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

FORM 10-Q

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2022

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
FOR THE TRANSITION PERIOD FROM_ TO _
Commission file number 000-19319

Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts
(State or other jurisdiction of incorporation or organization)

04-3039129
(I.R.S. Employer Identification No.)

50 Northern Avenue, Boston, Massachusetts
(Address of principal executive offices)

02210
(Zip Code)

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

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

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

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

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

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

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

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

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

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

Common Stock, par value \$0.01 per share

256,691,452

Outstanding at October 21, 2022

VERTEX PHARMACEUTICALS INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED SEPTEMBER 30, 2022

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

“Vertex®,” “KALYDECO®,” “ORKAMBI®,” “SYMDEKO®,” “SYMKEVI®,” “TRIKAFTA®” and “KAFTRIO®” are registered trademarks of Vertex. Other brands, names and trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners.

We use the brand name for our products when we refer to the product that has been approved and with respect to the indications on the approved label. Otherwise, including in discussions of our cystic fibrosis 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 Operations
(unaudited)
(in millions, except per share amounts)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|-------------|--|-------------|
| | 2022 | 2021 | 2022 | 2021 |
| Revenues: | | | | |
| Product revenues, net | \$ 2,334.3 | \$ 1,984.1 | \$ 6,628.0 | \$ 5,500.8 |
| Other revenues | — | — | — | 1.0 |
| Total revenues | 2,334.3 | 1,984.1 | 6,628.0 | 5,501.8 |
| Costs and expenses: | | | | |
| Cost of sales | 289.4 | 236.5 | 797.0 | 656.8 |
| Research and development expenses | 645.0 | 467.0 | 1,846.2 | 1,370.0 |
| Acquired in-process research and development expenses | 29.0 | 26.7 | 92.9 | 986.8 |
| Selling, general and administrative expenses | 246.8 | 198.2 | 677.3 | 584.9 |
| Change in fair value of contingent consideration | (2.6) | 1.2 | (59.3) | (1.1) |
| Total costs and expenses | 1,207.6 | 929.6 | 3,354.1 | 3,597.4 |
| Income from operations | 1,126.7 | 1,054.5 | 3,273.9 | 1,904.4 |
| Interest income | 46.2 | 1.1 | 58.6 | 3.7 |
| Interest expense | (13.7) | (15.2) | (43.2) | (46.4) |
| Other income (expense), net | 17.2 | 42.4 | (133.7) | (2.2) |
| Income before provision for income taxes | 1,176.4 | 1,082.8 | 3,155.6 | 1,859.5 |
| Provision for income taxes | 245.9 | 230.9 | 652.5 | 287.5 |
| Net income | \$ 930.5 | \$ 851.9 | \$ 2,503.1 | \$ 1,572.0 |
| Net income per common share: | | | | |
| Basic | \$ 3.63 | \$ 3.30 | \$ 9.78 | \$ 6.08 |
| Diluted | \$ 3.59 | \$ 3.28 | \$ 9.68 | \$ 6.03 |
| Shares used in per share calculations: | | | | |
| Basic | 256.5 | 257.9 | 255.8 | 258.7 |
| Diluted | 259.5 | 259.7 | 258.7 | 260.9 |

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 and nine months ended September 30, 2021.

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

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|---|-----------------|--|-------------------|
| | 2022 | 2021 | 2022 | 2021 |
| Net income | \$ 930.5 | \$ 851.9 | \$ 2,503.1 | \$ 1,572.0 |
| Other comprehensive income: | | | | |
| Unrealized holding losses on marketable securities, net | (0.6) | (0.0) | (3.6) | (0.3) |
| Unrealized gains on foreign currency forward contracts, net of tax of \$(16.0), \$(9.6), \$(34.3) and \$(21.2), respectively | 58.8 | 34.7 | 128.1 | 77.0 |
| Foreign currency translation adjustment | (18.1) | 2.0 | (42.8) | 3.3 |
| Total other comprehensive income | 40.1 | 36.7 | 81.7 | 80.0 |
| Comprehensive income | <u>\$ 970.6</u> | <u>\$ 888.6</u> | <u>\$ 2,584.8</u> | <u>\$ 1,652.0</u> |

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

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

| | September 30, 2022 | December 31, 2021 |
|--|-----------------------|----------------------|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 9,171.5 | \$ 6,795.0 |
| Marketable securities | 599.2 | 729.9 |
| Accounts receivable, net | 1,385.2 | 1,136.8 |
| Inventories | 388.2 | 353.1 |
| Prepaid expenses and other current assets | 726.9 | 545.8 |
| Total current assets | 12,271.0 | 9,560.6 |
| Property and equipment, net | 1,118.7 | 1,094.1 |
| Goodwill | 1,075.2 | 1,002.2 |
| Intangible assets | 603.6 | 400.0 |
| Deferred tax assets | 1,162.7 | 934.5 |
| Operating lease assets | 342.7 | 330.3 |
| Other assets | 132.5 | 110.8 |
| Total assets | \$ 16,706.4 | \$ 13,432.5 |
| Liabilities and Shareholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 126.9 | \$ 195.0 |
| Accrued expenses | 2,264.4 | 1,678.6 |
| Other current liabilities | 218.0 | 268.4 |
| Total current liabilities | 2,609.3 | 2,142.0 |
| Long-term finance lease liabilities | 442.3 | 509.8 |
| Long-term operating lease liabilities | 382.3 | 377.4 |
| Long-term contingent consideration | 127.2 | 186.5 |
| Other long-term liabilities | 115.7 | 116.8 |
| Total liabilities | 3,676.8 | 3,332.5 |
| 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, 256,645,737 and 254,479,046 shares issued and outstanding, respectively | 2.6 | 2.5 |
| Additional paid-in capital | 7,225.5 | 6,880.8 |
| Accumulated other comprehensive income | 97.6 | 15.9 |
| Retained earnings | 5,703.9 | 3,200.8 |
| Total shareholders' equity | 13,029.6 | 10,100.0 |
| Total liabilities and shareholders' equity | \$ 16,706.4 | \$ 13,432.5 |

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

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

| | Three Months Ended | | | | | |
|--|--------------------|--------|----------------------------------|---|----------------------|----------------------------------|
| | Common Stock | | Additional Paid-in Capital | Accumulated Other Comprehensive (Loss) Income | Retained Earnings | Total Shareholders' Equity |
| | Shares | Amount | | | | |
| Balance at June 30, 2021 | 259.1 | \$ 2.6 | \$ 7,640.2 | \$ (25.2) | \$ 1,578.8 | \$ 9,196.4 |
| Other comprehensive income, net of tax | — | — | — | 36.7 | — | 36.7 |
| Net income | — | — | — | — | 851.9 | 851.9 |
| Repurchase of common stock | (3.3) | (0.0) | (642.2) | — | — | (642.2) |
| Common stock withheld for employee tax obligations | (0.1) | (0.0) | (28.6) | — | — | (28.6) |
| Issuance of common stock under benefit plans | 0.5 | 0.0 | 12.9 | — | — | 12.9 |
| Stock-based compensation expense | — | — | 103.7 | — | — | 103.7 |
| Balance at September 30, 2021 | 256.2 | \$ 2.6 | \$ 7,086.0 | \$ 11.5 | \$ 2,430.7 | \$ 9,530.8 |
| Balance at June 30, 2022 | 256.0 | \$ 2.6 | \$ 7,100.0 | \$ 57.5 | \$ 4,773.4 | \$ 11,933.5 |
| Other comprehensive income, net of tax | — | — | — | 40.1 | — | 40.1 |
| Net income | — | — | — | — | 930.5 | 930.5 |
| Common stock withheld for employee tax obligations | (0.2) | (0.0) | (48.0) | — | — | (48.0) |
| Issuance of common stock under benefit plans | 0.8 | 0.0 | 38.2 | — | — | 38.2 |
| Stock-based compensation expense | — | — | 135.3 | — | — | 135.3 |
| Balance at September 30, 2022 | 256.6 | \$ 2.6 | \$ 7,225.5 | \$ 97.6 | \$ 5,703.9 | \$ 13,029.6 |

| | Nine Months Ended | | | | | |
|--|-------------------|--------|----------------------------------|---|----------------------|----------------------------------|
| | Common Stock | | Additional Paid-in Capital | Accumulated Other Comprehensive (Loss) Income | Retained Earnings | Total Shareholders' Equity |
| | Shares | Amount | | | | |
| Balance at December 31, 2020 | 259.9 | \$ 2.6 | \$ 7,894.0 | \$ (68.5) | \$ 858.7 | \$ 8,686.8 |
| Other comprehensive income, net of tax | — | — | — | 80.0 | — | 80.0 |
| Net income | — | — | — | — | 1,572.0 | 1,572.0 |
| Repurchase of common stock | (5.3) | (0.0) | (1,067.2) | — | — | (1,067.2) |
| Common stock withheld for employee tax obligations | (0.6) | (0.0) | (134.2) | — | — | (134.2) |
| Issuance of common stock under benefit plans | 2.2 | 0.0 | 66.8 | — | — | 66.8 |
| Stock-based compensation expense | — | — | 326.6 | — | — | 326.6 |
| Balance at September 30, 2021 | 256.2 | \$ 2.6 | \$ 7,086.0 | \$ 11.5 | \$ 2,430.7 | \$ 9,530.8 |
| Balance at December 31, 2021 | 254.5 | \$ 2.5 | \$ 6,880.8 | \$ 15.9 | \$ 3,200.8 | \$ 10,100.0 |
| Other comprehensive income, net of tax | — | — | — | 81.7 | — | 81.7 |
| Net income | — | — | — | — | 2,503.1 | 2,503.1 |
| Common stock withheld for employee tax obligations | (0.7) | (0.0) | (169.9) | — | — | (169.9) |
| Issuance of common stock under benefit plans | 2.8 | 0.1 | 135.2 | — | — | 135.3 |
| Stock-based compensation expense | — | — | 379.4 | — | — | 379.4 |
| Balance at September 30, 2022 | 256.6 | \$ 2.6 | \$ 7,225.5 | \$ 97.6 | \$ 5,703.9 | \$ 13,029.6 |

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

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

| | Nine Months Ended September 30, | |
|---|--|-------------------|
| | 2022 | 2021 |
| Cash flows from operating activities: | | |
| Net income | \$ 2,503.1 | \$ 1,572.0 |
| Adjustments to reconcile net income to net cash provided by operating activities: | | |
| Stock-based compensation expense | 379.8 | 322.8 |
| Depreciation expense | 109.9 | 91.8 |
| Decrease in fair value of contingent consideration | (59.3) | (1.1) |
| Deferred income taxes | (424.0) | (112.7) |
| Gains (losses) on equity securities | 143.1 | (5.0) |
| Other non-cash items, net | (32.8) | 20.5 |
| Changes in operating assets and liabilities: | | |
| Accounts receivable, net | (368.8) | (231.2) |
| Inventories | (58.1) | (65.8) |
| Prepaid expenses and other assets | (41.9) | (107.7) |
| Accounts payable | (39.1) | (22.0) |
| Accrued expenses | 980.3 | 254.2 |
| Other liabilities | (40.7) | (67.3) |
| Net cash provided by operating activities | <u>3,051.5</u> | <u>1,648.5</u> |
| Cash flows from investing activities: | | |
| Purchases of available-for-sale debt securities | (417.8) | (447.8) |
| Maturities of available-for-sale debt securities | 435.9 | 452.1 |
| Payment to acquire ViaCyte, Inc., net of cash acquired | (295.9) | — |
| Purchases of property and equipment | (171.1) | (173.2) |
| Investment in equity securities and notes receivable | (47.8) | (38.0) |
| Net cash used in investing activities | <u>(496.7)</u> | <u>(206.9)</u> |
| Cash flows from financing activities: | | |
| Issuances of common stock under benefit plans | 134.7 | 67.3 |
| Repurchases of common stock | — | (1,057.2) |
| Payments in connection with common stock withheld for employee tax obligations | (169.9) | (134.2) |
| Payments on finance leases | (75.1) | (34.6) |
| Proceeds from finance leases | — | 12.6 |
| Other financing activities | 2.4 | 4.3 |
| Net cash used in financing activities | <u>(107.9)</u> | <u>(1,141.8)</u> |
| Effect of changes in exchange rates on cash | <u>(70.0)</u> | <u>(8.5)</u> |
| Net increase in cash, cash equivalents and restricted cash | 2,376.9 | 291.3 |
| Cash, cash equivalents and restricted cash—beginning of period | 6,800.1 | 5,988.9 |
| Cash, cash equivalents and restricted cash—end of period | <u>\$ 9,177.0</u> | <u>\$ 6,280.2</u> |
| Supplemental disclosure of cash flow information: | | |
| Cash paid for interest | \$ 41.1 | \$ 42.7 |
| Cash paid for income taxes | \$ 840.1 | \$ 381.5 |

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 operations. To conform prior periods to current presentation, we reclassified \$26.7 million and \$986.8 million from “Research and development expenses” to “Acquired in-process research and development expenses” for the three and nine months ended September 30, 2021, respectively. Please refer to Note C, “Acquired In-Process Research and Development and Other Arrangements,” for further information on these transactions.

Certain information and footnote disclosures normally included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2021 (the “2021 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 operations for the interim periods ended September 30, 2022 and 2021.

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, 2021, which are contained in our 2021 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 2021 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 2021 Annual Report on Form 10-K.

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

B. Revenue Recognition

Disaggregation of Revenue

Revenues by Product

Product revenues, net consisted of the following:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|-----------------------------|---|-------------------|--|-------------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| TRIKAFTA/KAFTRIO | \$ 2,010.5 | \$ 1,555.8 | \$ 5,665.3 | \$ 4,004.6 |
| SYMDEKO/SYMKEVI | 38.2 | 81.4 | 145.7 | 340.0 |
| ORKAMBI | 146.2 | 184.5 | 399.9 | 624.2 |
| KALYDECO | 139.4 | 162.4 | 417.1 | 532.0 |
| Total product revenues, net | <u>\$ 2,334.3</u> | <u>\$ 1,984.1</u> | <u>\$ 6,628.0</u> | <u>\$ 5,500.8</u> |

Product Revenues by Geographic Location

Total net product revenues by geographic region, based on the location of the customer, consisted of the following:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|-------------------|--|-------------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| United States | \$ 1,455.6 | \$ 1,382.9 | \$ 4,238.9 | \$ 3,893.2 |
| Outside of the United States | | | | |
| Europe | 730.5 | 518.8 | 2,018.3 | 1,382.7 |
| Other | 148.2 | 82.4 | 370.8 | 224.9 |
| Total product revenues outside of the United States | <u>878.7</u> | <u>601.2</u> | <u>2,389.1</u> | <u>1,607.6</u> |
| Total product revenues, net | <u>\$ 2,334.3</u> | <u>\$ 1,984.1</u> | <u>\$ 6,628.0</u> | <u>\$ 5,500.8</u> |

Contract Liabilities

We had contract liabilities of \$119.5 million and \$171.7 million as of September 30, 2022 and December 31, 2021, 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.

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

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 \$29.0 million and \$92.9 million for the three and nine months ended September 30, 2022, respectively, and \$26.7 million and \$986.8 million, for the three and nine months ended September 30, 2021, respectively, related to upfront, contingent milestone, or other payments pursuant to our business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions.

Our collaboration, licensing and asset acquisition agreements that had a significant impact on our financial statements for the three and nine months ended September 30, 2022 and 2021, or were new or materially revised during the three and nine months ended September 30, 2022, are described below. Additional agreements were described in Note B, “Collaborative and Other Arrangements,” of our 2021 Annual Report on Form 10-K.

In-license Agreements

We have entered into a number of in-license agreements in order 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; the required length of notice is dependent on whether any product developed under the license agreement has received marketing approval. 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 net product sales.

In 2017, we entered into a joint development and commercialization agreement with CRISPR pursuant to the terms of the CRISPR Agreement (the “Original CTX001 JDCA”), under which we and CRISPR were co-developing and preparing to co-commercialize exagamglogene autotemcel (“exa-cel”), formerly known as CTX001, for the treatment of hemoglobinopathies, including treatments for sickle cell disease and transfusion-dependent beta thalassemia.

In the second quarter of 2021, we and CRISPR amended and restated the Original CTX001 JDCA (the “A&R JDCA”), pursuant to which the parties agreed to, among other things, (a) adjust the governance structure for the collaboration and adjust the responsibilities of each party thereunder; (b) adjust the allocation of net profits and net losses between the parties; and (c) exclusively license (subject to CRISPR’s reserved rights to conduct certain activities) certain intellectual property rights to us relating to the products that may be researched, developed, manufactured and commercialized under such agreement.

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

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

In connection with the A&R JDCA, we made a \$900.0 million upfront payment to CRISPR in the second quarter of 2021. We concluded that we did not have any alternative future use for the acquired in-process research and development and recorded this upfront payment to “Acquired in-process research and development expenses.” CRISPR has the potential to receive an additional 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 and CRISPR shared equally all expenses incurred under the Original CTX001 JDCA. On July 1, 2021, the net profits and net losses incurred with respect to exa-cel pursuant to the A&R JDCA began to be allocated 60% to us and 40% to CRISPR, subject to certain adjustments, while all other product candidates and products continue to have net profits and net losses shared equally between the parties. We concluded that the Original CTX001 JDCA and the A&R JDCA are cost-sharing arrangements, which result in the net impact of the arrangements being recorded in “Total costs and expenses” within our condensed consolidated statements of operations. During the three and nine months ended September 30, 2022 and 2021, we recognized the following amounts in total, not including amounts recorded to “Acquired in-process research and development expenses,” related to these agreements:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|----------------------------------|---------|---------------------------------|----------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| Total expenses incurred under the Original CTX001 JDCA and A&R JDCA | \$ 98.2 | \$ 58.7 | \$ 259.8 | \$ 147.4 |
| Vertex’s share recognized in “Total costs and expenses” in our condensed consolidated statements of operations | \$ 59.0 | \$ 35.2 | \$ 155.9 | \$ 79.6 |

Verve Therapeutics, Inc.

In July 2022, we entered into a research collaboration with Verve Therapeutics, Inc. (“Verve”) focused on discovering and developing an in vivo gene-editing program for a liver disease. Under the terms of the agreement, we made a \$25.0 million upfront payment to Verve and purchased \$35.0 million of Verve’s common stock. 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.” The investment in Verve’s common stock is recorded at fair value on our condensed consolidated balance sheet within “Marketable securities.”

Asset Acquisition

Catalyst Biosciences, Inc. - Complement 3 Degradar Program

In May 2022, pursuant to an asset purchase agreement, we acquired Catalyst Biosciences, Inc.’s portfolio of protease medicines that target the complement system (the “complement portfolio”) and related intellectual property, including CB 2782-PEG, which is a pre-clinical complement component 3 degrader program for geographic atrophy in dry age-related macular degeneration. We determined that substantially all the fair value acquired was concentrated in the CB-2782 PEG in-process research and development assets, which did not constitute a business, and for which we determined there was no alternative future use. As a result, we recorded our \$60.0 million upfront payment to “Acquired in-process research and development expenses” in the nine months ended September 30, 2022.

Cystic Fibrosis Foundation

We have a research, development and commercialization agreement that was originally entered into in 2004 with the Cystic Fibrosis Foundation, as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc. This agreement was most recently amended in 2016. Pursuant to the agreement, as amended, we agreed to pay royalties ranging from low-single digits to mid-single digits on potential sales of certain compounds first synthesized and/or tested between March 1, 2014 and August 31, 2016, including elxacaftor, and 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 KALYDECO (ivacaftor), ORKAMBI (lumacaftor in combination with ivacaftor) and SYMDEKO/SYMKEVI (tezacaftor in combination with ivacaftor). For combination products, such as ORKAMBI, SYMDEKO/SYMKEVI and TRIKAFTA/KAFTRIO (elxacaftor/tezacaftor/ivacaftor and ivacaftor), 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.”

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D. Business Combination

On September 27, 2022, we acquired all outstanding shares of ViaCyte, Inc. (“ViaCyte”), a privately held biotechnology company primarily focused on delivering novel stem cell-derived cell replacement therapies as a functional cure for type 1 diabetes, in exchange for \$315.0 million. ViaCyte’s intellectual property and assembled workforce complement our ongoing programs and have the potential to produce therapies for patients with type 1 diabetes. We accounted for the transaction as a business combination, and allocated the purchase price to the following assets acquired and liabilities assumed:

| | As of September 27, 2022 | |
|---------------------------|---------------------------------|-------|
| | (in millions) | |
| Cash and cash equivalents | \$ | 19.1 |
| Goodwill | | 73.0 |
| Intangible asset | | 216.6 |
| Net other assets | | 6.3 |
| Total purchase price | \$ | 315.0 |

The “Goodwill” represents the difference between the fair value of the consideration transferred and the fair value of the assets acquired and liabilities assumed. The goodwill is attributable to (i) the technological expertise in cell therapy of ViaCyte’s assembled workforce, (ii) the potential synergies from combining ViaCyte’s technology with our clinical development capabilities and (iii) the potential future benefits for other therapeutic programs that the acquired technology could be used for, but did not meet the definition of an in-process research and development asset. None of the goodwill is expected to be deductible for income tax purposes.

The “Intangible asset” is an in-process research and development asset of \$216.6 million related to ViaCyte’s technology and intellectual property associated with stem cell differentiation and manufacturing. The fair value of the intangible asset was determined through a discounted cash flow analysis using the relief from royalty method, which estimated the cost savings associated with owning an asset instead of paying a royalty to the previous owner. The relief from royalty method utilized Level 3 fair value inputs including (i) estimated future product sales, which were calculated using, among other assumptions, an estimated probability of obtaining marketing approval for the asset, (ii) estimated after-tax royalty savings expected from ownership of the asset, and (iii) an appropriate discount and tax rate.

As of September 30, 2022, our analysis of the fair value of certain assets acquired, including certain tax analyses, related to the ViaCyte business combination is preliminary and will be finalized during the measurement period of up to one year from the acquisition date. The financial results of ViaCyte did not have a material effect on our condensed consolidated statement of operations in the three and nine months ended September 30, 2022.

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E. 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 September 30, | | Nine Months Ended September 30, | |
|--|---|----------|---------------------------------|------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions, except per share amounts) | | | |
| Net income | \$ 930.5 | \$ 851.9 | \$ 2,503.1 | \$ 1,572.0 |
| Basic weighted-average common shares outstanding | 256.5 | 257.9 | 255.8 | 258.7 |
| Effect of potentially dilutive securities: | | | | |
| Stock options | 1.4 | 1.0 | 1.4 | 1.1 |
| Restricted stock units (including PSUs) | 1.6 | 0.8 | 1.4 | 1.1 |
| Employee stock purchase program | 0.0 | 0.0 | 0.1 | 0.0 |
| Diluted weighted-average common shares outstanding | 259.5 | 259.7 | 258.7 | 260.9 |
| Basic net income per common share | \$ 3.63 | \$ 3.30 | \$ 9.78 | \$ 6.08 |
| Diluted net income per common share | \$ 3.59 | \$ 3.28 | \$ 9.68 | \$ 6.03 |

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 September 30, | | Nine Months Ended September 30, | |
|--|----------------------------------|------|---------------------------------|------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| Stock options | — | 1.1 | 0.0 | 0.7 |
| Unvested restricted stock units (including PSUs) | 0.0 | 0.2 | 0.2 | 0.4 |

F. 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 investments separately from the investment policy that governs our other cash, cash equivalents and marketable securities as described in Note G, "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 operations.

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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 \$3.5 billion and \$3.3 billion of cash as of September 30, 2022 and December 31, 2021, respectively):

| | As of September 30, 2022 | | | | As of December 31, 2021 | | | |
|--|--------------------------|-------------------|-------------------|-------------------|-------------------------|-------------------|-----------------|-------------------|
| | 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 | \$ 3,610.9 | \$ 3,610.9 | \$ — | \$ — | \$ 3,478.1 | \$ 3,478.1 | \$ — | \$ — |
| Time deposits | 2,000.0 | — | 2,000.0 | — | — | — | — | — |
| Commercial paper | 18.2 | — | 18.2 | — | — | — | — | — |
| Marketable securities: | | | | | | | | |
| Corporate equity securities | 122.8 | 78.2 | 44.6 | — | 230.9 | 230.9 | — | — |
| U.S. Treasury securities | 130.0 | 130.0 | — | — | 86.4 | 86.4 | — | — |
| Government-sponsored enterprise securities | 10.5 | 10.5 | — | — | 69.0 | 69.0 | — | — |
| Corporate debt securities | 66.8 | — | 66.8 | — | 90.9 | — | 90.9 | — |
| Commercial paper | 269.1 | — | 269.1 | — | 252.7 | — | 252.7 | — |
| Prepaid expenses and other current assets: | | | | | | | | |
| Foreign currency forward contracts | 191.6 | — | 191.6 | — | 44.5 | — | 44.5 | — |
| Other assets: | | | | | | | | |
| Foreign currency forward contracts | 9.1 | — | 9.1 | — | 2.0 | — | 2.0 | — |
| Total financial assets | <u>\$ 6,429.0</u> | <u>\$ 3,829.6</u> | <u>\$ 2,599.4</u> | <u>\$ —</u> | <u>\$ 4,254.5</u> | <u>\$ 3,864.4</u> | <u>\$ 390.1</u> | <u>\$ —</u> |
| Financial instruments carried at fair value (liability positions): | | | | | | | | |
| Other current liabilities: | | | | | | | | |
| Foreign currency forward contracts | \$ (0.0) | \$ — | \$ (0.0) | \$ — | \$ (5.6) | \$ — | \$ (5.6) | \$ — |
| Long-term contingent consideration | (127.2) | — | — | (127.2) | (186.5) | — | — | (186.5) |
| Other long-term liabilities: | | | | | | | | |
| Foreign currency forward contracts | (0.0) | — | (0.0) | — | (2.7) | — | (2.7) | — |
| Total financial liabilities | <u>\$ (127.2)</u> | <u>\$ —</u> | <u>\$ (0.0)</u> | <u>\$ (127.2)</u> | <u>\$ (194.8)</u> | <u>\$ —</u> | <u>\$ (8.3)</u> | <u>\$ (186.5)</u> |

Please refer to Note G, “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. Please refer to Note G, “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 myotonic dystrophy type 1. 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 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 5.2% and 5.7% as of September 30, 2022, 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

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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:

| | Nine Months Ended September 30, 2022 |
|---|---|
| | (in millions) |
| Balance at December 31, 2021 | \$ 186.5 |
| Decrease in fair value of contingent payments | (59.3) |
| Balance at September 30, 2022 | <u>\$ 127.2</u> |

The decrease in fair value of contingent consideration during the nine months ended September 30, 2022 was primarily due to a revision to the scope of certain acquired gene-editing programs in the second quarter of 2022.

Fair Value of Intangible Assets

As of September 30, 2022 and December 31, 2021, we had \$603.6 million and \$400.0 million, respectively, of in-process research and development intangible assets classified as “Intangible assets” on our condensed consolidated balance sheets. In the third quarter of 2022, we recorded a \$216.6 million in-process research and development intangible asset resulting from our acquisition of ViaCyte, which is described in Note D, “Business Combination.” In the nine months ended September 30, 2022, we recorded a \$13.0 million impairment of an in-process research and development intangible asset to “Research and development expenses,” due to a decision to revise the scope of certain acquired gene-editing programs.

G. Marketable Securities and Equity Investments

A summary of our cash equivalents and marketable securities, which are recorded at fair value (and do not include \$3.5 billion and \$3.3 billion of cash as of September 30, 2022 and December 31, 2021, respectively), is shown below:

| | As of September 30, 2022 | | | | As of December 31, 2021 | | | |
|--|---------------------------------|-------------------------------|--------------------------------|-------------------|--------------------------------|-------------------------------|--------------------------------|-------------------|
| | 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 | \$ 3,610.9 | \$ — | \$ — | \$ 3,610.9 | \$ 3,478.1 | \$ — | \$ — | \$ 3,478.1 |
| Time deposits | 2,000.0 | — | — | 2,000.0 | — | — | — | — |
| Commercial paper | 18.2 | — | — | 18.2 | — | — | — | — |
| Total cash equivalents | \$ 5,629.1 | \$ — | \$ — | \$ 5,629.1 | \$ 3,478.1 | \$ — | \$ — | \$ 3,478.1 |
| Marketable securities: | | | | | | | | |
| U.S. Treasury securities | \$ 131.7 | \$ — | \$ (1.7) | \$ 130.0 | \$ 86.6 | \$ — | \$ (0.2) | \$ 86.4 |
| Government-sponsored enterprise securities | 10.6 | — | (0.1) | 10.5 | 69.0 | — | — | 69.0 |
| Corporate debt securities | 67.8 | — | (1.0) | 66.8 | 91.1 | — | (0.2) | 90.9 |
| Commercial paper | 270.4 | — | (1.3) | 269.1 | 252.8 | — | (0.1) | 252.7 |
| Total marketable debt securities | 480.5 | — | (4.1) | 476.4 | 499.5 | — | (0.5) | 499.0 |
| Corporate equity securities | 104.4 | 29.2 | (10.8) | 122.8 | 69.4 | 167.1 | (5.6) | 230.9 |
| Total marketable securities | \$ 584.9 | \$ 29.2 | \$ (14.9) | \$ 599.2 | \$ 568.9 | \$ 167.1 | \$ (6.1) | \$ 729.9 |

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

| | As of September 30, 2022 | | As of December 31, 2021 | |
|---------------------------|--------------------------|---------|-------------------------|---------|
| | (in millions) | | | |
| Cash and cash equivalents | \$ | 3,629.1 | \$ | 3,478.1 |
| Marketable securities | | 476.4 | | 499.0 |
| Total | \$ | 4,105.5 | \$ | 3,977.1 |

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

| | As of September 30, 2022 | | As of December 31, 2021 | |
|---|--------------------------|---------|-------------------------|---------|
| | (in millions) | | | |
| Matures within one year | \$ | 4,091.4 | \$ | 3,912.3 |
| Matures after one year through five years | | 14.1 | | 64.8 |
| Total | \$ | 4,105.5 | \$ | 3,977.1 |

We have a limited number of available-for-sale debt securities in insignificant loss positions as of September 30, 2022, which we do not intend to sell and have concluded we will not be required to sell before recovery of the amortized costs for the investments at maturity. 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 and nine months ended September 30, 2022 and 2021.

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 operations. During the three and nine months ended September 30, 2022 and 2021, our net unrealized gains (losses) on corporate equity securities held at the conclusion of each period were as follows:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|-------------------------------|---|-------------|--|-------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| Net unrealized gains (losses) | \$ | 16.7 | \$ | 46.7 |
| | | | \$ | (143.1) |
| | | | \$ | 5.0 |

As of September 30, 2022, 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.

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H. Accumulated Other Comprehensive Income (Loss)

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

| | Foreign Currency Translation Adjustment | Unrealized Holding Gains (Losses), Net of Tax | | | Total |
|---|---|--|---|----------------|-------|
| | | On Available-For- Sale Debt Securities | On Foreign Currency Forward Contracts | | |
| | | (in millions) | | | |
| Balance at December 31, 2021 | \$ (13.6) | \$ (0.5) | \$ 30.0 | \$ 15.9 | |
| Other comprehensive (loss) income before reclassifications | (42.8) | (3.6) | 230.2 | 183.8 | |
| Amounts reclassified from accumulated other comprehensive income (loss) | — | — | (102.1) | (102.1) | |
| Net current period other comprehensive (loss) income | (42.8) | (3.6) | 128.1 | 81.7 | |
| Balance at September 30, 2022 | <u>\$ (56.4)</u> | <u>\$ (4.1)</u> | <u>\$ 158.1</u> | <u>\$ 97.6</u> | |
| Balance at December 31, 2020 | \$ (15.6) | \$ 0.3 | \$ (53.2) | \$ (68.5) | |
| Other comprehensive income (loss) before reclassifications | 3.3 | (0.3) | 46.2 | 49.2 | |
| Amounts reclassified from accumulated other comprehensive income (loss) | — | — | 30.8 | 30.8 | |
| Net current period other comprehensive income (loss) | 3.3 | (0.3) | 77.0 | 80.0 | |
| Balance at September 30, 2021 | <u>\$ (12.3)</u> | <u>\$ 0.0</u> | <u>\$ 23.8</u> | <u>\$ 11.5</u> | |

I. 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 operations 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 September 30, 2022, all hedges were determined to be highly effective.

We consider the impact of our counterparties' credit risk on the fair value of the foreign currency forward contracts. As of September 30, 2022 and December 31, 2021, credit risk did not change the fair value of our foreign currency forward contracts.

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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:

| Foreign Currency | As of September 30, 2022 | | As of December 31, 2021 | |
|--|--------------------------|---------|-------------------------|---------|
| | (in millions) | | | |
| Euro | \$ | 1,081.9 | \$ | 1,364.5 |
| Canadian dollar | | 192.3 | | 89.9 |
| British pound sterling | | 183.5 | | 287.7 |
| Australian dollar | | 150.8 | | 96.3 |
| Swiss Franc | | 59.2 | | 54.1 |
| Total foreign currency forward contracts | \$ | 1,667.7 | \$ | 1,892.5 |

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 operations each period. As of September 30, 2022, the notional amount of our outstanding foreign currency forward contracts where hedge accounting under U.S. GAAP is not applied was \$641.6 million.

During the three and nine months ended September 30, 2022 and 2021, we recognized the following related to foreign currency forward contracts in our condensed consolidated statements of operations:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|----------------------------------|------------|---------------------------------|------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| <i>Designated as hedging instruments - Reclassified from AOCI</i> | | | | |
| Product revenues, net | \$ 65.2 | \$ (5.2) | \$ 130.3 | \$ (39.3) |
| <i>Not designated as hedging instruments</i> | | | | |
| Other income (expense), net | \$ (22.1) | \$ (0.4) | \$ (38.9) | \$ (9.4) |
| <i>Total reported in the Condensed Consolidated Statement of Operations</i> | | | | |
| Product revenues, net | \$ 2,334.3 | \$ 1,984.1 | \$ 6,628.0 | \$ 5,500.8 |
| Other income (expense), net | \$ 17.2 | \$ 42.4 | \$ (133.7) | \$ (2.2) |

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

| As of September 30, 2022 | | | |
|---|------------|-----------------------------|------------|
| Assets | | Liabilities | |
| Classification | Fair Value | Classification | Fair Value |
| (in millions) | | | |
| Prepaid expenses and other current assets | \$ 191.6 | Other current liabilities | \$ (0.0) |
| Other assets | 9.1 | Other long-term liabilities | (0.0) |
| Total assets | \$ 200.7 | Total liabilities | \$ (0.0) |

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As of December 31, 2021

| Assets | | Liabilities | |
|---|-------------------|-----------------------------|-------------------|
| Classification | Fair Value | Classification | Fair Value |
| (in millions) | | | |
| Prepaid expenses and other current assets | \$ 44.5 | Other current liabilities | \$ (5.6) |
| Other assets | 2.0 | Other long-term liabilities | (2.7) |
| Total assets | <u>\$ 46.5</u> | Total liabilities | <u>\$ (8.3)</u> |

As of September 30, 2022, 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 September 30, 2022 | | | | | |
|---|-------------------------------------|---------------------------------|------------------------------------|-------------------------------------|---------------------|
| | Gross Amounts Recognized | Gross Amounts Offset | Gross Amounts Presented | Gross Amounts Not Offset | Legal Offset |
| (in millions) | | | | | |
| Foreign currency forward contracts | | | | | |
| Total assets | \$ 200.7 | \$ — | \$ 200.7 | \$ (0.0) | \$ 200.7 |
| Total liabilities | (0.0) | — | (0.0) | 0.0 | — |

| As of December 31, 2021 | | | | | |
|---|-------------------------------------|---------------------------------|------------------------------------|-------------------------------------|---------------------|
| | Gross Amounts Recognized | Gross Amounts Offset | Gross Amounts Presented | Gross Amounts Not Offset | Legal Offset |
| (in millions) | | | | | |
| Foreign currency forward contracts | | | | | |
| Total assets | \$ 46.5 | \$ — | \$ 46.5 | \$ (8.3) | \$ 38.2 |
| Total liabilities | (8.3) | — | (8.3) | 8.3 | — |

J. Inventories

Inventories consisted of the following:

| | As of September 30, 2022 | As of December 31, 2021 |
|-----------------|---------------------------------|--------------------------------|
| | (in millions) | |
| Raw materials | \$ 32.2 | \$ 42.4 |
| Work-in-process | 229.3 | 224.0 |
| Finished goods | 126.7 | 86.7 |
| Total | <u>\$ 388.2</u> | <u>\$ 353.1</u> |

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K. Stock-based Compensation Expense and Share Repurchase Programs

Stock-based compensation expense

During the three and nine months ended September 30, 2022 and 2021, we recognized the following stock-based compensation expense:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|-----------------|--|-----------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions) | | | |
| Stock-based compensation expense by type of award: | | | | |
| Restricted stock units (including PSUs) | \$ 130.3 | \$ 89.5 | \$ 351.5 | \$ 279.1 |
| Stock options | 3.1 | 7.9 | 14.9 | 29.6 |
| ESPP share issuances | 1.9 | 6.3 | 13.0 | 17.9 |
| Stock-based compensation expense related to inventories | 0.3 | (0.7) | 0.4 | (3.8) |
| Total stock-based compensation expense included in "Total costs and expenses" | <u>\$ 135.6</u> | <u>\$ 103.0</u> | <u>\$ 379.8</u> | <u>\$ 322.8</u> |
| Stock-based compensation expense by line item: | | | | |
| Cost of sales | \$ 2.4 | \$ 1.6 | \$ 7.0 | \$ 4.6 |
| Research and development expenses | 80.0 | 61.0 | 229.9 | 196.4 |
| Selling, general and administrative expenses | 53.2 | 40.4 | 142.9 | 121.8 |
| Total stock-based compensation expense included in costs and expenses | 135.6 | 103.0 | 379.8 | 322.8 |
| Income tax effect | (38.8) | (21.6) | (101.3) | (73.7) |
| Total stock-based compensation expense, net of tax | <u>\$ 96.8</u> | <u>\$ 81.4</u> | <u>\$ 278.5</u> | <u>\$ 249.1</u> |

Share repurchase programs

In November 2020, our Board of Directors approved a share repurchase program (the "2020 Share Repurchase Program"), pursuant to which we repurchased \$500.0 million of our common stock in 2020 and the first quarter of 2021. During the three months ended March 31, 2021, we repurchased 2.0 million shares of our common stock under the 2020 Share Repurchase Program for an aggregate of \$424.9 million.

In June 2021, our Board of Directors approved a share repurchase program (the "2021 Share Repurchase Program"), pursuant to which we are authorized to repurchase up to \$1.5 billion of our common stock by December 31, 2022. During the nine months ended September 30, 2022, we did not repurchase any shares of our common stock under the 2021 Share Repurchase Program. As of September 30, 2022, a total of \$499.7 million remained authorized for repurchases of common stock under the 2021 Share Repurchase Program.

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

L. Income Taxes

We are subject to U.S. federal, state, and foreign income taxes. During the three and nine months ended September 30, 2022 and 2021, we recorded the following provisions for (benefits from) income taxes and effective tax rates as compared to our income before provision for income taxes:

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|--|--|-------------|--|-------------|
| | 2022 | 2021 | 2022 | 2021 |
| | (in millions, except percentages) | | | |
| Income before provision for income taxes | \$ 1,176.4 | \$ 1,082.8 | \$ 3,155.6 | \$ 1,859.5 |
| Provision for income taxes | 245.9 | 230.9 | 652.5 | 287.5 |
| Effective tax rate | 21 % | 21 % | 21 % | 15 % |

Our effective tax rate for the three and nine months ended September 30, 2022, and the three months ended September 30, 2021, was similar to the U.S. statutory rate.

Our effective tax rate for the nine months ended September 30, 2021 was different than the U.S. statutory rate primarily due to a \$99.7 million discrete tax benefit associated with an increase in the U.K.'s corporate tax rate from 19% to 25%, which was enacted in June 2021 and will become effective in April 2023.

We have reviewed the tax positions taken, or to be taken, in our tax returns for all tax years currently open to examination by a taxing authority. As of September 30, 2022 and December 31, 2021, we had \$144.9 million and \$129.5 million, respectively, of net unrecognized tax benefits, which would affect our tax rate if recognized.

Starting in 2022, our cash paid for income taxes is substantially increasing due to the elimination of the option in the U.S. to deduct research and development expenses in the period they are incurred and instead, as required by the Tax Cuts and Jobs Act of 2017, amortize them over a five year period if they are performed in the U.S. and fifteen years if they are performed in foreign jurisdictions.

In August 2022, the Inflation Reduction Act of 2022 ("IRA") was enacted into law. The IRA includes a 15% corporate alternative minimum tax and a 1% excise tax on share repurchases. We do not expect the IRA to have a significant impact on our consolidated financial statements.

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 2018 in jurisdictions that have a material impact on our consolidated financial statements.

M. 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 SOFR rate, 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).

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

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

The Credit Agreement contains customary representations and warranties and affirmative and negative covenants, including a financial covenant to maintain subject to certain limited exceptions, a consolidated leverage ratio of 3.50 to 1.00, subject to an increase to 4.00 to 1.00 following a material acquisition. As of September 30, 2022, 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.

Prior Credit Facilities

In July 2022, in conjunction with entering our new credit agreement, we terminated the \$500.0 million credit agreement we entered into in 2019. In September 2022, a \$2.0 billion credit agreement we entered into in 2020 expired in accordance with its terms.

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 a reserve for 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 F, "Fair Value Measurements," there were no material contingent liabilities accrued as of September 30, 2022 or December 31, 2021.

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

N. 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:

| | Nine Months Ended September 30, | | | |
|---|---------------------------------|-------------------|---------------------|-------------------|
| | 2022 | | 2021 | |
| | Beginning of period | End of period | Beginning of period | End of period |
| | (in millions) | | | |
| Cash and cash equivalents | \$ 6,795.0 | \$ 9,171.5 | \$ 5,988.2 | \$ 6,275.7 |
| Prepaid expenses and other current assets | 5.1 | 5.5 | 0.7 | 4.5 |
| Cash, cash equivalents and restricted cash per condensed consolidated statement of cash flows | <u>\$ 6,800.1</u> | <u>\$ 9,177.0</u> | <u>\$ 5,988.9</u> | <u>\$ 6,280.2</u> |

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

We invest in scientific innovation to create transformative medicines for people with serious diseases with a focus on specialty markets. We have four approved medicines to treat cystic fibrosis, or CF, a life-threatening genetic disease, and are focused on increasing the number of people with CF eligible and able to receive our medicines through label expansions, approval of new medicines, and expanded reimbursement. We are broadening our pipeline into additional disease areas through internal research efforts and accessing external innovation through business development transactions.

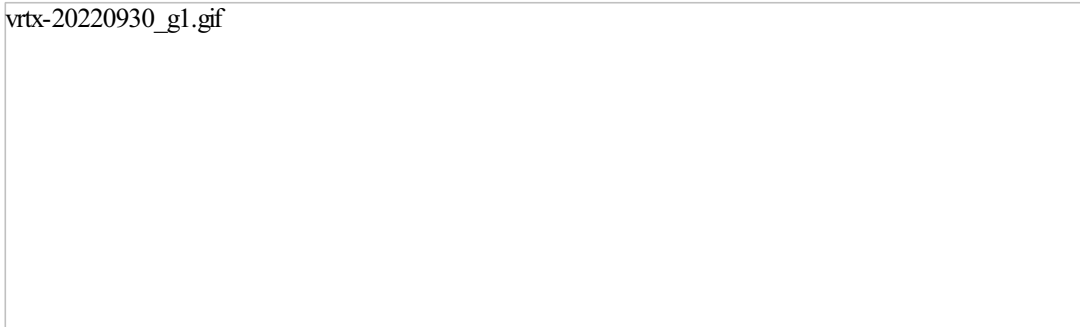
Our triple combination regimen, TRIKAFTA/KAFTRIO (elixacaftor/tezacaftor/ivacaftor and ivacaftor), was approved in 2019 in the United States, or U.S., and in 2020 in the European Union, or E.U. Collectively, our four medicines are being used by the majority of the approximately 83,000 people with CF 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 approximately 90% of people with CF. We also are pursuing genetic therapies for the remaining people with CF who may not be helped by our current CF medicines.

Beyond CF, we continue to research and develop product candidates for the treatment of serious diseases, including sickle cell disease, beta thalassemia, pain, APOL1-mediated kidney disease, type 1 diabetes, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy, and myotonic dystrophy type 1.

Financial Highlights

| | |
|-----------------|---|
| <i>Revenues</i> | In the third quarter of 2022, our net product revenues continued to increase as compared to the third quarter of 2021 primarily due to the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued steady performance of TRIKAFTA in the U.S. |
| <i>Expenses</i> | Our total research and development, or R&D, acquired in-process research and development, or AIPR&D, and selling, general and administrative, or SG&A, expenses increased to \$920.8 million in the third quarter of 2022 as compared to \$691.9 million in the third quarter of 2021. The increase was primarily due to the progression of several product candidates into mid- to late-stage clinical development. Cost of sales was 12% of our net product revenues in each of the third quarter of 2022 and 2021, respectively. |
| <i>Cash</i> | Our cash, cash equivalent and marketable securities increased to \$9.8 billion as of September 30, 2022 as compared to \$7.5 billion as of December 31, 2021 primarily due to our net product revenues and operating cash flows partially offset by income tax payments and our acquisition of ViaCyte, Inc., or ViaCyte. |

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Business Updates

Marketed Products

We expect to continue to grow our CF business by increasing the number of people with CF eligible and able to receive our medicines and providing improved treatment options for people who are already eligible for one of our medicines. Recent and anticipated progress in activities supporting these efforts is included below.

- We have received approval from the U.S. Food and Drug Administration, or the FDA, for ORKAMBI in children with CF 12 to less than 24 months of age who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator, or CFTR, gene.
- We have received reimbursement of KAFTRIO in Italy for children with CF 6 to 11 years of age with at least one F508del mutation in the CFTR gene.
- We expect to present results from the Phase 3 clinical trial for TRIKAFTA/KAFTRIO in children with CF 2 to 5 years of age at the North American Cystic Fibrosis Conference in November 2022. We anticipate global regulatory submissions for TRIKAFTA/KAFTRIO in children with CF 2 to 5 years of age before the end of 2022.
- We anticipate global regulatory submissions for KALYDECO in children with CF from 1 month to less than 4 months of age before the end of 2022.
- TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in more than 30 countries.

Pipeline

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

Cystic Fibrosis

- We are conducting two Phase 3 global, randomized, double-blind, active-controlled clinical trials evaluating our new once-daily investigational triple combination of vanzacaftor/tezacaftor/deutivacaftor, formerly known as VX-121/tezacaftor/VX-561, in people with CF 12 years of age and older. Enrollment in both clinical trials is expected to be completed by the end of 2022. We also have initiated a clinical trial of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age.
- In collaboration with Moderna, we are developing a CFTR mRNA therapeutic for the treatment of people with CF who do not produce any CFTR protein. We have completed IND-enabling studies and expect to submit an Investigational New Drug Application, or IND, for this program in the fourth quarter of 2022.

Beta Thalassemia and Sickle Cell Disease

- We are evaluating the use of a non-viral *ex vivo* CRISPR gene-editing therapy, exagamglogene autotemcel, or exa-cel, formerly known as CTX001, for the treatment of sickle cell disease, or SCD, and transfusion-dependent beta thalassemia, or TDT. We have concluded our discussions with the FDA, and the FDA granted exa-cel a rolling review. We plan to submit a biologics licensing application, or BLA, beginning in November 2022. We expect to complete the submission by the end of the first quarter of 2023. In the U.S., exa-cel has been granted Fast Track, Regenerative Medicine Advanced Therapy, Rare Pediatric Disease, and Orphan Drug designations.
- We anticipate submissions to the European Medicines Agency, or EMA, and the United Kingdom's Medicines and Healthcare products Regulatory Agency, or MHRA, for regulatory approval of exa-cel for TDT and SCD in Europe and the U.K. in the fourth quarter of 2022. Exa-cel has been granted EMA Priority Medicines, or PRIME, designation in Europe and Orphan Drug designation in Europe and the U.K.
- Two additional Phase 3 clinical trials evaluating exa-cel in pediatric patients with TDT and SCD are ongoing.

Pain

- We have discovered multiple selective small molecule inhibitors of NaV1.8 with the objective of creating a new class of pain medicines that have the potential to provide effective pain relief. In March, we announced positive Phase 2 data for VX-548, a NaV1.8 inhibitor, for the non-opioid treatment of acute pain. We have reached agreement with the FDA on the Phase 3 pivotal program design for VX-548 for moderate to severe acute pain. We have initiated this Phase 3 program and we expect to enroll two randomized, controlled trials with 2,000 patients with moderate to severe acute pain following bunionectomy and abdominoplasty surgery. We plan to evaluate the safety and effectiveness of VX-548 in multiple other types of moderate to severe acute pain in an additional clinical trial.
- The FDA granted VX-548 Fast Track and Breakthrough Therapy designations for the treatment of moderate to severe acute pain.
- We expect to initiate a Phase 2 clinical trial evaluating VX-548 in neuropathic pain by the end of 2022.

APOL1-Mediated Kidney Disease

- Inaxaplin, formerly known as VX-147, is our small molecule for the treatment of APOL1-mediated kidney disease, or AMKD, including APOL1-mediated focal segmental glomerulosclerosis, or FSGS. Based on positive Phase 2 data in FSGS, we initiated pivotal development of inaxaplin in a single Phase 2/3 clinical trial in patients with AMKD with two APOL1 mutations and proteinuric kidney disease. We continue to enroll patients in this Phase 2/3 clinical trial.
- 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 used in combination with immunosuppression to protect the implanted cells. VX-880 is being evaluated in a Phase 1/2 clinical trial as a potential treatment for type 1 diabetes, or T1D, and proof-of-concept has been achieved in the VX-880 program. Enrollment is ongoing in this Phase 1/2 clinical trial.
- 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 modified to produce hypimmune stem cells islets with the goal of eliminating the need for immunosuppression. We are conducting IND-enabling studies for the cells and device program, and we expect to submit an IND for this program in the fourth quarter of 2022.

Alpha-1 Antitrypsin Deficiency

- We are working to address the underlying genetic cause of alpha-1 antitrypsin, or AAT, deficiency by 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 AAT deficiency. We have initiated a 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 plan to initiate a 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.

Duchenne Muscular Dystrophy

- We are investigating a novel approach to treating Duchenne muscular dystrophy, or DMD, which delivers CRISPR/Cas9 gene-editing technology to muscle cells, with the goal of restoring near-full length dystrophin protein expression by targeting specific mutations in the dystrophin gene that cause the disease. We are conducting IND-enabling studies for our first in vivo gene-editing therapy for DMD and we expect to submit an IND for this program in 2023.

Investment in External Innovation

- In the third quarter of 2022, we acquired ViaCyte, a regenerative medicine company focused on delivering novel stem cell-derived cell replacement therapies as a potential functional cure for T1D.

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 actively pursuing a pipeline of product candidates for the treatment of serious diseases outside of CF. Our strategy is to combine transformative advances in the understanding of human disease biology and the science of therapeutics in order to discover and develop new medicines. This approach includes advancing multiple compounds from each program, spanning multiple modalities, into early clinical trials and evaluating patient data to inform discovery and development of additional compounds, with the goal of bringing first-in-class and best-in-class therapies to patients, and 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. 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 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 in order 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 in order 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. 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.

COVID-19

We continue to monitor the impacts of the COVID-19 global pandemic on our business, including in our clinical trials, manufacturing facilities and capabilities, and ability to access necessary resources. COVID-19 has not materially affected our supply chain or the demand for our medicines, and we believe that we will be able to continue to supply all of our approved medicines to patients globally. We adjusted our business operations in response to COVID-19 and have continued to monitor local COVID-19 trends and government guidance for each of our site locations. We are utilizing a site-specific approach to assess and permit employee access to our sites. Currently, our sites are open to certain employees where appropriate and permitted by local laws and guidelines.

Strategic Transactions

Acquisitions

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

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 Therapeutics AG, Kymira Therapeutics, Inc., Mammoth Biosciences, Inc., Moderna, Inc., Obsidian Therapeutics, Inc., and Verve Therapeutics, Inc., or Verve. 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 acquired in-process research and development expenses; however, depending on many factors, including the structure of the collaboration, the significance of the in-licensed product candidate to the collaborator's operations and the other activities in which our collaborators are engaged, the accounting for these transactions can vary significantly. We expect to continue to identify and evaluate collaboration and licensing opportunities that may be similar to or different from the collaborations and licenses that we have engaged in previously.

Acquired In-Process Research and Development

In the nine months ended September 30, 2022 and 2021, our AIPR&D included \$92.9 million and \$986.8 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 operations during the nine months ended September 30, 2022 and 2021.

Strategic Investments

In connection with our business development activities, we have periodically made equity investments in our collaborators. As of September 30, 2022, we held strategic equity investments in certain public and private companies, and we expect to make additional strategic equity investments in the future. While 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 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 operations.

RESULTS OF OPERATIONS

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|---|------------|--------|------------------------------------|------------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages and per share amounts) | | | | | |
| Revenues | \$ 2,334.3 | \$ 1,984.1 | 18% | \$ 6,628.0 | \$ 5,501.8 | 20% |
| Operating costs and expenses | 1,207.6 | 929.6 | 30% | 3,354.1 | 3,597.4 | (7)% |
| Income from operations | 1,126.7 | 1,054.5 | 7% | 3,273.9 | 1,904.4 | 72% |
| Other non-operating expense, net | 49.7 | 28.3 | 76% | (118.3) | (44.9) | 163% |
| Provision for income taxes | 245.9 | 230.9 | 6% | 652.5 | 287.5 | 127% |
| Net income | \$ 930.5 | \$ 851.9 | 9% | \$ 2,503.1 | \$ 1,572.0 | 59% |
| Net income per diluted common share | \$ 3.59 | \$ 3.28 | | \$ 9.68 | \$ 6.03 | |
| Diluted shares used in per share calculations | 259.5 | 259.7 | | 258.7 | 260.9 | |

Revenues

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|-----------------------|-------------------------------------|------------|--------|------------------------------------|------------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| TRIKAFTA/KAFTRIO | \$ 2,010.5 | \$ 1,555.8 | 29% | \$ 5,665.3 | \$ 4,004.6 | 41% |
| SYMDEKO/SYMKEVI | 38.2 | 81.4 | (53)% | 145.7 | 340.0 | (57)% |
| ORKAMBI | 146.2 | 184.5 | (21)% | 399.9 | 624.2 | (36)% |
| KALYDECO | 139.4 | 162.4 | (14)% | 417.1 | 532.0 | (22)% |
| Product revenues, net | 2,334.3 | 1,984.1 | 18% | 6,628.0 | 5,500.8 | 20% |
| Other revenues | — | — | ** | — | 1.0 | ** |
| Total revenues | \$ 2,334.3 | \$ 1,984.1 | 18% | \$ 6,628.0 | \$ 5,501.8 | 20% |

** Not meaningful

Product Revenues, Net

In the third quarter of 2022, our net product revenues increased by \$350.2 million, or 18% as compared to the third quarter of 2021, primarily due to the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued steady performance of TRIKAFTA in the U.S. In the nine months ended September 30, 2022, our net product revenues increased by \$1.1 billion, or 20% as compared to the nine months ended September 30, 2021, respectively, primarily due to the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and in the U.S., including the June 2021 launch of TRIKAFTA for children with CF 6 through 11 years of age. 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 September 30, | | | Nine Months Ended September 30, | | |
|-----------------------|-------------------------------------|------------|--------|------------------------------------|------------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| United States | \$ 1,455.6 | \$ 1,382.9 | 5% | \$ 4,238.9 | \$ 3,893.2 | 9% |
| ex-U.S. | 878.7 | 601.2 | 46% | 2,389.1 | 1,607.6 | 49% |
| Product revenues, net | \$ 2,334.3 | \$ 1,984.1 | 18% | \$ 6,628.0 | \$ 5,500.8 | 20% |

Other Revenues

We earned a collaborative milestone of \$1.0 million in the nine months ended September 30, 2021 and did not have any “Other revenues” in the nine months ended September 30, 2022. Our “Other revenues” have historically fluctuated significantly from one period to another based on our collaborative out-license activities and may continue to fluctuate in the future.

Operating Costs and Expenses

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|-----------------|--------|------------------------------------|-------------------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| Cost of sales | \$ 289.4 | \$ 236.5 | 22% | \$ 797.0 | \$ 656.8 | 21% |
| Research and development expenses | 645.0 | 467.0 | 38% | 1,846.2 | 1,370.0 | 35% |
| Acquired in-process research and development expenses | 29.0 | 26.7 | 9% | 92.9 | 986.8 | (91)% |
| Selling, general and administrative expenses | 246.8 | 198.2 | 25% | 677.3 | 584.9 | 16% |
| Change in fair value of contingent consideration | (2.6) | 1.2 | ** | (59.3) | (1.1) | ** |
| Total costs and expenses | <u>\$ 1,207.6</u> | <u>\$ 929.6</u> | 30% | <u>\$ 3,354.1</u> | <u>\$ 3,597.4</u> | (7)% |

** Not meaningful

In the second quarter of 2022, we began classifying upfront, contingent milestone, or 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,” or “AIPR&D,” in our condensed consolidated statements of operations. To conform prior periods to our current presentation, we have reclassified \$26.7 million and \$986.8 million from “Research and development expenses” to “AIPR&D” for the third quarter and nine months ended September 30, 2021, respectively.

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 slightly 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 12% in each of the third quarter and nine months ended September 30, 2022 and 2021.

Research and Development Expenses

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|-----------------|--------|------------------------------------|-------------------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| Research expenses | \$ 160.2 | \$ 121.9 | 31% | \$ 464.9 | \$ 372.2 | 25% |
| Development expenses | 484.8 | 345.1 | 40% | 1,381.3 | 997.8 | 38% |
| Total research and development expenses | <u>\$ 645.0</u> | <u>\$ 467.0</u> | 38% | <u>\$ 1,846.2</u> | <u>\$ 1,370.0</u> | 35% |

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 2020, 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 September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|-----------------|------------|------------------------------------|-----------------|------------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| Research Expenses: | | | | | | |
| Salary and benefits | \$ 40.7 | \$ 34.6 | 18% | \$ 119.0 | \$ 102.5 | 16% |
| Stock-based compensation expense | 23.3 | 17.4 | 34% | 65.7 | 56.4 | 16% |
| Outsourced services and other direct expenses | 49.2 | 35.8 | 37% | 132.7 | 114.9 | 15% |
| Intangible asset impairment charge | — | — | ** | 13.0 | — | ** |
| Infrastructure costs | 47.0 | 34.1 | 38% | 134.5 | 98.4 | 37% |
| Total research expenses | <u>\$ 160.2</u> | <u>\$ 121.9</u> | <u>31%</u> | <u>\$ 464.9</u> | <u>\$ 372.2</u> | <u>25%</u> |

** Not meaningful

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 and infrastructure. We expect to continue to invest in our research programs with a focus on creating transformative medicines for serious diseases.

Development Expenses

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|-----------------|------------|------------------------------------|-----------------|------------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| | (in millions, except percentages) | | | | | |
| Development Expenses: | | | | | | |
| Salary and benefits | \$ 133.5 | \$ 88.6 | 51% | \$ 344.4 | \$ 252.2 | 37% |
| Stock-based compensation expense | 56.7 | 43.6 | 30% | 164.2 | 140.0 | 17% |
| Outsourced services and other direct expenses | 215.9 | 149.6 | 44% | 639.2 | 426.4 | 50% |
| Infrastructure costs | 78.7 | 63.3 | 24% | 233.5 | 179.2 | 30% |
| Total development expenses | <u>\$ 484.8</u> | <u>\$ 345.1</u> | <u>40%</u> | <u>\$ 1,381.3</u> | <u>\$ 997.8</u> | <u>38%</u> |

Our development expenses increased by \$139.7 million and \$383.5 million, or 40% and 38%, in the third quarter and nine months ended September 30, 2022 as compared to the third quarter and nine months ended September 30, 2021, respectively, primarily due to increased costs to support clinical trials associated with our advancing pipeline programs, including our CF triple combination of vanzacaftor/tezacaftor/deutivacaftor, exa-cel, pain and T1D. We are investing in our internal headcount, leveraging outsourced services, and investing in infrastructure to support these programs. In the nine months ended September 30, 2022 and 2021, costs related to our CF programs represented the largest portion of our development costs.

Acquired In-process Research and Development Expenses

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|---|-------------------------------------|---------|--------|------------------------------------|----------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| (in millions, except percentages) | | | | | | |
| Acquired in-process research and development expenses | \$ 29.0 | \$ 26.7 | 9% | \$ 92.9 | \$ 986.8 | (91)% |

AIPR&D in the third quarter of 2022 was primarily related to a \$25.0 million upfront payment pursuant to our license agreement with Verve. AIPR&D in the nine months ended September 30, 2022 was primarily related to our payment to Verve and a \$60.0 million payment to Catalyst to acquire their complement portfolio and related intellectual property. AIPR&D in the nine months ended September 30, 2021 included the \$900.0 million upfront payment 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 business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions.

Selling, General and Administrative Expenses

| | Three Months Ended September 30, | | | Nine Months Ended September 30, | | |
|--|-------------------------------------|----------|--------|------------------------------------|----------|--------|
| | 2022 | 2021 | Change | 2022 | 2021 | Change |
| (in millions, except percentages) | | | | | | |
| Selling, general and administrative expenses | \$ 246.8 | \$ 198.2 | 25% | \$ 677.3 | \$ 584.9 | 16% |

Selling, general and administrative expenses increased by 25% and 16% in the third quarter and nine months ended September 30, 2022 as compared to the third quarter and nine months ended September 30, 2021, respectively, 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 contingent consideration potentially payable to former Exonics equity holders decreased by \$2.6 million and \$59.3 million in the third quarter and nine months ended September 30, 2022, respectively. The fair value of contingent consideration decreased in the nine months ended September 30, 2022 primarily as a result of a revision to the scope of certain gene-editing programs in the second quarter of 2022. The fair value of contingent consideration increased by \$1.2 million and decreased by \$1.1 million in the third quarter and nine months ended September 30, 2021, respectively.

Other Non-Operating Income (Expense), Net

Interest Income

Interest income increased to \$46.2 million and \$58.6 million in the third quarter and nine months ended September 30, 2022, respectively, as compared to \$1.1 million and \$3.7 million in the third quarter and nine months ended September 30, 2021, respectively. The increase in interest income was 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 \$13.7 million and \$15.2 million in the third quarter of 2022 and 2021, respectively, and \$43.2 million and \$46.4 million in the nine months ended September 30, 2022 and 2021, 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 \$17.2 million and \$42.4 million in the third quarter of 2022 and 2021, respectively, and expense of \$133.7 million and \$2.2 million in the nine months ended September 30, 2022 and 2021, respectively. The vast majority of these amounts relate to net unrealized gains or losses resulting from changes in the fair value of our strategic investments. As of September 30, 2022, the fair value of our investments in publicly traded companies was \$122.8 million. To the extent that we continue to hold strategic investments in publicly traded companies, we will record other income (expense) related to these strategic 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 investments.

Income Taxes

We recorded provisions for income taxes of \$245.9 million and \$652.5 million in the third quarter and nine months ended September 30, 2022, respectively, and \$230.9 million and \$287.5 million in the third quarter and nine months ended September 30, 2021. Our effective tax rate of 21% for the nine months ended September 30, 2022 was similar to the U.S. statutory rate. Our effective tax rate of 15% for the nine months ended September 30, 2021 was lower than the U.S. statutory rate primarily due to a \$99.7 million discrete tax benefit associated with an increase in the U.K.'s corporate tax rate from 19% to 25%, which was enacted in June 2021 and will become effective in April 2023.

Net Income

Our net income increased to \$930.5 million in the third quarter of 2022 as compared to \$851.9 million in the third quarter of 2021, primarily due to increased product revenues and interest income partially offset by increased operating costs and expenses.

Our net income increased to \$2.5 billion in the nine months ended September 30, 2022 as compared to \$1.6 billion in the nine months ended September 30, 2021, primarily due to the \$900.0 million upfront payment we made to CRISPR in the second quarter of 2021 and increased product revenues partially offset by increased operating costs and expenses. We also incurred significant unrealized losses on our strategic investments in third quarter and nine months ended September 30, 2022.

In each of the third quarter of 2022 as compared to the third quarter of 2021 and the nine months ended September 30, 2022 as compared to the nine months ended September 30, 2021, our increased operating costs and expenses consisted of increased cost of sales, development expenses to progress several product candidates into mid- to late-stage clinical development, selling, general and administrative expenses to support the commercialization of our medicines and increased support for our pipeline product candidates and income taxes.

LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of September 30, 2022 and December 31, 2021:

| | As of September 30, 2022 | | As of December 31, 2021 | | Change |
|--|-----------------------------------|-----------|-------------------------|-----------|--------|
| | (in millions, except percentages) | | | | |
| Cash, cash equivalents and marketable securities | \$ | 9,770.7 | \$ | 7,524.9 | 30% |
| Working Capital: | | | | | |
| Total current assets | | 12,271.0 | | 9,560.6 | 28% |
| Total current liabilities | | (2,609.3) | | (2,142.0) | 22% |
| Total working capital | \$ | 9,661.7 | \$ | 7,418.6 | 30% |

Working Capital

As of September 30, 2022, total working capital was \$9.7 billion, which represented an increase of \$2.2 billion from \$7.4 billion as of December 31, 2021. The increase in total working capital in the nine months ended September 30, 2022 was primarily related to \$3.1 billion of cash provided by operations.

Cash Flows

| | Nine Months Ended September 30, | | | |
|---------------------------------|---------------------------------|---------|------|-----------|
| | 2022 | | 2021 | |
| | (in millions) | | | |
| Net cash provided by (used in): | | | | |
| Operating activities | \$ | 3,051.5 | \$ | 1,648.5 |
| Investing activities | \$ | (496.7) | \$ | (206.9) |
| Financing activities | \$ | (107.9) | \$ | (1,141.8) |

Operating Activities

Cash provided by operating activities were \$3.1 billion in the nine months ended September 30, 2022 as compared to \$1.6 billion in the nine months ended September 30, 2021, primarily due to the \$900.0 million upfront payment to CRISPR in the nine months ended September 30, 2021 and an increase to accrued expenses from increased product revenues, offset by higher income tax payments.

Investing Activities

Cash used in investing activities were \$496.7 million and \$206.9 million in the nine months ended September 30, 2022 and 2021, respectively. In the third quarter of 2022, our investing activities included a net payment of \$295.9 million to acquire ViaCyte. Otherwise, our investing activities were primarily related to purchases of property and equipment in each of the nine months ended September 30, 2022 and 2021.

Financing Activities

Cash used in financing activities were \$107.9 million and \$1.1 billion in the nine months ended September 30, 2022 and 2021, respectively. In the nine months ended September 30, 2022, the largest portion of our financing activities were related to our employee stock benefit plans. In the nine months ended September 30, 2021, the largest portion of our financing activities were share repurchases pursuant to our share repurchase programs totaling \$1.1 billion.

Sources and Uses of Liquidity

As of September 30, 2022, we had cash, cash equivalents and marketable securities of \$9.8 billion, which represented an increase of \$2.2 billion from \$7.5 billion as of December 31, 2021. 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 products 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 the amounts of future revenues generated by our products, and the potential introduction of one or more of our other product candidates to the market, the level of 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 \$500.0 million pursuant to a revolving credit facility that we entered into in July 2022 and could repay and reborrow amounts under this revolving credit agreement without penalty. Subject to certain conditions, we could request that the borrowing capacity be increased by an additional \$500.0 million, for a total of \$1.0 billion. Negative covenants in our credit agreement could prohibit or limit our ability to access this source of liquidity. As of September 30, 2022, the facility was undrawn, and we were in compliance with these covenants.

In July 2022, in conjunction with entering our new credit agreement, we terminated the \$500.0 million credit agreement we entered into in 2019. In September 2022, a \$2.0 billion credit agreement we entered into in 2020 expired in accordance with its terms.

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 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 September 30, 2022, we had \$0.5 billion available under our 2021 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, 2021, which was filed with the Securities and Exchange Commission, or SEC, on February 9, 2022.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

Our discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the U.S. The preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate. During the nine months ended September 30, 2022, 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, 2021, which was filed with the SEC on February 9, 2022.

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, 2021, which was filed with the SEC on February 9, 2022.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management (under the supervision and with the participation of our chief executive officer and chief financial officer), after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q, has concluded that, based on such evaluation, as of September 30, 2022 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Controls Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended September 30, 2022 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, 2021, which was filed with the SEC on February 9, 2022. 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, including those related to net product revenues;
- our expectations regarding clinical trials, development timelines, regulatory authority filings, submissions, and potential approvals and label expansions for our product and product candidates, and other pipeline programs, including timing and structure of clinical trials, anticipated enrollment and dosing of patients, timing of availability of data from our ongoing and planned clinical trials, and timing of anticipated regulatory filings;
- our ability to obtain reimbursement for our medicines in the U.S. and ex-U.S. markets and our ability to launch, commercialize and market our products or any of our other product candidates for which we obtain regulatory approval;
- 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 product candidates and other pipeline programs for further investigation, clinical trials or potential use as a treatment;
- our beliefs regarding the number of people with CF and those potentially eligible for our medicines, and our ability to grow our CF business by increasing the number of people with CF eligible and able to receive our medicines;
- our expectations regarding the potential benefits and commercial potential of our product candidates, including the potential approach to treating or curing specific diseases;
- our plan 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;
- the potential future benefits of our acquisitions and collaborations, including our exa-cel collaboration with CRISPR;
- 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 expectations that our acquisition of ViaCyte may accelerate the development of our T1D program;
- our expectations regarding the effect of COVID-19 on, among other things, our financial performance, liquidity, business and operations, including manufacturing, supply chain, research and development activities and pipeline programs;
- potential fluctuations in foreign currency exchange rates;
- 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; 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, 2021, which was filed with the SEC on February 9, 2022, 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 June 2021, our Board of Directors approved a share repurchase program (the “2021 Share Repurchase Program”), pursuant to which we are authorized to repurchase up to \$1.5 billion of our common stock by December 31, 2022. We did not repurchase any shares of our common stock under the 2021 Share Repurchase Program in the three months ended September 30, 2022. As of September 30, 2022, \$499.7 million remained available to fund repurchases under this share repurchase program.

Under our 2021 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 |
|-----------------------|--|
| 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.INSXBRL | Instance - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document. |
| 101.SCHXBRL | Taxonomy Extension Schema |
| 101.CALXBRL | Taxonomy Extension Calculation |
| 101.LABXBRL | Taxonomy Extension Labels |
| 101.PREXBRL | Taxonomy Extension Presentation |
| 101.DEF XBRL | Taxonomy Extension Definition |
| 104 | Cover Page Interactive Data File—the cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document. |

SIGNATURES

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

Vertex Pharmaceuticals Incorporated

October 28, 2022

By:

/s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

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