

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**  
**WASHINGTON, D.C. 20549**

**FORM 10-K**

**ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**  
**For the Fiscal Year Ended December 31, 2025**

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 000-19319

**Vertex Pharmaceuticals Incorporated**

(Exact name of registrant as specified in its charter)

**Massachusetts**

(State or other jurisdiction of incorporation or organization)

**50 Northern Avenue, Boston, Massachusetts**

(Address of principal executive offices)

**04-3039129**

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

**02210**

(Zip Code)

Registrant's telephone number, including area code **(617) 341-6100**

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

<u>Title of Each Class</u>	<u>Trading Symbol</u>	<u>Name of Each Exchange on Which Registered</u>
Common Stock, \$0.01 Par Value Per Share	VRTX	The Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Exchange 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 Exchange 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 (Check one):

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

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

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

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

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

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

The aggregate market value of the registrant's common stock held by non-affiliates of the registrant based on the closing price on June 30, 2025 (the last business day of the registrant's most recently completed second fiscal quarter of 2025) was \$113.4 billion.

As of February 6, 2026, the registrant had 254,034,190 shares of common stock outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Portions of the definitive proxy statement for the 2026 Annual Meeting of Shareholders, which we expect to hold on May 13, 2026, are incorporated by reference into Part III of this Annual Report on Form 10-K.



**VERTEX PHARMACEUTICALS INCORPORATED**  
**ANNUAL REPORT ON FORM 10-K**  
**TABLE OF CONTENTS**

<b>PART I</b>		
Item 1.	Business	1
	Information about our Executive Officers	21
Item 1A.	Risk Factors	24
Item 1B.	Unresolved Staff Comments	39
Item 1C.	Cybersecurity	39
Item 2.	Properties	40
Item 3.	Legal Proceedings	41
Item 4.	Mine Safety Disclosures	41
<b>PART II</b>		
Item 5.	Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities	42
Item 6.	[Reserved]	43
Item 7.	Management’s Discussion and Analysis of Financial Condition and Results of Operations	43
Item 7A.	Quantitative and Qualitative Disclosures About Market Risk	61
Item 8.	Financial Statements and Supplementary Data	62
Item 9.	Changes in and Disagreements with Accountants on Accounting and Financial Disclosure	62
Item 9A.	Controls and Procedures	62
Item 9B.	Other Information	65
Item 9C.	Disclosure Regarding Foreign Jurisdictions that Prevent Inspections	65
<b>PART III</b>		
Item 10.	Directors, Executive Officers and Corporate Governance	66
Item 11.	Executive Compensation	66
Item 12.	Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters	66
Item 13.	Certain Relationships and Related Transactions, and Director Independence	66
Item 14.	Principal Accountant Fees and Services	66
<b>PART IV</b>		
Item 15.	Exhibits and Financial Statement Schedules	67
Item 16.	Form 10-K Summary	70
	Signatures	71

“Vertex,” “we,” “us” and “our” as used in this Annual Report on Form 10-K refer to Vertex Pharmaceuticals Incorporated, a Massachusetts corporation, and its subsidiaries.

“VERTEX<sup>®</sup>,” “KALYDECO<sup>®</sup>,” “ORKAMBI<sup>®</sup>,” “SYMDEKO<sup>®</sup>,” “SYMKEVI<sup>®</sup>,” “TRIKAFTA<sup>®</sup>,” “KAFTRIO<sup>®</sup>,” “CASGEVY<sup>®</sup>,” “ALYFTREK<sup>®</sup>,” and “JOURNAVX<sup>®</sup>” are registered trademarks of Vertex. Other brands, names and trademarks contained in this Annual Report on Form 10-K are the property of their respective owners.

We use the brand name for our products when we refer to the product that has been approved and with respect to the indications on the approved label. Otherwise, we refer to our product candidates by their scientific (or generic) name or VX developmental designation.

This Annual Report on Form 10-K contains forward-looking statements. Words such as “anticipates,” “may,” “forecasts,” “expects,” “intends,” “plans,” “potentially,” “believes,” “seeks,” “estimates,” variations of such words and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. Please refer to “Special Note Regarding Forward-Looking Statements” set forth in Part I, Item 1A, for a discussion of our forward-looking statements and the related risks and uncertainties of such statements.

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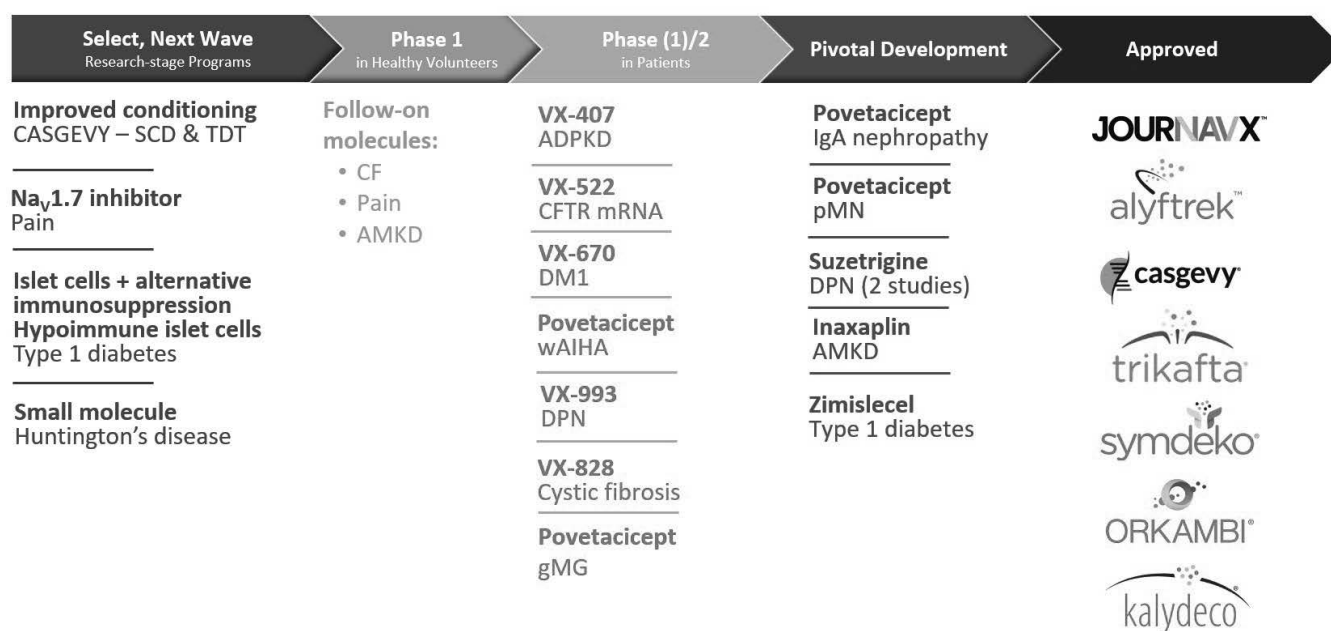
## PART I

### ITEM 1. BUSINESS

#### OVERVIEW

We are a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases, with a focus on specialty markets. We have approved medicines for cystic fibrosis (“CF”), sickle cell disease (“SCD”), transfusion dependent beta thalassemia (“TDT”), and acute pain, and we continue to serially innovate and advance next-generation clinical and research programs in these areas. Our mid- and late-stage clinical pipeline includes programs across a range of modalities in additional serious diseases, including IgA nephropathy, APOL1-mediated kidney disease, neuropathic pain, type 1 diabetes, primary membranous nephropathy, autosomal dominant polycystic kidney disease, and myotonic dystrophy type 1.

The following chart sets forth our approved products, clinical-stage programs, and select pre-clinical programs:



We are advancing five pivotal programs across multiple disease areas:

- **IgA Nephropathy.** We are developing povetacept, a dual inhibitor of the B cell activating factor (“BAFF”) and a proliferation-inducing ligand (“APRIL”) pathways, as a potentially best-in-class approach to treat IgA nephropathy (“IgAN”), a serious, progressive, life-threatening kidney disease that often progresses to end-stage renal disease. We completed enrollment in the IgAN Phase 3 clinical trial and submitted the first module of the rolling Biologics Licensing Application (“BLA”) for povetacept in IgAN in the fourth quarter of 2025. We expect to complete the submission for potential accelerated approval in the U.S. in the first half of 2026.
- **APOL1-Mediated Kidney Disease.** We are developing inaxaplin, a small molecule inhibitor of APOL1 as a potential first-in-class treatment for APOL1-mediated kidney disease (“AMKD”). We have completed the enrollment of the interim analysis cohort of the Phase 2/3 clinical trial and will conduct the pre-planned interim analysis once this cohort reaches 48 weeks of treatment. We expect to share data from the interim analysis in late 2026 or early 2027.
- **Peripheral Neuropathic Pain.** We are developing suzetrigine, a selective non-opioid NaV1.8 pain signal inhibitor, for diabetic peripheral neuropathy (“DPN”), a common form of peripheral neuropathic pain. We are evaluating suzetrigine for the treatment of DPN in two Phase 3 clinical trials. We expect to complete enrollment in both Phase 3 clinical trials by the end of 2026.








- *Type 1 Diabetes.* Zimislecel is an allogeneic stem-cell derived, fully differentiated islet cell therapy in pivotal development for the treatment of type 1 diabetes (“T1D”). We have completed enrollment in the Phase 1/2/3 clinical trial of zimislecel in people with T1D. We have temporarily postponed completion of the dosing in this clinical trial, pending an ongoing internal manufacturing analysis.
- *Primary Membranous Nephropathy.* We are also developing povetacicept to treat primary membranous nephropathy (“pMN”), a rare and serious autoimmune glomerular disease that can lead to kidney damage and renal failure, and which has no treatments specifically approved for this condition. We continue to enroll and dose patients in the adaptive Phase 2/3 pivotal trial in people with pMN. We expect to complete the Phase 2 portion of the clinical trial and to initiate the Phase 3 portion in mid-2026.

Our core strategy is to discover, develop, and commercialize innovative medicines by combining transformative advances in the understanding of human disease and the science of therapeutics, to dramatically advance human health. We focus on validated targets that address causal human biology, predictive lab assays and clinical biomarkers, rapid paths to registration and approval, and product candidates that hold the potential for transformative patient benefit. Our approach includes advancing multiple compounds or therapies from each program into early clinical trials to obtain patient data that can inform selection of the most promising therapies for later stage development as well as inform our ongoing discovery and development efforts. We aim to serially innovate in our disease areas of interest and follow our first-in-class therapies with potential best-in-class candidates. We plan to continue investing to advance our strategy, fostering scientific innovation by identifying additional product candidates through internal research efforts, and investing in business development transactions to access emerging technologies, products and product candidates.

Our serial innovation approach is intended to increase the likelihood of successfully bringing transformative medicines to patients and to provide durable clinical and commercial success. We are working to ensure broad access for eligible patients with these conditions in all countries with regulatory approval. Within our clinical pipeline, we are rapidly progressing multiple programs into pivotal development. We maintain a strong financial profile as we continue to invest in our serial innovation strategy, launch new products, advance our diverse pipeline, and expand geographically.

## MARKETED PRODUCTS

Information regarding our marketed products, including information regarding the disease area, initial approval and age group for which the therapy is approved, are set forth in the table below.

Disease	Initial Approval	Eligible Age Group <sup>(1)</sup>
<b><i>Cystic Fibrosis</i></b>		
	2024	6 years of age and older
	2019	2 years of age and older
	2018	6 years of age and older
	2015	1 year of age and older
	2012	1 month of age and older
<b><i>Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia</i></b>		
	2023	12 years of age and older
<b><i>Acute Pain</i></b>		
	2025	Adults

<sup>(1)</sup> Specifies the youngest eligible age group in any major market.

### CF

CF is a life-shortening genetic disease caused by a defective or missing cystic fibrosis transmembrane conductance receptor (“CFTR”) protein resulting from mutations in the CFTR gene. The absence of working CFTR protein results in poor flow of salt and water into and out of cells in a number of organs, including the lungs, where mucus builds up, causing chronic lung infections and progressive lung damage. Our CFTR modulators, including ivacaftor, deutivacaftor, lumacaftor, tezacaftor, elexacaftor, and vanzacaftor, target the underlying cause of disease by improving CFTR protein function, and as such have been shown to provide transformative benefit for people living with CF.

Our marketed CF medicines, ALYFTREK (vanzacaftor/tezacaftor/deutivacaftor), TRIKAFTA/KAFTRIO (elexacaftor/tezacaftor/ivacaftor and ivacaftor), SYMDEKO/SYMKEVI (tezacaftor/ivacaftor and ivacaftor), ORKAMBI (lumacaftor/ivacaftor) and KALYDECO (ivacaftor), are being used by nearly three quarters of the approximately 97,000 people with CF in the U.S., Europe, Australia, and Canada. We estimate that there are approximately 112,000 people with CF in all target markets.

Our CF medicines are reimbursed or accessible in more than 60 countries across six continents. ALYFTREK, our most-recently approved triple combination CF medicine, has the benefit of a once-daily dosing regimen and demonstrated non-inferiority to TRIKAFTA in ppFEV<sub>1</sub>, a measure of lung function, and an improvement in sweat chloride levels as compared to TRIKAFTA. We expect that the majority of people with CF will transition to ALYFTREK over time.

### ***Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia***

SCD and TDT are hemoglobinopathies, a group of inherited blood disorders that result from gene mutations that alter hemoglobin, a protein in red blood cells that delivers oxygen throughout the body.

SCD is caused by the change of a single amino acid in the  $\beta$ -hemoglobin gene that causes red cells to change shape in settings of low oxygen. These sickled cells block blood flow and can lead to severe pain (known as vaso-occlusive crises), organ damage, and shortened life span. Treatment is typically focused on relieving pain and minimizing organ damage, requiring medication and, for some patients, monthly blood transfusions and frequent hospital visits.

Beta thalassemia is caused by loss-of-function mutations in the same  $\beta$ -hemoglobin gene that lead to severe anemia in patients, which causes fatigue and shortness of breath. In infants, beta thalassemia causes failure to thrive, jaundice, and feeding problems. Complications of beta thalassemia can lead to an enlarged spleen, liver and/or heart, misshapen bones and delayed puberty. Treatment for beta thalassemia varies depending on the disease severity for each patient. People with TDT, the most severe form of the disease, require regular blood transfusions, as frequently as every two to four weeks. Repeated blood transfusions eventually cause an unhealthy buildup of iron in the patient, leading to organ damage.

CASGEVY (exagamglogene autotemcel), our ex-vivo, non-viral CRISPR/Cas9-based gene-editing therapy for severe SCD and TDT, is approved in the U.S. and across multiple geographies including Europe, Canada, and the Middle East. We estimate approximately 60,000 people with severe SCD or TDT are or could become eligible for CASGEVY in these geographies. To receive CASGEVY, patients first undergo a treatment at an authorized treatment center (“ATC”) that mobilizes a population of hematopoietic stem and progenitor cells (“HSPC”) from the bone marrow into the bloodstream. These cells are collected from the patient’s bloodstream and transferred to a manufacturing facility where the HSPCs are isolated and CRISPR/Cas9 gene-editing is performed on the cells. The gene-editing procedure results in a precise and specific gene-edit in a non-coding intron of the BCL11A gene. Following manufacturing, the edited cells, now called CASGEVY, are transferred back to the ATC. Patients are preconditioned with a myeloablative conditioning treatment that ablates their bone marrow to create space for the edited cells. After CASGEVY is infused into the patient and the edited cells engraft, the levels of fetal hemoglobin erythrocytes increase, thereby reducing or eliminating symptoms associated with disease. Efficacy data support the profile of CASGEVY as a potential one-time functional cure for people with severe SCD and TDT.

CASGEVY is broadly reimbursed by third-party payors in the U.S., including the federal government and commercial payors. In addition, we have agreements with national and regional payors covering more than 275 million lives, to provide access to CASGEVY. Outside of the U.S., patients have access to CASGEVY in Austria, Denmark, the U.K., Italy, Luxembourg, Bahrain, Saudi Arabia, the UAE, and Kuwait. We continue to expand access and pursue additional long-term reimbursement arrangements and to engage with payors in the E.U. and the Middle East.

Globally in 2025, approximately 300 people with SCD or TDT initiated treatment with CASGEVY, 147 people had their first cell collection for CASGEVY, and 64 people received infusions of CASGEVY. In 2026, we expect to reach more eligible patients and drive patient infusions through our global ATC network.

### ***Acute Pain***

Acute pain is a disabling condition that may occur suddenly but typically lasts less than 90 days and resolves in days or weeks (for example, following surgery or an injury). It is estimated that over 80 million people are prescribed a medicine for acute pain every year in the U.S. Currently available treatments have limitations around efficacy or side effects, including a risk of addiction with opioids. Because of these challenges, over- and under-utilization, as well as misutilization, of current pain medicines may occur.

JOURNAVX (suzetrigine) is a first-in-class, oral pain signal inhibitor that is highly selective for voltage-gated sodium channel NaV1.8. Through this mechanism, JOURNAVX provides effective relief of pain without evidence of the several limitations of other currently available therapies, including the addictive potential of opioids. JOURNAVX was approved by the U.S. Food and Drug Administration (“FDA”) in January 2025 for the treatment of moderate-to-severe acute pain in adults.

Since JOURNAVX became available at U.S. pharmacies in March 2025 and through the end of 2025, more than 550,000 prescriptions were written and filled across the hospital and retail settings in different acute pain conditions, consistent with the product's broad label. We have secured access for JOURNAVX with all three national pharmacy benefit managers, and as of January 2026, more than 200 million individuals across commercial and government payors have coverage to JOURNAVX, representing two-thirds of U.S. covered lives. In addition, 21 states provide coverage via Medicaid.

## **COMMERCIALIZATION OF OUR MEDICINES**

We sell our medicines primarily to a limited number of specialty pharmacy and specialty distributors globally, as well as to certain major wholesalers in the U.S. Our customers in the U.S. subsequently resell our medicines to patients, health care providers, retail pharmacies, hospitals, or ATCs. Outside of the U.S., we generate sales primarily through distributor arrangements and to retail pharmacies, as well as to hospitals and clinics, many of which are government-owned or supported customers. In certain markets, we may not utilize a specialty distributor or specialty pharmacy to distribute CASGEVY and instead may sell CASGEVY directly to ATCs. We contract with government agencies so that our medicines will be eligible for purchase by, or partial or full reimbursement from, such third-party payors.

We promote the use of our medicines directly to healthcare professionals and organizations such as doctors, nurse practitioners, physician assistants, pharmacists, hospitals, and pharmacy benefit managers. Through our field sales and medical organizations, we explain the risks and benefits of our medicines to these healthcare professionals and organizations. Our marketing is limited to the approved uses of the particular medicine. We also continue to develop scientific data and other information about potential additional uses of our medicines and provide such information through clinical or medical affairs teams as scientific exchange at scientific congresses or in other ways, including the development of publications, or in response to unsolicited inquiries from healthcare professionals and organizations. In the U.S., we also market directly to consumers by communicating the approved uses, benefits and risks.

We are dedicated to helping patients obtain access to our therapies. We work to gain access for our medicines on formularies and reimbursement plans (lists of formulary-recommended or approved medicines and other products) by providing information about the clinical profiles of our medicines. Our patient support representatives help patients understand their insurance coverage and, in the U.S., we have established programs that provide co-pay assistance or free medicine for qualified uninsured or underinsured patients, based on specific eligibility criteria.

## **RESEARCH AND DEVELOPMENT PROGRAMS**

We invest in research and development to discover and develop transformative medicines for people with serious diseases, with a focus on specialty markets. Our research strategy is to combine transformative advances in the understanding of human disease and in the science of therapeutics to dramatically advance human health. We focus on:

- disease areas with known causal human biology;
- targets validated by causal human biology;
- predictive lab assays and clinical biomarkers;
- potential for transformative benefit regardless of modality; and
- efficient path to registration and approval.

Our development-stage product candidates are focused on the treatment of serious diseases. In pursuit of serial innovation, our research and development approach includes advancing multiple candidates into clinical trials and pursuing multiple modalities with the goal of bringing first-in-class and/or best-in-class therapies to patients.

Our research and development strategy has been validated through our success in moving novel product candidates into clinical trials and obtaining marketing approvals for our five CF medicines, CASGEVY, and JOURNAVX. Our approach to drug discovery has been further validated by ongoing pivotal development in five additional disease areas: in IgAN and pMN with povetacept, in AMKD with inaxaplin, in T1D with zimislecel, and in diabetic peripheral neuropathy with suzetrigine.

To augment our internal programs, we acquire businesses and technologies and collaborate with biopharmaceutical and technology companies, leading academic research institutions, government laboratories, foundations and other organizations to advance research in our disease areas of interest, as well as to access technologies needed to execute on our strategy. Our

internal and external innovation approaches are based on the same strategy, which enables us to effectively integrate and execute on new internal capabilities as we invest in external innovation. Our investments in external innovation include our collaboration with CRISPR, which resulted in the successful development and approval of CASGEVY; our acquisition of Semma Therapeutics, Inc. (“Semma”), which established and advanced our T1D program; our expansion of our renal programs through our acquisition of Alpine Immune Sciences, Inc. (“Alpine”); our mRNA therapeutic, VX-522, for treatment of CF through our collaboration with Moderna; and our intracellular therapeutic for myotonic dystrophy type 1 (“DM1”), VX-670, through our collaboration with Entrada.

## **CF**

Our goal in CF is to continue to extend our leadership by developing treatment regimens that will provide benefits to all people with CF. We have completed the Phase 3 clinical trial evaluating TRIKAFTA/KAFTRIO in children one year to less than two years of age. The data showed that TRIKAFTA was generally safe and well-tolerated, consistent with the established safety profile. Treatment with TRIKAFTA in this age group resulted in rapid, robust, and clinically meaningful improvement in the secondary endpoint of sweat chloride reduction. We expect to begin submissions for global regulatory approvals in this age group in the first half of 2026. We completed the global trial evaluating ALYFTREK in children 2 to 5 years of age. The data showed that ALYFTREK was generally safe and well-tolerated, consistent with the established safety profile. Treatment with ALYFTREK in this age group resulted in a clinically meaningful improvement in the CFTR function as measured by sweat chloride. We expect to submit for approval with global regulators in this age group in the first half of 2026. In addition, we initiated a pivotal trial evaluating ALYFTREK in children one to less than two years of age.

We estimate that nearly 95% of people with CF could benefit from our five approved medicines, and, in connection with our serial innovation approach, we continue to identify and develop additional CFTR modulators with the goal of developing best-in-class medicines that can treat more people with CF. We have advanced several next-generation, 3.0 CFTR modulators into the clinic. VX-828 is the first of these and is being evaluated in a proof-of-concept clinical trial of people with CF. We expect to complete enrollment and dosing in the first half of 2026. We are also enrolling and dosing in a Phase 1 clinical trial of VX-581, another corrector in the next-generation 3.0 class, in healthy volunteers.

To treat people with CF who do not make full-length CFTR protein, and as a result, cannot benefit from our CFTR modulators, we are researching and developing genetic therapies, such as mRNA, and gene-editing approaches to CF. In collaboration with Moderna, we are developing VX-522, a nebulized CF mRNA therapeutic designed to treat the underlying cause of CF lung disease for these people by enabling cells in the lungs to produce functional CFTR protein. We are targeting completion of dosing in the multiple ascending dose portion of the Phase 1/2 clinical trial evaluating VX-522 and disclosure of the data in the second half of 2026.

## ***Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia***

In December 2025, we presented positive data from the pivotal trials evaluating CASGEVY in children 5 to 11 years of age with severe SCD (the CLIMB SCD-151 clinical trial) and TDT (the CLIMB THAL-141 clinical trial). We expect to initiate global regulatory submissions for this age group, including in the U.S., in the first half of 2026. In the U.S., CASGEVY has received a Commissioner’s National Priority Voucher for use in this age group, which is meant to accelerate the FDA’s review of the application once submitted.

In connection with our serial innovation approach, we are advancing preclinical assets for myeloablative conditioning agents with improved tolerability profiles, which we refer to as “improved conditioning agents,” which could be used in connection with treatment with CASGEVY, significantly broadening the eligible SCD and TDT patient population. We are also investigating in vivo gene-editing approaches and small molecules for the potential treatment of SCD and TDT.

## ***Pain***

Pain can be debilitating and develop from a variety of conditions. Most commonly, people with pain can be categorized as suffering from one of three types of pain: acute pain, chronic neuropathic pain (caused primarily by damage or dysfunction of peripheral nerves), or chronic musculoskeletal pain (caused primarily by damage to muscle, joints or bone). Acute pain usually resolves in days or weeks (for example, following surgery or an injury), while chronic pain generally lasts greater than three months due to unresolved or ongoing damage to tissues or nerves. Currently available treatments have limitations around efficacy or side effects, including a risk of addiction. Because of these challenges, over, under, and mis-utilization of current pain medicines may occur.

The sodium channels NaV1.8 and NaV1.7 play important roles in the physiology of pain. We have discovered multiple selective small molecule inhibitors of NaV1.8 as potential treatments for pain. We obtained pharmacological validation of NaV1.8 inhibition with a first generation NaV1.8 inhibitor in acute pain, chronic neuropathic pain, and chronic musculoskeletal pain.

### *Acute Pain*

In August 2025, we announced results from the Phase 2 placebo-controlled dose-ranging clinical trial evaluating the safety and efficacy of VX-993, an investigational selective NaV1.8 pain signal inhibitor, for the treatment of acute pain following bunionectomy surgery. The clinical trial was powered to determine whether VX-993 would result in higher clinical efficacy than previously demonstrated with the NaV1.8 pathway. Based on the efficacy results of the clinical trial, we did not expect VX-993 to be superior to suzetrigine and therefore chose not to further advance VX-993 as monotherapy in acute pain. VX-993 was generally safe and well-tolerated.

### *Peripheral Neuropathic Pain*

There are no approved medicines in the U.S. that are labeled for the treatment of peripheral neuropathic pain. We are evaluating suzetrigine, our selective non-opioid NaV1.8 pain signal inhibitor, for the treatment of DPN, a type of peripheral neuropathic pain, in two Phase 3 clinical trials. We expect to complete enrollment in both Phase 3 clinical trials by the end of 2026. The FDA granted Breakthrough Therapy Designation to suzetrigine in DPN. We are also enrolling and dosing people with DPN in a Phase 2 clinical trial evaluating VX-993.

In connection with our serial innovation approach, we are advancing multiple NaV1.8 inhibitors and NaV1.7 inhibitors, which could be used alone or in combination, for the treatment of acute pain and peripheral neuropathic pain.

### ***IgA Nephropathy***

IgAN is a serious, progressive, life-threatening chronic kidney disease driven by uncontrolled autoreactive B cell activity that causes inflammation and damage to the kidneys. It is the most common cause of primary glomerulonephritis worldwide. We estimate that IgAN affects approximately 330,000 people in the U.S. and Europe, and, globally, more than 1.5 million people are diagnosed with IgAN. A high percentage of people with IgAN progress to end-stage kidney disease.

IgAN is thought to occur when the body produces an abnormal form of IgA, a type of antibody that normally helps the body fight infections. The body generates an abnormal immune response, including antibodies (autoantibodies), against this abnormal IgA, and these antibodies can combine to create larger molecules called immune complexes. These immune complexes can deposit in the kidneys, triggering damage and inflammation, especially within the glomeruli, impairing the kidneys' ability to properly filter waste and fluid.

We are developing povetacept for multiple diseases and believe that it has pipeline-in-a-product potential. Povetacept is a potent dual inhibitor of the BAFF and APRIL cytokines, which promote B cell proliferation, differentiation and survival, and provides B cell control by inhibiting the ability of BAFF and APRIL to drive the pathogenesis of multiple autoimmune diseases, such as IgAN, pMN and generalized myasthenia gravis ("gMG") (as described below). Povetacept was specifically engineered to achieve improvements in binding affinity, potency, pharmacokinetics, and tissue distribution. Povetacept has demonstrated potential best-in-class efficacy in a global Phase 1/2 clinical trial in people with IgAN. A small volume dose of povetacept is expected to be self-administered at home once every four weeks via a subcutaneous auto-injector.

We completed enrollment in RAINIER, the global Phase 3 pivotal trial of povetacept versus placebo in people with IgAN. The clinical trial design contemplates a pre-planned interim analysis evaluating the change from baseline in urine protein-to-creatinine ratio ("UPCR") after a certain number of patients reach 36 weeks of treatment. We expect to share data from the interim analysis in the first half of 2026. If positive, the interim analysis may serve as the basis to seek accelerated approval in the U.S. The final analysis will occur when patients reach two years of treatment and will evaluate total eGFR (estimated glomerular filtration rate) slope. The FDA has granted Breakthrough Therapy Designation for povetacept in IgAN. We submitted the first module of the IgAN BLA to the FDA at the end of 2025 under the rolling submission pathway, and we expect to complete the submission in the first half of 2026, pending positive results from the interim analysis. We are using a priority review voucher to expedite the FDA review of the povetacept BLA from ten months to six months.

Our serial innovation approach continues with respect to IgAN and other B cell-mediated diseases.

## ***APOL1-Mediated Kidney Disease***

AMKD is a rapidly progressive, proteinuric kidney disease caused by variants in the APOL1 gene. In AMKD, the kidney's filtering units, known as the glomeruli, and within them the cells known as podocytes, are damaged, leading to leakage of protein into the urine, deterioration in kidney function, scarring, and, ultimately, end stage renal disease. People with AMKD progress to end stage kidney disease at a faster rate than those with other forms of chronic kidney disease and reach kidney failure at a median age of 45 years old. AMKD occurs in people with African ancestry, with an estimated patient population of approximately 150,000 people in the U.S. and Europe. In addition, we estimate that there are approximately 100,000 people with AMKD with comorbidities, such as type 2 diabetes, in the U.S. and Europe.

In a Phase 2 proof-of-concept clinical trial, people with APOL1-mediated focal segmental glomerulosclerosis ("FSGS") treated with inaxaplin on top of standard of care achieved a statistically significant, substantial, and clinically meaningful reduction of proteinuria. Inaxaplin was generally safe and well tolerated by patients. Based on the positive Phase 2 data, the FDA granted Breakthrough Therapy Designation to inaxaplin for FSGS and the European Medicines Agency ("EMA") granted Priority Medicines ("PRIME") designation to inaxaplin for AMKD. We initiated pivotal development of inaxaplin in a single Phase 2/3 adaptive clinical trial ("AMPLITUDE") in people with AMKD in 2022. We completed enrollment of the interim analysis cohort of AMPLITUDE in 2025 and we expect to conduct the pre-planned interim analysis once this cohort has been treated for 48 weeks. We expect to share data from the interim analysis in late 2026 or early 2027, and we expect to complete full enrollment in the AMPLITUDE clinical trial in the second half of 2026.

Our serial innovation strategy in AMKD focuses on indication expansion: evaluating inaxaplin in new populations of people with AMKD not included in the AMPLITUDE clinical trial. The Phase 2 clinical trial ("AMPLIFIED") evaluates inaxaplin as a treatment for people with AMKD with moderate proteinuria, or with AMKD and type 2 diabetes, two populations that are not being studied in the AMPLITUDE trial. We expect to complete the AMPLIFIED clinical trial and share results in mid-2026.

## ***Type 1 Diabetes***

T1D is a chronic metabolic disorder caused by insufficient insulin secretion by the beta cells in the pancreas. In people with T1D, the insulin-producing islet cells of the pancreas are destroyed by the person's own immune system, resulting in a lack of insulin and impairment of blood glucose control. While insulin therapy allows patients to live for decades with the disease, challenges of insulin therapy include inadequate control of blood sugar (both hyper- and hypo-glycemia), a substantial burden of care on patients and families, and long-term vascular complications. Current standards of care do not address the underlying causes of the disease, and there are limited treatment options beyond insulin for the management of T1D.

We are developing non-autologous (allogeneic) fully differentiated, stem-cell derived islet cell therapies designed to replace insulin-producing islet cells that are destroyed in people with T1D, with the goal of delivering a functional cure. Zimislecel, our first program, is a stem cell-derived, allogeneic, fully differentiated, insulin-producing islet cell replacement therapy, using standard immunosuppression to protect the implanted cells. We believe that zimislecel has the potential to transform the lives of eligible people with T1D. In the U.S. and Europe, we estimate that there are approximately four million people diagnosed with T1D. At initial launch, we expect there will be approximately 65,000 people with high unmet need who experience severe hypoglycemic events who will be eligible for zimislecel.

We have completed enrollment in the Phase 1/2/3 clinical trial evaluating the safety and efficacy of zimislecel. We have temporarily postponed completion of the dosing pending an ongoing internal manufacturing analysis. The most recent data from this trial, published online in the *New England Journal of Medicine* in June 2025, continue to demonstrate the transformative potential of zimislecel with consistent and durable patient benefit. The safety profile is generally consistent with the immunosuppressive regimen used in the trial, the infusion procedure, and complications from long-standing diabetes. Zimislecel has been granted Regenerative Medicine Advanced Therapy and Fast Track designations from the FDA, PRIME designation from the EMA, Breakthrough Medicine designation from the Kingdom of Saudi Arabia ("Saudi Arabia"), and has secured an Innovation Passport under the Innovative Licensing and Access Pathway from the U.K. Medicines and Healthcare products Regulatory Agency (the "MHRA").

In March 2025, we announced results from the Phase 1/2 clinical trial evaluating VX-264, which encapsulated zimislecel in an immunoprotective device. VX-264 was generally safe and well-tolerated but did not meet its efficacy endpoint, and we have discontinued development of this program.

In connection with our serial innovation approach, we are pursuing research-stage programs to evaluate additional approaches that could provide transformative benefits to people with T1D and reduce or eliminate the need for standard immunosuppressive regimens, including targeting improved immunosuppression for zimislecel.

### ***Primary Membranous Nephropathy***

pMN is a serious, progressive, life-threatening chronic kidney disease driven by uncontrolled autoreactive B cell activity that causes inflammation and damage to the kidneys. It is a rare autoimmune glomerular disease that occurs when the body generates an abnormal immune response, including antibodies (autoantibodies), against proteins that are part of the kidney. We estimate that pMN affects approximately 150,000 people in the U.S. and Europe, and more than 600,000 people globally. Autoantibodies trigger damage and inflammation, especially within the glomeruli, impairing the kidneys' ability to properly filter waste and fluid.

People with pMN can experience a variety of serious complications, including blood clots, infection, and heart disease. At time of diagnosis, most people with pMN are at risk of progression to end-stage renal disease. There are no therapies specifically approved for the treatment of pMN.

We believe povetacept represents a potentially best-in-class approach to control B cell activity in people with pMN. We have received Fast Track Designation from the FDA and PRIME designation from the EMA for povetacept in pMN. Based on the strength of the Phase 2 results in the RUBY-3 clinical trial, we completed the End of Phase 2 meeting with the FDA and reached agreement on an adaptive Phase 2/3 pivotal development program for pMN; we are enrolling and dosing people with pMN in that clinical trial. We expect to complete the Phase 2 portion of the clinical trial and to initiate the Phase 3 portion of the trial in mid-2026.

### ***Autosomal Dominant Polycystic Kidney Disease***

ADPKD is a life-shortening genetic kidney disease characterized by the growth of numerous kidney-enlarging cysts that impair kidney function and can ultimately lead to end stage renal disease. In most cases, ADPKD is caused by variants in the PKD1 and PKD2 genes; the majority of ADPKD patients have a variant in the PKD1 gene. Around half of people with ADPKD experience kidney failure by the age of 60. We estimate that there are approximately 300,000 people diagnosed with ADPKD in the U.S. and Europe.

VX-407 is a first-in-class small molecule corrector that is designed to target the underlying cause of ADPKD in people with a subset of PKD1 variants, which represents up to approximately 10% of the overall patient population living with ADPKD. We are enrolling and dosing patients in a Phase 2 proof-of-concept clinical trial evaluating VX-407 ("AGLOW") for the treatment of ADPKD. We expect to complete enrollment in the AGLOW clinical trial by the end of 2026.

In connection with our serial innovation approach, we are progressing multiple research-stage assets in ADPKD.

### ***Myotonic Dystrophy Type 1***

DM1 is an inherited disease that results in the weakening and destruction of skeletal muscles over time. Muscle weakness, muscle wasting and myotonia (sustained muscle contraction and difficulty relaxing muscles) are the hallmark features of DM1. It is a serious life-shortening disease with no approved treatments, and we estimate that it affects approximately 110,000 people in the U.S. and Europe.

VX-670, our lead approach for DM1, holds the potential to address the underlying cause of DM1. VX-670 is an oligonucleotide connected to a cyclic peptide to promote effective delivery into cells. We continue to enroll and dose in the multiple ascending dose portion of the global Phase 1/2 clinical trial of VX-670 in people with DM1 ("GALILEO"), which evaluates both safety and efficacy of VX-670. We expect to complete enrollment and dosing in this trial in mid-2026.

Our serial innovation approach in DM1 includes a small molecule program in preclinical development.

### ***Generalized Myasthenia Gravis***

gMG is a serious, chronic, and debilitating B cell-mediated immune disorder. This rare condition is caused by the formation of pathogenic autoantibodies to key proteins that function in neuromuscular transmission. These pathogenic antibodies block, alter, or damage the neuromuscular junction, which is the connection point between nerve cells and the muscles they control. As a result, people with gMG experience muscle weakness and fatigue, which can lead to inability to

perform the activities of daily living and, in severe cases, compromise of respiratory muscles that can lead to life-threatening respiratory failure. Current therapies address only subsets of the gMG population, and many advanced treatments require cyclic treatment and drug holidays due to safety challenges and immunosuppression. As a consequence, there is significant unmet medical need for improved therapies. We estimate that gMG affects approximately 175,000 people in the U.S. and Europe and more than 300,000 people globally.

Povetacept is a potent dual inhibitor of BAFF and APRIL, two cytokines that are elevated in gMG, where they play distinct roles in the proliferation, differentiation, and survival of B cells. In gMG, elevated expression of both BAFF and APRIL drives uncontrolled B cell growth and activation, triggering overproduction of the pathogenic autoantibodies driving disease activity. By inhibiting both BAFF and APRIL, we believe povetacept represents a potential best-in-class approach to reducing production of these pathogenic autoantibodies in gMG.

We expect to initiate a placebo-controlled, Phase 2 dose-ranging proof-of-concept clinical trial evaluating povetacept for the treatment of people with gMG in the first half of 2026.

## **STRATEGIC TRANSACTIONS**

As part of our business strategy, we seek to license or acquire technologies, products, product candidates, and businesses that are aligned with our corporate and research and development strategies and that complement and advance our ongoing research and development efforts. In addition, we establish business relationships with collaborators to support our research activities and to lead or support development and/or commercialization of certain product candidates. We expect to continue to identify and evaluate potential acquisitions, licenses and collaborations that may be similar to or different from the transactions that we have engaged in previously.

### ***Acquisitions***

In 2024, we acquired Alpine for approximately \$5.0 billion. Alpine's lead molecule, povetacept, is a highly potent and effective dual inhibitor of BAFF and APRIL. We are currently evaluating povetacept in a pivotal trial as a potentially best-in-class approach to treat IgAN. We also believe povetacept holds pipeline-in-a-product potential for other indications, such as pMN and gMG.

We previously made other acquisitions which have expanded and advanced our pipeline, including:

- In 2019, we established our T1D program through our acquisition of Semma, a privately held company focused on the use of stem cell-derived human islets as a potentially curative treatment for T1D. We are evaluating zimislecel for the potential treatment of T1D in a Phase 1/2/3 clinical trial.
- In 2017, we enhanced our CF portfolio through our acquisition of certain CF assets, including deutivacaftor, from Concert Pharmaceuticals Inc. In 2024, the FDA approved ALYFTREK for people with CF 6 years of age and older.

We expect to continue to identify and make acquisitions to expand and advance our pipeline and business.

### ***Collaboration and Licensing Arrangements***

#### ***Joint Development and Commercialization Agreement with CRISPR***

In 2017, we entered into a joint development and commercialization agreement ("Original JDCA") with CRISPR Therapeutics AG ("CRISPR"), pursuant to which we are co-developing and co-commercializing CASGEVY for SCD and TDT. In 2021, we and CRISPR amended and restated the Original JDCA (the "A&R JDCA").

Pursuant to the A&R JDCA, we lead global development, manufacturing and commercialization of CASGEVY, with support from CRISPR. Subject to the terms and conditions of the A&R JDCA, we have the right to conduct all research, development, manufacturing, and commercialization activities relating to the product candidates and products under the A&R JDCA (including CASGEVY) throughout the world, subject to CRISPR's reserved right to conduct certain activities.

The net profits and net losses incurred pursuant to the A&R JDCA with respect to CASGEVY are allocated 60% to us and 40% to CRISPR, subject to certain adjustments, while all other product candidates and products under the A&R JDCA have net profits and net losses shared equally between the parties.

Either party may terminate the A&R JDCA upon the other party's material breach, subject to specified notice and cure provisions, or, in our case, in the event that CRISPR becomes subject to specified bankruptcy, winding up, or similar circumstances. Either party may terminate the A&R JDCA in the event the other party commences or participates in any action or proceeding challenging the validity or enforceability of any patent that is licensed to such challenging party pursuant to the A&R JDCA. We also have the right to terminate the A&R JDCA for convenience at any time after giving prior written notice. If circumstances arise pursuant to which a party would have the right to terminate the A&R JDCA on account of an uncured material breach, such party may elect to keep the A&R JDCA in effect and cause such breaching party to be treated as if it had exercised its opt-out rights with respect to the products associated with such uncured material breach and the royalties payable to the breaching party would be reduced by a specified percentage.

Either party may opt out of the development of a product candidate under the A&R JDCA after predetermined points in the development of the product candidate, on a candidate-by-candidate basis. In the event of such opt-out, the party opting-out will no longer share in the net profits and net losses associated with such product candidate and, instead, the opting out party will be entitled to high single to mid-teen percentage royalties on the net sales of such product, if commercialized.

#### *In-License Agreements*

We have entered into various agreements pursuant to which we have obtained access to technologies from third parties and are conducting research and development activities with collaborators. Pursuant to these arrangements, we have obtained development and commercialization rights to resulting product candidates. Depending on the terms of the arrangements, we may be responsible for the costs of research activities, required to make upfront payments and/or milestone payments upon the achievement of certain research, development, and commercial objectives, and/or pay royalties on future sales, if any, of commercial products resulting from the collaboration. Our current in-license agreements include:

- CRISPR Therapeutics AG. In addition to our arrangement with CRISPR described above, we have exercised options to exclusively license treatments for specific targets, including CF, that were subject to the research program under the collaboration agreement we entered into with CRISPR in 2015. In 2019, we obtained exclusive worldwide rights to CRISPR's intellectual property for Duchenne muscular dystrophy ("DMD") and DM1 gene-editing products through a new agreement with CRISPR. In 2023, we obtained non-exclusive rights to CRISPR's intellectual property for the development of hypimmune gene-edited cell therapies for T1D through a new agreement with CRISPR.
- Moderna, Inc. In 2016, we entered into a collaboration with Moderna for the identification and development of mRNA therapeutics encoding CFTR for the treatment of CF. We are evaluating VX-522, an mRNA therapeutic, pursuant to this collaboration.
- Entrada Therapeutics, Inc. In 2022, we established a collaboration with Entrada focused on enabling efficient intracellular delivery of an oligonucleotide for DM1. This collaboration includes VX-670, an investigational candidate for the treatment of DM1 that is in clinical development. We are evaluating VX-670 in people with DM1 pursuant to this collaboration.

#### *Out-license Agreements*

We have entered into various agreements pursuant to which we have out-licensed rights to certain product candidates to third-party collaborators. Pursuant to these out-license arrangements, our collaborators are responsible for certain costs related to the continued development of such product candidates and obtain development and commercialization rights to these product candidates. Depending on the terms of the arrangements, our collaborators may be required to make upfront payments, milestone payments upon the achievement of certain research and development objectives and/or pay royalties on future sales, if any, of commercial products licensed under the agreement.

In 2025, we entered into agreements with Zai Lab Limited ("Zai") and Ono Pharmaceuticals Co., Ltd ("Ono") related to the development and commercialization of povetacept in certain Asian markets. Zai licensed povetacept for mainland China, Hong Kong SAR, Macau SAR, Taiwan region, and Singapore, while Ono licensed povetacept for Japan and South Korea. Zai and Ono will help advance povetacept clinical trials and will be responsible for obtaining marketing authorizations and commercialization activities in the licensed territories, if povetacept becomes an approved product.

## *Cystic Fibrosis Foundation*

In 2004, we entered into an agreement (the “CFF Agreement”) with the Cystic Fibrosis Foundation (the “CFF”), as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc., to support research and development activities. Pursuant to the CFF Agreement, as amended, we have agreed to pay tiered royalties ranging from single digits to sub-teens on covered compounds first synthesized and/or tested during a research term on or before February 28, 2014, including ivacaftor, lumacaftor and tezacaftor, and royalties ranging from low-single digits to mid-single digits on net sales of certain compounds first synthesized and/or tested between March 1, 2014 and August 31, 2016, including elexacaftor. We do not have any royalty obligations on compounds first synthesized and tested on or after September 1, 2016. For combination products, such as ORKAMBI, SYMDEKO/SYMKEVI, TRIKAFTA/KAFTRIO, and ALYFTREK, sales are allocated equally to each of the active pharmaceutical ingredients in the combination product, and royalties are then paid for any royalty-bearing components included in the combination. For TRIKAFTA/KAFTRIO, the CFF Agreement does not identify a specific date on which royalty obligations terminate. To qualify as a royalty bearing “Drug Product” as defined under the CFF Agreement, a compound must be covered by intellectual property protection (including patents) that Vertex has the legal right to license to another party.

## **INTELLECTUAL PROPERTY**

Patents and other intellectual property rights such as trademarks, trade secrets, and copyrights are critical to our business. We actively seek protection for our products and proprietary information by means of U.S. and foreign patents, trademarks, and copyrights, as appropriate. In addition, we rely upon trade secret protection and contractual arrangements to protect certain of our proprietary information.

Patents provide a period of exclusivity that can make it more difficult for competitors to market and use our technology. We own and control patents and pending patent applications that relate to compounds, formulations, synthetic routes, intermediates, devices, treatment of diseases, and other inventions.

To protect our intellectual property, we typically apply for patents several years before a product receives marketing approval. Under current law, a patent expires 20 years from its first effective filing date. Since the drug development process may last for many years, there may be a period of time in which we have an issued patent but not marketing approval to sell the drug. To compensate for patent term lost while a product is in clinical trials and undergoing review for marketing approval, we may be able to apply for patent term extensions or supplementary protection certificates (“SPCs”) in some countries. In addition to patent protection, we receive regulatory exclusivity from U.S. and European regulatory agencies for the active pharmaceutical and biological agents and, where applicable, their approved orphan indications for a certain time period. Regulatory exclusivity runs concurrently with patent exclusivity and provides complementary protection for our products.

For our approved commercial products, and those in development, we own or hold exclusive and non-exclusive licenses to several hundred patents around the world. In the U.S., once a New Drug Application (“NDA”), or a supplement thereto, is approved we are required to list with the FDA each U.S. patent with claims that cover our product or a method of using the product. The FDA publishes the patents we list in a book referred to as the Orange Book. We have fourteen issued U.S. patents listed in the Orange Book that cover the active pharmaceutical ingredients in KALYDECO, its marketed formulations, and/or its approved indication. We have 22 issued U.S. patents listed in the Orange Book that cover the active pharmaceutical ingredients in ORKAMBI, its marketed formulations, and/or its approved indication. We have 25 issued U.S. patents listed in the Orange Book that cover the active pharmaceutical ingredients in SYMDEKO, its marketed formulations, and/or its approved indication. We have 34 issued U.S. patents listed in the Orange Book that cover the active pharmaceutical ingredients in TRIKAFTA, its marketed formulations, and/or its approved indication. We have 35 issued U.S. patents listed in the Orange Book that cover the active pharmaceutical ingredients in ALYFTREK, its marketed formulations, and/or its approved indication. We have an issued patent listed in the Orange Book that covers the active pharmaceutical ingredient in JOURNAVX, its marketed formulation, and/or its approved indication.

Products approved by the FDA under a BLA, including CASGEVY, receive 12 years of regulatory exclusivity in the U.S. from a product’s approval date. Additionally, we have licenses to dozens of issued U.S. patents that cover CASGEVY, its approved indication, and/or its manufacture. Products approved by the FDA under a BLA are not subject to the Orange Book patent listing requirement.

The table below sets forth the year of projected expiration for the basic product patent covering each of our approved products. For products that are combinations of two or more active ingredients, the table lists the projected expiration of the latest expiring patent covering any of the active pharmaceutical ingredients (lumacaftor for ORKAMBI, tezacaftor for SYMDEKO/SYMKEVI, elexacaftor for TRIKAFTA/KAFTRIO and vanzacaftor for ALYFTREK). Unless otherwise noted, patent term extensions, and pediatric exclusivity periods are not reflected in the expiration dates listed in the table below and may extend protection. In some instances, we also own later-expiring patents and applications relating to solid forms, formulations, methods of manufacture, or the use of these drugs in the treatment of particular diseases or conditions. In some cases, however, such patents may not protect our drug from generic competition after the expiration of the basic patent.

<b>Product</b>	<b>Expiration Year of U.S. Basic Product Patent</b>	<b>Expiration Year of European Basic Product Patent</b>
KALYDECO	2028 <sup>1</sup>	2027 <sup>2,3</sup>
ORKAMBI	2031 <sup>1</sup>	2030 <sup>2</sup>
SYMDEKO/SYMKEVI	2027	2033 <sup>2</sup>
TRIKAFTA/KAFTRIO	2037	2037
CASGEVY	2035 <sup>4</sup>	2034 <sup>5,6</sup>
ALYFTREK	2039	2039
JOURNAVX	2040	2040

<sup>1</sup> Includes pediatric exclusivity.

<sup>2</sup> Expiration date reflects SPCs granted in the five major European markets (France, Germany, Italy, Spain and the U.K.).

<sup>3</sup> SPC expires in 2028 in Germany; application for pediatric extension pending in France, Italy, Spain, and the U.K.

<sup>4</sup> Expiration year reflects the expiration of regulatory exclusivity, which expires later than the basic product patent for this product in this market.

<sup>5</sup> Expiration year reflects the expiration of regulatory exclusivity in the E.U., which expires later than the basic product patent for this product in this market.

<sup>6</sup> Product is approved in Great Britain with regulatory exclusivity until November 2033, which is later than the expiration of the basic product patent.

In addition to protecting our marketed products, we actively file patent applications in the U.S. and in foreign countries on inventions relating to our pipeline. For example, we also own and/or control U.S. and foreign patents and/or patent applications relating to the following:

- Other CF potentiators and correctors and many other related compounds, and the use of those compounds for the treatment of CF.
- VX-522 and other mRNA-based approaches for treating CF.
- VX-993, VX-973, and other compounds being studied for the potential treatment of pain.
- Povetacept for the treatment of IgAN, pMN and gMG.
- Inaxaplin for the potential treatment of AMKD.
- Zimislecel and other cell-based approaches for treating T1D.
- VX-407 and other compounds being studied for the potential treatment of ADPKD.
- VX-670 for the treatment of DM1.
- Other pre-clinical and clinical candidates and the use of such candidates to treat specified diseases.
- The manufacture, pharmaceutical compositions, related solid forms, formulations, dosing regimens, and methods of use of many of the above compounds.

We and CRISPR intend to rely upon a combination of rights, including patent rights, trade secret protection, and regulatory exclusivities to protect CASGEVY. CRISPR has licensed certain rights to a worldwide patent portfolio that covers various aspects of the CRISPR/Cas9 editing platform technology including, for example, compositions of matter and methods

of use, including their use in targeting or cutting DNA, from Dr. Emmanuelle Charpentier. In addition to Dr. Charpentier, this patent portfolio has named inventors who assigned their rights to the Regents of the University of California or the University of Vienna, to whom we refer, together with Dr. Charpentier, as the CVC Group. CRISPR has non-exclusive or co-exclusive rights to the patent rights that protect the core CRISPR/Cas9 gene-editing technology. For example, certain third parties, including competitors, have reported obtaining a license to rights in this patent portfolio in certain fields. In addition, patents and patent applications in this patent portfolio are the subject of adversarial proceedings in the U.S., Europe, and other jurisdictions, including proceedings in the U.S. Patent and Trademark Office (the “USPTO”), between the CVC Group and, separately, Sigma-Aldrich, Co. LLC (“Sigma-Aldrich”), ToolGen, Inc. (“ToolGen”), and the Broad Institute, Harvard University, and Massachusetts Institute of Technology (collectively, “Broad”). To date, both the CVC Group and Broad have obtained granted patents that purport to cover aspects of CRISPR/Cas9 editing platform technology. The patents and patent applications within the patent portfolios of the CVC Group, Broad, Sigma-Aldrich and/or ToolGen are, or may in the future be, involved in proceedings similar to interferences or priority disputes in Europe or other foreign jurisdictions. In December 2023, we entered into an agreement with Editas Medicine, Inc. (“Editas”), providing us a non-exclusive sublicense to certain patents relating to CRISPR/Cas9 technology, owned by Broad and Harvard, which are licensed to Editas. In addition to the patent portfolios licensed from Dr. Charpentier, Broad, and Harvard, we own patents and/or patent applications relating to the composition, manufacture, and use of CASGEVY.

We and our CASGEVY manufacturing partners are engaged in patent litigation against ToolGen in the U.S., the U.K., and the Netherlands. In these cases, ToolGen alleges that the CASGEVY manufacturing process infringes its patents relating to CRISPR/Cas9. We have argued in the U.K. and the Netherlands that ToolGen’s patents are invalid, and we filed oppositions at the European Patent Office seeking the revocation of the patents asserted in the U.K. and the Netherlands cases. We intend to respond to the U.S. case in the first half of 2026.

From time to time, we enter into exclusive and non-exclusive license agreements for proprietary third-party technology used in connection with our research activities. These license agreements typically provide for the payment by us of a license fee but may also include terms providing for milestone payments or royalties for the development and/or commercialization of our drug products arising from the related research.

We cannot be certain that issued patents we own or license will be enforceable or provide adequate protection or that pending patent applications will result in issued patents. The existence of patents does not guarantee our right to practice the patented technology or commercialize the patented product. Litigation, interferences, oppositions, *inter partes* reviews, administrative challenges or other similar types of proceedings may be necessary in some instances to determine the validity and scope of certain patents, regulatory exclusivities or other proprietary rights, and in other instances to determine the validity, scope or non-infringement of intellectual property rights that may be claimed by third parties to be pertinent to the manufacture, use or sale of our products.

## **MANUFACTURING**

As we market and sell our approved products and advance our product candidates through clinical development toward commercialization, we continue to build and maintain our supply chain and quality assurance resources. We rely on internal capabilities and a global network of third parties to manufacture and distribute our product candidates for clinical trials, as well as our products for commercial sale and post-approval clinical trials. In addition to establishing supply chains for newly approved products, we must adapt our supply chains for existing products to increase scale of production or to include additional formulations. We are focused on ensuring the stability of the supply chains for our current products, including our CF medicines, CASGEVY, and JOURNAVX, and for our pipeline programs. We are also focused on identifying and ensuring efficient manufacturing and delivery processes for the biologics and cell and genetic therapies we are developing, including our stem cell therapy program for T1D, and biologics manufacturing for povetacept.

We have established our own small molecule manufacturing capabilities in Boston, which we use for clinical trial and commercial supplies, including certain manufacturing steps related to our commercial supply of TRIKAFTA/KAFTRIO. We expect to continue to rely on third parties to meet our commercial supply needs and a significant portion of our clinical supply needs for the foreseeable future.

Our supply chain for sourcing raw materials and manufacturing our products and product candidates, including obtaining all necessary supplies, is a multi-step, global endeavor. In general, these raw materials and other necessary supplies are available from multiple sources. Third-party contract manufacturers, including some based in China, perform different parts of our manufacturing process. Contract manufacturers supply us with raw materials, convert these raw materials into drug

substance and/or convert the drug substance or product into final dosage form. In addition, third parties assist us with packaging, warehousing, and global distribution of our products. Establishing and managing this global supply chain for each of our products and product candidates requires a significant financial commitment and the creation and maintenance of numerous third-party contractual relationships. We have established and we maintain second sources for the vast majority of our commercial products, including active ingredients, drug product, and finished dosage form packaging. Similarly, commercial manufacturing for the vast majority of our small molecule drug products is in the U.S.

The manufacturing processes for biologics and cell and genetic therapies are more complex than those required for small molecule drugs and require different systems, equipment, facilities, and expertise. Additionally, we are unable to utilize a single process for all of our biologics and cell and genetic therapies; they must be customized for each program and therapy. We are investing and plan to continue to invest significant resources in expanding and strengthening our manufacturing infrastructure and capabilities, such as current Good Manufacturing Practices (“cGMP”) clinical manufacturing, both independently and through third-party networks, in an effort to develop and commercialize our biologics and cell and genetic therapies. We have secured agreements to meet our current demands for these products and product candidates. We continue to evaluate additional suppliers for all of our late-stage clinical programs for additional capacity and redundancy to support commercial supply.

We rely on third-party manufacturers to produce or process cell culture reagents and gene-editing components, such as Cas9 protein and guide RNA molecules, for clinical trials and commercial supply of CASGEVY, and to generate gene-edited cells to supply CASGEVY. The manufacturing process for CASGEVY involves a number of steps prior to the final infusion of drug product into patients. Following mobilization and collection of blood cells from the patient, cells are transferred to a manufacturing site where HSPCs are purified and CRISPR/Cas9 gene-editing is performed. The edited cellular product, called CASGEVY, is frozen and transported back to the authorized treatment center where it is stored prior to infusion into the patient. Each step must be completed successfully, and in a timely manner, requiring coordination between us, authorized treatment centers, third-party manufacturers and shipping vendors. We are making investments to enhance the CASGEVY manufacturing process, to secure additional capacity, and to coordinate manufacturing, testing, and logistics activities at a larger scale across multiple facilities to serve the geographies in which we are treating and expect to treat additional people with CASGEVY.

In addition, we have established cell therapy manufacturing capabilities at our facilities in the Boston area to supply clinical and potentially commercial quantities of our cell therapies as our needs evolve, including our plans to utilize our own manufacturing capabilities in Boston for additional commercial supply of CASGEVY. To further expand our ability to supply clinical and potentially commercial quantities of our cell therapies, we have a strategic agreement with Lonza to support the manufacture of T1D cell therapy product candidates. We also rely on third-party manufacturers to produce drug substance and finished drug product for clinical trials for povetacept. In addition, we have obligations to supply product to global third parties that support the development and commercialization of povetacept.

We have developed systems and processes to track, monitor, and oversee our and our third-party manufacturers’ activities, including a quality assurance program intended to ensure that our third-party manufacturers comply with cGMP and the foreign jurisdictional equivalents when applicable. We devote substantial time, resources, and effort in the areas of production, quality control, and quality assurance to maintain cGMP compliance. We regularly evaluate the performance of our third-party manufacturers with the objective of confirming their continuing capabilities to meet our needs compliantly, efficiently, and economically. Manufacturing facilities, both foreign and domestic, are subject to inspections by or under the authority of the FDA and other U.S. and foreign government authorities. Although we actively engage with regulatory authorities, the timing of inspections and regulatory approvals for each of these facilities is the remit of the third-party manufacturer and not within our control and may be delayed for a number of reasons.

## **COMPETITION**

The pharmaceutical industry is characterized by extensive research efforts, rapid technological progress, and intense competition. There are many public and private companies, including pharmaceutical companies and biotechnology companies, engaged in developing products for the indications our medicines are approved to treat and the therapeutic areas we are targeting with our research and development activities. Potential competitors also include academic institutions, government agencies, other public and private research organizations and charitable venture philanthropy organizations that conduct research, seek patent protection and/or establish collaborative arrangements for research, development, manufacturing and commercialization. Mergers and acquisitions in the pharmaceutical, biotechnology and gene therapy

industries may result in a larger concentration of resources among a smaller number of our competitors. Some of our competitors may have substantially greater financial, technical, sales and marketing, and human resources than we do.

Competition may be based, among other things, on efficacy, safety, availability, patient convenience, frequency of dosing, ease of use, delivery devices and overall patient experience; formulary placement, price, payer coverage and reimbursement rates; regulatory approvals and exclusivity; patent and other intellectual property positions; marketing effectiveness; and research and development of new products, processes, modalities, indications, and uses. Early market entry and rapid patient access can also be important to achieve product acceptance and success. Accordingly, the relative speed with which we can develop therapies, complete the testing and approval process, and supply commercial quantities of such therapies will have a significant impact on our competitive position.

Our therapies must compete with other branded or generic products already on the market or those that are developed in the future. The introduction of new products or technologies, including the development of new processes or technologies by competitors or new information about existing products or technologies, results in increased competition for our marketed products and pricing pressure on our marketed products. For example, the number of compounds available to treat a particular disease typically increases over time and can result in slowed sales growth or reduced sales of our products in that therapeutic area. The development of new or improved treatment options could eliminate the use of our medicines or may limit the utility and application of ongoing clinical trials for our product candidates. Similarly, developments of new standards of care practices, treatment options or cures for the diseases our medicines treat could have similar impacts.

We believe our long-term competitive success depends on discovering and developing or acquiring transformative medicines for people with serious diseases and continuously improving the productivity of our operations in a highly competitive environment. There can be no assurance that our efforts will result in commercially successful medicines, and it is possible that our medicines will be, or will become, uncompetitive from time to time. See also *Item 1A., Risk Factors – “Competing products and technological advances from our competitors may negatively affect our business and market position.”* of this Annual Report on Form 10-K.

## **GOVERNMENT REGULATION**

Our operations and activities are subject to extensive regulation by numerous government authorities in the U.S., Europe and other countries, including with respect to the testing, manufacture, labeling, storage, record keeping, approval, pricing and price reporting, and advertising and promotion of our products.

### ***Regulations Concerning Product Development and Approval***

*United States.* The process for obtaining regulatory approvals to market a new pharmaceutical product, or an additional indication of an existing product, requires substantial effort and financial resources and takes several years to complete. The applicant must complete preclinical tests and submit protocols to the FDA before commencing clinical trials. Clinical trials are intended to establish the safety and efficacy of the pharmaceutical product and typically are conducted in sequential phases, although the phases may overlap or be combined. If the required clinical testing is successful, the results are submitted to the FDA in the form of an NDA or BLA requesting approval to market the product for one or more indications. The FDA reviews an NDA or BLA to determine whether a product is safe and effective for its intended use and whether its manufacturing is compliant with cGMP.

The FDA can employ several tools to facilitate the development of certain drugs or expedite certain applications, including fast track designation, Breakthrough Therapy designation, regenerative medicine advanced therapy designation, priority review, accelerated approval, incentives for orphan drugs developed for rare diseases and others.

Compliance with regulatory requirements is assured through periodic, announced or unannounced inspections by the FDA and other regulatory authorities, and these inspections associated with clinical development may include the sponsor, investigator sites, laboratories, hospitals and manufacturing facilities of our subcontractors or other third-party manufacturers. Failure to comply with applicable regulatory requirements can result in enforcement action by the FDA, including rejection of an NDA or BLA.

Even if an NDA or a BLA receives approval, the applicant must comply with post-approval requirements. For example, holders of an approval must report adverse reactions, provide updated safety and efficacy information and comply with requirements concerning advertising and promotional materials and activities. Also, quality control and manufacturing procedures must continue to conform to cGMP after approval, and certain changes to the manufacturing procedures and

finished product must be submitted and approved by the FDA prior to implementation. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which imposes extensive procedural and record keeping requirements. In addition, as a condition of approval, the FDA may require post-marketing testing and surveillance to further assess and monitor the product's safety or efficacy after commercialization, which may require additional clinical trials, patient registries, observational data or additional work on chemistry, manufacturing and controls. Any post-approval regulatory obligations, and the cost of complying with such obligations, could expand in the future. Further, the FDA continues to regulate product labeling and prohibits the promotion of products for unapproved or “off-label” uses along with other labeling restrictions.

*Outside the United States.* We are subject to similar regulatory requirements outside the United States for approval and marketing of pharmaceutical products. We must obtain approval of a clinical trial application or product from applicable supervising regulatory authorities before it can commence clinical trials or marketing of the product in target markets. The approval requirements and process for each country can vary, and the time required to obtain approval may be longer or shorter than that required for FDA approval in the United States. For example, we may submit marketing authorizations in the E.U. under either a centralized or decentralized procedure. The centralized procedure is mandatory for the approval of biotechnology products and many pharmaceutical products and provides for a single marketing authorization that is valid for all E.U. member states. Under the centralized procedure, a single marketing authorization application is submitted to the European Medicines Agency. After the agency evaluates the application, it makes a recommendation to the European Commission, which then makes the final determination on whether to approve the application. The decentralized procedure provides for mutual recognition of individual national approval decisions and is available for products that are not subject to the centralized procedure.

In April 2023, the European Commission adopted a proposal to revise the E.U. pharmaceutical legislation. In April 2024, the European Parliament introduced amendments to the European Commission’s proposal. The legislative process remains ongoing, with several stages still required before the reform can receive final approval. Once completed, the reform is likely to be the most comprehensive overhaul of E.U.’s medicines regulation in over 20 years, with a wide range of impacts including on approval procedures, regulatory data protection, and environmental protection measures. Once approved, certain provisions of the reform could potentially have an adverse impact on our business.

The requirements governing the conduct of clinical trials and product licensing also vary. In addition, post-approval regulatory obligations such as adverse event reporting and cGMP compliance generally apply and may vary by country. For example, after a marketing authorization has been granted in the E.U., periodic safety reports must be submitted and other pharmacovigilance measures may be required.

### ***Regulations Concerning Pricing and Reimbursement***

Sales of our products depend, to a large degree, on the extent to which our products will be reimbursed by third-party payors, such as government health programs, commercial insurance companies, and managed health care organizations. Increasingly, these third-party payors are becoming stricter in the ways they evaluate and reimburse medical products and services. Additionally, the containment of health care costs has become a priority of many governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could limit our revenues. Decisions by third-party payors to not cover a product could reduce physician usage of the product.

*United States.* In the U.S., we participate in the Medicaid Drug Rebate Program, Medicare, and other governmental pricing programs. Medicaid is a joint federal and state program that is administered by the states for low-income and disabled beneficiaries. Under the Medicaid Drug Rebate program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs, which includes select inpatient drugs for which there is “direct reimbursement.” Medicaid rebates are based on pricing data reported by us on a monthly and quarterly basis to CMS, the federal agency that administers the Medicaid and Medicare programs.

Any company that participates in the Medicaid Drug Rebate Program also must participate in the 340B drug pricing program (the “340B program”), and the Federal Supply Schedule (“FSS”) pricing program. The 340B program, which is administered by the Health Resources and Services Administration, requires participating companies to agree to charge statutorily defined “covered entities” no more than the 340B “ceiling price” for covered outpatient drugs. The 340B ceiling

price is calculated using a statutory formula, which is based on pricing data calculated under the Medicaid Drug Rebate Program. The FSS pricing program, which is administered by the Department of Veterans Affairs (“VA”), also requires participating companies to extend discounted prices to the VA, Department of Defense, Coast Guard, and Public Health Service. Similar to the 340B program, FSS prices are calculated utilizing pricing data reported by us to the VA on a quarterly and annual basis.

Medicare is a federal program that is administered by the federal government. The program covers individuals age 65 and over as well as those with certain disabilities. Medicare Part A generally covers certain inpatient hospital services for eligible beneficiaries. Prescription drugs that are used as part of an inpatient hospital stay will be covered by Medicare Part A, and these products typically are paid as part of a bundled or composite rate (e.g., diagnosis related group).

Medicare Part D provides coverage to enrolled Medicare patients for self-administered drugs (i.e., drugs that are not administered by a physician). Medicare Part D is administered by private prescription drug plans approved by the U.S. government. Subject to certain statutory parameters, each drug plan establishes its own Medicare Part D formulary for prescription drug coverage and pricing, which the drug plan may modify from time-to-time. The prescription drug plans negotiate pricing with manufacturers and pharmacies, and may condition formulary placement on the availability of manufacturer discounts.

The U.S. government has shown significant interest in implementing cost-containment programs for medicines and has enacted reforms at the federal level designed to, among other things, modify prescription drug reimbursement amounts and methodologies, and otherwise control health care costs. For example, the Patient Protection and Affordable Care Act (“ACA”) was enacted in March 2010 and was designed to expand coverage for the uninsured while at the same time containing overall health care costs. With regard to pharmaceutical products, among other things, the ACA was designed to expand and increase manufacturer rebates for drugs covered under Medicaid programs, impose an annual fee on branded pharmaceutical manufacturers, subject biological products to potential competition by lower-cost biosimilars, and make changes to the coverage requirements under the Medicare Part D program. Additionally, in August 2022, the Inflation Reduction Act (“IRA”) was enacted, establishing a Medicare Drug Price Negotiation Program, a Medicare inflationary rebate, and a redesign of the Part D benefit structure. Certain drugs, including our CF medicines and CASGEVY, currently are excluded from the IRA negotiation program. Nevertheless, other elements of the IRA may have a material impact on our business, including the redesign of the Part D benefit and the Manufacturer Discount Program, which requires manufacturers to take on more of the beneficiary cost previously subsidized by the federal government through the application of increased drug discounts.

We anticipate that the U.S. government will continue to engage in activities seeking to address drug pricing and reimbursement. Furthermore, certain states have enacted laws establishing Prescription Drug Affordability Boards (“PDABs”). Some state PDABs, including those in Colorado, Maryland, Washington, and Minnesota, either have the authority or have defined a pathway pursuant to which they may be granted the authority to establish upper payment limits for prescription drugs. In certain states, there is pending litigation that would establish a PDAB or expand the authority of an existing PDAB. Additionally, the U.S. government continues to focus on obtaining most-favored-nation pricing on U.S. prescription drug prices in government programs. For example, CMS recently issued a proposed rule called the Guarding U.S. Medicare Against Rising Drug Costs Model (“GUARD”). GUARD is a proposed mandatory model that would assess rebates for certain drugs payable under Medicare Part D if the prices exceed those paid in economically comparable countries. While there is significant uncertainty around the potential implementation of GUARD and related executive orders and rulemaking, implementation of mandatory initiatives could result in reduced pricing and reimbursement for our products.

*Outside the United States.* In Europe and other foreign jurisdictions, the success of our products depends largely on obtaining and maintaining government reimbursement, because patients are generally unable to access prescription pharmaceutical products that are not reimbursed by their governments. In some countries, such as Germany, commercial sales of a new product may begin while pricing and reimbursement terms are under discussion. In other countries, a company must complete reimbursement negotiations prior to the commencement of commercial supply of the pharmaceutical product.

The requirements governing drug pricing vary widely country-by-country and region-by-region. For example, the member states of the E.U. can restrict the range of drugs for which their national health insurance systems provide reimbursement and can control the prices of prescription drugs. Many countries in the E.U. also attempt to contain drug costs by engaging in some form of reference pricing in which authorities examine pre-determined internal or external markets for published prices of a product or national class of drugs. In addition, many ex-U.S. government payors require companies to provide health economic assessments of products, which are evaluated by government agencies set up for this purpose. A

member state may approve a specific price for the drug, or it may instead adopt a system of direct or indirect controls on the total amount of money that a company may receive for supply of a drug. Countries also may consider increasing mandatory discounts over time in an attempt to manage increased demands on healthcare budgets. Reimbursement discussions in foreign countries often result in a reimbursement price that is lower than the net price that companies can obtain for the product in the U.S.

In addition, reimbursement discussions may take a significant period of time resulting in commercialization delays. In some countries where reimbursement has not yet been obtained, or where there are a limited number of eligible people and our medicines or therapies are unregistered, the governments of such countries may agree to purchase our medicines and therapies on an unlicensed and/or named patient basis. Reimbursement for our products cannot be assured because a country or region may only provide for reimbursement on terms that we do not deem adequate.

Further, many governments outside of the U.S. have introduced or are in the process of introducing legislation focusing on cost containment measures in the pharmaceutical industry. The impact of these laws where finalized, the final form of laws under consideration, and their relevant practical application, are unknown at this time, but may lead to lower prices, paybacks, or other forms of discounts or special taxes. Reforms in our product markets, including those that may stem from periods of uneven economic growth or downturns or uncertainty, or as a result of high inflation, emergence, or escalation of, and responses to, international tension and conflicts, or government budgeting priorities, may continue to result in added pressure on pricing, access, and reimbursement for our products.

### ***Other Regulations***

The manufacturing process for pharmaceutical products is highly regulated and regulators may shut down manufacturing facilities that they observe are not complying with regulations. We, our commercial manufacturing organizations (“CMOs”) and our corporate partners are subject to cGMP, which are extensive regulations governing manufacturing processes, stability testing, record keeping and quality standards as defined by FDA and EMA. Similar regulations are in effect in other jurisdictions. Suppliers of key components and materials must be named in the NDA or marketing authorization application filed with the regulatory authority for any product candidate for which we are seeking marketing approval, and significant delays can occur if the qualification of a new supplier is required. Even after our facilities or a third-party supplier is qualified by the regulatory authority, investment and effort must continue to be expended in the areas of production and quality control to maintain full compliance with applicable regulatory requirements, including cGMP. Our manufacturing operations and third-party suppliers are subject to regular periodic inspections by regulatory authorities following initial approval.

Pharmaceutical companies must also monitor information on side effects and adverse events reported during clinical studies and after marketing approval and report such information and events to regulatory agencies. Non-compliance with the applicable safety reporting requirements may result in civil or criminal penalties. Side effects or adverse events that are reported during clinical trials can delay, impede or prevent marketing approval. Based on new safety information that emerges after approval, the FDA can mandate product labeling changes, impose risk evaluation and mitigation strategies, require new post-marketing studies (including additional clinical trials) or suspend or withdraw approval of the product. These requirements may affect our ability to maintain marketing approval of our products or require us to make significant expenditures to obtain or maintain such approvals.

Pharmaceutical companies are also subject to various laws pertaining to healthcare “fraud and abuse,” including the federal Anti-Kickback Statute (“AKS”), the False Claims Act (“FCA”), and other state and federal laws and regulations in and outside of the U.S. In the U.S., the Anti-Kickback Statute generally makes it illegal to knowingly and willfully solicit, offer, receive or pay any remuneration in return for or to induce the referral of business, including the purchase or prescription of a particular drug that is reimbursed by a state or federal health care program. The FCA prohibits knowingly and willingly presenting or causing to be presented for payment to third-party payors (including Medicare and Medicaid), any claims for reimbursed drugs or services that are false or fraudulent, claims for items or services not provided as claimed or claims for medically unnecessary items or services. Violations of fraud and abuse laws may be punishable by criminal and/or civil sanctions, including fines and civil monetary penalties, as well as by the possibility of exclusion from federal healthcare programs (including Medicare and Medicaid). Liability under the FCA may also arise when a violation of certain laws or regulations related to the underlying products (e.g., violations regarding improper promotional activity, manufacturing regulations, or unlawful payments) contributes to the submission of a false claim. If we were subject to allegations concerning, or convicted of violating, these laws, our business could be harmed.

Laws and regulations also have been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers. The laws and regulations generally limit financial interactions between manufacturers and health care providers, require manufacturers to adopt certain compliance standards or require disclosure to the government and public of such interactions. The laws include U.S. federal and state “sunshine” provisions. The federal sunshine provisions apply to pharmaceutical manufacturers with products reimbursed under certain government programs and require those manufacturers to disclose annually to the federal government (for re-disclosure to the public) certain payments and other transfers of value made to physicians, physicians assistants, advanced practice registered nurses, and teaching hospitals. State laws may also require disclosure of pharmaceutical pricing information and marketing expenditures. Many of these laws and regulations contain requirements that are subject to interpretation. Outside the U.S., other countries have implemented laws and regulations limiting financial interactions between manufacturers and health care providers and providing requirements for disclosure of financial interactions with healthcare providers and additional countries may consider or implement such laws.

We are subject to various federal and foreign laws that govern our international business practices with respect to payments to government officials. Those laws include the U.S. Foreign Corrupt Practices Act (“FCPA”), which prohibits U.S. companies and their representatives from paying, offering to pay, promising, or authorizing the payment of anything of value to any foreign government official, government staff member, political party, or political candidate for the purpose of obtaining or retaining business or to otherwise obtain favorable treatment or influence a person working in an official capacity. In many countries, the health care professionals we regularly interact with may meet the FCPA’s definition of a foreign government official. We are also subject to U.K. Bribery Act 2010 (“the Bribery Act”), which proscribes giving and receiving bribes in the public and private sectors, bribing a foreign public official, and failing to have adequate procedures to prevent employees and other agents from giving bribes. U.S. companies that conduct business in the U.K. generally will be subject to the Bribery Act.

We are subject to extensive privacy and data protection laws and regulations concerning the collection, use and sharing of personal data. We routinely collect and use sensitive personal information relating to health. The legislative, regulatory and litigation landscape for privacy and data protection requirements is rapidly evolving and changing, and may limit our ability to use data globally or across borders. For example, the E.U. General Data Protection Regulation (“GDPR”) imposes obligations on us with respect to our processing of personal data and the cross-border transfer of such data, including higher standards of obtaining consent, more robust transparency requirements, data breach notification requirements, requirements for contractual language with our data processors, and stronger individual data rights. In addition, several U.S. jurisdictions have similar data privacy laws, such as the California Consumer Privacy Act and California Privacy Rights Act. Data protection requirements are not universal and can conflict between jurisdictions. There has also been an increase in enforcement actions from the Federal Trade Commission, with a specific focus on companies operating health-related websites. Compliance with these laws and regulations is made more complex by the lack of consistent standards, common definitions, or clear regulatory expectations. At the same time, enforcement of these laws and regulations is increasing and litigation, fines, and penalties are also becoming more common.

In addition, as we expand our pipeline and contemplate different approaches that may incorporate the use of medical devices, such approaches may necessitate compliance with regulatory laws applicable to medical devices, including those governing the testing, manufacture, approval, distribution, and marketing of medical devices. Furthermore, the extent of government regulation, which might result from future legislation or administrative action, cannot accurately be predicted.

## **EMPLOYEES AND HUMAN CAPITAL MANAGEMENT**

As of December 31, 2025, we had approximately 6,400 employees. Of these employees, approximately 5,200 were based in the U.S. and approximately 1,200 were based outside the U.S. None of our U.S. employees are covered by a collective bargaining agreement. A small number of employees outside the U.S. are covered by such agreements due to local law or industry requirements. We consider our relations with our employees to be good.

We rely on skilled, experienced, and innovative employees to conduct the operations of our company. The biotechnology industry is very competitive, and recruiting and retaining such employees is important to the continued success of our business. We are committed to building an outstanding, committed, and passionate team, and we focus on a culture that values all employees. We focus on recruiting, retaining, and developing qualified and talented employees from a range of backgrounds to conduct our research, development, commercial, and other business activities because we believe that each employee brings unique perspectives and strengths, and by embracing these strengths, we can do our best work for patients.

We support our employees through a variety of initiatives including learning resources and forums that promote belonging in our workplaces; five global employee resource networks open to all employees that promote connectivity and collaboration across levels and functions; and investments that advance access to opportunity in our surrounding communities.

To promote our employees' continued well-being, we offer comprehensive benefits and resources, including those focused on health and income protection, such as life insurance and retirement savings programs. We continue to promote and enhance wellness tools supporting our employees' mental, social, physical and financial health. We continually review and augment our programs to include benefits that support the evolving needs of our workforce.

In addition, we provide our employees with career development and advancement opportunities, including job rotations, mentoring, and training. We are committed to identifying and developing our next generation of leaders, which is reflected in our manager excellence and talent readiness programs designed for critical roles in our organization.

## OTHER MATTERS

### *Financial Information and Significant Customers*

We operate in one segment, pharmaceuticals. Financial information about our revenue by product and significant customers is set forth in Note Q, "Segment Information," to our consolidated financial statements included in this Annual Report on Form 10-K.

### *Information Available on the Internet*

Our internet address is [www.vrtx.com](http://www.vrtx.com). Our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, and all amendments to those reports, are available to you free of charge through the "Investors/Financial Information/SEC Filings" section of our website as soon as reasonably practicable after those materials have been electronically filed with, or furnished to, the Securities and Exchange Commission.

### *Corporate Information*

Vertex was incorporated in Massachusetts in 1989, and our principal executive offices are located at 50 Northern Avenue Boston, Massachusetts 02210.

## INFORMATION ABOUT OUR EXECUTIVE OFFICERS

*The names, ages and positions held by our executive officers are as follows:*

<b>Name</b>	<b>Age</b>	<b>Position</b>
Reshma Kewalramani, M.D.	53	Chief Executive Officer and President
Jeffrey M. Leiden, M.D., Ph.D.	70	Executive Chairman
E. Morrow "Morrey" Atkinson, III, Ph.D.	60	Executive Vice President, Chief Technical Operations Officer, Head of Biopharmaceutical Science and Manufacturing Operations
Jonathan Biller, J.D.	62	Executive Vice President, Chief Legal Officer
Carmen Bozic, M.D.	63	Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer
Mark Bunnage, D.Phil	57	Executive Vice President, Chief Scientific Officer
Duncan J. McKechnie	57	Executive Vice President, Chief Commercial Officer
Amit K. Sachdev, J.D.	58	Executive Vice President, Chief Patient and External Affairs Officer
Ourania "Nia" Tatsis, Ph.D.	56	Executive Vice President, Chief Regulatory and Quality Officer
Charles F. Wagner, Jr.	57	Executive Vice President, Chief Operating and Financial Officer
Kristen C. Ambrose, CPA	49	Senior Vice President, Chief Accounting Officer

Dr. Kewalramani has been our Chief Executive Officer (CEO) and President since April 2020 and a member of our Board of Directors since February 2020. Dr. Kewalramani was our Executive Vice President and Chief Medical Officer from April 2018 through April 2020. She was our Senior Vice President, Late Development from February 2017 until April 2018.

Dr. Kewalramani also served on the board of Ginkgo Bioworks from September 2021 to June 2024. From August 2004 to January 2017, she served in roles of increasing responsibility at Amgen Inc., most recently as Vice President and Head of U.S. Medical Organization. From 2014 through 2019, Dr. Kewalramani was the industry representative to the FDA's Endocrine and Metabolic Drug Advisory Committee. She completed her internship and residency in Internal Medicine at the Massachusetts General Hospital and her fellowship in Nephrology at the Massachusetts General Hospital and Brigham and Women's Hospital combined program. Dr. Kewalramani holds a B.A. from Boston University and an M.D. from Boston University School of Medicine. She is an alumna of the Harvard Business School, having completed the General Management Program.

Dr. Leiden is our Executive Chairman, a position he has held since in April 2020. He was our Chief Executive Officer and President from 2012 through March 2020. He has been a member of our Board of Directors since July 2009, the Chairman of our Board of Directors since May 2012, and served as our lead independent director from October 2010 through December 2011. Dr. Leiden was a Managing Director at Clarus Ventures, a life sciences venture capital firm, from 2006 through January 2012. Dr. Leiden was President and Chief Operating Officer of Abbott Laboratories, Pharmaceuticals Products Group, and a member of the Board of Directors of Abbott Laboratories from 2001 to 2006. From 1987 to 2000, Dr. Leiden held several academic appointments, including the Rawson Professor of Medicine and Pathology and Chief of Cardiology and Director of the Cardiovascular Research Institute at the University of Chicago, the Elkan R. Blout Professor of Biological Sciences at the Harvard School of Public Health, and Professor of Medicine at Harvard Medical School. He is an elected member of both the American Academy of Arts and Sciences and the Institute of Medicine of the National Academy of Sciences. Dr. Leiden was a director and the non-executive Vice Chairman of the board of Shire plc, from 2006 to January 2012, a director of Quest Diagnostics, from December 2014 to May 2019, and the Chairman of Revolution Healthcare Acquisition Corp., from April 2021 to December 2022. Dr. Leiden received his M.D., Ph.D. and B.A. degrees from the University of Chicago.

Dr. Atkinson has been our Executive Vice President, Chief Technical Operations Officer, Head of Biopharmaceutical Sciences and Manufacturing Operations since August 2023. He previously served as our Senior Vice President, Head of Commercial Manufacturing and Supply Chain since July 2020. Prior to joining us, Dr. Atkinson served in various roles at Bristol-Myers Squibb Co., including as Senior Vice President, Global Manufacturing Operations from September 2019 to June 2020; Vice President and Integration Leader, Corporate Cell Therapy and Global Development and Manufacturing from January 2019 to September 2019; Vice President, Internal Manufacturing, Biologics from June 2017 to January 2019; and Vice President, Biologics Development and Clinical Manufacturing from 2012 to June 2017. Before Bristol-Myers Squibb, he held various roles at Cook Pharmica, LLC (now owned by Novo Holdings) and Eli Lilly. Dr. Atkinson served as a member of the Board of Directors of 89bio, Inc. from February 2022 until October 2025, when it was acquired by Roche. Dr. Atkinson holds a B.S. in Biology from Indiana University and a Ph.D. in Biological Sciences from Stanford University.

Mr. Biller has been our Executive Vice President, Chief Legal Officer since September 2022. From November 2019 until he joined us, Mr. Biller served in several executive roles at Agios Pharmaceuticals, Inc., including Chief Legal Officer and, most recently, Chief Financial Officer and Head of Corporate Affairs. Prior to Agios, he served as Executive Vice President, General Counsel at Celgene from July 2018 to November 2019, where he was responsible for their global legal function, and served as Senior Vice President, Tax and Treasury from 2011 to June 2018. Prior to Celgene, Mr. Biller was General Counsel, Chief Tax Officer and Secretary at Bunge Limited, a global publicly traded agriculture and food company. Earlier in his career he held various leadership roles at Alcon, Inc. and was a partner at Hopkins & Sutter and Foley & Lardner. Mr. Biller holds a B.A. from Brown University and a J.D. from Yale Law School.

Dr. Bozic is our Executive Vice President, Global Medicines Development and Medical Affairs, a position she has held since October 2019, and she has been our Chief Medical Officer since April 2020. She was our Senior Vice President and Head of Global Clinical Development from May 2019 to October 2019. Prior to joining us, Dr. Bozic spent more than 20 years at Biogen Inc., a biotechnology company focused on neurological diseases, most recently as Senior Vice President of Global Development and Portfolio Transformation from 2015 to May 2019 and as Senior Vice President of Clinical and Safety Sciences from 2013 to 2015. Dr. Bozic has served as the industry representative to the FDA's Risk Communication Advisory Committee, and was a member of PhRMA's Clinical and Preclinical Development Committee and the Board of Managers at BioMotiv. She received her M.D., C.M., completed her residency, and was Chief Resident in Internal Medicine at McGill University. She completed her fellowship in Pulmonary and Critical Care Medicine at Brigham and Women's Hospital and was an Associate Physician at Beth Israel Deaconess Medical Center and Harvard Medical School before joining the biopharmaceutical industry.

Dr. Bunnage is our Executive Vice President and Chief Scientific Officer, a position he has held since February 2026. He was our Senior Vice President & Head of Global Research from March 2024 through January 2026, our Senior Vice President & Head of Research from July 2021 to March 2024, and our Senior Vice President & Site Head, Boston Research, from August 2016 to July 2021. Prior to joining Vertex, Dr. Bunnage had a 20-year career at Pfizer Inc. where he held positions of increasing responsibility, including Vice President, Worldwide Medicinal Chemistry and Head of Medicinal Chemistry, Sandwich Laboratories. Dr. Bunnage is a Fellow of the Royal Society of Chemistry and a Fellow of the Royal Society of Biology. He also serves as a visiting professor in chemistry at the University of Oxford, United Kingdom, and is a member of the Strategic Advisory Board for the Department of Chemistry at the University of Durham, United Kingdom. Dr. Bunnage received his B.Sc in Chemistry from the University of Durham and his D.Phil in Chemistry from the University of Oxford. He completed his postdoctoral research as a NATO Fellow at The Scripps Research Institute in La Jolla, California.

Mr. McKechnie is our Executive Vice President, Chief Commercial Officer, a position he has held since July 1, 2025. Mr. McKechnie previously served as our Senior Vice President, Head of North America Commercial from October 2018 to July 2025, and as our Vice President of Global Marketing from June 2013 to September 2018. Prior to joining Vertex, Mr. McKechnie held positions of increasing responsibility at Novartis AG, including Vice President, Respiratory Franchise from January 2013 to June 2013; Vice President and Head Brand Maximization and Established Medicines from April 2012 to April 2013; and Vice President, Cardiovascular Marketing from November 2008 to March 2012. Before Novartis, Mr. McKechnie held various roles at GlaxoSmithKline plc. Mr. McKechnie holds a Business & Marketing degree from the University of Plymouth in England.

Mr. Sachdev is our Executive Vice President, Chief Patient and External Affairs Officer, a role he has held since July 2023. From October 2019 to July 2023, he was our Executive Vice President, Chief Patient Officer. In addition, Mr. Sachdev served in the role of Chief of Staff to the CEO from April 2020 to March 2023. He served as our Executive Vice President and Chief Regulatory Officer from January 2017 until September 2019, and as our Executive Vice President, Policy, Access and Value from October 2014 through December 2016. In 2010, he established our first international commercial operations in Canada. In 2007, he joined us as a Senior Vice President, to establish our government affairs and public policy activities, as well as our patient advocacy programs. Prior to joining us, Mr. Sachdev served as Executive Vice President, Health, of the Biotechnology Industry Organization (BIO) and was the Deputy Commissioner for Policy at the FDA, where he also served in several other senior positions. Prior to the FDA, Mr. Sachdev served as Majority Counsel to the Committee on Energy and Commerce in the U.S. House of Representatives and practiced law at the American Chemistry Council, and subsequently at the law firm of Ropes & Gray LLP. He served as a member of the Board of Directors of Eiger BioPharmaceuticals from April 2019 to September 2024. Mr. Sachdev holds a B.S from Carnegie Mellon University and a J.D. from Emory University School of Law.

Dr. Tatsis is our Executive Vice President, Chief Regulatory and Quality Officer, a position she has held since August 2020. Previously, she was our Senior Vice President and Chief Regulatory Officer from October 2019 to August 2020, and our Senior Vice President, Global Regulatory Affairs from September 2017 to October 2019. Prior to joining us, Dr. Tatsis held positions of increasing responsibility at several pharmaceutical companies, including Sanofi, Stemion, Pfizer, and Wyeth. Most recently, from 2014 to 2017, she was Vice President, Head of Global Regulatory Affairs, at the Sanofi Genzyme Business Unit focused on Inflammation/Immunology, Rare Disease, Multiple Sclerosis, Ophthalmology, Neurology, and Oncology/Immuno-Oncology. Dr. Tatsis also worked as an associate staff scientist and research fellow in Immunology and Vaccine Development at the Wistar Institute and completed a post-doctoral research fellowship in Immunology at Thomas Jefferson University. Dr. Tatsis has served as a member of the board of directors at Odyssey Therapeutics since October 2025, and previously served on the board of directors of Verve Therapeutics from June 2024 until July 2025, when it was acquired by Eli Lilly. She received her Ph.D. in Cell and Molecular Biology from the University of Vermont and holds a B.S. in Biology from Temple University.

Mr. Wagner is our Executive Vice President, Chief Operating & Financial Officer, a position he has held since July 2025. Mr. Wagner was our Executive Vice President, Chief Financial Officer from April 2019 through June 2025. Prior to his role at Vertex, Mr. Wagner was Chief Financial Officer and Executive Vice President, Finance, of Ortho Clinical Diagnostics, a Carlyle Group portfolio company, from June 2015 to March 2019. In that role, he led the finance, accounting, tax, treasury, global financial systems, lender relations, and acquisitions and divestiture groups. From July 2012 to June 2015, Mr. Wagner served as Executive Vice President, Chief Financial Officer of Bruker Corporation, a scientific instruments manufacturer. Prior to that, Mr. Wagner served as Chief Financial Officer for Progress Software Corporation, a provider of enterprise software, and Millipore Corporation, a global provider of products and services in the life science tools market. Mr. Wagner served as a director of Good Start Genetics, Inc., from April 2014 to August 2017 and served as a director and

member of the Audit Committee of Bruker Corporation from August 2010 to June 2012. He has served as a member of the Board of Directors of The TJX Companies, Inc., since September 2023. Mr. Wagner holds a B.S. in Accounting from Boston College and a M.B.A from Harvard Business School.

Ms. Ambrose is our Senior Vice President, Chief Accounting Officer, a position she has held since May 2021. Ms. Ambrose previously served as our Senior Vice President, Accounting, Tax, Treasury, Strategic Sourcing and Corporate Services since March 2021. From February 2003 until she joined us, Ms. Ambrose held roles of increasing responsibility at Boston Scientific Corporation, a medical device company, most recently as Vice President of Finance and Controller of the Global Endoscopy Division from July 2019 to March 2021 and as Vice President of Global Internal Audit from February 2017 to June 2019. Prior to Boston Scientific Corporation, Ms. Ambrose served as an accountant at Ernst & Young LLP. She received her B.S. in Commerce from the University of Virginia and is a Certified Public Accountant.

## ITEM 1A. RISK FACTORS

*Investing in our common stock involves a high degree of risk, and you should carefully consider the risks and uncertainties described below in addition to the other information included or incorporated by reference in this Annual Report on Form 10-K. If any of the following risks or uncertainties occur, our business, financial condition or results of operations would likely suffer, possibly materially. In that case, the trading price of our common stock could decline.*

### **Risks Related to Our Business and Products**

***Our success depends on our ability to develop and commercialize additional medicines.***

We invest significant resources in research and development to discover and develop transformative medicines for people with serious diseases. Product development is highly uncertain and expensive. Product candidates may appear promising in research and development but may fail to reach commercial success for many reasons, including:

- the failure to establish safety and efficacy through clinical trials;
- the failure to obtain marketing approval;
- the inability to manufacture on economically feasible terms;
- the failure to gain and maintain market acceptance among physicians and patients or other members of the medical community;
- the failure to obtain adequate pricing or reimbursement levels from third-party payors or foreign governments; and
- competition based on, among other factors, safety, efficacy, patient convenience, pricing and reimbursement.

If we are not able to successfully develop and commercialize additional medicines, our business would be materially harmed.

***Our business is substantially dependent on the success of our CF medicines.***

Substantially all our net product revenues have been derived from the sale of our CF medicines. We may be unable to sustain or increase revenues from sales of our CF medicines in the future for any number of reasons, including the potential introduction of competitive products or the inability to successfully develop and commercialize next-generation medications or medicines to treat people with CF who cannot benefit from our current CF medicines. Our concentrated source of revenue increases the risks associated with potential manufacturing or supply disruptions, safety issues that may be identified with respect to our CF medicines, and failure to gain and/or maintain market acceptance or adequate pricing or reimbursement for our CF medicines. If we are unable to sustain or increase revenues from sales of our CF medicines, or if we do not meet the expectations of investors, our business would be materially harmed and our ability to fund our operations could be adversely affected.

***If we are unable to successfully develop and commercialize medicines for acute and neuropathic pain, our business could be materially harmed.***

A portion of the value attributed to our company by investors is based on the expected commercial success of JOURNAVX for acute pain and on our development programs for both acute and peripheral neuropathic pain. JOURNAVX may not gain or maintain market acceptance among physicians, patients, or payors due to various factors, including the availability of lower-cost alternatives, and sales, marketing, pricing, and/or distribution challenges associated with introducing a product into a highly competitive market. Furthermore, we may not succeed in developing JOURNAVX for additional indications or in advancing other product candidates, including NaV1.8 or NaV1.7 inhibitors, for the treatment of acute or peripheral neuropathic pain. Even if we obtain marketing approvals for these product candidates, they will face significant competition and there can be no assurance of commercial success.

***We may not be able to increase or maintain CASGEVY product revenues.***

The future commercial success of CASGEVY depends on physicians, patients, or payors accepting it as medically useful, cost-effective, ethical, safe, and preferred with respect to current and potential future competitive therapies, and on

payors providing adequate reimbursement. In addition to risks generally associated with the commercialization of medicines, the cell collection processes, manufacturing and other procedures required to manufacture and administer CASGEVY are more complex, resource-intensive, and operationally demanding than for small molecules. For example, the cost of manufacturing CASGEVY as a percentage of revenue is significantly higher than for our CF medicines. Moreover, market acceptance continues to be dependent in part on the prevalence and severity of side effects associated with the procedure by which CASGEVY is administered, including those resulting from the myeloablative preconditioning regime. There can be no assurance that we will be able to increase or maintain our revenues from CASGEVY in future periods.

### **Risks Related to Commercialization**

***We are subject to pricing and reimbursement pressures that could have a material adverse effect on our business, revenues, and results of operations.***

Revenues from our products depend, to a large degree, on the extent to which the products are purchased by customers, such as wholesalers, pharmacies, and hospitals, and reimbursed by third-party payors, such as government health programs, commercial insurers, and managed health care organizations. Increasingly, these third-party payors are becoming more critical in evaluating and reimbursing medicines. The containment of health care costs continues to be a priority for many governments, and drug pricing has been a focus in this effort. The U.S. federal government and state legislatures and foreign governments have shown significant and evolving interest in implementing cost-containment programs, including price controls, restrictions on reimbursement, value-based and reference pricing, compulsory licensing, including the pursuit of so-called “march in” rights, and mandatory substitution with generic products, all of which could limit the prices of, or access to, our products. Decisions by third-party payors to not cover a product or restrict access to a product may shift over time and could reduce market acceptance of the product and limit product revenues. We must also compete to be placed on formularies of managed care providers, as exclusion of our products from a formulary would limit usage by managed care providers and patients.

In the U.S., pricing and access is primarily governed by practices of private managed care providers and institutional and governmental purchasers, federal laws and regulations related to Medicare and Medicaid, including the ACA and the IRA, and state activities, including the establishment of PDABs and price transparency rules. For example, in August 2023, the Colorado PDAB selected five drugs for an affordability review, including TRIKAFTA. Although the Colorado PDAB later found TRIKAFTA to be ineligible for an upper payment limit we cannot predict whether future reviews by the Colorado PDAB, or any other PDAB, will come to the same conclusion about TRIKAFTA or any of our other therapies, or the amount of any potential upper payment limit. Furthermore, changes to the health care system enacted as part of health care reform in the U.S., as well as increased purchasing power of entities that negotiate on behalf of Medicare, Medicaid, and private payors, could result in further pricing pressures. For example, initiatives by the U.S. government to impose most-favored-nation pricing on U.S. prescription drug prices in government programs, including the recently proposed GUARD Model by the CMS. While there is significant uncertainty around the related executive orders and rulemaking, mandatory initiatives could result in reduced pricing and reimbursement for our products.

In most markets outside of the U.S., the pricing and reimbursement medicines is subject to governmental control and governments are making greater efforts to reduce drug prices and limit drug spending. The reimbursement process in ex-U.S. markets vary widely and can take a significant time to complete, and reimbursement decisions are made on a country-by-country and region-by-region basis. Reimbursement for our products by governments, including the timing of any reimbursements, may also be affected by budgetary or political constraints, particularly in challenging economic environments. We have experienced challenges in obtaining timely reimbursement for our products in various countries outside the U.S., and our future revenues depend on maintaining such reimbursement. There is no assurance that coverage and reimbursement will continue for our current products or be available for our future products. Even if reimbursement is available, there is no assurance that the timing or level of reimbursement will be sufficient. Furthermore, many ex-U.S. governments are introducing new legislation focused on cost containment measures applicable to the pharmaceutical industry; such legislation, if finalized, could lead to lower prices, rebates or other forms of discounts or special taxes.

Our failure to obtain or maintain adequate prices, coverage, or reimbursement for our products would have an adverse effect on our business, revenue and results of operations, could curtail or eliminate our ability to adequately fund our research and development programs and/or could cause a decline or volatility in our stock price.

***Competing products and technological advances from our competitors may negatively affect our business and market position.***

Our products and product candidates face or may face competition from existing and potential competing products. See also *Item 1., Business – Competition* of this Annual Report on Form 10-K. Competing products may be more effective, safer, more effectively marketed, have lower prices or better coverage or reimbursement levels, eliminate or minimize the need for treatment with our products or product candidates, or have other differentiating factors that negatively affect the demand for our products or product candidates. If a competitor obtains approval and reimbursement before we do, approval and/or reimbursement of our products or product candidates could be delayed, denied, or otherwise adversely affected. We compete with an array of companies and other organizations, including those that have substantially greater resources, more mature development, manufacturing and commercial organizations, and/or other competitive advantages. Smaller companies with innovative programs or technologies are frequently acquired by and enter into collaborations with larger competitors, which may result in the acceleration or enhancement of competitive programs. We cannot predict the timing or impact of the introduction of competitive products. If a competing product is successfully developed and commercialized for a patient population we are currently treating or are seeking to treat, our revenues, business or market position could be materially adversely affected. In addition, the release of new information, including clinical data and regulatory approval timelines, by our competitors regarding competitive products or potentially competitive product candidates can affect investors' perceptions regarding the prospects of our products and product candidates, and has caused and may in the future cause our stock price to decline or experience periods of significant volatility.

***If we discover safety or efficacy issues with any of our products, commercialization efforts for the product could be negatively affected, the approved product could lose its approval, and our business could be materially harmed.***

After regulatory approval and launch, our products are used over longer periods of time and by larger populations of patients than during pre-approval clinical trials. Additional clinical and non-clinical studies, such as for label expansions, new combinations or otherwise, may also be conducted after regulatory approval. For example, as part of FDA approval for CASGEVY, we are required to conduct post-marketing safety studies to assess certain long-term risks associated with the treatment. Additionally, when post-marketing studies involve our marketed products, or an active pharmaceutical ingredient thereof, they can raise new safety issues for our existing products. The subsequent discovery or appearance of previously unknown or underestimated safety or efficacy concerns with a product could negatively affect commercial sales of the product, result in reduced coverage or reimbursement by payors, cause reputational harm, government investigations, and/or lawsuits against us. Subsequent adverse safety events, as well as safety or efficacy issues affecting suppliers or competing products, may also lead to recalls, denial or withdrawal of regulatory approvals, non-renewal of conditional regulatory approvals, label changes, obligations to conduct additional or more extensive clinical trials or to implement a risk management plan, and reductions in market acceptance. Each of our CF products shares at least one active pharmaceutical ingredient with another of our products. If any of our CF products were to experience safety issues or labeling modifications, our other CF products may be adversely affected. For example, in December 2024, the FDA required us to modify the TRIKAFTA label by revising information regarding liver injury and liver failure and moving that information from the “warnings and precautions” section to a “boxed warning” section; the FDA required similar language in the ALYFTREK label. In addition, safety or efficacy issues affecting suppliers' or competitors' products also may reduce the market acceptance of our products.

The discovery of safety events involving our products or public speculation about such events could limit or reduce product revenues and cause our stock price to decline or experience periods of volatility.

### **Risks Related to Product Development**

***The data from our product development activities may not support advancement or regulatory approval of our product candidates, or label expansions for our marketed products, or provide sufficient data to support the successful commercialization of our approved products.***

Extensive testing is required for our product candidates and for new indications of our marketed products. The outcomes of such clinical and non-clinical testing are highly uncertain, may not generate sufficient safety, efficacy, or other data, and may not support regulatory approval of our product candidates. Clinical and non-clinical testing, and in particular our later-stage clinical trials, are expensive and resource intensive. The data from our preclinical studies and other research activities have in the past and may in the future fail to predict results in clinical trials. For example, despite considerable non-clinical testing, the clinical study of VX-264 in T1D did not meet its efficacy endpoint. Similarly, results from earlier-stage clinical

trials may not be predictive of the results from later-stage clinical trials, or of the likelihood of approval of a product candidate for commercial sale. In addition, interim or preliminary data from a clinical trial may not be predictive of final results from the clinical trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment and treatment continues, more patient data become available, or as patients continue other treatments for their disease.

The data from our clinical programs may not support approval or successful commercialization of our product candidates, and we may be unable to recoup the significant research and development, clinical trial, acquisition-related, and other expenses incurred, which could have an adverse effect on our business, financial condition and results of operations, and/or cause our stock price to decline or experience periods of volatility.

In addition, results of our clinical trials and findings from nonclinical studies could lead to abrupt changes in our development activities, including the possible cessation of development activities associated with a particular product candidate or program. For example, after VX-264 did not meet its efficacy endpoint, we announced that the program would not advance into further clinical studies. Failure to advance product candidates through clinical development would impair our ability to commercialize products, which could materially harm our business, financial condition and long-term prospects.

Our research and development activities are highly regulated, and it is possible that the FDA and other regulatory authorities:

- pause or halt our clinical trials based on their assessment of the potential or actual risks of continuing;
- disagree with our conclusions about the results from our clinical trials;
- require additional clinical trials, including confirmatory trials, or disagree with our clinical trial design or endpoint;
- fail to approve the facilities or processes used to manufacture a product candidate, or our dosing or delivery methods;
- grant marketing approval that is more restricted than anticipated, including limiting indications to narrow patient populations and imposing safety monitoring requirements, or risk evaluation and mitigation strategies;
- withdraw approval of a product or indication, including when the product or indication was approved under an accelerated approval pathway and confirmatory studies were unsuccessful.

Furthermore, we periodically release new information, including clinical data, regarding our products and product candidates, which may affect investors' perceptions regarding our products and product candidates, and cause our stock price to decline or experience periods of significant volatility. For example, our stock price decreased in August 2025 after we released Phase 2 data for VX-993 and informed investors that the FDA did not see a path toward a broad peripheral neuropathic pain label for suzetrigine at that time. The timing of the release of information by us regarding our product development programs is often beyond our control and is influenced by the timing of receipt of communications from regulators and data from our clinical trials, among other things.

***If we fail to successfully conduct our clinical activities, our clinical trials or future regulatory approvals may be delayed or denied.***

Conducting clinical trials is a complex, lengthy and expensive process. Our ability to complete clinical trials on our anticipated timelines depends on numerous factors, including proper and efficient protocol design, regulatory and institutional review board approval, adequate patient enrollment and retention rates, and compliance with current good clinical practices. Delays or complications in clinical trials may arise from difficulties in enrolling or retaining patients, competition from other clinical trials, the occurrence of significant and/or unexpected adverse safety events, changes in regulatory requirements, supply chain issues or disruptions at clinical trial sites. Further, we may face additional challenges identifying and enrolling sufficient patients for clinical trials for rare diseases and cell and gene therapies due to small patient populations. With respect to cell and genetic therapies there may be additional concerns regarding the safety of these more novel therapeutic approaches to the treatment of these diseases. If we or our third-party clinical trial providers, including contract research organizations ("CROs"), do not successfully conduct and manage our clinical activities or adequately comply with regulatory requirements, our clinical trials may experience delays or increased costs, and the potential regulatory approval of a product candidate or

expansion of a label for a marketed product may be delayed or denied. Any delay in obtaining required regulatory approvals could adversely affect our ability to successfully commercialize a product candidate.

### **Regulatory, Intellectual Property and Other Legal Risks**

***The extensive regulatory framework governing the health care industry could adversely affect our ability to obtain approval and market our medicines and failure to comply with these regulations could result in fines, penalties or other non-monetary remedies.***

The health care industry is highly regulated and subject to complex and increasing regulations. U.S. federal and state regulators, including the FDA and comparable ex-U.S. regulators directly regulate our most critical business activities, including those related to research, development, manufacturing, and commercialization, as described in Item 1, “*Business – Government Regulation.*”

The process for obtaining regulatory approvals to market a product is costly and time consuming, and approvals may not be granted for future products, or additional indications of existing products, on a timely basis or at all. In addition, we cannot guarantee that we will remain compliant with applicable regulatory requirements once approval has been obtained. These requirements govern, among other things, our manufacturing practices, communications regarding our products, and reporting of safety events. Maintaining compliance with these extensive regulations is complex, expensive, and time consuming, and failure to comply may result in additional regulatory actions, including recalls, withdrawal or suspension of product approvals, civil and criminal charges, reputational harm, and fines, penalties, or other monetary or non-monetary remedies, including exclusion from receipt of payment from U.S. federal and state healthcare programs like Medicare and Medicaid. Compliance with the regulatory requirements for biologics and cell and gene therapies can be more burdensome, expensive and time-consuming than for other, better known or more extensively studied types of medicines, such as small molecules. Regulatory requirements governing cell and genetic therapy products have changed frequently and may continue to change in the future. Furthermore, risks relating to compliance with laws and regulations may be heightened as we continue to expand our global operations and enter new therapeutic areas with different patient populations, which may require different commercialization activities from those we currently utilize.

We expect that regulation of the healthcare industry will continue to evolve through political and legal action, as future proposals to reform healthcare systems are considered by U.S. and foreign governments and regulatory authorities. We cannot predict when additional changes in the healthcare industry in general, or the pharmaceutical industry in particular, will occur, or what the impact of such changes may be. For example, new proposals or requirements regarding local manufacturing of pharmaceutical products, enhanced data security and privacy measures, sustainability, importation restrictions, embargoes, or trade sanctions may negatively impact our business. In addition, our development and commercialization activities could be harmed or delayed by a shutdown of the U.S. government or events that affect the manner in which the FDA operates.

***Commercialization of our products requires that we operate in compliance with applicable health care laws, including laws regulating promotional activities, prohibiting fraud and abuse and requiring reporting of government pricing information.***

We market our products to health care providers and provide promotional materials and informational programs regarding the use of each product in these patient populations. In jurisdictions where permitted, we also market our products to patients for whom the applicable product has been approved, as well as to their caregivers. If a regulatory authority interprets any of our conduct, including our marketing practices or patient support programs, as promotion of unapproved uses or otherwise false and misleading, it could request that we modify or withdraw our promotional materials or issue corrective advertising. It could also take enforcement action, such as issuing warning or untitled letters, prohibiting certain of our activities, seizing products, and imposing civil fines and criminal penalties. It is also possible that other federal, state, or foreign enforcement authorities might take action if they believe that the alleged conduct led to the submission and payment of claims for unapproved uses of our product, which could result in significant fines or penalties. Even if it is later determined we were not in violation of these laws, we may be faced with negative publicity, incur significant expenses defending our actions, and have to divert significant management resources from other matters.

Our interactions with health care providers that prescribe or purchase our products are also subject to laws and regulations designed to prevent fraud and abuse in the sale and use of medicines and that place significant restrictions on the marketing practices of biopharmaceutical companies. The relationships between companies and health care providers are

scrutinized and have been the target of lawsuits and investigations alleging various problematic conduct, including submission of incorrect pricing information, improper promotion of pharmaceutical products, payments intended to influence the referral of health care business, submission of false claims for government reimbursement, and anticompetitive behavior. We are required to track and disclose financial interactions with health care providers and health care organizations, which may increase government and public scrutiny of these financial interactions. Failure to comply with these reporting requirements could result in significant civil monetary penalties. As we commercialize products for new patient populations and in new geographies, we will have more interactions with a broader set of healthcare providers and we must continue to expend significant efforts to establish, maintain and enhance systems and processes to comply with laws and regulations governing those interactions.

Government price reporting and payment regulations are also complex, requiring us to continually assess the methods by which we calculate and report pricing in accordance with these obligations. Our methodologies for calculations are inherently subject to assumptions and may be subject to review and challenge by various government agencies, which may disagree with our interpretation. If the government disagrees with our reported calculations, we may need to restate previously reported data and could be subject to additional financial and legal liability.

***If we are unable to obtain, maintain and enforce our intellectual property rights, our business could be harmed.***

Our success depends, in significant part, on our ability to obtain, maintain, and enforce patents and intellectual property rights such as trademarks and copyrights that protect our products, product candidates, and technologies. In addition, we rely upon trade secret protection and contractual arrangements to protect certain of our proprietary information. Due to the complexity of the legal standards and factual questions relating to the patentability, validity, and enforceability of patents covering pharmaceutical and biotechnological inventions and the scope of claims made under these patents, our ability to obtain, maintain and enforce our patents is uncertain. The initial grant of patents or regulatory exclusivity in the U.S. and ex-U.S. markets depends upon decisions of the patent offices, courts, and governments in those countries. We may fail to obtain, defend or otherwise preserve patent and other intellectual property rights, including certain forms of regulatory exclusivity, and our current intellectual property rights or protections and those we obtain in the future may not be broad enough or sufficient to protect our commercial interests in all countries where we conduct business.

In the U.S. and ex-U.S. markets, third parties have challenged and may continue to challenge, invalidate, or circumvent our patents and patent applications relating to our products, product candidates, and technologies. We have had and may continue to have disputes with respect to the rights to products, product candidates, and technologies developed in collaboration with other parties. If we cannot resolve disputes and obtain adequate intellectual property right protections, we may not be able to develop or market our products. Settlements of such proceedings could also result in reducing the period of exclusivity and other protections, resulting in a reduction in revenue from affected products. Any litigation, including litigation related to Abbreviated New Drug Applications (“ANDA”), litigation related to 505(b)(2) applications, interference proceedings to determine priority of inventions, derivations proceedings, inter partes review, oppositions to patents in foreign countries, litigation against our collaborators, or similar actions, could harm our business.

Difficulties in, or preclusion from, protecting our intellectual property rights in foreign jurisdictions could substantially harm our business. Third-party manufacturers may be able to sell generic versions of our products in countries that do not provide effective mechanisms for enforcement of our patents or other intellectual property rights. For example, we have experienced a violation of our intellectual property rights in Russia, where a copy product that infringes our patents has been made available. In addition, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties in certain circumstances. Compulsory licenses have been used in certain countries for market access purposes and, in some cases, as a cost-containment measure. Compulsory licenses issued for our patents may diminish or reduce revenue from those jurisdictions and negatively affect our results of operations. Third parties may also illegally distribute and sell counterfeit versions of our products. Copy or counterfeit products may not meet our rigorous manufacturing and testing standards and a patient who receives such product may be at risk for a number of dangerous health consequences. Our business and reputation could suffer harm as a result of illegally produced and distributed generic versions of our products, as well as counterfeit products sold under our brand name. The diversion of products from their authorized market into other channels may result in reduced revenues and negatively affect our profitability.

***If we are not able to operate without infringing upon intellectual property rights of third parties, our business could be harmed.***

Our competitors seek to protect their products, product candidates and proprietary information through patents, trademarks, trade secrets, and copyrights. Third parties have claimed and may claim in the future that our products or other activities infringe their intellectual property rights or that our employees have misappropriated their intellectual property rights. See also *Item 1., Business – Intellectual Property* of this Annual Report on Form 10-K. Resolving an intellectual property infringement or other claim can be costly and time consuming and may require us to enter into license agreements, which may not be available on commercially reasonable terms. A successful claim of patent infringement or other violation or misappropriation of intellectual property rights by a third party could subject us to significant damages and/or an injunction preventing the manufacture, sale, or use of the affected product or products, and/or require us to pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

***Our business has a substantial risk of product liability claims and other litigation liability.***

The testing, manufacturing, marketing and use of our products and product candidates involve substantial risk of product liability claims. These claims may be made directly by consumers, patients, healthcare providers, or others. Product liability claims and lawsuits and safety alerts or product recalls, regardless of their ultimate outcome, may decrease demand for our products or any product candidate for which we obtain marketing approval, and may have a material adverse effect on our business, results of operations, reputation, and our ability to market our products. Our product liability and clinical trial insurance may not provide adequate coverage against all potential liabilities.

There continues to be a significant volume of government and regulatory investigations and litigation against companies operating in our industry, as well as robust regulatory enforcement and whistleblower claims. Investigations into aspects of our business include inquiries, subpoenas, and other types of information demands from government and regulatory authorities. We are also involved in and are subject to other various legal proceedings, including litigation, and other dispute-related proceedings. These activities require significant financial and internal resources. This includes the arbitration initiated by the third party to whom the CFF has assigned its ALYFTREK royalty rights. Please see *Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations* of this Annual Report on Form 10-K for more information. The outcome of such legal proceedings, investigations or any other dispute-related proceedings are inherently uncertain and adverse developments or outcomes can result in significant expenses, monetary damages, penalties, injunctions, or other relief against us, and in the ALYFTREK arbitration, could result in higher future costs of goods if royalty fees are higher than anticipated. For a description of our litigation, investigation and other dispute-related matters, see Note P., *Commitments and Contingencies – Legal Matters and Other Contingencies*, included in this Annual Report on Form 10-K.

***We are subject to various and evolving laws and regulations governing the privacy and security of personal data.***

We are subject to a variety of evolving and developing data privacy and security laws and regulations in various jurisdictions related to the collection, storage, use, sharing, and security of personal data, including health information. Regulators globally are imposing data privacy and security requirements, such as the E.U.'s GDPR and other domestic data privacy and security laws, such as the California Consumer Privacy Act and the California Privacy Rights Act. These and other similar types of laws and regulations that have been or may be passed often include requirements with respect to personal information. Compliance with privacy laws and regulations is a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices. Failure to comply may result in liability through government enforcement, private actions, civil and criminal fines and penalties, litigation, and reputational harm. Although we are not directly subject to HIPAA, we could face penalties, including criminal liability, for knowingly obtaining or disclosing protected health information from non-compliant HIPAA-covered entities. The commercialization of cell and genetic therapies involves processing more personal data than traditional therapies, increasing our risk exposure. Furthermore, the number of government investigations, enforcement actions, and class action lawsuits related to data security incidents and privacy violations, particularly focused on online data sharing, continue to increase. Government investigations typically require significant resources and generate negative publicity, which could harm our business and reputation.

## Risks Related to Our Operations

***We may face manufacturing, supply, and distribution delays, difficulties, and disruptions, among other challenges, including at our third-party providers.***

We could be subject to significant supply interruptions for our commercial products or product candidates as a result of disruptions to our internal manufacturing capabilities or those of our suppliers or partners. Supply disruptions may result from a variety of factors, including shortages in product raw materials or labor, technical difficulties, regulatory inspections or restrictions, delays in construction, regulatory approval, and inspection of new facilities or the expansion of existing facilities, shipping or customs delays, inability to maintain compliance with quality or other regulations, including cGMP requirements, general global supply chain disruptions, and performance failures by us or any third-party manufacturer on which we rely. Disruption in our supply chain or manufacturing capabilities can result in shipment delays, inventory shortages, lot failures, product withdrawals, recalls and other interruptions in the commercial and clinical supply of our products and product candidates. Any such disruption with respect to our commercial products could result in a failure to meet market demand, could negatively affect our patients, could reduce our net product revenues and/or increase our costs. Any such disruption in the supply of product candidates to our clinical trials could negatively affect the subjects enrolled in our clinical trials and/or cause delays in our clinical trials and applications for regulatory approval.

Additionally, unfavorable geopolitical events could affect our ability to interact with or conduct business with specific vendors within our global supply network or could prevent or delay the transportation of supplies or products to their planned destination. For example, we depend on China-based suppliers for portions of our supply chain. Finding alternative suppliers due to geopolitical developments or otherwise may not be feasible or could take a significant amount of time and involve significant expense due to the nature of our products and the need to obtain regulatory approvals.

***If we are unable to maintain and expand our supply chain and manufacturing capabilities, our ability to develop our product candidates and manufacture our products would be harmed.***

We continue to invest in and expand our manufacturing capabilities and supplier relationships to ensure the stability of our supply chains and to support the anticipated demand for our products. Establishing, managing and expanding our global manufacturing capabilities and supply chain, particularly as we enter new therapeutic modalities, requires significant financial commitment. This includes the creation and maintenance of numerous third-party contractual relationships upon which we rely. There can be no assurance that we will be able to identify, establish and maintain additional manufacturers or capacity for our product candidates and products on a timely basis, on commercially reasonable terms, or at all. The foregoing risks may be heightened where our products and the materials that we utilize in our operations are manufactured by only one supplier or at only one facility. In addition, in the course of providing its services, a contract manufacturer may develop process technology related to the manufacture of our products or product candidates that the manufacturer owns, either independently or jointly with us. This would increase our reliance on that manufacturer or require us to obtain a license from that manufacturer to have our products or product candidates manufactured by other suppliers utilizing the same process.

In addition, we and our CMOs and corporate partners are subject to cGMP, as well as comparable regulations in other jurisdictions. Manufacturing operations are also subject to routine inspections by regulatory agencies. Even after a supplier is qualified by the regulatory authority, the supplier must continue to expend time, money and effort in the area of production and quality control to maintain full compliance with applicable regulatory requirements, including cGMP. If, as a result of these inspections, a regulatory authority determines that the equipment, facilities, laboratories or processes do not comply with applicable regulations and conditions of product approval, the regulatory authority may suspend the manufacturing operations. There can be no assurance that we or our CMOs and corporate partners will be able to remedy any deficiencies cited by FDA or other regulatory agencies in their inspections.

Furthermore, the manufacturing and logistics for drug products are highly complex and can require significant investment, including to scale-up manufacturing processes and to secure capacity at third parties with expertise to meet our requirements. This capacity may be limited by the number of other clinical trials and commercial manufacturing ongoing for other companies seeking similar support. There are many risks that could result in delays and additional costs, including the need to hire and train qualified employees and obtain access to necessary equipment and third-party technology. Additionally, even with relevant experience and expertise, drug manufacturers often encounter difficulties in scale-up and production, including difficulties with production costs and yields, quality control, and compliance with federal, state and foreign

regulations, which can prevent manufacturers from completing clinical trials or commercializing products on a timely or profitable basis, if at all.

***Reliance on third-party relationships could adversely affect our business.***

Our business depends on relationships with third parties, including activities critical to research, development, manufacturing, commercialization, and technology. For example, we rely on third parties such as CROs for the day-to-day management and oversight of our clinical trials, on CMOs for active ingredient manufacturing and finishing operations, and on logistics providers for the distribution of our products. We are expanding our relationships with CROs, CMOs, and other third parties as we enter markets in which we have no or limited experience. Failure by any of our third parties to meet their contractual, regulatory, or other obligations, any disruption in the relationship between Vertex and a third party upon whom we rely, or the failure of a third party to conduct activities in accordance with our expectations, could adversely affect the relevant research, development, manufacturing, commercial, or administrative activity and our business. The foregoing risks may be heightened as a result of the limited number or specialized nature of certain third parties, as we may not be able to replace such third party in a timely manner, on commercially reasonable terms, or at all.

The third parties upon which we rely are subject to their own operational and financial risks, as well as other difficulties, which, if realized, could negatively affect our business. If any of our third parties violate, or are alleged to have violated, any laws or regulations, including anti-corruption or anti-bribery regulations, the GDPR, or other laws and regulations, during the performance of their obligations to us, we could suffer financial and reputational harm or other negative outcomes, including possible legal consequences.

***If we fail to scale our operations to accommodate growth, our business may suffer.***

As we continue to expand our global operations and capabilities, we face increasing demands on our management and infrastructure. To effectively manage our growing business, we need to:

- implement and clearly communicate corporate-wide strategies and effectively prioritize resources;
- enhance our operational and financial infrastructure, including data and information controls;
- effectively leverage technology and automation where appropriate to enable efficient growth and remain competitive;
- improve our administrative, financial and management processes, including decision-making processes and budget prioritization;
- effectively grow, train and manage our global employee base; and
- expand our compliance and legal resources.

***A variety of risks associated with operating in foreign countries could materially adversely affect our business.***

Our global operations subject us to risks that could adversely affect our business and revenue. In addition to the ex-U.S. risks we face with respect to compliance with local laws and regulatory requirements, pricing and reimbursement, intellectual property, manufacturing capabilities and supply chain, foreign exchange risks, and reliance on third parties, risks associated with operating a global biotechnology company include the potential for:

- economic weakness, including recession and inflation, or political instability globally or with respect to particular foreign economies and markets;
- business interruptions resulting from geo-political actions, including war and terrorism;
- import and export licensing requirements, tariffs, trade barriers, and other trade and travel restrictions, the risks of which appear to have increased in the current political environment;
- credit risks related to our customers, which may be higher in less developed markets; and
- global or regional public health emergencies.

If any of the above risks were to occur, our revenues, results of operations, financial condition or business could be materially harmed.

Current or future U.S. legislation, including executive orders, or other new changes in laws, regulations or policies in the U.S. or other countries could negatively impact our business by increasing costs, decreasing demand for our products, and increasing government cost controls, among other risks. For example, U.S. legislation has been introduced to limit certain U.S. biotechnology companies from using equipment or services from select Chinese biotechnology companies, and others in Congress have advocated for limitations on those Chinese service providers' ability to engage in business in the U.S. We cannot predict what actions may ultimately be taken with respect to trade relations between the United States and China or other countries, what products and services may be subject to such actions, the effective date or duration of such actions, or what actions may be taken by the other countries in response to actions by the United States. If we are unable to obtain or use services from existing service providers or become unable to export or sell our products to any of our customers or service providers, our business could be materially and adversely affected.

***A breakdown or breach of our information technology systems, or unauthorized access to confidential information could adversely affect our business.***

We maintain and rely extensively on information technology systems and network infrastructures, internally and with third parties for the effective operation of our business. We collect, store, and transmit confidential information, including personal information, financial information and intellectual property. Disruption, infiltration, or failure of our information technology systems because of software or hardware malfunctions, computer viruses, cyber-attacks, employee theft or misuse, power disruptions, natural disasters or accidents could cause breaches of data security and/or loss of critical data, which in turn could materially adversely affect our business.

Cyber-attacks and incidents are increasing in their frequency, sophistication, and intensity, and are difficult to detect. Cyber-attacks are carried out by well-resourced groups and individuals with a wide range of motives and expertise. Due to the nature of some cyber-attacks and incidents, there is a risk that they may remain undetected for a period of time. Recent developments in the threat landscape include the use of adversarial artificial intelligence techniques and machine learning, as well as an increased number of cyber extortion attacks with higher financial ransom demand amounts and increasing sophistication and variety of ransomware techniques. Cyber-attacks and incidents also include manufacturing, hardware or software supply chain attacks, which could cause disruption to or a delay in the manufacturing of our products or product candidates, or lead to data privacy or security breach. We use cloud technologies and any failure by cloud or other technology service providers to adequately safeguard their systems and prevent cyber-attacks or data privacy incidents could disrupt our operations and result in misappropriation, corruption, or loss of confidential or proprietary information. The third parties upon which we rely face similar risks and when they experience a security breach of their systems, our security can be adversely affected.

Like many companies, we have experienced immaterial cybersecurity incidents, including temporary service interruptions of third-party suppliers. There can be no assurance that our efforts to protect our data and information systems will prevent breakdowns or breaches in our systems that could adversely affect our business. While we maintain cyber liability insurance, this insurance may not be sufficient to cover the financial, legal, business or reputational losses that may result from an interruption or breach of our systems and those of critical third parties. Cybersecurity incidents can cause the loss of critical or sensitive information, including personal information, and could give rise to legal liability and regulatory action under data protection and privacy laws.

In addition, we face certain risks as we seek to leverage artificial intelligence to optimize productivity and efficiency in various aspects of the organization. Flaws, biases, or malfunctions in these systems could lead to operational disruptions, data loss, or erroneous decision-making, impacting our operations, financial condition, and reputation. Ethical and legal challenges may arise, including biases or discrimination in generated outcomes, non-compliance with data protection regulations and laws specifically governing the use of artificial intelligence systems and tools, and lack of transparency. Furthermore, the deployment of artificial intelligence systems could expose us to increased cybersecurity threats, such as data breaches and unauthorized access. We also face competitive risks if we do not implement artificial intelligence or other machine learning technologies in a timely fashion.

***Our operations may be disrupted by the occurrence of a natural disaster, catastrophic event, or by other serious accidents occurring at our facilities.***

Most of our operations, including our research and development activities, are conducted in a limited number of facilities. If any of our major facilities were to experience a catastrophic loss due to an earthquake, flood, severe storms, fire or similar event, our operations would be seriously harmed. For example, our corporate headquarters, as well as additional leased space that we use for certain logistical and laboratory operations and manufacturing, are located in a flood zone along the Massachusetts coast. If we are unable to effectively implement our business continuity plans, we may experience delays in recovery of data and/or an inability to perform vital corporate functions, which could result in a significant disruption in our operations, large expenses to repair or replace the facility and/or the loss of critical data. Additionally, we use hazardous materials in some of our facilities, and any accident, injury or other loss related thereto could result in substantial liability. Our property or other relevant insurance may not be sufficient to cover all potential losses that may result from an interruption to our operations or damage resulting from these risks.

### **Strategic and Financial Risks**

***Our business development strategy, including strategic transactions and collaborations, may not be successful, and there may be delays or failures in realizing the anticipated benefits of these activities.***

As part of our business strategy, we seek to enter into strategic transactions to acquire, license, or collaborate with other entities, in each case that have potential to complement and advance our ongoing research, development, manufacturing, and commercialization efforts. Over the last several years we have engaged in a number of strategic transactions and collaborations, including our acquisition of Alpine and its lead asset, povetacicept, as well as several smaller transactions and collaboration arrangements. See also *Item 1., Business – Strategic Transactions* of this Annual Report on Form 10-K. Our future transactions and collaborations may be similar to prior transactions, may be structured differently from prior transactions, or may involve larger transactions or later-stage assets. We face significant competition for potential strategic transactions and collaborations from a variety of other companies, some of which have significantly more financial resources and experience in business development activities. We may not complete future transactions in a timely manner, or at all, including due to the possibility that a governmental entity or regulatory body may delay or refuse to grant approval for the consummation of the transaction.

We may not realize the anticipated benefits of our completed or future strategic transactions. The product candidates or products contemplated by those transactions may be delayed or terminated at any point during research or clinical development. Even if a product is approved, we may not be able to successfully commercialize it. As a result, we may fail to generate expected revenue growth or income contribution within the anticipated timeframe or at all. We also face risks that we:

- may not effectively integrate acquired assets or businesses into our ongoing business;
- may incur additional expenses or fail to achieve anticipated cost savings related to the strategic transactions;
- may incur impairment charges related to assets acquired in any such transactions; or
- may acquire unanticipated liabilities.

In addition, future strategic transactions could result in potentially dilutive issuances of equity securities or the incurrence of debt.

We continue to collaborate with outside partners on research, development, manufacturing, and/or commercialization activities with respect to product candidates and products. We face the same research, development, manufacturing, and commercialization risks with respect to product candidates and products that are subject to collaborations as with product candidates and products that we have developed ourselves. We face additional risks in connection with our current and future collaborative arrangements, including with respect to the performance of the collaborator and their compliance with contractual obligations.

***Our effective tax rate fluctuates, and changes in tax laws, regulations and treaties, unfavorable resolution to the tax positions we have taken, and exposure to additional income tax liabilities could have a material impact on our future taxable income.***

Our effective tax rate is derived from a combination of applicable tax rates in the various places that we operate globally. Our effective tax rate may be different than experienced in the past due to numerous factors, including:

- changes in the mix of our profitability from country to country;
- tax authority examinations/audits of our tax filings;
- adjustments to the value of our uncertain tax positions;
- changes in accounting for income taxes; and
- changes in tax laws or modifications of treaties in various jurisdictions.

Any of these factors could cause us to experience an effective tax rate that is significantly different from previous periods or our current expectations. For example, actions taken with respect to tax-related matters by associations such as the Organisation for Economic Co-operation and Development and the European Commission could influence tax laws in jurisdictions in which we operate, such as the enactments by both E.U. and non-E.U. member countries of a global minimum tax. We are subject to ongoing tax audits in various jurisdictions, and local tax authorities may disagree with certain positions we have taken and assess additional taxes. We regularly assess the probable outcomes of these audits to determine the appropriateness of our tax provision, and we have established contingency reserves for material tax exposures. However, there can be no assurance that we will accurately predict the outcomes of these disputes or other tax audits or that issues raised by tax authorities will be resolved at a financial cost that does not exceed our related reserves and the actual outcomes of these disputes and other tax audits could have a material impact on our results of operations or financial condition.

***Changes in foreign currency rates, interest rate risks, the value of our investment portfolio, and inflation affect our results of operations and financial condition.***

Fluctuations in currency exchange rates and interest rates, changes in the value of our investment portfolio, and inflation have affected and will continue to affect our cash flows, results of operations, and financial condition. The exchange rates among our reporting currency, the U.S. dollar, and the currencies in which we do business are volatile and our efforts to mitigate against these risks may not be successful. We invest our available cash in a range of investments, including investments in cash equivalents and debt securities, and fluctuations in interest rates, among other factors, could materially negatively affect the value of this investment portfolio. In addition, systemic economic downturns, as well as inflationary pressures, such as those observed in recent periods, may adversely impact our business and financial results. See also *Item 7A., Quantitative and Qualitative Disclosures About Market Risk* of this Annual Report on Form 10-K.

***Future indebtedness could materially and adversely affect our financial condition, and the terms of our credit agreements impose restrictions on our business.***

If we borrow under our current credit agreement or any future credit agreements, or otherwise issue or incur additional debt, such indebtedness could have important consequences to our business. The credit agreement requires that we comply with certain financial covenants, including a consolidated leverage ratio covenant and negative covenants, restricting or limiting our ability and the ability of our subsidiaries to, among other things, incur additional indebtedness, grant liens, engage in certain investment, acquisition and disposition transactions, and enter into transactions with affiliates. As a result, we may be restricted from engaging in business activities that may otherwise improve our business. Failure to comply with the covenants could result in an event of default that could trigger acceleration of our indebtedness. If we incur additional indebtedness, the risks related to our business and our ability to service or repay our indebtedness would increase.

***There can be no assurance that we will repurchase shares of common stock or that we will repurchase shares at favorable prices.***

In May 2025, our Board of Directors approved a share repurchase program pursuant to which we are authorized to repurchase up to \$4.0 billion of our common stock from time to time through open market or privately negotiated transactions, of which \$618.5 million has been repurchased as of December 31, 2025. Our stock repurchases will depend

upon, among other factors, market conditions, our cash balances and potential future capital requirements, results of operations, financial condition, and other factors that we may deem relevant. We can provide no assurance that we will repurchase stock at favorable prices, if at all.

### **General Risk Factors**

#### ***Our stock price is volatile.***

Our stock price is subject to significant fluctuations. From January 1, 2025 to December 31, 2025, our common stock traded between \$362.50 and \$519.68 per share. Our future stock price could be significantly and adversely affected by:

- announcements or investor analyst commentary regarding the clinical development of our product candidates as new information, including efficacy and safety information becomes available;
- our financial guidance and/or financial results, including quarterly and annual fluctuations resulting from factors such as the timing and amount of our revenues and expenses; and
- other factors including the risks described in these “*Risk Factors.*”

Fluctuations in our stock price can result in substantial losses for shareholders. Following periods of volatility in the market price of a company’s securities, shareholder derivative lawsuits and securities class action litigation are common. Such litigation, if instituted against us or our officers and directors, could result in substantial costs and other harm to our business.

#### ***If we fail to attract and retain skilled employees, our business could be materially harmed.***

We must attract and retain highly qualified and trained scientists, as well as employees with experience in the development, manufacture, and commercialization of medicines, including biologic and cell and genetic therapies. We face intense competition for such talent from our competitors, other companies, academic institutions, and other organizations throughout our industry, especially with respect to employees with expertise in cell or genetic therapies. Our compensation program, including equity awards, may not be sufficient to retain employees, especially if our stock price declines or other employers offer more attractive opportunities. Our ability to commercialize our products and achieve our research and development objectives depends on our ability to respond effectively to these demands. If we are unable to hire and retain qualified personnel, our ability to advance our pipeline, commercialize our products, and achieve our business objectives could be materially adversely affected.

#### ***The use of social media platforms presents risks and challenges.***

Social media is increasingly used by patients, advocacy groups, and other third parties to discuss our products and product candidates. Social media posts may include statements about efficacy or adverse events that could create reporting obligations or regulatory scrutiny. Our employees’ use of social media also presents risks, including potential noncompliance with legal or regulatory requirements, inappropriate disclosure of confidential information or personal information, and loss of intellectual property. In addition, misinformation, negative sentiment, or impersonation of our business on social media could cause reputational damage or otherwise harm our business. Failure to appropriately manage these risks could result in regulatory actions, liability, or other adverse consequences.

#### ***We have adopted provisions in our articles of organization and by-laws and are subject to Massachusetts corporate laws that may frustrate any attempt to remove or replace members of our board or to effectuate certain types of business combinations involving us.***

Provisions of our articles of organization, by-laws and Massachusetts state laws may frustrate any attempt to remove or replace members of our current Board of Directors and may discourage certain types of business combinations involving us. Our by-laws allow the Board of Directors to adjourn any meetings of shareholders prior to the time the meeting has been convened. We may issue shares of any class or series of preferred stock in the future without shareholder approval and upon such terms as our Board of Directors may determine. The rights of the holders of common stock will be subject to, and may be adversely affected by, the rights of the holders of any class or series of preferred stock that may be issued in the future. Massachusetts state law also prohibits us from engaging in specified business combinations with an interested stockholder, subject to certain exceptions, unless the combination is approved or consummated in a prescribed manner, and places

restrictions on voting by any shareholder who acquires 20% or more of the aggregate shareholder voting power without approval by non-interested shareholders. As a result, shareholders or other parties may find it difficult to remove or replace our directors or to effectuate certain types of business combinations involving us.

## **SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This Annual Report on Form 10-K, including the descriptions of our Business set forth in Part I, Item 1, our Risk Factors set forth in Part I, Item 1A, and our Management's Discussion and Analysis of Financial Condition and Results of Operations set forth in Part II, Item 7, contains forward-looking statements. Forward-looking statements are not purely historical and may be accompanied by words such as "anticipates," "may," "forecasts," "expects," "intends," "plans," "potentially," "believes," "seeks," "estimates," and other words and terms of similar meaning. Such statements may relate to:

- our expectations regarding the amount of, timing of, and trends with respect to our financial performance, including revenues, costs and expenses, and other gains and losses;
- our expectations regarding clinical trials, including expectations for patient enrollment, development timelines, the expected timing of data from our ongoing and planned clinical trials, and regulatory authority filings and other submissions for our therapies;
- our beliefs, expectations, and plans with respect to the commercial launches of CASGEVY for the treatment of SCD and TDT, ALYFTREK for the treatment of CF, and JOURNAVX for the treatment of moderate-to-severe acute pain, and the anticipated launch of povetacicept for the treatment of IgAN;
- our ability to maintain and obtain adequate reimbursement for our products and product candidates, our ability to launch, commercialize and market our products or any of our other therapies for which we obtain regulatory approval, and our ability to obtain label expansions for existing therapies;
- our expectations regarding our ability to continue to grow our CF business by increasing the number of people with CF eligible and able to receive our medicines and providing improved treatment options for people who are already eligible for one of our medicines, and our beliefs that the majority of people with CF will transition to ALYFTREK over time;
- our beliefs regarding the support provided by clinical trials and preclinical and nonclinical studies of our therapies for further investigation, clinical trials or potential use as a treatment, including with respect to povetacicept as a pipeline-in-a-product and as a potential best-in-class approach for the treatment of IgAN, pMN, and gMG;
- the data that will be generated by ongoing and planned clinical trials and the ability to use that data to advance compounds, continue development, support regulatory filings, or accelerate regulatory approval, including our plans to complete the full submission for potential accelerated approval of povetacicept in IgAN in the first half of 2026 and to share data from the interim analysis of the Phase 2/3 clinical trial of inaxaplin in AMKD in late 2026 or early 2027 and from the Phase 2 trial in people with AMKD in mid-2026;
- our beliefs that ALYFTREK will provide additional clinical benefits to eligible people with CF, regarding the durable efficacy and effectiveness of CASGEVY as one-time functional cure for people with SCD and TDT, and regarding the clinical benefits of JOURNAVX without the evidence of the several limitations of other available therapies;
- our plans to continue investing in our research and development programs, including anticipated timelines for our programs, and our strategy to develop our pipeline programs, alone or with third-party collaborators;
- our beliefs regarding the approximate patient populations for the disease areas on which we focus;
- the potential benefits and therapeutic scope of our acquisitions and collaborations, including our acquisition of Alpine and its lead asset, povetacicept, its potential to become a pipeline-in-a-product, and our expectations regarding our agreements with Zai, Ono and WuXi;
- our expectations regarding the lower royalty burden for ALYFTREK;
- our plans to expand, strengthen, and invest in our global supply chains and manufacturing infrastructure and capabilities, including for biologic and cell and gene therapies;
- the effects of import and export licensing requirements, tariffs, trade barriers, and other trade and travel restrictions;
- potential business development activities, including the identification of potential collaborative partners or acquisition targets;

- our ability to expand and protect our intellectual property portfolio and otherwise maintain exclusive rights to products;
- our expectations or beliefs regarding any legal proceedings in which we are involved, including any litigation, arbitration or other similar proceedings involving our products, product candidates or activities;
- the establishment, development and maintenance of collaborative relationships, including potential milestone payments or other obligations;
- potential fluctuations in foreign currency exchange rates and the effectiveness of our foreign currency management program;
- our expectations regarding the amount of cash to generated by operations, our cash balance and expected generation and interest income;
- our expectations regarding our provision for or benefit from income taxes and the utilization of our deferred tax assets;
- our ability to use our research programs to identify and develop new product candidates to address serious diseases and significant unmet medical needs;
- the effectiveness of our governance, plans and strategy with respect to managing cybersecurity risks and other threats to our information technology systems;
- our ability to effectively implement artificial intelligence systems and tools;
- our ability to attract and retain skilled personnel;
- our expectations involving governmental cost containment and other regulatory efforts;
- our expectations surrounding the competitive landscape facing our products and product candidates; and
- our liquidity and our expectations regarding the possibility of raising additional capital.

Forward-looking statements are subject to certain risks, uncertainties, or other factors that are difficult to predict and could cause actual events or results to differ materially from those indicated in any such statements. These risks, uncertainties, and other factors include, but are not limited to, those described in our Risk Factors, set forth in Part I, Item 1A, and elsewhere in this report and those described from time to time in our future reports filed with the Securities and Exchange Commission.

Any such forward-looking statements are made on the basis of our views and assumptions as of the date of the filing and are not estimates of future performance. Except as required by law, we undertake no obligation to publicly update any forward-looking statements. The reader is cautioned not to place undue reliance on any such statements.

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

We did not receive any written comments from the Securities and Exchange Commission prior to the date 180 days before the end of the fiscal year ended December 31, 2025 regarding our filings under the Securities Exchange Act of 1934, as amended, that have not been resolved.

#### **ITEM 1C. CYBERSECURITY**

##### ***Risk Management and Strategy***

We recognize the critical importance of developing, implementing, and maintaining robust cybersecurity measures to maintain the security, confidentiality, integrity, and availability of our business systems and confidential information, including personal information and intellectual property. Our cybersecurity program includes systems and processes for assessing, identifying and managing material risks from cybersecurity threats and include maintenance and monitoring of information security policies aligned with global regulatory controls and aligned with National Institute of Standards and Technology Cybersecurity Framework and System and Organization Controls 2. The program includes user and employee awareness of cyber policies and practices; information systems configuration management; third-party risk management systems; identity and information asset protection; infrastructure security systems; and cyber threat operations with continuous monitoring and threat hunting. This program also includes processes to oversee and identify material risks from cybersecurity threats associated with our use of third-party service providers. We engage a range of third-party experts in connection with various development, implementation, and maintenance activities related to our cybersecurity program, including audit and compliance, threat hunting, monitoring, and end-user support.

Our cybersecurity program is integrated into our overall risk management systems, including our annual enterprise risk management program, internal audit program, business continuity and crisis management programs, third-party risk management program, insurance risk management program, and employee compliance programs. As part of our overall risk management program, we maintain a global insurance portfolio with comprehensive cyber coverage. Our Chief Information Security Officer (“CISO”) and the Information Security function advises, consults with, or provides input to each of these programs to ensure that material risks from cybersecurity threats are appropriately assessed, identified, and managed.

As of the date of this report, there have been no cybersecurity threats that have materially affected or are reasonably likely to materially affect our business, operations, or financial condition. Similar to other companies, we have experienced cybersecurity incidents, including temporary service interruptions of third-party suppliers. As of the date of this report, however, known cybersecurity incidents, individually or in aggregate, have not had a material impact on our company. Over the last three years, net expenses incurred from any information security breaches, including any penalties and settlements, are not material relative to our total revenue. For additional discussion on cybersecurity risks we face, see *Item 1.A, Risk Factors – “A breakdown or breach of our information technology systems, or unauthorized access to confidential information could adversely affect our business.”* of this Annual Report on Form 10-K.

### *Governance*

While our board of directors has oversight responsibility for risk management generally, the Audit and Finance Committee (“Audit Committee”) is specifically responsible for overseeing our cybersecurity risk management program to ensure that cybersecurity risks are identified, assessed, managed, and monitored. Our CISO provides quarterly updates to the Audit Committee in this regard, and covers the state of our cybersecurity program, supported by key performance indicators across the range of cybersecurity functions related to risk management and governance, identity and information asset protection, core security and endpoint security, and cyber threat operations. These updates include descriptions of cybersecurity incidents of interest, including those associated with our third-party service providers; the board will be informed promptly of material risks from cybersecurity threats.

We strive to create a culture of cybersecurity resilience and awareness and believe that cybersecurity is the responsibility of every employee and contractor. At the same time, primary responsibility for assessing, monitoring, and managing our cybersecurity risks lies with our CISO. Our CISO has more than 35 years of experience in security and information systems and spent 25 years with Raytheon Technologies, most recently as Chief Technology Officer of Cybersecurity, Special Missions, Training & Services. Our CISO supported the U.S. President's National Security Telecommunications Advisory Committee for more than 20 years, is a member of the Massachusetts Cybersecurity Strategy Council, and previously served as Chair of the Kogod Cybersecurity Governance Center at American University. He also served on the Rhode Island Homeland Security Advisory Board and was a member of various commercial cyber product councils.

Our CISO oversees a team of skilled cybersecurity professionals who have Certified Information Systems Security Professional credentials, Global Information Assurance Certification from the SANS Institute, and other security and network certifications. The cybersecurity team monitors and evaluates our cybersecurity posture and performance on an ongoing basis, including through regular vulnerability scans, penetration tests, and threat intelligence feeds. The cybersecurity team uses various tools and methodologies to manage cybersecurity risk that are tested on a regular cadence, and assesses and evaluates cybersecurity incidents, escalating certain cybersecurity incidents to the CISO according to protocol. The CISO is continually informed regarding the performance of the cybersecurity program, as well as the latest developments in cybersecurity, including potential threats and innovative risk management techniques aligned with industry standards. The CISO reports to our Chief Digital and Information Officer, who is a Senior Vice President of the Company and reports directly to our Chief Operating and Financial Officer (“COFO”). Our COFO is an Executive Vice President and an executive officer of the Company, and reports directly to our CEO.

## **ITEM 2. PROPERTIES**

### ***Corporate Headquarters***

We lease approximately 1.1 million square feet of office and laboratory space at our corporate headquarters in Boston, Massachusetts in two buildings pursuant to two leases that we entered into in May 2011 and amended in August 2024 to, among other terms, extend the lease termination dates from December 2028 to June 2044. We have the option to extend the term of the leases for up to two additional ten-year periods.

### ***Additional United States and Worldwide Locations***

In addition to our corporate headquarters, we lease an aggregate of approximately 865,000 square feet of space globally. This space includes logistical, laboratory, commercial and manufacturing operations, as well as laboratory and office space to support our research and development organizations. We also own approximately 213,000 square feet at our continuous manufacturing facility in Massachusetts. Additionally, we are constructing the second building of our Leiden Campus in Massachusetts (“Leiden II”), which will include approximately 348,000 square feet of office and laboratory space. We expect Leiden II to be operational in late 2026.

### **ITEM 3. LEGAL PROCEEDINGS**

Other than as described in Note P, “Commitments and Contingencies,” to our consolidated financial statements, we are not currently subject to any material legal proceedings.

### **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**

*Market Information*

Our common stock is traded on The Nasdaq Global Select Market under the symbol “VRTX.”

*Shareholders*

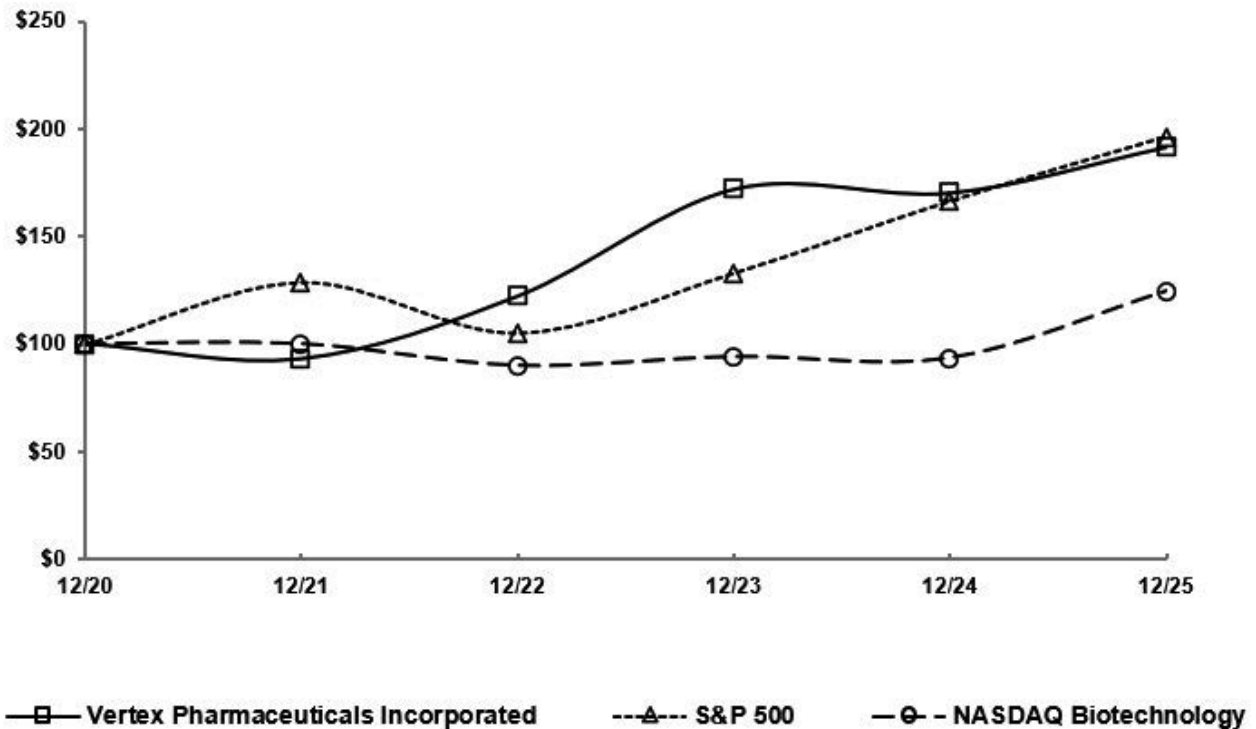
As of February 6, 2026, there were 94 holders of record of our common stock.

*Performance Graph*

Our performance graph includes the NASDAQ Biotechnology Index, which we believe is a comparable index consisting of companies with similar industry classifications, and which we plan to use in our future performance graphs.

**COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN\***

Among Vertex Pharmaceuticals Incorporated, the S&P 500 Index  
and the NASDAQ Biotechnology Index



\*\$100 invested on 12/31/20 in stock or index, including reinvestment of dividends. Fiscal year ending December 31.

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## Dividends

We have never paid any cash dividends on our common stock, and we do not anticipate paying any in the foreseeable future.

## Issuer Repurchases of Equity Securities

In May 2025, our Board of Directors approved a share repurchase program (our “2025 Share Repurchase Program”), pursuant to which we are authorized to repurchase up to \$4.0 billion of our common stock. The 2025 Share Repurchase Program does not have an expiration date and can be discontinued at any time.

The table set forth below shows repurchases of securities by us during the three months ended December 31, 2025 under our 2025 Share Repurchase Program.

<b>Period</b>	<b>Total Number of Shares Purchased</b>	<b>Average Price Paid per Share</b>	<b>Total Number of Shares Purchased as Part of Publicly Announced Programs (1)</b>	<b>Approximate Dollar Value of Shares that May Yet Be Purchased Under the Programs (1)</b>
Oct. 1, 2025 to Oct. 31, 2025	256,788	\$ 409.11	256,788	\$ 3,381,462,793
Nov. 1, 2025 to Nov. 30, 2025	—	\$ —	—	\$ 3,381,462,793
Dec. 1, 2025 to Dec. 31, 2025	—	\$ —	—	\$ 3,381,462,793
Total	<u>256,788</u>	\$ 409.11	<u>256,788</u>	\$ 3,381,462,793

(1) Under our 2025 Share Repurchase Program, we are authorized to purchase shares from time to time through open market or privately negotiated transactions. Such purchases may be made pursuant to Rule 10b5-1 plans or other means as determined by our management and in accordance with the requirements of the Securities and Exchange Commission.

## ITEM 6. [RESERVED]

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

*Our discussion and analysis of our financial condition and results of operations for 2025 as compared to 2024 are discussed below. For a discussion of our financial condition and results of operations for 2024 as compared to 2023, please refer to Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our 2024 Annual Report on Form 10-K, except as set forth below.*

### OVERVIEW

We are a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases, with a focus on specialty markets. We have approved medicines for cystic fibrosis (“CF”), sickle cell disease (“SCD”), transfusion dependent beta thalassemia (“TDT”), and acute pain, and we continue to serially innovate and advance next-generation clinical and research programs in these areas. Our mid- and late-stage clinical pipeline includes programs across a range of modalities in additional serious diseases, including IgA nephropathy, APOL1-mediated kidney disease, neuropathic pain, type 1 diabetes, primary membranous nephropathy, autosomal dominant polycystic kidney disease, and myotonic dystrophy type 1.

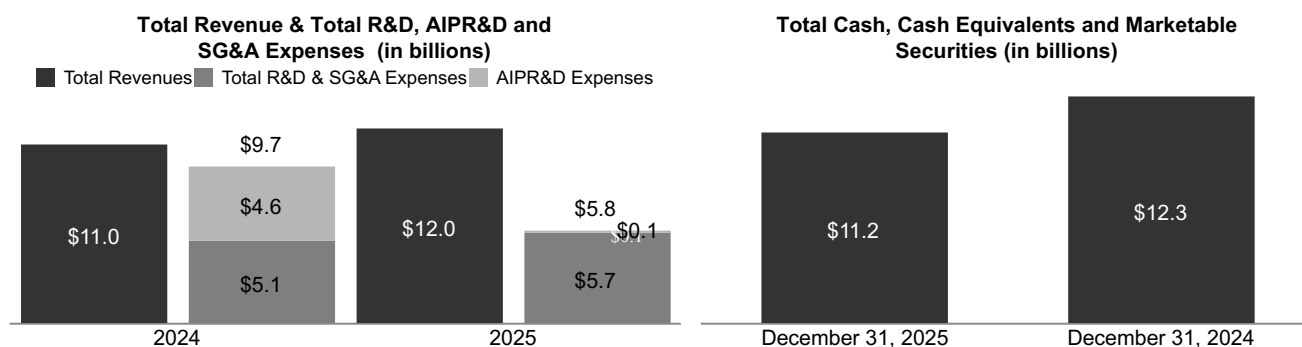
Collectively, our five CF medicines, led by TRIKAFTA/KAFTRIO, are being used to treat nearly three quarters of the people with CF in the U.S., Europe, Australia, and Canada. ALYFTREK, our newest CF medicine, is approved in the United States (the “U.S.”), the United Kingdom (the “U.K.”), the European Union (the “E.U.”), Canada, New Zealand, Switzerland, Australia and Israel.

CASGEVY, our ex-vivo, non-viral CRISPR/Cas9 gene-edited cell therapy, is approved in the U.S., the E.U., the U.K., the Kingdom of Saudi Arabia (“Saudi Arabia”), the Kingdom of Bahrain (“Bahrain”), Qatar, the United Arab Emirates (the “UAE”), Kuwait, Switzerland and Canada for the treatment of people 12 years of age and older with SCD or TDT.

JOURNAVX, our selective non-opioid NaV1.8 pain signal inhibitor, is approved in the U.S. for the treatment of people with moderate-to-severe acute pain. We are continuing our commercial launch of JOURNAVX for eligible adults.

## Financial Highlights

<i>Total Revenues</i>	In 2025, our total revenues increased to \$12.0 billion as compared to \$11.0 billion in 2024, primarily due to continued strong demand for TRIKAFTA/KAFTRIO as well as contributions from our launches of ALYFTREK, JOURNAVX and CASGEVY.
<i>Cost of Sales</i>	Our cost of sales as a percentage of our net product revenues decreased from 13.9% in 2024 to 13.8% in 2025 as a result of a lower overall royalty rate for our CF medicines, partially offset by changes in our product mix, and investments in network expansion and manufacturing process improvements.
<i>Total R&amp;D and SG&amp;A Expenses</i>	Our total research and development (“R&D”) and selling, general and administrative (“SG&A”) expenses increased to \$5.7 billion in 2025 as compared to \$5.1 billion in 2024, primarily due to increased investment to commercialize our new products and to advance our R&D pipeline.
<i>AIPR&amp;D Expenses</i>	In 2025, our acquired in-process research and development expenses (“AIPR&D”) of \$133.0 million included various upfront and milestone payments related to our collaboration and in-licensing arrangements. In 2024, AIPR&D included \$4.4 billion resulting from our acquisition of Alpine Immune Sciences, Inc. (“Alpine”), which was accounted for as an asset acquisition.
<i>Cash</i>	Our total cash, cash equivalents and marketable securities increased to \$12.3 billion as of December 31, 2025 as compared to \$11.2 billion as of December 31, 2024 primarily due to cash flows provided by our operating activities partially offset by repurchases of our common stock.



Note: Charts above may not add due to rounding.

## **Business Updates**

### *Marketed Products*

#### Cystic Fibrosis

We expect that the number of people with CF taking our medicines will continue to grow through new approvals and reimbursement agreements, treatment of younger patients, increased survival and expansion into additional geographies.

- ALYFTREK is reimbursed for eligible people with CF in the U.S., England, Ireland, Germany, Denmark, Northern Ireland, Norway, Wales, Italy, Australia, New Zealand and Luxembourg. We are working to secure access for eligible patients in additional countries.

#### Sickle Cell Disease and Beta Thalassemia

- In 2025, we recorded \$115.8 million of CASGEVY product revenues. This reflects 64 patients receiving infusions of CASGEVY in 2025, including 30 people infused in the fourth quarter. Globally, in 2025, 147 people with SCD or TDT had their first cell collection for CASGEVY.
- As of the end of 2025, approximately 90 percent of people with SCD or TDT in the U.S. have reimbursed access to CASGEVY, which is also reimbursed in the U.K., Italy, Austria, Denmark, Luxembourg, Saudi Arabia, the UAE, Bahrain, and Kuwait. In January 2026, we secured reimbursed access to CASGEVY for eligible people with SCD in Scotland, consistent with the reimbursement agreement reached in 2025 for people with TDT.
- We expect to begin global regulatory submissions for approvals for CASGEVY in children 5 to 11 years of age, in the first half of 2026. The FDA awarded Vertex with a Commissioner's National Priority Voucher for this pediatric submission, indicating an accelerated timeline for review once the submission is complete.

#### Acute Pain

- Since pharmacy availability in March 2025 through year-end 2025, more than 550,000 prescriptions for JOURNAVX were written and filled across the hospital and retail settings in different acute pain conditions, consistent with JOURNAVX's broad label.
- We have secured access for JOURNAVX with all three national pharmacy benefit managers, and, as of January 2026, over 200 million individuals across commercial and government payers have coverage, representing two-thirds of U.S. covered lives. In addition, 21 states provide coverage via Medicaid.
- More than 100 of the targeted 150 healthcare systems and more than 950 individual hospitals of the 2,000 targeted institutions have added JOURNAVX to formularies, protocols or order sets.

### *Select R&D Pipeline Programs*

We continue to advance a diversified pipeline of potentially transformative medicines for serious diseases utilizing a range of modalities. Recent and anticipated progress in activities supporting these efforts is included below:

#### Cystic Fibrosis

- We completed the global trial evaluating ALYFTREK in children 2 to 5 years of age. Following positive results from this clinical trial, we expect to submit for approval with global regulators in this age group in the first half of 2026. We also initiated a pivotal trial of ALYFTREK in children 1 year to less than 2 years of age.
- Following positive results from the clinical trial evaluating TRIKAFTA in children 1 year to less than 2 years of age, we expect to begin submissions for global regulatory approvals in this age group in the first half of 2026.

#### IgA Nephropathy

- We are developing povetacept, a dual inhibitor of B cell activating factor ("BAFF") and a proliferation-inducing ligand ("APRIL") cytokines, for multiple diseases. Povetacept represents a potentially best-in-class approach to control B cell activity in immunoglobulin A nephropathy ("IgAN").

- We completed enrollment in the Phase 3 clinical trial evaluating povetacept for IgAN and, in the fourth quarter of 2025, we initiated the rolling Biologics Licensing Application (“BLA”) filing for U.S. accelerated approval with submission of the first module. We expect to release interim analysis data in the first half of 2026 and we expect to complete the submission in the first half of 2026, if data from the interim analysis are supportive. We are using a priority review voucher to expedite the review of the povetacept BLA from ten months to six months.

#### APOL1-Mediated Kidney Disease

- Inaxaplin is our small molecule for the treatment of APOL1-mediated kidney disease (“AMKD”). We completed enrollment in the interim analysis cohort of the global Phase 2/3 pivotal clinical trial evaluating inaxaplin in people with primary AMKD (“AMPLITUDE”). We expect to conduct the pre-planned interim analysis once this cohort has been treated for 48 weeks and we expect to share data from the interim analysis in late 2026 or early 2027. We expect to complete full enrollment in AMPLITUDE in the second half of 2026.

#### Peripheral Neuropathic Pain

- We previously initiated the first Phase 3 clinical trial evaluating suzetrigine for the treatment of people with diabetic peripheral neuropathy (“DPN”), a common form of peripheral neuropathic pain, and have initiated a second Phase 3 clinical trial evaluating suzetrigine in DPN in the fourth quarter of 2025. We expect to complete enrollment in both Phase 3 clinical trials by the end of 2026.

#### Type 1 Diabetes

- Zimislecel is an allogeneic, stem cell-derived, fully differentiated, insulin-producing islet cell replacement therapy, using standard immunosuppression to protect the implanted cells. We have completed enrollment in the Phase 1/2/3 clinical trial of zimislecel in people with type 1 diabetes (“T1D”). We have temporarily postponed completion of dosing in this clinical trial, pending an internal manufacturing analysis.

#### Primary Membranous Nephropathy

- Povetacept represents a potentially best-in-class approach to control B cell activity in primary membranous nephropathy (“pMN”), another B cell-mediated disease. We are enrolling and dosing patients in the adaptive Phase 2/3 pivotal clinical trial of povetacept for the treatment of people with pMN. We expect to complete the Phase 2 portion of the clinical trial and to initiate the Phase 3 portion in mid-2026.

#### *External Innovation*

Recent investments in external innovation include:

- An exclusive global license agreement with WuXi Biologics to develop and commercialize a trispecific T cell engager for B cell-mediated autoimmune diseases, which is currently in preclinical development.

### ***Our Business Environment***

In 2025, our net product revenues were primarily from the sale of our medicines for the treatment of CF. Our CF strategy involves continuing to develop and obtain approval and reimbursement for treatment regimens that will provide benefits to all people with CF and increasing the number of people with CF eligible and able to receive our medicines. Outside of CF, we continue to advance the commercialization of CASGEVY for the treatment of SCD and TDT, and JOURNAVX for the treatment of acute pain. In addition, we are advancing our pipeline of product candidates for the treatment of serious diseases outside of CF, SCD, TDT and acute pain.

Our strategy is to combine transformative advances in the understanding of causal human biology and the science of therapeutics to discover and develop innovative medicines. This approach includes advancing multiple compounds or therapies from each program, spanning multiple modalities, into early clinical trials to obtain patient data that can inform selection of the most promising therapies for later-stage development, as well as to inform discovery and development efforts. We aim to serially innovate in our disease areas of interest and follow our first-in-class therapies with potential best-in-class candidates to provide durable clinical and commercial success.

In pursuit of new product candidates and therapies in specialty markets, we invest in research and development. We believe that pursuing research in diverse areas allows us to balance the risks inherent in product development and may provide product candidates that will form our pipeline in future years. To supplement our internal research programs, we acquire technologies and programs and collaborate with biopharmaceutical and technology companies, leading academic research institutions, government laboratories, foundations and other organizations, as needed, to advance research in our areas of therapeutic interest and to access technologies needed to execute on our strategy.

Discovery and development of a new pharmaceutical or biological product is a difficult and lengthy process that requires significant financial resources along with extensive technical and regulatory expertise. Across the industry, most potential drug or biological products never progress into development, and most products that advance into development never receive marketing approval. Our investments in product candidates are subject to considerable risks. We closely monitor our research and development activities, and frequently evaluate our pipeline programs in light of new data and scientific, business and commercial insights, with the objective of balancing risk and potential. This process can result in rapid changes in focus and priorities as new information becomes available and as we gain additional understanding of our ongoing programs and potential new programs, as well as those of our competitors. In addition, our product candidates must satisfy rigorous standards of safety and efficacy before they can be approved for sale by regulatory authorities. Our analysis of data obtained from nonclinical and clinical activities is subject to confirmation and interpretation by regulatory authorities, which could delay, limit or prevent regulatory approval.

Our business also requires ensuring appropriate manufacturing and supply of our products. As we advance our product candidates through clinical development toward commercialization and market and sell our approved products, we build and maintain our supply chain and quality assurance resources. We rely on a global network of third parties, including some in China, and our internal capabilities to manufacture and distribute our products for commercial sale and post-approval clinical trials and to manufacture and distribute our product candidates for clinical trials. In addition to establishing supply chains for each newly approved product, we adapt our supply chain for existing products to include additional formulations or to increase scale of production for existing products as needed. The processes for biological and cell and genetic therapies can be more complex than those required for small molecule drugs and require additional investments in different systems, equipment, facilities and expertise. We are focused on ensuring the stability of the supply chains for our current products, as well as for our pipeline programs.

Sales of our products depend, to a large degree, on the extent to which our products are reimbursed by third-party payors, such as government health programs, commercial insurance and managed health care organizations. Reimbursement for our products, including our potential pipeline therapies, cannot be assured and may take significant periods of time to obtain. We dedicate substantial management and other resources to obtain and maintain appropriate levels of reimbursement for our products from third-party payors, including governmental organizations in the U.S. and ex-U.S. markets. In the U.S., we work with government and commercial payors to obtain and maintain appropriate levels of reimbursement for our medicines. In ex-U.S. markets, we seek government reimbursement for our medicines on a country-by-country or region-by-region, as required. This is necessary for each new medicine, as well as for label expansions for our current medicines. We expect to continue to focus significant resources to expand and maintain reimbursement for our CF medicines, CASGEVY, JOURNAVX, and, ultimately, our pipeline therapies, in U.S. and ex-U.S. markets.

## ***Strategic Transactions***

### *Acquisitions*

As part of our business strategy, we seek to acquire technologies, products, product candidates and other businesses that are aligned with our corporate and research and development strategies and complement and advance our ongoing research and development efforts. We have acquired multiple biotechnology companies over the last several years and expect to continue to identify and evaluate such opportunities. The accounting for these acquisitions can vary significantly based on whether we conclude the transactions represent business combinations or asset acquisitions. In 2024, we acquired Alpine and its lead molecule, povetacicept, for approximately \$5.0 billion. Povetacicept has shown potential to treat multiple diseases or conditions and become a pipeline-in-a-product. We accounted for the Alpine transaction as an asset acquisition because povetacicept represented substantially all of the fair value of the gross assets that we acquired. As a result, \$4.4 billion of the fair value attributed to povetacicept was expensed as AIPR&D in 2024. In 2019 and 2022, we acquired Semma Therapeutics, Inc. (“Semma”) and ViaCyte, Inc. (“ViaCyte”), respectively, pursuant to which we established and accelerated the development of our T1D program. We accounted for each of these acquisitions as a business combination.

Please refer to our critical accounting policies, “Acquisitions,” for further information regarding the significant judgments and estimates related to our acquisitions.

#### *Collaboration and In-Licensing Arrangements*

We enter into arrangements with third parties, including collaboration and licensing arrangements, for the development, manufacture and commercialization of products, product candidates, and other technologies that have the potential to complement our ongoing research and development efforts.

Over the last several years, we entered into collaboration agreements with a number of companies, including CRISPR Therapeutics AG (“CRISPR”), Entrada Therapeutics, Inc. (“Entrada”), and Moderna, Inc.

Generally, when we in-license a technology or product candidate, we make upfront payments to the collaborator, assume the costs of the program and/or agree to make contingent payments, which could consist of milestone, royalty and option payments. Most of these collaboration payments are expensed as AIPR&D, including, a \$75.0 million milestone paid to Entrada in 2024, and, in 2023, total payments of \$242.6 million to Entrada and total upfront and milestone payments of \$170.0 million to CRISPR related to T1D. These payments were expensed to AIPR&D because they were primarily attributable to acquired in-process research and development for which there was no alternative future use. However, depending on many factors, including the structure of the collaboration, the stage of development of the acquired technology, the significance of the in-licensed product candidate to the collaborator’s operations and the other activities in which our collaborators are engaged, the accounting for these transactions can vary significantly. We expect to continue to identify and evaluate collaboration and licensing opportunities that may be similar to or different from the collaborations and licenses that we have engaged in previously.

#### Joint Development and Commercialization Agreement with CRISPR

In 2017, we entered into a joint development and commercialization agreement with CRISPR (the “CRISPR JDCA”), which we amended and restated in 2021.

Pursuant to the CRISPR JDCA, we lead global development, manufacturing and commercialization of CASGEVY, with support from CRISPR. We also conduct all research, development, manufacturing and commercialization activities relating to other product candidates and products under the CRISPR JDCA throughout the world subject to CRISPR’s reserved right to conduct certain activities.

CASGEVY was approved by the FDA in December 2023 for the treatment of SCD. In connection with this approval, we made a \$200.0 million milestone payment to CRISPR in January 2024. We are recording intangible asset amortization expense to “Cost of sales” related to this intangible asset. Subsequent to receiving marketing approval for CASGEVY, we continue to lead the research and development activities under the CRISPR JDCA, subject to CRISPR’s reserved right to conduct certain activities. We are reimbursed by CRISPR for its 40% share of these research and development activities, subject to certain adjustments, and we record this reimbursement from CRISPR as a credit within “Research and development expenses.” We also share with CRISPR 40% of the net commercial profits or losses incurred with respect to CASGEVY, subject to certain adjustments, which is recorded to “Cost of sales.” The net commercial profits or losses equal the sum of the product revenues, cost of sales and selling, general and administrative expenses that we have recognized related to the CRISPR JDCA.

Prior to receiving marketing approval from the FDA for CASGEVY in December 2023, we accounted for the CRISPR JDCA as a cost-sharing arrangement, with costs incurred related to CASGEVY allocated 60% to us and 40% to CRISPR, subject to certain adjustments. In 2023, we recognized net reimbursements from CRISPR as credits to “Research and development expenses” and to “Selling, general and administrative expenses,” related to CRISPR’s share of the CRISPR JDCA’s operating expenses.

#### *Acquired In-Process Research and Development Expenses*

In 2025 and 2024, our AIPR&D included \$133.0 million and \$4.6 billion, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions, including the asset acquisitions, collaborations, and licenses of third-party technologies described above. Please refer to Note B, “Collaboration, License and Other Arrangements,” for further information regarding our asset acquisitions, collaborations, and in-license agreements.

### *Out-licensing Arrangements*

We also have out-licensed certain development programs to collaborators who are leading the development or commercialization of these programs, either globally or within certain geographic regions.

In 2025, we entered into agreements with Zai Lab Limited (“Zai”) and Ono Pharmaceuticals, Co Ltd (“Ono”) respectively, for the development and commercialization of povetacicept in various Asian markets. Zai licensed povetacicept for mainland China, Hong Kong SAR, Macau SAR, Taiwan region, and Singapore, while Ono licensed povetacicept for Japan and South Korea. Zai and Ono will help advance povetacicept clinical trials, and will be responsible for obtaining marketing authorizations and commercialization activities, if povetacicept becomes an approved product, in their licensed territories. We are eligible to receive certain future milestone payments and tiered royalties on future net sales of povetacicept in these regions.

## RESULTS OF OPERATIONS

### Total Revenues

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	(in millions, except percentages)				
TRIKAFTA/KAFTRIO	\$ 10,312.7	1%	\$ 10,238.6	14%	\$ 8,944.7
ALYFTREK	837.8	**	—	**	—
Other product revenues	820.1	5%	781.5	(15)%	924.5
Product revenues, net	11,970.6	9%	11,020.1	12%	9,869.2
Other revenues	30.7	**	—	**	—
Total revenues	<u>\$ 12,001.3</u>	9%	<u>\$ 11,020.1</u>	12%	<u>\$ 9,869.2</u>

\*\* Not meaningful

### Product Revenues, Net

In 2025, our net product revenues increased \$950.5 million, or 9%, as compared to 2024, primarily due to continued strong demand for TRIKAFTA/KAFTRIO as well as contributions from our launches of ALYFTREK, JOURNAVX and CASGEVY. In 2025, “Other product revenues” included \$115.8 million from CASGEVY and \$59.6 million from JOURNAVX. In 2024, “Other product revenues” included CASGEVY product revenues of \$10.0 million. Our remaining “Other product revenues” are related to KALYDECO, ORKAMBI, and SYMDEKO/SYMKEVI, our other CF products.

### Other Revenues

In 2025, other revenues were \$30.7 million, which included \$20.6 million and \$10.0 million related to upfront payments received from our agreements with Ono and Zai, respectively.

### Revenues by Geographic Location

Our total revenues from the U.S. and from ex-U.S. markets were as follows:

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	(in millions, except percentages)				
United States	\$ 7,548.6	13%	\$ 6,684.9	11%	\$ 6,040.4
ex-U.S.	4,452.7	3%	4,335.2	13%	3,828.8
Total revenues	<u>\$ 12,001.3</u>	9%	<u>\$ 11,020.1</u>	12%	<u>\$ 9,869.2</u>

Our U.S. total revenues increased 13% in 2025, as compared to 2024, due to continued strong patient demand, new patient initiations and higher realized net prices. Our ex-U.S. total revenues increased 3% in 2025, as compared to 2024, primarily due to solid CF performance across multiple geographies and increased CASGEVY product revenues, partially offset by a decline in product revenues in Russia, where we are continuing to experience a violation of our intellectual property rights.

In 2026, we expect our total revenues to increase due to continued growth of our CF product revenues, including from ALYFTREK globally, and increased contributions from CASGEVY and JOURNAVX.

## Operating Costs and Expenses

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	<b>(in millions, except percentages)</b>				
Cost of sales	\$ 1,651.3	8%	\$ 1,530.5	21%	\$ 1,262.2
Research and development expenses	3,909.5	8%	3,630.3	15%	3,162.9
Acquired in-process research and development expenses	133.0	**	4,628.4	**	527.1
Selling, general and administrative expenses	1,753.1	20%	1,464.3	29%	1,136.6
Intangible asset impairment charge	379.0	**	—	**	—
Change in fair value of contingent consideration	2.1	**	(0.5)	**	(51.6)
Total costs and expenses	<u>\$ 7,828.0</u>	(30)%	<u>\$ 11,253.0</u>	86%	<u>\$ 6,037.2</u>

\*\* Not meaningful

### Cost of Sales

Our cost of sales primarily consists of third-party royalties payable on net sales of our CF products as well as the cost of producing inventories. Pursuant to our agreement (the “CFF Agreement”) with the Cystic Fibrosis Foundation (the “CFF”), our tiered third-party royalties on sales of ALYFTREK, TRIKAFTA/KAFTRIO, SYMDEKO/SYMKEVI, KALYDECO, and ORKAMBI, calculated as a percentage of net sales, range from the single digits to the sub-teens, with lower royalties on sales of ALYFTREK and TRIKAFTA/KAFTRIO than for our other products. The royalty burden associated with TRIKAFTA/KAFTRIO is 9.33% and our position is that the royalty burden associated with ALYFTREK is 4%. On October 10, 2025, Royalty Pharma plc (“RP”), the third party to whom the CFF assigned its rights (and the CFF, which remains a party to the CFF Agreement), initiated a confidential arbitration alleging the royalty burden on ALYFTREK is approximately 8%. RP is seeking a declaratory judgment regarding the royalty burden on ALYFTREK as well as alleged unpaid royalties and other alleged damages available under the CFF Agreement or applicable law, costs, expenses, attorneys’ fees, and interest. We believe RP’s position is contrary to the plain terms of the CFF Agreement and intend to vigorously defend our position under the CFF Agreement.

Our cost of sales as a percentage of our net product revenues was 13.8% and 13.9% in 2025 and 2024, respectively, primarily due to ALYFTREK sales in 2025, which has the royalty burden lower than TRIKAFTA/KAFTRIO, partially offset by changes in product mix, and investments in network expansion and manufacturing process improvements.

In 2026, we expect our cost of sales as a percentage of our net product revenues to increase due to a higher proportion of products outside of CF, which currently have greater manufacturing costs relative to their net product revenue contributions, and continued investments in efficient manufacturing and delivery processes.

### Research and Development Expenses

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	<b>(in millions, except percentages)</b>				
Research expenses	\$ 827.9	3%	\$ 804.5	14%	\$ 705.6
Development expenses	3,081.6	9%	2,825.8	15%	2,457.3
Total research and development expenses	<u>\$ 3,909.5</u>	8%	<u>\$ 3,630.3</u>	15%	<u>\$ 3,162.9</u>

Over the past three years, we have incurred approximately \$10.7 billion in research and development expenses associated with product discovery and development. Our research and development expenses include internal and external costs incurred for research and development of our products and product candidates. We assign external costs of services provided to us by clinical research organizations and other outsourced research by individual program. Our internal costs include salary and benefits, stock-based compensation expense, laboratory supplies and other direct expenses and infrastructure costs, the majority of which are not assigned to individual products or product candidates.

### Research Expenses

	<u>2025</u>	<u>Change %</u>	<u>2024</u>	<u>Change %</u>	<u>2023</u>
	(in millions, except percentages)				
Research Expenses:					
Salary and benefits	\$ 203.9	(3)%	\$ 210.7	14%	\$ 184.1
Stock-based compensation expense	94.8	(15)%	112.1	21%	92.4
Outsourced services and other direct expenses	286.2	5%	271.4	15%	237.0
Infrastructure costs	243.0	16%	210.3	9%	192.1
Total research expenses	<u>\$ 827.9</u>	3%	<u>\$ 804.5</u>	14%	<u>\$ 705.6</u>

Our research expenses reflect investment in our pipeline and expansion of our cell and genetic therapy capabilities, which has increased our outsourced services and other direct expenses and infrastructure costs in 2025 as compared to 2024. Salary and benefits in 2024 included \$13.1 million associated with cash-settled unvested Alpine equity awards. Compared to 2024, our total research expenses in 2025 increased \$23.4 million, or 3%. We expect to continue to invest in our research programs with a focus on creating transformative medicines for serious diseases.

### Development Expenses

	<u>2025</u>	<u>Change %</u>	<u>2024</u>	<u>Change %</u>	<u>2023</u>
	(in millions, except percentages)				
Development Expenses:					
Salary and benefits	\$ 744.8	8%	\$ 686.7	16%	\$ 590.9
Stock-based compensation expense	320.6	2%	313.7	20%	262.5
Compensation expense for cash-settled unvested Alpine equity awards	—	**	151.9	**	—
Outsourced services and other direct expenses	1,493.5	21%	1,239.1	0%	1,238.7
Infrastructure costs	522.7	20%	434.4	19%	365.2
Total development expenses	<u>\$ 3,081.6</u>	9%	<u>\$ 2,825.8</u>	15%	<u>\$ 2,457.3</u>

\*\* Not meaningful

As we have advanced our pipeline of transformative medicines, we have invested in internal headcount and infrastructure to support multiple mid- and late-stage clinical development programs. These include our povetacept programs acquired from Alpine, pain and T1D programs, which together have increased our outsourced services and other direct expenses. In conjunction with our acquisition of Alpine, we incurred \$151.9 million associated with cash-settled unvested Alpine equity awards within development expenses in 2024. Compared to 2024, our total development expenses in 2025 increased by \$255.8 million, or 9%. In 2026, we expect our development expenses to continue to increase due to our advancing pipeline programs, including our T1D programs.

Our stock-based compensation expenses, including those recorded as research and development expenses, have historically fluctuated and are expected to continue to fluctuate from one period to another primarily due to changes in the probability of achieving milestones associated with our performance-based awards.

### Acquired In-Process Research and Development Expenses

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	(in millions, except percentages)				
Acquired in-process research and development expenses	\$ 133.0	**	\$ 4,628.4	**	\$ 527.1

\*\* Not meaningful

In 2025, AIPR&D included various upfront and milestone payments related to our collaboration and in-licensing arrangements. In 2024, AIPR&D included \$4.4 billion resulting from our acquisition of Alpine, which was accounted for as an asset acquisition, and various other upfront and milestone payments. Our AIPR&D has historically fluctuated, and is expected to continue to fluctuate, from one period to another due to upfront, contingent milestone, and other payments pursuant to our existing and future business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions.

### Selling, General and Administrative Expenses

	<u>2025</u>	<u>% Change</u>	<u>2024</u>	<u>% Change</u>	<u>2023</u>
	<b>(in millions, except percentages)</b>				
Selling, general and administrative expenses	\$ 1,753.1	20%	\$ 1,464.3	29%	\$ 1,136.6

Selling, general and administrative expenses increased by 20% in 2025 as compared to 2024, primarily due to increased commercial investment to support the launch of JOURNAVX. We expect our selling, general and administrative expenses to continue to increase in 2026 to as we expand the commercialization of JOURNAVX, prepare for our anticipated launch of povetacept for the treatment of IgAN, and further investments in infrastructure to scale our organization.

### Intangible Asset Impairment Charge

In the first quarter of 2025, based on results from a Phase 1/2 clinical trial evaluating our VX-264 clinical program in patients with T1D, we concluded that VX-264 will not be advancing further in clinical development. Based on this event, we performed an interim impairment test on the fair value of our VX-264 indefinite-lived in-process research and development asset that we acquired from Semma Therapeutics, Inc. As a result, we recorded a full intangible asset impairment charge of \$379.0 million associated with VX-264 in the first quarter of 2025.

### Non-Operating Income (Expense), Net

#### Interest Income

Interest income decreased from \$598.1 million in 2024 to \$490.9 million in 2025, primarily due to decreased market interest rates. Our future interest income is dependent on the amount of, and prevailing market interest rates on, our outstanding cash, cash equivalents and available-for-sale debt securities.

#### Other Income (Expense), Net

Other income (expense), net were expenses of \$7.7 million and \$86.1 million in 2025 and 2024, respectively. These amounts primarily related to net unrealized and realized losses resulting from changes in the fair value of certain of our strategic equity investments and net foreign currency exchange losses.

### Income Taxes

Our effective tax rate fluctuates from year to year due to the global nature of our operations. The factors that most significantly impact our effective tax rate include changes in tax laws, variability in the amount and allocation of our taxable earnings among multiple jurisdictions, the amount and characterization of our research and development expenses, the levels of certain deductions and credits, adjustments to the value of our uncertain tax positions, acquisitions and third-party collaboration and licensing transactions.

In July 2025, the U.S. enacted H.R.1, which includes significant provisions modifying the U.S. tax framework, including the ability for companies to immediately deduct research and development expenditures for 2025 and provisions for deducting previously capitalized amounts. H.R.1 does not have a material impact on our 2025 U.S. taxes, but we expect further guidance to be issued. We will review guidance when issued for impacts on future years and disclose any impacts if needed at that time. These legislative changes could have an impact on our future effective tax rates, tax liabilities, and cash taxes.

Our provision for income taxes was \$690.0 million in 2025 and \$784.1 million in 2024. In 2025, our 14.9% effective tax rate was lower than the U.S. statutory rate primarily due to research and development tax credits, increased utilization of foreign tax credits, and excess tax benefits related to stock-based compensation.

In 2024, our 315.5% effective tax rate was materially different than the U.S. statutory rate primarily due to the \$4.4 billion of non-deductible AIPR&D resulting from our acquisition of Alpine, which significantly lowered our pre-tax income. The non-deductible AIPR&D was partially offset by a benefit from a research and development tax credit study that was completed in 2024 and excess tax benefits related to stock-based compensation.

## LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of December 31, 2025 and 2024:

	2025	2024	% Change
	(in millions, except percentages)		
<b>Cash, cash equivalents and marketable securities:</b>			
Cash and cash equivalents	\$ 5,084.8	\$ 4,569.6	
Marketable securities	1,523.3	1,546.3	
Long-term marketable securities	5,712.3	5,107.9	
Total cash, cash equivalents and marketable securities	<u>\$ 12,320.4</u>	<u>\$ 11,223.8</u>	10%
<b>Working Capital:</b>			
Total current assets	\$ 11,201.0	\$ 9,596.4	17%
Total current liabilities	(3,861.2)	(3,564.6)	8%
Total working capital	<u>\$ 7,339.8</u>	<u>\$ 6,031.8</u>	22%

### *Working Capital*

As of December 31, 2025, total working capital was \$7.3 billion, which represented an increase of \$1.3 billion, or 22%, from \$6.0 billion as of December 31, 2024, primarily due to increased cash and marketable securities due to product revenue growth, as well as increased inventories to support our recent commercial launches.

### *Cash Flows*

	2025	2024	2023
	(in millions)		
<b>Net cash provided by (used in):</b>			
Operating activities	\$ 3,631.4	\$ (492.6)	\$ 3,537.3
Investing activities	\$ (945.4)	\$ (3,770.0)	\$ (3,141.7)
Financing activities	\$ (2,261.3)	\$ (1,494.9)	\$ (562.2)

### **Operating Activities**

Cash provided by operating activities was \$3.6 billion in 2025, primarily due to income from operations of \$4.2 billion driven by our net product revenues partially offset by purchases of inventory and other changes in operating assets and liabilities. Cash used in operating activities was \$492.6 million in 2024, primarily due to our acquisition of Alpine partially offset by cash flows provided by other operating activities.

### **Investing Activities**

Cash used in investing activities was \$945.4 million in 2025, primarily related to net purchases of available-for-sale debt securities and purchases of property and equipment. Cash used in investing activities was \$3.8 billion in 2024, which included net purchases of available-for-sale debt securities of \$3.0 billion.

## **Financing Activities**

Cash used in financing activities were \$2.3 billion and \$1.5 billion in 2025 and 2024, respectively. Our financing activities in each year were primarily related to repurchases of our common stock pursuant to our share repurchase programs and payments in connection with common stock withheld for employee tax obligations.

### ***Sources and Uses of Liquidity***

We intend to rely on our existing cash, cash equivalents and current marketable securities together with our operating profitability as our primary source of liquidity. We expect that cash flows from our product sales together with our cash, cash equivalents and current marketable securities will be sufficient to fund our operations for at least the next twelve months. The adequacy of our available funds to meet our future operating and capital requirements will depend on many factors, including our future sales of currently marketed products, and the potential introduction of one or more new product candidates to the market, our business development activities, and the number, breadth and cost of our research and development programs.

### **Credit Facilities & Financing Strategy**

We may borrow up to a total of \$500.0 million pursuant to a revolving credit facility that we entered into in July 2022 and could repay and reborrow amounts under this revolving credit agreement without penalty. Subject to certain conditions, we could request that the borrowing capacity be increased by an additional \$500.0 million, for a total of \$1.0 billion. Negative covenants in our credit agreement could prohibit or limit our ability to access this source of liquidity. As of December 31, 2025, the facility was undrawn, and we were in compliance with these covenants.

We may also raise additional capital by borrowing under credit agreements, through public offerings or private placements of our securities, or securing new collaborative agreements or other methods of financing. We will continue to manage our capital structure and will consider all financing opportunities, whenever they may occur, that could strengthen our long-term liquidity profile. There can be no assurance that any such financing opportunities will be available on acceptable terms, if at all.

### ***Future Capital Requirements***

We have significant future capital requirements, including:

- Expected operating expenses to conduct research and development activities, manufacture and commercialize our existing and future products, and to operate our organization.
- Cash that we pay for income taxes.
- Royalties we pay related to sales of our CF products.
- Facility, operating and finance lease obligations as described below.
- Firm purchase obligations related to our supply and manufacturing processes.

In addition, other potential significant future capital requirements may include:

- We have entered into certain agreements with third parties that include the funding of certain research, development, manufacturing and commercialization efforts. Certain of our transactions, including collaborations, licensing arrangements, and asset acquisitions, include the potential for future milestone and royalty payments by us upon the achievement of pre-established developmental and regulatory targets and/or commercial targets. Other transactions include the potential for future lease-related expenses and other costs. Our obligation to fund these research and development and commercialization efforts and to pay these potential milestones, expenses and royalties is contingent upon continued involvement in the programs and/or the lack of any adverse events that could cause their discontinuance. We may enter into additional agreements, including acquisitions, collaborations, licensing arrangements and equity investments, which require additional capital.
- To the extent we borrow amounts under our existing credit agreement, we would be required to repay any outstanding principal amounts in 2027.

- As of December 31, 2025, we had \$3.4 billion remaining authorization available under the share repurchase program that our Board of Directors approved in May 2025. The program does not have an expiration date and can be discontinued at any time. We expect to fund the program through a combination of cash on hand and cash generated by operations.

Additional information on several of our future capital requirements is provided below.

### **Research and Development Costs**

We have ongoing clinical trials of product candidates at various stages of clinical development. Our clinical trial costs are dependent on, among other things, the size, number, and length of our clinical trials. These costs can increase as product candidates move from earlier-stage clinical trials into later-stage clinical development.

### **Leases**

We account for the majority of our real estate leases and each of our embedded leases with contract manufacturing organizations as operating leases. These include leases for our corporate headquarters at Fan Pier in Boston, Massachusetts, which continues through June 2044, and office and laboratory space at the Jeffrey Leiden Center for Biologics, Cell and Genetic Therapies Campus (the “Leiden Campus”) near our corporate headquarters. As of December 31, 2025, the longest lease at the Leiden Campus continues through the first quarter of 2042. We also have several embedded leases with contract manufacturing organizations related to the manufacturing and commercialization of our products with remaining lease terms up to 7 years as of December 31, 2025.

Our total future minimum lease payments for our leases for each of the next five years and in total are included in Note L, “Leases.” The total future undiscounted minimum lease payments were \$3.2 billion and \$178.1 million related to our operating and finance leases, respectively, as of December 31, 2025.

In addition to the items described above, we have a strategic agreement with Lonza to support the manufacture of T1D cell therapy product candidates, pursuant to which we have partnered with Lonza to build a 130,000 square foot dedicated new facility operated by Lonza in New Hampshire. Lease payments will begin in the first quarter of 2026 and continue through the tenth anniversary of the facility’s regulatory approval for commercial production. We may enter into additional lease agreements to support future product development and commercialization efforts, which would require additional capital.

## **CRITICAL ACCOUNTING POLICIES AND ESTIMATES**

Our discussion and analysis of our financial condition and results of operations are based upon our consolidated financial statements prepared in accordance with generally accepted accounting principles in the U.S. The preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate.

We believe that our application of the following accounting policies, each of which requires significant judgments and estimates on the part of management, are the most critical to aid in fully understanding and evaluating our reported financial results:

- revenue recognition;
- acquisitions, including intangible assets;
- pre-launch inventories; and
- income taxes.

Our accounting policies, including the ones discussed below, are more fully described in Note A, “Nature of Business and Accounting Policies.”

## **Revenue Recognition**

### **Product Revenues, Net**

We generate product revenues from sales in the U.S. and in international markets. We sell our products principally to a limited number of specialty pharmacy and specialty distributors as well as certain major wholesalers in the U.S., which account for the largest portion of our total revenues. Our customers in the U.S. subsequently resell our products to patients, health care providers, retail pharmacies, hospitals, or authorized treatment centers (“ATCs”) for CASGEVY. We contract with government agencies so that our products will be eligible for purchase by, or partial or full reimbursement from, such third-party payors. We make international sales primarily through distributor arrangements and to retail pharmacies, as well as to hospitals and clinics, many of which are government-owned or supported customers. In certain markets, we may not utilize a specialty distributor or specialty pharmacy to distribute CASGEVY. In these markets, we sell CASGEVY directly to ATCs. We recognize net product revenues from sales of our products when our customers obtain control of our products, which typically occurs upon delivery to customers for our small molecule products, including our CF products and JOURNAVX, and upon infusion of our gene-therapy products, including CASGEVY. Revenues from our product sales are recorded at the net sales price, or transaction price, which requires us to make several significant estimates regarding the net sales price.

We are required to make estimates for our product revenues related to government, commercial, and private payor rebates, chargebacks, discounts and fees, collectively rebates. The values of the rebates provided to third-party payors per course of treatment vary significantly and are based on government-mandated discounts and our arrangements with other third-party payors. Our most significant estimate relates to determining amounts due pursuant to the Medicaid Drug Rebate Program, including estimating the level of expected utilization of the rebates based on the amount of product sold to eligible patients. We track available information regarding changes, if any, to the payor mix for our products, to our contractual terms with third-party payors and to applicable governmental programs and regulations and levels of our products in the distribution channel. We adjust our estimated rebates based upon new information as it becomes available, including information regarding actual rebates for our products. Claims by third-party payors for rebates are submitted to us significantly after the related sales, potentially resulting in adjustments in the period in which the new information becomes known.

The following table summarizes activity related to our product revenue accruals for rebates for 2025, 2024 and 2023:

	<b>(in millions)</b>
Balance at December 31, 2022	<u>\$ 1,291.4</u>
Provision related to 2023 sales	3,481.4
Adjustments related to prior year(s) sales	(6.5)
Credits/payments made	(3,064.7)
Balance at December 31, 2023	<u>\$ 1,701.6</u>
Provision related to 2024 sales	3,673.0
Adjustments related to prior year(s) sales	(42.1)
Credits/payments made	(3,725.4)
Balance at December 31, 2024	<u>\$ 1,607.1</u>
Provision related to 2025 sales	3,780.4
Adjustments related to prior year(s) sales	(90.4)
Credits/payments made	(3,519.5)
Balance at December 31, 2025	<u>\$ 1,777.6</u>

We have also entered into annual contracts with government-owned and supported customers in international markets that limit the amount of annual reimbursement we can receive for our products. Upon exceeding the annual reimbursement amount provided by the customer’s contract with us, products are provided free of charge, which is a material right. If we estimate that the annual reimbursement amount under a contract will be exceeded for an annual period, we defer a portion of

the consideration received, which includes upfront payments and fees, for shipments made up to the annual reimbursement limit as “Other current liabilities.” Once the annual reimbursement limit has been reached, we recognize the deferred amount as revenue when we deliver the free products. To estimate the portion of the consideration received to be recognized as revenue and the portion of the amount to be deferred, we rely on our forecast of the number of units we will distribute during the applicable annual period in each international market in which our contracts with government-owned and supported customers limit the amount of annual reimbursement we can receive. Our forecasts are based on, among other things, our historical experience.

The preceding estimates and judgments materially affect our recognition of net product revenues. Changes in our estimates of net product revenues could have a material effect on net product revenues recorded in the period in which we determine that change occurs.

### ***Acquisitions***

As part of our business strategy, we seek to acquire products, product candidates and other technologies and businesses that are aligned with our corporate and research and development strategies and complement and advance our ongoing research and development efforts.

We are required to make several significant judgments and estimates to determine the accounting treatment for each acquisition transaction. If we determine that substantially all the fair value associated with an acquisition is concentrated in a single asset, or the acquisition does not constitute a business, we account for it as an asset acquisition. For example, we accounted for our \$5.0 billion acquisition of Alpine in 2024 as an asset acquisition because povetacept, Alpine’s lead molecule, represented substantially all of the fair value of the gross assets that we acquired. As a result, \$4.4 billion of the fair value attributed to povetacept was expensed to AIPR&D in 2024. If the fair value that we acquired in an acquisition is distributed among more than one asset, and the acquisition constitutes a business, we account for it as a business combination.

For an asset acquisition involving rights to intellectual property related to in-process research and development that is not yet associated with a product that has achieved regulatory approval, we generally expense our upfront payment to AIPR&D, because there is no alternative future use for the asset that was acquired.

For business combinations, we are required to make several significant judgments and estimates to calculate and allocate the purchase price, including the fair value of contingent consideration liabilities, to the assets that we have acquired and the liabilities that we have assumed on our consolidated balance sheet. The most significant judgment and estimate we have made for our business combinations relates to the fair value of the in-process research and development assets.

### **In-process Research and Development Intangible Assets**

As of December 31, 2025 and 2024, we had \$224.6 million and \$603.6 million, respectively, of in-process research and development assets on our consolidated balance sheet within “Other intangible assets, net.” During 2025, we recorded a \$379.0 million impairment of one of these assets, which was classified as an “Intangible asset impairment charge.” As of December 31, 2025, our remaining indefinite-lived in-process research and development assets were associated with our T1D program.

We characterize in-process research and development assets on our consolidated balance sheets as indefinite-lived intangible assets until the completion or abandonment of the associated research and development efforts. We test our in-process research and development intangible assets for impairment on an annual basis, and more frequently if indicators are present or changes in circumstances suggest that impairment may exist. When we determine that an indefinite-lived intangible asset has become impaired or we abandon the associated research and development project, we write down the carrying value to its fair value and record an impairment charge in the period in which the impairment occurs.

For example, in 2025, based on results from a Phase 1/2 clinical trial evaluating our VX-264 clinical program in patients with T1D, we concluded that VX-264 will not be advancing further in clinical development. Based on this event, we performed an interim impairment test on the fair value of our VX-264 indefinite-lived in-process research and development asset that we acquired from Semma Therapeutics, Inc. in 2019. We recorded the \$379.0 million impairment charge based on the results of this impairment test.

We use significant judgment to determine the fair value of our in-process research and development assets and have utilized either the multi-period excess earnings or the relief from royalty methods of the income approach. Each method requires us to estimate the probability of technical and regulatory success, revenue projections and growth rates, and appropriate discount and tax rates. The multi-period excess earnings method also requires us to estimate development and commercial costs. The relief from royalty method also requires us to estimate the after-tax royalty savings expected from ownership of the asset that we acquired. In 2025, we used the multi-period earnings method to record the impairment described above.

If one of our product candidates achieves regulatory approval, the in-process research and development intangible assets associated with the product candidate become finite-lived intangible assets as described below.

### **Finite-lived Intangible Assets**

As of December 31, 2025 and 2024, we had \$199.6 million and \$222.3 million, respectively, of finite-lived intangible assets on our consolidated balance sheet within “Other intangible assets, net.” These finite-lived intangible assets primarily relate to \$208.0 million of CASGEVY regulatory approval milestones recorded in 2023.

We amortize our finite-lived intangible assets related to our marketed products, which represent the majority of our finite-lived intangible assets, using the straight-line method within “Cost of sales” over the remaining estimated life of the assets beginning in the period in which regulatory approval is achieved or the assets are acquired and continuing through the period that we no longer have either exclusive rights to market the products associated with the assets or in-license rights to the intellectual property underlying the assets. We test finite-lived intangible assets for impairment if indicators are present or changes in circumstances suggest that the carrying value of an asset may not be recoverable. If we determine that the carrying value of a finite-lived intangible asset may not be recoverable, we compare the carrying value of the asset to the undiscounted cash flows that we expect the asset to generate. When we determine that a finite-lived intangible asset has become impaired, we write down the carrying value of the asset to its fair value and record an impairment charge in the period in which the impairment occurs.

### ***Pre-Launch Inventories***

We capitalize inventories prior to regulatory approval when we consider the related product candidate to have a high likelihood of regulatory approval and expect to recover the related costs. In making this determination, we evaluate, among other factors, the status of regulatory submissions and communications with regulatory authorities, information regarding the product candidate’s safety and efficacy, and the outlook for commercial sales, including the existence of any competition. As an example, during the first quarter of 2024, following positive results related to our Phase 3 trials for JOURNAVX, we began capitalizing inventories produced in preparation for our planned product launch. In January 2025, we received approval from the FDA to market JOURNAVX in the U.S. Prior to making this determination, we expensed inventoriable and related costs associated with JOURNAVX as “Research and development expenses.”

### ***Income Taxes***

We utilize the asset and liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement carrying amounts and tax basis of assets and liabilities using enacted tax rates in effect for years in which the temporary differences are expected to reverse. If our estimate of the tax effect of reversing temporary differences is (i) not reflective of actual outcomes, (ii) modified to reflect new developments or interpretations of the tax law, or (iii) revised to incorporate new accounting principles, or changes in the expected timing or manner of the reversal, our results of operations could be materially impacted.

We provide a valuation allowance when it is more likely than not that deferred tax assets will not be realized. On a periodic basis, we reassess our valuation allowances on our deferred tax assets, weighing positive and negative evidence to assess the recoverability of the deferred tax assets. Judgment is required in making these assessments to maintain or adjust our valuation allowances and, to the extent our future expectations change we would have to assess the recoverability of these deferred tax assets at that time. As of December 31, 2025, we maintained a valuation allowance of \$326.2 million related primarily to U.S. state tax attributes.

We record liabilities related to uncertain tax positions by prescribing a minimum recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. We adjust our liability to reflect any subsequent changes in the relevant facts and circumstances surrounding the

uncertain positions. We are subject to tax laws and audits in multiple jurisdictions and judgment is required in making this assessment. Consequently, we regularly re-evaluate uncertain tax positions and consider various factors, including changes in tax law, the measurement of tax positions taken or expected to be taken in tax returns, and changes in facts or circumstances related to a tax position. As of December 31, 2025, our liability for uncertain tax positions was \$852.1 million.

## **RECENT ACCOUNTING PRONOUNCEMENTS**

Refer to Note A, “Nature of Business and Accounting Policies,” in the accompanying notes to the consolidated financial statements for a discussion of recent accounting pronouncements and new accounting pronouncements adopted during 2025.

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

### ***Interest Rate Risk***

#### **Financial Instruments**

As part of our investment portfolio, we own financial instruments that are sensitive to market risks. The investment portfolio is used to preserve our capital, provide adequate liquidity and earn returns commensurate with our risk appetite. We invest in instruments that meet the credit quality standards outlined in our investment policy, which also limits the amount of credit exposure to any one issue or type of instrument. These instruments primarily include securities issued by the U.S. government and its agencies, investment-grade corporate bonds, asset-backed securities and money market funds. These investments are primarily denominated in U.S. Dollars and none are held for trading purposes.

All of our interest-bearing securities are subject to interest rate risk and could change in value if interest rates fluctuate. Substantially all of our investment portfolio consists of marketable securities with active secondary or resale markets to help ensure portfolio liquidity, and we have implemented guidelines limiting the term-to-maturity of our investment instruments. Since we account for these securities as available-for-sale, no gains or losses are realized due to changes in the fair value of our investments unless we sell our investments prior to maturity or incur a credit loss. Due to the conservative nature of these instruments, we do not believe that the fair value of our investments has a material exposure to interest rate risk.

While we are exposed to global interest rate fluctuations, our investment portfolio is most affected by fluctuations in U.S. interest rates, which affect the interest earned on our cash, cash equivalents and marketable securities.

#### **Credit Agreement**

In 2022, we entered into a \$500.0 million unsecured revolving credit facility (“credit agreement”). Loans under this credit agreement bear interest, at our option, at a base rate or a Secured Overnight Financing Rate (“SOFR”), plus an applicable margin based on our consolidated leverage ratio (the ratio of our total consolidated funded indebtedness to our consolidated EBITDA for the most recently completed four fiscal quarter period). Pursuant to our credit agreement, the applicable margin on base rate loans ranges from 0.000% to 0.500% and the applicable margin on SOFR loans ranges from 1.000% to 1.500%. We do not believe that changes in interest rates related to our credit agreement would have a material effect on our consolidated financial statements. As of December 31, 2025, we had no principal or interest outstanding under our credit facility. A portion of our “Interest expense” in 2026 will be dependent on whether, and to what extent, we borrow amounts under this facility.

### ***Foreign Exchange Market Risk***

As a result of our foreign operations, we face significant exposure to movements in foreign currency exchange rates between the U.S. dollar and various foreign currencies, the most significant of which is the Euro. Fluctuations in the amounts of our foreign revenues and fluctuations in foreign currency exchange rates, may have a positive or negative effect on our foreign exchange rate exposure. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, payables, and accruals, and inventories.

We have a foreign currency management program, which is separate from our investment policy and portfolio, with the objective of reducing the effect of exchange rate fluctuations on our operating results and forecasted revenues denominated in foreign currencies. We have cash flow hedges related to a portion of our forecasted product revenues that qualify for hedge accounting treatment under U.S. GAAP. We do not seek hedge accounting treatment for our foreign currency forward contracts related to monetary assets and liabilities that impact our operating results. As of December 31, 2025, we held

foreign exchange forward contracts that were designated as cash flow hedges with notional amounts totaling \$6.1 billion representing a net liability of \$111.5 million on our consolidated balance sheet.

Although not predictive in nature, we believe a hypothetical 10% threshold reflects a reasonably possible near-term change in exchange rates. If the December 31, 2025 exchange rates were to change by a hypothetical 10%, the fair value recorded on our consolidated balance sheet related to our foreign exchange forward contracts that were designated as cash flow hedges as of December 31, 2025 would change by approximately \$608.0 million. However, since these contracts hedge a specific portion of our forecasted product revenues denominated in certain foreign currencies, any change in the fair value of these contracts is recorded in “Accumulated other comprehensive (loss) income” on our consolidated balance sheets and is reclassified to earnings in the same periods during which the underlying product revenues affect earnings. Therefore, any change in the fair value of these contracts that would result from a hypothetical 10% change in exchange rates would be entirely offset by the change in value associated with the underlying hedged product revenues resulting in no impact on our future anticipated earnings and cash flows with respect to the hedged portion of our forecasted product revenues.

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

The information required by this Item 8 is contained on pages F-1 through F-48 of this Annual Report on Form 10-K.

## **ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE**

Not applicable.

## **ITEM 9A. CONTROLS AND PROCEDURES**

**(1) Evaluation of Disclosure Controls and Procedures.** Our chief executive officer and chief financial officer, after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rule 13a-15(e) and Rule 15d-15(e) promulgated under the Securities Exchange Act of 1934, as amended (the “Exchange Act”)) as of the end of the period covered by this Annual Report on Form 10-K, have concluded that, based on such evaluation, our disclosure controls and procedures were effective. In designing and evaluating the disclosure controls and procedures, management recognized 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 necessarily was required to apply our judgment in evaluating the cost-benefit relationship of possible controls and procedures.

**(2) Management’s Annual Report on Internal Control Over Financial Reporting.** Management is responsible for establishing and maintaining adequate internal control over financial reporting. Internal control over financial reporting is defined in Rule 13a-15(f) and Rule 15d-15(f) promulgated under the Exchange Act, as a process designed by, or under the supervision of, our principal executive and principal financial officers 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 generally accepted accounting principles. Our internal control over financial reporting include those policies and procedures that:

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

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2025. In making this assessment, we used the criteria set forth in the *Internal Control—Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (“COSO”). Based on our assessment, management has concluded that, as of December 31, 2025, our internal control over financial reporting is effective based on those criteria.

Our independent registered public accounting firm, Ernst & Young LLP, issued an attestation report on our internal control over financial reporting. See Section 4 below.

**(3) Changes in Internal Controls.** During the quarter ended December 31, 2025, there were no changes in our internal control over financial reporting that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

#### **(4) Report of Independent Registered Public Accounting Firm**

To the Shareholders and the Board of Directors of Vertex Pharmaceuticals Incorporated

#### **Opinion on Internal Control Over Financial Reporting**

We have audited Vertex Pharmaceuticals Incorporated's internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Vertex Pharmaceuticals Incorporated (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2025, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the 2025 consolidated financial statements of the Company and our report dated February 13, 2026 expressed an unqualified opinion thereon.

#### **Basis for Opinion**

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Annual Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

#### **Definition and Limitations of Internal Control Over Financial Reporting**

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting 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 the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (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.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Boston, Massachusetts  
February 13, 2026

## ITEM 9B. OTHER INFORMATION

### *Rule 10b5-1 Trading Plans*

Our policy governing transactions in our securities by our directors, officers, and employees permits our officers, directors and employees to enter into trading plans complying with Rule 10b5-1 under the Exchange Act. The following table describes the written plans for the sale of our securities adopted by our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) during the fourth quarter of 2025, each of which is intended to satisfy the affirmative defense conditions of Rule 10b5-1 (each, a “Trading Plan”). Other than as described in the table below, none of our directors or officers adopted, modified or terminated a Trading Plan in the fourth quarter of 2025.

<b>Name and Title</b>	<b>Date of Adoption of Trading Plan</b>	<b>Scheduled Expiration Date of Trading Plan<sup>(1)</sup></b>	<b>Maximum Shares Subject to Trading Plan</b>
Reshma Kewalramani <i>Chief Executive Officer and President</i>	11/17/2025	11/16/2026	40,000
Amit Sachdev <i>EVP, Chief Patient and External Affairs Officer</i>	11/18/2025	10/30/2026	70,498 <sup>(2)</sup>
Carmen Bozic <i>EVP, Global Medicines Development and Medical Affairs, Chief Medical Officer</i>	11/20/2025	11/02/2026	34,733 <sup>(2)</sup>
Duncan McKechnie <i>EVP, Chief Commercial Officer</i>	11/25/2025	11/13/2026	17,367 <sup>(2)</sup>

(1) A Trading Plan may expire on an earlier date if all contemplated transactions are completed before such Trading Plan’s expiration date, upon termination by broker or the holder of the Trading Plan, or as otherwise provided in the Trading Plan.

(2) The maximum shares listed has not been reduced by the number of shares of common stock that will be withheld to satisfy tax withholding obligations at future vesting dates because such number of shares is not yet determinable.

### *2026 Restated Articles of Organization*

On February 12, 2026, the Company filed Restated Articles of Organization with the Secretary of the Commonwealth of Massachusetts to consolidate its Articles of Organization and all prior amendments and to remove references to the Series A Junior Participating Preferred Stock, which is no longer outstanding. The restatement was effected for clarity only and did not result in any changes to the rights of holders of the Company’s common stock.

A copy of the Restated Articles of Organization is filed as Exhibit 3.1 to this Annual Report on Form 10-K and is incorporated herein by reference.

## ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS.

None.

### **PART III**

Portions of our definitive Proxy Statement for the 2026 Annual Meeting of Shareholders (“2026 Proxy Statement”) are incorporated by reference into this Part III of our Annual Report on Form 10-K.

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

The information regarding directors required by this Item 10 will be included in our 2026 Proxy Statement and is incorporated herein by reference. We expect this information to be provided under “Election of Directors,” “Corporate Governance and Risk Management,” “Shareholder Proposals for the 2027 Annual Meeting and Nominations for Director,” “Delinquent Section 16(a) Reports” and “Code of Conduct.” The information regarding executive officers required by this Item 10 is included in Part I of this Annual Report on Form 10-K.

We have adopted insider trading policies and procedures governing the purchase, sale and/or other dispositions of our securities by directors, officers and employees, or Vertex itself, that are reasonably designed to promote compliance with insider trading laws, rules and regulations and any listing standards applicable to us. A copy of our Insider Trading Policy is filed as Exhibit 19.1 to this Annual Report on Form 10-K.

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

The information required by this Item 11 will be included in the 2026 Proxy Statement and is incorporated herein by reference. We expect this information to be provided under “Compensation Committee Interlocks and Insider Participation,” “Compensation Discussion and Analysis,” “Compensation and Equity Tables,” “Director Compensation,” “Management Development and Compensation Committee Report” and/or “Corporate Governance and Risk Management.”

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

The information required by this Item 12 will be included in the 2026 Proxy Statement and is incorporated herein by reference. We expect this information to be provided under “Security Ownership of Certain Beneficial Owners and Management” and “Equity Compensation Plan Information.”

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

The information required by this Item 13 will be included in the 2026 Proxy Statement and is incorporated herein by reference. We expect this information to be provided under “Election of Directors,” “Corporate Governance and Risk Management,” and “Audit and Finance Committee.”

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

The information required by this Item 14 will be included in the 2026 Proxy Statement and is incorporated herein by reference. We expect this information to be provided under “Ratification of the Appointment of Independent Registered Public Accounting Firm.”

## PART IV

### ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a)(1) The Financial Statements required to be filed by Items 8 and 15(c) of Form 10-K, and filed herewith, are as follows:

	<b>Page Number in this Form 10-K</b>
Report of Independent Registered Public Accounting Firm (PCAOB ID: 42) .....	F-1
Consolidated Statements of Income (Loss) .....	F-3
Consolidated Statements of Comprehensive Income (Loss) .....	F-4
Consolidated Balance Sheets .....	F-5
Consolidated Statements of Shareholders' Equity .....	F-6
Consolidated Statements of Cash Flows .....	F-7
Notes to Consolidated Financial Statements .....	F-8

(a)(2) Financial Statement Schedules have been omitted because they are either not applicable or the required information is included in the consolidated financial statements or notes thereto listed in (a)(1) above.

(a)(3) Exhibits.

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

Exhibit Number	Exhibit Description	Filed with this report	Incorporated by Reference herein from—Form or Schedule	Filing Date/ Period Covered	SEC File/ Reg. Number
<b>Governance Documents</b>					
3.1	Restated Articles of Organization of Vertex Pharmaceuticals Incorporated, as amended.	X			
3.2	Amended and Restated By-Laws of Vertex Pharmaceuticals Incorporated.		10-K (Exhibit 3.2)	February 13, 2025	000-19319
<b>Stock Certificate</b>					
4.1	Specimen Stock Certificate.		10-K (Exhibit 4.1)	February 15, 2018	000-19319
4.2	Description of Securities.		10-K (Exhibit 4.2)	February 13, 2025	000-19319
<b>Collaboration Agreement</b>					
10.1	Research, Development and Commercialization Agreement, dated as of May 24, 2004, between Vertex Pharmaceuticals Incorporated and Cystic Fibrosis Foundation Therapeutics Incorporated.†		10-Q (Exhibit 10.1)	November 3, 2021	000-19319
10.2	Amendment No. 1 to Research, Development and Commercialization Agreement, dated as of January 6, 2006, between Vertex Pharmaceuticals Incorporated and Cystic Fibrosis Foundation Therapeutics Incorporated.†		10-Q (Exhibit 10.2)	November 3, 2021	000-19319
10.3	Amendment No. 2 to Research, Development and Commercialization Agreement, dated as of March 17, 2006, between Vertex Pharmaceuticals Incorporated and Cystic Fibrosis Foundation Therapeutics Incorporated.		10-Q/A (Exhibit 10.6)	August 19, 2011	000-19319
10.4	Amendment No. 5 to Research, Development and Commercialization Agreement, effective as of April 1, 2011, between Vertex Pharmaceuticals Incorporated and Cystic Fibrosis Foundation Therapeutics Incorporated.†		10-Q (Exhibit 10.3)	November 3, 2021	000-19319
10.5	Amendment No. 7 to Research, Development and Commercialization Agreement, dated October 13, 2016, between Vertex Pharmaceuticals Incorporated and Cystic Fibrosis Foundation Therapeutics Incorporated.†		10-Q (Exhibit 10.4)	November 3, 2021	000-19319

<b>Exhibit Number</b>	<b>Exhibit Description</b>	<b>Filed with this report</b>	<b>Incorporated by Reference herein from—Form or Schedule</b>	<b>Filing Date/ Period Covered</b>	<b>SEC File/ Reg. Number</b>
10.6	Amended and Restated Joint Development and Commercialization Agreement, dated April 16, 2021, between Vertex Pharmaceuticals Incorporated, Vertex Pharmaceuticals (Europe) Limited and CRISPR Therapeutics AG, CRISPR Therapeutics Limited, CRISPR Therapeutics, Inc., TRACR Hematology Ltd.†		10-Q (Exhibit 10.1)	July 30, 2021	000-19319
10.7	Amendment No. 1 to Amended and Restated Joint Development and Commercialization Agreement, dated December 12, 2023, between Vertex Pharmaceuticals Incorporated, Vertex Pharmaceuticals (Europe) Limited and CRISPR Therapeutics AG, CRISPR Therapeutics Limited, CRISPR Therapeutics, Inc., TRACR Hematology Ltd.†		10-K (Exhibit 10.7)	February 15, 2024	000-19319
<b>Leases</b>					
10.8	Lease, dated May 5, 2011, between Fifty Northern Avenue LLC and Vertex Pharmaceuticals Incorporated.†		10-Q (Exhibit 10.2)	July 30, 2021	000-19319
10.9	2024 Amendment to the Lease (50 Northern Avenue), dated August 15, 2024, between Vertex Pharmaceuticals Incorporated and SNH Seaport LLC. †		10-Q (Exhibit 10.1)	November 5, 2024	000-19319
10.10	Lease, dated May 5, 2011, between Eleven Fan Pier Boulevard LLC and Vertex Pharmaceuticals Incorporated.†		10-Q (Exhibit 10.3)	July 30, 2021	000-19319
10.11	2024 Amendment to Lease (11 Fan Pier Boulevard), dated August 15, 2024, between Vertex Pharmaceuticals Incorporated and SNH Seaport LLC.†		10-Q (Exhibit 10.2)	November 5, 2024	000-19319
<b>Financing Agreements</b>					
10.12	Credit Agreement, dated as of July 1, 2022, by and among Vertex Pharmaceuticals Incorporated, Bank of America, N.A. and the other lenders party thereto.		10-Q (Exhibit 10.1)	August 5, 2022	000-19319
10.13	First Amendment to Credit Agreement, dated June 20, 2024 by and between Vertex Pharmaceuticals Incorporated and Bank of America N.A.		10-Q (Exhibit 10.1)	August 2, 2024	000-19319
<b>Equity Plans</b>					
10.14	Amended and Restated 2006 Stock and Option Plan.*		10-Q (Exhibit 10.1)	October 25, 2018	000-19319
10.15	Form of Stock Option Agreement under Amended and Restated 2006 Stock and Option Plan (granted on or after July 30, 2013).*		10-K (Exhibit 10.20)	February 13, 2015	000-19319
10.16	Amended and Restated 2013 Stock and Option Plan.*		DEF 14A (Appendix A)	April 7, 2022	000-19319
10.17	Form of Non-Qualified Stock Option Agreement under 2013 Stock and Option Plan.*		10-K (Exhibit 10.17)	February 13, 2015	000-19319
10.18	Form of Restricted Stock Unit Agreement under 2013 Stock and Option Plan (U.S.).*		10-K (Exhibit 10.25)	February 16, 2016	000-19319
10.19	Form of Restricted Stock Unit Agreement under 2013 Stock and Option Plan (International).*		10-K (Exhibit 10.19)	February 13, 2015	000-19319
10.20	Form of Restricted Stock Unit Agreement Under 2013 Stock and Option Plan.*		10-K (Exhibit 10.17)	February 13, 2020	000-19319
10.21	Form of Restricted Stock Unit Agreement under 2013 Stock and Option Plan (granted on or after January 1, 2025).*		10-K (Exhibit 10.21)	February 13, 2025	000-19319
10.22	Form of Restricted Stock Unit Agreement (with performance conditions) under 2013 Stock and Option Plan.*		10-K (Exhibit 10.22)	February 13, 2025	000-19319
10.23	Non-Employee Director Deferred Compensation Plan.*		10-K (Exhibit 10.27)	February 16, 2016	000-19319
10.24	Vertex Pharmaceuticals Incorporated Employee Stock Purchase Plan.*		DEF 14A (Appendix B)	April 26, 2019	000-19319
<b>Agreements with Executive Officers and Directors</b>					
10.25	Employment Agreement, dated as of April 1, 2020, by and between Vertex Pharmaceuticals Incorporated and Jeffrey M. Leiden, M.D., Ph.D.*		8-K (Exhibit 10.1)	April 1, 2020	000-19319
10.26	Amendment No. 1 to Employment Agreement, between Jeffrey M. Leiden and Vertex Pharmaceuticals Incorporated, dated as of February 7, 2022.*		10-K (Exhibit 10.24)	February 9, 2022	000-19319
10.27	Amendment No. 2 to Employment Agreement, between Jeffrey M. Leiden and Vertex Pharmaceuticals Incorporated, dated as of February 8, 2023*		10-K (Exhibit 10.23)	February 10, 2023	000-19319

<b>Exhibit Number</b>	<b>Exhibit Description</b>	<b>Filed with this report</b>	<b>Incorporated by Reference herein from—Form or Schedule</b>	<b>Filing Date/ Period Covered</b>	<b>SEC File/ Reg. Number</b>
10.28	Amendment No.3 to Employment Agreement, between Jeffrey M. Leiden and Vertex Pharmaceuticals Incorporated, dated as of November 1, 2024.*		10-Q (Exhibit 10.3)	November 5, 2024	000-19319
10.29	Employee Non-disclosure, Non-competition and Inventions Agreement between Jeffrey M. Leiden and Vertex Pharmaceuticals Incorporated, dated December 14, 2011.*		10-K (Exhibit 10.35)	February 22, 2012	000-19319
10.30	Employment Agreement, dated as of July 24, 2019, between Vertex Pharmaceuticals Incorporated and Reshma Kewalramani.*		8-K (Exhibit 10.1)	July 25, 2019	000-19319
10.31	Change of Control Agreement, dated as of July 24, 2019, between Vertex Pharmaceuticals Incorporated and Reshma Kewalramani.*		8-K (Exhibit 10.2)	July 25, 2019	000-19319
10.32	Employment Agreement, dated as of August 27, 2012, between Vertex Pharmaceuticals Incorporated and Stuart Arbuckle.*		10-Q (Exhibit 10.1)	November 6, 2012	000-19319
10.33	Change of Control Agreement, dated as of August 27, 2012, between Vertex Pharmaceuticals Incorporated and Stuart Arbuckle.*		10-Q (Exhibit 10.2)	November 6, 2012	000-19319
10.34	Employment Agreement, dated as of December 12, 2014, between Vertex Pharmaceuticals Incorporated and David Altshuler.*		10-K (Exhibit 10.34)	February 16, 2016	000-19319
10.35	Change of Control Agreement, dated as of December 10, 2014, between Vertex Pharmaceuticals Incorporated and David Altshuler.*		10-K (Exhibit 10.35)	February 16, 2016	000-19319
10.36	Third Amended and Restated Employment Agreement, dated as of February 26, 2013, between Vertex Pharmaceuticals Incorporated and Amit Sachdev.*		10-K (Exhibit 10.42)	February 23, 2017	000-19319
10.37	Third Amended and Restated Change of Control Agreement, dated as of February 26, 2013, between Vertex Pharmaceuticals Incorporated and Amit Sachdev.*		10-K (Exhibit 10.43)	February 23, 2017	000-19319
10.38	Employment Agreement, dated February 7, 2025, by and between Vertex Pharmaceuticals Incorporated and Charles F. Wagner, Jr.*		10-Q (Exhibit 10.1)	May 6, 2025	000-19319
10.39	Change of Control Agreement, dated as of February 7, 2025, by and between Vertex Pharmaceuticals Incorporated and Charles F. Wagner, Jr.*		10-K (Exhibit 10.39)	February 13, 2025	000-19319
10.40	Employment Agreement, dated August 1, 2020, by and between Vertex Pharmaceuticals Incorporated and Nia Tatsis.*		10-K (Exhibit 10.36)	February 9, 2022	000-19319
10.41	Change of Control Agreement, dated August 1, 2020, by and between Vertex Pharmaceuticals Incorporated and Nia Tatsis.*		10-K (Exhibit 10.37)	February 9, 2022	000-19319
10.42	Employment Agreement, dated October 3, 2022, by and between Vertex Pharmaceuticals Incorporated and Carmen Bozic.*	X			
10.43	Change of Control Agreement, dated October 3, 2022, by and between Vertex Pharmaceuticals Incorporated and Carmen Bozic.*	X			
10.44	Vertex Pharmaceuticals Employee Compensation Plan.*	X			
10.45	Vertex Pharmaceuticals Non-Employee Board Compensation.*		10-K (Exhibit 10.43)	February 13, 2025	000-19319
<b>Insider Trading Policy</b>					
19.1	Vertex Pharmaceuticals Incorporated Insider Trading Policy.*		10-K (Exhibit 19.1)	February 13, 2025	000-19319
<b>Subsidiaries</b>					
21.1	Subsidiaries of Vertex Pharmaceuticals Incorporated.	X			
<b>Consent</b>					
23.1	Consent of Independent Registered Public Accounting Firm, Ernst & Young LLP.	X			
<b>Certifications</b>					
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.	X			
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.	X			
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.	X			
<b>Clawback Policy</b>					
97.1	Policy Relating to Recovery of Erroneously Awarded Compensation		10-K (Exhibit 97.1)	February 15, 2024	000-19319

<b>Exhibit Number</b>	<b>Exhibit Description</b>	<b>Filed with this report</b>	<b>Incorporated by Reference herein from—Form or Schedule</b>	<b>Filing Date/ Period Covered</b>	<b>SEC File/ Reg. Number</b>
101.INS	XBRL Instance	X			
101.SCH	XBRL Taxonomy Extension Schema	X			
101.CAL	XBRL Taxonomy Extension Calculation	X			
101.LAB	XBRL Taxonomy Extension Labels	X			
101.PRE	XBRL Taxonomy Extension Presentation	X			
101.DEF	XBRL Taxonomy Extension Definition	X			
104	Cover Page Interactive Data File—the cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.	X			

\* Management contract, compensatory plan or agreement.

† Confidential portions of this document have been redacted according to the applicable rules.

#### **ITEM 16. FORM 10-K SUMMARY**

Not applicable.



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## **Report of Independent Registered Public Accounting Firm**

To the Shareholders and the Board of Directors of Vertex Pharmaceuticals Incorporated

### **Opinion on the Financial Statements**

We have audited the accompanying consolidated balance sheets of Vertex Pharmaceuticals Incorporated (the Company) as of December 31, 2025 and 2024, the related consolidated statements of income (loss), comprehensive income (loss), shareholders' equity and cash flows for each of the three years in the period ended December 31, 2025, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2025 and 2024, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2025, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2025, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated February 13, 2026 expressed an unqualified opinion thereon.

### **Basis for Opinion**

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

### **Critical Audit Matter**

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the 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.

***Medicaid Drug Rebate Program in the U.S.***

*Description of  
the Matter*

As discussed in Note A to the Company's consolidated financial statements, the Company recognizes revenue from product sales based on amounts due from customers net of allowances for variable consideration, which include, among others, rebates mandated by law under Medicaid and other government pricing programs. The most significant estimates relate to government and private payor rebates, chargebacks, discounts and fees, collectively rebates. The Company includes an estimate of variable consideration in its transaction price at the time of sale, when control of the product transfers to the customer. The Company estimates its Medicaid and other government pricing accruals based on monthly sales, historical experience of claims submitted by the various states and jurisdictions, historical rebate rates and estimated lag time of the rebate invoices. Rebate accruals inclusive of estimated amounts due for claims not yet received or processed as part of the Company's Medicaid program are recorded within accrued expenses on the Company's consolidated balance sheet.

Auditing the allowances for rebates owed pursuant to the Medicaid Drug Rebate Program in the U.S. was complex and judgmental due to the significant estimation required in determining certain assumptions including the levels of expected utilization of these rebates based on the amount of product sold to eligible patients, as well as the complexity of the government mandated rebate calculations. The allowances for rebates owed pursuant to the Medicaid Drug Rebate Program in the U.S. are sensitive to these significant assumptions and calculations.

*How We  
Addressed the  
Matter in Our  
Audit*

We obtained an understanding, evaluated the design and tested the operating effectiveness of controls over the Company's revenue recognition process, including controls over management's computation and review of the allowances for Medicaid rebates. We tested the Company's controls to assess the completeness and accuracy of the current and historical data that supports the Medicaid estimate, significant assumptions related to the inputs utilized as well as management's review of the application of the government pricing regulations.

Our audit procedures to test the allowances for rebates owed pursuant to the Medicaid Drug Rebate Program in the U.S., included the following: we assessed the methodology used to determine the estimate and tested the significant assumptions as well as the underlying data used by the Company in its analysis. We also assessed the historical accuracy of the Company's estimates of Medicaid rebates by comparing assumptions to historical trends and evaluating the change from prior periods. We further tested the completeness and accuracy of the underlying data used in the Company's calculations through reconciliation to third-party invoices, claims data and actual cash payments. In addition, we involved our government pricing specialists to assist in evaluating management's methodology and calculations used in the measurement of certain estimated rebates.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2005.

Boston, Massachusetts  
February 13, 2026

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Consolidated Statements of Income (Loss)**  
(in millions, except per share amounts)

	Year Ended December 31,		
	2025	2024	2023
Revenues:			
Product revenues, net	\$ 11,970.6	\$ 11,020.1	\$ 9,869.2
Other revenues	30.7	—	—
Total revenues	12,001.3	11,020.1	9,869.2
Costs and expenses:			
Cost of sales	1,651.3	1,530.5	1,262.2
Research and development expenses	3,909.5	3,630.3	3,162.9
Acquired in-process research and development expenses	133.0	4,628.4	527.1
Selling, general and administrative expenses	1,753.1	1,464.3	1,136.6
Intangible asset impairment charge	379.0	—	—
Change in fair value of contingent consideration	2.1	(0.5)	(51.6)
Total costs and expenses	7,828.0	11,253.0	6,037.2
Income (loss) from operations	4,173.3	(232.9)	3,832.0
Interest income	490.9	598.1	614.7
Interest expense	(13.3)	(30.6)	(44.1)
Other expense, net	(7.7)	(86.1)	(22.8)
Income before provision for income taxes	4,643.2	248.5	4,379.8
Provision for income taxes	690.0	784.1	760.2
Net income (loss)	<u>\$ 3,953.2</u>	<u>\$ (535.6)</u>	<u>\$ 3,619.6</u>
Net income (loss) per common share:			
Basic	\$ 15.46	\$ (2.08)	\$ 14.05
Diluted	\$ 15.32	\$ (2.08)	\$ 13.89
Shares used in per share calculations:			
Basic	255.7	257.9	257.7
Diluted	258.0	257.9	260.5

The accompanying notes are an integral part of these consolidated financial statements.

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Consolidated Statements of Comprehensive Income (Loss)**  
(in millions)

	Year ended December 31,		
	2025	2024	2023
Net income (loss)	\$ 3,953.2	\$ (535.6)	\$ 3,619.6
Other comprehensive (loss) income:			
Unrealized holding gains (losses) on available-for-sale debt securities, net of tax of \$(7.6), \$0.6 and \$(2.7), respectively	26.9	(2.5)	9.7
Unrealized (losses) gains on foreign currency forward contracts, net of tax of \$56.0, \$(38.2) and \$14.0, respectively	(198.1)	136.0	(50.9)
Foreign currency translation adjustment	27.5	8.6	26.1
Total other comprehensive (loss) income	(143.7)	142.1	(15.1)
Comprehensive income (loss)	<u>\$ 3,809.5</u>	<u>\$ (393.5)</u>	<u>\$ 3,604.5</u>

The accompanying notes are an integral part of these consolidated financial statements.

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Consolidated Balance Sheets**  
(in millions, except share and per share data)

	December 31,	
	2025	2024
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 5,084.8	\$ 4,569.6
Marketable securities	1,523.3	1,546.3
Accounts receivable, net	2,052.8	1,609.4
Inventories	1,686.8	1,205.4
Prepaid expenses and other current assets	853.3	665.7
Total current assets	11,201.0	9,596.4
Property and equipment, net	1,520.3	1,227.8
Goodwill	1,088.0	1,088.0
Other intangible assets, net	424.2	825.9
Deferred tax assets	2,897.9	2,331.1
Operating lease assets	1,562.7	1,356.8
Long-term marketable securities	5,712.3	5,107.9
Other assets	1,236.6	999.3
Total assets	<u>\$ 25,643.0</u>	<u>\$ 22,533.2</u>
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 461.7	\$ 413.0
Accrued expenses	2,971.2	2,788.6
Other current liabilities	428.3	363.0
Total current liabilities	3,861.2	3,564.6
Long-term operating lease liabilities	1,846.5	1,544.4
Other long-term liabilities	1,269.5	1,014.6
Total liabilities	<u>6,977.2</u>	<u>6,123.6</u>
Commitments and contingencies (Note P)		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding	—	—
Common stock, \$0.01 par value; 500,000,000 shares authorized, 253,991,224 and 256,940,382 shares issued and outstanding, respectively	2.5	2.6
Additional paid-in capital	5,119.2	6,672.4
Accumulated other comprehensive (loss) income	(15.9)	127.8
Retained earnings	13,560.0	9,606.8
Total shareholders' equity	<u>18,665.8</u>	<u>16,409.6</u>
Total liabilities and shareholders' equity	<u>\$ 25,643.0</u>	<u>\$ 22,533.2</u>

The accompanying notes are an integral part of these consolidated financial statements.

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Consolidated Statements of Shareholders' Equity**  
(in millions)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings	Total Shareholders' Equity
	Shares	Amount				
<b>Balance at December 31, 2022</b>	257.0	\$ 2.6	\$ 7,386.5	\$ 0.8	\$ 6,522.8	\$ 13,912.7
Other comprehensive loss, net of tax	—	—	—	(15.1)	—	(15.1)
Net income	—	—	—	—	3,619.6	3,619.6
Repurchases of common stock	(1.3)	—	(427.6)	—	—	(427.6)
Common stock withheld for employee tax obligations	(0.7)	—	(226.1)	—	—	(226.1)
Issuance of common stock under benefit plans	2.7	—	133.4	—	—	133.4
Stock-based compensation expense	—	—	583.5	—	—	583.5
<b>Balance at December 31, 2023</b>	<u>257.7</u>	<u>\$ 2.6</u>	<u>\$ 7,449.7</u>	<u>\$ (14.3)</u>	<u>\$ 10,142.4</u>	<u>\$ 17,580.4</u>
Other comprehensive income, net of tax	—	—	—	142.1	—	142.1
Net loss	—	—	—	—	(535.6)	(535.6)
Repurchases of common stock	(2.7)	—	(1,194.9)	—	—	(1,194.9)
Common stock withheld for employee tax obligations	(0.9)	—	(405.0)	—	—	(405.0)
Issuance of common stock under benefit plans	2.8	—	113.5	—	—	113.5
Stock-based compensation expense	—	—	709.1	—	—	709.1
<b>Balance at December 31, 2024</b>	<u>256.9</u>	<u>\$ 2.6</u>	<u>\$ 6,672.4</u>	<u>\$ 127.8</u>	<u>\$ 9,606.8</u>	<u>\$ 16,409.6</u>
Other comprehensive loss, net of tax	—	—	—	(143.7)	—	(143.7)
Net income	—	—	—	—	3,953.2	3,953.2
Repurchases of common stock	(4.8)	(0.1)	(2,011.5)	—	—	(2,011.6)
Common stock withheld for employee tax obligations	(0.7)	—	(369.9)	—	—	(369.9)
Issuance of common stock under benefit plans	2.6	—	127.9	—	—	127.9
Stock-based compensation expense	—	—	700.3	—	—	700.3
<b>Balance at December 31, 2025</b>	<u>254.0</u>	<u>\$ 2.5</u>	<u>\$ 5,119.2</u>	<u>\$ (15.9)</u>	<u>\$ 13,560.0</u>	<u>\$ 18,665.8</u>

The accompanying notes are an integral part of these consolidated financial statements.

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Consolidated Statements of Cash Flows**  
(in millions)

	Year Ended December 31,		
	2025	2024	2023
Cash flows from operating activities:			
Net income (loss)	\$ 3,953.2	\$ (535.6)	\$ 3,619.6
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:			
Stock-based compensation expense	685.9	698.5	581.2
Depreciation and amortization expense	209.8	207.2	181.3
Intangible asset impairment charges	379.0	—	—
Deferred income taxes	(510.8)	(348.8)	(536.5)
Other non-cash items, net	113.4	0.9	(42.6)
Changes in operating assets and liabilities:			
Accounts receivable, net	(347.3)	(99.3)	(84.1)
Inventories	(524.2)	(517.3)	(322.9)
Prepaid expenses and other assets	(396.0)	(200.3)	(545.7)
Accounts payable	36.8	49.5	48.7
Accrued expenses	(116.9)	212.9	429.4
Other liabilities	148.5	39.7	208.9
Net cash provided by (used in) operating activities	3,631.4	(492.6)	3,537.3
Cash flows from investing activities:			
Purchases of available-for-sale debt securities	(6,396.5)	(7,438.2)	(3,786.5)
Sales and maturities of available-for-sale debt securities	5,897.4	4,465.6	839.1
Purchases of property and equipment	(437.6)	(297.7)	(200.4)
Proceeds related to equity securities	16.0	—	95.1
Net payments related to finite-lived intangible assets	—	(187.7)	(58.0)
Acquisition of available-for-sale debt securities from Alpine Immune Sciences, Inc.	—	(258.0)	—
Other investing activities	(24.7)	(54.0)	(31.0)
Net cash used in investing activities	(945.4)	(3,770.0)	(3,141.7)
Cash flows from financing activities:			
Issuances of common stock under benefit plans	127.7	114.6	134.6
Repurchases of common stock	(2,017.4)	(1,177.1)	(427.6)
Payments in connection with common stock withheld for employee tax obligations	(369.9)	(405.0)	(226.1)
Payments on finance leases	(5.4)	(33.6)	(44.9)
Other financing activities	3.7	6.2	1.8
Net cash used in financing activities	(2,261.3)	(1,494.9)	(562.2)
Effect of changes in exchange rates on cash	90.9	(42.6)	26.9
Net increase (decrease) in cash, cash equivalents and restricted cash	515.6	(5,800.1)	(139.7)
Cash, cash equivalents and restricted cash—beginning of period	4,572.2	10,372.3	10,512.0
Cash, cash equivalents and restricted cash—end of period	\$ 5,087.8	\$ 4,572.2	\$ 10,372.3
Supplemental disclosure of cash flow information:			
Cash paid for income taxes	\$ 1,566.7	\$ 1,082.1	\$ 1,677.3
Cash paid for interest	\$ 12.4	\$ 30.5	\$ 43.1
Net payments due to CRISPR Therapeutics AG related to finite-lived intangible assets	\$ —	\$ —	\$ 180.0

The accompanying notes are an integral part of these consolidated financial statements.

# VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Consolidated Financial Statements

### A. Nature of Business and Accounting Policies

#### *Business*

Vertex Pharmaceuticals Incorporated (“Vertex,” “we,” “us” or “our”) is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases, with a focus on specialty markets. We have approved medicines for cystic fibrosis (“CF”), sickle cell disease (“SCD”), transfusion dependent beta thalassemia (“TDT”), and acute pain, and we continue to serially innovate and advance next-generation clinical and research programs in these areas. Our mid- and late-stage clinical pipeline includes programs across a range of modalities in additional serious diseases, including IgA nephropathy (“IgAN”), APOL1-mediated kidney disease, neuropathic pain, type 1 diabetes (“T1D”), primary membranous nephropathy (“pMN”), autosomal dominant polycystic kidney disease, and myotonic dystrophy type 1 (“DM1”).

Our marketed CF medicines are ALYFTREK (vanzacaftor/tezacaftor/deutivacaftor), which was approved by the U.S. Food and Drug Administration (“FDA”) in December 2024, TRIKAFTA/KAFTRIO (elexacaftor/tezacaftor/ivacaftor and ivacaftor), SYMDEKO/SYMKEVI (tezacaftor/ivacaftor and ivacaftor), ORKAMBI (lumacaftor/ivacaftor) and KALYDECO (ivacaftor).

CASGEVY (exagamglogene autotemcel), our ex-vivo, non-viral CRISPR/Cas9-based gene-editing therapy for severe SCD and TDT, is approved in the United States (“U.S.”) and across multiple geographies including Europe, Canada, and the Middle East. CASGEVY was initially approved by the FDA in December 2023.

In January 2025, the FDA approved JOURNAVX (suzetrigine), our first-in-class, oral pain signal inhibitor that is highly selective for voltage-gated sodium channel NaV1.8, for the treatment of moderate-to-severe acute pain in adults.

#### *Basis of Presentation*

The accompanying consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the U.S. (“U.S. GAAP”), reflect the operations of Vertex and our wholly owned subsidiaries. All material intercompany balances and transactions have been eliminated. We operate in one segment, pharmaceuticals. Please refer to Note Q, “Segment Information,” for enterprise-wide disclosures regarding our revenues, major customers, significant segment expenses, and long-lived assets by geographic area.

#### *Use of Estimates*

The preparation of consolidated financial statements in accordance with U.S. GAAP requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of our consolidated financial statements, and the amounts of revenues and expenses during the reported periods. We base our estimates on historical experience and various other assumptions, including in certain circumstances future projections that we believe to be reasonable under the circumstances. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

#### *Revenue Recognition*

We recognize revenue when a customer obtains control of promised goods or services. We record the amount of revenue that reflects the consideration that we expect to receive in exchange for those goods or services. We apply the following five-step model to determine this amount: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) we satisfy each performance obligation.

We only apply the five-step model to contracts when it is probable that we will collect the consideration to which we are entitled in exchange for the goods or services that we transfer to the customer. Once a contract is determined to be within the

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

scope of Accounting Standards Codification (“ASC”) 606, *Revenue from Contracts with Customers* at contract inception, we review the contract to determine which performance obligations we must deliver and which of these performance obligations are distinct. We recognize as revenue the amount of the transaction price that is allocated to each performance obligation when that performance obligation is satisfied or as it is satisfied. Generally, our performance obligations are transferred to customers at a point in time, typically upon delivery.

#### **Product Revenues, Net**

We sell our products primarily to a limited number of specialty pharmacy and specialty distributors globally, as well as to certain major wholesalers in the U.S. and to retail pharmacies, hospitals and clinics internationally. Many of the international hospitals and clinics are government-owned or supported. Our customers in the U.S. subsequently resell our products to patients, health care providers, retail pharmacies, hospitals, or authorized treatment centers (“ATCs”). In certain markets, we may sell CASGEVY directly to ATCs. Revenue recognition typically occurs upon delivery of our small molecule products, including our CF medicines and JOURNAVX, and upon infusion of our gene-therapy products, including CASGEVY.

Revenues from product sales are recorded at the net sales price, or “transaction price,” which includes estimates of variable consideration that result from (a) invoice discounts for prompt payment and distribution fees, (b) government and private payor rebates, chargebacks, discounts and fees, (c) product returns, and (d) other adjustments for certain indirect customers, including costs of co-pay assistance programs for patients. Reserves are established for the estimates of variable consideration based on the amounts earned or to be claimed on the related sales. The reserves are classified as reductions to “Accounts receivable, net” if payable to a customer or “Accrued expenses” if payable to a third-party. Where appropriate, we utilize the expected value method to determine the appropriate amount for estimates of variable consideration based on factors such as our historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. The amount of variable consideration that is included in the transaction price may be constrained and is included in our net product revenues 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. Actual amounts of consideration ultimately received may differ from our estimates. If actual results vary from our estimates, we adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

*Invoice Discounts and Distribution Fees:* In the U.S., we may provide invoice discounts on product sales to our customers for prompt payment and pay distribution and administrative fees, such as fees for certain data that customers provide to us. These fees are based on a fixed percentage of sales. We estimate that, based on our experience, our customers will earn these discounts and fees, and deduct the full amount of these discounts and fees from our gross product revenues and accounts receivable at the time such revenues are recognized.

*Rebates, Chargebacks, Discounts and Fees:* We contract with government agencies and commercial payors (our “Third-party Payors”) so that products will be eligible for purchase by, or partial or full reimbursement from, such Third-party Payors. We estimate the rebates, chargebacks, discounts and fees we will provide to Third-party Payors and deduct these estimated amounts from our gross product revenues at the time the revenues are recognized. For each product, we estimate the aggregate rebates, chargebacks and discounts that we will provide to Third-party Payors based upon (i) our contracts with these Third-party Payors, (ii) the government-mandated discounts and fees applicable to government-funded programs, (iii) information obtained from our customers and other third-party data regarding the payor mix for such product and (iv) historical experience.

*Product Returns:* Return policies vary by product and market. We typically permit returns if our product is damaged, defective, or otherwise cannot be used by our customer. In specific cases, we will allow returns for expired product as defined within specific customer agreements. We record deductions from our gross product revenues for estimated sales returns in the period the related revenue is recognized and base our estimate for returns on historical experience and known or expected changes in the marketplace specific to each product.

*Other Adjustments:* We offer patient support programs to eligible patients, such as co-pay assistance programs, which require us to establish accruals based on an estimated cost per claim that we expect to receive.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

We make significant estimates and judgments that materially affect our recognition of net product revenues. Our most significant estimate relates to determining amounts due pursuant to the Medicaid Drug Rebate Program, including estimating the level of expected utilization of the rebates based on the amount of product sold to eligible patients. We adjust our estimated rebates, chargebacks and discounts based on new information, including information regarding actual rebates, chargebacks and discounts for our products, as it becomes available. Claims by Third-party Payors for rebates, chargebacks and discounts frequently are submitted to us significantly after the related sales, potentially resulting in adjustments in the period in which the new information becomes known. Our credits to product revenue related to prior period sales have not been significant and primarily related to rebates and discounts.

Our payment terms, which typically range from 30 to 150 days depending on the product and market, are consistent with prevailing market practice. We do not adjust our net product revenues for the effects of a significant financing component for transactions where we expect, at contract inception, the period between our customer obtaining control of our product and when we receive payment to be one year or less.

We exclude taxes collected from customers relating to product sales and remitted to governmental authorities from revenues.

#### Contract Liabilities

We had contract liabilities of \$171.8 million and \$206.8 million as of December 31, 2025 and 2024, respectively, primarily related to annual contracts with government-owned and supported customers in international markets that limit the amount of annual reimbursement we can receive for our CF products. Upon exceeding the annual reimbursement amount provided by the customer's contract with us, our CF products are provided free of charge, which is a material right. These contracts include upfront payments and fees. If we estimate that we will exceed the annual reimbursement amount under a contract, we defer a portion of the consideration received for shipments made up to the annual reimbursement limit as a portion of "Other current liabilities." Once the reimbursement limit has been reached, we recognize the deferred amount as revenue when we deliver the free products. Our CF product revenue contracts include performance obligations that are one year or less.

Our contract liabilities at the end of each fiscal year relate to contracts with CF annual reimbursement limits in international markets in which the annual period associated with the contract is not the same as our fiscal year. In these markets we recognize revenues related to performance obligations satisfied in previous years; however, these revenues do not relate to any performance obligations that were satisfied more than 12 months prior to the beginning of the current year. During the years ended December 31, 2025, 2024 and 2023, we recorded \$206.8 million, \$170.3 million and \$159.6 million, respectively, of CF product revenues that were recorded as contract liabilities at the beginning of the year.

#### **Other Revenues**

We have not recognized significant revenues other than our product revenues during the three years ended December 31, 2025. In 2025, our "Other revenues" were primarily related to \$20.6 million and \$10.0 million associated with upfront payments, for licenses that we concluded were distinct, received from our agreements with Ono Pharmaceuticals Co., Ltd. ("Ono") and Zai Lab Limited ("Zai"), respectively. Please refer to Note B, "Collaboration, License and Other Arrangements," for further information about these agreements. In future periods, we may recognize additional other revenues generated through collaborative research, development and/or commercialization agreements related to one or more of the following: nonrefundable upfront license fees; development and commercial milestones; funding of research and development activities; and royalties on net sales of licensed products. Revenue is recognized upon satisfaction of a performance obligation by transferring control of a good or service to our collaborator.

For each agreement that results in revenue, we identify all material performance obligations and determine the transaction price by estimating the amount of variable consideration at the outset of the contract. We constrain (reduce) the estimate of variable consideration such that it is probable that a significant reversal of previously recognized revenue will not occur throughout the life of the contract. We utilize the sales- and usage-based royalty exception in arrangements that resulted from the license of intellectual property, recognizing revenues generated from royalties as the underlying sales occur.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

Once the estimated transaction price is established, amounts are allocated to each separate performance obligation that has been identified on a relative standalone selling price basis. The consideration allocated to each distinct performance obligation is recognized as revenue when control of the related goods or services is transferred.

#### *Cost of Sales*

Our cost of sales primarily includes royalty expenses, cost of product sales, intangible asset amortization expenses, and other items related to our manufacturing processes, adjusted by CRISPR Therapeutics AG's ("CRISPR") share of the net commercial profits or losses for CASGEVY. Please refer to Note B, "Collaboration, License and Other Arrangements," for further information on our royalties related to our CF products and our agreements with CRISPR related to the treatment of net commercial profits or losses for CASGEVY.

Shipping and handling costs incurred for inventory purchases are capitalized and recorded upon sale in "Cost of sales" in our consolidated statements of income (loss). Shipping and handling costs incurred for product shipments are recorded as incurred in "Cost of sales" in our consolidated statements of income (loss).

#### *Research and Development Expenses*

Research and development expenses are comprised of costs we incur in performing research and development activities, including salary and benefits; stock-based compensation expense; outsourced services and other direct expenses, including clinical trial, pharmaceutical development and drug supply costs; and infrastructure costs, including facilities costs and depreciation expense. We recognize research and development expenses as incurred. We capitalize nonrefundable advance payments we make for research and development activities and expense the payments as the related goods are delivered or the related services are performed.

#### *Acquired In-process Research and Development Expenses*

Our research and development activities include upfront, contingent milestone, and other payments pursuant to our business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions. In-process research and development that is acquired in a transaction that does not qualify as a business combination under U.S. GAAP and that does not have an alternative future use is recorded to "Acquired in-process research and development expenses" ("AIPR&D") in our consolidated statements of income (loss) in the period in which it is acquired.

In transactions that do not qualify as a business combination, we present the cost to acquire AIPR&D within our "Cash flows from operating activities" in our consolidated statements of cash flows.

#### *Stock-based Compensation Expense*

We expense the fair value of employee restricted stock units and other forms of stock-based employee compensation over the associated employee service period on a straight-line basis. Stock-based compensation expense is determined based on the fair value of the award at the grant date and is adjusted each period to reflect actual forfeitures and the outcomes of certain performance conditions.

For awards with performance conditions in which the award does not vest unless the performance condition is met, we recognize expense if, and to the extent that, we estimate that achievement of the performance condition is probable. If we conclude that vesting is probable, we recognize expense from the date we reach this conclusion through the estimated vesting date.

We provide to employees who have rendered a certain number of years of service to Vertex and meet certain age requirements, partial or full acceleration of vesting of these equity awards, subject to certain conditions including a notification period, upon a termination of employment other than for cause. A low percentage of our employees were eligible for partial or full acceleration of any of their equity awards as of December 31, 2025. We recognize stock-based compensation expense related to these awards over a service period reflecting qualified employees' eligibility for partial or full acceleration of vesting.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

Please refer to Note N, “Stock-based Compensation Expense,” for tables displaying our stock-based compensation expense by type of award and by line item within our consolidated statements of income (loss).

#### *Advertising Costs*

Advertising costs, including promotional expenses were \$202.8 million, \$85.7 million and \$45.8 million in 2025, 2024 and 2023, respectively. Our advertising costs are expensed as incurred and recorded to “Selling, general and administrative expenses,” in our consolidated statements of income (loss).

#### *Fair Value of Contingent Consideration*

We base our estimates of the probability of achieving the milestones relevant to the fair value of contingent payments on industry data and our knowledge of the programs and viability of the programs. Estimates included in the discounted cash flow models pertaining to contingent payments also include: (i) estimates regarding the timing of the relevant development and commercial milestones and royalties, and (ii) and appropriate discount rates. We record any increases or decreases in the fair value of our contingent payments to “Change in fair value of contingent consideration” in our consolidated statements of income (loss). We record our contingent consideration liabilities at fair value on our consolidated balance sheets as “Other current liabilities” or “Other long-term liabilities” depending on when we estimate we will pay them. Please refer to Note D, “Fair Value Measurements,” for further information.

#### *Income Taxes*

Our provision for income taxes is accounted for under the asset and liability method and includes federal, state, local and foreign taxes.

Deferred tax assets and liabilities are recognized for the estimated future tax consequences of temporary differences between the financial statement carrying amounts and the income tax bases of assets and liabilities. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the year in which the temporary differences are expected to be recovered or settled. A valuation allowance is applied against any net deferred tax asset if, based on the available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized. On a periodic basis, we reassess the valuation allowance on our deferred income tax assets weighing positive and negative evidence to assess the recoverability of our deferred tax assets. We include, among other things, our recent financial performance and our future projections in this periodic assessment.

We record liabilities related to uncertain tax positions by prescribing a minimum recognition threshold and measurement attribute for the financial statement recognition and measurement of a tax position taken or expected to be taken in a tax return. We evaluate our uncertain tax positions on a quarterly basis and consider various factors, including, but not limited to, changes in tax law, the measurement of tax positions taken or expected to be taken in our tax returns, and changes in facts or circumstances related to a tax position. We adjust our liabilities to reflect any subsequent changes in the relevant facts and circumstances surrounding the uncertain positions. We accrue interest and penalties related to unrecognized tax benefits as a component of our “Provision for income taxes.”

As part of the U.S. Tax Cut and Jobs Act of 2017, we are subject to a territorial tax system, under which we must establish an accounting policy to provide for tax on Net Controlled Foreign Corporation Tested Income (“NCTI”) (formerly Global Intangible Low Taxed Income) earned by certain foreign subsidiaries. We have elected to treat the impact of NCTI as a current tax expense in our “Provision for income taxes.”

#### *Net Income (Loss) Per Common Share*

Basic net income (loss) per common share is based upon the weighted-average number of common shares outstanding during the period. Diluted net income (loss) per common share utilizing the treasury-stock method is based upon the weighted-average number of common shares outstanding during the period plus additional weighted-average common equivalent shares outstanding during the period when the effect is dilutive. Potentially dilutive shares result from the assumed (i) vesting of restricted stock units (“RSUs”) and performance-based restricted stock units (“PSUs”), and (ii) exercise of outstanding stock options. The proceeds of such vestings or exercises are assumed to have been used to repurchase outstanding stock using the treasury-stock method.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

#### *Comprehensive Income (Loss)*

Comprehensive income (loss) consists of net income (loss) and other comprehensive (loss) income, which includes foreign currency translation adjustments and unrealized gains and losses on foreign currency forward contracts and our available-for-sale debt securities. For purposes of comprehensive income disclosures, we record provisions for or benefits from income taxes related to the unrealized gains and losses on foreign currency forward contracts and our available-for-sale debt securities. We record provisions for or benefits from income taxes related to our cumulative translation adjustment only for those undistributed earnings in our foreign subsidiaries that we do not intend to permanently reinvest.

#### *Cash and Cash Equivalents*

We consider all highly liquid investments with original maturities of three months or less at the date of purchase to be cash equivalents.

#### *Marketable Securities*

As of December 31, 2025, our marketable securities consisted of investments in available-for-sale debt securities and corporate equity securities with readily determinable fair values. We classify marketable securities with current maturities of less than one year as current assets on our consolidated balance sheets. The remainder of our marketable securities are classified as long-term assets within “Long-term marketable securities” on our consolidated balance sheets. The fair value of these securities is based on quoted prices for identical or similar assets.

We record unrealized gains (losses) on available-for-sale debt securities as a component of “Accumulated other comprehensive (loss) income,” which is a separate component of shareholders’ equity on our consolidated balance sheets, until such gains and losses are realized. Realized gains and losses, if any, are determined using the specific identification method.

For available-for-sale debt securities in unrealized loss positions, we are required to assess whether to record an allowance for credit losses using an expected loss model. A credit loss is limited to the amount by which the amortized cost of an investment exceeds its fair value. A previously recognized credit loss may be decreased in subsequent periods if our estimate of fair value for the investment increases. To determine whether to record a credit loss, we consider issuer specific credit ratings and historical losses as well as current economic conditions and our expectations for future economic conditions.

We record changes in the fair value of our investments in corporate equity securities to “Other expense, net” in our consolidated statements of income (loss). Realized gains and losses, which are also included in “Other expense, net,” are determined on an original weighted-average cost basis.

#### *Accounts Receivable*

We deduct invoice discounts for prompt payment and fees for distribution services from our accounts receivable based on our experience that our customers will earn these discounts and fees. Our estimates for our allowance for credit losses, which has not been significant to date, is determined based on existing contractual payment terms, historical payment patterns, current economic conditions and our expectation for future economic conditions.

#### *Concentration of Credit Risk*

Financial instruments that potentially subject us to concentration of credit risk consist principally of cash equivalents and marketable securities. We place these investments with highly rated financial institutions, and, by policy, limit the amount of credit exposure to any one financial institution. We also maintain a foreign currency hedging program that includes foreign currency forward contracts with several counterparties. We have not experienced any credit losses related to these financial instruments and do not believe we are exposed to any significant credit risk related to these instruments.

We are also subject to credit risk from our accounts receivable related to our product sales and collaborators. We evaluate the creditworthiness of each of our customers and have determined that all our material customers are creditworthy. To date, we have not experienced significant losses with respect to the collection of our accounts receivable. We believe that

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

our allowances, which are not significant to our consolidated financial statements, are adequate at December 31, 2025. Please refer to Note Q, “Segment Information,” for further information.

#### *Inventories*

We value our inventories at the lower-of-cost or net realizable value. We determine the cost of our inventories, which include amounts related to materials and manufacturing overhead, on a first-in, first-out basis. We perform an assessment of the recoverability of our capitalized inventory during each reporting period and write down any excess and obsolete inventories to their net realizable value in the period in which the impairment is first identified.

We capitalize inventories prior to regulatory approval when we consider the related product candidate to have a high likelihood of regulatory approval and expect to recover the related costs. In making this determination, we evaluate, among other factors, the status of regulatory submissions and communications with regulatory authorities, information regarding the product candidate’s safety and efficacy, and the outlook for commercial sales, including the existence of any competition.

#### *Property and Equipment*

Property and equipment are recorded at cost, net of accumulated depreciation. Depreciation expense is recorded using the straight-line method over the estimated useful life of the related asset generally as follows:

Description	Estimated Useful Life
Buildings and improvements	15 to 40 years
Laboratory equipment, other equipment and furniture	7 to 10 years
Leasehold improvements; assets under finance leases	The shorter of the useful life of the assets or the estimated remaining term of the associated lease
Computers and software	3 to 5 years

Maintenance and repairs to an asset that do not improve or extend its life are expensed as incurred. When assets are retired or otherwise disposed of, the assets and related accumulated depreciation are eliminated from the accounts and any resulting gain or loss is reflected in our consolidated statements of income (loss). We perform an assessment of the fair value of the assets if indicators of impairment are identified during a reporting period and record the assets at the lower of the net book value or the fair value of the assets.

We capitalize costs incurred to develop software for internal use during the application development stage, which are depreciated over the useful life of the related asset.

#### *Goodwill*

The difference between the purchase price and the fair value of assets acquired and liabilities assumed in a business combination is allocated to goodwill. Goodwill is evaluated for impairment by reporting unit on an annual basis as of October 1, and more frequently if indicators are present or changes in circumstances suggest that impairment may exist. As noted in *Basis of Presentation* above, we have one operating segment, pharmaceuticals, which is our only reporting unit.

#### *In-process Research and Development Assets*

We record the fair value of in-process research and development assets as of the transaction date of a business combination on our consolidated balance sheets as “Other intangible assets, net.” These assets are used in research and development activities but have not yet reached technological feasibility, which occurs when we complete the research and development efforts by obtaining regulatory approval to market an underlying product candidate. We characterize in-process research and development assets on our consolidated balance sheets as indefinite-lived intangible assets until either they achieve regulatory approval and become finite-lived intangible assets, or the assets are impaired. Upon completion of the associated research and development efforts, we will determine the remaining estimated life of the marketed product and begin amortizing the carrying value of the assets over this period. If the assets become impaired or are abandoned, the carrying value is written down to fair value, and we record an impairment charge in the period in which the impairment

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

occurs. We test in-process research and development assets for impairment on an annual basis as of October 1, and more frequently if indicators are present or changes in circumstances suggest that impairment may exist.

The fair value of our in-process research and development assets is determined using either the multi-period excess earnings or the relief from royalty methods of the income approach. Each method requires us to make: (i) assumptions regarding the probability of obtaining marketing approval for a product candidate; (ii) estimates of future cash flows from potential product sales with respect to a product candidate; and (iii) appropriate discount and tax rates. The multi-period excess earnings method also requires us to estimate the timing of and the expected costs to develop and commercialize a product candidate. The relief from royalty method also requires us to estimate the after-tax royalty savings expected from ownership of a product candidate that we acquired.

#### *Finite-lived Intangible Assets*

We record finite-lived intangible assets at cost, net of accumulated amortization, on our consolidated balance sheets as “Other intangible assets, net.” Most of these assets relate to our marketed products and may include, among other things, completed research and development projects that were previously reflected on our consolidated balance sheets as in-process research and development assets, or rights to developed technology associated with in-licenses, regulatory approval milestones due to our collaborators, or other payments. We amortize our finite-lived intangible assets related to our marketed products using the straight-line method within “Cost of sales” over the remaining estimated life of the assets beginning in the period in which regulatory approval is achieved or the assets are acquired and continuing through the period that we no longer have either exclusive rights to market the products associated with the assets or in-license rights to the intellectual property underlying the assets.

We test our finite-lived intangible assets for impairment if indicators are present or changes in circumstances suggest that the carrying value of the assets may not be recoverable. If we determine that the carrying value of a finite-lived intangible asset may not be recoverable, we compare the carrying value of the asset’s group to the undiscounted cash flows that we expect the asset group to generate. When we determine that a finite-lived intangible asset has become impaired, we write down the carrying value of the asset to its fair value and record an impairment charge in the period in which the impairment occurs.

#### *Leases*

We determine whether an arrangement contains a lease at inception. If a lease is identified in an arrangement, we recognize a right-of-use asset and liability on our consolidated balance sheet and determine whether the lease should be classified as a finance or operating lease. We do not recognize assets or liabilities for leases with lease terms of less than 12 months.

A lease qualifies as a finance lease if any of the following criteria are met at the inception of the lease: (i) there is a transfer of ownership of the leased asset to Vertex by the end of the lease term, (ii) we hold an option to purchase the leased asset that we are reasonably certain to exercise, (iii) the lease term is for a major part of the remaining economic life of the leased asset, (iv) the present value of the sum of lease payments equals or exceeds substantially all of the fair value of the leased asset, or (v) the nature of the leased asset is specialized to the point that it is expected to provide the lessor no alternative use at the end of the lease term. All other leases are recorded as operating leases.

Finance and operating lease assets and liabilities are recognized at the lease commencement date based on the present value of the lease payments over the lease term using the discount rate implicit in the lease. If the rate implicit is not readily determinable, we utilize our incremental borrowing rate at the lease commencement date. Operating lease assets are further adjusted for prepaid or accrued lease payments. Operating lease payments are expensed using the straight-line method as an operating expense over the lease term. Finance lease assets are amortized to depreciation expense using the straight-line method over the shorter of the useful life of the related asset or the lease term. Finance lease payments are bifurcated into (i) a portion that is recorded as imputed interest expense and (ii) a portion that reduces the finance liability associated with the lease.

For our real estate leases, we account for lease and fixed non-lease components together as a single lease component. For our embedded leases with contract manufacturing organizations, we account for the lease component separately from the

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

non-lease components. Variable lease payments are expensed as incurred. If a lease includes an option to extend or terminate the lease, we reflect the option in the lease term if it is reasonably certain we will exercise the option.

Finance leases are recorded in “Property and equipment, net,” “Other current liabilities” and “Other long-term liabilities,” and operating leases are recorded in “Operating lease assets,” “Other current liabilities” and “Long-term operating lease liabilities” on our consolidated balance sheets.

#### *Cloud Computing Service Contracts*

We classify costs incurred to implement cloud computing service contracts as “Other assets” on our consolidated balance sheets. Amortization is recorded over the noncancellable term of the cloud computing service contract, plus any optional renewal periods that are reasonably certain to be exercised.

#### *Hedging Activities*

We recognize the fair value of our foreign currency forward contracts that are designated and qualify as hedging instruments pursuant to U.S. GAAP as either assets or liabilities on our consolidated balance sheets. Changes in the fair value of these instruments are recorded each period in “Accumulated other comprehensive (loss) income” as unrealized gains and losses until the forecasted underlying transaction occurs. Unrealized gains and losses on these foreign currency forward contracts are included in “Prepaid expenses and other current assets” or “Other assets,” and “Other current liabilities” or “Other long-term liabilities,” respectively, on our consolidated balance sheets depending on the remaining period until their contractual maturity. Realized gains and losses for the effective portion of such contracts are recognized in “Product revenues, net” in our consolidated statement of income in the same period that we recognize the product revenues that were impacted by the hedged foreign exchange rate changes. We classify the cash flows from hedging instruments in the same category as the cash flows from the hedged items.

Certain of our hedging instruments are subject to master netting arrangements to reduce the risk arising from such transactions with our counterparties. We present unrealized gains and losses on our foreign currency forward contracts on a gross basis within our consolidated balance sheets.

We also enter into foreign currency forward contracts designed to mitigate the effect of changes in foreign exchange rates on monetary assets and liabilities. Realized gains and losses for these contracts are recognized in “Other expense, net” in our consolidated statements of income (loss) each period because they are not designated as hedge instruments pursuant to U.S. GAAP.

#### *Legal Matters*

We are and may become subject to claims and legal proceedings in the ordinary course of our business activities. If we determine that it is probable that future expenditures will be made for a particular matter and such expenditures can be reasonably estimated, we accrue a loss contingency based on our best estimate of the probable range of loss. We accrue the minimum amount within the probable range of loss if no amount within the range is more likely than another. If we determine that future expenditures are not probable, or probable but not reasonably estimated, we do not accrue a loss contingency. If we determine that a material loss is reasonably possible and the range of loss can be estimated, we disclose the possible range of loss.

#### *Foreign Currency Translation and Transactions*

The majority of our operations occur in entities that have the U.S. dollar denominated as their functional currency. The assets and liabilities of our entities with functional currencies other than the U.S. dollar are translated into U.S. dollars at exchange rates in effect at the end of the year. Revenue and expense amounts for these entities are translated using the average exchange rates for the period. Changes resulting from foreign currency translation are included in “Accumulated other comprehensive (loss) income.” Net foreign currency exchange transaction losses, which are included in “Other expense, net” on our consolidated statements of income (loss), were \$13.7 million, \$27.3 million and \$24.6 million for 2025, 2024 and 2023, respectively. These net foreign currency exchange losses are presented net of the impact of the foreign currency forward contracts designed to mitigate their effect on our consolidated statements of income (loss).

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

#### *Share Repurchase Programs*

Repurchases of our common stock are recorded as reductions to “Common Stock” and “Additional paid-in capital” pursuant to our established accounting policy. Repurchases in excess of the par value will be recorded as reductions to “Retained earnings” in the event that “Additional paid-in capital” is reduced to zero.

#### *Recently Adopted Accounting Standards*

##### **Segment Reporting**

In 2023, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* (“ASU 2023-07”), which requires public entities to disclose significant segment expenses and other segment items. ASU 2023-07 also requires public entities to provide in interim periods all disclosures about a reportable segment’s profit or loss and assets that are currently required annually. ASU 2023-07 became effective for the annual period starting on January 1, 2024, and for the interim periods starting on January 1, 2025. We have disclosed significant segment expenses, other segment items, and our measure of segment profit or loss in Note Q, “Segment Information.”

##### **Income Tax Disclosures**

In 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures* (“ASU 2023-09”), which requires public entities to disclose in their rate reconciliation table additional categories of information about federal, state and foreign income taxes and to provide more details about the reconciling items in some categories if items meet a quantitative threshold. ASU 2023-09 became effective for the annual period starting on January 1, 2025. The adoption of ASU 2023-09, on a prospective basis, resulted in expansion of our income tax footnote disclosures in Note O, “Income Taxes,” including a more detailed effective tax rate reconciliation.

#### *Recently Issued Accounting Standards*

##### **Disaggregation of Income Statement Expenses**

In 2024, the FASB issued ASU 2024-03, *Income Statement—Reporting Comprehensive Income—Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses* (“ASU 2024-03”), which requires public entities, among other items, to disclose in a tabular format, on an annual and interim basis, purchases of inventory, employee compensation, depreciation, intangible asset amortization and depletion for each income statement line item that contains those expenses. ASU 2024-03 becomes effective for the annual period starting on January 1, 2027 and interim periods starting on January 1, 2028. We are in the process of analyzing the impact that the adoption of ASU 2024-03 will have on our disclosures.

##### **Internal-Use Software**

In 2025, the FASB issued ASU 2025-06, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software* (“ASU 2025-06”), which eliminates consideration of the software project development stages and replaces them with modernized recognition and measurement guidance designed to reflect current internal-use software development practices. ASU 2025-06 becomes effective for the annual and interim periods starting on January 1, 2028. We are in the process of analyzing the impact that the adoption of ASU 2025-06 will have on our consolidated financial statements and related disclosures.

## **B. Collaboration, License and Other Arrangements**

#### *Acquired In-Process Research and Development*

We have entered into numerous business development agreements with third parties to collaborate on research, development and commercialization programs, license technologies, or acquire assets. Our AIPR&D included \$133.0 million, \$4.6 billion and \$527.1 million in 2025, 2024 and 2023, respectively, related to upfront, contingent milestone, or other

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

payments pursuant to our business development transactions. In 2024, our AIPR&D included \$4.4 billion associated with our acquisition of Alpine Immune Sciences, Inc. (“Alpine”) as discussed below.

#### Asset Acquisitions

##### Alpine Immune Sciences, Inc. - povetacept

On May 20, 2024, we acquired all of the issued and outstanding shares of common stock of Alpine, a publicly traded biotechnology company focused on discovering and developing innovative, protein-based immunotherapies for approximately \$5.0 billion. We funded the Alpine acquisition with our cash and cash equivalents.

Alpine’s lead molecule, povetacept, is a dual inhibitor of B cell activating factor (“BAFF”) and a proliferation-inducing ligand (“APRIL”) pathways. As of the acquisition date, povetacept was in Phase 2 development and had shown potential best-in-class efficacy in IgAN, a serious progressive, life-threatening kidney disease that often progresses to end-stage-renal disease. Due to its mechanism of action as a dual BAFF/APRIL inhibitor, povetacept also holds the potential to benefit patients with multiple diseases, such as pMN and generalized myasthenia gravis. We accounted for the Alpine transaction as an asset acquisition because povetacept represented substantially all of the fair value of the gross assets that we acquired. As a result, \$4.4 billion of fair value attributed to povetacept was expensed to AIPR&D in 2024.

We paid total cash of \$5.0 billion at the acquisition date, which included \$4.8 billion to acquire Alpine and \$197.6 million for cash-settled unvested Alpine equity awards. The \$197.6 million represented post-acquisition expense, which was recorded as \$165.0 million of “Research and development expenses” and \$32.6 million of “Selling, general and administrative expenses.”

The total cash paid to acquire Alpine, allocation of consideration to the assets acquired and liabilities assumed and AIPR&D was as follows:

	<b>(in millions)</b>
Cash consideration to acquire Alpine’s outstanding common stock	\$ 4,536.9
Cash consideration for Alpine’s vested and unvested equity awards	420.6
Total cash consideration paid to Alpine	4,957.5
Less: Expense related to unvested equity awards	(197.6)
Transaction costs	40.7
Total consideration allocated	<u>\$ 4,800.6</u>
Cash and cash equivalents	\$ 31.9
Current marketable securities	209.5
Long-term marketable securities	48.5
Deferred tax asset	105.5
Total other assets	19.5
Total liabilities	(37.5)
Total identifiable assets acquired, net	<u>377.4</u>
Acquired in-process research and development expense	4,423.2
Total consideration allocated	<u>\$ 4,800.6</u>

#### In-license Agreements

We have entered into several in-license agreements to advance and obtain access to technologies and services related to our research and early-development activities. We are generally required to make an upfront payment upon execution of our license agreements; development, regulatory and commercialization milestones payments upon the achievement of certain

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

product research, development and commercialization objectives; and royalty payments on future sales, if any, of commercial products resulting from our collaborations.

Pursuant to the terms of our in-license agreements, our collaborators typically lead the discovery efforts and we lead all preclinical, development and commercialization activities associated with the advancement of any product candidates and fund all expenses.

We typically can terminate our in-license agreements by providing advance notice to our collaborators. Our license agreements may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, these license agreements generally remain in effect until the date on which the royalty term and all payment obligations with respect to all products in all countries have expired.

#### CRISPR Therapeutics AG

##### *CRISPR-Cas9 Gene-editing Therapies Agreements*

In 2015, we entered into a strategic collaboration, option and license agreement (the “CRISPR Agreement”) with CRISPR and its affiliates to collaborate on the discovery and development of potential new treatments aimed at the underlying genetic causes of human diseases using CRISPR-Cas9 gene-editing technology. We had the exclusive right to license certain targets. In 2019, we elected to exclusively license three targets, including CF, pursuant to the CRISPR Agreement. For each of the three targets that we elected to license, CRISPR has the potential to receive up to an additional \$410.0 million in development, regulatory and commercial milestones as well as royalties on resulting net product sales.

In 2017, we entered into a joint development and commercialization agreement with CRISPR (the “CRISPR JDCA”), which we amended and restated in 2021, pursuant to the terms of the CRISPR Agreement. Under the CRISPR JDCA, we and CRISPR were co-developing and preparing to co-commercialize CASGEVY for the treatment of hemoglobinopathies, including treatments for SCD and TDT.

Pursuant to the CRISPR JDCA, we lead global development, manufacturing and commercialization of CASGEVY, with support from CRISPR. We also conduct all research, development, manufacturing and commercialization activities relating to other product candidates and products under the CRISPR JDCA throughout the world subject to CRISPR’s reserved right to conduct certain activities.

CASGEVY was approved by the FDA in December 2023 for the treatment of SCD. In connection with this approval, we made a \$200.0 million milestone payment to CRISPR in January 2024. Please refer to Note J, “Goodwill and Other Intangible Assets,” for further information. Subsequent to receiving marketing approval for CASGEVY, we continue to lead the research and development activities under the CRISPR JDCA, subject to CRISPR’s reserved right to conduct certain activities. We are reimbursed by CRISPR for its 40% share of these research and development activities, subject to certain adjustments, and we record this reimbursement from CRISPR as a credit within “Research and development expenses.” We also share with CRISPR 40% of the net commercial profits or losses incurred with respect to CASGEVY, subject to certain adjustments, which is recorded to “Cost of sales.” The net commercial profits or losses equal the sum of the product revenues, cost of sales and selling, general and administrative expenses that we have recognized related to the CRISPR JDCA. In 2025 and 2024, we recognized net reimbursements from CRISPR pursuant to the CRISPR JDCA as credits to “Cost of sales” of \$146.8 million and \$73.5 million, respectively, related to CRISPR’s share of the CRISPR JDCA’s net commercial loss, and to “Research and development expenses” of \$62.2 million and \$31.6 million, respectively, related to CRISPR’s share of the CRISPR JDCA’s research and development activities.

During 2025, we received \$12.5 million from CRISPR, pursuant to the CRISPR JDCA, for its share of our upfront payment paid to Orna Therapeutics in December 2024, which we recorded as a credit to AIPR&D in 2025.

Prior to receiving marketing approval from the FDA for CASGEVY in December 2023, we accounted for the CRISPR JDCA as a cost-sharing arrangement, with costs incurred related to CASGEVY allocated 60% to us and 40% to CRISPR, subject to certain adjustments. In 2023, we recognized net reimbursements from CRISPR as credits to “Research and development expenses” of \$61.9 million to “Selling, general and administrative expenses” of \$32.0 million, related to CRISPR’s share of the CRISPR JDCA’s operating expenses.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

#### CRISPR-Cas9 Gene-editing Hypoimmune Cell Therapies Agreement

In 2023, we entered into a non-exclusive license agreement (the “CRISPR T1D Agreement”) for the use of CRISPR’s CRISPR-Cas9 gene-editing technology to accelerate the development of our hypoimmune cell therapies for T1D. Pursuant to the CRISPR T1D Agreement, we made a \$100.0 million upfront payment to CRISPR, and we determined that substantially all the fair value of our upfront payment was attributable to in-process research and development, for which there is no alternative future use, and that no substantive processes were acquired that would constitute a business. In the second quarter of 2023, we achieved a research milestone that resulted in a \$70.0 million payment to CRISPR. We recorded the upfront payment and the research milestone, totaling \$170.0 million, to AIPR&D in 2023. In 2024, we achieved additional research milestones totaling \$35.0 million, which were recorded to AIPR&D. CRISPR is eligible to receive up to an additional \$125.0 million in research, development, regulatory and commercial milestones, as well as royalties on resulting net product sales.

#### **Entrada Therapeutics, Inc.**

In 2023, we entered into a strategic collaboration and license agreement (the “Entrada Agreement”) with Entrada Therapeutics, Inc. (“Entrada”) focused on discovering and developing intracellular therapeutics for DM1. Upon closing, we made an upfront payment of \$225.1 million to Entrada, and purchased \$24.9 million of Entrada’s common stock in connection with the Entrada Agreement. We determined that substantially all the fair value of our upfront payment was attributable to in-process research and development, for which there was no alternative future use, and that no substantive processes were acquired that would constitute a business. In 2024 and 2023, Entrada also earned milestones of \$75.0 million and \$17.5 million, respectively. As a result, we recorded \$75.0 million and \$242.6 million in total to AIPR&D in 2024 and 2023, respectively. We recorded the investment in Entrada’s common stock at fair value on our consolidated balance sheet within “Marketable securities.” Entrada is eligible to receive up to an additional \$335.0 million in development, regulatory and commercial milestones for any products that may result from the Entrada Agreement, as well as royalties on resulting net product sales.

#### **Moderna, Inc.**

In 2016, we entered into a strategic collaboration and licensing agreement with Moderna, Inc. (“Moderna”), pursuant to which the parties are seeking to identify and develop messenger ribonucleic acid (“mRNA”) therapeutics encoding cystic fibrosis transmembrane conductance regulator for the treatment of CF. Moderna is eligible to receive up to \$270.0 million in development and regulatory milestones as well as royalties on net product sales related to this agreement.

#### *Additional In-License Agreements and Other Arrangements*

In addition to the agreements described above, we recorded upfront, option and milestone payments totaling \$145.5 million in 2025, \$95.2 million in 2024 and \$114.5 million in 2023 to AIPR&D related to additional in-license agreements and other business development transactions that we do not consider to be individually significant to our consolidated financial statements. For each of these transactions, we determined that substantially all the fair value of the consideration for each individual agreement was attributable to in-process research and development, for which we did not have any alternative future use, and no substantive processes were acquired that would constitute a business.

Please refer to Note D, “Fair Value Measurements,” and Note E, “Marketable Securities and Equity Investments,” for further information regarding our investments in our collaborators.

#### *Out-license Agreements*

We have entered into licensing agreements pursuant to which we have out-licensed rights to certain product candidates to third-party collaborators. Pursuant to these out-license agreements, our collaborators may become responsible for all costs related to the continued development of such product candidates and obtain development and commercialization rights to these product candidates, either globally or within certain geographic regions. Depending on the terms of the agreements, our collaborators may be required to make upfront payments, milestone payments upon the achievement of certain product research, development and regulatory objectives and may also be required to pay royalties on future sales, if any, of

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

commercial products resulting from the collaboration. The termination provisions associated with these collaborations are generally the same as those described above related to our in-license agreements.

#### **Zai Lab Limited**

In January 2025, we entered into an agreement with Zai for the development and commercialization of povetacept for mainland China, Hong Kong SAR, Macau SAR, Taiwan region, and Singapore. Under the agreement, Zai will help advance the povetacept clinical trials and will be responsible for obtaining marketing authorizations in the licensed territories. Zai will also be responsible for commercialization activities in the licensed territories, if povetacept becomes an approved product. Under the terms of the agreement, we received a \$10.0 million upfront payment in the first quarter of 2025, which was recorded as “Other revenues.” We are eligible to receive from Zai certain milestone payments and tiered royalties on future net sales of povetacept in the region of focus.

#### **Ono Pharmaceuticals Co., Ltd.**

In June 2025, we entered into an agreement with Ono for the development and commercialization of povetacept for Japan and South Korea. Under the agreement, Ono will help advance the povetacept clinical trials and will be responsible for obtaining marketing authorizations in Japan and South Korea. Ono will also be responsible for commercialization activities in Japan and South Korea, if povetacept becomes an approved product. Under the terms of the agreement, we received a \$20.6 million upfront payment in the second quarter of 2025, which was recorded as “Other revenues.” We are eligible to receive from Ono certain milestone payments and tiered royalties on future net sales of povetacept in Japan and South Korea.

#### *Cystic Fibrosis Foundation*

In 2004, we entered into an agreement (the “CFF Agreement”) with the Cystic Fibrosis Foundation (the “CFF”), as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc., to support research and development activities. Pursuant to the CFF Agreement, as amended, we have agreed to pay tiered royalties ranging from single digits to sub-teens on covered compounds first synthesized and/or tested during a research term on or before February 28, 2014, including ivacaftor, lumacaftor and tezacaftor, and royalties ranging from low-single digits to mid-single digits on net sales of certain compounds first synthesized and/or tested between March 1, 2014 and August 31, 2016, including elexacaftor. We do not have any royalty obligations on compounds first synthesized and tested on or after September 1, 2016. For combination products, such as ORKAMBI, SYMDEKO/SYMKEVI, TRIKAFTA/KAFTRIO, and ALYFTREK, sales are allocated equally to each of the active pharmaceutical ingredients in the combination product, and royalties are then paid for any royalty-bearing components included in the combination. We record expenses related to these royalty obligations to “Cost of sales.”

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

**C. Earnings Per Share**

The following table sets forth the computation of basic and diluted net income (loss) per common share for the periods ended:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions, except per share amounts)</b>		
Net income (loss)	\$ 3,953.2	\$ (535.6)	\$ 3,619.6
Basic weighted-average common shares outstanding	255.7	257.9	257.7
Effect of potentially dilutive securities:			
Restricted stock units (including PSUs)	1.4	—	1.6
Stock options	0.9	—	1.2
Diluted weighted-average common shares outstanding	<u>258.0</u>	<u>257.9</u>	<u>260.5</u>
Basic net income (loss) per common share	\$ 15.46	\$ (2.08)	\$ 14.05
Diluted net income (loss) per common share	\$ 15.32	\$ (2.08)	\$ 13.89

During the three years ended December 31, 2025, the number of anti-dilutive securities that were excluded from the computation of our diluted net income (loss) per common share were as follows:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
Unvested restricted stock units (including PSUs)	0.2	0.8	0.1
Stock options	—	0.4	—

**D. Fair Value Measurements**

The following fair value hierarchy is used to classify assets and liabilities based on observable inputs and unobservable inputs used to determine the fair value of our financial assets and liabilities:

- Level 1: Quoted prices in active markets for identical assets or liabilities. An active market for an asset or liability is a market in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis.
- Level 2: Observable inputs other than Level 1 inputs. Examples of Level 2 inputs include quoted prices in active markets for similar assets or liabilities and quoted prices for identical assets or liabilities in markets that are not active.
- Level 3: Unobservable inputs based on our assessment of the assumptions that market participants would use in pricing the asset or liability.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

The following table sets forth our financial assets and liabilities subject to fair value measurements by level within the fair value hierarchy:

	As of December 31, 2025				As of December 31, 2024			
	Total	Fair Value Hierarchy			Total	Fair Value Hierarchy		
		Level 1	Level 2	Level 3		Level 1	Level 2	Level 3
(in millions)								
Financial instruments carried at fair value (asset positions):								
Cash equivalents	\$ 2,779.1	\$ 1,770.7	\$ 1,008.4	\$ —	\$ 1,687.1	\$ 613.3	\$ 1,073.8	\$ —
Marketable securities:								
Corporate equity securities	16.6	16.6	—	—	36.6	36.6	—	—
U.S. Treasury securities	1,864.9	1,864.9	—	—	1,602.0	1,566.8	35.2	—
U.S. government agency securities	262.4	—	262.4	—	240.5	—	240.5	—
Asset-backed securities	1,357.0	—	1,357.0	—	1,244.2	—	1,244.2	—
Certificates of deposit	26.2	—	26.2	—	—	—	—	—
Corporate debt securities	3,693.9	—	3,693.9	—	3,525.9	—	3,525.9	—
Commercial paper	14.6	—	14.6	—	5.0	—	5.0	—
Prepaid expenses and other current assets:								
Foreign currency forward contracts	6.2	—	6.2	—	130.1	—	130.1	—
Other assets:								
Foreign currency forward contracts	12.7	—	12.7	—	12.4	—	12.4	—
<b>Total financial assets</b>	<b>\$ 10,033.6</b>	<b>\$ 3,652.2</b>	<b>\$ 6,381.4</b>	<b>\$ —</b>	<b>\$ 8,483.8</b>	<b>\$ 2,216.7</b>	<b>\$ 6,267.1</b>	<b>\$ —</b>
Financial instruments carried at fair value (liability positions):								
Other current liabilities:								
Foreign currency forward contracts	\$ (79.4)	\$ —	\$ (79.4)	\$ —	\$ —	\$ —	\$ —	\$ —
Other long-term liabilities:								
Foreign currency forward contracts	(51.0)	—	(51.0)	—	—	—	—	—
Contingent consideration	(79.0)	—	—	(79.0)	(76.9)	—	—	(76.9)
<b>Total financial liabilities</b>	<b>\$ (209.4)</b>	<b>\$ —</b>	<b>\$ (130.4)</b>	<b>\$ (79.0)</b>	<b>\$ (76.9)</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ (76.9)</b>

Please refer to Note E, “Marketable Securities and Equity Investments,” for the carrying amount and related unrealized gains (losses) by type of investment. Our cash equivalents primarily include money market funds and time deposits.

*Fair Value of Corporate Equity Securities*

We classify our investments in publicly traded corporate equity securities as “Marketable securities” on our consolidated balance sheets. Generally, our investments in the common stock of publicly traded companies are valued based on Level 1 inputs because they have readily determinable fair values.

Please refer to Note E, “Marketable Securities and Equity Investments,” for further information on these investments.

*Fair Value of Contingent Consideration*

Our Level 3 contingent consideration liabilities are related to \$678.3 million of development and regulatory milestones potentially payable to former equity holders of Exonics Therapeutics, Inc., a privately-held company we acquired in 2019. We base our estimates of the probability of achieving the milestones relevant to the fair value of contingent payments on industry data attributable to gene therapies and our knowledge of the progress and viability of the associated Duchenne muscular dystrophy programs. The discount rates used in the valuation model for contingent payments, which were between 4.1% and 4.5% as of December 31, 2025, represent a measure of credit risk and market risk associated with settling the liabilities. Significant judgment is used in determining the appropriateness of these assumptions at each reporting period.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

The following table represents a rollforward of the fair value of our contingent consideration liabilities:

	<b>Year Ended</b>
	<b>December 31, 2025</b>
	<b>(in millions)</b>
Balance at December 31, 2024	\$ 76.9
Increase in fair value of contingent payments	2.1
Balance at December 31, 2025	<u>\$ 79.0</u>

**E. Marketable Securities and Equity Investments**

A summary of our cash equivalents and marketable debt and equity securities, which are recorded at fair value, is shown below:

	As of December 31, 2025				As of December 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in millions)							
Cash equivalents	\$ 2,779.1	\$ —	\$ —	\$ 2,779.1	\$ 1,216.6	\$ —	\$ —	\$ 1,216.6
Marketable securities:								
U.S. Treasury securities	\$ 1,852.9	\$ 12.1	\$ (0.1)	\$ 1,864.9	\$ 1,926.0	\$ 11.9	\$ (0.2)	\$ 1,937.7
U.S. government agency securities	261.2	1.2	—	262.4	189.6	1.1	(0.1)	190.6
Asset-backed securities	1,351.1	6.0	(0.1)	1,357.0	1,354.6	6.6	(0.2)	1,361.0
Certificates of deposit	26.2	—	—	26.2	24.4	—	—	24.4
Corporate debt securities	3,669.3	25.0	(0.4)	3,693.9	3,513.2	25.5	(0.3)	3,538.4
Commercial paper	14.6	—	—	14.6	8.7	—	—	8.7
Total marketable available-for-sale debt securities	7,175.3	44.3	(0.6)	7,219.0	7,016.5	45.1	(0.8)	7,060.8
Corporate equity securities	25.0	—	(8.4)	16.6	25.0	—	(15.6)	9.4
Total marketable securities	7,200.3	44.3	(9.0)	7,235.6	7,041.5	45.1	(16.4)	7,070.2
Total cash equivalents and marketable securities	<u>\$ 9,979.4</u>	<u>\$ 44.3</u>	<u>\$ (9.0)</u>	<u>\$ 10,014.7</u>	<u>\$ 8,258.1</u>	<u>\$ 45.1</u>	<u>\$ (16.4)</u>	<u>\$ 8,286.8</u>

Amounts in the table above at fair value were classified on our consolidated balance sheets as follows:

	December 31,	
	2025	2024
	(in millions)	
Cash and cash equivalents	\$ 2,779.1	\$ 1,216.6
Marketable securities	1,523.3	1,347.4
Long-term marketable securities	5,712.3	5,722.8
Total	<u>\$ 10,014.7</u>	<u>\$ 8,286.8</u>

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

Marketable available-for-sale debt securities by contractual maturity were as follows:

	<b>December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Matures within one year	\$ 1,506.7	\$ 1,338.0
Matures after one year through five years	5,595.8	5,633.2
Matures after five years	116.5	89.6
Total	\$ 7,219.0	\$ 7,060.8

We did not record any allowances for credit losses to adjust the fair value of our marketable available-for-sale debt securities in 2025, 2024 or 2023. Additionally, we did not record any realized gains or losses that were material to our consolidated statements of income (loss) in 2025, 2024 or 2023. As of December 31, 2025, we held marketable available-for-sale debt securities with a total fair value of \$631.9 million that were in unrealized loss positions totaling \$0.6 million. Included in this amount were marketable available-for sale debt securities with a total fair value of \$9.7 million and total unrealized loss of \$0.1 million that had been in unrealized loss positions for greater than twelve months. We intend to hold these investments until maturity and do not expect to incur realized losses on these investments when they mature.

We record changes in the fair value of our investments in corporate equity securities to “Other expense, net” in our consolidated statements of income (loss). During the three years ended December 31, 2025, our net unrealized losses on corporate equity securities with readily determinable fair values held at the conclusion of each period were as follows:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
Net unrealized losses	\$ (11.3)	\$ (9.5)	\$ (7.5)

In 2023, we received proceeds of \$95.1 million related to the sale of the common stock of a publicly traded company, which had a total original cost basis of \$57.3 million.

As of December 31, 2025 and 2024, the carrying value of our equity investments without readily determinable fair values, which were recorded in “Other assets” on our consolidated balance sheets, were \$81.5 million and \$64.8 million, respectively. During 2024, we reduced the carrying value of our equity investments without readily determinable fair values by \$48.2 million based on observable changes in price.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

**F. Accumulated Other Comprehensive Income (Loss)**

The following table summarizes the changes in accumulated other comprehensive income (loss) (“AOCI”) by component:

	Foreign Currency Translation Adjustment	Unrealized Holding Gains (Losses), Net of Tax		Total
		On Available- For-Sale Debt Securities	On Foreign Currency Forward Contracts	
(in millions)				
<b>Balance at December 31, 2022</b>	\$ (25.0)	\$ (0.1)	\$ 25.9	\$ 0.8
Other comprehensive income (loss) before reclassifications	26.1	9.7	(27.2)	8.6
Amounts reclassified from accumulated other comprehensive income (loss)			(23.7)	(23.7)
Net current period other comprehensive income (loss)	26.1	9.7	(50.9)	(15.1)
<b>Balance at December 31, 2023</b>	<u>\$ 1.1</u>	<u>\$ 9.6</u>	<u>\$ (25.0)</u>	<u>\$ (14.3)</u>
Other comprehensive income (loss) before reclassifications	8.6	(4.4)	163.8	168.0
Amounts reclassified from accumulated other comprehensive income (loss)		1.9	(27.8)	(25.9)
Net current period other comprehensive income (loss)	8.6	(2.5)	136.0	142.1
<b>Balance at December 31, 2024</b>	<u>\$ 9.7</u>	<u>\$ 7.1</u>	<u>\$ 111.0</u>	<u>\$ 127.8</u>
Other comprehensive income (loss) before reclassifications	27.5	34.5	(255.3)	(193.3)
Amounts reclassified from accumulated other comprehensive income (loss)	—	(7.6)	57.2	49.6
Net current period other comprehensive income (loss)	27.5	26.9	(198.1)	(143.7)
<b>Balance at December 31, 2025</b>	<u>\$ 37.2</u>	<u>\$ 34.0</u>	<u>\$ (87.1)</u>	<u>\$ (15.9)</u>

**G. Hedging**

*Foreign currency forward contracts - Designated as hedging instruments*

We maintain a hedging program intended to mitigate the effect of changes in foreign exchange rates for a portion of our forecasted product revenues denominated in certain foreign currencies. The program includes foreign currency forward contracts that are designated as cash flow hedges under U.S. GAAP having contractual durations from one to 36 months. We recognize realized gains and losses for the effective portion of such contracts in “Product revenues, net” in our consolidated statements of income (loss) in the same period that we recognize the product revenues that were impacted by the hedged foreign exchange rate changes.

We formally document the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as our risk management objective and strategy for undertaking various hedging activities, which includes matching all foreign currency forward contracts that are designated as cash flow hedges to forecasted transactions. Using regression analysis, we assess, both at the hedge’s inception and on an ongoing basis, whether the foreign currency forward contracts are highly effective in offsetting changes in cash flows of hedged items on a prospective and retrospective basis. As of December 31, 2025, all hedges were determined to be highly effective.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

We consider the impact of our counterparties' credit risk on the fair value of the foreign currency forward contracts. As of December 31, 2025 and December 31, 2024, credit risk did not change the fair value of our foreign currency forward contracts.

The following table summarizes the notional amount in U.S. dollars of our outstanding foreign currency forward contracts designated as cash flow hedges under U.S. GAAP:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
<b>Foreign Currency</b>	<b>(in millions)</b>	
Euro	\$ 4,677.9	\$ 1,977.4
Canadian dollar	516.1	322.0
British pound sterling	492.6	301.7
Australian dollar	267.5	179.2
Swiss franc	126.0	79.7
Total foreign currency forward contracts	<u>\$ 6,080.1</u>	<u>\$ 2,860.0</u>

*Foreign currency forward contracts - Not designated as hedging instruments*

We enter into foreign currency forward contracts, typically with contractual maturities of approximately one month, which are designed to mitigate the effect of changes in foreign exchange rates on monetary assets and liabilities, including intercompany balances. These contracts are not designated as hedging instruments under U.S. GAAP. We recognize realized gains and losses for such contracts in "Other expense, net" in our consolidated statements of income (loss) each period. As of December 31, 2025 and 2024, the notional amount of our outstanding foreign currency forward contracts where hedge accounting under U.S. GAAP was not applied was \$612.6 million and \$367.0 million, respectively.

During the three years ended December 31, 2025, we recognized the following related to foreign currency forward contracts in our consolidated statements of income (loss):

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
<i>Designated as hedging instruments - Reclassified from AOCI</i>			
Product revenues, net	\$ (73.3)	\$ 35.7	\$ 30.2
<i>Not designated as hedging instruments</i>			
Other expense, net	\$ (34.7)	\$ 11.7	\$ 4.4
<i>Total reported in the Consolidated Statements of Income (Loss)</i>			
Product revenues, net	\$ 11,970.6	\$ 11,020.1	\$ 9,869.2
Other expense, net	\$ (7.7)	\$ (86.1)	\$ (22.8)

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

The following table summarizes the fair value of our outstanding foreign currency forward contracts designated as cash flow hedges under U.S. GAAP included on our consolidated balance sheets:

**As of December 31, 2025**

<b>Assets</b>		<b>Liabilities</b>	
<b>Classification</b>	<b>Fair Value</b>	<b>Classification</b>	<b>Fair Value</b>
<b>(in millions)</b>			
Prepaid expenses and other current assets	\$ 6.2	Other current liabilities	\$ (79.4)
Other assets	12.7	Other long-term liabilities	(51.0)
Total assets	<u>\$ 18.9</u>	Total liabilities	<u>\$ (130.4)</u>

**As of December 31, 2024**

<b>Assets</b>		<b>Liabilities</b>	
<b>Classification</b>	<b>Fair Value</b>	<b>Classification</b>	<b>Fair Value</b>
<b>(in millions)</b>			
Prepaid expenses and other current assets	\$ 130.1	Other current liabilities	\$ —
Other assets	12.4	Other long-term liabilities	—
Total assets	<u>\$ 142.5</u>	Total liabilities	<u>\$ —</u>

As of December 31, 2025, we expect the amounts that are related to foreign currency forward contracts designated as cash flow hedges under U.S. GAAP recorded in “Prepaid expenses and other current assets” and “Other current liabilities” to be reclassified to earnings within twelve months.

As discussed in “Note A, “Nature of Business and Accounting Policies,” we present the fair value of our foreign currency forward contracts on a gross basis within our consolidated balance sheets. The following table summarizes the potential effect of offsetting derivatives by type of financial instrument designated as cash flow hedges under U.S. GAAP on our consolidated balance sheets:

**As of December 31, 2025**

	<b>Gross Amounts Recognized</b>	<b>Gross Amounts Offset</b>	<b>Gross Amounts Presented</b>	<b>Gross Amounts Not Offset</b>	<b>Legal Offset</b>
<b>(in millions)</b>					
<b>Foreign currency forward contracts</b>					
Total assets	\$ 18.9	\$ —	\$ 18.9	\$ (18.9)	\$ —
Total liabilities	(130.4)	—	(130.4)	18.9	(111.5)

**As of December 31, 2024**

	<b>Gross Amounts Recognized</b>	<b>Gross Amounts Offset</b>	<b>Gross Amounts Presented</b>	<b>Gross Amounts Not Offset</b>	<b>Legal Offset</b>
<b>(in millions)</b>					
<b>Foreign currency forward contracts</b>					
Total assets	\$ 142.5	\$ —	\$ 142.5	\$ —	\$ 142.5
Total liabilities	—	—	—	—	—

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

**H. Inventories**

“Inventories” consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Raw materials	\$ 259.8	\$ 252.0
Work-in-process	1,196.9	768.8
Finished goods	230.1	184.6
Total	\$ 1,686.8	\$ 1,205.4

**I. Property and Equipment**

“Property and equipment, net” consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Buildings and improvements	\$ 483.8	\$ 461.2
Laboratory equipment, other equipment and furniture	788.1	684.5
Leasehold improvements	1,009.3	737.6
Computers and software	410.1	376.2
Land	33.1	33.1
Total property and equipment, gross	2,724.4	2,292.6
Less: accumulated depreciation	(1,204.1)	(1,064.8)
Total property and equipment, net	\$ 1,520.3	\$ 1,227.8

We recorded depreciation expense of \$155.8 million, \$160.4 million and \$167.8 million in 2025, 2024 and 2023, respectively, which includes our finance lease amortization.

**J. Goodwill and Other Intangible Assets**

*Intangible Assets*

“Other intangible assets, net” consisted of the following:

	<b>Estimated Useful lives</b>	<b>As of December 31, 2025</b>			<b>As of December 31, 2024</b>		
		<b>Gross Carrying Amount</b>	<b>Accumulated Amortization</b>	<b>Net Carrying Amount</b>	<b>Gross Carrying Amount</b>	<b>Accumulated Amortization</b>	<b>Net Carrying Amount</b>
		<b>(in millions, except useful lives)</b>					
In-process research and development	Indefinite	\$ 224.6	\$ —	\$ 224.6	\$ 603.6	\$ —	\$ 603.6
Finite-lived intangible assets - marketed products	10 to 12 years	238.0	(42.1)	195.9	238.0	(21.9)	216.1
Finite-lived intangible assets - assembled workforce	3 years	7.7	(4.0)	3.7	7.7	(1.5)	\$ 6.2
Total other intangible assets, net		\$ 470.3	\$ (46.1)	\$ 424.2	\$ 849.3	\$ (23.4)	\$ 825.9

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

In March 2025, based on results from a Phase 1/2 clinical trial evaluating our VX-264 clinical program in patients with T1D, we concluded that VX-264 will not be advancing further in clinical development. Based on this event, we performed an interim impairment test on the fair value of our VX-264 indefinite-lived in-process research and development asset that we acquired from Semma Therapeutics, Inc. in 2019. As a result, using the multi period earnings method of the income approach, we recorded a full intangible asset impairment charge of \$379.0 million in the first quarter of 2025. As of December 31, 2025, our remaining indefinite-lived in-process research and development assets were associated with our T1D program.

In 2023, we recorded a total of \$238.0 million of finite-lived intangible assets following the regulatory approval of CASGEVY in several markets, which we are amortizing on a straight-line basis over the longer of the last underlying patents to expire or the period that we have exclusive rights to market CASGEVY. We recorded intangible asset amortization expense of \$20.2 million, \$20.2 million and \$1.7 million to “Cost of sales” related to these assets in in 2025, 2024 and 2023, respectively.

As of December 31, 2025, the estimated future amortization of our finite-lived intangible assets was as follows:

Year	Estimated Amortization Expense (in millions)	
2026	\$	22.7
2027	\$	21.3
2028	\$	20.2
2029	\$	20.2
2030	\$	20.2

*Goodwill*

As of December 31, 2025 and 2024, we had goodwill of \$1.1 billion on our consolidated balance sheets.

**K. Additional Balance Sheet & Cash Flow Information**

*Cash, Cash Equivalents and Restricted Cash Presented in Consolidated Statements of Cash Flows*

The cash, cash equivalents and restricted cash balances at the beginning and ending of each period presented in our consolidated statements of cash flows consisted of the following:

	As of December 31,			
	2025	2024	2023	2022
	(in millions)			
Cash and cash equivalents	\$ 5,084.8	\$ 4,569.6	\$ 10,369.1	\$ 10,504.0
Prepaid expenses and other current assets	3.0	2.6	3.2	8.0
Cash, cash equivalents and restricted cash per consolidated statements of cash flows	<u>\$ 5,087.8</u>	<u>\$ 4,572.2</u>	<u>\$ 10,372.3</u>	<u>\$ 10,512.0</u>

Our restricted cash, if any, is included in “Prepaid expenses and other current assets” and “Other assets” on our consolidated balance sheets.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

*Additional Balance Sheet Information*

“Prepaid expenses and other current assets” consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Tax-related prepaid and receivables	\$ 634.5	\$ 357.0
Prepaid expenses	101.7	102.2
Fair value of cash flow hedges	6.2	130.1
Other	110.9	76.4
Total	\$ 853.3	\$ 665.7

As of December 31, 2025 and 2024, “Other assets” included \$66.6 million and \$62.6 million, respectively, related to costs incurred to implement cloud computing service contracts. We recorded amortization associated with cloud computing service contracts of \$31.2 million, \$25.2 million and \$11.8 million in 2025, 2024 and 2023, respectively.

“Accrued expenses” consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Product revenue accruals	\$ 1,814.1	\$ 1,618.9
Payroll and benefits	397.7	352.1
Research, development and commercial contract costs	246.9	272.7
Royalty payable	276.7	271.0
Tax related accruals	103.1	161.1
Capital related accruals	86.0	43.5
Other	46.7	69.3
Total	\$ 2,971.2	\$ 2,788.6

“Other current liabilities” consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Contract liabilities	\$ 171.8	\$ 206.8
Operating lease liabilities	77.3	87.1
Foreign currency forward contracts	79.4	—
Upfront and milestones payable	51.5	32.5
Other	48.3	36.6
Total	\$ 428.3	\$ 363.0

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

“Other long-term liabilities” consisted of the following:

	As of December 31,	
	2025	2024
	(in millions)	
Tax-related liabilities	\$ 895.4	\$ 698.6
Finance lease liabilities	106.7	112.8
Contingent consideration	79.0	76.9
Other	188.4	126.3
Total	\$ 1,269.5	\$ 1,014.6

**L. Leases**

A summary of our most significant leases, including real estate and embedded leases with contract manufacturing organizations, is as follows:

**Corporate Headquarters**

In 2011, we entered into two lease agreements, pursuant to which we lease approximately 1.1 million square feet of office and laboratory space in two buildings in Boston, Massachusetts for a term of 15 years (our “Corporate Headquarters”). In August 2024, we amended the existing lease agreements to, among other terms, extend the lease termination dates from December 2028 to June 2044 (the “Amendments”). We have the option to extend the amended leases for up to two additional ten-year periods.

The Amendments did not grant us any additional rights of use not contemplated in the existing lease agreements. As a result, we accounted for the Amendments as modifications that extended the terms of the existing leases and reassessed the classification of the leases as of their effective dates. We remeasured the lease liabilities using our incremental borrowing rate as of the effective date of the Amendments and classified the leases associated with our Corporate Headquarters as operating leases. As a result, we obtained right-of-use operating lease assets of \$847.9 million in exchange for operating lease obligations of \$1.0 billion and reduced our finance lease liabilities and property and equipment by \$275.3 million and \$107.5 million, respectively.

**Jeffrey Leiden Center for Biologics, Cell and Genetic Therapies Campus (“Leiden Campus”)**

In 2019, we entered into an agreement to lease approximately 269,000 square feet of office and laboratory space at our Leiden Campus near our corporate headquarters in Boston, Massachusetts for a term of 16 years (“Leiden I”), which is classified as an operating lease. Base rent payments commenced in 2021 and will continue through November 2036. We utilize the initial period as our lease term. We have an option to extend the lease term for up to two additional ten-year periods.

In 2024, we entered into a lease agreement for a second building (“Leiden II”) at our Leiden Campus. The Leiden II lease, which commenced in 2025, includes approximately 348,000 square feet of office and laboratory space for a term of approximately 16 years. Upon lease commencement, we recorded a right-of-use asset and corresponding lease liability, net of tenant allowances, of \$296.7 million within each of “Operating lease assets” and “Long-term operating lease liabilities” on our consolidated balance sheet. We anticipate that base rent payments will commence in the first quarter of 2027 and expect them to continue through the first quarter of 2042. We have an option to extend the Leiden II term for up to two additional ten-year periods. We utilize the initial period as our lease term.

**Lonza Portsmouth - T1D Facility**

In 2023, we entered into a strategic agreement with Lonza to support the manufacture of T1D cell therapy product candidates, pursuant to which we have partnered with Lonza to build a 130,000 square foot dedicated new facility operated by Lonza in New Hampshire. The lease commencement for the facility occurred in the first quarter of 2026. Lease payments

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

will continue through the tenth anniversary of the facility’s regulatory approval for commercial production. We will complete the lease accounting analysis for this facility in the first quarter of 2026.

Please refer to our accounting policy, *Leases*, in Note A, “Nature of Business and Accounting Policies,” for further information on the accounting treatment for our leases.

*Aggregate Lease Information*

The components of lease cost recorded in our consolidated statements of income (loss) were as follows:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
Operating lease cost	\$ 194.7	\$ 103.9	\$ 47.8
Finance lease cost			
Amortization of leased assets	7.2	30.9	42.7
Interest on lease liabilities	5.7	25.2	38.8
Variable lease cost	50.1	43.6	44.6
Sublease income	(0.2)	(1.6)	(2.7)
Net lease cost	<u>\$ 257.5</u>	<u>\$ 202.0</u>	<u>\$ 171.2</u>

Our variable lease cost during 2025, 2024 and 2023 primarily related to operating expenses, taxes and insurance associated with our real estate leases.

Our leases are included on our consolidated balance sheets as follows:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
<b>Operating leases</b>		
Operating lease assets	\$ 1,562.7	\$ 1,356.8
Total operating lease assets	<u>\$ 1,562.7</u>	<u>\$ 1,356.8</u>
Other current liabilities	\$ 77.3	\$ 87.1
Long-term operating lease liabilities	1,846.5	1,544.4
Total operating lease liabilities	<u>\$ 1,923.8</u>	<u>\$ 1,631.5</u>
<b>Finance leases</b>		
Property and equipment, net	\$ 81.5	\$ 57.9
Total finance lease assets	<u>\$ 81.5</u>	<u>\$ 57.9</u>
Other current liabilities	\$ 5.5	\$ 5.2
Other long-term liabilities	106.7	112.8
Total finance lease liabilities	<u>\$ 112.2</u>	<u>\$ 118.0</u>

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

Maturities of our finance and operating lease liabilities as of December 31, 2025 were as follows:

Year	Operating Leases	Finance Leases	Total
(in millions)			
2026	\$ 155.2	\$ 10.5	\$ 165.7
2027	196.9	11.8	208.7
2028	193.3	12.2	205.5
2029	139.9	12.5	152.4
2030	197.8	12.8	210.6
Thereafter	2,316.4	118.3	2,434.7
Total lease payments	3,199.5	178.1	3,377.6
Less: tenant allowance	(220.8)	—	(220.8)
Less: amount representing interest	(1,054.9)	(65.9)	(1,120.8)
Present value of lease liabilities	<u>\$ 1,923.8</u>	<u>\$ 112.2</u>	<u>\$ 2,036.0</u>

The weighted-average remaining lease terms and discount rates related to our leases were as follows:

	As of December 31,	
	2025	2024
Weighted-average remaining lease term (in years)		
Operating leases	15.17	15.58
Finance leases	21.94	22.17
Weighted-average discount rate		
Operating leases	4.75 %	4.61 %
Finance leases	4.51 %	4.58 %

Supplemental cash flow information related to our leases was as follows:

	Year ended December 31,		
	2025	2024	2023
(in millions)			
Cash paid for amounts included in the measurement of lease liabilities			
Operating cash flows from operating leases	\$ 183.2	\$ 113.5	\$ 62.8
Operating cash flows from finance leases	\$ 5.6	\$ 25.7	\$ 38.4
Financing cash flows from finance leases	\$ 5.4	\$ 33.6	\$ 44.9
Right-of-use assets obtained in exchange for lease obligations			
Operating leases	\$ 311.0	\$ 1,120.9	\$ 2.4

The majority of right-of-use assets obtained in exchange for lease obligations in 2025 and 2024 are described above.

**M. Common Stock, Preferred Stock and Equity Plans**

*Common Stock and Preferred Stock*

We are authorized to issue 500.0 million shares of common stock. Holders of common stock are entitled to one vote per share. Holders of common stock are entitled to receive dividends, if and when declared by our Board of Directors, and to share ratably in our assets legally available for distribution to our shareholders in the event of liquidation. Holders of common

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

stock have no preemptive, subscription, redemption or conversion rights. The holders of common stock do not have cumulative voting rights.

We are authorized to issue 1.0 million shares of preferred stock in one or more series and to fix the powers, designations, preferences and relative participating, option or other rights thereof, including dividend rights, conversion rights, voting rights, redemption terms, liquidation preferences and the number of shares constituting any series, without any further vote or action by our shareholders. As of December 31, 2025 and 2024, we had no shares of preferred stock issued or outstanding.

#### *Share Repurchase Programs*

In February 2023, our Board of Directors approved a share repurchase program (the “2023 Share Repurchase Program”), pursuant to which we were authorized to repurchase up to \$3.0 billion of our common stock. As of December 31, 2025, we had repurchased the full amount authorized under the 2023 Share Repurchase Program. In May 2025, our Board of Directors approved an additional share repurchase program (the “2025 Share Repurchase Program”), pursuant to which we are authorized to repurchase up to \$4.0 billion of our common stock.

In 2025, 2024 and 2023, we repurchased 4.8 million, 2.7 million and 1.3 million shares of our common stock, respectively, under these programs for an aggregate of \$2.0 billion, \$1.2 billion and \$427.6 million, respectively. As of December 31, 2025, we had \$3.4 billion remaining authorization under the 2025 Share Repurchase Program, which does not have an expiration date and can be discontinued at any time.

#### *Stock and Option Plans*

The purpose of each of our stock and option plans is to attract, retain and motivate our employees, consultants and directors. Awards granted under these plans can be nonstatutory stock options (“NSOs”), incentive stock options (“ISOs”), RSUs including PSUs, restricted stock (“RSs”), or other equity-based awards, as specified in the individual plans.

Shares issued under all of our plans are funded through the issuance of new shares. The following table contains information about our equity plans:

<u>Title of Plan</u>	<u>Group Eligible</u>	<u>Type of Award Granted</u>
2013 Stock and Option Plan	Employees, Non-employee Directors and Consultants	NSO, RS, RSU and PSU
2006 Stock and Option Plan	Employees, Non-employee Directors and Consultants	NSO, RS and RSU

As of December 31, 2025, we are authorized to grant 10.9 million additional awards under our 2013 Stock and Option Plan and have 4.4 million awards outstanding. We are no longer authorized to grant additional awards under our 2006 Stock and Option Plan.

#### **Restricted Stock Units (excluding PSUs)**

The following table summarizes our restricted stock unit activity during the year ended December 31, 2025:

	<u>Restricted Stock Units (excluding PSUs)</u>	
	<u>Number of Shares</u>	<u>Weighted-average Grant-date Fair Value</u>
	<u>(in thousands)</u>	<u>(per share)</u>
Unvested at December 31, 2024	2,688	\$ 372.54
Granted	1,469	\$ 456.01
Vested	(1,393)	\$ 345.20
Cancelled	(218)	\$ 425.63
Unvested at December 31, 2025	<u>2,546</u>	<u>\$ 431.34</u>

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

The total fair value of restricted stock units that vested during 2025, 2024 and 2023 (measured based on the market price of our common stock on the date of vesting) was \$654.6 million, \$666.0 million and \$433.4 million, respectively.

#### Performance-based RSUs (PSUs)

Certain members of senior management receive approximately 50% of their annual equity compensation in the form of PSUs. 50% of the number of PSUs are eligible to vest based on the achievement of one-year financial goals and the remaining PSUs are eligible to vest based on the achievement of non-financial goals, such as clinical development, regulatory and/or manufacturing-related milestones. The financial PSUs, if earned, vest in annual installments over a three-year period measured from the date of grant, and the non-financial PSUs, if earned, cliff vest at the conclusion of the performance period. The potential shares earned pursuant to these PSU awards range from 0% to 200% of the target number of shares, with the number of shares issued determined by the achievement of the financial and non-financial performance goals.

The following table summarizes our PSU activity during the year ended December 31, 2025:

	<b>Performance-Based RSU</b>	
	<b>Number of Units</b>	<b>Weighted-average Grant-date Fair Value</b>
	<b>(in thousands)</b>	<b>(per share)</b>
Unvested at December 31, 2024 (1)	862	\$ 346.01
Granted (2)	481	\$ 450.83
Vested	(584)	\$ 288.14
Cancelled	(31)	\$ 392.20
Unvested at December 31, 2025	<u>728</u>	<u>\$ 412.52</u>

(1) “Unvested” represents our PSUs at target to the extent performance has not been certified plus the actual number of shares that continue to be subject to service conditions for which the performance has been achieved and certified.

(2) “Granted” represents (i) the target number of shares issuable for grants during 2025 and (ii) any change in the number of shares issuable pursuant to outstanding PSUs based on performance certification during 2025.

The total fair value of PSUs that vested during 2025, 2024 and 2023 (measured on the date of vesting) was \$276.6 million, \$347.1 million and \$160.4 million, respectively.

#### Stock Options

All options have been granted with an exercise price equal to the fair value of the underlying common stock on the date of grant. All options awarded under our stock and option plans, cannot have an exercise price less than fair market value on the date of grant and cannot expire more than 10 years from the grant date. In each of the three years ended December 31, 2025, we only granted stock options to certain of our non-employee directors.

The following table summarizes information related to the outstanding and exercisable options during the year ended December 31, 2025:

	<b>Stock Options</b>	<b>Weighted-average Exercise Price</b>	<b>Weighted-average Remaining Contractual Life</b>	<b>Aggregate Intrinsic Value</b>
	<b>(in thousands)</b>	<b>(per share)</b>	<b>(in years)</b>	<b>(in millions)</b>
Outstanding at December 31, 2024	1,594	\$ 156.36		
Granted	7	\$ 502.97		
Exercised	<u>(447)</u>	\$ 125.30		
Outstanding at December 31, 2025	<u>1,154</u>	\$ 170.54	2.63	\$ 326.7
Exercisable at December 31, 2025	1,154	\$ 170.54	2.63	\$ 326.7

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

The aggregate intrinsic value in the table above represents the total pre-tax amount, net of exercise price, that would have been received by option holders if all option holders had exercised all options with an exercise price lower than the market price on the last business day of 2025, which was \$453.36 based on the closing price of our common stock on that date.

The total intrinsic value (the amount by which the fair market value exceeded the exercise price) of stock options exercised during 2025, 2024 and 2023 was \$147.8 million, \$112.8 million and \$128.4 million, respectively. The total cash we received as a result of stock option exercises during 2025, 2024 and 2023 was \$55.6 million, \$50.0 million and \$80.8 million, respectively.

The following table summarizes information about stock options outstanding as of December 31, 2025, which were all exercisable:

Range of Exercise Prices	Options Outstanding and Exercisable		
	Number Outstanding (in thousands)	Weighted-average Remaining Contractual Life (in years)	Weighted-average Exercise Price (per share)
\$86.52–\$100.00	177	0.74	\$ 88.39
\$100.01–\$150.00	27	1.42	\$ 125.59
\$150.01–\$200.00	848	2.60	\$ 172.79
\$200.01–\$502.97	102	6.37	\$ 305.38
Total	1,154	2.63	\$ 170.54

*Employee Stock Purchase Plan*

We have an employee stock purchase plan (the “ESPP”). The ESPP permits eligible employees to enroll in a twelve-month offering period comprising two six-month purchase periods. Participants may purchase shares of our common stock, through payroll deductions, at a price equal to 85% of the fair market value of the common stock on the first day of the applicable twelve-month offering period, or the last day of the applicable six-month purchase period, whichever is lower. Purchase dates under the ESPP occur on or about May 14 and November 14 of each year. As of December 31, 2025, there were 0.9 million shares of common stock authorized for issuance pursuant to the ESPP.

In 2025, the following shares were issued to employees under the ESPP:

	Year Ended December 31, 2025
Number of shares (in thousands)	199
Average price paid per share	\$ 362.43

*Employee Benefits*

We have a 401(k) retirement plan (the “Vertex 401(k) Plan”) in which substantially all of our permanent U.S. employees are eligible to participate. Participants may contribute up to 60% of their annual compensation to the Vertex 401(k) Plan, subject to statutory limitations. We may declare discretionary matching contributions to the Vertex 401(k) Plan. We pay matching contributions in the form of cash. In ex-U.S. markets, we have similar benefit plans. In 2025, 2024 and 2023, we recorded approximately \$63.1 million, \$52.3 million and \$43.6 million of expense related to these plans, respectively.

**N. Stock-based Compensation Expense**

We recognize share-based payments to employees as compensation expense using the fair value method. The fair value of restricted stock units, including PSUs, is based on the intrinsic value on the date of grant. The fair value of shares purchased pursuant to the ESPP and stock options is calculated using the Black-Scholes option pricing model. Stock-based compensation expense, measured at the grant date based on the fair value of the award, is typically recognized ratably over the requisite service period.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

During the three years ended December 31, 2025, we recognized the following stock-based compensation expense:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
<b>Stock-based compensation expense by type of award:</b>			
Restricted stock units (including PSUs)	\$ 672.1	\$ 689.1	\$ 563.7
ESPP share issuances	27.0	18.2	15.8
Stock options	1.2	1.8	4.0
Stock-based compensation expense related to inventories	(14.4)	(10.6)	(2.3)
Total stock-based compensation expense included in “Total costs and expenses”	<u>\$ 685.9</u>	<u>\$ 698.5</u>	<u>\$ 581.2</u>
<b>Stock-based compensation expense by line item:</b>			
Cost of sales	\$ 11.1	\$ 7.5	\$ 7.5
Research and development expenses	415.4	425.8	354.9
Selling, general and administrative expenses	259.4	265.2	218.8
Total stock-based compensation expense included in “Total costs and expenses”	685.9	698.5	581.2
Income tax effect	(128.6)	(251.6)	(167.5)
Total stock-based compensation expense, net of tax	<u>\$ 557.3</u>	<u>\$ 446.9</u>	<u>\$ 413.7</u>

We capitalize a portion of our stock-based compensation expense to inventories, all of which is attributable to employees who support the manufacturing of our products.

The following table sets forth our unrecognized stock-based compensation expense as of December 31, 2025, by type of award and the weighted-average period we expect to recognize the expense:

	<b>As of December 31, 2025</b>	
	<b>Unrecognized Expense</b>	<b>Weighted-average Recognition Period</b>
	<b>(in millions)</b>	<b>(in years)</b>
<b>Type of award:</b>		
Restricted stock units (including PSUs)	\$ 737.1	1.89
ESPP share issuances	5.2	0.46
Total unrecognized stock-based compensation expense	<u>\$ 742.3</u>	

*Restricted Stock Units and Performance-based Restricted Stock Units*

We award restricted stock units with service conditions, which are generally the vesting periods of the awards.

Our PSUs granted to certain members of senior management are described in Note M, “Common Stock, Preferred Stock and Equity Plans.” The financial-based PSUs, with a one-year performance period, are expensed ratably over their three-year vesting period. During the performance period, they are expensed based upon an assessment of the likely level of achievement. The non-financial based PSUs cliff vest at the end of their performance period, which is approximately three years. They are expensed on a straight-line basis over the same period based upon an assessment of the likely level of achievement.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

*Employee Stock Purchase Plan*

The weighted-average fair value of each purchase right granted during 2025, 2024 and 2023 was \$113.26, \$117.89 and \$90.91, respectively. The following table reflects the weighted-average assumptions used in our Black-Scholes option pricing model:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
Expected stock price volatility	31.81%	29.37%	28.52%
Risk-free interest rate	4.02%	4.63%	5.13%
Expected term (in years)	0.74	0.73	0.71
Expected annual dividends	—	—	—

*Stock Options*

We issued stock options to our non-employee directors with total grant date fair values of \$2.0 million or less in each of the three years ended December 31, 2025.

**O. Income Taxes**

We are subject to U.S. federal, state, and foreign income taxes. The components of income before provision for income taxes consisted of the following:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
United States	\$ 2,821.2	\$ (1,369.7)	\$ 3,089.1
Foreign	1,822.0	1,618.2	1,290.7
Income before provision for income taxes	<u>\$ 4,643.2</u>	<u>\$ 248.5</u>	<u>\$ 4,379.8</u>

The components of our provision for income taxes consisted of the following:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
<b>Current taxes:</b>			
Federal	\$ 679.1	\$ 704.9	\$ 900.4
State	35.9	118.2	46.2
Foreign	485.8	309.8	350.1
Total current taxes	<u>1,200.8</u>	<u>1,132.9</u>	<u>1,296.7</u>
<b>Deferred taxes:</b>			
Federal	(527.0)	(438.7)	(569.9)
State	(22.1)	(48.7)	(21.9)
Foreign	38.3	138.6	55.3
Total deferred taxes	<u>(510.8)</u>	<u>(348.8)</u>	<u>(536.5)</u>
Provision for income taxes	<u>\$ 690.0</u>	<u>\$ 784.1</u>	<u>\$ 760.2</u>

*Unremitted Earnings*

As of December 31, 2025, we do not consider a portion of the earnings of our foreign subsidiaries to be indefinitely reinvested. Upon repatriation of the non-indefinitely invested earnings in the form of distributions or otherwise, we could be

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

subject to immaterial U.S. federal withholding taxes payable to various foreign countries and income taxes in certain states. There are no material deferred taxes recorded on the excess of financial statement reporting over the tax basis of our investments in our foreign subsidiaries. Any permanently reinvested basis differences could reverse if we sell our foreign subsidiaries or various other events occur, none of which were considered probable as of December 31, 2025. The tax liabilities described above would not be material to our consolidated financial statements.

*Effective Tax Rate Reconciliation*

A reconciliation of our provision for income taxes and our effective tax rate as compared to the U.S. federal statutory rate of 21% for the year ended December 31, 2025 was as follows:

	<b>Year ended December 31, 2025</b>	
	<b>Amount</b>	<b>Percentage</b>
	<b>(in millions, except percentages)</b>	
Income before provision for income taxes	\$ 4,643.2	
Federal statutory tax rate	975.1	21.0 %
State and local income taxes, net of federal income tax effect <sup>(1)</sup>	(1.9)	— %
Foreign tax effects		
United Kingdom (“U.K.”)		
Statutory tax rate difference between U.K. and U.S.	64.7	1.4 %
Other	8.3	0.2 %
Other foreign jurisdictions	10.5	0.2 %
Effect of cross-border tax laws		
Deferred charges related to intra-entity transfers	(61.5)	(1.3)%
Foreign-derived deduction eligible income	(58.5)	(1.3)%
Subpart F income, net of credits	(97.4)	(2.1)%
Other	0.8	— %
Tax credits		
Research and development tax credits	(154.4)	(3.3)%
Nontaxable or nondeductible items		
Stock compensation (benefit), shortfalls and cancellations	(61.9)	(1.3)%
Other	25.4	0.5 %
Changes in unrecognized tax benefits	64.3	1.4 %
Other adjustments	(23.5)	(0.5)%
Provision for income taxes and effective tax rate	<u>\$ 690.0</u>	<u>14.9 %</u>

(1) The state that contributes to the majority of the state and local tax effect is New Jersey.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

A reconciliation of our provision for income taxes and our effective tax rate as compared to the U.S. federal statutory rate of 21% for the years ended December 31, 2024 and 2023 was as follows:

	<b>Year ended December 31,</b>	
	<b>2024</b>	<b>2023</b>
Federal statutory tax rate	21.0 %	21.0 %
State taxes, net of federal benefit	29.5 %	0.3 %
Foreign income tax rate differential	21.3 %	(0.6)%
U.S. tax on foreign earnings, net of credits	(12.6)%	0.7 %
Foreign derived intangible income deduction	(28.3)%	(1.7)%
Tax credits	(102.9)%	(6.0)%
Stock compensation (benefit), shortfalls and cancellations	(25.5)%	(0.8)%
Uncertain tax positions	11.3 %	3.4 %
Non-deductible AIPR&D	373.8 %	— %
Other	27.9 %	1.1 %
Effective tax rate	315.5 %	17.4 %

Our 14.9% effective tax rate for 2025 was lower than the U.S. statutory rate primarily due to research and development tax credits, increased utilization of foreign tax credits, and excess tax benefits related to stock-based compensation.

Our 315.5% effective tax rate for 2024 was materially different than the U.S. statutory rate primarily due to the \$4.4 billion of non-deductible AIPR&D resulting from our acquisition of Alpine, which significantly lowered our pre-tax income. The non-deductible AIPR&D was partially offset by a benefit from a research and development tax credit study that was completed in 2024 and excess tax benefits related to stock-based compensation.

Our 17.4% effective tax rate for 2023 was lower than the U.S. statutory rate primarily due to a benefit from a research and development tax credit study that was completed in 2023 and excess tax benefits related to stock-based compensation, partially offset by changes in uncertain tax positions.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

*Deferred Tax Assets and Liabilities*

Deferred tax assets and liabilities are determined based on the difference between financial statement and tax bases using enacted tax rates in effect for the year in which the differences are expected to reverse. The components of the deferred taxes were as follows:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
<b>Deferred tax assets:</b>		
Tax credit carryforwards	\$ 355.8	\$ 298.3
Intangible assets	651.6	769.3
Stock-based compensation	171.8	164.2
Operating lease assets	390.7	333.5
R&D capitalization	1,920.5	1,404.1
Other	233.5	192.0
Gross deferred tax assets	3,723.9	3,161.4
Valuation allowance	(326.2)	(272.9)
Total deferred tax assets	3,397.7	2,888.5
<b>Deferred tax liabilities:</b>		
Operating lease liabilities	(312.7)	(271.9)
Other	(187.1)	(285.5)
Total deferred tax liabilities	(499.8)	(557.4)
Net deferred tax assets	\$ 2,897.9	\$ 2,331.1

On a periodic basis, we reassess the valuation allowance on our deferred income tax assets, weighing positive and negative evidence to assess the recoverability of our deferred tax assets. As of December 31, 2025, we maintained a valuation allowance of \$326.2 million related to U.S. state tax attributes.

In addition to deferred tax assets and liabilities, we have recorded deferred charges related to intra-entity sales of inventory. As of December 31, 2025 and 2024, the total deferred charges were \$318.4 million and \$279.3 million, respectively.

*Tax Attributes*

As of December 31, 2025, we had the following net operating losses (“NOLs”), capital losses, and tax credit carryforwards, which if not utilized, will begin to expire in the year listed below:

	<b>As of December 31, 2025</b>	
	<b>Amount</b>	<b>Year</b>
	<b>(in millions)</b>	
Federal net operating loss carryforwards	\$ 57.3	2028
Federal capital loss carryforwards	\$ 27.6	2027
Federal research and development tax credit carryforwards	\$ 3.6	2034
State net operating loss carryforwards	\$ 450.2	2027
State research and development tax credit carryforwards	\$ 471.7	2026
Foreign net operating loss carryforwards	\$ 30.3	2041
Foreign tax credit carryforwards	\$ 15.9	2026

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

Included in the amounts above are \$84.9 million of NOLs, and \$64.6 million of credits that have unlimited carryforward periods.

Our NOLs and credits could be subject to annual limitations due to ownership change limitations provided by U.S. Internal Revenue Service (“IRS”) Code Section 382 and similar state provisions. An annual limitation could result in the expiration of NOLs and tax credit carryforward before utilization. There are limitations on the tax attributes of acquired entities however, we do not believe the limitations will have a material impact on the utilization of the NOLs or tax credits.

*Cash Paid for Income Taxes*

Cash paid for income taxes was as follows:

	<b>Year ended December 31, 2025</b>	
	<b>(in millions)</b>	
Federal	\$	968.9
State		74.2
Foreign		
United Kingdom		473.2
Other		50.4
Total cash paid for income taxes	\$	1,566.7

*Unrecognized Tax Benefits*

Unrecognized tax benefits were as follows:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
Balance at beginning of the period	\$ 706.2	\$ 615.9	\$ 459.6
Increases related to current period tax positions	119.5	119.2	116.0
Increases related to prior period tax positions	5.2	4.6	62.5
Decreases related to prior period tax positions	(5.7)	(1.9)	(14.4)
Statute of limitations expiration	(10.7)	(16.6)	(8.1)
Settlement with tax authorities	(6.3)	(14.5)	—
Changes in foreign exchange rates	43.9	(0.5)	0.3
Balance at end of period	\$ 852.1	\$ 706.2	\$ 615.9

During 2025, we increased our gross unrecognized tax benefits by \$145.9 million, primarily associated with intercompany transfer pricing matters. The unrecognized tax benefits were recorded as a \$7.4 million decrease to our gross deferred tax assets and a \$138.5 million increase to our gross tax liability.

During 2024, we increased our gross unrecognized tax benefits by \$90.3 million, primarily associated with intercompany transfer pricing matters. The unrecognized tax benefits were recorded as a \$1.6 million increase to our gross deferred tax assets and a \$91.9 million increase to our gross tax liability.

During 2023, we increased our gross unrecognized tax benefits by \$156.3 million, primarily associated with intercompany transfer pricing matters. This unrecognized tax benefit was recorded as a \$3.7 million increase to our gross deferred tax assets and a \$160.0 million increase to our gross tax liability.

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

As of December 31, 2025, we have classified \$46.2 million, and \$805.9 million of our unrecognized tax benefits as credits to “Deferred tax assets,” and “Other long-term liabilities,” respectively, on our consolidated balance sheet.

Included in our unrecognized tax benefits as of December 31, 2025, 2024 and 2023, we had \$436.6 million, \$341.4 million and \$288.7 million (net of the federal benefit on state issues), respectively, of unrecognized tax benefits, which would affect our effective income tax rate if recognized.

We recognize potential interest and penalties related to unrecognized tax benefits in our provision for income taxes. In 2025 and 2023, we recognized total net interest and penalty expenses of \$13.1 million and \$84.9 million, respectively. In 2024, we recognized total net interest and penalty credits of \$41.5 million. As of December 31, 2025 and 2024, our accrual for interest and penalties was \$95.7 million and \$82.6 million, respectively.

The IRS and other local and foreign tax authorities routinely examine our tax returns, including intercompany transfer pricing, and it is reasonably possible that we will adjust the value of our uncertain tax positions related these matters and other issues as we receive additional information from various taxing authorities, including reaching settlements with such authorities. In the case of intercompany transfer pricing, it is reasonably possible that taxing authorities do not agree with each other on the reallocation of income or the valuation of intellectual property, in which case we could be subject to double taxation, despite bilateral treaty agreements available to prevent this. In 2023, we came to settlement with the U.K.’s HM Revenue & Customs (“HMRC”) with respect to our tax positions for 2015 through 2020 and subsequently received Closure Notices for those periods in 2024. Due to the nature of the adjustments, we have asserted our rights under the U.S./U.K. Income Tax Convention pursuant to the mutual agreement procedures for the relief of double taxation for these matters.

We file U.S. federal income tax returns and income tax returns in various state, local and foreign jurisdictions. We have various income tax audits ongoing at any time throughout the world. Except for jurisdictions where we have NOLs or tax credit carryforwards, we are no longer subject to any tax assessment from tax authorities for years prior to 2014 in jurisdictions that have a material impact on our consolidated financial statements.

In December 2022, E.U. member states reached an agreement to implement the minimum tax component (“Pillar Two”) of the Organization for Economic Co-operation and Development’s (the “OECD’s”), global international tax reform initiative with effective dates of January 1, 2024 and 2025. On January 5, 2026, the OECD announced that a ‘side-by-side’ agreement was reached with member countries creating safe harbors to exempt U.S. multi-nationals from certain of the taxes under the Pillar Two regime by recognizing the U.S. tax system as a compatible domestic minimum tax regime. Our exposure to other countries’ minimum tax regimes was limited before these changes but the side-by-side agreement allows for certainty as our structure may change in the future.

In July 2025, the U.S. enacted H.R.1, which includes significant provisions modifying the U.S. tax framework, including the ability for companies to immediately deduct research and development expenditures for 2025 and provisions for deducting previously capitalized amounts. H.R.1 does not have a material impact on our 2025 U.S. taxes, but we expect further guidance to be issued. We will review guidance when issued for impacts on future years and disclose any impacts if needed at that time. These legislative changes could have an impact on our future effective tax rates, tax liabilities, and cash taxes.

#### **P. Commitments and Contingencies**

##### *2022 Credit Facility*

In July 2022, Vertex and certain of its subsidiaries entered into a \$500.0 million unsecured revolving facility (the “Credit Agreement”) with Bank of America, N.A., as administrative agent and the lenders referred to therein (the “Lenders”), which matures on July 1, 2027. The Credit Agreement was not drawn upon at closing and we have not drawn upon it to date. Amounts drawn pursuant to the Credit Agreement, if any, will be used for general corporate purposes. Subject to satisfaction of certain conditions, we may request that the borrowing capacity for the Credit Agreement be increased by an additional \$500.0 million. Additionally, the Credit Agreement provides a sublimit of \$100.0 million for letters of credit.

Any amounts borrowed under the Credit Agreement will bear interest, at our option, at either a base rate or a Secured Overnight Financing Rate (“SOFR”), in each case plus an applicable margin. Under the Credit Agreement, the applicable

## VERTEX PHARMACEUTICALS INCORPORATED

### Notes to Consolidated Financial Statements (Continued)

margins on base rate loans range from 0.000% to 0.500% and the applicable margins on SOFR loans range from 1.000% to 1.500%, in each case based on our consolidated leverage ratio (the ratio of our total consolidated funded indebtedness to our consolidated EBITDA for the most recently completed four fiscal quarter period).

Any amounts borrowed pursuant to the Credit Agreement are guaranteed by certain of our existing and future domestic subsidiaries, subject to certain exceptions.

The Credit Agreement contains customary representations and warranties and affirmative and negative covenants, including a financial covenant to maintain subject to certain limited exceptions, a consolidated leverage ratio of 3.50 to 1.00, subject to an increase to 4.00 to 1.00 following a material acquisition. As of December 31, 2025, we were in compliance with the covenants described above. The Credit Agreement also contains customary events of default. In the case of a continuing event of default, the administrative agent would be entitled to exercise various remedies, including the acceleration of amounts due under outstanding loans.

Direct costs related to the Credit Agreement are recorded over its term and are not material to our financial statements.

#### *Guaranties and Indemnifications*

As permitted under Massachusetts law, our Articles of Organization and By-laws provide that we will indemnify certain of our officers and directors for certain claims asserted against them in connection with their service as an officer or director. The maximum potential amount of future payments that we could be required to make under these indemnification provisions is unlimited. However, we have purchased directors' and officers' liability insurance policies that could reduce our monetary exposure and enable us to recover a portion of any future amounts paid. No indemnification claims currently are outstanding, and we believe the estimated fair value of these indemnification arrangements is minimal.

We customarily agree in the ordinary course of our business to indemnification provisions in agreements with clinical trial investigators and sites in our product development programs, sponsored research agreements with academic and not-for-profit institutions, various comparable agreements involving parties performing services for us, and our real estate leases. We also customarily agree to certain indemnification provisions in our drug discovery, development and commercialization collaboration agreements. With respect to our clinical trials and sponsored research agreements, these indemnification provisions typically apply to any claim asserted against the investigator or the investigator's institution relating to personal injury or property damage, violations of law or certain breaches of our contractual obligations arising out of the research or clinical testing of our compounds or product candidates. With respect to lease agreements, the indemnification provisions typically apply to claims asserted against the landlord relating to personal injury or property damage caused by us, to violations of law by us or to certain breaches of our contractual obligations. The indemnification provisions appearing in our collaboration agreements are similar to those for the other agreements discussed above, but in addition provide some limited indemnification for our collaborator in the event of third-party claims alleging infringement of intellectual property rights. In each of the cases above, the indemnification obligation generally survives the termination of the agreement for some extended period, although we believe the obligation typically has the most relevance during the contract term and for a short period of time thereafter. The maximum potential amount of future payments that we could be required to make under these provisions is generally unlimited. We have purchased insurance policies covering personal injury, property damage and general liability that reduce our exposure for indemnification and would enable us in many cases to recover all or a portion of any future amounts paid. We have never paid any material amounts to defend lawsuits or settle claims related to these indemnification provisions. Accordingly, we believe the estimated fair value of these indemnification arrangements is minimal.

#### *Legal Matters and Other Contingencies*

As described in Note B, "Collaboration, License and Other Arrangements," we have an agreement with the CFF (the "CFF Agreement") pursuant to which we owe third-party royalties payable on net sales of certain CF products, including ALYFTREK. Since inception, our ALYFTREK net product revenues total \$837.8 million. Based on the CFF Agreement, our position is that the royalty burden associated with ALYFTREK is 4%. On October 10, 2025, Royalty Pharma plc ("RP"), the third party to whom the CFF assigned its rights (and the CFF, which remains a party to the CFF Agreement), initiated a confidential arbitration alleging the royalty burden on ALYFTREK is approximately 8%. RP is seeking a declaratory judgment regarding the royalty burden on ALYFTREK as well as alleged unpaid royalties and other alleged damages

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

available under the CFF Agreement or applicable law, costs, expenses, attorneys' fees, and interest. We believe RP's position is contrary to the plain terms of the CFF Agreement and intend to vigorously defend our position under the CFF Agreement.

On a quarterly basis, we evaluate developments with claims, whether asserted or unasserted, and legal proceedings that could result in a loss contingency accrual, or an increase or decrease to a previously accrued loss contingency. There were no material loss contingencies accrued as of December 31, 2025 or 2024.

We also have certain contingent liabilities that arise in the ordinary course of our business activities. We accrue for such contingent liabilities when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. Other than our contingent consideration liabilities discussed in Note D, "Fair Value Measurements," there were no significant contingent liabilities accrued as of December 31, 2025 or 2024.

**Q. Segment Information**

Segment reporting is prepared on the same basis that our chief executive officer, who is our chief operating decision maker ("CODM"), manages the business, makes operating decisions and assesses performance. We operate in one segment, pharmaceuticals. We have selected net income (loss) as our reported measure of segment profit or loss because it is regularly provided to our CODM, allows our CODM to allocate resources because it encapsulates the results of our processes that generate revenues and expenses, and is important to the users of our financial statements. Enterprise-wide disclosures about revenues, significant customers, significant segment expenses, and property and equipment, net by location are presented below.

*Revenues by Product*

"Product revenues, net" consisted of the following:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
TRIKAFTA/KAFTRIO	\$ 10,312.7	\$ 10,238.6	\$ 8,944.7
ALYFTREK	837.8	—	—
Other product revenues	820.1	781.5	924.5
Total product revenues, net	\$ 11,970.6	\$ 11,020.1	\$ 9,869.2

In 2025, "Other product revenues" included \$115.8 million from CASGEVY and \$59.6 million from JOURNAVX. In 2024, "Other product revenues" included CASGEVY product revenues of \$10.0 million and there were no revenues from JOURNAVX. The remaining "Other product revenues" are related to KALYDECO, ORKAMBI, and SYMDEKO/SYMKEVI, our other CF products.

*Revenues by Geographic Location*

"Product revenues, net" are allocated based on the location of the customer. "Other revenues" are allocated based on the location of the Vertex entity associated with such revenues. Our "Total revenues" consisted of the following:

	<b>Year ended December 31,</b>		
	<b>2025</b>	<b>2024</b>	<b>2023</b>
	<b>(in millions)</b>		
United States	\$ 7,548.6	\$ 6,684.9	\$ 6,040.4
Outside of the United States			
Europe	3,460.3	3,453.9	3,109.0
Other	992.4	881.3	719.8
Total revenues outside of the United States	4,452.7	4,335.2	3,828.8
Total revenues	\$ 12,001.3	\$ 11,020.1	\$ 9,869.2

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

In 2025, our “Other revenues” of \$30.7 million were attributed to the U.S. We did not have any “Other revenues” in 2024 or 2023.

*Significant Customers*

Gross product revenues and net accounts receivable from each of our customers who individually accounted for 10% or more of total gross product revenues and/or 10% or more of total accounts receivable consisted of the following:

	<u>Percentage of Total Gross Product Revenues</u>			<u>Percentage of Accounts Receivable</u>	
	<u>Year Ended December 31,</u>			<u>As of December 31,</u>	
	<u>2025</u>	<u>2024</u>	<u>2023</u>	<u>2025</u>	<u>2024</u>
McKesson Corporation	22%	26%	26%	19%	17%
Accredo Health Group, Inc.	12%	11%	11%	<10%	<10%
Lloyds Pharmacy	<10%	<10%	<10%	10%	13%

*Significant Segment Expenses*

Significant segment expenses are set forth in the following table:

	<u>Year ended December 31,</u>		
	<u>2025</u>	<u>2024</u>	<u>2023</u>
	<u>(in millions)</u>		
Total revenues	\$ 12,001.3	\$ 11,020.1	\$ 9,869.2
Costs and expenses:			
Cost of sales - products	601.5	516.3	349.2
Cost of sales - royalty	1,049.8	1,014.2	913.0
Research expenses	827.9	804.5	705.6
Development expenses	3,081.6	2,825.8	2,457.3
Acquired in-process research and development expenses	133.0	4,628.4	527.1
Selling and other commercial expenses	1,102.8	838.5	592.4
General and administrative expenses	650.3	625.8	544.2
Intangible asset impairment charge	379.0	—	—
Interest income	(490.9)	(598.1)	(614.7)
Other Segment items <sup>(1)</sup>	23.1	116.2	15.3
Provision for income taxes	690.0	784.1	760.2
Net income (loss)	<u>\$ 3,953.2</u>	<u>\$ (535.6)</u>	<u>\$ 3,619.6</u>

- (1) Other segment items included in “Net income (loss)” primarily include changes in the fair value of contingent consideration, interest expense and changes in the fair value of equity investments.

**VERTEX PHARMACEUTICALS INCORPORATED**

**Notes to Consolidated Financial Statements (Continued)**

*Long-lived Assets by Location*

Long-lived assets by location consisted of the following:

	<b>As of December 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
United States	\$ 2,888.4	\$ 2,392.4
Outside of the United States		
United Kingdom	167.6	176.6
Other	27.0	15.6
Total long-lived assets outside of the United States	194.6	192.2
Total long-lived assets	\$ 3,083.0	\$ 2,584.6

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