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

FORM 10-Q

☒ QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2023

or

❑ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
FOR THE TRANSITION PERIOD FROM _ TO _

Commission file number 000-19319

Vertex Pharmaceuticals Incorporated
(Exact name of registrant as specified in its charter)

Massachusetts	**04-3039129**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
50 Northern Avenue, Boston, Massachusetts	**02210**
(Address of principal executive offices)	(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	257,551,600	Outstanding at April 25, 2023

VERTEX PHARMACEUTICALS INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED MARCH 31, 2023

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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.

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 development programs, we refer to our compounds 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
(in millions, except per share amounts)(unaudited)

	Three Months Ended March 31,	
	2023	**2022**
Product revenues, net	$ 2,374.8	$ 2,097.5
Costs and expenses:		
Cost of sales	266.9	245.8
Research and development expenses	742.6	601.1
Acquired in-process research and development expenses	347.1	2.0
Selling, general and administrative expenses	241.1	215.2
Change in fair value of contingent consideration	(1.9)	(7.5)
Total costs and expenses	1,595.8	1,056.6
Income from operations	779.0	1,040.9
Interest income	122.6	1.6
Interest expense	(11.4)	(14.9)
Other income (expense), net	1.3	(72.8)
Income before provision for income taxes	891.5	954.8
Provision for income taxes	191.7	192.7
Net income	$ 699.8	$ 762.1
Net income per common share:		
Basic	$ 2.72	$ 2.99
Diluted	$ 2.69	$ 2.96
Shares used in per share calculations:		
Basic	257.4	255.1
Diluted	260.3	257.9

Please refer to Note A, “Basis of Presentation and Accounting Policies,” for an explanation of amounts reclassified from “Research and development expenses” to “Acquired in-process research and development expenses” for the three months ended March 31, 2022.

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

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

	Three Months Ended March 31,	
	2023	**2022**
Net income	$ 699.8	$ 762.1
Other comprehensive income:		
Unrealized holding gains (losses) on marketable securities, net of tax of $(0.8) and zero, respectively	2.9	(2.3)
Unrealized (losses) gains on foreign currency forward contracts, net of tax of $7.4 and $(2.2), respectively	(26.8)	10.1
Foreign currency translation adjustment	10.0	(12.4)
Total other comprehensive loss	(13.9)	(4.6)
Comprehensive income	$ 685.9	$ 757.5

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

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

	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 9,289.9	$ 10,504.0
Marketable securities	1,124.2	274.5
Accounts receivable, net	1,547.8	1,442.2
Inventories	535.1	460.6
Prepaid expenses and other current assets	468.7	553.5
Total current assets	12,965.7	13,234.8
Property and equipment, net	1,111.7	1,108.4
Goodwill	1,088.0	1,088.0
Intangible assets	603.6	603.6
Deferred tax assets	1,359.9	1,246.9
Operating lease assets	336.3	347.4
Long-term marketable securities	1,081.5	112.2
Other assets	427.5	409.6
Total assets	$ 18,974.2	$ 18,150.9
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 323.2	$ 303.9
Accrued expenses	2,326.0	2,126.7
Other current liabilities	377.0	311.5
Total current liabilities	3,026.2	2,742.1
Long-term finance lease liabilities	417.6	430.8
Long-term operating lease liabilities	371.6	379.5
Other long-term liabilities	726.5	685.8
Total liabilities	4,541.9	4,238.2
Commitments and contingencies	—	—
Shareholders' equity:		
Preferred stock, $0.01 par value; 1,000,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.01 par value; 500,000,000 shares authorized, 257,509,761 and 257,011,628 shares issued and outstanding, respectively	2.6	2.6
Additional paid-in capital	7,220.2	7,386.5
Accumulated other comprehensive (loss) income	(13.1)	0.8
Retained earnings	7,222.6	6,522.8
Total shareholders' equity	14,432.3	13,912.7
Total liabilities and shareholders' equity	$ 18,974.2	$ 18,150.9

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

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

	Three Months Ended					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings	Total Shareholders' Equity
	Shares	Amount				
Balance at December 31, 2021	254.5	$ 2.5	$ 6,880.8	$ 15.9	$ 3,200.8	$ 10,100.0
Other comprehensive loss, net of tax	—	—	—	(4.6)	—	(4.6)
Net income	—	—	—	—	762.1	762.1
Common stock withheld for employee tax obligations	(0.5)	(0.0)	(117.5)	—	—	(117.5)
Issuance of common stock under benefit plans	1.6	0.1	36.4	—	—	36.5
Stock-based compensation expense	—	—	130.5	—	—	130.5
Balance at March 31, 2022	255.6	$ 2.6	$ 6,930.2	$ 11.3	$ 3,962.9	$ 10,907.0
Balance at December 31, 2022	257.0	$ 2.6	$ 7,386.5	$ 0.8	$ 6,522.8	$ 13,912.7
Other comprehensive loss, net of tax	—	—	—	(13.9)	—	(13.9)
Net income	—	—	—	—	699.8	699.8
Repurchase of common stock	(0.5)	(0.0)	(135.6)	—	—	(135.6)
Common stock withheld for employee tax obligations	(0.6)	(0.0)	(166.6)	—	—	(166.6)
Issuance of common stock under benefit plans	1.6	0.0	13.1	—	—	13.1
Stock-based compensation expense	—	—	122.8	—	—	122.8
Balance at March 31, 2023	257.5	$ 2.6	$ 7,220.2	$ (13.1)	$ 7,222.6	$ 14,432.3

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

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

	Three Months Ended March 31,	
	2023	**2022**
Cash flows from operating activities:		
Net income	$ 699.8	$ 762.1
Adjustments to reconcile net income to net cash provided by operating activities:		
Stock-based compensation expense	122.4	130.3
Depreciation expense	38.8	35.9
Deferred income taxes	(113.4)	(12.3)
(Gains) losses on equity securities	(6.4)	75.6
Decrease in fair value of contingent consideration	(1.9)	(7.5)
Other non-cash items, net	21.7	4.9
Changes in operating assets and liabilities:		
Accounts receivable, net	(90.5)	(165.2)
Inventories	(82.6)	2.0
Prepaid expenses and other assets	46.2	67.6
Accounts payable	35.7	(14.5)
Accrued expenses	140.7	61.6
Other liabilities	89.4	15.7
Net cash provided by operating activities	899.9	956.2
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(1,816.6)	(117.1)
Maturities of available-for-sale debt securities	50.0	129.7
Purchases of property and equipment	(42.1)	(63.6)
Investment in equity securities	(24.9)	—
Net cash used in investing activities	(1,833.6)	(51.0)
Cash flows from financing activities:		
Issuances of common stock under benefit plans	14.2	33.7
Repurchases of common stock	(132.8)	—
Payments in connection with common stock withheld for employee tax obligations	(166.6)	(117.5)
Payments on finance leases	(10.6)	(12.9)
Other financing activities	1.1	1.3
Net cash used in financing activities	(294.7)	(95.4)
Effect of changes in exchange rates on cash	12.0	(5.9)
Net (decrease) increase in cash, cash equivalents and restricted cash	(1,216.4)	803.9
Cash, cash equivalents and restricted cash—beginning of period	10,512.0	6,800.1
Cash, cash equivalents and restricted cash—end of period	$ 9,295.6	$ 7,604.0
Supplemental disclosure of cash flow information:		
Cash paid for income taxes	$ 120.3	$ 85.0
Cash paid for interest	$ 11.1	$ 13.7

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.

Beginning with the second quarter of 2022, we are separately classifying upfront, contingent milestone, and other payments pursuant to our business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions as "Acquired in-process research and development expenses" in our condensed consolidated statements of income. To conform prior periods to current presentation, we reclassified $2.0 million from "Research and development expenses" to "Acquired in-process research and development expenses" for the three months ended March 31, 2022. Please refer to Note C, "Acquired In-Process Research and Development and Other Arrangements," for further information on these transactions. We have reclassified certain items from the prior year's condensed consolidated balance sheet to conform to the current year's presentation.

Certain information and footnote disclosures normally included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022 (the "2022 Annual Report on Form 10-K") have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of income for the interim periods ended March 31, 2023 and 2022.

The results of operations for the interim periods 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, 2022, which are contained in our 2022 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 and Issued Accounting Standards

For a discussion of recently adopted accounting pronouncements please refer to Note A, "Nature of Business and Accounting Policies," in our 2022 Annual Report on Form 10-K. We do not expect any recently issued accounting standards to have a significant impact on our condensed consolidated financial statements.

Summary of Significant Accounting Policies

Our significant accounting policies are described in Note A, "Nature of Business and Accounting Policies," in our 2022 Annual Report on Form 10-K.

B. Revenue Recognition

Disaggregation of Revenue

Revenues by Product

"Product revenues, net" consisted of the following:

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
TRIKAFTA/KAFTRIO	$ 2,096.7	$ 1,761.6
KALYDECO	125.0	139.0
ORKAMBI	122.5	132.1
SYMDEKO/SYMKEVI	30.6	64.8
Total product revenues, net	$ 2,374.8	$ 2,097.5

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 March 31,	
	2023	2022
	(in millions)	
United States	$ 1,403.8	$ 1,368.2
Outside of the United States		
Europe	807.2	632.3
Other	163.8	97.0
Total product revenues outside of the United States	971.0	729.3
Total product revenues, net	$ 2,374.8	$ 2,097.5

Contract Liabilities

We had contract liabilities of $236.4 million and $159.6 million as of March 31, 2023 and December 31, 2022, respectively, related to annual contracts with government-owned and supported customers in international markets that limit the amount of annual reimbursement we can receive. Upon exceeding the annual reimbursement amount, products are provided free of charge, which is a material right. These contracts include upfront payments and fees. We defer a portion of the consideration received for shipments made up to the annual reimbursement limit as a portion of "Other current liabilities." The deferred amount is recognized as revenue when the free products are shipped. Our 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 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.

C. Acquired In-Process Research and Development and Other Arrangements

We have entered into numerous 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" included $347.1 million for the three months ended March 31, 2023, and $2.0 million, for the three months ended March 31, 2022, related to upfront, contingent milestone, or other payments pursuant to our business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions that qualify as in-process research and development.

Our collaboration, licensing and asset acquisition agreements that had a significant impact on our financial statements for the three months ended March 31, 2023 and 2022, or were new or materially revised during the three months ended March 31, 2023, are described below. Additional agreements were described in Note B, "Acquired In-Process Research and Development and Other Arrangements," of our 2022 Annual Report on Form 10-K.

In-license Agreements

We have entered into several in-license agreements to advance and obtain access to technologies and services related to our research and early-development activities. We are generally required to make an upfront payment upon execution of our license agreements; development, regulatory and commercialization milestones payments upon the achievement of certain product research, development and commercialization objectives; and royalty payments on future sales, if any, of commercial products resulting from our collaborations.

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

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

CRISPR Therapeutics AG - CRISPR-Cas9 Gene-editing Therapies

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, 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 are co-developing and preparing to co-commercialize exagamglogene autotemcel ("exa-cel"), for the treatment of hemoglobinopathies, including treatments for sickle cell disease and transfusion-dependent beta thalassemia. Pursuant to the CRISPR JDCA, we lead global development, manufacturing, and commercialization of exa-cel, 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.

In connection with the CRISPR JDCA, CRISPR has the potential to receive a one-time $200.0 million milestone payment upon receipt of the first marketing approval of exa-cel from the U.S. Food and Drug Administration or the European Commission.

We account for the CRISPR JDCA as a cost-sharing arrangement, with costs incurred related to exa-cel allocated 60% to us and 40% to CRISPR, subject to certain adjustments. During the three months ended March 31, 2023 and 2022, we recognized the net impact of the CRISPR JDCA as "Research and development expenses" of $60.5 million and $36.2

million, respectively, and "Selling, general and administrative expenses" of $16.5 million and $9.8 million, respectively, within our condensed consolidated statements of income.

In March 2023, we entered into a non-exclusive license agreement ("the CRISPR T1D Agreement") for the use of CRISPR's CRISPR-Cas9 gene-editing technology to accelerate the development of our hypoimmune cell therapies for type 1 diabetes. Pursuant to the CRISPR T1D Agreement, we made a $100.0 million upfront payment to CRISPR. CRISPR is also eligible to receive up to an additional $230.0 million in research, development, regulatory and commercial milestones for any products that may result from the agreement, as well as royalties on resulting net product sales. We determined that substantially all the fair value of the collaboration agreement was attributable to in-process research and development and no substantive processes were acquired that would constitute a business. We concluded that there is no alternative future use for the acquired in-process research and development and recorded the upfront payment to "Acquired in-process research and development expenses."

Entrada Therapeutics, Inc.

In February 2023, we closed a strategic collaboration and license agreement (the "Entrada Agreement") with Entrada Therapeutics, Inc. ("Entrada") focused on discovering and developing intracellular Endosomal Escape Vehicle (EEV) therapeutics for myotonic dystrophy type 1 ("DM1"). Upon closing, we made an upfront payment of $225.1 million to Entrada, and purchased $24.9 million of Entrada's common stock in connection with the Entrada Agreement. Entrada is eligible to receive up to an additional $485.0 million in research, development, regulatory and commercial milestones for any products that may result from the Entrada Agreement, as well as royalties on resulting net product sales. We determined that substantially all the fair value of the collaboration agreement was attributable to in-process research and development and no substantive processes were acquired that would constitute a business. We concluded that there is no alternative future use for the acquired in-process research and development and recorded the upfront payment to "Acquired in-process research and development expenses." We recorded the investment in Entrada's common stock at fair value within our condensed consolidated balance sheet within "Marketable securities."

Cystic Fibrosis Foundation

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

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

D. Earnings Per Share

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

	Three Months Ended March 31,	
	2023	2022
	(in millions, except per share amounts)	
Net income	$ 699.8	$ 762.1
Basic weighted-average common shares outstanding	257.4	255.1
Effect of potentially dilutive securities:		
Stock options	1.3	1.3
Restricted stock units (including PSUs)	1.6	1.4
Employee stock purchase program	0.0	0.1
Diluted weighted-average common shares outstanding	260.3	257.9
Basic net income per common share	$ 2.72	$ 2.99
Diluted net income per common share	$ 2.69	$ 2.96

We did not include the securities in the following table in the computation of the diluted net income per common share because the effect would have been anti-dilutive during each period:

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
Stock options	0.0	0.0
Unvested restricted stock units (including PSUs)	0.6	0.6

E. Fair Value Measurements

The following fair value hierarchy is used to classify assets and liabilities based on observable inputs and unobservable inputs used in order 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.

Our investment strategy is focused on capital preservation. We invest in instruments that meet the credit quality standards outlined in our investment policy, which also limits the amount of credit exposure to any one issue or type of instrument. We maintain strategic equity investments separately from the investment policy that governs our other cash, cash equivalents and marketable securities as described in Note F, “Marketable Securities and Equity Investments.” Additionally, we utilize foreign currency forward contracts intended to mitigate the effect of changes in foreign exchange rates on our condensed consolidated statement of income.

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

The following tables set forth our financial assets and liabilities subject to fair value measurements by level within the fair value hierarchy (and does not include $2.1 billion and $3.1 billion of cash as of March 31, 2023 and December 31, 2022, respectively):

	As of March 31, 2023				As of December 31, 2022			
	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:								
Money market funds	$ 4,954.1	$ 4,954.1	$ —	$ —	$ 5,162.6	$ 5,162.6	$ —	$ —
Time deposits	1,600.0	—	1,600.0	—	2,000.0	—	2,000.0	—
U.S. Treasury securities	86.4	86.4	—	—	—	—	—	—
Government-sponsored enterprise securities	129.3	129.3	—	—	—	—	—	—
Corporate debt securities	1.7	—	1.7	—	5.8	—	5.8	—
Commercial paper	408.0	—	408.0	—	204.5	—	204.5	—
Marketable securities:								
Corporate equity securities	148.1	127.2	20.9	—	116.8	88.8	28.0	—
U.S. Treasury securities	276.6	276.6	—	—	—	—	—	—
Government-sponsored enterprise securities	532.4	532.4	—	—	127.1	127.1	—	—
Asset-backed securities	117.9	—	117.9	—	—	—	—	—
Certificates of deposit	44.9	—	44.9	—	—	—	—	—
Corporate debt securities	771.5	—	771.5	—	87.0	—	87.0	—
Commercial paper	314.3	—	314.3	—	55.8	—	55.8	—
Prepaid expenses and other current assets:								
Foreign currency forward contracts	19.5	—	19.5	—	47.5	—	47.5	—
Other assets:								
Foreign currency forward contracts	0.3	—	0.3	—	0.8	—	0.8	—
Total financial assets	$ 9,405.0	$ 6,106.0	$ 3,299.0	$ —	$ 7,807.9	$ 5,378.5	$ 2,429.4	$ —
Financial instruments carried at fair value (liability positions):								
Other current liabilities:								
Foreign currency forward contracts	$ (20.5)	$ —	$ (20.5)	$ —	$ (14.3)	$ —	$ (14.3)	$ —
Contingent consideration	(14.8)	—	—	(14.8)	(14.6)	—	—	(14.6)
Other long-term liabilities:								
Foreign currency forward contracts	(0.5)	—	(0.5)	—	(0.9)	—	(0.9)	—
Contingent consideration	(112.3)	—	—	(112.3)	(114.4)	—	—	(114.4)
Total financial liabilities	$ (148.1)	$ —	$ (21.0)	$ (127.1)	$ (144.2)	$ —	$ (15.2)	$ (129.0)

Please refer to Note F, “Marketable Securities and Equity Investments,” for the carrying amount and related unrealized gains (losses) by type of investment.

Fair Value of Corporate Equity Securities

We classify our investments in publicly traded corporate equity securities as “Marketable securities” on our condensed consolidated balance sheets. Generally, our investments in the common stock of publicly traded companies are valued based on Level 1 inputs because they have readily determinable fair values. However, certain of our investments in publicly traded companies have been or continue to be valued based on Level 2 inputs due to transfer restrictions associated with these investments.

As of March 31, 2023, several of our investments in publicly traded corporate equity securities were subject to contractual sales restrictions expiring in 2023, 2024 and 2025 with a total fair value of $42.8 million. We purchased these investments directly from these publicly traded companies in 2022 and the first quarter of 2023, and do not anticipate any circumstances that would cause these restrictions to lapse prior to the periods listed above.

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

Fair Value of Contingent Consideration

In 2019, we acquired Exonics Therapeutics, Inc. (“Exonics”), a privately-held company focused on creating transformative gene-editing therapies to repair mutations that cause Duchenne muscular dystrophy and other severe neuromuscular diseases, including DM1. Our Level 3 contingent consideration liabilities are related to $678.3 million of development and regulatory milestones potentially payable to former Exonics equity holders. We base our estimates of the probability of achieving the milestones relevant to the fair value of contingent payments, which could include milestone, royalty and option payments, on industry data attributable to rare diseases and our knowledge of the progress and viability of the programs. The discount rates used in the valuation model for contingent payments, which were between 4.7% and 5.8% as of March 31, 2023, 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. Due to the uncertainties associated with development and commercialization of product candidates in the pharmaceutical industry and the effects of changes in other assumptions including discount rates, we expect our estimates regarding the fair value of contingent consideration to change in the future, resulting in adjustments to the fair value of our contingent consideration liabilities, and the effect of any such adjustments could be material.

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

	Three Months Ended March 31, 2023
	(in millions)
Balance at December 31, 2022	$ 129.0
Decrease in fair value of contingent payments	(1.9)
Balance at March 31, 2023	$ 127.1

F. Marketable Securities and Equity Investments

A summary of our cash equivalents and marketable securities, which are recorded at fair value (and do not include $2.1 billion and $3.1 billion of cash as of March 31, 2023 and December 31, 2022, respectively), is shown below:

	As of March 31, 2023				As of December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in millions)							
Cash equivalents:								
Money market funds	$ 4,954.1	$ —	$ —	$ 4,954.1	$ 5,162.6	$ —	$ —	$ 5,162.6
Time deposits	1,600.0	—	—	1,600.0	2,000.0	—	—	2,000.0
U.S. Treasury securities	86.3	0.1	—	86.4	—	—	—	—
Government-sponsored enterprise securities	129.2	0.1	—	129.3	—	—	—	—
Corporate debt securities	1.7	—	—	1.7	5.8	—	—	5.8
Commercial paper	408.0	—	—	408.0	204.5	—	—	204.5
Total cash equivalents	$ 7,179.3	$ 0.2	$ —	$ 7,179.5	$ 7,372.9	$ —	$ —	$ 7,372.9
Marketable securities:								
U.S. Treasury securities	$ 274.8	$ 1.8	$ —	$ 276.6	$ —	$ —	$ —	$ —
Government-sponsored enterprise securities	532.0	0.5	(0.1)	532.4	127.0	0.2	(0.1)	127.1
Asset-backed securities	118.1	—	(0.2)	117.9	—	—	—	—
Certificates of deposit	44.9	—	—	44.9	—	—	—	—
Corporate debt securities	770.0	2.9	(1.4)	771.5	87.2	—	(0.2)	87.0
Commercial paper	314.4	—	(0.1)	314.3	55.8	—	—	55.8
Total marketable debt securities	2,054.2	5.2	(1.8)	2,057.6	270.0	0.2	(0.3)	269.9
Corporate equity securities	129.4	47.4	(28.7)	148.1	104.4	30.9	(18.5)	116.8
Total marketable securities	$ 2,183.6	$ 52.6	$ (30.5)	$ 2,205.7	$ 374.4	$ 31.1	$ (18.8)	$ 386.7

Available-for-sale debt securities were classified on our condensed consolidated balance sheets at fair value as follows:

	As of March 31, 2023	As of December 31, 2022
	(in millions)	
Cash and cash equivalents	$ 5,579.5	$ 5,372.9
Marketable securities	976.1	157.7
Long-term marketable securities	1,081.5	112.2
Total	$ 7,637.1	$ 5,642.8

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

	As of March 31, 2023	As of December 31, 2022
	(in millions)	
Matures within one year	$ 6,555.6	$ 5,530.6
Matures after one year through five years	1,081.5	112.2
Total	$ 7,637.1	$ 5,642.8

We did not record any allowances for credit losses to adjust the fair value of available-for-sale debt securities or gross realized gains or losses in the three months ended March 31, 2023 and 2022. As of March 31, 2023, we held available-for-sale debt securities with a total fair value of $702.7 million that were in unrealized loss positions; however, none of these investments had been in an unrealized loss position for greater than twelve months.

We record changes in the fair value of our investments in corporate equity securities to "Other income (expense), net" in our condensed consolidated statements of income. During the three months ended March 31, 2023 and 2022, our net unrealized gains (losses) on corporate equity securities held at the conclusion of each period were as follows:

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
Net unrealized gains (losses)	$ 6.4	$ (75.6)

As of March 31, 2023, 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 $98.6 million.

G. 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, 2022	$ (25.0)	$ (0.1)	$ 25.9	$ 0.8
Other comprehensive income (loss) before reclassifications	10.0	2.9	(9.6)	3.3
Amounts reclassified from accumulated other comprehensive income (loss)	—	—	(17.2)	(17.2)
Net current period other comprehensive income (loss)	10.0	2.9	(26.8)	(13.9)
Balance at March 31, 2023	$ (15.0)	$ 2.8	$ (0.9)	$ (13.1)
Balance at December 31, 2021	$ (13.6)	$ (0.5)	$ 30.0	$ 15.9
Other comprehensive (loss) income before reclassifications	(12.4)	(2.3)	25.9	11.2
Amounts reclassified from accumulated other comprehensive income (loss)	—	—	(15.8)	(15.8)
Net current period other comprehensive (loss) income	(12.4)	(2.3)	10.1	(4.6)
Balance at March 31, 2022	$ (26.0)	$ (2.8)	$ 40.1	$ 11.3

H. 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 eighteen months. We recognize realized gains and losses for the effective portion of such contracts in "Product revenues, net" in our condensed consolidated statements of income in the same period that we recognize the product revenues that were impacted by the hedged foreign exchange rate changes.

We formally document the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as our risk management objective and strategy for undertaking various hedging

activities, which includes matching all foreign currency forward contracts that are designated as cash flow hedges to forecasted transactions. We also formally assess, both at the hedge's inception and on an ongoing basis, whether the foreign currency forward contracts are highly effective in offsetting changes in cash flows of hedged items on a prospective and retrospective basis. If we were to determine that a (i) foreign currency forward contract is not highly effective as a cash flow hedge, (ii) foreign currency forward contract has ceased to be a highly effective hedge or (iii) forecasted transaction is no longer probable of occurring, we would discontinue hedge accounting treatment prospectively. We measure effectiveness based on the change in fair value of the forward contracts and the fair value of the hypothetical foreign currency forward contracts with terms that match the critical terms of the risk being hedged. As of March 31, 2023, all hedges were determined to be highly effective.

We consider the impact of our counterparties' credit risk on the fair value of the foreign currency forward contracts. As of March 31, 2023 and December 31, 2022, credit risk did not change the fair value of our foreign currency forward contracts.

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

	As of March 31, 2023	As of December 31, 2022
Foreign Currency	**(in millions)**	
Euro	$ 1,325.9	$ 1,497.7
British pound sterling	243.5	247.4
Canadian dollar	231.1	216.3
Australian dollar	180.3	174.9
Swiss Franc	65.9	65.2
Total foreign currency forward contracts	$ 2,046.7	$ 2,201.5

Foreign currency forward contracts - Not designated as hedging instruments

We also enter into foreign currency forward contracts with contractual maturities of less than 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 income (expense), net" in our condensed consolidated statements of income each period. As of March 31, 2023, the notional amount of our outstanding foreign currency forward contracts where hedge accounting under U.S. GAAP is not applied was $541.3 million.

During the three months ended March 31, 2023 and 2022, we recognized the following related to foreign currency forward contracts in our condensed consolidated statements of income:

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
Designated as hedging instruments - Reclassified from AOCI		
Product revenues, net	$ 22.0	$ 20.1
Not designated as hedging instruments		
Other income (expense), net	$ 3.6	$ (8.4)
Total reported in the Condensed Consolidated Statements of Income		
Product revenues, net	$ 2,374.8	$ 2,097.5
Other income (expense), net	$ 1.3	$ (72.8)

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 March 31, 2023			
Assets		**Liabilities**	
Classification	**Fair Value**	**Classification**	**Fair Value**
	(in millions)		
Prepaid expenses and other current assets	$ 19.5	Other current liabilities	$ (20.5)
Other assets	0.3	Other long-term liabilities	(0.5)
Total assets	$ 19.8	Total liabilities	$ (21.0)

As of December 31, 2022			
Assets		**Liabilities**	
Classification	**Fair Value**	**Classification**	**Fair Value**
	(in millions)		
Prepaid expenses and other current assets	$ 47.5	Other current liabilities	$ (14.3)
Other assets	0.8	Other long-term liabilities	(0.9)
Total assets	$ 48.3	Total liabilities	$ (15.2)

As of March 31, 2023, we expect the amounts that are related to foreign exchange forward contracts designated as cash flow hedges under U.S. GAAP recorded in “Prepaid expenses and other current assets” and “Other current liabilities” to be reclassified to earnings within twelve months.

We present the fair value of our foreign currency forward contracts on a gross basis within our condensed consolidated balance sheets. The following table summarizes the potential effect of offsetting derivatives by type of financial instrument designated as cash flow hedges under U.S. GAAP on our condensed consolidated balance sheets:

	As of March 31, 2023				
	Gross Amounts Recognized	**Gross Amounts Offset**	**Gross Amounts Presented**	**Gross Amounts Not Offset**	**Legal Offset**
Foreign currency forward contracts			**(in millions)**		
Total assets	$ 19.8	$ —	$ 19.8	$ (19.8)	$ —
Total liabilities	(21.0)	—	(21.0)	19.8	(1.2)

	As of December 31, 2022				
	Gross Amounts Recognized	**Gross Amounts Offset**	**Gross Amounts Presented**	**Gross Amounts Not Offset**	**Legal Offset**
Foreign currency forward contracts			**(in millions)**		
Total assets	$ 48.3	$ —	$ 48.3	$ (15.2)	$ 33.1
Total liabilities	(15.2)	—	(15.2)	15.2	—

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

I. Inventories

Inventories consisted of the following:

	As of March 31, 2023	As of December 31, 2022
	(in millions)	
Raw materials	$ 58.1	$ 38.1
Work-in-process	341.1	260.7
Finished goods	135.9	161.8
Total	$ 535.1	$ 460.6

J. Stock-based Compensation Expense and Share Repurchase Programs

Stock-based compensation expense

During the three months ended March 31, 2023 and 2022, we recognized the following stock-based compensation expense:

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
Stock-based compensation expense by type of award:		
Restricted stock units (including PSUs)	$ 115.9	$ 118.2
Stock options	1.4	5.5
ESPP share issuances	5.5	6.8
Stock-based compensation expense related to inventories	(0.4)	(0.2)
Total stock-based compensation expense included in "Total costs and expenses"	$ 122.4	$ 130.3
Stock-based compensation expense by line item:		
Cost of sales	$ 1.9	$ 2.2
Research and development expenses	76.3	80.4
Selling, general and administrative expenses	44.2	47.7
Total stock-based compensation expense included in costs and expenses	122.4	130.3
Income tax effect	(40.6)	(36.0)
Total stock-based compensation expense, net of tax	$ 81.8	$ 94.3

Share repurchase program

In February 2023, our Board of Directors approved a share repurchase program (our "Share Repurchase Program"), pursuant to which we are authorized to repurchase up to $3.0 billion of our common stock. Our Share Repurchase Program does not have an expiration date and can be discontinued at any time. During the three months ended March 31, 2023, we repurchased 459,017 shares of our common stock under our Share Repurchase Program for an aggregate of $135.6 million. As of March 31, 2023, a total of $2.9 billion remained authorized for future repurchases.

K. Income Taxes

We are subject to U.S. federal, state, and foreign income taxes. During the three months ended March 31, 2023 and 2022, we recorded the following provisions for income taxes and effective tax rates as compared to our income before provision for income taxes:

	Three Months Ended March 31,	
	2023	2022
	(in millions, except percentages)	
Income before provision for income taxes	$ 891.5	$ 954.8
Provision for income taxes	$ 191.7	$ 192.7
Effective tax rate	21.5 %	20.2 %

Our effective tax rate for the three months ended March 31, 2023 was higher than the U.S. statutory rate primarily due to an increase in our unrecognized tax benefits partially offset by excess tax benefits related to stock-based compensation. Our effective tax rate for the three months ended March 31, 2022 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation.

We have reviewed the tax positions taken, or to be taken, in our tax returns for all tax years currently open to examination by a taxing authority. As of March 31, 2023 and December 31, 2022, we had $232.2 million and $208.5 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.

L. Commitments and Contingencies

2022 Credit Facility

In July 2022, Vertex and certain of its subsidiaries entered into a $500.0 million unsecured revolving facility (the “Credit Agreement”) with Bank of America, N.A., as administrative agent and the lenders referred to therein (the “Lenders”), which matures on July 1, 2027. The Credit Agreement was not drawn upon at closing and we have not drawn upon it to date. Amounts drawn pursuant to the Credit Agreement, if any, will be used for general corporate purposes. Subject to satisfaction of certain conditions, we may request that the borrowing capacity for the Credit Agreement be increased by an additional $500.0 million. Additionally, the Credit Agreement provides a sublimit of $100.0 million for letters of credit.

Any amounts borrowed under the Credit Agreement will bear interest, at our option, at either a base rate or a Secured Overnight Financing Rate (“SOFR”), in each case plus an applicable margin. Under the Credit Agreement, the applicable margins on base rate loans range from 0.000% to 0.500% and the applicable margins on SOFR loans range from 1.000% to 1.500%, in each case based on our consolidated leverage ratio (the ratio of our total consolidated funded indebtedness to our consolidated EBITDA for the most recently completed four fiscal quarter period).

Any amounts borrowed pursuant to the Credit Agreement are guaranteed by certain of our existing and future domestic subsidiaries, subject to certain exceptions.

The Credit Agreement contains customary representations and warranties and affirmative and negative covenants, including a financial covenant to maintain subject to certain limited exceptions, a consolidated leverage ratio of 3.50 to 1.00, subject to an increase to 4.00 to 1.00 following a material acquisition. As of March 31, 2023, we were in compliance with the covenants described above. The Credit Agreement also contains customary events of default. In the case of a continuing event of default, the administrative agent would be entitled to exercise various remedies, including the acceleration of amounts due under outstanding loans.

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

Guaranties and Indemnifications

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

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.

Other Contingencies

We 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 E, "Fair Value Measurements," there were no material contingent liabilities accrued as of March 31, 2023 or December 31, 2022.

M. Additional Cash Flow Information

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

	Three Months Ended March 31,			
	2023		2022	
	Beginning of period	End of period	Beginning of period	End of period
	(in millions)			
Cash and cash equivalents	$ 10,504.0	$ 9,289.9	$ 6,795.0	$ 7,600.1
Prepaid expenses and other current assets	8.0	5.7	5.1	3.9
Cash, cash equivalents and restricted cash per condensed consolidated statement of cash flows	$ 10,512.0	$ 9,295.6	$ 6,800.1	$ 7,604.0

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 four approved medicines that treat the underlying cause of cystic fibrosis ("CF"), a life-threatening genetic disease, and we continue to focus on developing additional treatments for CF. Beyond CF, we have a pipeline that includes mid- and late-stage clinical programs in sickle cell disease, beta thalassemia, acute and neuropathic pain, APOL1-mediated kidney disease, type 1 diabetes, and alpha-1 antitrypsin deficiency, and earlier-stage programs in diseases such as muscular dystrophies.

Our triple combination regimen, TRIKAFTA/KAFTRIO (elexacaftor/tezacaftor/ivacaftor and ivacaftor), was approved in 2019 in the United States ("U.S.") and in 2020 in the European Union ("E.U."). Collectively, our four medicines are being used to treat more than two-thirds of the approximately 88,000 people with CF in North America, Europe, and Australia. We are evaluating our medicines in additional patient populations, including younger children, with the goal of having small molecule treatments for all people who have at least one mutation in their cystic fibrosis transmembrane conductance regulator ("CFTR") gene that is responsive to our CFTR modulators. We also are pursuing messenger ribonucleic acid ("mRNA") and genetic therapies for people with CF who do not make CFTR protein and, as a result, cannot benefit from our current CF medicines.

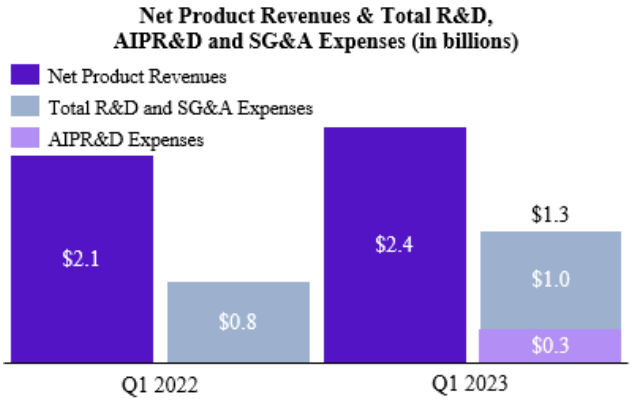
In addition, we are preparing for near-term launches of potential new products in sickle cell disease ("SCD"), beta thalassemia, CF and acute pain. We recently completed regulatory submissions in the U.S., E.U. and the United Kingdom ("U.K.") for exagamglogene autotemcel ("exa-cel") for the treatment of SCD and transfusion-dependent beta thalassemia ("TDT").

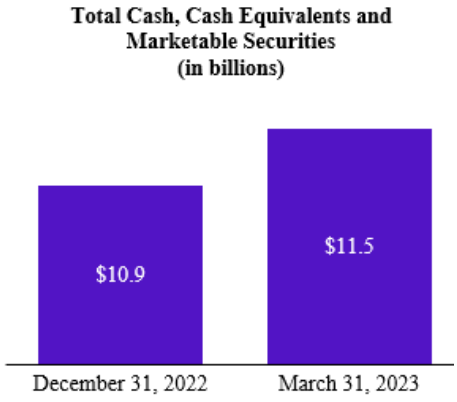
Financial Highlights

Revenues — In the first quarter of 2023, our net product revenues increased to $2.4 billion as compared to $2.1 billion in the first quarter of 2022 primarily due to the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued performance of TRIKAFTA in the U.S.

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 $1.3 billion in the first quarter of 2023 as compared to $818.3 million in the first quarter of 2022. The increase was primarily due to increased AIPR&D and the progression of several product candidates in mid- to late-stage clinical development. Cost of sales was 11% and 12% of our net product revenues in the first quarter of 2023 and 2022, respectively.

Cash — Our total cash, cash equivalents and marketable securities increased to $11.5 billion as of March 31, 2023 as compared to $10.9 billion as of December 31, 2022 primarily due to our net product revenues and operating cash flows partially offset by our upfront payments to Entrada Therapeutics, Inc. ("Entrada") and CRISPR Therapeutics AG ("CRISPR"), and repurchases of our common stock.





Business Updates

Marketed Products

We expect to grow our CF business with (i) continued uptake by patients in countries where we are early in our launch, such as those with recently achieved reimbursement agreements, (ii) label expansions, including into younger patient groups, (iii) the development of mRNA therapies for people with CF who are not eligible for our approved CFTR modulators, and (iv) growth in the number of people living with CF. Recent progress in activities supporting continued uptake and label expansions is included below.

- The U.S. Food and Drug Administration ("FDA") approved TRIKAFTA in children with CF 2 to 5 years of age with at least one F508del mutation in the CFTR gene or a mutation in the CFTR gene that is responsive to TRIKAFTA. We have completed regulatory submissions with the European Medicines Agency ("EMA"), the Medicines and Healthcare products Regulatory Agency ("MHRA") in the U.K., Health Canada, and the Therapeutic Goods Administration in Australia for the use of KAFTRIO/TRIKAFTA in children with CF 2 to 5 years of age.
- We received a positive opinion from the EMA Committee for Medicinal Products for Human Use for the use of ORKAMBI in children with CF 1 year to less than 2 years of age with two copies of the F508del mutation in the CFTR gene.
- We have submitted a supplemental new drug application to the FDA and marketing authorization applications ("MAAs") to the EMA, MHRA, and Health Canada for the use of KALYDECO in children with CF from 1 month to less than 4 months of age. KALYDECO has been granted Priority Review designation in the U.S.
- TRIKAFTA/KAFTRIO is approved and reimbursed or accessible in more than 30 countries outside the U.S.

Potential Near-Term Launch Opportunities

We are preparing for the following near-term launches of potential new products:

Exa-cel in SCD and TDT

- We recently completed rolling submissions of our biologics licensing applications ("BLAs") for exa-cel in the U.S. Exa-cel has been granted Fast Track, Regenerative Medicine Advanced Therapy, Orphan Drug and Rare Pediatric Disease designations in the U.S.
- In the fourth quarter of 2022, we completed submissions for exa-cel with the EMA and MHRA in the E.U. and U.K., respectively. The EMA and MHRA have validated the MAAs, and exa-cel has been granted Priority Medicines and Orphan Drug designations in the E.U. In the U.K., exa-cel has been granted an Innovation Passport under the Innovative Licensing and Access Pathway from the MHRA.

Vanzacaftor/tezacaftor/deutivacaftor in CF

- In the fourth quarter of 2022, we completed enrollment in the pivotal SKYLINE 102 and SKYLINE 103 clinical trials, which evaluate the efficacy and safety of our new once-daily investigational triple combination vanzacaftor/tezacaftor/deutivacaftor relative to TRIKAFTA in people with CF 12 years of age and older. We expect to complete these clinical trials by the end of 2023. In parallel, we have initiated a study of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age, known as the RIDGELINE clinical trial, and we expect to complete this clinical trial by the end of 2023.

VX-548 in Acute Pain

- We continue to enroll the Phase 3 pivotal program for our lead compound, VX-548, for the treatment of moderate to severe acute pain, and we expect to complete the pivotal program in late 2023 or early 2024. VX-548 has been granted Breakthrough Therapy and Fast Track designations in the U.S. for moderate to severe acute pain.

Pipeline

We continue to advance a pipeline of potentially transformative small molecule, mRNA, and cell and genetic therapies aimed at treating serious diseases. Recent and anticipated progress in activities supporting these efforts is included below.

Cystic Fibrosis

- In collaboration with Moderna, we are developing VX-522, a CFTR mRNA therapeutic for the treatment of people with CF who do not produce any CFTR protein. We have initiated a single-ascending dose clinical trial for VX-522 in people with CF, which is active and enrolling patients. We expect to complete this single-ascending dose clinical trial and initiate a multiple-ascending dose clinical trial in 2023. The FDA has granted Fast Track designation for VX-522.

Beta Thalassemia and Sickle Cell Disease

- We are evaluating the use of exa-cel, a non-viral *ex vivo* CRISPR gene-editing therapy, for the treatment of SCD and TDT.
- Dosing in the Phase 3 CLIMB-111 and CLIMB 121 clinical trials evaluating exa-cel continues, as does the CLIMB 131 long-term follow-up clinical trial in patients 12 years of age and older.
- Two additional Phase 3 clinical trials evaluating exa-cel in children with SCD or TDT 5 to 11 years of age are ongoing.

Neuropathic Pain

- We have discovered multiple selective small molecule inhibitors of NaV1.8, with the objective of creating a new class of pain medicines that provide effective non-opioid pain relief, without abuse potential.
- We continue to enroll and dose patients in a Phase 2 dose-ranging clinical trial evaluating VX-548 in diabetic peripheral neuropathy, a common form of chronic peripheral neuropathic pain. We expect to complete this clinical trial in late 2023 or early 2024.

APOL1-Mediated Kidney Disease

- Inaxaplin is our small molecule for the treatment of APOL1-mediated kidney disease (“AMKD”), including APOL1-mediated focal segmental glomerulosclerosis (“FSGS”). We continue to enroll and dose patients in the pivotal program for inaxaplin, a single Phase 2/3 clinical trial, and we expect to complete the Phase 2B dose-ranging portion of the trial in 2023.
- The FDA granted inaxaplin Breakthrough Therapy designation for APOL1-mediated FSGS and the EMA granted inaxaplin Orphan Drug and PRIME designations for AMKD.

Type 1 Diabetes

- VX-880 is a stem cell-derived, fully differentiated, insulin-producing islet cell replacement therapy, using standard immunosuppression to protect the implanted cells. A clinical trial is ongoing to evaluate VX-880 as a potential treatment for type 1 diabetes (“T1D”), and proof-of-concept has been achieved. We have completed enrollment and dosing in Part B of the Phase 1/2 clinical trial and we expect to begin Part C of the trial with concurrent dosing. The EMA granted VX-880 PRIME designation.
- We continue to advance additional programs in T1D, in which these same stem cell-derived, fully differentiated, insulin-producing islet cells are encapsulated and implanted in an immunoprotective device or are modified to produce hypoimmune cells with the goal of eliminating the need for immunosuppression. The Investigational New Drug Application in the U.S., and the Clinical Trial Application (“CTA”) in Canada for VX-264, the cells and device program, have been cleared, and we plan to begin enrollment and dosing in a Phase 1/2 clinical trial in the near term.
- Our hypoimmune cell research program continues to progress.

- A Phase 1/2 clinical trial evaluating VCTX-211, a hypoimmune cell program using ViaCyte cells that originated under the CRISPR and ViaCyte collaboration, is active and enrolling patients.

Alpha-1 Antitrypsin Deficiency

- We are working to address the underlying genetic cause of alpha-1 antitrypsin ("AAT") deficiency ("AATD"). We are developing novel small molecule correctors of Z-AAT protein folding, with the goal of enabling the secretion of functional AAT into the blood and addressing both the lung and the liver aspects of AATD. We continue to enroll and dose healthy volunteers in a Phase 1 clinical trial for VX-634, which is the first in a series of next-wave investigational molecules with significantly improved potency and drug-like properties as compared to our previous AAT correctors.
- We initiated a second Phase 2 clinical trial of VX-864, a first-generation AAT corrector, to assess the impact of longer-term treatment on the liver, as well as the levels of functional AAT in the plasma. This Phase 2 clinical trial continues to enroll and dose patients.

Additional Earlier Stage R&D Programs

Consistent with our overall strategy, we are taking a portfolio approach to all of our programs, with additional assets in CF, SCD, TDT, pain, AMKD, T1D and AATD in earlier stages of development.

We are also advancing preclinical assets in new disease areas, such as DMD and myotonic dystrophy type 1 ("DM1"). Additionally, we are working on preclinical molecules with the potential to expand our leadership in existing disease areas, including assets targeting gentler conditioning for exa-cel and NaV1.7 inhibitors in pain.

Investments in External Innovation

Recent investments in external innovation are included below:

- We announced a new licensing agreement for the use of CRISPR's gene-editing technology, known as CRISPR/Cas9, to accelerate the development of our hypoimmune cell therapies for T1D.
- We closed our previously announced strategic collaboration and licensing agreement with Entrada Therapeutics, Inc. ("Entrada"), focused on discovering and developing intracellular Endosomal Escape Vehicle ("EEV") therapeutics for DM1.

Our Business Environment

Our net product revenues come 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, including through label expansions, expanded reimbursement, and the development of new medicines. We are advancing our pipeline of product candidates for the treatment of serious diseases outside of CF. 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 from each program, spanning multiple modalities, into early clinical trials to obtain patient data that can inform selection of the most promising compounds for later-stage development, and to inform discovery and development of additional compounds. 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 do advance into development never

receive marketing approval. Our investments in product candidates are subject to considerable risks. We closely monitor the results of our discovery, research, clinical trials and nonclinical studies and frequently evaluate our product development 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.

Our business also requires ensuring appropriate manufacturing and reimbursement 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 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 new approved product, we adapt our supply chain for existing products to include additional formulations or to increase scale of production for existing products as needed. The processes for cell and genetic therapies can be more complex than those required for small molecule drugs and require additional investments in different systems, equipment, facilities and expertise. We are focused on ensuring the stability of the supply chains for our current products, as well as for our pipeline programs.

Sales of our products depend, to a large degree, on the extent to which our products are reimbursed by third-party payors, such as government health programs, commercial insurance and managed health care organizations. Reimbursement for our products, including our potential pipeline therapies, cannot be assured and may take significant periods of time to obtain. We dedicate substantial management and other resources to obtain and maintain appropriate levels of reimbursement for our products from third-party payors, including governmental organizations in the U.S. and ex-U.S. markets.

In the U.S., we have worked successfully with third-party payors to promptly obtain appropriate levels of reimbursement for our CF medicines. 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 our medicines provide and provide patients with appropriate levels of access to our medicines now and in the future. We cannot, however, predict how recent changes in the law, including through the Inflation Reduction Act of 2022, will affect our ability to negotiate successfully with third-party payors in the future. 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 expect to continue to focus significant resources to obtain expanded reimbursement for our CF medicines and, ultimately, 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.

In the second quarter of 2022, we acquired Catalyst Biosciences, Inc.'s, or Catalyst's, portfolio of protease medicines that target the complement system and related intellectual property for $60.0 million. In the third quarter of 2022, we acquired ViaCyte, a privately held biotechnology company with intellectual property, tools, technologies and assets with potential to accelerate development of our T1D programs, for $315.0 million.

We expect to continue to identify and evaluate potential acquisitions and may include larger transactions or later-stage assets.

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 Arbor Biotechnologies, Inc., CRISPR, Entrada, ImmunoGen, Inc., Kymera Therapeutics, Inc., Mammoth Biosciences, Inc., Moderna, Inc., Obsidian Therapeutics, Inc., and Verve Therapeutics, Inc. Generally, when we in-license a technology or product candidate, we make upfront payments to the collaborator, assume the costs of the program and/or agree to make contingent payments, which could consist of milestone, royalty and option

payments. Most of these collaboration payments are expensed as AIPR&D; 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.

In February 2023, we closed our strategic collaboration and licensing agreement with Entrada. Upon closing, we made an upfront payment of $225.1 million to Entrada, and purchased $24.9 million of Entrada's common stock.

In March 2023, we entered into a non-exclusive license agreement for the use of CRISPR's CRISPR-Cas9 gene-editing technology to accelerate the development of our hypoimmune cell therapies for T1D, and made a $100.0 million upfront payment to CRISPR.

Acquired In-Process Research and Development Expenses

In the first quarter of 2023 and 2022, our AIPR&D included $347.1 million, which primarily related to our upfront payments to Entrada and CRISPR, and $2.0 million, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions, including the collaborations, licenses of third-party technologies, and asset acquisitions described above.

Out-License Agreements

We also have out-licensed internally developed programs to collaborators who are leading the development of these programs. Pursuant to these out-licensing arrangements, our collaborators are responsible for the research, development, and commercialization costs associated with these programs, and we are entitled to receive contingent milestone and/or royalty payments. As a result, we do not expect to incur significant expenses in connection with these programs and have the potential for future collaborative and royalty revenues resulting from these programs. None of our out-license agreements had a significant impact on our condensed consolidated statement of income during the first quarter of 2023 and 2022.

Strategic Equity Investments

In connection with our business development activities, we have periodically made equity investments in our collaborators. As of March 31, 2023, we held strategic equity investments in certain public and private companies, and we expect to make additional strategic equity investments in the future. We invest the majority of our cash, cash equivalents and marketable securities in instruments that meet specific credit quality standards and limit our exposure to any one issue or type of instrument. Our strategic equity investments are maintained and managed separately from our other cash, cash equivalents and marketable securities. As discussed below in "Other Income (Expense), Net" in our *Results of Operations*, any changes in the fair value of equity investments with readily determinable fair values (including publicly traded securities) are recorded to other income (expense), net in our condensed consolidated statement of income.

RESULTS OF OPERATIONS

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages and per share amounts)		
Product revenues, net	$ 2,374.8	$ 2,097.5	13%
Operating costs and expenses	1,595.8	1,056.6	51%
Income from operations	779.0	1,040.9	(25)%
Other non-operating income (expense), net	112.5	(86.1)	**
Provision for income taxes	191.7	192.7	(1)%
Net income	$ 699.8	$ 762.1	(8)%
Net income per diluted common share	$ 2.69	$ 2.96	
Diluted shares used in per share calculations	260.3	257.9	

** Not meaningful

Product Revenues, net

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
TRIKAFTA/KAFTRIO	$ 2,096.7	$ 1,761.6	19%
KALYDECO	125.0	139.0	(10)%
ORKAMBI	122.5	132.1	(7)%
SYMDEKO/SYMKEVI	30.6	64.8	(53)%
Product revenues, net	$ 2,374.8	$ 2,097.5	13%

In the first quarter of 2023, our net product revenues increased by $277.3 million, or 13%, as compared to the first quarter of 2022, primarily due to the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and the continued performance of TRIKAFTA in the U.S. Decreases in revenues for our products other than TRIKAFTA/KAFTRIO were primarily the result of patients switching from these medicines to TRIKAFTA/KAFTRIO.

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

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
United States	$ 1,403.8	$ 1,368.2	3%
ex-U.S.	971.0	729.3	33%
Product revenues, net	$ 2,374.8	$ 2,097.5	13%

Operating Costs and Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Cost of sales	$ 266.9	$ 245.8	9%
Research and development expenses	742.6	601.1	24%
Acquired in-process research and development expenses	347.1	2.0	**
Selling, general and administrative expenses	241.1	215.2	12%
Change in fair value of contingent consideration	(1.9)	(7.5)	**
Total costs and expenses	$ 1,595.8	$ 1,056.6	51%

** Not meaningful

Cost of Sales

Our cost of sales primarily consists of third-party royalties payable on net sales of our products as well as the cost of producing inventories. Pursuant to our agreement with the Cystic Fibrosis Foundation our tiered third-party royalties on sales of TRIKAFTA/KAFTRIO, SYMDEKO/SYMKEVI, KALYDECO, and ORKAMBI, calculated as a percentage of net sales, range from the single digits to the sub-teens, with royalties on sales of TRIKAFTA/KAFTRIO lower than for our other products. Over the last several years, our cost of sales has been increasing due to increased net product revenues. Our cost of sales as a percentage of our net product revenues was 11% and 12% in the first quarter of 2023 and 2022, respectively.

Research and Development Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Research expenses	$ 166.8	$ 143.8	16%
Development expenses	575.8	457.3	26%
Total research and development expenses	$ 742.6	$ 601.1	24%

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

Since January 2021, we have incurred approximately $6.8 billion in total research and development and AIPR&D expenses associated with product discovery and development. The successful development of our product candidates is highly uncertain and subject to a number of risks. In addition, the duration of clinical trials may vary substantially according to the type, complexity and novelty of the product candidate and the disease indication being targeted. The FDA and comparable agencies in foreign countries impose substantial requirements on the introduction of therapeutic pharmaceutical products, typically requiring lengthy and detailed laboratory and clinical testing procedures, sampling activities and other costly and time-consuming procedures. Data obtained from nonclinical and clinical activities at any step in the testing process may be adverse and lead to discontinuation or redirection of development activities. Data obtained from these activities also are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The duration and cost of discovery, nonclinical studies and clinical trials may vary significantly over the life of a project and are difficult to predict. Therefore, accurate and meaningful estimates of the ultimate costs to bring our product candidates to market are not available.

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

Research Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Research Expenses:			
Salary and benefits	$ 45.5	$ 40.2	13%
Stock-based compensation expense	20.2	22.9	(12)%
Outsourced services and other direct expenses	53.6	39.5	36%
Infrastructure costs	47.5	41.2	15%
Total research expenses	$ 166.8	$ 143.8	16%

Our research expenses have been increasing over the last several years as we have invested in our pipeline and expanded our cell and genetic therapy capabilities, resulting in increased headcount, outside services and other direct expenses and infrastructure costs associated with our research facilities. We expect to continue to invest in our research programs with a focus on creating transformative medicines for serious diseases.

Development Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Development Expenses:			
Salary and benefits	$ 144.2	$ 109.9	31%
Stock-based compensation expense	56.1	57.5	(2)%
Outsourced services and other direct expenses	295.3	212.7	39%
Infrastructure costs	80.2	77.2	4%
Total development expenses	$ 575.8	$ 457.3	26%

Our development expenses increased by $118.5 million, or 26%, in the first quarter of 2023 as compared to the first quarter of 2022, primarily due to increased costs to support clinical trials associated with our advancing pipeline programs, including pain, our CF triple combination of vanzacaftor/tezacaftor/deutivacaftor, exa-cel and T1D. We are significantly investing in internal headcount, leveraging outsourced services, and investing in infrastructure to support these programs.

Acquired In-process Research and Development Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Acquired in-process research and development expenses	$ 347.1	$ 2.0	**

** Not meaningful

AIPR&D in the first quarter of 2023 was primarily related to our upfront payments of $225.1 million to Entrada and $100.0 million to CRISPR. Our AIPR&D has historically fluctuated, and is expected to continue to fluctuate, from one period

to another due to upfront, contingent milestone, and other payments pursuant to our existing and future business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions.

Selling, General and Administrative Expenses

	Three Months Ended March 31,		
	2023	2022	Change
	(in millions, except percentages)		
Selling, general and administrative expenses	$ 241.1	$ 215.2	12%

Selling, general and administrative expenses increased by 12% in the first quarter of 2023 as compared to the first quarter of 2022, primarily due to the continued investment to support the commercialization of our medicines and increased support for our pipeline product candidates.

Contingent Consideration

The fair value of our contingent consideration decreased by $1.9 million and $7.5 million in the first quarter of 2023 and 2022, respectively.

Other Non-Operating Income (Expense), Net

Interest Income

Interest income increased to $122.6 million in the first quarter of 2023, as compared to $1.6 million in the first quarter of 2022, primarily due to increased market interest rates and increased cash equivalents and available-for-sale debt securities. 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 $11.4 million and $14.9 million in the first quarter of 2023 and 2022, respectively. The majority of our interest expense in these periods was related to imputed interest expense associated with our leased corporate headquarters in Boston.

Other Income (Expense), Net

Other income (expense), net was income of $1.3 million and expense of $72.8 million in the first quarter of 2023 and 2022, respectively. The vast majority of these amounts relate to net unrealized gains or losses resulting from changes in the fair value of our strategic equity investments. As of March 31, 2023, the fair value of our investments in publicly traded companies was $148.1 million. To the extent that we continue to hold strategic equity investments in publicly traded companies, we will record other income (expense) related to these investments on a quarterly basis. We expect that due to the volatility of the stock price of biotechnology companies, our other income (expense), net will fluctuate in future periods based on increases or decreases in the fair value of our strategic equity investments.

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 allocation of our taxable earnings among multiple jurisdictions, the amount and characterization of our research and development expenses, the levels of certain deductions and credits, adjustments to the value of our uncertain tax positions, acquisitions and third-party collaboration and licensing transactions.

We recorded provisions for income taxes of $191.7 million and $192.7 million in the first quarter of 2023 and 2022, respectively. Our effective tax rate of 21.5% in the first quarter of 2023 was higher than the U.S. statutory rate primarily due to an increase in our unrecognized tax benefits partially offset by excess tax benefits related to stock-based compensation. Our effective tax rate of 20.2% in the first quarter of 2022 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation.

LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of March 31, 2023 and December 31, 2022:

	As of March 31, 2023	As of December 31, 2022	Change
	(in millions, except percentages)		
Total cash, cash equivalents and marketable securities	$ 11,495.6	$ 10,890.7	6%
Working Capital:			
Total current assets	$ 12,965.7	$ 13,234.8	(2)%
Total current liabilities	(3,026.2)	(2,742.1)	10%
Total working capital	$ 9,939.5	$ 10,492.7	(5)%

Working Capital

As of March 31, 2023, total working capital was $9.9 billion, which represented a decrease of $553.2 million from $10.5 billion as of December 31, 2022. The decrease in total working capital in the first quarter of 2023 was primarily related to increased investment in long-term marketable securities and repurchases of our common stock partially offset by $899.9 million of cash provided by operations.

Cash Flows

	Three Months Ended March 31,	
	2023	2022
	(in millions)	
Net cash provided by (used in):		
Operating activities	$ 899.9	$ 956.2
Investing activities	$ (1,833.6)	$ (51.0)
Financing activities	$ (294.7)	$ (95.4)

Operating Activities

Cash provided by operating activities were $899.9 million in the first quarter of 2023 as compared to $956.2 million in the first quarter of 2022, primarily due to a $62.3 million decrease in net income resulting from increased AIPR&D partially offset by increased net product revenues.

Investing Activities

Cash used in investing activities were $1.8 billion and $51.0 million in the first quarter of 2023 and 2022, respectively. In the first quarter of 2023, the largest portion of our investing activities were purchases of marketable securities. In the first quarter of 2022, the largest portion of our investing activities were purchases of property and equipment.

Financing Activities

Cash used in financing activities were $294.7 million and $95.4 million in the first quarter of 2023 and 2022, respectively. In the first quarter of 2023, the largest portions of our financing activities were payments related to our employee stock benefit plans and repurchases of our common stock pursuant to our Share Repurchase Program. In the first quarter of 2022, the largest portion of our financing activities were payments related to our employee stock benefit plans.

Sources and Uses of Liquidity

As of March 31, 2023, we had total cash, cash equivalents and marketable securities of $11.5 billion, which represented an increase of $604.9 million from $10.9 billion as of December 31, 2022. We intend to rely on our existing cash, cash equivalents and marketable securities together with cash flows from product sales as our primary source of liquidity.

We expect that cash flows from our product sales together with our current cash, cash equivalents and 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 product sales, and the potential introduction of one or more of our other product candidates to the market, our business development activities, and the number, breadth, cost and prospects of our research and development programs.

Credit Facilities & Financing Strategy

We may borrow up to a total of $500.0 million pursuant to a revolving credit facility that we entered into in July 2022 and could repay and reborrow amounts under this revolving credit agreement without penalty. Subject to certain conditions, we could request that the borrowing capacity be increased by an additional $500.0 million, for a total of $1.0 billion. Negative covenants in our credit agreement could prohibit or limit our ability to access this source of liquidity. As of March 31, 2023, 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.
- Facility and finance lease obligations.
- Royalties we pay to the Cystic Fibrosis Foundation on sales of our CF products.
- Cash paid for income taxes.

In addition, we have significant potential future capital requirements including:

- We have entered into certain business development-related agreements with third parties that include the funding of certain research, development, 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. Our obligation to fund these research and development and commercialization efforts and to pay these potential milestone and royalties is contingent upon continued involvement in the programs and/or the lack of any adverse events that could cause the discontinuance of the programs associated with our collaborations, licensing arrangements and acquisitions. We may enter into additional business development transactions, including acquisitions, collaborations, licensing arrangements and equity investments, that require additional capital.
- To the extent we borrow amounts under our existing credit agreement, we would be required to repay any outstanding principal amounts in 2027.
- As of March 31, 2023, we had $2.9 billion remaining authorization available under our Share Repurchase Program.

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, 2022, which was filed with the Securities and Exchange Commission, or SEC, on February 10, 2023.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

Our discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the U.S. The

preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate. During the three months ended March 31, 2023, 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, 2022, which was filed with the SEC on February 10, 2023.

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, 2022, which was filed with the SEC on February 10, 2023.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management (under the supervision and with the participation of our chief executive officer and chief financial officer), after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q, has concluded that, based on such evaluation, as of March 31, 2023 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Controls Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended March 31, 2023 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. Other Information

Item 1. Legal Proceedings

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors

Information regarding risk factors appears in Part I, Item 1A. “Risk Factors” of our Annual Report on Form 10-K for the year ended December 31, 2022, which was filed with the SEC on February 10, 2023. There have been no material changes from the risk factors previously disclosed in the Annual Report on Form 10-K.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q and, in particular, our Management’s Discussion and Analysis of Financial Condition and Results of Operations set forth in Part I, Item 2, contain a number of forward-looking statements. Forward-looking statements are not purely historical and may be accompanied by words such as “anticipates,” “may,” “forecasts,” “expects,” “intends,” “plans,” “potentially,” “believes,” “seeks,” “estimates,” and other words and terms of similar meaning. Such statements may relate to:

- our expectations regarding the amount of, timing of, and trends with respect to our financial performance, including revenues, costs and expenses, and other gains and losses;
- our expectations regarding clinical trials, including expectations for patient enrollment, development timelines, the expected timing of data from our ongoing and planned clinical trials, and regulatory authority filings and other submissions for our therapies;
- our ability to maintain and obtain adequate reimbursement for our products, our ability to launch, commercialize and market our products or any of our other therapies for which we obtain regulatory approval and our ability to obtain label expansions for existing therapies;
- our expectations regarding our ability to continue to grow our CF business by increasing the number of people with CF eligible and able to receive our medicines, providing improved treatment options for people who are already eligible for one of our medicines, and pursuing genetic therapies for people with CF who cannot currently benefit from our medicines;
- the data that will be generated by ongoing and planned clinical trials and the ability to use that data to advance compounds, continue development or support regulatory filings;
- our beliefs regarding the support provided by clinical trials and preclinical and nonclinical studies of our therapies for further investigation, clinical trials or potential use as a treatment;
- our plans to continue investing in our research and development programs, including anticipated timelines for our programs, and our strategy to develop our pipeline programs, alone or with third party-collaborators;
- our beliefs regarding the approximate patient populations for the disease areas on which we focus;
- the potential benefits and therapeutic scope of our acquisitions and collaborations, including the expectation that the use of CRISPR/Cas9 gene editing technology will accelerate the development of our T1D hypoimmune cell therapy program;
- the establishment, development and maintenance of collaborative relationships, including potential milestone payments or other obligations;
- potential business development activities, including the identification of potential collaborative partners or acquisition targets;
- our ability to expand and protect our intellectual property portfolio and otherwise maintain exclusive rights to products;
- potential fluctuations in foreign currency exchange rates and the effectiveness of our foreign currency management program;
- our expectations regarding cash generated by operations, our cash balance and expected generation and interest income;

- our expectations regarding our provision for or benefit from income taxes and the utilization of our deferred tax assets;
- our ability to use our research programs to identify and develop new product candidates to address serious diseases and significant unmet medical needs;
- our plans to expand, strengthen, and invest in our global supply chains and manufacturing infrastructure and capabilities, including for cell and gene therapies; and
- our liquidity and our expectations regarding the possibility of raising additional capital.

Forward-looking statements are subject to certain risks, uncertainties, or other factors that are difficult to predict and could cause actual events or results to differ materially from those indicated in any such statements. These risks, uncertainties, and other factors include, but are not limited to, those described in our "Risk Factors" in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2022, which was filed with the SEC on February 10, 2023, and those described from time to time in our future reports filed with the Securities and Exchange Commission.

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

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Issuer Repurchases of Equity Securities

In February 2023, our Board of Directors approved a share repurchase program (our "Share Repurchase Program"), pursuant to which we are authorized to repurchase up to $3.0 billion of our common stock. Our Share Repurchase Program does not have an expiration date and can be discontinued at any time. The table set forth below shows repurchases of securities by us during the three months ended March 31, 2023 under our Share Repurchase Program.

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs (1)	Approximate Dollar Value of Shares that May Yet be Purchased Under the Plans or Programs (1)
January 1, 2023 to January 31, 2023	N/A	N/A	N/A	N/A
February 1, 2023 to February 28, 2023	172,784	$ 293.43	172,784	$ 2,949,299,420
March 1, 2023 to March 31, 2023	286,233	$ 296.56	286,233	$ 2,864,413,554
Total	459,017	$ 295.38	459,017	$ 2,864,413,554

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

Item 6. Exhibits

Exhibit Number	Exhibit Description
10.1	Amendment No. 2 to Employment Agreement, between Jeffrey M. Leiden and Vertex Pharmaceuticals Incorporated, dated as of February 8, 2023. (incorporated by reference to Exhibit 10.23 to our Annual Report on Form 10-K filed on February 10, 2023).*
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.

* Management contract, compensatory plan or agreement.

SIGNATURES

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

Vertex Pharmaceuticals Incorporated

May 2, 2023 By: /s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

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

Exhibit 31.1

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: May 2, 2023

/s/ Reshma Kewalramani

Reshma Kewalramani
Chief Executive Officer and President

Exhibit 31.2

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: May 2, 2023

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.
Executive Vice President and Chief Financial Officer

Exhibit 32.1

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 March 31, 2023 (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: May 2, 2023

/s/ Reshma Kewalramani

Reshma Kewalramani
Chief Executive Officer and President

Date: May 2, 2023

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.
Executive Vice President and Chief Financial Officer