

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 SEPTEMBER 30, 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	253,718,831	Outstanding at October 31, 2025
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VERTEX PHARMACEUTICALS INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED SEPTEMBER 30, 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 (Loss)
(unaudited; in millions, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenues:				
Product revenues, net	\$ 3,076.4	\$ 2,771.9	\$ 8,780.6	\$ 8,108.1
Other revenues	—	—	30.7	—
Total revenues	3,076.4	2,771.9	8,811.3	8,108.1
Costs and expenses:				
Cost of sales	414.8	392.6	1,185.3	1,107.1
Research and development expenses	977.7	875.9	2,935.8	2,631.6
Acquired in-process research and development expenses	54.5	15.0	76.5	4,540.9
Selling, general and administrative expenses	445.1	371.8	1,266.1	1,086.7
Intangible asset impairment charge	—	—	379.0	—
Change in fair value of contingent consideration	(1.9)	0.3	1.2	0.7
Total costs and expenses	1,890.2	1,655.6	5,843.9	9,367.0
Income (loss) from operations	1,186.2	1,116.3	2,967.4	(1,258.9)
Interest income	125.7	132.2	369.0	469.9
Interest expense	(3.3)	(7.5)	(10.0)	(27.8)
Other expense, net	(9.8)	(16.9)	(14.2)	(71.2)
Income (loss) before provision for income taxes	1,298.8	1,224.1	3,312.2	(888.0)
Provision for income taxes	215.9	178.7	550.1	560.6
Net income (loss)	<u>\$ 1,082.9</u>	<u>\$ 1,045.4</u>	<u>\$ 2,762.1</u>	<u>\$ (1,448.6)</u>
Net income (loss) per common share:				
Basic	\$ 4.24	\$ 4.05	\$ 10.77	\$ (5.61)
Diluted	\$ 4.20	\$ 4.01	\$ 10.68	\$ (5.61)
Shares used in per share calculations:				
Basic	255.6	258.0	256.4	258.1
Diluted	257.6	261.0	258.6	258.1

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net income (loss)	\$ 1,082.9	\$ 1,045.4	\$ 2,762.1	\$ (1,448.6)
Other comprehensive income (loss):				
Unrealized holding gains on available-for-sale debt securities, net of tax of \$(1.0), \$(18.3), \$(7.7) and \$(11.4), respectively	3.5	66.7	27.4	41.6
Unrealized gains (losses) on foreign currency forward contracts, net of tax of \$(9.4), \$17.3, \$70.3 and \$1.8, respectively	33.5	(63.0)	(248.7)	(6.7)
Foreign currency translation adjustment	(0.4)	2.6	29.0	8.2
Total other comprehensive income (loss)	36.6	6.3	(192.3)	43.1
Comprehensive income (loss)	<u>\$ 1,119.5</u>	<u>\$ 1,051.7</u>	<u>\$ 2,569.8</u>	<u>\$ (1,405.5)</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)

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 4,939.6	\$ 4,569.6
Marketable securities	1,347.4	1,546.3
Accounts receivable, net	1,946.4	1,609.4
Inventories	1,626.8	1,205.4
Prepaid expenses and other current assets	709.4	665.7
Total current assets	10,569.6	9,596.4
Property and equipment, net	1,425.1	1,227.8
Goodwill	1,088.0	1,088.0
Other intangible assets, net	429.8	825.9
Deferred tax assets	2,937.2	2,331.1
Operating lease assets	1,591.8	1,356.8
Long-term marketable securities	5,722.8	5,107.9
Other assets	1,098.0	999.3
Total assets	\$ 24,862.3	\$ 22,533.2
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$ 420.3	\$ 413.0
Accrued expenses	3,613.5	2,788.6
Other current liabilities	441.5	363.0
Total current liabilities	4,475.3	3,564.6
Long-term operating lease liabilities	1,834.8	1,544.4
Other long-term liabilities	1,233.4	1,014.6
Total liabilities	7,543.5	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, 253,951,475 and 256,940,382 shares issued and outstanding, respectively	2.5	2.6
Additional paid-in capital	5,011.9	6,672.4
Accumulated other comprehensive (loss) income	(64.5)	127.8
Retained earnings	12,368.9	9,606.8
Total shareholders' equity	17,318.8	16,409.6
Total liabilities and shareholders' equity	\$ 24,862.3	\$ 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				
Balance at June 30, 2024	258.0	\$ 2.6	\$ 7,101.2	\$ 22.5	\$ 7,648.4	\$ 14,774.7
Other comprehensive income, net of tax	—	—	—	6.3	—	6.3
Net income	—	—	—	—	1,045.4	1,045.4
Repurchases of common stock	(0.6)	—	(307.6)	—	—	(307.6)
Common stock withheld for employee tax obligations	(0.2)	—	(83.7)	—	—	(83.7)
Issuance of common stock under benefit plans	0.5	—	7.6	—	—	7.6
Stock-based compensation expense	—	—	188.2	—	—	188.2
Balance at September 30, 2024	257.7	\$ 2.6	\$ 6,905.7	\$ 28.8	\$ 8,693.8	\$ 15,630.9
Balance at June 30, 2025	256.3	\$ 2.6	\$ 5,987.9	\$ (101.1)	\$ 11,286.0	\$ 17,175.4
Other comprehensive income, net of tax	—	—	—	36.6	—	36.6
Net income	—	—	—	—	1,082.9	1,082.9
Repurchases of common stock	(2.7)	(0.1)	(1,092.4)	—	—	(1,092.5)
Common stock withheld for employee tax obligations	(0.2)	—	(91.0)	—	—	(91.0)
Issuance of common stock under benefit plans	0.6	—	8.1	—	—	8.1
Stock-based compensation expense	—	—	199.3	—	—	199.3
Balance at September 30, 2025	254.0	\$ 2.5	\$ 5,011.9	\$ (64.5)	\$ 12,368.9	\$ 17,318.8

	Nine Months Ended					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings	Total Shareholders' Equity
	Shares	Amount				
Balance at December 31, 2023	257.7	\$ 2.6	\$ 7,449.7	\$ (14.3)	\$ 10,142.4	\$ 17,580.4
Other comprehensive income, net of tax	—	—	—	43.1	—	43.1
Net loss	—	—	—	—	(1,448.6)	(1,448.6)
Repurchases of common stock	(1.7)	—	(763.8)	—	—	(763.8)
Common stock withheld for employee tax obligations	(0.9)	—	(397.7)	—	—	(397.7)
Issuance of common stock under benefit plans	2.6	—	79.3	—	—	79.3
Stock-based compensation expense	—	—	538.2	—	—	538.2
Balance at September 30, 2024	257.7	\$ 2.6	\$ 6,905.7	\$ 28.8	\$ 8,693.8	\$ 15,630.9
Balance at December 31, 2024	256.9	\$ 2.6	\$ 6,672.4	\$ 127.8	\$ 9,606.8	\$ 16,409.6
Other comprehensive loss, net of tax	—	—	—	(192.3)	—	(192.3)
Net income	—	—	—	—	2,762.1	2,762.1
Repurchases of common stock	(4.5)	(0.1)	(1,906.6)	—	—	(1,906.7)
Common stock withheld for employee tax obligations	(0.8)	—	(367.4)	—	—	(367.4)
Issuance of common stock under benefit plans	2.4	—	74.0	—	—	74.0
Stock-based compensation expense	—	—	539.5	—	—	539.5
Balance at September 30, 2025	254.0	\$ 2.5	\$ 5,011.9	\$ (64.5)	\$ 12,368.9	\$ 17,318.8

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)

	Nine Months Ended September 30,	
	2025	2024
Cash flows from operating activities:		
Net income (loss)	\$ 2,762.1	\$ (1,448.6)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities:		
Stock-based compensation expense	528.3	530.7
Depreciation and amortization expense	154.1	160.7
Intangible asset impairment charge	379.0	—
Deferred income taxes	(541.3)	(400.4)
Losses on equity securities	12.2	50.5
Other non-cash items, net	90.3	(15.5)
Changes in operating assets and liabilities:		
Accounts receivable	(247.9)	(168.3)
Inventories	(446.8)	(369.8)
Prepaid expenses and other assets	(145.4)	(35.7)
Accounts payable	(1.5)	41.1
Accrued expenses	547.6	533.0
Other liabilities	42.7	45.1
Net cash provided by (used in) operating activities	3,133.4	(1,077.2)
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(5,165.9)	(5,279.6)
Sales and maturities of available-for-sale debt securities	4,805.6	3,005.4
Acquisition of available-for-sale debt securities from Alpine Immune Sciences, Inc.	—	(258.0)
Purchases of property and equipment	(288.2)	(205.1)
Net payments related to finite-lived intangible assets	—	(187.7)
Other investing activities	(8.8)	(23.1)
Net cash used in investing activities	(657.3)	(2,948.1)
Cash flows from financing activities:		
Issuances of common stock under benefit plans	73.7	80.3
Repurchases of common stock	(1,889.0)	(759.2)
Payments in connection with common stock withheld for employee tax obligations	(367.4)	(397.7)
Payments on finance leases	(4.0)	(32.3)
Other financing activities	2.6	5.3
Net cash used in financing activities	(2,184.1)	(1,103.6)
Effect of changes in exchange rates on cash	83.6	4.7
Net increase (decrease) in cash, cash equivalents and restricted cash	375.6	(5,124.2)
Cash, cash equivalents and restricted cash—beginning of period	4,572.2	10,372.3
Cash, cash equivalents and restricted cash—end of period	\$ 4,947.8	\$ 5,248.1
Supplemental disclosure of cash flow information:		
Cash paid for income taxes	\$ 1,177.3	\$ 810.3
Cash paid for interest	\$ 9.4	\$ 27.8

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 September 30, 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 anticipate that the adoption of ASU 2023-09 will expand our income tax footnote disclosures, including a more detailed effective tax rate reconciliation.

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 \$54.5 million and \$76.5 million in the three and nine months ended September 30, 2025, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions. Our AIPR&D included \$15.0 million and \$4.5 billion in the three and nine months ended September 30, 2024, respectively. Our AIPR&D in the nine months ended September 30, 2024, primarily included \$4.4 billion associated with our acquisition of Alpine Immune Sciences, Inc. (“Alpine”) as discussed below.

Our collaboration, licensing and asset acquisition agreements that had a significant impact on our financial statements for the three and nine months ended September 30, 2025 and 2024 or were new or materially revised during the three and nine months ended September 30, 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.

Asset Acquisition

Alpine Immune Sciences, Inc. - povetacept

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

Alpine’s lead molecule, povetacept, is a highly potent and effective dual inhibitor of B cell activating factor (“BAFF”) and a proliferation inducing ligand (“APRIL”). As of the acquisition date, povetacept was in Phase 2 development and had shown potential best-in-class efficacy in IgA nephropathy (“IgAN”), a serious, progressive, autoimmune disease of the kidney that can lead to end-stage-renal disease. Due to its mechanism of action as a dual BAFF/APRIL inhibitor, povetacept also holds the potential to benefit patients with other serious autoimmune diseases of the kidney, such as primary membranous nephropathy and lupus nephritis. We accounted for the Alpine transaction as an asset acquisition because povetacept represented substantially all of the fair value of the gross assets that we acquired. As a result, \$4.4 billion of fair value attributed to povetacept was expensed to AIPR&D in the nine months ended September 30, 2024.

We paid total cash of \$5.0 billion at the acquisition date, which included \$4.8 billion to acquire Alpine and \$197.6 million for cash-settled unvested Alpine equity awards. The \$197.6 million represented post-acquisition expense, which was recorded as \$165.0 million of “Research and development expenses” and \$32.6 million of “Selling, general and administrative expenses” in the nine months ended September 30, 2024.

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

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

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

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.

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

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 Orna Therapeutics in December 2024, which we recorded as a credit to AIPR&D in the nine months ended September 30, 2025.

During the three and nine months ended September 30, 2025 and 2024, the credits recognized in our condensed consolidated statements of income (loss) for CRISPR's share of CRISPR JDCA activities were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
Cost of sales	\$ 43.3	\$ 15.8	\$ 109.6	\$ 47.5
Research and development expenses	\$ 14.3	\$ 11.7	\$ 45.4	\$ 35.0
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 AIPR&D in the nine months ended September 30, 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 an agreement with Zai Lab Limited (“Zai”) for the development and commercialization of povetacept in mainland China, Hong Kong SAR, Macau SAR, Taiwan region and Singapore. Under the agreement, Zai is responsible for the povetacept clinical trials and regulatory submissions in the licensed territories. Zai will also be responsible for commercialization activities in the licensed territories, if povetacept becomes an approved product. Under the terms of the agreement, we received a \$10.0 million upfront payment in the first quarter of 2025, which was recorded as “Other revenues” in the nine months ended September 30, 2025. We are eligible to receive from Zai certain regulatory milestone payments and tiered royalties on future net sales of povetacept in the region of focus.

Ono Pharmaceuticals Co., Ltd.

In June 2025, we entered into an agreement with Ono Pharmaceuticals Co., Ltd. (“Ono”) for the development and commercialization of povetacept in Japan and South Korea. Under the agreement, Ono is responsible for the povetacept clinical trials and regulatory submissions in Japan and South Korea. Ono will also be responsible for commercialization activities in Japan and South Korea, if povetacept becomes an approved product. Under the terms of the agreement, we received a \$20.6 million upfront payment in the second quarter of 2025, which was recorded as “Other revenues” in the nine months ended September 30, 2025. We are eligible to receive from Ono certain regulatory milestone payments and tiered royalties on future net sales of povetacept in Japan and South Korea.

Cystic Fibrosis Foundation

In 2004, we entered into an agreement with the Cystic Fibrosis Foundation (the “CFF”), as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc., to support research and development activities. Pursuant to the 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 elxacaftor. 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.”

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C. Earnings Per Share

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions, except per share amounts)			
Net income (loss)	\$ 1,082.9	\$ 1,045.4	\$ 2,762.1	\$ (1,448.6)
Basic weighted-average common shares outstanding	255.6	258.0	256.4	258.1
Effect of potentially dilutive securities:				
Restricted stock units (including performance-based restricted stock units ("PSUs"))	1.1	1.9	1.3	—
Stock options	0.9	1.1	0.9	—
Diluted weighted-average common shares outstanding	257.6	261.0	258.6	258.1
Basic net income (loss) per common share	\$ 4.24	\$ 4.05	\$ 10.77	\$ (5.61)
Diluted net income (loss) per common share	\$ 4.20	\$ 4.01	\$ 10.68	\$ (5.61)

During the three and nine months ended September 30, 2025 and 2024, the number of anti-dilutive securities that were excluded from the computation of our diluted net income per common share were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
Unvested restricted stock units (including PSUs)	0.7	—	0.2	1.1
Stock options	—	—	—	0.6

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.

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

	As of September 30, 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	\$ 1,216.6	\$ 505.5	\$ 711.1	\$ —	\$ 1,687.1	\$ 613.3	\$ 1,073.8	\$ —
Marketable securities:								
Corporate equity securities	9.4	9.4	—	—	36.6	36.6	—	—
U.S. Treasury securities	1,937.7	1,937.7	—	—	1,602.0	1,566.8	35.2	—
U.S. government agency securities	190.6	—	190.6	—	240.5	—	240.5	—
Asset-backed securities	1,361.0	—	1,361.0	—	1,244.2	—	1,244.2	—
Certificates of deposit	24.4	—	24.4	—	—	—	—	—
Corporate debt securities	3,538.4	—	3,538.4	—	3,525.9	—	3,525.9	—
Commercial paper	8.7	—	8.7	—	5.0	—	5.0	—
Prepaid expenses and other current assets:								
Foreign currency forward contracts	6.7	—	6.7	—	130.1	—	130.1	—
Other assets:								
Foreign currency forward contracts	9.4	—	9.4	—	12.4	—	12.4	—
Total financial assets	<u>\$ 8,302.9</u>	<u>\$ 2,452.6</u>	<u>\$ 5,850.3</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	\$ (114.3)	\$ —	\$ (114.3)	\$ —	\$ —	\$ —	\$ —	\$ —
Other long-term liabilities:								
Foreign currency forward contracts	(78.3)	—	(78.3)	—	—	—	—	—
Contingent consideration	(78.1)	—	—	(78.1)	(76.9)	—	—	(76.9)
Total financial liabilities	<u>\$ (270.7)</u>	<u>\$ —</u>	<u>\$ (192.6)</u>	<u>\$ (78.1)</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.

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 4.2% and 4.5% as of September 30, 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:

	Nine Months Ended September 30, 2025
	(in millions)
Balance at December 31, 2024	\$ 76.9
Increase in fair value of contingent payments	1.2
Balance at September 30, 2025	<u>\$ 78.1</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:

	As of September 30, 2025				As of December 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in millions)							
Cash equivalents	\$ 1,216.6	\$ —	\$ —	\$ 1,216.6	\$ 1,687.1	\$ —	\$ —	\$ 1,687.1
Marketable securities:								
U.S. Treasury securities	1,926.0	11.9	(0.2)	1,937.7	1,603.9	3.6	(5.5)	1,602.0
U.S. government agency securities	189.6	1.1	(0.1)	190.6	240.5	0.5	(0.5)	240.5
Asset-backed securities	1,354.6	6.6	(0.2)	1,361.0	1,239.6	5.1	(0.5)	1,244.2
Certificates of deposit	24.4	—	—	24.4	—	—	—	—
Corporate debt securities	3,513.2	25.5	(0.3)	3,538.4	3,519.4	10.6	(4.1)	3,525.9
Commercial paper	8.7	—	—	8.7	5.0	—	—	5.0
Total marketable available-for-sale debt securities	7,016.5	45.1	(0.8)	7,060.8	6,608.4	19.8	(10.6)	6,617.6
Corporate equity securities	25.0	—	(15.6)	9.4	72.1	3.0	(38.5)	36.6
Total marketable securities	7,041.5	45.1	(16.4)	7,070.2	6,680.5	22.8	(49.1)	6,654.2
Total cash equivalents and marketable securities	<u>\$ 8,258.1</u>	<u>\$ 45.1</u>	<u>\$ (16.4)</u>	<u>\$ 8,286.8</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:

	As of September 30, 2025	As of December 31, 2024
	(in millions)	
Cash and cash equivalents	\$ 1,216.6	\$ 1,687.1
Marketable securities	1,347.4	1,546.3
Long-term marketable securities	5,722.8	5,107.9
Total	<u>\$ 8,286.8</u>	<u>\$ 8,341.3</u>

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

	As of September 30, 2025	As of December 31, 2024
	(in millions)	
Matures within one year	\$ 1,338.0	\$ 1,509.7
Matures after one year through five years	5,633.2	5,034.4
Matures after five years	89.6	73.5
Total	<u>\$ 7,060.8</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 and nine months ended September 30, 2025 and 2024. Additionally, we did not record any realized gains or losses that were material to our condensed consolidated statements of income (loss) during the three and nine months ended September 30, 2025 and 2024. As of September 30, 2025, we held marketable available-for-sale debt securities with a total fair value of \$463.1 million that were in unrealized loss positions totaling \$0.8 million. Included in this amount were marketable available-for sale debt securities with a total fair value of \$9.9 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 (loss). During the three and nine months ended September 30, 2025 and 2024, our net unrealized (losses) gains on corporate equity securities with readily determinable fair values held at the conclusion of each period were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
Net unrealized (losses) gains	\$ (1.5)	\$ 2.8	\$ (18.6)	\$ (12.6)

As of September 30, 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 \$81.5 million. During the three and nine months ended September 30, 2024, we reduced the carrying value of one of our equity investments without a readily determinable fair value by \$13.3 million and \$37.6 million, respectively, 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:

	Unrealized Holding Gains (Losses), Net of Tax			
	Foreign Currency Translation Adjustment	On Available-For- Sale Debt Securities	On Foreign Currency Forward Contracts	Total
	(in millions)			
Balance at December 31, 2024	\$ 9.7	\$ 7.1	\$ 111.0	\$ 127.8
Other comprehensive income (loss) before reclassifications	29.0	32.9	(276.9)	(215.0)
Amounts reclassified from accumulated other comprehensive income (loss)	—	(5.5)	28.2	22.7
Net current period other comprehensive income (loss)	29.0	27.4	(248.7)	(192.3)
Balance at September 30, 2025	<u>\$ 38.7</u>	<u>\$ 34.5</u>	<u>\$ (137.7)</u>	<u>\$ (64.5)</u>
Balance at December 31, 2023	\$ 1.1	\$ 9.6	\$ (25.0)	\$ (14.3)
Other comprehensive income before reclassifications	8.2	39.2	2.1	49.5
Amounts reclassified from accumulated other comprehensive income (loss)	—	2.4	(8.8)	(6.4)
Net current period other comprehensive income (loss)	8.2	41.6	(6.7)	43.1
Balance at September 30, 2024	<u>\$ 9.3</u>	<u>\$ 51.2</u>	<u>\$ (31.7)</u>	<u>\$ 28.8</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 (loss) in the same period that we recognize the product revenues that were impacted by the hedged foreign exchange rate changes.

We formally document the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as our risk management objective and strategy for undertaking various hedging activities, which includes matching all foreign currency forward contracts that are designated as cash flow hedges to forecasted transactions. Using regression analysis, we assess, both at the hedge’s inception and on an ongoing basis, whether the foreign currency forward contracts are highly effective in offsetting changes in cash flows of hedged items on a prospective and retrospective basis. As of September 30, 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 September 30, 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:

	<u>As of September 30, 2025</u>	<u>As of December 31, 2024</u>
Foreign Currency	(in millions)	
Euro	\$ 5,249.4	\$ 1,977.4
Canadian dollar	570.4	322.0
British pound sterling	545.2	301.7
Australian dollar	296.1	179.2
Swiss franc	139.5	79.7
Total foreign currency forward contracts	<u>\$ 6,800.6</u>	<u>\$ 2,860.0</u>

Foreign currency forward contracts - Not designated as hedging instruments

We enter into foreign currency forward contracts, typically with contractual maturities of approximately one month, which are designed to mitigate the effect of changes in foreign exchange rates on monetary assets and liabilities, including intercompany balances. These contracts are not designated as hedging instruments under U.S. GAAP. We recognize realized gains and losses for such contracts in “Other expense, net” in our condensed consolidated statements of income (loss) each period. As of September 30, 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 \$619.3 million and \$367.0 million, respectively.

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During the three and nine months ended September 30, 2025 and 2024, we recognized the following related to foreign currency forward contracts in our condensed consolidated statements of income (loss):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
(in millions)				
<i>Designated as hedging instruments - Reclassified from AOCI</i>				
Product revenues, net	\$ (39.0)	\$ (3.1)	\$ (36.2)	\$ 11.2
<i>Not designated as hedging instruments</i>				
Other expense, net	\$ (22.9)	\$ 0.1	\$ (27.2)	\$ (15.7)
<i>Total reported in the Condensed Consolidated Statements of Income (Loss)</i>				
Product revenues, net	\$ 3,076.4	\$ 2,771.9	\$ 8,780.6	\$ 8,108.1
Other expense, net	\$ (9.8)	\$ (16.9)	\$ (14.2)	\$ (71.2)

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:

As of September 30, 2025			
Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in millions)			
Prepaid expenses and other current assets	\$ 6.7	Other current liabilities	\$ (114.3)
Other assets	9.4	Other long-term liabilities	(78.3)
Total assets	<u>\$ 16.1</u>	Total liabilities	<u>\$ (192.6)</u>

As of December 31, 2024			
Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in millions)			
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 September 30, 2025, we expect the amounts that are related to foreign currency forward contracts designated as cash flow hedges under U.S. GAAP recorded in “Prepaid expenses and other current assets” and “Other current liabilities” to be reclassified to earnings within twelve months.

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:

	As of September 30, 2025				
	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
Foreign currency forward contracts					
(in millions)					
Total assets	\$ 16.1	\$ —	\$ 16.1	\$ (16.1)	\$ —
Total liabilities	(192.6)	—	(192.6)	16.1	(176.5)

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

H. Inventories

“Inventories” consisted of the following:

	As of September 30, 2025	As of December 31, 2024
	(in millions)	
Raw materials	\$ 278.6	\$ 252.0
Work-in-process	1,096.2	768.8
Finished goods	252.0	184.6
Total	<u>\$ 1,626.8</u>	<u>\$ 1,205.4</u>

I. Intangible Assets

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

		As of September 30, 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	(37.0)	201.0	238.0	(21.9)	216.1
Finite-lived intangible assets - assembled workforce	3 years	7.7	(3.5)	4.2	7.7	(1.5)	6.2
Total other intangible assets, net		\$ 470.3	\$ (40.5)	\$ 429.8	\$ 849.3	\$ (23.4)	\$ 825.9

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 first quarter of 2025. As of September 30, 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 and nine months ended September 30, 2025 and 2024, we recognized the following stock-based compensation expense:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
Stock-based compensation expense by type of award:				
Restricted stock units (including PSUs)	\$ 190.8	\$ 184.8	\$ 517.1	\$ 522.7
ESPP share issuances	8.5	3.4	21.2	13.7
Stock options	—	—	1.2	1.8
Stock-based compensation expense related to inventories	(4.4)	(3.6)	(11.2)	(7.5)
Total stock-based compensation expense included in “Total costs and expenses”	<u>\$ 194.9</u>	<u>\$ 184.6</u>	<u>\$ 528.3</u>	<u>\$ 530.7</u>
Stock-based compensation expense by line item:				
Cost of sales	\$ 2.8	\$ 1.9	\$ 7.9	\$ 5.5
Research and development expenses	116.0	111.0	315.7	327.5
Selling, general and administrative expenses	76.1	71.7	204.7	197.7
Total stock-based compensation expense included in “Total costs and expenses”	<u>194.9</u>	<u>184.6</u>	<u>528.3</u>	<u>530.7</u>
Income tax effect	(47.5)	(64.6)	(159.2)	(224.3)
Total stock-based compensation expense, net of tax	<u>\$ 147.4</u>	<u>\$ 120.0</u>	<u>\$ 369.1</u>	<u>\$ 306.4</u>

Share repurchase program

In February 2023, our Board of Directors approved a share repurchase program (the “2023 Share Repurchase Program”), pursuant to which we were authorized to repurchase up to \$3.0 billion of our common stock. As of September 30, 2025, we had repurchased the full amount authorized under the 2023 Share Repurchase Program.

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

During the nine months ended September 30, 2025 and 2024, we repurchased 4.5 million and 1.7 million shares of our common stock under our share repurchase programs, respectively, for aggregate repurchases of \$1.9 billion and \$763.8 million, respectively. As of September 30, 2025, we had remaining authorization of \$3.5 billion under the 2025 Share Repurchase Program.

K. Income Taxes

We are subject to U.S. federal, state, and foreign income taxes. During the three and nine months ended September 30, 2025 and 2024, we recorded the following provisions for income taxes and effective tax rates as compared to our income (loss) before provision for income taxes.

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	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions, except percentages)			
Income (loss) before provision for income taxes	\$ 1,298.8	\$ 1,224.1	\$ 3,312.2	\$ (888.0)
Provision for income taxes	\$ 215.9	\$ 178.7	\$ 550.1	\$ 560.6
Effective tax rate	16.6 %	14.6 %	16.6 %	(63.1)%

Our effective tax rates for the three and nine months ended September 30, 2025 were lower than the U.S. statutory rate primarily due to a benefit from a research and development tax credit study that was completed in the third quarter of 2025, excess tax benefits related to stock-based compensation, and increased utilization of foreign tax credits, partially offset by changes in uncertain tax positions.

Our effective tax rate for the three months ended September 30, 2024 was lower than the U.S. statutory rate primarily due to a benefit from a research and development tax credit study that was completed in the third quarter of 2024 and excess tax benefits related to stock-based compensation, partially offset by changes in uncertain tax positions.

Our effective tax rate for the nine months ended September 30, 2024 was materially different than the U.S. statutory rate primarily due to the \$4.4 billion of non-deductible AIPR&D resulting from our acquisition of Alpine, which drove our pre-tax loss for the nine months ended September 30, 2024.

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 September 30, 2025 and December 31, 2024, we had \$421.6 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%.

In July 2025, the U.S. enacted H.R.1, which includes significant provisions modifying the U.S. tax framework, including the ability for companies to immediately deduct research and development expenditures for 2025 and provisions for deducting previously capitalized amounts. We continue to assess the impact that H.R.1. has on us and will review guidance as it becomes available. These legislative changes could have an impact on our future effective tax rates, tax liabilities, and cash taxes.

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

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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 September 30, 2025, we were in compliance with the covenants described above. The Credit Agreement also contains customary events of default. In the case of a continuing event of default, the administrative agent would be entitled to exercise various remedies, including the acceleration of amounts due under outstanding loans.

Direct costs related to the Credit Agreement are recorded over its term and are not material to our financial statements.

Guaranties and Indemnifications

As permitted under Massachusetts law, our Articles of Organization and By-laws provide that we will indemnify certain of our officers and directors for certain claims asserted against them in connection with their service as an officer or director. The maximum potential amount of future payments that we could be required to make under these indemnification provisions is unlimited. However, we have purchased directors’ and officers’ liability insurance policies that could reduce our monetary exposure and enable us to recover a portion of any future amounts paid. No indemnification claims currently are outstanding, and we believe the estimated fair value of these indemnification arrangements is minimal.

We customarily agree in the ordinary course of our business to indemnification provisions in agreements with clinical trial investigators and sites in our product development programs, sponsored research agreements with academic and not-for-profit institutions, various comparable agreements involving parties performing services for us, and our real estate leases. We also customarily agree to certain indemnification provisions in our drug discovery, development and commercialization collaboration agreements. With respect to our clinical trials and sponsored research agreements, these indemnification provisions typically apply to any claim asserted against the investigator or the investigator’s institution relating to personal injury or property damage, violations of law or certain breaches of our contractual obligations arising out of the research or clinical testing of our compounds or product candidates. With respect to lease agreements, the indemnification provisions typically apply to claims asserted against the landlord relating to personal injury or property damage caused by us, to violations of law by us or to certain breaches of our contractual obligations. The indemnification provisions appearing in our collaboration agreements are similar to those for the other agreements discussed above, but in addition provide some limited indemnification for our collaborator in the event of third-party claims alleging infringement of intellectual property rights. In each of the cases above, the indemnification obligation generally survives the termination of the agreement for some extended period, although we believe the obligation typically has the most relevance during the contract term and for a short period of time thereafter. The maximum potential amount of future payments that we could be required to make under these provisions is generally unlimited. We have purchased insurance policies covering personal injury, property damage and general liability that reduce our exposure for indemnification and would enable us in many cases to recover all or a portion of any future amounts paid. We have never paid any material amounts to defend lawsuits or settle claims related to these indemnification provisions. Accordingly, we believe the estimated fair value of these indemnification arrangements is minimal.

Legal Matters and Other Contingencies

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

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As described in Note B, “Collaboration, License and Other Arrangements,” we have an agreement with the CFF (the “CFF Agreement”) pursuant to which we owe third party royalties payable on net sales of certain CF products, including ALYFTREK. Since inception, our ALYFTREK net product revenues total \$457.7 million. Based on the CFF Agreement, our position is that the royalty burden associated with ALYFTREK is 4%. On October 10, 2025, Royalty Pharma plc (“RP”), the third party to whom the CFF assigned its rights (and the CFF, which remains a party to the CFF Agreement), initiated a confidential arbitration alleging the royalty burden on ALYFTREK is approximately 8%. RP is seeking a declaratory judgment regarding the royalty burden on ALYFTREK as well as alleged unpaid royalties and other alleged damages available under the CFF Agreement or applicable law, costs, expenses, attorneys’ fees, and interest. We believe RP’s position is contrary to the plain terms of the CFF Agreement and intend to vigorously defend our position under the CFF Agreement.

On a quarterly basis, we evaluate developments with claims, whether asserted or unasserted, and legal proceedings that could result in a loss contingency accrual, or an increase or decrease to a previously accrued loss contingency. There were no material loss contingencies accrued as of September 30, 2025 or December 31, 2024.

We also have certain contingent liabilities that arise in the ordinary course of our business activities. We accrue for such contingent liabilities when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. Other than our contingent consideration liabilities discussed in Note D, “Fair Value Measurements,” there were no significant contingent liabilities accrued as of September 30, 2025 or December 31, 2024.

M. Segment Information

Revenues by Product

“Product revenues, net” consisted of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
TRIKAFTA/KAFTRIO	\$ 2,653.6	\$ 2,585.0	\$ 7,740.2	\$ 7,517.8
ALYFTREK	247.0	—	457.7	—
Other product revenues	175.8	186.9	582.7	590.3
Total product revenues, net	<u>\$ 3,076.4</u>	<u>\$ 2,771.9</u>	<u>\$ 8,780.6</u>	<u>\$ 8,108.1</u>

In the three and nine months ended September 30, 2025, “Other product revenues” included \$16.9 million and \$61.5 million, respectively, from CASGEVY, and \$19.6 million and \$32.9 million, respectively, from JOURNAVX. In the three and nine months ended September 30, 2024, “Other product revenues” were \$2.0 million from CASGEVY and there were no revenues from JOURNAVX. 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:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
United States	\$ 1,976.3	\$ 1,713.5	\$ 5,457.3	\$ 4,847.7
Outside of the United States				
Europe	836.9	847.7	2,574.4	2,621.9
Other	263.2	210.7	748.9	638.5
Total product revenues outside of the United States	<u>1,100.1</u>	<u>1,058.4</u>	<u>3,323.3</u>	<u>3,260.4</u>
Total product revenues, net	<u>\$ 3,076.4</u>	<u>\$ 2,771.9</u>	<u>\$ 8,780.6</u>	<u>\$ 8,108.1</u>

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Significant Segment Expenses

Significant segment expenses are set forth in the following table:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
	(in millions)			
Total revenues	\$ 3,076.4	\$ 2,771.9	\$ 8,811.3	\$ 8,108.1
Costs and expenses:				
Cost of sales - products	139.6	131.6	410.7	363.9
Cost of sales - royalty	275.2	261.0	774.6	743.2
Research expenses	210.0	193.8	625.4	597.2
Development expenses	767.7	682.1	2,310.4	2,034.4
Acquired in-process research and development expenses	54.5	15.0	76.5	4,540.9
Selling and other commercial expenses	278.3	216.9	784.0	604.9
General and administrative expenses	166.8	154.9	482.1	481.8
Intangible asset impairment charge	—	—	379.0	—
Interest income	(125.7)	(132.2)	(369.0)	(469.9)
Other segment items ⁽¹⁾	11.2	24.7	25.4	99.7
Provision for income taxes	215.9	178.7	550.1	560.6
Net income (loss)	<u>\$ 1,082.9</u>	<u>\$ 1,045.4</u>	<u>\$ 2,762.1</u>	<u>\$ (1,448.6)</u>

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

Additional Segment Information

During the three and nine months ended September 30, 2025, we recorded total depreciation and amortization expense of \$54.0 million and \$154.1 million, respectively. During the three and nine months ended September 30, 2024, we recorded total depreciation and amortization expense of \$53.2 million and \$160.7 million, respectively.

N. Additional Balance Sheet & Cash Flow Information

Contract Liabilities

We had contract liabilities of \$182.0 million and \$206.8 million as of September 30, 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 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.

Operating Lease Assets and Liabilities

In 2024, we entered into a lease agreement for a second building (“Leiden II”) at our Jeffrey Leiden Center for Biologics, Cell and Genetic Therapies Campus (“Leiden Campus”) near our corporate headquarters in Boston, Massachusetts. The

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Leiden II lease includes approximately 348,000 square feet of office and laboratory space for a term of approximately 16 years. We anticipate that base rent payments will commence in the first quarter of 2027 and expect them to continue through the first quarter of 2042. We have an option to extend the Leiden II term for up to two additional ten-year periods.

In the third quarter of 2025, we were given access to begin construction of leasehold improvements in Leiden II, which resulted in lease commencement for accounting purposes. We classified the Leiden II lease as an operating lease because none of the finance lease criteria were met. On the Leiden II lease commencement date, a right-of-use asset and corresponding lease liability, net of tenant allowances, of \$296.7 million was recorded within each of “Operating lease assets” and “Long-term operating lease liabilities” on our condensed consolidated balance sheet. We utilize the initial approximate 16-year period as our Leiden II lease term.

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:

	Nine Months Ended September 30,			
	2025		2024	
	Beginning of period	End of period	Beginning of period	End of period
	(in millions)			
Cash and cash equivalents	\$ 4,569.6	\$ 4,939.6	\$ 10,369.1	\$ 5,239.2
Prepaid expenses and other current assets	2.6	8.2	3.2	8.9
Cash, cash equivalents and restricted cash per condensed consolidated statement of cash flows	<u>\$ 4,572.2</u>	<u>\$ 4,947.8</u>	<u>\$ 10,372.3</u>	<u>\$ 5,248.1</u>

Supplemental Cash Flow Information

We obtained \$310.8 million and \$1.1 billion of right-of-use operating lease assets in exchange for lease obligations during the nine months ended September 30, 2025 and 2024, respectively, which represent non-cash operating activities associated with our condensed consolidated statement of cash flows. These amounts primarily relate to the Leiden II lease for the nine months ended September 30, 2025, and an amendment to our corporate headquarters leases for the nine months ended September 30, 2024.

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, neuropathic pain, type 1 diabetes, IgA nephropathy, primary membranous nephropathy and other autoimmune renal diseases and cytopenias, APOL1-mediated kidney disease, autosomal dominant polycystic kidney disease and myotonic dystrophy type 1.

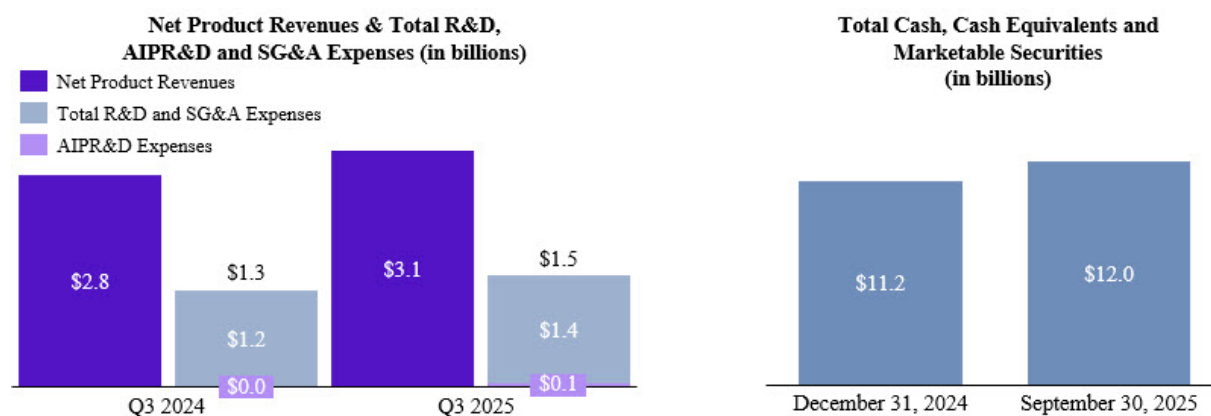
In December 2024, the U.S. Food and Drug Administration (the “FDA”) approved ALYFTREK (vanzacaftor/tezacaftor/deutivacaftor), our once-daily next-in-class triple combination for the treatment of people with CF 6 years of age and older, and our fifth CF medicine. ALYFTREK is also approved in the United Kingdom (the “U.K.”), the European Union (“E.U.”), Canada, New Zealand and Switzerland. Collectively, our five medicines, led by TRIKAFTA/KAFTRIO (elixacaftor/tezacaftor/ivacaftor and ivacaftor), are being used to treat more than three quarters of the approximately 94,000 people with CF in the U.S., Europe, Australia, and Canada.

CASGEVY (exagamglogene autotemcel), our ex-vivo, non-viral CRISPR/Cas9 gene-edited cell therapy, is approved in the U.S., the E.U., the U.K., the Kingdom of Saudi Arabia (“Saudi Arabia”), the Kingdom of Bahrain (“Bahrain”), Qatar, the United Arab Emirates (the “UAE”), 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, and the Middle East.

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.

Financial Highlights

Revenues	In the third quarter of 2025, our net product revenues increased to \$3.1 billion as compared to \$2.8 billion in the third quarter of 2024, primarily due to continued strong patient demand for TRIKAFTA/KAFTRIO and early contributions from three ongoing launches.
Expenses	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 \$1.5 billion in the third quarter of 2025 as compared to \$1.3 billion in the third quarter of 2024, primarily due to increased commercial investments to support the launch of JOURNAVX, continued investment in support of multiple mid-to-late stage clinical development programs, and higher AIPR&D. Cost of sales was 13.5% and 14.2% in the third quarter of 2025 and 2024, respectively.
Cash	Our total cash, cash equivalents and marketable securities increased to \$12.0 billion as of September 30, 2025 as compared to \$11.2 billion as of December 31, 2024 primarily due to cash flows provided by our operating activities partially offset by repurchases of our common stock.



Note: Charts above may not add due to rounding.

Business Updates

Marketed Products

Cystic Fibrosis

We expect that the number of people with CF taking our medicines will continue to grow through new approvals and reimbursement agreements, treatment of younger patients, increased survival and expansion into additional geographies. Recent and anticipated progress in activities expanding our CF business is included below:

- Medsafe New Zealand and Swissmedic approved ALYFTREK for the treatment of people with CF ages 6 years of age and older who have at least one F508del mutation or another responsive mutation in the cystic fibrosis transmembrane conductance regulator (“CFTR”) gene.
- Regulatory submissions for ALYFTREK are under review in Australia.
- Eligible people with CF in England, Ireland, Germany, Denmark and Northern Ireland have reimbursed access to ALYFTREK, and we are working to secure access for eligible patients in additional countries.

Sickle Cell Disease and Beta Thalassemia

- In September, we secured reimbursement for CASGEVY for people with SCD and TDT in Italy. Italy has the largest population of people living with TDT in Europe, with approximately 5,000 people 12 years of age and older with TDT and approximately 2,300 people 12 years of age and older with SCD.
- Globally, since launch through September 30, 2025, approximately 165 people with SCD or TDT have had their first cell collection for CASGEVY, including 50 people in the third quarter of 2025, and 39 people with SCD or TDT have received infusions of CASGEVY, including 10 people infused in the third quarter of 2025.

Acute Pain

- Since JOURNAVX became available at pharmacies in March, through mid-October more than 300,000 prescriptions for JOURNAVX have been written and filled across the hospital and retail settings in different acute pain conditions, consistent with its broad label.
- As of mid-October, across commercial and government payers, more than 170 million individuals have covered access to JOURNAVX, representing more than half of U.S. covered lives. This includes formal coverage with two of the three large national pharmacy benefit managers and unrestricted access within 19 state Medicaid plans. We expect access to JOURNAVX to continue to expand over the remainder of 2025 and into 2026.
- Approximately 90 of the targeted 150 large healthcare systems and more than 750 individual hospitals of the 2,000 targeted institutions have added JOURNAVX to formularies, protocols or order sets.

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

- In October, we completed the pivotal clinical trial of TRIKAFTA in children 12 months to less than 24 months of age. The data showed that TRIKAFTA was generally safe and well-tolerated, consistent with the established safety profile. We expect to submit for approval in this age group with global regulators in the first half of 2026.
- We have completed enrollment in a global trial evaluating ALYFTREK in children 2 to 5 years of age. In this pivotal clinical trial, approximately 65 children will receive ALYFTREK for 24 weeks. We expect to share data from this clinical trial in the first half of 2026.

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 and expect to complete dosing in the fourth quarter of 2025. We expect to share emerging data from these clinical trials in an upcoming medical conference.

Peripheral Neuropathic Pain

- We previously initiated the first Phase 3 clinical trial evaluating suzetrigine for the treatment of people with diabetic peripheral neuropathy, a common form of chronic peripheral neuropathic pain, and we expect to start the second Phase 3 clinical trial in November. We expect to complete enrollment in both Phase 3 clinical trials by the end of 2026.

Type 1 Diabetes

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

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

- We are developing povetacicept, a dual inhibitor of B cell activating factor (“BAFF”) and a proliferation-inducing ligand (“APRIL”) cytokines, for multiple diseases. Povetacicept represents a potentially best-in-class approach to control B cell activity in immunoglobulin A nephropathy (“IgAN”) and primary membranous nephropathy (“pMN”), and we believe it has pipeline-in-a-product potential.
- The FDA has granted a rolling review to the Biologics Licensing Application (“BLA”) submission for povetacicept for the treatment of IgAN. We have completed enrollment of the interim analysis cohort for potential accelerated approval in the U.S., and we have completed the clinical trials to support the launch of povetacicept for monthly, at-home self-administration with an autoinjector. We expect to submit the first module of the IgAN BLA to the FDA before the end of 2025, and we expect to complete the full BLA submission in the first half of 2026 for potential accelerated approval in the U.S. In addition, we have completed full enrollment of the Phase 3 clinical trial evaluating IgAN.
- We have initiated the Phase 2/3 pivotal clinical trial of povetacicept for the treatment of people with pMN.

APOL1-Mediated Kidney Disease

- Inaxaplin is our small molecule for the treatment of APOL1-mediated kidney disease (“AMKD”). We completed enrollment in the interim analysis cohort of the global Phase 2/3 pivotal clinical trial evaluating inaxaplin (“AMPLITUDE”). 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.

Our Business Environment

In the nine months ended September 30, 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 continue to progress commercialization of CASGEVY, which has received marketing approvals in the U.S. and across multiple geographies, including countries in Europe and the Middle East, 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., for example, recently enacted changes to the Medicaid program may result in a less favorable coverage environment that may impact our revenues.

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 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. In addition, federal and state governments in the U.S. continue to consider policies to lower prescription drug costs. The logistics of such initiatives continue to be discussed, and we cannot anticipate how these actions, if enacted, may impact our revenues and our business. 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 across multiple geographies, including countries in Europe and the Middle East. 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.

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, 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 nine months ended September 30, 2025 and 2024, our AIPR&D included \$76.5 million and \$4.5 billion, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions, including the asset acquisitions, collaborations, and licenses of third-party technologies described above. Please refer to Note B, “Collaboration, License and Other Arrangements,” for further information regarding our asset acquisitions, collaborations and in-license agreements.

Out-licensing Arrangements

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

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

RESULTS OF OPERATIONS

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages and per share amounts)						
Total revenues	\$ 3,076.4	\$ 2,771.9	11%	\$ 8,811.3	\$ 8,108.1	9%
Acquired in-process research and development expenses	54.5	15.0	**	76.5	4,540.9	**
Intangible asset impairment charge	—	—	**	379.0	—	**
Other operating costs and expenses	1,835.7	1,640.6	12%	5,388.4	4,826.1	12%
Income (loss) from operations	1,186.2	1,116.3	6%	2,967.4	(1,258.9)	**
Other non-operating income, net	112.6	107.8	4%	344.8	370.9	(7)%
Provision for income taxes	215.9	178.7	21%	550.1	560.6	(2)%
Net income (loss)	<u>\$ 1,082.9</u>	<u>\$ 1,045.4</u>	4%	<u>\$ 2,762.1</u>	<u>\$ (1,448.6)</u>	**
Net income (loss) per diluted common share	\$ 4.20	\$ 4.01		\$ 10.68	\$ (5.61)	
Diluted shares used in per share calculations	257.6	261.0		258.6	258.1	

** Not meaningful

Total Revenues

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
TRIKAFTA/KAFTRIO	\$ 2,653.6	\$ 2,585.0	3%	\$ 7,740.2	\$ 7,517.8	3%
ALYFTREK	247.0	—	**	457.7	—	**
Other product revenues	175.8	186.9	(6)%	582.7	590.3	(1)%
Product revenues, net	3,076.4	2,771.9	11%	8,780.6	8,108.1	8%
Other revenues	—	—	**	30.7	—	**
Total revenues	<u>\$ 3,076.4</u>	<u>\$ 2,771.9</u>	11%	<u>\$ 8,811.3</u>	<u>\$ 8,108.1</u>	9%

** Not meaningful

Product Revenues, Net

In the third quarter and nine months ended September 30, 2025, our net product revenues increased by \$304.5 million and \$672.5 million, or 11% and 8%, as compared to the third quarter and nine months ended September 30, 2024, respectively, primarily due to continued strong demand for TRIKAFTA/KAFTRIO and early contributions from three

ongoing launches. In the third quarter and nine months ended September 30, 2025, "Other product revenues" included \$16.9 million and \$61.5 million, respectively, from CASGEVY, and \$19.6 million and \$32.9 million, respectively, from JOURNAVX. The third quarter and nine months ended September 30, 2024 included \$2.0 million related to CASGEVY.

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
	(in millions, except percentages)					
United States	\$ 1,976.3	\$ 1,713.5	15%	\$ 5,457.3	\$ 4,847.7	13%
ex-U.S.	1,100.1	1,058.4	4%	3,323.3	3,260.4	2%
Product revenues, net	<u>\$ 3,076.4</u>	<u>\$ 2,771.9</u>	11%	<u>\$ 8,780.6</u>	<u>\$ 8,108.1</u>	8%

In the third quarter and nine months ended September 30, 2025, our net product revenues increased 15% and 13% in the U.S., as compared to the third quarter and nine months ended September 30, 2024, respectively, due to continued strong patient demand, new patient initiations and higher net realized pricing. In the third quarter and nine months ended September 30, 2025, our ex-U.S. net product revenues increased 4% and 2%, respectively, as compared to the third quarter and nine months ended September 30, 2024, primarily due to solid performance across multiple geographies, partially offset by an expected decline in product revenues in Russia, where we are continuing to experience a violation of our intellectual property rights.

Other Revenues

Other revenues were \$30.7 million in the nine months ended September 30, 2025, which included \$20.6 million and \$10.0 million related to upfront payments received from our collaboration agreements with Ono and Zai, respectively, in the first half of 2025.

Operating Costs and Expenses

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
	(in millions, except percentages)					
Cost of sales	\$ 414.8	\$ 392.6	6%	\$ 1,185.3	\$ 1,107.1	7%
Research and development expenses	977.7	875.9	12%	2,935.8	2,631.6	12%
Acquired in-process research and development expenses	54.5	15.0	**	76.5	4,540.9	**
Selling, general and administrative expenses	445.1	371.8	20%	1,266.1	1,086.7	17%
Intangible asset impairment charge	—	—	**	379.0	—	**
Change in fair value of contingent consideration	(1.9)	0.3	**	1.2	0.7	**
Total costs and expenses	<u>\$ 1,890.2</u>	<u>\$ 1,655.6</u>	14%	<u>\$ 5,843.9</u>	<u>\$ 9,367.0</u>	(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 (the "CFF Agreement") with the Cystic Fibrosis Foundation (the "CFF"), our tiered third-party royalties on sales of ALYFTREK, TRIKAFTA/KAFTRIO, SYMDEKO/SYMKEVI, KALYDECO, and ORKAMBI, calculated as a percentage of net sales, range from the single digits to the sub-teens, with lower royalties on sales of ALYFTREK and TRIKAFTA/KAFTRIO than for our other products. The royalty burden associated with TRIKAFTA is 9.33% and our position is that the royalty burden associated with ALYFTREK is 4%. On October 10, 2025, Royalty Pharma plc ("RP"), the third party to whom the CFF assigned its rights (and the CFF, which remains a party to the CFF Agreement), initiated a confidential arbitration alleging the royalty burden on ALYFTREK is approximately 8%. RP is seeking a declaratory judgment regarding the royalty burden on ALYFTREK as well as alleged unpaid royalties and other alleged damages available under the CFF Agreement or applicable law, costs, expenses, attorneys' fees, and interest. We believe

RP's position is contrary to the plain terms of the CFF Agreement and intend to vigorously defend our position under the CFF Agreement.

In the third quarter and nine months ended September 30, 2025, our cost of sales increased \$22.2 million and \$78.2 million, or 6% and 7%, as compared to the third quarter and nine months ended September 30, 2024, respectively, primarily due to increased sales volume and changes in product mix. Our cost of sales as a percentage of our net product revenues was 13.5% and 14.2% in the third quarter of 2025 and 2024, respectively, and 13.5% and 13.7% in the nine months ended September 30, 2025 and 2024, respectively.

Research and Development Expenses

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
Research expenses	\$ 210.0	\$ 193.8	8%	\$ 625.4	\$ 597.2	5%
Development expenses	767.7	682.1	13%	2,310.4	2,034.4	14%
Total research and development expenses	<u>\$ 977.7</u>	<u>\$ 875.9</u>	12%	<u>\$ 2,935.8</u>	<u>\$ 2,631.6</u>	12%

Since January 2023, we have incurred approximately \$9.7 billion in research and development expenses associated with product discovery and development. Our research and development expenses include internal and external costs incurred for research and development of our products and product candidates. We assign external costs of services provided to us by clinical research organizations and other outsourced research by individual program. Our internal costs include salary and benefits, stock-based compensation expense, laboratory supplies and other direct expenses and infrastructure costs, the majority of which are not assigned to individual products or product candidates.

Research Expenses

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
Research Expenses:						
Salary and benefits	\$ 49.6	\$ 48.3	3%	\$ 155.8	\$ 163.2	(5)%
Stock-based compensation expense	28.0	29.4	(5)%	72.8	87.9	(17)%
Outsourced services and other direct expenses	72.2	64.1	13%	216.3	195.5	11%
Infrastructure costs	60.2	52.0	16%	180.5	150.6	20%
Total research expenses	<u>\$ 210.0</u>	<u>\$ 193.8</u>	8%	<u>\$ 625.4</u>	<u>\$ 597.2</u>	5%

Our research expenses reflect investment in our pipeline and expansion of our cell and genetic therapy capabilities, which has increased our outsourced services and other direct expenses and infrastructure costs in the third quarter and nine months ended September 30, 2025 as compared to the third quarter and nine months ended September 30, 2024. Salary and benefits in the nine months ended September 30, 2024 included \$13.1 million associated with cash-settled unvested Alpine equity awards. Compared to the third quarter and nine months ended September 30, 2024, our research expenses increased by

\$16.2 million, or 8%, and \$28.2 million, or 5%, respectively. 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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
Development Expenses:						
Salary and benefits	\$ 182.2	\$ 172.8	5%	\$ 565.4	\$ 510.1	11%
Stock-based compensation expense	88.0	81.6	8%	242.9	239.6	1%
Compensation expense for cash-settled unvested Alpine equity awards	—	—	**	—	151.9	**
Outsourced services and other direct expenses	364.7	319.4	14%	1,116.9	822.7	36%
Infrastructure costs	132.8	108.3	23%	385.2	310.1	24%
Total development expenses	<u>\$ 767.7</u>	<u>\$ 682.1</u>	13%	<u>\$ 2,310.4</u>	<u>\$ 2,034.4</u>	14%

** Not meaningful

As we have advanced our pipeline of transformative medicines, we have invested in internal headcount and infrastructure to support multiple mid- and late-stage clinical development programs, including from our povetacept programs acquired from Alpine, pain and T1D programs. In conjunction with our acquisition of Alpine, we incurred \$151.9 million associated with cash-settled unvested Alpine equity awards within development expenses in the nine months ended September 30, 2024. Compared to the third quarter and nine months ended September 30, 2024, our development expenses increased by \$85.6 million, or 13%, and \$276.0 million, or 14%, respectively.

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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
Acquired in-process research and development expenses	\$ 54.5	\$ 15.0	**	\$ 76.5	\$ 4,540.9	**

** Not meaningful

AIPR&D in the third quarter and nine months ended September 30, 2025 and third quarter ended September 30, 2024 included various upfront and milestone payments. AIPR&D in the nine months ended September 30, 2024 was primarily related to \$4.4 billion AIPR&D resulting from our acquisition of Alpine, which was accounted for as an asset acquisition. AIPR&D in the nine months ended September 30, 2024 also included the \$75.0 million milestone paid to Entrada during the first quarter of 2024. 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 September 30,			Nine Months Ended September 30,		
	2025	2024	Change	2025	2024	Change
(in millions, except percentages)						
Selling, general and administrative expenses	\$ 445.1	\$ 371.8	20%	\$ 1,266.1	\$ 1,086.7	17%

Selling, general and administrative expenses increased by 20% and 17% in the third quarter and nine months ended September 30, 2025, respectively, as compared to the third quarter and nine months ended September 30, 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 in the first quarter of 2025.

Contingent Consideration

The fair value of our contingent consideration decreased by \$1.9 million and increased by \$1.2 million in the third quarter and nine months ended September 30, 2025, respectively, and increased by \$0.3 million and \$0.7 million in the third quarter and nine months ended September 30, 2024, respectively.

Other Non-Operating Income (Expense), Net

Interest Income

Interest income decreased from \$132.2 million in the third quarter of 2024 to \$125.7 million in the third quarter of 2025, primarily due to decreased market interest rates partially offset by increased cash, cash equivalents and available-for-sale debt securities. Interest income decreased from \$469.9 million in the nine months ended September 30, 2024 to \$369.0 million in the nine months ended September 30, 2025, primarily due to decreased market interest rates and decreased cash, cash equivalents and available-for-sale debt securities following our acquisition of Alpine in the second 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 \$3.3 million and \$10.0 million in the third quarter and nine months ended September 30, 2025, respectively, and \$7.5 million and \$27.8 million in the third quarter and nine months ended September 30, 2024, respectively. Prior to the third quarter of 2024, the majority of our interest expense related to imputed interest expense associated with our corporate headquarters leases in Boston. Following the amendment of these leases in the third quarter of 2024 that changed their classifications from finance to operating leases, the operating lease costs associated with the leases are recorded entirely within operating expenses in our condensed consolidated statements of income (loss).

Other Income (Expense), Net

Other income (expense), net were expenses of \$9.8 million and \$16.9 million in the third quarter of 2025 and 2024, respectively, and \$14.2 million and \$71.2 million in the nine months ended September 30, 2025 and 2024, respectively. These amounts primarily related to net unrealized and realized losses resulting from changes in the fair value of certain of our strategic equity investments and net foreign currency exchange losses.

Income Taxes

Our effective tax rate fluctuates from 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.

In July 2025, the U.S. enacted H.R.1, which includes significant provisions modifying the U.S. tax framework, including the ability for companies to immediately deduct research and development expenditures for 2025 and provisions for deducting previously capitalized amounts. We continue to assess the impact that H.R.1. has on us and will review guidance as it becomes available. These legislative changes could have an impact on our future effective tax rates, tax liabilities, and cash taxes.

We recorded provisions for income taxes of \$215.9 million and \$178.7 million in the third quarter of 2025 and 2024, respectively, and \$550.1 million and \$560.6 million in the nine months ended September 30, 2025 and 2024, respectively.

Our effective tax rate of 16.6% in the nine months ended September 30, 2025 was lower than the U.S. statutory rate primarily due to a benefit from a research and development tax credit study that was completed in the third quarter of 2025, excess tax benefits related to stock-based compensation, and increased utilization of foreign tax credits, partially offset by changes in uncertain tax positions. Our effective tax rate of (63.1)% in the nine months ended September 30, 2024 was materially different than the U.S. statutory rate primarily due to the \$4.4 billion of AIPR&D resulting from our acquisition of Alpine, which drove our pre-tax loss in the nine months ended September 30, 2024.

LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of September 30, 2025 and December 31, 2024:

	As of September 30, 2025	As of December 31, 2024	Change
	(in millions, except percentages)		
Cash, cash equivalents and marketable securities:			
Cash and cash equivalents	\$ 4,939.6	\$ 4,569.6	
Marketable securities	1,347.4	1,546.3	
Long-term marketable securities	5,722.8	5,107.9	
Total cash, cash equivalents and marketable securities	<u>\$ 12,009.8</u>	<u>\$ 11,223.8</u>	7%
Working Capital:			
Total current assets	\$ 10,569.6	\$ 9,596.4	10%
Total current liabilities	(4,475.3)	(3,564.6)	26%
Total working capital	<u>\$ 6,094.3</u>	<u>\$ 6,031.8</u>	1%

Working Capital

As of September 30, 2025, total working capital was \$6.1 billion, which represented an increase of \$62.5 million, or 1%, compared to December 31, 2024. Our working capital was similar to December 31, 2024 primarily due to increased accounts receivable and inventories resulting from our growing portfolio of products, partially offset by product revenue accruals from the same products.

Cash Flows

	Nine Months Ended September 30,	
	2025	2024
	(in millions)	
Net cash provided by (used in):		
Operating activities	\$ 3,133.4	\$ (1,077.2)
Investing activities	\$ (657.3)	\$ (2,948.1)
Financing activities	\$ (2,184.1)	\$ (1,103.6)

Operating Activities

Cash provided by operating activities was \$3.1 billion in the nine months ended September 30, 2025 primarily due to income from operations of \$3.0 billion driven by our net product revenues. Cash used by operations was \$1.1 billion in the nine months ended September 30, 2024 primarily due to our acquisition of Alpine partially offset by cash flows provided by other operating activities.

Investing Activities

Cash used in investing activities was \$657.3 million in the nine months ended September 30, 2025, primarily related to net purchases of available-for-sale debt securities and purchases of property and equipment. Cash used in investing activities

was \$2.9 billion in the nine months ended September 30, 2024, which included net purchases of available-for-sale debt securities of \$2.3 billion.

Financing Activities

Cash used in financing activities were \$2.2 billion and \$1.1 billion in the nine months ended September 30, 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 programs and payments in connection with common stock withheld for employee tax obligations.

Sources and Uses of Liquidity

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

Credit Facilities & Financing Strategy

We may borrow up to a total of \$500.0 million pursuant to a revolving credit facility that we entered into in July 2022 and could repay and reborrow amounts under this revolving credit agreement without penalty. Subject to certain conditions, we could request that the borrowing capacity be increased by an additional \$500.0 million, for a total of \$1.0 billion. Negative covenants in our credit agreement could prohibit or limit our ability to access this source of liquidity. As of September 30, 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 September 30, 2025, we had \$3.5 billion remaining authorization available under the share repurchase program that our Board of Directors approved in May 2025. The program does not have an expiration date and can be discontinued at any time. We expect to fund the program through a combination of cash on hand and cash generated by operations.

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 nine months ended September 30, 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 September 30, 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 September 30, 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

Other than as described in Part I—Note L, “Commitments and Contingencies,” to our condensed consolidated financial statements, 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 financial performance, including revenues, costs and expenses, taxes, and other gains and losses;
- product development, including our development timelines, timing of data from our ongoing and planned clinical trials, regulatory authority filings and other submissions for our therapies, including potential to file for accelerated approvals, and communications with regulatory authorities;
- our ability to continue to grow our CF business by increasing the number of people with CF eligible and able to receive our medicines through new approvals and reimbursement agreements, treatment of younger patients, increased survival, and expansion into additional geographies;
- our ability to continue to launch, commercialize and market our products and our ability to obtain label expansions for existing therapies;
- our ability to obtain and maintain adequate coverage, pricing, and reimbursement from third-party payors for our products;
- the data that will be generated by ongoing and planned clinical trials, preclinical and nonclinical studies, and the ability to use that data to advance compounds, continue development or support regulatory filings;
- 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 ability to use our research programs to identify and develop new product candidates to address serious diseases and significant unmet medical needs;
- 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, and other potential business development activities, including the identification of potential collaborative partners or acquisition targets;
- our plans to build and maintain our global supply chains and manufacturing infrastructure and capabilities, including for biologics, cell and gene therapies;
- our ability to expand and protect our intellectual property portfolio and otherwise maintain exclusive rights to products;
- our expectations or beliefs regarding any legal proceedings in which we are involved, including any litigation, arbitration or other similar proceedings involving our products, product candidates or activities;
- 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; 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 “2023 Share Repurchase Program”), pursuant to which we were authorized to repurchase up to \$3.0 billion of our common stock. As of September 30, 2025, we had repurchased the full amount authorized under the 2023 Share Repurchase Program.

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

The table set forth below shows repurchases of securities by us during the three months ended September 30, 2025 under our share repurchase programs.

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)
July 1, 2025 to July 31, 2025	258,000	\$ 466.69	258,000	\$ 4,449,478,512
August 1, 2025 to August 31, 2025	1,221,770	\$ 389.38	1,221,770	\$ 3,973,743,262
September 1, 2025 to September 30, 2025	1,248,390	\$ 390.28	1,248,390	\$ 3,486,517,264
Total	2,728,160	\$ 397.11	2,728,160	\$ 3,486,517,264

(1) Under our share repurchase programs, 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 third quarter of 2025, and one from the second quarter of 2025 that was previously not disclosed, 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
Carmen Bozic <i>EVP, Global Medicines Development and Medical Affairs, Chief Medical Officer</i>	5/30/2025	11/21/2025	19,404
Kristen Ambrose <i>SVP, Chief Accounting Officer</i>	8/08/2025	8/03/2026	7,539 ⁽²⁾
Jeffrey Leiden <i>Executive Chairman</i>	8/06/2025	1/21/2026	144,644
E. Morrow "Morrey" Atkinson, III <i>EVP, Chief Technical Operations Officer, Head of Biopharmaceutical Sciences and Manufacturing Operations</i>	8/11/2025	4/30/2026	12,709 ⁽²⁾
Ourania "Nia" Tatsis <i>EVP, Chief Regulatory and Quality Officer</i>	8/21/2025	7/31/2026	24,301 ⁽²⁾

(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
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.
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.

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

November 4, 2025

By:

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

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

CERTIFICATION

I, Reshma Kewalramani, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Vertex Pharmaceuticals Incorporated;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2025

/s/ Reshma Kewalramani

Reshma Kewalramani
Chief Executive Officer and President

CERTIFICATION

I, Charles F. Wagner, Jr., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Vertex Pharmaceuticals Incorporated;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2025

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

Executive Vice President and Chief Operating & Financial Officer

SECTION 906 CEO/CFO CERTIFICATION

Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code) each of the undersigned officers of Vertex Pharmaceuticals Incorporated, a Massachusetts corporation (the “Company”), does hereby certify, to such officer’s knowledge, that the Quarterly Report on Form 10-Q for the quarter ended September 30, 2025 (the “Form 10-Q”) of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and the information contained in the Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 4, 2025

/s/ Reshma Kewalramani

Reshma Kewalramani

Chief Executive Officer and President

Date: November 4, 2025

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

Executive Vice President and Chief Operating & Financial Officer
