabilities consisted of the following:

	December 31,	
	2022	2021
	(in thousands)	
Accrued outsourced clinical trials and preclinical studies	\$ 12,576	\$ 6,316
Accrued employee compensation and related taxes	4,772	3,652
Operating lease liability, short-term	613	520
Accrued legal fees and expenses	177	80
Accrued other professional service fees	392	140
Value of liability-classified stock purchase warrants	100	100
Other accrued liabilities	197	210
Total accrued liabilities	<u>\$ 18,827</u>	<u>\$ 11,018</u>

## 6. DEBT

### *Loan and Security Agreement with Hercules Capital, Inc.*

In April 2022, the Company entered into a loan and security agreement (the “Hercules Loan and Security Agreement”) among the Company, certain of its subsidiaries from time to time party thereto (together with the Company, collectively, the “Borrower”), Hercules Capital, Inc. (“Hercules”) and certain other lenders named therein (the “Lenders”). Under the Hercules Loan and Security Agreement, the Lenders provided the Company with access to a term loan with an aggregate principal amount of up to \$75.0 million, in four tranches (collectively the “Term Loan”), consisting of (1) an initial tranche of \$25.0 million, available to the Company through June 15, 2023; (2) a second tranche of \$10.0 million, subject to the achievement of certain regulatory milestones, available through June 15, 2023; (3) a third tranche of \$15.0 million, subject to the achievement of certain regulatory milestones, available through March 15, 2024; and (4) a fourth tranche of \$25.0 million, subject to approval by the Lenders’ investment committee(s), available through December 15, 2024. The milestones for the second and third tranches have not yet been achieved. The obligations of the Borrower under the Loan Agreement are secured by substantially all of the assets of the Borrower, excluding the Borrower’s intellectual property. The Term Loan has a maturity date of October 1, 2026.

The Term Loan bears interest at a floating per annum rate equal to the greater of (i) 7.45% and (ii) 4.2% above the Prime Rate, provided that the Term Loan interest rate shall not exceed a per annum rate of 8.95%. Interest is payable monthly in arrears on the first day of each month. The interest rate as of December 31, 2022 was 8.95% based upon an increase in the prime rate in June 2022.

Per the terms of the Hercules Loan and Security Agreement, the Company was originally obligated to make interest-only payments through April 1, 2024. However, upon the achievement of a development milestone in August 2022 the interest-only period was extended to October 1, 2024. If additional development milestones are met, the interest-only period will be further extended to April 1, 2025. The Borrower is required to repay the Term Loan amount in equal monthly installments of the principal amount and interest between the end of the interest-only period and the maturity date of October 1, 2026. In addition, the Borrower is required to pay an end-of-term fee equal to 6% of the principal amount of funded Term Loan advances at maturity, which are being accreted as additional interest expense over the term of the loan.

Upon signing the Hercules Loan and Security Agreement, the Company drew an initial principal amount of \$5.0 million. The Company incurred debt issuance costs of \$0.2 million in connection with the Term Loan. In addition, in connection with the initial draw, the Company paid to the Lenders a facility fee of \$0.1 million, as well as \$0.1 million of other expenses incurred by the Lenders and reimbursed by the Company (“Lender Expenses”). The debt issuance costs and the Lender Expenses are being amortized as additional interest expense over the term of the loan. Debt issuance costs and the Lender Expenses are presented on the consolidated balance sheet as a direct deduction from the related debt liability rather than capitalized as an



asset in accordance with ASU No. 2015-03, *Interest - Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs*.

The total cost of all items (cash interest, the amortization/accretion of the debt issuance costs and the end-of-term fee) is being recognized as interest expense using an effective interest rate of approximately 9.3%. The Company recorded interest expense of \$0.5 million during the year ended December 31, 2022.

The following table summarizes the impact of the Term Loan, on the Company’s consolidated balance sheet at December 31, 2022:

	<b>December 31, 2022</b>
	<b>(in thousands)</b>
Gross proceeds	5,000
Unamortized debt issuance costs	(355)
Carrying value	<u>\$ 4,645</u>

Future principal payments, which exclude the end of term charge, in connection with the Hercules Loan and Security Agreement as of December 31, 2022 are as follows (in thousands):

<b>Fiscal Year</b>	
2023	—
2024	551
2025	2,331
2026	2,118
<b>Total</b>	<u>\$ 5,000</u>

## 7. COLLABORATION AGREEMENTS

### *License Agreement with Zenas BioPharma*

In October 2020, the Company became party to a license agreement with Zenas BioPharma (Cayman) Limited (“Zenas BioPharma”) to license technology comprising certain materials, patent rights, and know-how to Zenas BioPharma. Since February 2021, the Company has entered into several letter agreements with Zenas BioPharma pursuant to which the Company agreed to provide assistance to Zenas BioPharma with certain development activities, including manufacturing. In May 2022, the Company entered into a Manufacturing Development and Supply Agreement with Zenas BioPharma (the “Supply Agreement”) to manufacture and supply, or to have manufactured and supplied, clinical drug product for developmental purposes. The license agreement and subsequent letter agreements and supply agreement (collectively, the “Zenas Agreements”) were negotiated with a single commercial objective and are treated as a combined contract for accounting purposes. Under the terms of the Zenas Agreements, the Company granted Zenas BioPharma an exclusive license to develop, manufacture, and commercialize certain IGF-1R directed antibody products for non-oncology indications in the greater area of China.

As consideration for the Zenas Agreements, the transaction price included upfront non-cash consideration and variable consideration in the form of payment for the Company’s goods and services and milestone payments due upon the achievement of specified events. Under the Zenas Agreements, the Company can receive non-refundable milestone payments upon achieving specific milestone events during the contract term. Additionally, the Company may receive royalty payments based on a percentage of the annual net sales of any licensed products sold on a country-by-country basis in the greater area of China. The royalty percentage may vary based on different tiers of annual net sales of the licensed products made. Zenas BioPharma is obligated to make royalty payments to the Company for the royalty term in the Zenas Agreements.

The Zenas Agreements would qualify as a collaborative arrangement under the scope of Accounting Standards Codification, Topic 808, *Collaborative Arrangements* (“ASC 808”). While this arrangement is in the scope of ASC 808, the Company applied ASC 606 to account for certain aspects of this arrangement. The Company applied ASC 606 for certain activities within the arrangement associated with the Company’s transfer of a good or service (i.e., a unit of account) that is part of the Company’s ongoing major or central operations. The Company allocated the transaction price based on the relative estimated

standalone selling prices of each performance obligation or, in the case of certain variable consideration, to one or more performance obligations. Research and development activities are priced generally at cost. The Company's license of goods and services to Zenas BioPharma during the contract term was determined to be a single performance obligation satisfied over time. The Company will recognize the transaction price from the license agreement over the Company's estimated period to complete its activities.

At the inception of the arrangement, the Company evaluated whether the milestones were considered probable of being reached and estimated the amount to be included in the transaction price using the most likely amount method. As it was not probable that a significant revenue reversal would not occur, none of the associated milestone payments were included in the transaction price at contract inception. For the sales-based royalties included in the arrangement, the license was deemed to be the predominant item to which the royalties relate. The Company will recognize royalty revenues at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). During the years ended December 31, 2022 and 2021, the Company recognized \$1.8 million and \$3.0 million, respectively, of collaboration revenue related to the Zenas Agreements.

As of December 31, 2022, the Zenas Agreements are considered related party transactions because Fairmount Funds Management LLC beneficially owns more than 5% of the Company's common stock and is also a 5% or greater stockholder of Zenas BioPharma and has a seat on Zenas BioPharma's board of directors.

#### ***Antibody and Discovery Option Agreement with Paragon Therapeutics, Inc.***

In January 2022, the Company and Paragon Therapeutics, Inc. ("Paragon") entered into an antibody and discovery option agreement (the "Paragon Agreement") under which the Company and Paragon will cooperate to develop one or more antibodies. Under the terms of the Paragon Agreement, Paragon will perform certain development activities in accordance with an agreed upon research plan, and the Company will pay Paragon agreed upon development fees in exchange for Paragon's commitment of the necessary personnel and resources to perform these activities. The Paragon Agreement stipulates a final deliverable to the Company comprising of a report summarizing the experiments and processes performed under the research plan (the "Final Deliverable").

Additionally, Paragon agreed to grant the Company an option for an exclusive license to all of Paragon's right, title and interest in and to certain antibody technology and the Final Deliverable, and a non-exclusive license to certain background intellectual property owned by Paragon solely to research, develop, make, use, sell, offer for sale and import of the licensed intellectual property and resulting products worldwide (each, an "Option" and together, the "Options"). Paragon also granted to the Company a limited, exclusive, royalty-free license, without the right to sublicense, to certain antibody technology and the Final Deliverable, and a non-exclusive, royalty-free license without the right to sublicense, under certain background intellectual property owned by Paragon, solely to evaluate the antibody technology and Option and for the purpose of allowing the Company to determine whether to exercise the Option with respect to certain programs. The Company may, at its sole discretion, exercise the Option with respect to specified programs at any time until the date that is 90 days after the Company's receipt of the Final Deliverable the applicable program, or such longer period as agreed upon by the parties ("Option Period") by delivering written notice of such exercise to Paragon. If the Company fails to exercise an Option prior to expiration of the applicable Option Period, such Option for such Program will terminate. In consideration for Paragon's grant of the Options to the Company, the Company paid to Paragon a non-refundable, non-creditable one-time fee of \$2.5 million, which was recorded as research and development expense during the three months ended March 31, 2022. In December 2022, the Company and Paragon entered into a first amendment to the Paragon Agreement, under which the Company obtained an additional limited license for the purpose of conducting certain activities. In consideration for the rights and licenses obtained under the first amendment, Viridian paid Paragon a non-refundable fee of \$2.3 million (the "First Amendment Payment"), which was recorded as research and development expense during the three months ended December 31, 2022. The non-refundable upfront fee and the First Amendment Payment are separate from any development costs or cost advance paid or owing with respect to the specified program. During the year ended December 31, 2022, the Company recorded \$5.6 million in research and development costs related to the Paragon Agreement.

As of December 31, 2022, the Paragon Agreement is considered a related party transaction because Fairmount beneficially owns more than 5% of the Company's capital stock and has two seats on the Company's board of directors, and beneficially owns more than 5% of Paragon, which is a joint venture between Fairmount and FairJourney Biologics, and has appointed the sole director on Paragon's board of directors and has the contractual right to approve the appointment of any executive officers.

## 8. COMMITMENTS AND CONTINGENCIES

### *License Agreement with ImmunoGen, Inc.*

In October 2020, the Company became party to a license agreement (the “ImmunoGen License Agreement”) with Immunogen, Inc. (“ImmunoGen”), under which the Company obtained an exclusive, sublicensable, worldwide license to certain patents and other intellectual property rights to develop, manufacture, and commercialize certain products for non-oncology and non-radiopharmaceutical indications. In consideration for rights granted by ImmunoGen, the Company is obligated to make certain future development milestone payments of up to \$48.0 million upon the achievement of specified clinical and regulatory milestones. In December 2021, the Company paid a \$2.5 million milestone payment to ImmunoGen upon the submission of an investigational new drug (“IND”) application for VRDN-001 with the FDA. In May 2022, the Company paid a \$3.0 million milestone payment to ImmunoGen related to the first patient dosed in the clinical trial for VRDN-001. In December 2022, the Company recorded \$10.0 million as research and development expense related to a milestone owed to ImmunoGen related to the first patient dosed in a Pivotal Clinical Trial for VRDN-001, amount which was paid in January 2023 and which was included in accounts payable in the consolidated balance sheet as of December 31, 2022. Additionally, if the Company successfully commercializes any product candidate subject to the ImmunoGen License Agreement, it is responsible for royalty payments equal to a percentage in the mid-single digits of net sales and commercial milestone payments of up to \$95.0 million. The Company is obligated to make any such royalty payments on a product-by-product and country-by-country basis from the first commercial sale of a specified product in each country until the later of (i) the expiration of the last patent claim subject to the ImmunoGen License Agreement in such country, (ii) the expiration of any applicable regulatory exclusivity obtained for each product in such country, or (iii) the 12th anniversary of the date of the first commercial sale of such product in such country.

### *License Agreements with Xencor, Inc.*

In December 2021, the Company entered into a subsequent technology license agreement (the “2021 Xencor License Agreement”) with Xencor for a non-exclusive license to certain antibody libraries developed by Xencor. Under the 2021 Xencor License Agreement, the Company received a one-year research license to review the antibodies and the right to select up to three antibodies for further development. In consideration for rights granted by Xencor, the Company issued 394,737 shares of our common stock to Xencor in December 2021. The shares were valued at \$7.5 million and recorded as research and development expense during the year ended December 31, 2021. Under the terms of the 2021 Xencor License Agreement, if successful, for each licensed product, the Company would be obligated to make future milestone payments of up to \$27.75 million, which includes development milestone payments of up to \$4.75 million, special milestone payments of up to \$3.0 million, and commercial milestone payments of up to \$20.0 million. Additionally, for each licensed product that the Company successfully commercializes, it would be responsible for royalty payments equal to a percentage in the mid-single digits of net sales.

In December 2020, the Company entered into a license agreement (the “Xencor License Agreement”) with Xencor, under which Xencor granted the Company rights to an exclusive, worldwide, sublicensable, non-transferable, royalty-bearing license to use specified Xencor technology for the research, development, manufacturing, and commercialization of therapeutic antibodies targeting IGF-1R indications. In consideration for rights granted by Xencor, the Company issued 322,407 shares of its common stock in December 2020. The shares were valued at \$6.0 million and recorded as research and development expense in 2020. Under the terms of the Xencor License Agreement, the Company is obligated to make future development milestone payments of up to \$30.0 million. Additionally, if the Company successfully commercializes any product candidate subject to the Xencor License Agreement, it is responsible for royalty payments equal to a percentage in the mid-single digits of net sales and commercial milestone payments of up to \$25.0 million. The Company is obligated to make any such royalty payments on a product-by-product and country-by-country basis from the first commercial sale of products containing the licensed technology in each country until the later of (i) the expiration of the last patent claim subject to the Xencor License Agreement in such country, (ii) the expiration of any applicable regulatory exclusivity obtained, or (iii) the 12<sup>th</sup> anniversary of the date of the first commercial sale.

### *Contingent Value Rights Agreement*

In accordance with the merger agreement with miRagen Therapeutics, Inc., on November 4, 2020, the Company and the Rights Agent (as defined therein) executed and delivered a contingent value rights agreement (the “CVR Agreement”), pursuant to which each holder of the Company’s common stock as of November 6, 2020, other than former stockholders of Private Viridian, was entitled to one contractual contingent value right issued by the Company, subject to and in accordance with the terms and conditions of the CVR Agreement, for each share of its common stock held by such holder. No CVRs have been or may in the future be issued pursuant to the terms of the CVR Agreement as of December 31, 2022.

## Lease Obligations

### Colorado-based Office and Lab Space

The Company is party to a multi-year, non-cancelable lease agreement for its Colorado-based office and lab space. The lease agreement includes rent escalation clauses through the lease term and a Company option to extend the lease term for up to three terms of three years each. Minimum base lease payments under the lease agreement, including the impact of tenant improvement allowances, are recognized on a straight-line basis over the full term of the lease. The lease term was amended in March 2021 to extend the lease maturity date to December 31, 2024. Upon adoption of ASC 842 and upon subsequent modification of the lease in 2020 and in March 2021, the Company recognized a right-of-use asset and corresponding lease liability for the lease agreement of approximately \$1.6 million by calculating the present value of lease payments, discounted at 6%, the Company's estimated incremental borrowing rate, over the 12 months expected remaining term.

### Massachusetts-based Office Space

The Company is party to a multi-year, non-cancelable lease agreement for its Massachusetts-based office space (as subsequently amended, the "Original Lease"). The Original Lease included rent escalation clauses through the lease term. Minimum base lease payments under the lease agreement are recognized on a straight-line basis over the full term of the lease. Upon assumption of the Original Lease, the Company recognized a right-of-use asset and corresponding lease liability for the Original Lease of \$0.1 million by calculating the present value of lease payments, discounted at 6%, the Company's estimated incremental borrowing rate, over the expected remaining term. In July 2021, the Company entered into an amendment to the Original Lease to increase its Massachusetts-based office space (the "First Amendment"). In April 2022, the Company entered into a second amendment to the Original Lease of its Massachusetts-based office space (the "Second Amendment"). The Second Amendment made certain modifications to both the Original Lease and First Amendment, including (i) the addition of 2,432 square feet of office space in the same building and (the "April 2022 Expansion Premises"), (ii) the termination of the 1,087 square feet of leased space under the Original Lease seven days after the delivery of the April 2022 Expansion Premises, which occurred in the fourth quarter of 2022, and (iii) the extension of the expiration date of the 3,284 square feet of leased space under the First Amendment from October 2024 through November 2026.

Under the Second Amendment, the Company has the option to extend the lease term for an additional period of three years upon notice to the landlord. The Second Amendment provides for annual base rent for the April 2022 Expansion Premises of approximately \$0.4 million during the lease term. The Company is also obligated to pay the landlord certain costs, taxes and operating expenses.

In July 2022, the Company entered into a third amendment to the Original Lease of its Massachusetts-based office space (the "Third Amendment"). The Third Amendment makes certain modifications to the Original Lease, including (i) the addition of 5,240 square feet of office space in the same building (the "July 2022 Expansion Premises"), (ii) the termination of the 1,087 square feet of leased space under the Original Lease, and (iii) the extension of the expiration date of the 3,284 square feet of leased space under the First Amendment to four years from the delivery of the July 2022 Expansion Premises.

Under the Third Amendment, the Company has the option to extend the lease term for an additional period of three years upon notice to the landlord. The Third Amendment provides for annual base rent for the July 2022 Expansion Premises of approximately \$0.9 million during the lease term. The Company is also obligated to pay the landlord certain costs, taxes and operating expenses.

Future lease payments under noncancellable leases as of December 31, 2022 are as follows:

	(in thousands)	
2023	\$	717
2024		748
2025		245
2026		230
Total future minimum lease payments		1,940
Less: imputed interest		(217)
Total	\$	1,723

As of December 31, 2022, the Company's operating lease obligations were reflected as short-term operating lease liabilities of \$0.6 million within accrued liabilities and \$1.1 million of long-term lease obligations as other liabilities in the Company's consolidated balance sheets.

Amortization of the operating lease right-of-use assets, and corresponding reduction of operating lease obligations, amounted to \$0.5 million for both the years ended December 31, 2022 and 2021, and which was included in operating expense in the consolidated statements of operations and comprehensive loss.

The Company is also required to pay for operating expenses related to the leased space, which were \$0.3 million for both the years ended December 31, 2022 and 2021. The operating expenses are incurred separately and were not included in the present value of lease payments.

## **9. CAPITAL STOCK**

### ***Common Stock***

Under the Company's second restated certificate of incorporation, the Company is authorized to issue 205,000,000 shares of its stock, of which 200,000,000 shares have been designated as common stock and 5,000,000 shares have been designated as preferred stock, both with a par value of \$0.01 per share. The number of authorized shares of common stock may be increased or decreased by the affirmative vote of the holders of a majority of the Company's stock who are entitled to vote. Each share of common stock is entitled to one vote. The holders of common stock are entitled to receive dividends when and as declared or paid by its board of directors.

### ***Public Offerings***

In August 2022, the Company entered into an underwriting agreement (the "2022 Underwriting Agreement") with Jefferies LLC ("Jefferies"), SVB Securities LLC and Evercore Group L.L.C. ("Evercore") relating to the offer and sale (the "2022 Offering") of 11,352,640 shares of the Company's common stock, which includes 1,725,000 shares of common stock issued in connection with the exercise in full by the underwriters of their option to purchase additional shares at a public offering price of \$23.50 per share, and 28,084 shares of Series B Non-Voting Convertible Preferred Stock, par value \$0.01 per share, at a public offering price of \$1,566.745 per share (collectively the "2022 Public Offering"). The aggregate gross proceeds to the Company from the 2022 Public Offering, including the exercise of the option were approximately \$311.0 million, before deducting underwriting discounts and commissions and other offering expenses payable by the Company.

In September 2021, the Company entered into an underwriting agreement (the "2021 Underwriting Agreement") with Jefferies, SVB Leerink LLC and Evercore for the sale and issuance of 7,344,543 shares of common stock, which includes 1,159,089 shares of common stock issued in connection with the exercise in full by the underwriters of their option to purchase additional shares at a public offering price of \$11.00 per share and 23,126 shares of Series B Non-Voting Convertible Preferred Stock at a public offering price of \$733.37 per share (collectively the "2021 Public Offering"). The aggregate gross proceeds to the Company from the 2021 Public Offering, including the exercise of the option, were approximately \$97.7 million, before deducting underwriting discounts and commissions and estimated offering expenses payable by the Company.

### ***Common Stock Sales Agreements - Jefferies LLC***

In September 2022, the Company entered into an Open Market Sale Agreement<sup>SM</sup> (the "September 2022 ATM Agreement") with Jefferies, pursuant to which the Company may offer and sell shares of its common stock having an aggregate offering price of up to \$175.0 million from time to time at prices and on terms to be determined by market conditions at the time of offering, with Jefferies acting as its sales agent. Jefferies will receive a commission of 3.0% of the gross proceeds of any shares of common stock sold under the September 2022 ATM Agreement. During the quarter ended December 31, 2022, 964,357 shares have been sold under the September 2022 ATM Agreement with Jefferies at a weighted average price of \$26.01 per share, for aggregate net proceeds of approximately \$24.2 million, including commissions to Jefferies as a sales agent.

In November 2021, the Company entered into an Open Market Sale Agreement<sup>SM</sup> (the "November 2021 ATM Agreement") with Jefferies, pursuant to which the Company could offer and sell shares of its common stock having an aggregate offering price of up to \$75.0 million from time to time at prices and on terms to be determined by market conditions at the time of offering, with Jefferies acting as its sales agent. The November 2021 ATM Agreement was terminated in connection with the September 2022 ATM Agreement. No shares were sold under the November 2021 ATM Agreement.



In April 2021, the Company entered into an Open Market Sale Agreement<sup>SM</sup> (the “April 2021 ATM Agreement”) with Jefferies pursuant to which the Company could offer and sell shares of its common stock having an aggregate offering price of up to \$50.0 million from time to time at prices and on terms to be determined by market conditions at the time of offering, with Jefferies as its sales agent. Jefferies could receive a commission equal to 3.0% of the gross sales proceeds of any common stock sold through Jefferies under the April 2021 ATM Agreement. The April 2021 ATM Agreement was terminated in connection with the November 2021 ATM Agreement. An aggregate of 2,551,269 shares of common stock were sold pursuant to the April 2021 ATM Agreement prior to its termination, at a volume weighted average price of \$13.13 per share, for aggregate net proceeds of approximately \$32.4 million, including commissions to Jefferies as sales agent.

### ***Common Stock Purchase Agreement - Aspire Capital Fund, LLC***

In December 2019, the Company entered into a common stock purchase agreement (the “Aspire Stock Purchase Agreement”), with Aspire Capital Fund, LLC (“Aspire Capital”), which provides that, subject to the terms, conditions, and limitations set forth therein, Aspire Capital is committed to purchase up to an aggregate of \$20.0 million of shares of common stock over the 30-month term of the Aspire Stock Purchase Agreement.

Through December 31, 2020, the Company sold 412,187 shares of common stock to Aspire Capital at a weighted-average price of \$21.35 per share for aggregate net proceeds of \$8.8 million. The Company did not sell any shares to Aspire Capital during the years ended December 31, 2021 or 2022. As of December 31, 2022, the Company has the ability to sell an additional \$10.2 million of shares of common stock to Aspire Capital. Under the Aspire Stock Purchase Agreement, the Company has the right, in its sole discretion, on any trading day selected by it, and within certain specified limitations, to present Aspire Capital with a purchase notice, directing Aspire Capital (as principal) to purchase up to 13,333 shares of common stock per business day at a per share price equal to the lesser of (i) the lowest sale price of common stock on the purchase date or (ii) the average of the three lowest closing sale prices for the common stock during the 10 consecutive business days ending on the business day immediately preceding the purchase date. The Company also has the right to require Aspire Capital to purchase up to an additional 30% of the trading volume of the shares for the next business day at a purchase price (the “VWAP Purchase Price”), equal to the lesser of: (i) the closing sale price of the shares on the purchase date, or (ii) ninety-seven percent (97%) of the next business day’s volume weighted average-price (each such purchase, a “VWAP Purchase”). The Company shall have the right, in its sole discretion, to determine a maximum number of shares and set a minimum market price threshold for each VWAP Purchase. The Company can only require a VWAP Purchase if the Company has also submitted a regular purchase on the notice date for the VWAP Purchase. There are no limits on the number of VWAP purchases that the Company may require.

The Aspire Stock Purchase Agreement may be terminated by the Company at any time, at the Company’s discretion, without any cost to the Company. There are no limitations on use of proceeds, financial or business covenants, restrictions on future financings, rights of first refusal, participation rights, penalties, or liquidated damages in the Aspire Stock Purchase Agreement.

### ***Preferred Stock***

Under the Company’s second restated certificate of incorporation, the Company’s board of directors has the authority to designate and issue up to 5,000,000 shares of preferred stock, at its discretion, in one or more classes or series and to fix the powers, preferences and rights, and the qualifications, limitations, or restrictions thereof, including dividend rights, conversion rights, voting rights, terms of redemption, and liquidation preferences, without further vote or action by the Company’s stockholders.

#### ***Series A Preferred Stock***

Holders of Series A Preferred Stock are entitled to receive dividends on shares of Series A Preferred Stock equal, on an as-if-converted-to-Common-Stock basis, and in the same form as dividends actually paid on shares of the common stock. Except as otherwise required by law, the Series A Preferred Stock does not have voting rights. However, as long as any shares of Series A Preferred Stock are outstanding, the Company will not, without the affirmative vote of the holders of a majority of the then outstanding shares of the Series A Preferred Stock, (i) alter or change adversely the powers, preferences or rights given to the Series A Preferred Stock, (ii) alter or amend the Certificate of Designation, (iii) amend its certificate of incorporation or other charter documents in any manner that adversely affects any rights of the holders of Series A Preferred Stock, (iv) increase the number of authorized shares of Series A Preferred Stock, (v) at any time while at least 30% of the originally issued Series A Preferred Stock remains issued and outstanding, consummate a Fundamental Transaction (as defined in the Certificate of Designation) or (vi) enter into any agreement with respect to any of the foregoing. The Series A Preferred Stock does not have a preference upon any liquidation, dissolution, or winding-up of the Company. Each share of Series A Preferred Stock is convertible into 66.67 shares of common stock at any time at the option of the holder thereof, subject to certain limitations, including that a holder of Series A Preferred Stock is prohibited from converting shares of Series A Preferred Stock into shares

of common stock if, as a result of such conversion, such holder, together with its affiliates, would beneficially own more than a specified percentage (to be established by the holder between 4.99% and 19.99%) of the total number of shares of common stock issued and outstanding immediately after giving effect to such conversion.

As of December 31, 2022 and 2021, there were 188,381 and 260,437 shares of Series A Preferred Stock outstanding, respectively.

### *Series B Preferred Stock*

Each share of Series B Preferred Stock is convertible into 66.67 shares of common stock, subject to certain limitations, including that a holder of Series B Preferred Stock is prohibited from converting shares of Series B Preferred Stock into shares of common stock if, as a result of such conversion, such holder, together with its affiliates, would beneficially own more than a specified percentage (to be established by the holder between 4.9% and 19.9%) of the total number of shares of common stock issued and outstanding immediately after giving effect to such conversion. The powers, preferences, rights, qualifications, limitations, and restrictions applicable to the Series B Preferred Stock are set forth in the Certificate of Designation filed in connection with the Offering.

Holders of Series B Preferred Stock are entitled to receive dividends on shares of Series B Preferred Stock equal, on an as-if-converted-to-Common-Stock basis, and in the same form as dividends actually paid on shares of the common stock. Except as otherwise required by law, the Series B Preferred Stock does not have voting rights. However, as long as any shares of Series B Preferred Stock are outstanding, the Company will not, without the affirmative vote of the holders of a majority of the then outstanding shares of the Series B Preferred Stock, (i) alter or change adversely the powers, preferences or rights given to the Series B Preferred Stock, (ii) alter or amend the Certificate of Designation, or (iii) amend its certificate of incorporation or other charter documents in any manner that adversely affects any rights of the holders of Series B Preferred Stock. The Series B Preferred Stock does not have a preference upon any liquidation, dissolution, or winding-up of the Company. As of December 31, 2022 and 2021, there were 51,210 and 23,126 shares of Series B Preferred Stock outstanding, respectively.

## 10. WARRANTS

The following table presents information about the Company's outstanding warrants:

	Number of Underlying Shares (1)		Weighted-Average Exercise Price at December 31, 2022	Remaining Contractual Life at December 31, 2022 (No. Years)
	December 31,			
	2022	2021		
<b>Liability-classified warrants</b>				
Issued April 2017	781	781	\$127.95	2.33
<b>Equity-classified warrants</b>				
Acquired October 2020	29,446	29,446	\$0.01	7.73
Issued February 2020 (2)	332,130	388,796	\$15.09	2.12
Issued November 2017	1,606	1,606	\$0.47	1.87
Subtotal	363,182	419,848	\$15.57	
Total warrants	363,963	420,629	\$15.82	

- (1) If the Company subdivides (by any stock split, stock dividend, recapitalization, or otherwise) its outstanding shares of its common stock into a smaller number of shares, the warrant exercise price is proportionately reduced and the number of shares under outstanding warrants is proportionately increased. Additionally, if the Company combines (by combination, reverse stock split, or otherwise) its outstanding shares of common stock into a smaller number of shares, the warrant exercise price is proportionately increased and the number of shares under outstanding warrants is proportionately decreased.
- (2) Subject to specified conditions, the Company may voluntarily reduce the warrant exercise price of the warrants issued in February 2020.

A summary of the Company’s warrant activity during the year ended December 31, 2022 is as follows:

	Common Stock Warrants	
	Number	Weighted-Average Exercise Price
Outstanding at December 31, 2021	420,629	\$15.82
Exercised	(56,666)	\$16.50
Outstanding at December 31, 2022	363,963	\$15.82

## 11. SHARE-BASED COMPENSATION

### *Equity Incentive Plans*

The Company has grants outstanding under its 2008 Equity Incentive Plan (the “2008 Plan”), its amended and restated 2016 Equity Incentive Plan (the “2016 Plan”), and the Viridian 2020 Equity Incentive Plan (the “2020 Plan” and collectively with the 2008 Plan and the 2016 Plan, the “Equity Incentive Plans”). Additionally, beginning in July 2021, the Company granted stock options outside of its Equity Incentive Plans to certain employees to induce them to accept employment with the Company (the “Inducement Awards”). The terms and conditions of the Inducement Awards are substantially similar to those awards granted under the Company’s Equity Incentive Plans.

On June 8, 2022 (the “Effective Date”), the Company’s stockholders approved the amendment and restatement of the 2016 Plan (the “Amended 2016 Plan”), which increased the number of shares reserved for issuance under the Amended 2016 Plan by 3,050,000 shares and transferred the number of shares that remain available for issuance under the 2020 Plan as of the Effective Date into the Amended 2016 Plan so that the Company operates from a single equity plan going forward. The term of the Amended 2016 Plan is 10 years following the Effective Date.

As of December 31, 2022, the Company had the following balances by plan:

	Stock Options Outstanding	Shares Available for Issuance
Inducement Awards	1,513,174	—
2020 Plan	930,332	—
2016 Plan	3,278,757	3,781,888
2008 Plan	186	—
Total	5,722,449	3,781,888

Options granted under the Equity Incentive Plans and the Inducement Awards have an exercise price equal to the market value of the common stock at the date of grant and expire 10 years from the date of grant. Generally, options vest 25% on the first anniversary of the vesting commencement date and 75% ratably in equal monthly installments over the remaining 36 months. The Company has also granted options that vest in equal monthly or quarterly amounts over periods up to 48 months.

The Company does not currently hold any treasury shares. Upon stock option exercise, the Company issues new shares and delivers them to the participant.

A summary of common stock option activity is as follows:

	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2021	3,683,839	\$ 16.94	8.94	\$ 15,969
Granted	2,545,500	17.72		
Exercised	(191,291)	14.44		
Forfeited or expired	(315,599)	19.48		
Outstanding as of December 31, 2022	5,722,449	17.23	8.64	\$ 68,977
Vested or expected to vest as of December 31, 2022	5,722,449	\$ 17.23	8.64	\$ 68,977
Exercisable as of December 31, 2022	1,697,616	\$ 16.95	8.07	\$ 21,258
Vested as of December 31, 2022	1,697,616	\$ 16.95	8.07	\$ 21,258

### ***Fair Value Assumptions***

The Company uses the Black-Scholes option pricing model to estimate the fair value of stock options granted under its equity compensation plans. The Black-Scholes model requires inputs for risk-free interest rate, dividend yield, volatility, and expected lives of the options. Because the Company has a limited history of stock purchase and sale activity, expected volatility is based on a blend of historical data from public companies that are similar to the Company in size and nature of operations, as well as the Company's own volatility. The Company will continue to use similar entity volatility information until its historical volatility is relevant to measure expected volatility for option grants. The Company accounts for forfeitures as they occur. The risk-free rate for periods within the contractual life of each option is based on the U.S. Treasury yield curve in effect at the time of the grant for a period commensurate with the expected term of the grant. The expected term (without regard to forfeitures) for options granted represents the period of time that options granted are expected to be outstanding and is derived from the contractual terms of the options granted, and actual and expected option-exercise behaviors. The fair value of the underlying common stock is based on the closing price of the common stock on The Nasdaq Capital Market at the date of grant.

The weighted-average grant-date fair value of options granted to the Company's employees and members of its board of directors during the years ended December 31, 2022 and 2021 was \$12.95 and \$17.50, respectively. The fair value was determined by the Black-Scholes option pricing model using the following weighted-average assumptions:

	Year Ended December 31,	
	2022	2021
Expected term, in years	5.81	5.92
Expected volatility	87.8 %	117.7 %
Risk-free interest rate	2.5 %	0.8 %
Expected dividend yield	— %	— %
Weighted average exercise price	\$ 17.72	\$ 20.56

### ***Employee Stock Purchase Plan***

The 2016 Employee Stock Purchase Plan ("ESPP") allows qualified employees to purchase shares of common stock at a price equal to 85% of the lower of: (i) the closing price at the beginning of the offering period or (ii) the closing price at the end of the offering period. New six-month offering periods begin each August 22 and February 22. As of December 31, 2022, the Company had 309,541 shares available for issuance, and 27,074 cumulative shares had been issued under the ESPP.

### Share-Based Compensation Expense

Share-based compensation related to all equity awards issued pursuant to the Equity Incentive Plans and for estimated shares to be issued under the ESPP for the purchase periods active during each respective period is included in the consolidated statements of operations and comprehensive loss as follows:

	Year Ended December 31,	
	2022	2021
	(in thousands)	
Research and development	\$ 7,298	\$ 4,701
General and administrative	12,467	9,764
Total share-based compensation expense	<u>\$ 19,765</u>	<u>\$ 14,465</u>

As of December 31, 2022, the Company had \$54.9 million of total unrecognized employee and non-employee share-based compensation costs, which the Company expects to recognize over a weighted-average remaining period of 2.77 years.

### 12. NET LOSS PER SHARE

Basic net loss per share is computed by dividing the net loss available to common stockholders by the weighted-average number of common stock outstanding. Diluted net loss per share is computed similarly to basic net loss per share except that the denominator is increased to include the number of additional shares of common stock that would have been outstanding if the potential shares of common stock had been issued and if the additional shares of common stock were dilutive. Diluted net loss per share is the same as basic net loss per share of common stock, as the effects of potentially dilutive securities are antidilutive.

Potentially dilutive securities include the following:

	December 31,	
	2022	2021
	(in thousands)	
Series A Preferred Stock, as converted to shares of common stock	12,559	17,363
Series B Preferred Stock, as converted to shares of common stock	3,414	1,542
Options to purchase common stock	5,722	3,684
Warrants to purchase common stock	364	421
Total	<u>22,059</u>	<u>23,010</u>

### 13. INCOME TAXES

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to reverse.

Since its inception, the Company has incurred net taxable losses, and accordingly, no current provision for income taxes has been recorded. This amount differs from the amount computed by applying the U.S. federal income tax rate of 21% to pretax loss due to the provision of a valuation allowance to the extent of the Company's net deferred tax asset, as well as to state income taxes and nondeductible expenses.



The effective income tax rate of the provision for income taxes differs from the federal statutory rate as follows:

	Year Ended December 31,	
	2022	2021
Federal statutory income tax rate	21.0 %	21.0 %
Federal and state tax credits	1.6	1.5
State income taxes, net of federal benefit	4.2	8.3
Change in valuation allowance	(24.5)	(28.5)
Other permanent items	(0.8)	—
Section 382 limit	—	—
Stock-based compensation	(1.5)	(2.3)
Effective income tax rate	— %	— %

The tax effects of temporary differences that give rise to significant portions of the deferred income tax assets and liabilities are presented below:

	Year Ended December 31,	
	2022	2021
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	\$ 46,000	\$ 40,992
Tax credits	3,803	1,686
Accruals and reserves	4,986	3,055
Stock-based expense	4,161	3,123
Start-up costs and amortized costs	9,460	5,013
IRC § 174 capitalized costs	18,252	—
Operating lease right-of-use asset, net	22	—
Total deferred tax assets	86,684	53,869
Valuation allowance	(86,602)	(53,430)
Net deferred tax assets	82	439
Deferred tax liabilities:		
Unrealized gains/losses	(82)	—
Operating lease right-of-use asset, net	—	(439)
Total deferred tax liabilities	(82)	(439)
Total deferred tax assets, net	\$ —	\$ —

At December 31, 2022, the Company had approximately \$175.6 million and \$3.5 million of federal net operating loss and research and experimentation tax carryforwards, respectively, which will begin to expire in 2029. At December 31, 2022, the Company had approximately \$196.7 million of state net operating loss carryforwards which will begin to expire in 2030. In addition, the realization of net operating losses to offset potential future taxable income and related income taxes that would otherwise be due is subject to annual limitations under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the “Code”), and similar state provisions, which may result in the expiration of additional net operating losses before future utilization as a result of ownership changes. As a result of these ownership change provisions during 2020, the Company estimated an aggregate limitation on the utilization of net operating loss carryforwards of \$59.0 million. In addition to the limitation of net operating losses of \$59.0 million, approximately \$15.3 million of research and development tax credits were derecognized with the inability of the Company to ever realize a benefit from those credits in the future. The Company determines on an annual basis whether net operating loss carryforwards will be limited. An IRC 382 analysis has been completed through December 31, 2022 and determined that there were no additional ownership changes. The Company will continue to evaluate changes in ownership and the related limitations on a go forward basis.

As of December 31, 2022 and 2021, the Company’s net deferred tax assets before valuation allowance was \$86.6 million and \$53.4 million, respectively. In assessing the realizability of its deferred tax assets, the Company considers whether it is more likely than not that some portion or all of its deferred tax assets will not be realized. The ultimate realization of deferred tax

assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. The Company considers the scheduled reversal of deferred tax liabilities, projected future taxable income, and tax planning strategies in making this assessment. As the Company does not have any historical taxable income or projections of future taxable income over the periods in which the deferred tax assets are deductible, and after consideration of its history of operating losses, the Company does not believe it is more likely than not that it will realize the benefits of its net deferred tax assets, and accordingly, has established a valuation allowance equal to 100% of its net deferred tax assets at December 31, 2022 and 2021. The change in valuation allowance was an increase of \$33.2 million in 2022 and an increase of \$24.4 million in 2021.

The Company concluded that there were no significant uncertain tax positions relevant to the jurisdictions where it is required to file income tax returns requiring recognition in the consolidated financial statements for the years ended 2022 and 2021. As of December 31, 2022 and 2021, the Company had no accrued interest related to uncertain tax positions.

The Company's federal and state returns for 2018 through 2022 remain open to examination by tax authorities.

#### **14. SUBSEQUENT EVENTS**

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.

### VIRIDIAN THERAPEUTICS, INC.

Date: March 9, 2023

By: /s/ Scott Myers

Scott Myers

President, Chief Executive Officer, and Director  
(Principal Executive Officer)

Date: March 9, 2023

By: /s/ Kristian Humer

Kristian Humer

Chief Financial Officer

(Principal Financial Officer; Principal Accounting Officer)

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Scott Myers and Kristian Humer, and each of them, as his or her attorneys-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to this Annual Report on Form 10-K, and to file the same, with exhibits thereto and other documents in connection therewith with the Securities and Exchange Commission, hereby ratifying and confirming all that said attorneys-in-fact, and each of them, or his substitute or substitutes may do or cause to be done by virtue hereof.

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.

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ Scott Myers</u> Scott Myers	President, Chief Executive Officer, and Director <i>(Principal Executive Officer)</i>	March 9, 2023
<u>/s/ Kristian Humer</u> Kristian Humer	Chief Financial Officer <i>(Principal Financial Officer; Principal Accounting Officer)</i>	March 9, 2023
<u>/s/ Tomas Kiselak</u> Tomas Kiselak	Chairman of the Board	March 9, 2023
<u>/s/ Peter Harwin</u> Peter Harwin	Director	March 9, 2023
<u>/s/ Arlene Morris</u> Arlene Morris	Director	March 9, 2023
<u>/s/ Jennifer Moses</u> Jennifer Moses	Director	March 9, 2023









## OFFICERS & DIRECTORS

---

### LEADERSHIP

**Scott Myers, M.B.A.**  
*President and  
Chief Executive Officer*

**Barrett Katz, M.D., M.B.A.**  
*Chief Medical Officer*

**Bernie Huyghe, Ph.D.**  
*SVP, Technical Operations*

**Deepa Rajagopalan,  
M.D., M.B.A.**  
*Chief Product  
and Strategy Officer*

**Helen Milton, Ph.D.**  
*SVP, Regulatory & Quality*

**Janielle Newland**  
*Chief Administration Officer*

**Kristian Humer, M.B.A.**  
*Chief Financial Officer  
and Chief Business Officer*

**Lara Meisner, J.D.**  
*Chief Legal Officer*

**Rob Henderson, Ph.D.**  
*Chief Scientific Officer*

**Todd James**  
*SVP, Corporate Affairs  
and Investor Relations*

**Tony Casciano**  
*Chief Commercial Officer*

**Vahe Bedian, Ph.D.**  
*Co-Founder  
and Scientific Advisor*

### BOARD OF DIRECTORS

**Tomas Kiselak**  
**Chairman**  
*Managing Member,  
Fairmount Funds  
Management LLC*

**Arlene Morris**  
**Director**  
*Chief Executive Officer,  
Willow Advisors*

**Jennifer Moses**  
**Director**  
*Chief Financial Officer,  
Investors Management  
Corporation*

**Peter Harwin**  
**Director**  
*Managing Member,  
Fairmount Funds  
Management LLC*

**Scott Myers, M.B.A.**  
**Director**  
*President and  
Chief Executive Officer,  
Viridian Therapeutics*

## CORPORATE INFORMATION

---

### Annual Meeting

The Annual Meeting of Stockholders will be held at 10:00 a.m. ET on June 14, 2023 at [virtualshareholdermeeting.com/VRDN2023](https://virtualshareholdermeeting.com/VRDN2023).

### Independent Auditors

KPMG LLP; Boulder, CO

### Investor Inquires

[IR@viridiantherapeutics.com](mailto:IR@viridiantherapeutics.com)

### Transfer Agent

Vstock Transfer  
18 Lafayette Place  
Woodmere, NY 11598

Phone: 212-828-8436  
Toll-Free: 855-9VSTOCK  
Fax: 646-536-3179  
Email: [info@vstocktransfer.com](mailto:info@vstocktransfer.com)

### Stock Listing

NASDAQ: VRDN

### Annual Report on Form-10K

A copy of our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) is available free of charge on the SEC's website at [www.sec.gov](http://www.sec.gov) and in the "Filings" tab of the "Investors" section of the Company's website at [www.viridiantherapeutics.com](http://www.viridiantherapeutics.com).



221 Crescent Street, Suite 401  
Waltham, MA 02453  
T: 617.272.4600  
[viridiantherapeutics.com](http://viridiantherapeutics.com)