

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

☒ QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934  
 FOR THE QUARTERLY PERIOD ENDED MARCH 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)

04-3039129

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

50 Northern Avenue, Boston, Massachusetts

(Address of principal executive offices)

02210

(Zip Code)

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

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

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, \$0.01 Par Value Per Share	VRTX	The Nasdaq Global Select Market

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

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

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

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

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

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

Indicate the number of shares outstanding of each of the issuer’s classes of common stock, as of the latest practicable date.

Common Stock, par value \$0.01 per share	256,797,187	Outstanding at April 30, 2025
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**VERTEX PHARMACEUTICALS INCORPORATED**  
**FORM 10-Q**  
**FOR THE QUARTER ENDED MARCH 31, 2025**

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

“Vertex®,” “KALYDECO®,” “ORKAMBI®,” “SYMDEKO®,” “SYMKEVI®,” “TRIKAFTA®,” “KAFTRIO®,” “CASGEVY®,” “ALYFTREK™,” and “JOURNAVX™” are registered trademarks of Vertex. Other brands, names and trademarks contained in this Quarterly Report on Form 10-Q 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, including in discussions of our cystic fibrosis, sickle cell disease, beta thalassemia, and pain development programs, we refer to our product candidates by their scientific (or generic) name or VX developmental designation.

**Part I. Financial Information**
**Item 1. Financial Statements**

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

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
Revenues:		
Product revenues, net	\$ 2,760.2	\$ 2,690.6
Other revenues	10.0	—
Total revenues	2,770.2	2,690.6
Costs and expenses:		
Cost of sales	363.0	342.6
Research and development expenses	979.7	789.1
Acquired in-process research and development expenses	19.8	76.8
Selling, general and administrative expenses	396.4	342.7
Intangible asset impairment charge	379.0	—
Change in fair value of contingent consideration	2.2	(0.1)
Total costs and expenses	2,140.1	1,551.1
Income from operations	630.1	1,139.5
Interest income	120.9	181.2
Interest expense	(3.0)	(10.4)
Other expense, net	(17.6)	(31.2)
Income before provision for income taxes	730.4	1,279.1
Provision for income taxes	84.1	179.5
Net income	\$ 646.3	\$ 1,099.6
Net income per common share:		
Basic	\$ 2.52	\$ 4.26
Diluted	\$ 2.49	\$ 4.21
Shares used in per share calculations:		
Basic	256.9	258.2
Diluted	259.5	261.1

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

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Condensed Consolidated Statements of Comprehensive Income**  
(unaudited; in millions)

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
Net income	\$ 646.3	\$ 1,099.6
Other comprehensive (loss) income:		
Unrealized holding gains (losses) on available-for-sale debt securities, net of tax of \$(4.6) and \$5.4, respectively	16.5	(19.7)
Unrealized (losses) gains on foreign currency forward contracts, net of tax of \$25.6 and \$(12.3), respectively	(90.3)	44.5
Foreign currency translation adjustment	14.1	6.8
Total other comprehensive (loss) income	(59.7)	31.6
Comprehensive income	<u>\$ 586.6</u>	<u>\$ 1,131.2</u>

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

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

	March 31, 2025	December 31, 2024
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 4,674.7	\$ 4,569.6
Marketable securities	1,526.5	1,546.3
Accounts receivable, net	1,805.1	1,609.4
Inventories	1,359.7	1,205.4
Prepaid expenses and other current assets	642.8	665.7
Total current assets	10,008.8	9,596.4
Property and equipment, net	1,295.9	1,227.8
Goodwill	1,088.0	1,088.0
Other intangible assets, net	441.2	825.9
Deferred tax assets	2,544.3	2,331.1
Operating lease assets	1,338.5	1,356.8
Long-term marketable securities	5,156.5	5,107.9
Other assets	1,007.3	999.3
Total assets	\$ 22,880.5	\$ 22,533.2
<b>Liabilities and Shareholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 445.0	\$ 413.0
Accrued expenses	2,951.8	2,788.6
Other current liabilities	386.4	363.0
Total current liabilities	3,783.2	3,564.6
Long-term operating lease liabilities	1,537.7	1,544.4
Long-term finance lease liabilities	111.4	112.8
Other long-term liabilities	951.9	901.8
Total liabilities	6,384.2	6,123.6
Commitments and contingencies (Note L)		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued	—	—
Common stock, \$0.01 par value; 500,000,000 shares authorized, 256,967,767 and 256,940,382 shares issued and outstanding, respectively	2.6	2.6
Additional paid-in capital	6,172.5	6,672.4
Accumulated other comprehensive income	68.1	127.8
Retained earnings	10,253.1	9,606.8
Total shareholders' equity	16,496.3	16,409.6
Total liabilities and shareholders' equity	\$ 22,880.5	\$ 22,533.2

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

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

	Three Months Ended					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings	Total Shareholders' Equity
	Shares	Amount				
<b>Balance at December 31, 2023</b>	257.7	\$ 2.6	\$ 7,449.7	\$ (14.3)	\$ 10,142.4	\$ 17,580.4
Other comprehensive income, net of tax	—	—	—	31.6	—	31.6
Net income	—	—	—	—	1,099.6	1,099.6
Repurchases of common stock	(0.3)	(0.0)	(140.4)	—	—	(140.4)
Common stock withheld for employee tax obligations	(0.6)	(0.0)	(233.5)	—	—	(233.5)
Issuance of common stock under benefit plans	1.5	0.0	15.9	—	—	15.9
Stock-based compensation expense	—	—	193.0	—	—	193.0
<b>Balance at March 31, 2024</b>	<u>258.3</u>	<u>\$ 2.6</u>	<u>\$ 7,284.7</u>	<u>\$ 17.3</u>	<u>\$ 11,242.0</u>	<u>\$ 18,546.6</u>
<b>Balance at December 31, 2024</b>	256.9	\$ 2.6	\$ 6,672.4	\$ 127.8	\$ 9,606.8	\$ 16,409.6
Other comprehensive loss, net of tax	—	—	—	(59.7)	—	(59.7)
Net income	—	—	—	—	646.3	646.3
Repurchases of common stock	(0.9)	(0.0)	(416.9)	—	—	(416.9)
Common stock withheld for employee tax obligations	(0.6)	(0.0)	(270.5)	—	—	(270.5)
Issuance of common stock under benefit plans	1.6	0.0	18.5	—	—	18.5
Stock-based compensation expense	—	—	169.0	—	—	169.0
<b>Balance at March 31, 2025</b>	<u>257.0</u>	<u>\$ 2.6</u>	<u>\$ 6,172.5</u>	<u>\$ 68.1</u>	<u>\$ 10,253.1</u>	<u>\$ 16,496.3</u>

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

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

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
Cash flows from operating activities:		
Net income	\$ 646.3	\$ 1,099.6
Adjustments to reconcile net income to net cash provided by operating activities:		
Stock-based compensation expense	166.1	191.9
Depreciation and amortization expense	48.4	53.5
Intangible asset impairment charge	379.0	—
Deferred income taxes	(191.6)	(158.3)
Loss on equity securities	15.0	27.0
Other non-cash items, net	24.2	(36.4)
Changes in operating assets and liabilities:		
Accounts receivable	(169.6)	(251.6)
Inventories	(167.1)	(80.1)
Prepaid expenses and other assets	(28.1)	99.2
Accounts payable	26.2	0.1
Accrued expenses	48.1	194.1
Other liabilities	22.0	167.6
Net cash provided by operating activities	818.9	1,306.6
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(1,647.4)	(2,598.5)
Sales and maturities of available-for-sale debt securities	1,637.6	710.5
Purchases of property and equipment	(40.7)	(68.4)
Net payments related to finite-lived intangible assets	—	(180.0)
Other investing activities	(5.3)	—
Net cash used in investing activities	(55.8)	(2,136.4)
Cash flows from financing activities:		
Issuances of common stock under benefit plans	16.7	16.9
Repurchases of common stock	(426.1)	(131.2)
Payments in connection with common stock withheld for employee tax obligations	(270.5)	(233.5)
Payments on finance leases	(1.3)	(13.2)
Other financing activities	0.8	3.5
Net cash used in financing activities	(680.4)	(357.5)
Effect of changes in exchange rates on cash	30.5	(15.6)
Net increase (decrease) in cash, cash equivalents and restricted cash	113.2	(1,202.9)
Cash, cash equivalents and restricted cash—beginning of period	4,572.2	10,372.3
Cash, cash equivalents and restricted cash—end of period	\$ 4,685.4	\$ 9,169.4
Supplemental disclosure of cash flow information:		
Cash paid for income taxes	\$ 184.4	\$ 34.8
Cash paid for interest	\$ 2.7	\$ 10.0

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

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Notes to Condensed Consolidated Financial Statements (unaudited)**

**A. Basis of Presentation and Accounting Policies**

*Basis of Presentation*

The accompanying condensed consolidated financial statements are unaudited and have been prepared by Vertex Pharmaceuticals Incorporated (“Vertex,” “we,” “us” or “our”) in accordance with accounting principles generally accepted in the United States of America (“U.S. GAAP”).

The condensed consolidated financial statements 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.

Certain information and footnote disclosures normally included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2024 (the “2024 Annual Report on Form 10-K”) have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of income for the interim periods ended March 31, 2025 and 2024.

The results of operations for the interim period are not necessarily indicative of the results of operations to be expected for the full fiscal year. These interim financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2024, which are contained in our 2024 Annual Report on Form 10-K.

*Use of Estimates*

The preparation of condensed 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 condensed 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.

*Recently Adopted Accounting Standards*

**Segment Reporting**

As noted in Note A, “Nature of Business and Accounting Policies,” in our 2024 Annual Report on Form 10-K, we adopted Accounting Standards Update (“ASU”) 2023-07, *Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures* (“ASU 2023-07”) for our annual period ended December 31, 2024. ASU 2023-07 requires public entities to disclose significant segment expenses and other segment items for both interim and annual periods. For interim periods, ASU 2023-07 also requires all disclosures about a reportable segment’s profit or loss and assets that were previously required annually. These disclosures are included in Note M, “Segment Information.”

*Recently Issued Accounting Standards*

**Income Tax Disclosures**

In 2023, the Financial Accounting Standards Board (“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 becomes effective for the annual period starting on January 1, 2025. We are in the process of analyzing the impact that the adoption of ASU 2023-09 will have on our income tax disclosures.

**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.



**VERTEX PHARMACEUTICALS INCORPORATED**  
**Notes to Condensed Consolidated Financial Statements (unaudited)**

*Summary of Significant Accounting Policies*

Our significant accounting policies are described in Note A, “Nature of Business and Accounting Policies,” in our 2024 Annual Report on Form 10-K.

**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 “Acquired in-process research and development expenses” (“AIPR&D”) included \$19.8 million and \$76.8 million in the three months ended March 31, 2025 and 2024, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions.

Our collaboration, licensing and asset acquisition agreements that had a significant impact on our financial statements for the three months ended March 31, 2025 and 2024 or were new or materially revised during the three months ended March 31, 2025, are described below. Additional agreements are described in Note B, “Collaboration, License and Other Arrangements,” of our 2024 Annual Report on Form 10-K.

**In-license Agreements**

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 Therapeutics AG and its affiliates (“CRISPR”) 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 cystic fibrosis (“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 severe sickle cell disease (“SCD”) and transfusion-dependent beta thalassemia.

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 U.S. Food and Drug Administration 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. 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.

During the first quarter of 2025, we received \$12.5 million from CRISPR, pursuant to the CRISPR JDCA, for its share of our upfront payment paid to Orma Therapeutics in December 2024, which we recorded as a credit to AIPR&D in the three months ended March 31, 2025.

**VERTEX PHARMACEUTICALS INCORPORATED**  
**Notes to Condensed Consolidated Financial Statements (unaudited)**

During the three months ended March 31, 2025 and 2024, the credits recognized in our condensed consolidated statements of income for CRISPR's share of CRISPR JDCA activities were as follows:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Cost of sales	\$ 36.2	\$ 15.8
Research and development expenses	\$ 16.0	\$ 11.7
Acquired in-process research and development expenses	\$ 12.5	\$ —

**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 myotonic dystrophy type 1 ("DM1"). In the first quarter of 2024, Entrada earned a \$75.0 million milestone, which we recorded to AI&D in the three months ended March 31, 2024 because we determined that substantially all the fair value of the milestone payment was attributable to in-process research and development, for which there is no alternative future use. 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.

***Out-license Agreements***

**Zai Lab Limited**

In January 2025, we entered into a collaboration agreement with Zai Lab Limited ("Zai") for the development and commercialization of povetacicept in mainland China, Hong Kong SAR, Macau SAR, Taiwan region and Singapore. Under this collaboration, Zai is responsible for the povetacicept clinical trials and regulatory submissions in the licensed territories. Zai will also be responsible for commercialization activities in the licensed territories, if povetacicept 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" in the three months ended March 31, 2025. We are eligible to receive certain regulatory milestone payments and tiered royalties on future net sales of povetacicept in the region of focus for Zai.

***Cystic Fibrosis Foundation***

In 2004, we entered into a collaboration agreement with the Cystic Fibrosis Foundation, as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc., to support research and development activities. Pursuant to the collaboration 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 Condensed Consolidated Financial Statements (unaudited)**

**C. Earnings Per Share**

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

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions, except per share amounts)</b>	
Net income	\$ 646.3	\$ 1,099.6
Basic weighted-average common shares outstanding	256.9	258.2
Effect of potentially dilutive securities:		
Restricted stock units (including performance-based restricted stock units ("PSUs"))	1.6	1.7
Stock options	1.0	1.2
Employee stock purchase program	0.0	0.0
Diluted weighted-average common shares outstanding	259.5	261.1
Basic net income per common share	\$ 2.52	\$ 4.26
Diluted net income per common share	\$ 2.49	\$ 4.21

During the three months ended March 31, 2025 and 2024, the number of anti-dilutive securities that were excluded from the computation of our diluted net income per common share was not significant.

**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 Condensed Consolidated Financial Statements (unaudited)**

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

	As of March 31, 2025				As of December 31, 2024			
	Fair Value Hierarchy				Fair Value Hierarchy			
	Total	Level 1	Level 2	Level 3	Total	Level 1	Level 2	Level 3
	(in millions)							
Financial instruments carried at fair value (asset positions):								
Cash equivalents	\$ 1,411.0	\$ 750.8	\$ 660.2	\$ —	\$ 1,687.1	\$ 613.3	\$ 1,073.8	\$ —
Marketable securities:								
Corporate equity securities	21.6	21.6	—	—	36.6	36.6	—	—
U.S. Treasury securities	1,610.4	1,610.4	—	—	1,602.0	1,566.8	35.2	—
U.S. government agency securities	218.3	—	218.3	—	240.5	—	240.5	—
Asset-backed securities	1,255.7	—	1,255.7	—	1,244.2	—	1,244.2	—
Certificates of deposit	4.0	—	4.0	—	—	—	—	—
Corporate debt securities	3,569.5	—	3,569.5	—	3,525.9	—	3,525.9	—
Commercial paper	3.5	—	3.5	—	5.0	—	5.0	—
Prepaid expenses and other current assets:								
Foreign currency forward contracts	50.5	—	50.5	—	130.1	—	130.1	—
Other assets:								
Foreign currency forward contracts	0.6	—	0.6	—	12.4	—	12.4	—
Total financial assets	<u>\$ 8,145.1</u>	<u>\$ 2,382.8</u>	<u>\$ 5,762.3</u>	<u>\$ —</u>	<u>\$ 8,483.8</u>	<u>\$ 2,216.7</u>	<u>\$ 6,267.1</u>	<u>\$ —</u>
Financial instruments carried at fair value (liability positions):								
Other current liabilities:								
Foreign currency forward contracts	\$ (18.3)	\$ —	\$ (18.3)	\$ —	\$ —	\$ —	\$ —	\$ —
Other long-term liabilities:								
Foreign currency forward contracts	(6.3)	—	(6.3)	—	—	—	—	—
Contingent consideration	(79.1)	—	—	(79.1)	(76.9)	—	—	(76.9)
Total financial liabilities	<u>\$ (103.7)</u>	<u>\$ —</u>	<u>\$ (24.6)</u>	<u>\$ (79.1)</u>	<u>\$ (76.9)</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ (76.9)</u>

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 condensed 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. However, certain of our investments in publicly traded companies have been or continue to be valued based on Level 2 inputs due to transfer restrictions associated with these investments.

As of December 31, 2024, one of our investments in publicly traded corporate equity securities with a \$14.0 million fair value was subject to a contractual sales restriction, which expired in the first quarter of 2025.

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 4.5% and 4.6% as of March 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.

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

	<b>Three Months Ended March 31, 2025</b>
	<b>(in millions)</b>
Balance at December 31, 2024	\$ 76.9
Increase in fair value of contingent payments	2.2
Balance at March 31, 2025	<u>\$ 79.1</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:

	<b>As of March 31, 2025</b>				<b>As of December 31, 2024</b>			
	<b>Amortized Cost</b>	<b>Gross Unrealized Gains</b>	<b>Gross Unrealized Losses</b>	<b>Fair Value</b>	<b>Amortized Cost</b>	<b>Gross Unrealized Gains</b>	<b>Gross Unrealized Losses</b>	<b>Fair Value</b>
	<b>(in millions)</b>							
Cash equivalents	\$ 1,411.0	\$ —	\$ —	\$ 1,411.0	\$ 1,687.1	\$ —	\$ —	\$ 1,687.1
Marketable securities:								
U.S. Treasury securities	1,602.7	8.5	(0.8)	1,610.4	1,603.9	3.6	(5.5)	1,602.0
U.S. government agency securities	217.6	0.8	(0.1)	218.3	240.5	0.5	(0.5)	240.5
Asset-backed securities	1,251.3	4.9	(0.5)	1,255.7	1,239.6	5.1	(0.5)	1,244.2
Certificates of deposit	4.0	—	—	4.0	—	—	—	—
Corporate debt securities	3,552.0	18.3	(0.8)	3,569.5	3,519.4	10.6	(4.1)	3,525.9
Commercial paper	3.5	—	—	3.5	5.0	0.0	(0.0)	5.0
Total marketable available-for-sale debt securities	6,631.1	32.5	(2.2)	6,661.4	6,608.4	19.8	(10.6)	6,617.6
Corporate equity securities	60.0	—	(38.4)	21.6	72.1	3.0	(38.5)	36.6
Total marketable securities	6,691.1	32.5	(40.6)	6,683.0	6,680.5	22.8	(49.1)	6,654.2
Total cash equivalents and marketable securities	<u>\$ 8,102.1</u>	<u>\$ 32.5</u>	<u>\$ (40.6)</u>	<u>\$ 8,094.0</u>	<u>\$ 8,367.6</u>	<u>\$ 22.8</u>	<u>\$ (49.1)</u>	<u>\$ 8,341.3</u>

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

	<b>As of March 31, 2025</b>	<b>As of December 31, 2024</b>
	<b>(in millions)</b>	
Cash and cash equivalents	\$ 1,411.0	\$ 1,687.1
Marketable securities	1,526.5	1,546.3
Long-term marketable securities	5,156.5	5,107.9
Total	<u>\$ 8,094.0</u>	<u>\$ 8,341.3</u>

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

	<b>As of March 31, 2025</b>	<b>As of December 31, 2024</b>
	<b>(in millions)</b>	
Matures within one year	\$ 1,504.9	\$ 1,509.7
Matures after one year through five years	5,050.9	5,034.4
Matures after five years	105.6	73.5
Total	<u>\$ 6,661.4</u>	<u>\$ 6,617.6</u>

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We did not record any allowances for credit losses to adjust the fair value of our marketable available-for-sale debt securities during the three months ended March 31, 2025 and 2024. Additionally, we did not record any realized gains or losses that were material to our condensed consolidated statements of income during the three months ended March 31, 2025 and 2024. As of March 31, 2025, we held marketable available-for-sale debt securities with a total fair value of \$1.1 billion that were in unrealized loss positions totaling \$2.2 million. Included in this amount were marketable available-for-sale debt securities with a total fair value of \$7.6 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 condensed consolidated statements of income. During the three months ended March 31, 2025 and 2024, our net unrealized losses on corporate equity securities with readily determinable fair values held at the conclusion of each period were as follows:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Net unrealized losses	\$ (15.0)	\$ (2.7)

As of March 31, 2025, the carrying value of our equity investments without readily determinable fair values, which are recorded in “Other assets” on our condensed consolidated balance sheets, was \$64.7 million. During the three months ended March 31, 2024, we reduced the carrying value of one of our equity investments without a readily determinable fair value by \$24.3 million based on an observable change in price.

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

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

	<b>Foreign Currency Translation Adjustment</b>	<b>Unrealized Holding Gains (Losses), Net of Tax</b>		<b>Total</b>
		<b>On Available-For- Sale Debt Securities</b>	<b>On Foreign Currency Forward Contracts</b>	
		<b>(in millions)</b>		
<b>Balance at December 31, 2024</b>	\$ 9.7	\$ 7.1	\$ 111.0	\$ 127.8
Other comprehensive income (loss) before reclassifications	14.1	18.3	(71.5)	(39.1)
Amounts reclassified from accumulated other comprehensive income (loss)	—	(1.8)	(18.8)	(20.6)
Net current period other comprehensive income (loss)	14.1	16.5	(90.3)	(59.7)
<b>Balance at March 31, 2025</b>	<u>\$ 23.8</u>	<u>\$ 23.6</u>	<u>\$ 20.7</u>	<u>\$ 68.1</u>
<b>Balance at December 31, 2023</b>	\$ 1.1	\$ 9.6	\$ (25.0)	\$ (14.3)
Other comprehensive income (loss) before reclassifications	6.8	(20.5)	47.2	33.5
Amounts reclassified from accumulated other comprehensive income (loss)	—	0.8	(2.7)	(1.9)
Net current period other comprehensive income (loss)	6.8	(19.7)	44.5	31.6
<b>Balance at March 31, 2024</b>	<u>\$ 7.9</u>	<u>\$ (10.1)</u>	<u>\$ 19.5</u>	<u>\$ 17.3</u>

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**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 condensed consolidated statements of income 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. We also formally 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. If we were to determine that a (i) foreign currency forward contract is not highly effective as a cash flow hedge, (ii) foreign currency forward contract has ceased to be a highly effective hedge or (iii) forecasted transaction is no longer probable of occurring, we would discontinue hedge accounting treatment prospectively. We measure effectiveness based on the change in fair value of the forward contracts and the fair value of the hypothetical foreign currency forward contracts with terms that match the critical terms of the risk being hedged. As of March 31, 2025, all hedges were determined to be highly effective.

We consider the impact of our counterparties’ credit risk on the fair value of the foreign currency forward contracts. As of March 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:

	As of March 31, 2025	As of December 31, 2024
Foreign Currency	(in millions)	
Euro	\$ 2,652.3	\$ 1,977.4
British pound sterling	314.5	301.7
Canadian dollar	272.2	322.0
Australian dollar	147.6	179.2
Swiss Franc	67.3	79.7
Total foreign currency forward contracts	\$ 3,453.9	\$ 2,860.0

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

We also 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 condensed consolidated statements of income each period. As of March 31, 2025 and December 31, 2024, the notional amount of our outstanding foreign currency forward contracts where hedge accounting under U.S. GAAP was not applied was \$44.2 million and \$367.0 million, respectively.

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During the three months ended March 31, 2025 and 2024, we recognized the following related to foreign currency forward contracts in our condensed consolidated statements of income:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
<i>Designated as hedging instruments - Reclassified from AOCI</i>		
Product revenues, net	\$ 24.1	\$ 3.4
<i>Not designated as hedging instruments</i>		
Other expense, net	\$ (1.2)	\$ (2.4)
<i>Total reported in the Condensed Consolidated Statements of Income</i>		
Product revenues, net	\$ 2,760.2	\$ 2,690.6
Other expense, net	\$ (17.6)	\$ (31.2)

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 condensed consolidated balance sheets:

<b>As of March 31, 2025</b>			
<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	\$ 50.5	Other current liabilities	\$ (18.3)
Other assets	0.6	Other long-term liabilities	(6.3)
Total assets	<u>\$ 51.1</u>	Total liabilities	<u>\$ (24.6)</u>

<b>As of December 31, 2024</b>			
<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 March 31, 2025, we expect the amounts that are related to foreign exchange 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.

We present the fair value of our foreign currency forward contracts on a gross basis within our condensed 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 condensed consolidated balance sheets:

	<b>As of March 31, 2025</b>				
	<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	\$ 51.1	\$ —	\$ 51.1	\$ (24.6)	\$ 26.5
Total liabilities	(24.6)	—	(24.6)	24.6	—



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	As of December 31, 2024				
	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
<b>Foreign currency forward contracts</b>	(in millions)				
Total assets	\$ 142.5	\$ —	\$ 142.5	\$ —	\$ 142.5
Total liabilities	—	—	—	—	—

#### H. Inventories

“Inventories” consisted of the following:

	As of March 31, 2025	As of December 31, 2024
	(in millions)	
Raw materials	\$ 250.5	\$ 252.0
Work-in-process	901.2	768.8
Finished goods	208.0	184.6
Total	<u>\$ 1,359.7</u>	<u>\$ 1,205.4</u>

#### I. Intangible Assets

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

		As of March 31, 2025			As of December 31, 2024		
	Estimated Useful Lives	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount	Gross Carrying Amount	Accumulated Amortization	Net Carrying Amount
		(in millions, except useful lives)					
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	(26.9)	211.1	238.0	(21.9)	216.1
Finite-lived intangible assets - assembled workforce	3 years	7.7	(2.2)	5.5	7.7	(1.5)	6.2
Total other intangible assets, net		<u>\$ 470.3</u>	<u>\$ (29.1)</u>	<u>\$ 441.2</u>	<u>\$ 849.3</u>	<u>\$ (23.4)</u>	<u>\$ 825.9</u>

In March 2025, based on results from a Phase 1/2 clinical trial evaluating our VX-264 clinical program in patients with type 1 diabetes (“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 three months ended March 31, 2025. As of March 31, 2025, our remaining indefinite-lived in-process research and development assets were associated with our T1D program.

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**J. Stock-based Compensation Expense and Share Repurchase Programs**

*Stock-based compensation expense*

During the three months ended March 31, 2025 and 2024, we recognized the following stock-based compensation expense:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Stock-based compensation expense by type of award:		
Restricted stock units (including PSUs)	\$ 163.4	\$ 187.2
ESPP share issuances	5.6	5.8
Stock-based compensation expense related to inventories	(2.9)	(1.1)
Total stock-based compensation expense included in "Total costs and expenses"	<u>\$ 166.1</u>	<u>\$ 191.9</u>
Stock-based compensation expense by line item:		
Cost of sales	\$ 2.6	\$ 1.8
Research and development expenses	100.1	119.4
Selling, general and administrative expenses	63.4	70.7
Total stock-based compensation expense included in "Total costs and expenses"	<u>166.1</u>	<u>191.9</u>
Income tax effect	<u>(75.2)</u>	<u>(79.0)</u>
Total stock-based compensation expense, net of tax	<u>\$ 90.9</u>	<u>\$ 112.9</u>

*Share repurchase program*

In February 2023, our Board of Directors approved a share repurchase program, pursuant to which we are authorized to repurchase up to \$3.0 billion of our common stock. The program does not have an expiration date and can be discontinued at any time. During the three months ended March 31, 2025 and 2024, we repurchased 0.9 million and 0.3 million shares of our common stock under the program, respectively, for aggregate repurchases of \$416.9 million and \$140.4 million, respectively. As of March 31, 2025, we had \$964.4 million remaining authorization under this program.

**K. Income Taxes**

We are subject to U.S. federal, state, and foreign income taxes. During the three months ended March 31, 2025 and 2024, we recorded the following provisions for income taxes and effective tax rates as compared to our income before provision for income taxes.

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions, except percentages)</b>	
Income before provision for income taxes	\$ 730.4	\$ 1,279.1
Provision for income taxes	\$ 84.1	\$ 179.5
Effective tax rate	11.5 %	14.0 %

Our effective tax rate for the three months ended March 31, 2025 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation.

Our effective tax rate for the three months ended March 31, 2024 was lower than the U.S. statutory rate primarily due to changes in our unrecognized tax positions as well as excess tax benefits related to stock-based compensation.

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We have reviewed the tax positions taken, or to be taken, in our tax returns for all tax years currently open to examination by a taxing authority. As of March 31, 2025 and December 31, 2024, we had \$357.4 million and \$341.4 million, respectively, of net unrecognized tax benefits, which would affect our tax rate if recognized.

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 net operating losses 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 2023, we came to settlement with the United Kingdom's HM Revenue & Customs ("HMRC") with respect to our tax positions for 2015 through 2020 and subsequently received Closure Notices for those periods during the three months ended March 31, 2024. Due to the nature of the adjustments, we are asserting 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.

In December 2022, European Union 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. In July 2023, the OECD published Administrative Guidance proposing certain safe harbors that effectively extend certain effective dates to January 1, 2027. The assessment of our potential 2025 exposure for the global per-country minimum tax of 15%, based on our forecasted 2025 results, is immaterial to our condensed consolidated financial statements as the effective tax rates in most of the jurisdictions in which we operate are above 15%.

## **L. 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 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 March 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 were 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.

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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*

We are 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. On a quarterly basis, we evaluate developments with these claims 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 March 31, 2025 or December 31, 2024.

#### *Other Contingencies*

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 material contingent liabilities accrued as of March 31, 2025 or December 31, 2024.

### **M. Segment Information**

#### *Revenues by Product*

"Product revenues, net" consisted of the following:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
TRIKAFTA/KAFTRIO	\$ 2,535.5	\$ 2,483.6
ALYFTREK	53.9	—
Other product revenues	170.8	207.0
Total product revenues, net	<u>\$ 2,760.2</u>	<u>\$ 2,690.6</u>

In the three months ended March 31, 2025, "Other product revenues" included \$14.2 million from CASGEVY, and an insignificant amount from JOURNAVX. In the three months ended March 31, 2024, there were no revenues for these

**VERTEX PHARMACEUTICALS INCORPORATED**  
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products. The remaining “Other product revenues” are related to KALYDECO, ORKAMBI, and SYMDEKO/SYMKEVI, our other CF products.

*Product Revenues by Geographic Location*

“Product revenues, net” by geographic region, based on the location of the customer, consisted of the following:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
United States	\$ 1,653.5	\$ 1,519.9
Outside of the United States		
Europe	826.6	967.4
Other	280.1	203.3
Total product revenues outside of the United States	1,106.7	1,170.7
Total product revenues, net	\$ 2,760.2	\$ 2,690.6

*Significant Segment Expenses*

Significant segment expenses are set forth in the following table:

	<b>Three Months Ended March 31,</b>	
	<b>2025</b>	<b>2024</b>
	<b>(in millions)</b>	
Total revenues	\$ 2,770.2	\$ 2,690.6
Costs and expenses:		
Cost of sales - products	130.6	108.8
Cost of sales - royalty	232.4	233.8
Research expenses	206.1	196.1
Development expenses	773.6	593.0
Acquired in-process research and development expenses	19.8	76.8
Selling and other commercial expenses	241.1	191.7
General and administrative expenses	155.3	151.0
Intangible asset impairment charge	379.0	—
Interest income	(120.9)	(181.2)
Other segment items <sup>(1)</sup>	22.8	41.5
Provision for income taxes	84.1	179.5
Net income	\$ 646.3	\$ 1,099.6

(1) Other segment items included in “Net income” primarily include changes in the fair value of contingent consideration, interest expense and changes in the fair value of equity investments.

*Additional Segment Information*

During the three months ended March 31, 2025 and 2024, we recorded total depreciation and amortization expense of \$48.4 million and \$53.5 million, respectively.

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

*Contract Liabilities*

We had contract liabilities of \$228.4 million and \$206.8 million as of March 31, 2025 and December 31, 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

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**Notes to Condensed Consolidated Financial Statements (unaudited)**

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 ship 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.

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

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

	Three Months Ended March 31,			
	2025		2024	
	Beginning of period	End of period	Beginning of period	End of period
	(in millions)			
Cash and cash equivalents	\$ 4,569.6	\$ 4,674.7	\$ 10,369.1	\$ 9,158.0
Prepaid expenses and other current assets	2.6	10.7	3.2	11.4
Cash, cash equivalents and restricted cash per condensed consolidated statement of cash flows	<u>\$ 4,572.2</u>	<u>\$ 4,685.4</u>	<u>\$ 10,372.3</u>	<u>\$ 9,169.4</u>

*Supplemental Cash Flow Information*

We obtained \$5.2 million and \$32.3 million of right-of-use operating lease assets in exchange for lease obligations during the three months ended March 31, 2025 and 2024, respectively, which represent non-cash operating activities associated with our condensed consolidated statement of cash flows.

## Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

### 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 seven approved medicines: five that treat the underlying cause of cystic fibrosis ("CF"), a life-threatening genetic disease, one that treats severe sickle cell disease ("SCD") and transfusion dependent beta thalassemia ("TDT"), life shortening inherited blood disorders, and one that treats moderate-to-severe acute pain. Our clinical-stage pipeline includes programs in CF, SCD, beta thalassemia, acute and peripheral neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy and other autoimmune renal diseases and cytopenias, type 1 diabetes, myotonic dystrophy type 1, and autosomal dominant polycystic kidney disease.

In December 2024, the U.S. Food and Drug Administration (the "FDA") approved ALYFTREK (vanzacaftor/tezacaftor/deutivacaftor), our once-daily next-in-class triple combination for the treatment of people with CF 6 years of age and older, and our fifth CF medicine. ALYFTREK is also approved in the United Kingdom (the "U.K."). Collectively, our five medicines, led by TRIKAFTA/KAFTRIO (elixacaftor/tezacaftor/ivacaftor and ivacaftor), are being used to treat nearly three quarters of the approximately 94,000 people with CF in the U.S., Europe, Australia, and Canada. Through approvals of new medicines, label expansions, and expanded reimbursement, we are focused on increasing the number of people with CF who are eligible and able to receive our medicines. In addition, we are evaluating our CF medicines in additional patient populations, including younger children, with the goal of having small molecule treatments for all people who have at least one mutation in their CFTR gene that is responsive to our CFTR modulators. We also are pursuing messenger ribonucleic acid ("mRNA") and genetic therapies for people with CF who do not make full-length CFTR protein and, as a result, cannot benefit from our current CF medicines.

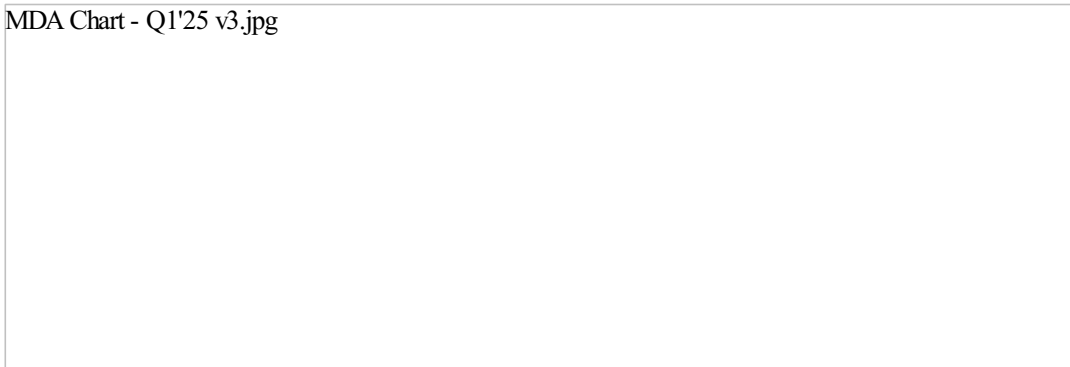
CASGEVY (exagamglogene autotemcel), our ex-vivo, non-viral CRISPR/Cas9 gene-edited cell therapy, is approved in the U.S., the European Union ("E.U."), the U.K., the Kingdom of Saudi Arabia ("Saudi Arabia"), the Kingdom of Bahrain ("Bahrain"), the United Arab Emirates (the "UAE"), Switzerland and Canada for the treatment of people 12 years of age and older with SCD or TDT. We estimate approximately 60,000 people with severe SCD or TDT are or could become eligible for CASGEVY in the U.S., Canada, Europe, Saudi Arabia and Bahrain.

In January 2025, the FDA approved JOURNAVX, our selective non-opioid NaV1.8 pain signal inhibitor, for the treatment of people with moderate-to-severe acute pain. We have begun our commercial launch of JOURNAVX in the U.S. for eligible adults. In addition, we are enrolling and dosing patients in a Phase 3 clinical trial evaluating suzetrigine for the treatment of diabetic peripheral neuropathy, a common form of peripheral neuropathic pain.

### Financial Highlights

<i>Revenues</i>	In the first quarter of 2025, our net product revenues increased to \$2.8 billion as compared to \$2.7 billion in the first quarter of 2024, primarily due to the continued performance of TRIKAFTA/KAFTRIO and initial contributions from ALYFTREK.
<i>Expenses</i>	Our total research and development ("R&D"), acquired in-process research and development ("AIPR&D"), and selling, general and administrative ("SG&A") expenses increased to \$1.4 billion in the first quarter of 2025 as compared to \$1.2 billion in the first quarter of 2024, primarily due to increased commercial investments to support the launch of JOURNAVX and continued investment to support additional therapies in mid-to-late stage development, partially offset by decreased AIPR&D. Cost of sales was 13% in each of the first quarter of 2025 and 2024.
<i>Cash</i>	Our total cash, cash equivalents and marketable securities increased to \$11.4 billion as of March 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.

MDA Chart - Q1'25 v3.jpg



*Note: Charts above may not add due to rounding.*

## Business Updates

### Marketed Products

#### Cystic Fibrosis

We expect to grow our CF business by increasing the number of people with CF who are eligible and able to receive our medicines.

Recent and anticipated progress in activities expanding our CF business is included below:

- ALYFTREK, the once-daily next-in-class combination CFTR modulator for the treatment of people with CF 6 years of age and older who have at least one F508del mutation or another responsive mutation in the CFTR gene, is approved in the U.S. and the U.K. We are working with the National Health Service in the U.K. to secure coverage for eligible patients.
- The European Medical Agency's (the "EMA") Committee for Medicinal Products for Human Use adopted a positive opinion for ALYFTREK for the treatment of people with CF 6 years of age and older who have at least one non-class I mutation in the CFTR gene. European Commission approval for ALYFTREK is expected in the second half of 2025. Regulatory submissions for ALYFTREK are under review in Canada, Switzerland, Australia and New Zealand.
- KAFTRIO is approved by the European Commission for the treatment of people with CF 2 years of age and older who have at least one non-class I mutation in the CFTR gene. With this approval, approximately 4,000 people in the European Union are newly eligible for a medicine that treats the underlying cause of their disease. We are working to ensure access for all eligible patients.

#### Sickle Cell Disease and Beta Thalassemia

- We entered into a reimbursement agreement with NHS England for eligible people with SCD to access CASGEVY, and a similar agreement in Wales for eligible people with SCD or TDT. Following a positive assessment, we also have finalized national reimbursement in Austria. In the Middle East, following regulatory approval in the UAE, we have entered into a reimbursement agreement for eligible people with SCD or TDT in the majority of emirates.
- We have activated more than 65 authorized treatment centers globally, and approximately 90 patients have had their first cell collection.
- We filed a manufacturing license submission with the FDA and we expect to begin manufacturing CASGEVY in Portsmouth, NH in the second half of 2025. This submission is part of the planned ramp of CASGEVY manufacturing capacity as demand for the therapy increases.



### Acute Pain

- In January 2025, the FDA approved JOURNAVX, for the treatment of adults with moderate-to-severe acute pain. JOURNAVX is available and stocked at pharmacies across the U.S., including major national and regional retail pharmacy chains.
- Since JOURNAVX became available in early March, more than 20,000 prescriptions have been written and filled across the hospital and retail settings in different acute pain conditions, consistent with its broad label.
- Across commercial and government payers, approximately 94 million lives have covered access to JOURNAVX, and approximately 42 million have unrestricted access (i.e., without the need for prior authorization or step edits). We have reached a formal coverage agreement with a large national Pharmacy Benefit Manager to make JOURNAVX available to their customers, representing approximately 22 million commercial lives. A total of ten state Medicaid plans are also providing unrestricted access to JOURNAVX and 20 more are currently evaluating their policies.
- More than 50 large healthcare systems have taken steps to initiate pharmacy and therapeutics (“P&T”) committee reviews of JOURNAVX, and some have already added it on formularies.
- We recently initiated two Phase 4 clinical trials in various moderate-to-severe acute pain conditions to provide additional data on the effectiveness and safety of JOURNAVX as part of real-world clinical practice, in both inpatient and outpatient settings.
- We expect JOURNAVX to be added to the list of treatments eligible for an add-on payment under the Non-Opioids Prevent Addiction in the Nation (“NOPAIN”) Act, which became effective on January 1, 2025.

### *Pipeline*

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 are enrolling and dosing patients in a Phase 3 clinical trial of TRIKAFTA/KAFTRIO in children 1 to 2 years of age and in a Phase 3 clinical trial of ALYFTREK in children 2 to 5 years of age to expand the labels and enable earlier treatment of children with CF.
- We continue to advance new oral small molecule combination therapies through preclinical and clinical development. We expect to advance the once-daily, next-generation 3.0 VX-828 combination into a clinical trial in people with CF in 2025.
- In collaboration with Moderna, Inc. (“Moderna”), we are developing VX-522, a nebulized CFTR mRNA therapy for the treatment of people with CF who do not produce full-length CFTR protein. We have implemented a temporary pause to the multiple ascending dose portion of the Phase 1/2 clinical trial of VX-522 in order to assess a tolerability issue.

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

- We have completed enrollment in two global Phase 3 clinical trials evaluating CASGEVY in children 5 to 11 years of age with SCD or TDT.
- We continue to advance preclinical assets for myeloablative conditioning agents that would have milder side-effects and could be used in connection with CASGEVY, which could broaden the eligible patient population.

### Acute Pain

- We expect to complete the Phase 2 clinical trial evaluating an oral formulation of VX-993 in the second quarter of 2025. VX-993 is a next-generation selective NaV1.8 pain signal inhibitor, for the treatment of moderate-to-severe acute pain following bunionectomy surgery. We expect to report data from this clinical trial in the second half of 2025.
- We have completed a Phase 1 clinical trial evaluating an intravenous formulation of VX-993 in healthy volunteers.

- The FDA has granted Fast Track Designation to VX-993 in moderate-to-severe acute pain in both the oral and intravenous formulations.

#### Peripheral Neuropathic Pain

- We are enrolling and dosing people with diabetic peripheral neuropathy, a common form of chronic peripheral neuropathic pain, in a Phase 3 pivotal trial evaluating suzetrigine. The FDA has granted suzetrigine Fast Track designation in peripheral neuropathic pain and Breakthrough Therapy designation in diabetic peripheral neuropathy.
- We are enrolling and dosing patients in a Phase 2 clinical trial evaluating the oral formulation of VX-993 for the treatment of diabetic peripheral neuropathy.

#### 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 are enrolling and dosing patients in the Phase 3 portion of the Phase 1/2/3 clinical trial of zimislecel in people with type 1 diabetes ("T1D") with severe hypoglycemic events and impaired awareness of hypoglycemia in the U.S., Canada, U.K. and E.U. We expect to complete enrollment and dosing in this pivotal clinical trial in the second quarter of 2025 and we expect to submit marketing applications to global regulators in 2026. We also expect to share updated clinical trial data in the second quarter of 2025.
- Zimislecel has been granted Regenerative Medicine Advanced Therapy and Fast Track designations from the FDA, Priority Medicines designation from the EMA, and has secured an Innovation Passport under the Innovative Licensing and Access Pathway from the UK Medicines and Healthcare products Regulatory Agency.
- 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.
- We are pursuing research-stage programs to evaluate additional approaches that could provide transformative benefit to people with T1D and reduce or eliminate the need for standard immunosuppressive regimens. These approaches include alternative immunosuppressives, gene editing, and novel immunoprotection to encapsulate the islet cells.

#### IgA Nephropathy, Primary Membranous Nephropathy and Other B Cell-Mediated Diseases

- We are developing povetacept, a dual antagonist of B cell activating factor ("BAFF") and a proliferation-inducing ligand ("APRIL") cytokines, as a potentially best-in-class approach to treat immunoglobulin A nephropathy ("IgAN") and primary membranous nephropathy ("pMN").
- The global Phase 3 RAINIER trial evaluating povetacept in people with IgAN has completed enrollment of the interim analysis cohort. The interim analysis will be conducted once this cohort reaches 36 weeks of treatment, with the potential to file for Accelerated Approval in the U.S. in the first half of 2026, if results are supportive. Clinical trials to support the launch of povetacept for home administration are underway.
- Based on positive results of povetacept in pMN in the RUBY-3 clinical trial, we have reached agreement with the FDA to advance povetacept into pivotal development for this disease. We expect to initiate a single, adaptive Phase 2/3 clinical trial of povetacept vs. standard-of-care in 2025, and the selected dose will be advanced to Phase 3. We expect to present updated data from the IgAN and pMN cohorts of the RUBY-3 clinical trial at upcoming medical congresses.
- We are studying additional renal diseases in the RUBY-3 basket trial and hematologic conditions in the RUBY-4 basket trial. We expect to share data and next steps from these trials in 2025.

#### APOL1-Mediated Kidney Disease

- Inaxaplin is our small molecule for the treatment of APOL1-mediated kidney disease ("AMKD"). We expect to complete enrollment in the interim analysis cohort of the Phase 3 portion of the global Phase 2/3 pivotal clinical trial ("AMPLITUDE") in the second half of 2025. We expect to conduct the pre-planned interim analysis once this cohort has been treated for 48 weeks, with potential to file for accelerated approval in the U.S. if the results are supportive.

- We continue to enroll and dose in the Phase 2 proof-of-concept clinical trial (“AMPLIFIED”) evaluating inaxaplin in people with AMKD and diabetes or other co-morbidities.

#### Myotonic Dystrophy Type 1

- Our lead approach for myotonic dystrophy type 1 (“DM1”), VX-670, was in-licensed from Entrada Therapeutics, Inc. (“Entrada”). VX-670 is an oligonucleotide connected to a cyclic peptide to promote effective delivery into cells, which holds the potential to address the underlying cause of DM1.
- We are enrolling and dosing in the multiple ascending dose portion of the global Phase 1/2 clinical trial evaluating VX-670 in people with DM1, which will evaluate both safety and efficacy.

#### Autosomal Dominant Polycystic Kidney Disease

- We have completed a Phase 1 clinical trial in healthy volunteers evaluating VX-407, our first-in-class small molecule corrector that targets the underlying cause of autosomal dominant polycystic kidney disease (“ADPKD”) in people with a subset of PKD1 variants.
- We expect to advance VX-407 into a Phase 2 proof-of-concept clinical trial in 2025 in people with ADPKD with a subset of variants in the PKD1 gene.

### **Our Business Environment**

In the first quarter of 2025, our net product revenues came 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. We are continuing to progress commercialization of CASGEVY, which has received marketing approvals in the U.S., the E.U., the U.K., Saudi Arabia, Bahrain, the UAE, Switzerland and Canada for the treatment of SCD and TDT. In addition, we have begun our commercial launch of JOURNAVX for the treatment of acute pain, which received marketing approval in the U.S. in January 2025. We also continue to advance 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 rapidly follow our first-in-class therapies that achieve proof-of-concept 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. Our foreign third-party manufacturers and suppliers may be subject to U.S. legislation, including the BIOSECURE Act, tariffs, sanctions, trade restrictions and other foreign regulatory requirements which could increase costs or reduce the supply of material available to us, or delay the procurement or supply of such material. 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 have worked successfully with third-party payors to promptly obtain appropriate levels of reimbursement for our CF medicines. In addition, we are working with U.S. government and commercial payors with respect to CASGEVY and JOURNAVX. We anticipate broad access with government and commercial payors for CASGEVY in the U.S., and we have recently entered into multiple agreements with government and commercial health insurance providers to provide such access. For JOURNAVX in the U.S., we have been working with government and commercial payors pre- and post-approval to support rapid and broad access. We plan to continue to engage in discussions with numerous commercial insurers and managed health care organizations, along with government health programs that are typically managed by authorities in the individual states, to ensure that payors recognize the significant benefits that all our therapies provide and provide patients with appropriate levels of access to our medicines and therapies now and in the future. We cannot, however, predict how changes in the law, including through the Inflation Reduction Act of 2022 and passage of state laws (e.g., transparency laws and prescription drug affordability boards), will affect our ability to negotiate successfully with third-party payors and distribute our products. Similarly, in ex-U.S. markets, we seek government reimbursement for our medicines on a country-by-country or region-by-region basis, as required. This is necessary for each new medicine, as well as for label expansions for our current medicines. We are working with ex-U.S. payors with respect to CASGEVY, and we are pursuing long-term reimbursement agreements. We have secured reimbursed access for people with SCD or TDT in Saudi Arabia, Bahrain, Luxembourg, and England. In addition, the Italian Medicines Agency has approved early access for CASGEVY, on a case-by-case basis, to treat people with SCD and TDT. 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 May 2024, we acquired Alpine Immune Sciences, Inc. (“Alpine”) for approximately \$5.0 billion in cash. Alpine’s lead molecule, 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 the second quarter of 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.

### *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 due to Entrada in the first quarter of 2024. 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.

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

In the first quarter of 2025 and 2024, our AIPR&D included \$19.8 million and \$76.8 million, 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 January 2025, we entered into a collaboration agreement with Zai Lab Limited (“Zai”) for the development and commercialization of povetacicept in mainland China, Hong Kong SAR, Macau SAR, Taiwan region and Singapore. Zai is responsible for the povetacicept clinical trials and regulatory submissions in the licensed territories. Zai will also be responsible for commercialization activities in the licensed territories, if povetacicept becomes an approved product. We are eligible to receive certain regulatory milestone payments and tiered royalties on future net sales of povetacicept in the region of focus for Zai.

## RESULTS OF OPERATIONS

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages and per share amounts)		
Total revenues	\$ 2,770.2	\$ 2,690.6	3%
Acquired in-process research and development expenses	19.8	76.8	(74)%
Intangible asset impairment charge	379.0	—	N/A
Other operating costs and expenses	1,741.3	1,474.3	18%
Income from operations	630.1	1,139.5	(45)%
Other non-operating income, net	100.3	139.6	(28)%
Provision for income taxes	84.1	179.5	(53)%
Net income	\$ 646.3	\$ 1,099.6	(41)%
Net income per diluted common share	\$ 2.49	\$ 4.21	
Diluted shares used in per share calculations	259.5	261.1	

### Total Revenues

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
TRIKAFTA/KAFTRIO	\$ 2,535.5	\$ 2,483.6	2%
ALYFTREK	53.9	—	N/A
Other product revenues	170.8	207.0	(17)%
Product revenues, net	2,760.2	2,690.6	3%
Other revenues	10.0	—	N/A
Total revenues	\$ 2,770.2	\$ 2,690.6	3%

### Product Revenues, Net

In the first quarter of 2025, our net product revenues increased by \$69.6 million, or 3%, as compared to the first quarter of 2024, primarily due to the continued performance of TRIKAFTA/KAFTRIO and initial contributions from ALYFTREK. In the first quarter of 2025, "Other product revenues" included \$14.2 million from CASGEVY, and an insignificant amount from JOURNAVX. In the first quarter of 2024, there were no revenues for these products.

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

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
United States	\$ 1,653.5	\$ 1,519.9	9%
ex-U.S.	1,106.7	1,170.7	(5)%
Product revenues, net	\$ 2,760.2	\$ 2,690.6	3%

Our net product revenues increased 9% in the U.S. due to higher net realized pricing and continued strong patient demand. Our net product revenues decreased 5% outside the U.S. primarily due to an expected decline in product revenues in Russia, where we are continuing to experience a violation of our intellectual property rights.

### Other Revenues

In the first quarter of 2025, "Other revenues" included a \$10.0 million upfront payment we received from our collaboration agreement with Zai.

### Operating Costs and Expenses

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Cost of sales	\$ 363.0	\$ 342.6	6%
Research and development expenses	979.7	789.1	24%
Acquired in-process research and development expenses	19.8	76.8	(74)%
Selling, general and administrative expenses	396.4	342.7	16%
Intangible asset impairment charge	379.0	—	N/A
Change in fair value of contingent consideration	2.2	(0.1)	**
Total costs and expenses	\$ 2,140.1	\$ 1,551.1	38%

\*\* 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 with the Cystic Fibrosis Foundation, 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.

In the first quarter of 2025, our cost of sales increased \$20.4 million, or 6%, as compared to the first quarter of 2024, primarily due to increased sales volume and changes in product mix. Our cost of sales as a percentage of our net product revenues was 13% in each of the first quarter of 2025 and 2024.

## Research and Development Expenses

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Research expenses	\$ 206.1	\$ 196.1	5%
Development expenses	773.6	593.0	30%
Total research and development expenses	\$ 979.7	\$ 789.1	24%

Our research and development expenses include internal and external costs incurred for research and development of our products and product candidates. We do not assign our internal costs, such as salary and benefits, stock-based compensation expense, laboratory supplies and other direct expenses and infrastructure costs, to individual products or product candidates, because the employees within our research and development groups typically are deployed across multiple research and development programs. We assign external costs of services provided to us by clinical research organizations and other outsourced research by individual program. Our internal costs are greater than our external costs. All research and development costs for our products and product candidates are expensed as incurred.

Since January 2023, we have incurred approximately \$7.8 billion in research and development expenses associated with product discovery and development. The successful development of our product candidates is highly uncertain and subject to a number of risks. In addition, the duration of clinical trials may vary substantially according to the type, complexity and novelty of the product candidate and the disease indication being targeted. The FDA and comparable agencies in foreign countries impose substantial requirements on the introduction of therapeutic pharmaceutical products, typically requiring lengthy and detailed laboratory and clinical testing procedures, sampling activities and other costly and time-consuming procedures. Data obtained from nonclinical and clinical activities at any step in the testing process may be adverse and lead to discontinuation or redirection of development activities. Data obtained from these activities also are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The duration and cost of discovery, nonclinical studies and clinical trials may vary significantly over the life of a project and are difficult to predict. Therefore, accurate and meaningful estimates of the ultimate costs to bring our product candidates to market are not available.

Any estimates regarding development and regulatory timelines for our product candidates are highly subjective and subject to change. Until we have data from Phase 3 clinical trials, we cannot make a meaningful estimate regarding when, or if, a clinical development program will generate revenues and cash flows.

### Research Expenses

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Research Expenses:			
Salary and benefits	\$ 53.1	\$ 53.0	0%
Stock-based compensation expense	22.3	29.9	(25)%
Outsourced services and other direct expenses	73.1	64.4	14%
Infrastructure costs	57.6	48.8	18%
Total research expenses	\$ 206.1	\$ 196.1	5%

Our research expenses have been increasing over the last several years as we invested in our pipeline and expanded our cell and genetic therapy capabilities, which resulted in increased outsourced services and other direct expenses and infrastructure costs in the first quarter of 2025 as compared to the first quarter of 2024. We expect to continue to invest in our research programs with a focus on creating transformative medicines for serious diseases.

#### *Development Expenses*

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Development Expenses:			
Salary and benefits	\$ 195.9	\$ 170.1	15%
Stock-based compensation expense	77.8	89.5	(13)%
Outsourced services and other direct expenses	379.7	236.1	61%
Infrastructure costs	120.2	97.3	24%
Total development expenses	\$ 773.6	\$ 593.0	30%

Our development expenses increased by \$180.6 million, or 30%, in the first quarter of 2025 as compared to the first quarter of 2024, due to continued investments in internal headcount and infrastructure to support multiple mid- and late-stage clinical development programs, driving increases in our outsourced services and other direct expenses. Our outsourced services and other direct expenses increased as compared to the first quarter of 2024 primarily due to the acquisition of our povetacicept program in the second quarter of 2024, expansion of our T1D study of VX-880, and next-generation pain product candidates.

Our stock-based compensation expenses, including those recorded as research and development expenses, have historically fluctuated and is 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**

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Acquired in-process research and development expenses	\$ 19.8	\$ 76.8	(74)%

AIPR&D in the first quarter of 2025 was primarily related to milestone payments. AIPR&D in the first quarter of 2024 was primarily related to the \$75.0 million milestone to Entrada. 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**

	Three Months Ended March 31,		
	2025	2024	Change
	(in millions, except percentages)		
Selling, general and administrative expenses	\$ 396.4	\$ 342.7	16%

Selling, general and administrative expenses increased by 16% in the first quarter of 2025 as compared to the first quarter of 2024 primarily due to increased commercial investment to support the launch of JOURNAVX.

#### **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. in 2019. As a result, we recorded a full intangible asset impairment charge of \$379.0 million associated with VX-264.

#### **Contingent Consideration**

The fair value of our contingent consideration increased by \$2.2 million in the first quarter of 2025 and decreased by \$0.1 million in the first quarter of 2024.

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

##### **Interest Income**

Interest income decreased from \$181.2 million in the first quarter of 2024 to \$120.9 million in the first quarter of 2025, primarily due to decreased cash equivalents and available-for-sale debt securities following our acquisition of Alpine in the second quarter of 2024 and decreased market interest rates compared to the first quarter of 2024. Our future interest income is dependent on the amount of, and prevailing market interest rates on, our outstanding cash equivalents and available-for-sale debt securities.

##### **Interest Expense**

Interest expense was \$3.0 million and \$10.4 million in the first quarter of 2025 and 2024, respectively. The majority of our interest expense in these periods was related to imputed interest expense associated with our finance leases, including interest expense imputed on our corporate headquarters leases in Boston in the first quarter of 2024. Our corporate headquarters leases were amended in the third quarter of 2024 resulting in a classification change from finance to operating leases, which are recorded entirely within operating expenses in our condensed consolidated statements of operations.

##### **Other Income (Expense), Net**

Other income (expense), net were expenses of \$17.6 million and \$31.2 million in the first quarter of 2025 and 2024, respectively. These amounts primarily related to net unrealized losses resulting from changes in the fair value of certain of our strategic equity investments, which consist of investments in our collaborators that may be public or privately-held companies. To the extent that we continue to hold strategic equity investments in publicly traded biotechnology companies, we expect that our other income (expense), net will continue to fluctuate in future periods due to the volatility in the stock prices of these companies that impacts the fair value of our investments. As of March 31, 2025, the fair value of our investments in publicly traded companies was \$21.6 million.

#### ***Income Taxes***

Our effective tax rate fluctuates from period to period 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.

We recorded provisions for income taxes of \$84.1 million and \$179.5 million in the first quarter of 2025 and 2024, respectively. Our effective tax rate of 11.5% in the first quarter of 2025 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation. Our effective tax rate of 14.0% in the first quarter of 2024 was lower than the U.S. statutory rate primarily due to changes in our unrecognized tax positions as well as excess tax benefits related to stock-based compensation.

## LIQUIDITY AND CAPITAL RESOURCES

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

	As of March 31, 2025	As of December 31, 2024	Change
(in millions, except percentages)			
Cash, cash equivalents and marketable securities:			
Cash and cash equivalents	\$ 4,674.7	\$ 4,569.6	
Marketable securities	1,526.5	1,546.3	
Long-term marketable securities	5,156.5	5,107.9	
Total cash, cash equivalents and marketable securities	<u>\$ 11,357.7</u>	<u>\$ 11,223.8</u>	1%
Working Capital:			
Total current assets	\$ 10,008.8	\$ 9,596.4	4%
Total current liabilities	(3,783.2)	(3,564.6)	6%
Total working capital	<u>\$ 6,225.6</u>	<u>\$ 6,031.8</u>	3%

### Working Capital

As of March 31, 2025, total working capital was \$6.2 billion, which represented an increase of \$193.8 million, or 3%, compared to December 31, 2024, primarily due to increased cash and cash equivalents resulting from our operations and increased inventories following the regulatory approvals of ALYFTREK and JOURNA VX.

### Cash Flows

	Three Months Ended March 31,	
	2025	2024
(in millions)		
Net cash provided by (used in):		
Operating activities	\$ 818.9	\$ 1,306.6
Investing activities	\$ (55.8)	\$ (2,136.4)
Financing activities	\$ (680.4)	\$ (357.5)

### Operating Activities

Cash provided by operating activities decreased from \$1.3 billion in the first quarter of 2024 to \$818.9 million in the first quarter of 2025, primarily due to the timing of tax payments and accruals related to our product revenue contracts.

### Investing Activities

Cash used in investing activities was \$55.8 million in the first quarter of 2025, primarily related to purchases of property and equipment. Cash used in investing activities was \$2.1 billion in the first quarter of 2024. The largest portion of our investing activities in the first quarter of 2024 were net purchases of available-for-sale debt securities.

### Financing Activities

Cash used in financing activities were \$680.4 million and \$357.5 million in the first quarter of 2025 and 2024, respectively. Our financing activities in each of these periods were primarily related to repurchases of our common stock pursuant to our share repurchase program and payments related to our employee stock benefit plans.

### 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 March 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.
- 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 March 31, 2025, we had \$964.4 million remaining authorization available under our \$3.0 billion Share Repurchase Program that our Board of Directors approved in February 2023. This program does not have an expiration date and can be discontinued at any time. We expect to fund these programs through a combination of cash on hand and cash generated by operations.

There have not been any material changes to our future capital requirements disclosed in our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the Securities and Exchange Commission, or SEC, on February 13, 2025.

### **CRITICAL ACCOUNTING POLICIES AND ESTIMATES**

Our discussion and analysis of our financial condition and results of operations are based upon our condensed 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 condensed 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. During the three months ended March 31, 2025, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on February 13, 2025.

## **RECENT ACCOUNTING PRONOUNCEMENTS**

For a discussion of recent accounting pronouncements, please refer to Note A, “Basis of Presentation and Accounting Policies.”

## **Item 3. Quantitative and Qualitative Disclosures About Market Risk**

Information required by this item is incorporated by reference from the discussion in Part II, Item 7A, “Quantitative and Qualitative Disclosures About Market Risk,” of our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on February 13, 2025.

## **Item 4. Controls and Procedures**

### **Evaluation of Disclosure Controls and Procedures**

Our management (under the supervision and with the participation of our chief executive officer and chief financial officer), after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q, has concluded that, based on such evaluation, as of March 31, 2025 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. In designing and evaluating our disclosure controls and procedures, our 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 our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

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

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended March 31, 2025 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

## **PART II Other Information**

### **Item 1. Legal Proceedings**

We are not currently subject to any material legal proceedings.

## Item 1A. Risk Factors

The information presented below supplements the risk factors set forth in Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on February 13, 2025. There have been no material changes from the risk factors previously disclosed in the Annual Report on Form 10-K.

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

This Quarterly Report on Form 10-Q and, in particular, our Management’s Discussion and Analysis of Financial Condition and Results of Operations set forth in Part I, Item 2, contain a number of 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, taxes, and other gains and losses;
- our expectations regarding our clinical trials and pipeline programs, including expectations for patient enrollment, development timelines, the expected timing of data from our ongoing and planned clinical trials, regulatory authority filings and other submissions for our therapies, communications with regulatory authorities and anticipated regulatory approvals;
- our ability to maintain and obtain adequate reimbursement for our products, 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, providing improved treatment options for people who are already eligible for one of our medicines, and pursuing genetic therapies for people with CF who cannot currently benefit from our medicines;
- the data that will be generated by ongoing and planned clinical trials and the ability to use that data to advance compounds, continue development or support regulatory filings;
- 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;
- 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;
- plans for and prospects of our business development activities, including the potential benefits and therapeutic scope of our collaborations, our ability to integrate and continue operations of acquired businesses, and our ability to successfully capitalize on these opportunities;
- the establishment, development and maintenance of collaborative relationships, including potential milestone payments or other obligations;
- 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;
- potential fluctuations in foreign currency exchange rates and the effectiveness of our foreign currency management program;
- our expectations regarding cash 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;
- our plans to build and maintain our global supply chains and manufacturing infrastructure and capabilities, including for cell and gene therapies; 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” in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2024, which was filed with the SEC on February 13, 2025, 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 2. Unregistered Sales of Equity Securities and Use of Proceeds

### Issuer Repurchases of Equity Securities

In February 2023, our Board of Directors approved a share repurchase program (our “Share Repurchase Program”), pursuant to which we are authorized to repurchase up to \$3.0 billion of our common stock. Our 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 March 31, 2025 under our Share Repurchase Program.

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs (1)	Approximate Dollar Value of Shares that May Yet be Purchased Under the Plans or Programs (1)
January 1, 2025 to January 31, 2025	533,000	\$ 419.45	533,000	\$ 1,157,685,653
February 1, 2025 to February 28, 2025	213,006	\$ 471.35	213,006	\$ 1,057,284,913
March 1, 2025 to March 31, 2025	186,504	\$ 498.08	186,504	\$ 964,391,824
Total	932,510	\$ 447.03	932,510	\$ 964,391,824

(1) Under our Share Repurchase Program, we are authorized to purchase shares from time to time through open market or privately negotiated transactions. Such purchases may be 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 5. 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 Securities Exchange Act of 1934, as amended. 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 Securities Exchange Act of 1934) during the first quarter of 2025, each of which is intended to satisfy the affirmative defense conditions of Rule 10b5-1 (each, a “Trading Plan”).

Name and Title	Date of Adoption of Trading Plan	Scheduled Expiration Date of Trading Plan (1)	Maximum Shares Subject to Trading Plan
Amit Sachdev <i>EVP, Chief Patient and External Affairs Officer</i>	2/21/2025	1/30/2026	48,505
Jonathan Biller <i>EVP, Chief Legal Officer</i>	2/24/2025	2/27/2026	22,217 <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.

## Item 6. Exhibits

Exhibit Number	Exhibit Description
10.1	<a href="#">Employment Agreement, dated February 7, 2025, by and between Vertex Pharmaceuticals Incorporated and Charles F. Wagner, Jr.*</a>
31.1	<a href="#">Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</a>
31.2	<a href="#">Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</a>
32.1	<a href="#">Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.</a>
101.INS	XBRL Instance - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation
101.LAB	XBRL Taxonomy Extension Labels
101.PRE	XBRL Taxonomy Extension Presentation
101.DEF	XBRL Taxonomy Extension Definition
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.
*	Management contract, compensatory plan or agreement.

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

**Vertex Pharmaceuticals Incorporated**

May 6, 2025

By:

\_\_\_\_\_  
/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

*Executive Vice President, Chief Financial Officer  
(principal financial officer and  
duly authorized officer)*