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

FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2021

or

☐ TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

FOR THE TRANSITION PERIOD FROM _ TO _

Commission file number 000-19319

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

Massachusetts	**04-3039129**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
50 Northern Avenue, Boston, Massachusetts	**02210**
(Address of principal executive offices)	(Zip Code)

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

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

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

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

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

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

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

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

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

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

Common Stock, par value $0.01 per share	258,865,671	Outstanding at April 22, 2021

VERTEX PHARMACEUTICALS INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED MARCH 31, 2021

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"We," "us," "Vertex" and the "Company" 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®" and "TRIKAFTA®" are registered trademarks of Vertex. The trademark for "KAFTRIO™" is pending in the United States and registered in the European Union. 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 thousands, except per share amounts)

	Three Months Ended March 31,	
	2021	**2020**
Revenues:		
Product revenues, net	$ 1,723,305	$ 1,515,107
Other revenues	1,000	—
Total revenues	1,724,305	1,515,107
Costs and expenses:		
Cost of sales	192,329	162,497
Research and development expenses	455,973	448,528
Sales, general and administrative expenses	192,077	182,258
Change in fair value of contingent consideration	(3,900)	1,600
Total costs and expenses	836,479	794,883
Income from operations	887,826	720,224
Interest income	1,465	12,576
Interest expense	(15,678)	(14,136)
Other expense, net	(52,653)	(61,130)
Income before provision for income taxes	820,960	657,534
Provision for income taxes	167,822	54,781
Net income	$ 653,138	$ 602,753
Net income per common share:		
Basic	$ 2.52	$ 2.32
Diluted	$ 2.49	$ 2.29
Shares used in per share calculations:		
Basic	259,369	259,815
Diluted	261,916	263,515

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

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

	Three Months Ended March 31,	
	2021	**2020**
Net income	$ 653,138	$ 602,753
Other comprehensive income:		
Unrealized holding losses on marketable securities, net	(218)	(764)
Unrealized gains on foreign currency forward contracts, net of tax of $(9.3) million and $(5.0) million, respectively	33,966	18,782
Foreign currency translation adjustment	1,430	(2,662)
Total other comprehensive income	35,178	15,356
Comprehensive income	$ 688,316	$ 618,109

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

VERTEX PHARMACEUTICALS INCORPORATED
Condensed Consolidated Balance Sheets
(unaudited)
(in thousands, except per share amounts)

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 6,304,330	$ 5,988,187
Marketable securities	619,638	670,710
Accounts receivable, net	977,551	885,352
Inventories	298,863	280,777
Prepaid expenses and other current assets	338,925	308,353
Total current assets	8,539,307	8,133,379
Property and equipment, net	986,123	958,534
Goodwill	1,002,158	1,002,158
Intangible assets	400,000	400,000
Deferred tax assets	815,890	882,779
Operating lease assets	322,319	325,564
Other assets	49,262	49,394
Total assets	$ 12,115,059	$ 11,751,808
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 127,839	$ 155,139
Accrued expenses	1,532,037	1,404,971
Other current liabilities	284,174	317,423
Total current liabilities	1,944,050	1,877,533
Long-term finance lease liabilities	530,330	539,042
Long-term operating lease liabilities	368,467	350,463
Long-term contingent consideration	185,700	189,600
Other long-term liabilities	106,258	108,355
Total liabilities	3,134,805	3,064,993
Commitments and contingencies	—	—
Shareholders' equity:		
Preferred stock, $0.01 par value; 1,000 shares authorized; none issued and outstanding	—	—
Common stock, $0.01 par value; 500,000 shares authorized, 258,829 and 259,890 shares issued and outstanding, respectively	2,588	2,599
Additional paid-in capital	7,499,161	7,894,027
Accumulated other comprehensive loss	(33,302)	(68,480)
Retained earnings	1,511,807	858,669
Total shareholders' equity	8,980,254	8,686,815
Total liabilities and shareholders' equity	$ 12,115,059	$ 11,751,808

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

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

	Three Months Ended					
	Common Stock					
	Shares	Amount	Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Retained Earnings (Accumulated Deficit)	Total Shareholders' Equity
Balance at December 31, 2019	258,993	$ 2,589	$ 7,937,606	$ (1,973)	$ (1,852,978)	$ 6,085,244
Other comprehensive income, net of tax	—	—	—	15,356	—	15,356
Net income	—	—	—	—	602,753	602,753
Repurchase of common stock	(1,404)	(14)	(300,012)	—	—	(300,026)
Common stock withheld for employee tax obligations	(575)	(6)	(136,161)	—	—	(136,167)
Issuance of common stock under benefit plans	2,065	22	77,572	—	—	77,594
Stock-based compensation expense	—	—	116,900	—	—	116,900
Balance at March 31, 2020	259,079	$ 2,591	$ 7,695,905	$ 13,383	$ (1,250,225)	$ 6,461,654
Balance at December 31, 2020	259,890	$ 2,599	$ 7,894,027	$ (68,480)	$ 858,669	$ 8,686,815
Other comprehensive income, net of tax	—	—	—	35,178	—	35,178
Net income	—	—	—	—	653,138	653,138
Repurchase of common stock	(1,989)	(20)	(424,932)	—	—	(424,952)
Common stock withheld for employee tax obligations	(472)	(5)	(102,135)	—	—	(102,140)
Issuance of common stock under benefit plans	1,400	14	15,164	—	—	15,178
Stock-based compensation expense	—	—	117,037	—	—	117,037
Balance at March 31, 2021	258,829	$ 2,588	$ 7,499,161	$ (33,302)	$ 1,511,807	$ 8,980,254

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

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

	Three Months Ended March 31,	
	2021	**2020**
Cash flows from operating activities:		
Net income	$ 653,138	$ 602,753
Adjustments to reconcile net income to net cash provided by operating activities:		
Stock-based compensation expense	115,174	115,706
Depreciation expense	28,834	26,821
(Decrease) increase in fair value of contingent consideration	(3,900)	1,600
Deferred income taxes	57,043	36,705
Losses on equity securities	52,295	44,870
Other non-cash items, net	2,332	9,668
Changes in operating assets and liabilities:		
Accounts receivable, net	(98,373)	(223,672)
Inventories	(22,785)	(27,450)
Prepaid expenses and other assets	(13,319)	2,790
Accounts payable	(10,644)	14,285
Accrued expenses	152,983	153,814
Other liabilities	8,277	57,808
Net cash provided by operating activities	921,055	815,698
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(121,455)	(75,265)
Maturities of available-for-sale debt securities	118,072	60,145
Sale of equity securities	—	72,036
Purchases of property and equipment	(70,926)	(19,450)
Investment in equity securities	—	(5,800)
Net cash (used in) provided by investing activities	(74,309)	31,666
Cash flows from financing activities:		
Issuances of common stock under benefit plans	15,559	79,597
Repurchases of common stock	(424,952)	(300,026)
Payments in connection with common stock withheld for employee tax obligations	(102,140)	(136,167)
Payments on finance leases	(12,233)	(10,287)
Proceeds from finance leases	3,632	5,833
Other financing activities	1,480	1,620
Net cash used in financing activities	(518,654)	(359,430)
Effect of changes in exchange rates on cash	(4,030)	(6,651)
Net increase in cash, cash equivalents and restricted cash	324,062	481,283
Cash, cash equivalents and restricted cash—beginning of period	5,988,845	3,120,681
Cash, cash equivalents and restricted cash—end of period	$ 6,312,907	$ 3,601,964
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ 14,534	$ 13,771
Cash paid for income taxes	$ 10,691	$ 5,845

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

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" or the "Company") 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 the Company and its wholly-owned subsidiaries. All material intercompany balances and transactions have been eliminated. The Company operates in one segment, pharmaceuticals. The Company has reclassified certain items from the prior year's condensed consolidated financial statements to conform to the current year's presentation.

Certain information and footnote disclosures normally included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2020 (the "2020 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 March 31, 2021 and 2020.

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, 2020, which are contained in the Company's 2020 Annual Report on Form 10-K.

Use of Estimates

The preparation of condensed consolidated financial statements in accordance with U.S. GAAP requires management 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 the condensed consolidated financial statements, and the amounts of revenues and expenses during the reported periods. The Company bases its estimates on historical experience and various other assumptions, including in certain circumstances future projections that management believes 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

Income Taxes

In 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740)* ("ASU 2019-12"), which simplifies the accounting for income taxes. ASU 2019-12 became effective on January 1, 2021. The adoption of ASU 2019-12 did not have a significant impact on the Company's condensed consolidated financial statements.

For a discussion of other recent accounting pronouncements please refer to Note A, "Nature of Business and Accounting Policies," in the Company's 2020 Annual Report on Form 10-K.

Summary of Significant Accounting Policies

The Company's significant accounting policies are described in Note A, "Nature of Business and Accounting Policies," in its 2020 Annual Report on Form 10-K.

B. Revenue Recognition

Disaggregation of Revenue

Revenues by Product

Product revenues, net consisted of the following:

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
TRIKAFTA/KAFTRIO	$ 1,193,217	$ 895,233
SYMDEKO/SYMKEVI	125,049	173,159
ORKAMBI	218,697	234,138
KALYDECO	186,342	212,577
Total product revenues, net	$ 1,723,305	$ 1,515,107

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 March 31,	
	2021	2020
	(in thousands)	
United States	$ 1,253,433	$ 1,187,588
Outside of the United States		
Europe	404,969	257,391
Other	64,903	70,128
Total product revenues outside of the United States	469,872	327,519
Total product revenues, net	$ 1,723,305	$ 1,515,107

Contract Liabilities

The Company had contract liabilities of $181.2 million and $191.5 million as of March 31, 2021 and December 31, 2020, respectively, related to annual contracts with government-owned and supported customers in international markets that limit the amount of annual reimbursement the Company 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. The Company defers 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. The Company's product revenue contracts include performance obligations that are one year or less.

The Company's 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 the Company's fiscal year. In these markets, the Company recognizes revenues related to performance obligations satisfied in previous years; however, these revenues do not relate to any performance obligations that were satisfied more than 12 months prior to the beginning of the current year.

C. Collaborative Arrangements

The Company has entered into numerous agreements pursuant to which it collaborates with third parties on research, development and commercialization programs, including in-license and out-license agreements.

The Company's in-license and out-license agreements that had a significant impact on its financial statements for the three months ended March 31, 2021 and 2020, or were new or otherwise revised during the three months ended March 31,

2021, are described below. Additional in-license and out-license agreements were described in Note B, "Collaborative Arrangements," of the Company's 2020 Annual Report on Form 10-K.

In-license Agreements

The Company has entered into a number of license agreements in order to advance and obtain access to technologies and services related to its research and early-development activities. The Company is generally required to make an upfront payment upon execution of the license agreement; 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 the collaboration.

Pursuant to the terms of its in-license agreements, the Company's collaborators typically lead the discovery efforts and the Company leads all preclinical, development and commercialization activities associated with the advancement of any drug candidates and funds all expenses.

The Company typically can terminate its in-license agreements by providing advance notice to its collaborators; the required length of notice is dependent on whether any product developed under the license agreement has received marketing approval. The Company's 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, the Company 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. The Company had the exclusive right to license certain targets. In 2019, the Company elected to exclusively license three targets, including cystic fibrosis, pursuant to the CRISPR Agreement. For each of the three targets that the Company 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, the Company entered into a joint development and commercialization agreement with CRISPR pursuant to the terms of the CRISPR Agreement (the "Original CTX001 JDCA"), under which the Company and CRISPR are co-developing and preparing to co-commercialize CTX001 for the treatment of hemoglobinopathies, including treatments for sickle cell disease and beta thalassemia. The Company concluded that the Original CTX001 JDCA is a cost-sharing arrangement, which results in the net impact of the arrangement being recorded in "Research and development expenses" in its condensed consolidated statements of operations. During the three months ended March 31, 2021 and 2020, the net expense related to the Original CTX001 JDCA was $20.0 million and $9.3 million, respectively.

In April 2021, the Company 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 the Company relating to the products that may be researched, developed, manufactured and commercialized under such agreement. The closing of the transaction contemplated under the A&R JDCA is subject to certain conditions, including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and any other required antitrust clearance.

After the effective date of the closing of the transaction (the "Effective Date"), the Company will lead global development, manufacturing and commercialization of CTX001, with support from CRISPR. Subject to the terms and conditions of the A&R JDCA, the Company will have the right to conduct all research, development, manufacturing and commercialization activities relating to the product candidates and products under the A&R JDCA (including CTX001) throughout the world subject to CRISPR's reserved right to conduct certain activities.

In connection with the closing of the transaction contemplated by the A&R JDCA, the Company will pay a $900.0 million upfront payment to CRISPR and an additional one-time $200.0 million milestone payment upon receipt of the first marketing approval of CTX001 from the U.S. Food or Drug Administration or the European Commission. The Company and CRISPR will continue to share equally all net profits and net losses incurred under the A&R JDCA through June 30, 2021, subject to adjustment based on the timing of antitrust clearance. Beginning July 1, 2021 (or the first day of the calendar quarter in which antitrust clearance occurs), with respect to CTX001, the net profits and net losses incurred pursuant to the A&R JDCA will be allocated 60% to the Company and 40% to CRISPR, while all other product candidate and products will continue to have net profits and net losses shared equally. The Company expects to record the $900.0 million upfront payment to "Research and development expenses" upon closing of the transaction contemplated by the A&R JDCA.

Out-license Agreements

The Company has entered into licensing agreements pursuant to which it has out-licensed rights to certain drug candidates to third-party collaborators. Pursuant to these out-license agreements, the Company's collaborators become responsible for all costs related to the continued development of such drug candidates and obtain development and commercialization rights to these drug candidates. Depending on the terms of the agreements, the Company's collaborators may be required to make upfront payments, milestone payments upon the achievement of certain product research and development objectives and may also be required to pay royalties on future sales, if any, of commercial products resulting from the collaboration. The termination provisions associated with these collaborations are generally the same as those described above related to the Company's in-license agreements. None of the Company's out-license agreements had a significant impact on the Company's condensed consolidated statement of operations during the three months ended March 31, 2021 and 2020.

Cystic Fibrosis Foundation

The Company has 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, the Company 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 elexacaftor, 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 (elexacaftor/tezacaftor/ivacaftor and ivacaftor), sales are allocated equally to each of the active pharmaceutical ingredients in the combination product.

D. Earnings Per Share

Basic net income per common share is based upon the weighted-average number of common shares outstanding during the period. Diluted net income per common share utilizing the treasury-stock method is based upon the weighted-average number of common shares outstanding during the period plus additional weighted-average common equivalent shares outstanding during the period when the effect is dilutive.

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

	Three Months Ended March 31,	
	2021	2020
	(in thousands, except per share amounts)	
Net income	$ 653,138	$ 602,753
Basic weighted-average common shares outstanding	259,369	259,815
Effect of potentially dilutive securities:		
Stock options	1,263	1,868
Restricted stock units (including PSUs)	1,276	1,801
Employee stock purchase program	8	31
Diluted weighted-average common shares outstanding	261,916	263,515
Basic net income per common share	$ 2.52	$ 2.32
Diluted net income per common share	$ 2.49	$ 2.29

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

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Stock options	356	879
Unvested restricted stock units (including PSUs)	712	430

E. Fair Value Measurements

The following fair value hierarchy is used to classify assets and liabilities based on observable inputs and unobservable inputs used in order to determine the fair value of the Company's 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 the Company's assessment of the assumptions that market participants would use in pricing the asset or liability.

The Company's investment strategy is focused on capital preservation. The Company invests in instruments that meet the credit quality standards outlined in the Company's investment policy. This policy also limits the amount of credit exposure to any one issue or type of instrument. The Company maintains strategic investments separately from the investment policy that governs its other cash, cash equivalents and marketable securities as described in Note F, "Marketable Securities and Equity Investments." Additionally, the Company utilizes foreign currency forward contracts intended to mitigate the effect of changes in foreign exchange rates on its condensed consolidated statement of operations.

During the three months ended March 31, 2021 and 2020, the Company did not record any other-than-temporary impairment charges related to its financial assets.

The following tables set forth the Company's financial assets and liabilities subject to fair value measurements by level within the fair value hierarchy (and does not include $2.4 billion and $2.8 billion of cash as of March 31, 2021 and December 31, 2020, respectively):

	As of March 31, 2021				As of December 31, 2020			
	Total	Level 1	Level 2	Level 3	Total	Level 1	Level 2	Level 3
	(in thousands)							
Financial instruments carried at fair value (asset positions):								
Cash equivalents:								
Money market funds	$ 3,890,303	$ 3,890,303	$ —	$ —	$ 3,141,053	$ 3,141,053	$ —	$ —
Marketable securities:								
Corporate equity securities	143,486	143,486	—	—	195,781	15,650	180,131	—
Government-sponsored enterprise securities	62,362	62,362	—	—	80,063	80,063	—	—
Corporate debt securities	155,716	—	155,716	—	231,598	—	231,598	—
Commercial paper	258,074	—	258,074	—	163,268	—	163,268	—
Prepaid expenses and other current assets:								
Foreign currency forward contracts	7,039	—	7,039	—	—	—	—	—
Other assets:								
Foreign currency forward contracts	1,944	—	1,944	—	—	—	—	—
Total financial assets	$ 4,518,924	$ 4,096,151	$ 422,773	$ —	$ 3,811,763	$ 3,236,766	$ 574,997	$ —
Financial instruments carried at fair value (liability positions):								
Other current liabilities:								
Foreign currency forward contracts	$ (28,743)	$ —	$ (28,743)	$ —	$ (59,184)	$ —	$ (59,184)	$ —
Long-term contingent consideration	(185,700)	—	—	(185,700)	(189,600)	—	—	(189,600)
Other long-term liabilities:								
Foreign currency forward contracts	(433)	—	(433)	—	(4,283)	—	(4,283)	—
Total financial liabilities	$ (214,876)	$ —	$ (29,176)	$ (185,700)	$ (253,067)	$ —	$ (63,467)	$ (189,600)

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

Fair Value of Corporate Equity Securities

The Company classifies its investments in publicly traded corporate equity securities as "Marketable securities" on its condensed consolidated balance sheets. Generally, the Company's investments in the common stock of these publicly traded companies are valued based on Level 1 inputs because they have readily determinable fair values. However, certain of the Company's 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 F, "Marketable Securities and Equity Investments," for further information on these investments.

Fair Value of Contingent Consideration

In 2019, the Company acquired Exonics Therapeutics, Inc. ("Exonics"), a privately-held company focused on creating transformative gene-editing therapies to repair mutations that cause DMD and other severe neuromuscular diseases, including DM1. The Company's Level 3 contingent consideration liabilities are related to $678.3 million of development and regulatory milestones potentially payable to Exonics' former equity holders. The Company bases its estimates of the probability of achieving the milestones relevant to the fair value of contingent payments on industry data attributable to rare diseases. The discount rates used in the valuation model for contingent payments, which were between 0.6% and 2.4% as of March 31, 2021, represent a measure of credit risk and market risk associated with settling the liabilities. Significant judgment is used in determining the appropriateness of these assumptions at each reporting period. Due to the uncertainties associated with development and commercialization of drug candidates in the pharmaceutical industry and the effects of

changes in other assumptions including discount rates, the Company expects its estimates regarding the fair value of contingent consideration to change in the future, resulting in adjustments to the fair value of the Company's contingent consideration liabilities, and the effect of any such adjustments could be material.

The following table represents a rollforward of the fair value of the Company's contingent consideration liabilities:

	Three Months Ended March 31, 2021
	(in thousands)
Balance at December 31, 2020	$ 189,600
Decrease in fair value of contingent payments	(3,900)
Balance at March 31, 2021	$ 185,700

F. Marketable Securities and Equity Investments

A summary of the Company's cash equivalents and marketable securities, which are recorded at fair value (and do not include $2.4 billion and $2.8 billion of cash as of March 31, 2021 and December 31, 2020, respectively), is shown below:

	As of March 31, 2021				As of December 31, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)							
Cash equivalents:								
Money market funds	$ 3,890,303	$ —	$ —	$ 3,890,303	$ 3,141,053	$ —	$ —	$ 3,141,053
Total cash equivalents	$ 3,890,303	$ —	$ —	$ 3,890,303	$ 3,141,053	$ —	$ —	$ 3,141,053
Marketable securities:								
Government-sponsored enterprise securities	$ 62,332	$ 30	$ —	$ 62,362	$ 80,046	$ 17	$ —	$ 80,063
Corporate debt securities	155,636	126	(46)	155,716	231,263	377	(42)	231,598
Commercial paper	258,068	27	(21)	258,074	163,286	19	(37)	163,268
Total marketable debt securities	476,036	183	(67)	476,152	474,595	413	(79)	474,929
Corporate equity securities	51,427	92,059	—	143,486	51,427	144,354	—	195,781
Total marketable securities	$ 527,463	$ 92,242	$ (67)	$ 619,638	$ 526,022	$ 144,767	$ (79)	$ 670,710

Available-for-sale debt securities were classified on the Company's condensed consolidated balance sheets at fair value as follows:

	As of March 31, 2021	As of December 31, 2020
	(in thousands)	
Cash and cash equivalents	$ 3,890,303	$ 3,141,053
Marketable securities	476,152	474,929
Total	$ 4,366,455	$ 3,615,982

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

	As of March 31, 2021	As of December 31, 2020
	(in thousands)	
Matures within one year	$ 4,358,433	$ 3,526,185
Matures after one year through five years	8,022	89,797
Total	$ 4,366,455	$ 3,615,982

The Company has a limited number of available-for-sale debt securities in insignificant loss positions as of March 31, 2021, which it does not intend to sell and has concluded it will not be required to sell before recovery of the amortized costs

for the investments at maturity. The Company did not record any charges for other-than-temporary declines in the fair value of available-for-sale debt securities or gross realized gains or losses in the three months ended March 31, 2021 and 2020.

The Company records changes in the fair value of its investments in corporate equity securities to "Other expense, net" on its condensed consolidated statements of operations. During the three months ended March 31, 2021 and 2020, the Company's net unrealized losses on corporate equity securities held at the conclusion of each period were as follows:

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Net unrealized losses	$ (52,295)	$ (39,440)

During the three months ended March 31, 2020, the Company received proceeds of $72.0 million related to the sale of the common stock of publicly traded companies, which had a total original weighted-average cost basis of $26.7 million. There were no sales of the common stock of publicly traded companies during the three months ended March 31, 2021.

As of March 31, 2021, the carrying value of the Company's equity investments without readily determinable fair values, which are recorded in "Other assets" on its condensed consolidated balance sheets, was $20.8 million.

G. 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 thousands)			
Balance at December 31, 2020	$ (15,678)	$ 334	$ (53,136)	$ (68,480)
Other comprehensive income (loss) before reclassifications	1,430	(218)	21,019	22,231
Amounts reclassified from accumulated other comprehensive income (loss)	—	—	12,947	12,947
Net current period other comprehensive income (loss)	1,430	(218)	33,966	35,178
Balance at March 31, 2021	$ (14,248)	$ 116	$ (19,170)	$ (33,302)
Balance at December 31, 2019	$ (895)	$ 503	$ (1,581)	$ (1,973)
Other comprehensive (loss) income before reclassifications	(2,662)	(764)	25,772	22,346
Amounts reclassified from accumulated other comprehensive income (loss)	—	—	(6,990)	(6,990)
Net current period other comprehensive (loss) income	(2,662)	(764)	18,782	15,356
Balance at March 31, 2020	$ (3,557)	$ (261)	$ 17,201	$ 13,383

H. Hedging

Foreign currency forward contracts - Designated as hedging instruments

The Company maintains a hedging program intended to mitigate the effect of changes in foreign exchange rates for a portion of the Company's 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. The Company recognizes realized gains and losses for the effective portion of such contracts in "Product revenues, net" in its condensed consolidated statements of operations in the same period that it recognizes the product revenues that were impacted by the hedged foreign exchange rate changes.

The Company formally documents the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as the Company's 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. The Company also formally assesses, 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 the Company 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, the Company would discontinue hedge accounting treatment prospectively. The Company measures effectiveness based on the change in fair value of the forward contracts and the fair value of the hypothetical foreign currency forward contracts with terms that match the critical terms of the risk being hedged. As of March 31, 2021, all hedges were determined to be highly effective.

The Company considers the impact of its counterparties' credit risk on the fair value of the foreign currency forward contracts. As of March 31, 2021 and December 31, 2020, credit risk did not change the fair value of the Company's foreign currency forward contracts.

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

	As of March 31, 2021	As of December 31, 2020
Foreign Currency	**(in thousands)**	
Euro	$ 892,995	$ 745,099
British pound sterling	250,528	160,427
Australian dollar	100,733	99,922
Canadian dollar	80,122	86,468
Total foreign currency forward contracts	$ 1,324,378	$ 1,091,916

Foreign currency forward contracts - Not designated as hedging instruments

The Company also enters 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. The Company recognizes realized gains and losses for such contracts in "Other expense, net" in its condensed consolidated statements of operations each period. As of March 31, 2021, the notional amount of the Company's outstanding foreign currency forward contracts where hedge accounting under U.S. GAAP is not applied was $386.7 million.

During the three months ended March 31, 2021 and 2020, the Company recognized the following related to foreign currency forward contacts in its condensed consolidated statements of operations:

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Designated as hedging instruments - Reclassified from AOCI		
Product revenues, net	$ (16,518)	$ 8,922
Not designated as hedging instruments		
Other expense, net	$ (7,997)	$ (16,229)
Total reported in the Condensed Consolidated Statement of Operations		
Product revenues, net	$ 1,723,305	$ 1,515,107
Other expense, net	$ (52,653)	$ (61,130)

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

As of March 31, 2021			
Assets		**Liabilities**	
Classification	**Fair Value**	**Classification**	**Fair Value**
	(in thousands)		
Prepaid expenses and other current assets	$ 7,039	Other current liabilities	$ (28,743)
Other assets	1,944	Other long-term liabilities	(433)
Total assets	$ 8,983	Total liabilities	$ (29,176)

As of December 31, 2020			
Assets		**Liabilities**	
Classification	**Fair Value**	**Classification**	**Fair Value**
	(in thousands)		
Prepaid expenses and other current assets	$ —	Other current liabilities	$ (59,184)
Other assets	—	Other long-term liabilities	(4,283)
Total assets	$ —	Total liabilities	$ (63,467)

As of March 31, 2021, the Company expects 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.

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 the Company's condensed consolidated balance sheets:

	As of March 31, 2021				
	Gross Amounts Recognized	**Gross Amounts Offset**	**Gross Amounts Presented**	**Gross Amounts Not Offset**	**Legal Offset**
Foreign currency forward contracts			**(in thousands)**		
Total assets	$ 8,983	$ —	$ 8,983	$ (8,983)	$ —
Total liabilities	(29,176)	—	(29,176)	8,983	(20,193)

	As of December 31, 2020				
	Gross Amounts Recognized	**Gross Amounts Offset**	**Gross Amounts Presented**	**Gross Amounts Not Offset**	**Legal Offset**
Foreign currency forward contracts			**(in thousands)**		
Total assets	$ —	$ —	$ —	$ —	$ —
Total liabilities	(63,467)	—	(63,467)	—	(63,467)

I. Inventories

Inventories consisted of the following:

	As of March 31, 2021	**As of December 31, 2020**
	(in thousands)	
Raw materials	$ 44,921	$ 46,232
Work-in-process	170,721	161,324
Finished goods	83,221	73,221
Total	$ 298,863	$ 280,777

J. Stock-based Compensation Expense and Share Repurchase Programs

Stock-based compensation expense

During the three months ended March 31, 2021 and 2020, the Company recognized the following stock-based compensation expense:

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Stock-based compensation expense by type of award:		
Restricted stock units (including PSUs) and restricted stock	$ 100,826	$ 97,149
Stock options	10,596	17,266
ESPP share issuances	5,615	2,485
Stock-based compensation expense related to inventories	(1,863)	(1,194)
Total stock-based compensation expense included in costs and expenses	$ 115,174	$ 115,706
Stock-based compensation expense by line item:		
Cost of sales	$ 1,431	$ 1,361
Research and development expenses	72,802	72,687
Sales, general and administrative expenses	40,941	41,658
Total stock-based compensation expense included in costs and expenses	115,174	115,706
Income tax effect	(31,251)	(64,246)
Total stock-based compensation expense, net of tax	$ 83,923	$ 51,460

Share repurchase programs

In 2019, the Company's Board of Directors approved a share repurchase program (the "2019 Share Repurchase Program"), pursuant to which the Company repurchased $500.0 million of its common stock in 2019 and 2020. During the three months ended March 31, 2020, the Company repurchased 1,403,868 shares of its common stock under the 2019 Share Repurchase Program for an aggregate of $300.0 million.

In November 2020, the Company's Board of Directors approved a new share repurchase program (the "2020 Share Repurchase Program"), pursuant to which the Company was authorized to repurchase up to $500.0 million of its common stock by December 31, 2022. During the three months ended March 31, 2021, the Company repurchased 1,988,941 shares of its common stock under the 2020 Share Repurchase Program for an aggregate of $424.9 million. As of March 31, 2021, the Company had repurchased the entire $500.0 million it was authorized to repurchase of its common stock under the 2020 Share Repurchase Program.

K. Income Taxes

The Company is subject to U.S. federal, state, and foreign income taxes. For the three months ended March 31, 2021 and 2020, the Company recorded provisions for income taxes of $167.8 million and $54.8 million, respectively.

The Company's effective tax rate of 20% for the three months ended March 31, 2021 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation. The Company's effective tax rate of 8% for the three months ended March 31, 2020 was lower than the U.S. statutory rate primarily due to a discrete benefit related to the write-off of a long-term intercompany receivable and excess tax benefits related to stock-based compensation.

On a periodic basis, the Company reassesses any valuation allowances that it maintains on its deferred tax assets, weighing positive and negative evidence to assess the recoverability of the deferred tax assets. As of December 31, 2020, the Company maintained a valuation allowance of $213.8 million related primarily to U.S. state and foreign tax attributes.

As part of the Tax Cut and Jobs Act of 2017, the Company is subject to a territorial tax system in which the requirement is to establish an accounting policy in providing for tax on Global Intangible Low Taxed Income ("GILTI") earned by certain foreign subsidiaries. The Company has elected to treat the impact of GILTI as a current tax expense in its provision for income taxes.

The Company has reviewed the tax positions taken, or to be taken, in its tax returns for all tax years currently open to examination by a taxing authority. Unrecognized tax benefits represent the aggregate tax effect of differences between tax return positions and the benefits recognized in the consolidated financial statements. As of March 31, 2021 and December 31, 2020, the Company had $81.8 million and $75.8 million, respectively, of net unrecognized tax benefits, which would affect the Company's tax rate if recognized. The Company does not expect that its unrecognized tax benefits will materially change within the next twelve months. The Company accrues interest and penalties related to unrecognized tax benefits as a component of its provision for income taxes. The Company did not recognize any material interest or penalties related to uncertain tax positions during the three months ended March 31, 2021 and 2020.

As of March 31, 2021, foreign earnings, which were not significant, have been retained by foreign subsidiaries for indefinite reinvestment. Upon repatriation of those earnings, in the form of dividends or otherwise, the Company could be subject to withholding taxes payable to the various foreign countries.

The Company files U.S. federal income tax returns and income tax returns in various state, local and foreign jurisdictions. The Company is no longer subject to any tax assessment from an income tax examination in the U.S. or any other major taxing jurisdiction for years before 2011, except where the Company has net operating losses or tax credit carryforwards that originate before 2011. The Company has various income tax audits ongoing at any time throughout the world.

L. Commitments and Contingencies

Revolving Credit Facilities

The Company and certain of its subsidiaries have entered into two credit agreements (the "Credit Agreements") with Bank of America, N.A., as administrative agent and the lenders referred to therein (the "Lenders"). The Credit Agreements were not drawn upon at closing and the Company has not drawn upon them to date. Amounts drawn pursuant to the Credit Agreements, if any, will be used for general corporate purposes. Any amounts borrowed under the Credit Agreements will bear interest, at the Company's option, at either a base rate or a Eurocurrency rate, in each case plus an applicable margin based on the Company's consolidated leverage ratio (the ratio of the Company's total consolidated funded indebtedness to the Company's consolidated EBITDA for the most recently completed four fiscal quarter period).

In September 2019, the Company and certain of its subsidiaries entered into a $500.0 million unsecured revolving facility (the "2019 Credit Agreement") with the Lenders, which matures on September 17, 2024. Under the 2019 Credit Agreement, the applicable margins on base rate loans range from 0.125% to 0.500% and the applicable margins on Eurocurrency loans range from 1.125% to 1.500%. The 2019 Credit Agreement provides a sublimit of $50.0 million for letters of credit.

In September 2020, the Company and certain of its subsidiaries entered into a $2.0 billion unsecured revolving facility (the "2020 Credit Agreement") with the Lenders, which matures on September 18, 2022. Under the 2020 Credit Agreement, the applicable margins on base rate loans range from 0.500% to 0.875% and the applicable margins on Eurocurrency loans range from 1.500% to 1.875%. The 2020 Credit Agreement does not support letters of credit.

Subject to satisfaction of certain conditions, the Company may request that the borrowing capacity for each of the Credit Agreements be increased by an additional $500.0 million. Any amounts borrowed pursuant to the Credit Agreements are guaranteed by certain of the Company's existing and future domestic subsidiaries, subject to certain exceptions.

The Credit Agreements contain customary representations and warranties and affirmative and negative covenants, including financial covenants to maintain (x) 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 and (y) a consolidated interest coverage ratio of

2.50 to 1.00, in each case measured on a quarterly basis. As of March 31, 2021, the Company was in compliance with the covenants described above. The Credit Agreements also contain 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 Agreements, which were not material to the Company's financial statements, were deferred and recorded over the term of the Credit Agreements.

Guaranties and Indemnifications

As permitted under Massachusetts law, the Company's Articles of Organization and By-laws provide that the Company will indemnify certain of its 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 the Company could be required to make under these indemnification provisions is unlimited. However, the Company has purchased directors' and officers' liability insurance policies that could reduce its monetary exposure and enable it to recover a portion of any future amounts paid. No indemnification claims currently are outstanding, and the Company believes the estimated fair value of these indemnification arrangements is minimal.

The Company customarily agrees in the ordinary course of its business to indemnification provisions in agreements with clinical trial investigators and sites in its drug development programs, sponsored research agreements with academic and not-for-profit institutions, various comparable agreements involving parties performing services for the Company and its real estate leases. The Company also customarily agrees to certain indemnification provisions in its drug discovery, development and commercialization collaboration agreements. With respect to the Company's 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 the Company's contractual obligations arising out of the research or clinical testing of the Company's compounds or drug 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 the Company, to violations of law by the Company or to certain breaches of the Company's contractual obligations. The indemnification provisions appearing in the Company's collaboration agreements are similar to those for the other agreements discussed above, but in addition provide some limited indemnification for its 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 the Company believes 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 the Company could be required to make under these provisions is generally unlimited. The Company has purchased insurance policies covering personal injury, property damage and general liability that reduce its exposure for indemnification and would enable it in many cases to recover all or a portion of any future amounts paid. The Company has never paid any material amounts to defend lawsuits or settle claims related to these indemnification provisions. Accordingly, the Company believes the estimated fair value of these indemnification arrangements is minimal.

Other Contingencies

The Company has certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a reserve for contingent liabilities when it is probable that future expenditures will be made, and such expenditures can be reasonably estimated. There were no material contingent liabilities accrued as of March 31, 2021 or December 31, 2020.

M. Additional Cash Flow Information

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

	Three Months Ended March 31,			
	2021		2020	
	Beginning of period	End of period	Beginning of period	End of period
	(in thousands)			
Cash and cash equivalents	$ 5,988,187	$ 6,304,330	$ 3,109,322	$ 3,593,412
Prepaid expenses and other current assets	658	8,577	8,004	8,552
Other assets	—	—	3,355	—
Cash, cash equivalents and restricted cash per condensed consolidated statement of cash flows	$ 5,988,845	$ 6,312,907	$ 3,120,681	$ 3,601,964

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

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 current 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, 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 approved to treat 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 up to 90% of people with CF. We are also pursuing genetic therapies to address the remaining 10% of people with CF.

Beyond CF, we continue to research and develop small molecule drug candidates for the treatment of serious diseases, including alpha-1 antitrypsin, or AAT, deficiency, APOL1-mediated kidney diseases and pain. We are also focused on developing cell and genetic therapies for various diseases in our pipeline, including sickle cell disease, or SCD, beta thalassemia, type 1 diabetes, or T1D, Duchenne muscular dystrophy, or DMD, myotonic dystrophy, or DM1, and CF. We are evaluating CTX001, a genetic therapy, as a potential treatment for SCD and transfusion-dependent beta thalassemia, or TDT, the most severe form of beta thalassemia, in Phase 1/2 clinical trials in collaboration with CRISPR Therapeutics AG, or CRISPR. In T1D, we are pursuing two programs for the transplant of functional islets into patients: transplantation of islet cells alone, using immunosuppression to protect the implanted cells, and implantation of the islet cells inside a novel immunoprotective device.

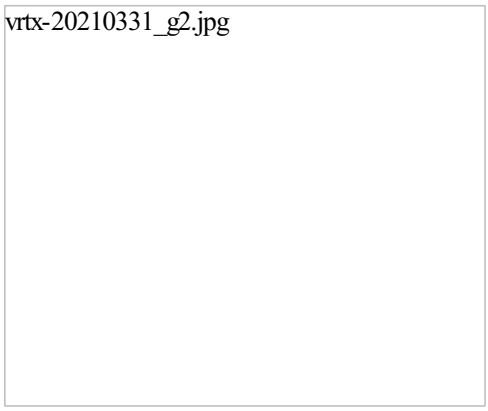
Financial Highlights

Revenues

In the first quarter of 2021, our net product revenues continued to increase due to the uptake of KAFTRIO in Europe and continued performance of TRIKAFTA in the U.S.

Expenses

Our total R&D and SG&A expenses increased to $648.1 million in the first quarter of 2021 as compared to $630.8 million in the first quarter of 2020. In the first quarter of 2021, cost of sales was approximately 11% of our net product revenues.



Business Updates

Cystic Fibrosis

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

Since the beginning of 2021, we have made the following progress with respect to our CF business.

- The Australian Therapeutic Goods Administration approved the use of TRIKAFTA (elexacaftor/tezacaftor/ivacaftor and ivacaftor) for people with CF 12 years of age and older who have at least one F508del mutation.
- We filed our post-marketing application with the European Medicines Agency, or EMA, for the expanded indication of KAFTRIO (elexacaftor/tezacaftor/ivacaftor and ivacaftor) to include children with CF 6 to 11 years of age.
- TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in 12 countries outside the U.S., including Denmark, Germany, Ireland, Israel, Switzerland and the countries within the UK.

Pipeline

We continue to advance a pipeline of potentially transformative small molecule, cell and genetic therapies aimed at serious diseases. Since the beginning of 2021, we have made the following progress in activities supporting these efforts.

Beta Thalassemia and Sickle Cell Disease

- We and our collaborator, CRISPR, are evaluating the use of a non-viral ex vivo CRISPR gene-editing therapy for the treatment of TDT and SCD. This approach aims to edit a person's hematopoietic stem cells to produce fetal hemoglobin in red blood cells, which has the potential to reduce or eliminate symptoms associated with the diseases.
- To date, more than 30 patients have been dosed in the clinical trials to evaluate CTX001 as a potential one-time curative therapy for people with severe SCD and TDT. Enrollment and dosing are ongoing in these clinical trials, and we expect to complete enrollment in 2021.
- In April, the EMA granted Priority Medicines Designation to CTX001 for TDT.

Alpha-1 Antitrypsin Deficiency

- We are evaluating multiple compounds with the potential to correct the misfolding of Z-AAT protein in the liver, in order to increase the systemic levels of functional AAT. Misfolded Z-AAT protein is the root cause of AAT deficiency and our small molecule corrector program targets both the liver and lung manifestations of the disease.
- Patients enrolled in the Phase 2 proof-of-concept clinical trial for the Z-AAT corrector, VX-864, have completed the 28-day dosing period. This clinical trial includes a 28-day safety follow-up period that is ongoing. We expect results from this clinical trial in the second quarter of 2021.

APOL1-Mediated Kidney Diseases

- We are evaluating the potential of inhibitors of APOL1 function in people with APOL1-mediated kidney diseases, including focal segmental glomerulosclerosis, or FSGS.
- Enrollment is ongoing in a Phase 2 proof-of-concept clinical trial designed to evaluate the reduction in proteinuria in people with APOL1-mediated FSGS after treatment with VX-147. We expect results from this clinical trial in the second half 2021.

Type 1 Diabetes

- We are evaluating a cell therapy designed to replace insulin-producing islet cells in people with T1D. We are pursuing two programs for the transplant of these fully-differentiated functional islets into patients: transplantation of islet cells alone, using immunosuppression to protect the implanted cells, and implantation of the islet cells inside a novel immunoprotective device.

- In March, the U.S. Food and Drug Administration, or FDA, granted Fast Track Designation and we initiated a Phase 1/2 clinical trial evaluating VX-880, our islet cells alone program. This clinical trial involves an infusion of fully differentiated, functional islet cells, and chronic administration of concomitant immunosuppressive therapy, to protect the islet cells from immune rejection.

Pain

- We are evaluating selective small molecule inhibitors of NaV1.8, a genetically validated, novel target for the treatment of pain, with the goal of preventing pain signals traveling from the sensory nerves to the central nervous system. We have previously demonstrated clinical proof-of-concept with a small molecule investigational treatment targeting NaV1.8, VX-150, in multiple pain indications including acute pain, neuropathic pain and musculoskeletal pain.
- Our selective NaV1.8 inhibitor, VX-548, demonstrated favorable safety, tolerability and pharmacokinetic profiles in Phase 1 clinical trials. In these clinical trials, the molecule exhibited a favorable profile at doses considerably lower than those required with our previous NaV1.8 inhibitors.
- We expect to advance VX-548 into Phase 2 proof-of-concept clinical trials for acute pain in the second half of 2021.

Investments in External Innovation

- In April, we amended our collaboration with CRISPR for the CTX001 programs in TDT and SCD. Under the terms of the revised collaboration agreement, we will lead worldwide development, manufacturing and commercialization of CTX001. Under the revised collaboration agreement, 60% of the net profits and net losses for CTX001 will be allocated to us and 40% of the net profits and net losses for CTX001 will be allocated to CRISPR. In connection with the closing of the transaction, we will pay a $900.0 million upfront payment to CRISPR and an additional one-time $200.0 million milestone payment upon regulatory approval of CTX001.
- In April, we entered into a research collaboration with Obsidian Therapeutics, Inc., or Obsidian, aimed at the discovery of novel therapies that regulate gene-editing for the treatment of serious diseases. This collaboration enables us to leverage Obsidian's cytoDRiVE® platform technology to discover gene-editing medicines whose therapeutic activity can be precisely controlled using small molecules.

COVID-19

We continue to monitor the impacts of the COVID-19 global pandemic on our business. COVID-19 has not 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 have adjusted our business operations in response to COVID-19, with a majority of our employees continuing to work remotely. We continue to monitor local COVID-19 trends and government guidance for each of our site locations, and are utilizing a phased, site-specific approach to assess employee access to our sites. Currently, all of our research and manufacturing sites are open to essential employees, as permitted by local laws. To provide a safe working environment for our on-site employees, we have, among other things, limited employee numbers at our open sites and increased safety measures, including at home and on-site testing in the U.S., enhanced cleaning and sanitation protocols, required use of personal protective equipment for all on-site employees, hand sanitation stations throughout our open sites and implementation of various social distancing measures while on-site.

Research

We continue to invest in our research programs and foster scientific innovation in order to identify and develop transformative medicines. Our strategy is to combine transformative advances in the understanding of human disease and the science of therapeutics in order to identify and develop new medicines. We believe that pursuing research in diverse areas allows us to balance the risks inherent in drug development and may provide drug 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.

Drug Discovery and Development

Discovery and development of a new pharmaceutical product is a difficult and lengthy process that requires significant financial resources along with extensive technical and regulatory expertise. Potential drug candidates are subjected to rigorous evaluations, driven in part by stringent regulatory considerations, designed to generate information concerning efficacy, side effects, proper dosage levels and a variety of other physical and chemical characteristics that are important in determining whether a drug candidate should be approved for marketing as a pharmaceutical product. Most chemical compounds that are investigated as potential drug candidates never progress into development, and most drug candidates that do advance into development never receive marketing approval. Our investments in drug candidates are subject to considerable risks. We closely monitor the results of our discovery, research, clinical trials and nonclinical studies and frequently evaluate our drug 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. For example, in 2020, we discontinued development of VX-814, a drug candidate for the treatment of AAT deficiency, based on the safety and pharmacokinetic profile of VX-814 observed in a Phase 2 clinical trial.

If we believe that data from a completed registration program support approval of a drug candidate, we submit an NDA or BLA to the FDA requesting approval to market the drug candidate in the U.S. and seek analogous approvals from comparable regulatory authorities in jurisdictions outside the U.S. To obtain approval, we must, among other things, demonstrate with evidence gathered in nonclinical studies and well-controlled clinical trials that the drug candidate is safe and effective for the disease it is intended to treat and that the manufacturing facilities, processes and controls for the manufacture of the drug candidate are adequate. The FDA and ex-U.S. regulatory authorities have substantial discretion in deciding whether or not a drug candidate should be granted approval based on the benefits and risks of the drug candidate in the treatment of a particular disease, and could delay, limit or deny regulatory approval. If regulatory delays are significant or regulatory approval is limited or denied altogether, our financial results and the commercial prospects for the drug candidate involved will be harmed.

Regulatory Compliance

Our marketing of pharmaceutical products is subject to extensive and complex laws and regulations. We have a corporate compliance program designed to actively identify, prevent and mitigate risk through the implementation of compliance policies and systems and through the promotion of a culture of compliance. Among other laws, regulations and standards, we are subject to various U.S. federal and state laws, and comparable laws in other jurisdictions, pertaining to health care fraud and abuse, including anti-kickback and false claims laws, and laws prohibiting the promotion of drugs for unapproved or off-label uses. Anti-kickback laws generally make it illegal for a prescription drug manufacturer to knowingly and willfully solicit, offer, receive or pay any remuneration in return for or to induce the referral of business, including the purchase or prescription of a particular drug that is reimbursed by a state or federal health care program. False claims laws prohibit anyone from knowingly or willfully presenting for payment to third-party payors, including Medicare and Medicaid, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. We are subject to laws and regulations that regulate the sales and marketing practices of pharmaceutical manufacturers, as well as laws such as the U.S. Foreign Corrupt Practices Act, which govern our international business practices with respect to payments to government officials. In addition, we are subject to various data protection and privacy laws and regulations in the U.S., E.U., U.K., Canada, Australia and other jurisdictions. We expect to continue to devote substantial resources to maintain, administer and expand these compliance programs globally.

Reimbursement

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. 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 by treating the underlying cause of CF and continue to provide access to our medicines.

In Europe and other ex-U.S. markets, we seek government reimbursement for our medicines on a country-by-country basis. This is necessary for each new medicine, as well as for label expansions for our current medicines. We successfully obtained reimbursement for KALYDECO in each significant ex-U.S. market within two years of approval, but experienced significant challenges in obtaining reimbursement for ORKAMBI in certain ex-U.S. markets. With the completion of reimbursement discussions in England and France in 2019, we have reimbursement for ORKAMBI or SYMKEVI in most of our significant ex-U.S. markets. In addition, in several ex-U.S. markets, including England, Ireland, Denmark and Australia, our reimbursement agreements include innovative arrangements that provide a pathway to access and rapid reimbursement for certain future CF medicines. For example, our existing reimbursement agreements in England, Ireland, and Denmark have been expanded to include KAFTRIO. We expect to continue to focus significant resources to obtain appropriate reimbursement for our products in ex-U.S. markets.

Strategic Transactions

Acquisitions

As part of our business strategy, we seek to acquire drugs, drug candidates and other technologies and businesses that have the potential to complement our ongoing research and development efforts. In 2019, we invested significantly in business development transactions designed to augment our pipeline, including the acquisition of Semma Therapeutics, Inc., or Semma, a privately-held company focused on the use of stem cell-derived human islets as a potentially curative treatment for T1D, and Exonics Therapeutics, Inc., or Exonics, a privately-held company focused on creating transformative gene-editing therapies to repair mutations that cause DMD and other severe neuromuscular diseases, including DM1. We expect to continue to identify and evaluate potential acquisitions and may include larger transactions or later-stage assets.

Collaboration and Licensing Arrangements

We enter into arrangements with third parties, including collaboration and licensing arrangements, for the development, manufacture and commercialization of drugs, drug candidates and other technologies that have the potential to complement our ongoing research and development efforts. We expect to continue to identify and evaluate collaboration and licensing opportunities that may be similar to or different from the collaborations and licenses that we have engaged in previously.

In-License Agreements

We have entered into collaborations with biotechnology and pharmaceutical companies in order to acquire rights or to license drug candidates or technologies that enhance our pipeline and/or our research capabilities. Over the last several years, we entered into collaboration agreements with a number of companies, including Affinia Therapeutics, Inc., Arbor Biotechnologies, Inc., CRISPR, Kymera Therapeutics, Inc., Moderna, Inc., Molecular Templates, Inc., Obsidian, and Skyhawk Therapeutics, Inc. Generally, when we in-license a technology or drug 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 research and development expenses; however, depending on many factors, including the structure of the collaboration, the significance of the in-licensed drug candidate to the collaborator's operations and the other activities in which our collaborators are engaged, the accounting for these transactions can vary significantly. In the first quarter of 2021 and 2020, our research and development expenses included $1.7 million and $36.3 million, respectively, related to upfront and milestones payments pursuant to our collaboration agreements.

Joint Development and Commercialization Agreement with CRISPR

In 2017, we entered into a joint development and commercialization agreement, or JDCA, with CRISPR pursuant to which we are co-developing and preparing to co-commercialize CTX001 for TDT and SCD. This JDCA was entered into following our exercise of an option to co-develop and co-commercialize the hemoglobinopathies program that was contained in the collaboration agreement that we entered into with CRISPR in 2015.

In April 2021, we and CRISPR entered into an amended and restated joint development and commercialization agreement, or A&R JDCA. Under the terms of the A&R JDCA, we will lead worldwide development, manufacturing and commercialization of CTX001. Additionally, 60% of the net profits and net losses for CTX001 will be allocated to us and

40% of the net profits and net losses for CTX001 will be allocated to CRISPR. In connection with the closing of the transaction, we will pay a $900.0 million upfront payment to CRISPR and an additional one-time $200.0 million milestone payment upon regulatory approval of CTX001. The closing of the transaction contemplated by the A&R JDCA is subject to certain conditions including the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act and any other required antitrust clearance. We expect to record the $900.0 million upfront payment to "Research and development expenses" upon closing of the transaction contemplated by the A&R JDCA.

Out-License Agreements

We also have out-licensed internally developed programs to collaborators who are leading the development of these programs. These out-license arrangements include our agreement with Merck KGaA, Darmstadt, Germany, which licensed oncology research and development programs from us in early 2017. 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.

Please refer to Note C, "Collaborative Arrangements," for further information regarding our in-license agreements and out-license agreements.

Strategic Investments

In connection with our business development activities, we have periodically made equity investments in our collaborators. As of March 31, 2021, we held strategic equity investments in public companies and certain private companies, and we plan 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. 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.

In the first quarter of 2021 and 2020, we recorded within other income (expense), losses of $52.3 million and $44.9 million, respectively, related to changes in the fair value of our strategic investments, and from sales of certain equity investments. As of March 31, 2021, the fair value of our investments in publicly traded companies was $143.5 million. To the extent that we continue to hold strategic investments, particularly strategic investments in publicly traded companies, we will record other income (expense) related to these strategic investments on a quarterly basis. Due to the increased volatility of the global markets, including as a result of COVID-19, and the high volatility of stocks in the biotechnology industry, we expect the value of these strategic investments to fluctuate and that the increases or decreases in the fair value of these strategic investments will continue to have material impacts on our net income (expense) and our profitability on a quarterly and/or annual basis.

RESULTS OF OPERATIONS

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages and per share amounts)			
Revenues	$ 1,724,305	$ 1,515,107	$ 209,198	14 %
Operating costs and expenses	836,479	794,883	41,596	5 %
Income from operations	887,826	720,224	167,602	23 %
Other non-operating expense, net	(66,866)	(62,690)	(4,176)	7 %
Provision for income taxes	167,822	54,781	113,041	206 %
Net income	$ 653,138	$ 602,753	$ 50,385	8 %
Net income per diluted common share	$ 2.49	$ 2.29		
Diluted shares used in per share calculations	261,916	263,515		

Net Income

Our net income increased in the first quarter of 2021 as compared to the first quarter of 2020 primarily due to increased revenues resulting from the uptake of KAFTRIO in Europe and continued performance of TRIKAFTA in the U.S. Our increased revenues were partially offset by increased operating costs and expenses, primarily as a result of increased cost of sales consistent with increased product revenues, and an increase in the provision for income taxes. The increase in our provision for income taxes was the result of our increased profitability and a discrete tax benefit recognized in the first quarter of 2020 for which there was no corresponding benefit in the first quarter of 2021.

Revenues

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Product revenues, net	$ 1,723,305	$ 1,515,107	$ 208,198	14 %
Other revenues	1,000	—	1,000	**
Total revenues	$ 1,724,305	$ 1,515,107	$ 209,198	14 %

** Not meaningful

Product Revenues, Net

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
TRIKAFTA/KAFTRIO	$ 1,193,217	$ 895,233	$ 297,984	33 %
SYMDEKO/SYMKEVI	125,049	173,159	(48,110)	(28) %
ORKAMBI	218,697	234,138	(15,441)	(7) %
KALYDECO	186,342	212,577	(26,235)	(12) %
Total product revenues, net	$ 1,723,305	$ 1,515,107	$ 208,198	14 %

In the first quarter of 2021, our net product revenues increased by $208.2 million as compared to the first quarter of 2020. The increase in our net product revenues in the first quarter of 2021 was primarily due to the uptake of KAFTRIO, which was approved in Europe in the third quarter of 2020, and the continued performance of TRIKAFTA in the U.S. Decreases in revenues for our products other than TRIKAFTA/KAFTRIO were primarily the result of patients switching from these medicines to TRIKAFTA/KAFTRIO. In the first quarter of 2021 and 2020, our net product revenues included $469.9 million and $327.5 million, respectively, from ex-U.S. markets.

Other Revenues

Our other revenues were $1.0 million related to a collaborative milestone that we earned in the first quarter of 2021. We did not record any other revenues in the first quarter of 2020. 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. Our

future royalty revenues will be dependent on if, and when, our collaborators are able to successfully develop drug candidates that we have out-licensed to them.

Operating Costs and Expenses

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Cost of sales	$ 192,329	$ 162,497	$ 29,832	18 %
Research and development expenses	455,973	448,528	7,445	2 %
Sales, general and administrative expenses	192,077	182,258	9,819	5 %
Change in fair value of contingent consideration	(3,900)	1,600	(5,500)	**
Total costs and expenses	$ 836,479	$ 794,883	$ 41,596	5 %

** Not meaningful

Cost of Sales

Our cost of sales primarily consists of third-party royalties payable on our net sales of our products as well as the cost of producing inventories that corresponded to product revenues for the reporting period. Pursuant to our agreement with the Cystic Fibrosis Foundation, or CFF, 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 approximately 11% in each of the first quarter of 2021 and 2020, respectively.

Research and Development Expenses

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Research expenses	$ 129,748	$ 157,270	$ (27,522)	(17) %
Development expenses	326,225	291,258	34,967	12 %
Total research and development expenses	$ 455,973	$ 448,528	$ 7,445	2 %

Our research and development expenses include internal and external costs incurred for research and development of our drugs and drug candidates and expenses related to certain technology that we acquire or license through business development transactions. 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 drugs or drug candidates, because the employees within our research and development groups typically are deployed across multiple research and development programs. These internal costs are significantly greater than our external costs, such as the costs of services provided to us by clinical research organizations and other outsourced research, which we allocate by individual program. All research and development costs for our drugs and drug candidates are expensed as incurred.

Since January 2019, we have incurred approximately $4.0 billion in research and development expenses associated with drug discovery and development. The successful development of our drug 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 drug 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 drug candidates to market are not available.

In 2020 and the first quarter of 2021, costs related to our CF programs represented the largest portion of our development costs. Any estimates regarding development and regulatory timelines for our drug candidates are highly subjective and

subject to change. Until we have data from Phase 3 clinical trials, we cannot make a meaningful estimate regarding when, or if, a clinical development program will generate revenues and cash flows.

Research Expenses

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Research Expenses:				
Salary and benefits	$ 34,742	$ 34,269	$ 473	1 %
Stock-based compensation expense	21,002	26,409	(5,407)	(20) %
Outsourced services and other direct expenses	40,106	30,853	9,253	30 %
Collaborative payments	1,650	36,250	(34,600)	(95) %
Infrastructure costs	32,248	29,489	2,759	9 %
Total research expenses	$ 129,748	$ 157,270	$ (27,522)	(17) %

We expect to continue to invest in our research programs with a focus on creating transformative medicines for serious diseases. Our research expenses have historically fluctuated, and are expected to continue to fluctuate, from one period to another due to upfront and milestone payments related to our business development activities that are reflected in the preceding table as collaborative payments. Our research expenses, excluding these collaborative payments, have been increasing over the last several years as we have invested in our pipeline and expanded our cell and genetic therapy capabilities.

Development Expenses

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Development Expenses:				
Salary and benefits	$ 84,530	$ 79,598	$ 4,932	6 %
Stock-based compensation expense	51,800	46,278	5,522	12 %
Outsourced services and other direct expenses	132,812	116,433	16,379	14 %
Infrastructure costs	57,083	48,949	8,134	17 %
Total development expenses	$ 326,225	$ 291,258	$ 34,967	12 %

Our development expenses increased by 12% in the first quarter of 2021 as compared to the first quarter of 2020, primarily due to increased expenses related to our diversifying pipeline, including clinical trials, headcount and infrastructure costs. Upon closing of the transaction contemplated by the A&R JDCA, we expect to record our $900.0 million upfront payment to CRISPR as a development expense.

Sales, General and Administrative Expenses

	Three Months Ended March 31,		Increase/(Decrease)	
	2021	2020	$	%
	(in thousands, except percentages)			
Sales, general and administrative expenses	$ 192,077	$ 182,258	$ 9,819	5 %

Sales, general and administrative expenses increased by 5% in the first quarter of 2021 as compared to the first quarter of 2020, primarily due to the incremental investment to support the commercialization of our medicines and increased support for our CF pipeline products and other disease areas.

Contingent Consideration

The fair value of contingent consideration potentially payable to Exonics' former equity holders decreased $3.9 million in the first quarter of 2021 and increased $1.6 million in the first quarter of 2020.

Other Non-Operating Income (Expense), Net

Interest Income

Interest income decreased from $12.6 million in the first quarter of 2020 to $1.5 million in the first quarter of 2021 primarily due to a decrease in prevailing market interest rates despite an increase in our cash equivalents and available-for-sale debt securities. Our future interest income will be 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 $15.7 million in the first quarter of 2021 as compared to $14.1 million in the first quarter of 2020. The majority of our interest expense in these periods was related to imputed interest expense associated with our leased corporate headquarters in Boston. Our future interest expense will be dependent on whether, and to what extent, we borrow amounts under our credit facilities.

Other Income (Expense), Net

We recorded expenses of $52.7 million and $61.1 million to other income (expense), net in the first quarter of 2021 and 2020, respectively. Our other income (expense), net in these periods was primarily related to changes in the fair value of our strategic investments. 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

Our provision for income taxes was $167.8 million and $54.8 million in the first quarter of 2021 and 2020, respectively. Our effective tax rate of 20% for the first quarter of 2021 was lower than the U.S. statutory rate primarily due to excess tax benefits related to stock-based compensation. Our effective tax rate of 8% for the first quarter of 2020 was lower than the U.S. statutory rate primarily due to a discrete benefit related to the write off of a long-term intercompany receivable and excess tax benefits related to stock-based compensation.

LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of March 31, 2021 and December 31, 2020:

	March 31, 2021	December 31, 2020	Increase/(Decrease) $	Increase/(Decrease) %
	(in thousands)			
Cash, cash equivalents and marketable securities	$ 6,923,968	$ 6,658,897	$ 265,071	4 %
Working Capital				
Total current assets	8,539,307	8,133,379	405,928	5 %
Total current liabilities	(1,944,050)	(1,877,533)	66,517	4 %
Total working capital	$ 6,595,257	$ 6,255,846	$ 339,411	5 %

As of March 31, 2021, total working capital was $6.6 billion, which represented an increase of $339 million from $6.3 billion as of December 31, 2020. The increase in total working capital in the first quarter of 2021 was primarily related to $921.1 million of cash provided by operations partially offset by $425.0 million of cash used to repurchase our common stock pursuant to our share repurchase program and expenditures for property and equipment of $70.9 million.

Sources of Liquidity

As of March 31, 2021, we had cash, cash equivalents and marketable securities of $6.9 billion, which represented an increase of $265.1 million from $6.7 billion as of December 31, 2020. 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 may borrow up to a total of $2.5 billion pursuant to two revolving credit facilities. We may repay and reborrow amounts under these revolving credit agreements without penalty. Subject to certain conditions, we may request that the

borrowing capacity for each of the credit agreements be increased by an additional $500.0 million, for a total of $3.5 billion collectively.

Other possible sources of future liquidity include commercial debt, public and private offerings of our equity and debt securities, strategic sales of assets or businesses and financial transactions. Negative covenants in our credit agreement may prohibit or limit our ability to access these sources of liquidity. As of March 31, 2021, we were in compliance with these covenants.

Future Capital Requirements

We have significant future capital requirements, including:

- significant expected operating expenses to conduct research and development activities and to operate our organization; and
- substantial facility and finance lease obligations.

In addition:

- We have entered into certain collaboration agreements with third parties that include the funding of certain research, development and commercialization efforts. Certain of our business development transactions, including collaborations and 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. We may enter into additional business development transactions, including acquisitions, collaborations and equity investments, that require additional capital.
- To the extent we borrow amounts under the credit agreements we entered into in 2020 and 2019, we would be required to repay any outstanding principal amounts in 2022 or 2024, respectively.
- Upon closing of the transaction contemplated by the A&R JDCA, we will pay a $900.0 million upfront payment to CRISPR.

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 and do not expect COVID-19 to have an adverse effect on our liquidity. 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 drug candidates to the market, the level of our business development activities and the number, breadth, cost and prospects of our research and development programs.

Financing Strategy

We may 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.

CONTRACTUAL COMMITMENTS AND OBLIGATIONS

Our commitments and obligations were reported in our Annual Report on Form 10-K for the year ended December 31, 2020, which was filed with the Securities and Exchange Commission, or SEC, on February 11, 2021. Except for the following, there have been no material changes from the contractual commitments and obligations previously disclosed in that Annual Report on Form 10-K:

- Upon closing of the transaction contemplated by the A&R JDCA, we will pay a $900.0 million upfront payment to CRISPR, and CRISPR may earn an additional one-time $200.0 million milestone payment upon receipt of the first marketing approval of CTX001 from the FDA or the European Commission.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

Our discussion and analysis of our financial condition and results of operations are based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the U.S. The preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate. During the three months ended March 31, 2021, 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, 2020, which was filed with the SEC on February 11, 2021.

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, 2020, which was filed with the SEC on February 11, 2021.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management (under the supervision and with the participation of our chief executive officer and chief financial officer), after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q, has concluded that, based on such evaluation, as of March 31, 2021 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Controls Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended March 31, 2021 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, 2020, which was filed with the SEC on February 11, 2021. 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 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;
- our expectations regarding clinical trials, development timelines, regulatory authority filings, submissions and potential approvals and label expansions for ivacaftor, lumacaftor, tezacaftor, elexacaftor, and any combination regimen;
- 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 medicines or any of our other drug candidates for which we obtain regulatory approval;
- our expectations regarding the timing and structure of clinical trials of our drugs, drug candidates and other pipeline programs and the expected timing of our receipt of data from our ongoing and planned clinical trials;
- 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 drug 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;
- our expectations regarding the potential benefits and commercial potential of our product candidates, including the potential approach to treating 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 CTX001 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;
- the timing of the potential closing of the transaction contemplated by the A&R JDCA, including satisfaction of closing conditions, and the future activities of the parties pursuant to the A&R JDCA;

- our expectation that we will record the $900.0 million upfront payment to CRISPR as a research and development expense upon the closing of the transaction contemplated by the A&R JDCA;
- 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, including the impact of the Coronavirus Aid, Relief and Economic Security Act;
- our ability to use our research programs to identify and develop new drug 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, 2020, which was filed with the SEC on February 11, 2021, 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 November 2020, our Board of Directors approved a share repurchase program (the "2020 Share Repurchase Program"), pursuant to which we were authorized to repurchase up to $500.0 million of our common stock by December 31, 2022. As of March 31, 2021, we had repurchased the entire $500.0 million of common stock that was authorized under the 2020 Share Repurchase Program. The table set forth below shows repurchases of securities by us during the three months ended March 31, 2021 under our 2020 Share Repurchase Program.

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs (1)	Approximate Dollar Value of Shares that May Yet be Purchased Under the Plans or Programs (1)
January 1, 2021 to January 31, 2021	—	$ —	—	$ 424,912,410
February 1, 2021 to February 28, 2021	1,988,941	$ 213.64	1,988,941	$ —
March 1, 2021 to March 31, 2021	—	$ —	—	$ —
Total	1,988,941	$ 213.64	1,988,941	$ —

(1) Under our 2020 Share Repurchase Program, we were authorized to purchase shares from time to time through open market or privately negotiated transactions. Such purchases were made pursuant to Rule 10b5-1 plans or other means as determined by our management and in accordance with the requirements of the Securities and Exchange Commission.

Item 5. Other Information

As previously announced, Paul Silva, our Senior Vice President and Chief Accounting Officer, retired from the Company on April 30, 2021.

Effective May 1, 2021, Kristen Ambrose, 45, will become our Chief Accounting Officer. Ms. Ambrose has been our Senior Vice President, Accounting, Tax, Treasury, Strategic Sourcing and Corporate Services since March 2021. From February 2003 until she joined the Company, Ms. Ambrose held roles of increasing responsibility at Boston Scientific Corporation, a medical device company, most recently as Vice President of Finance and Controller of the Global Endoscopy Division from July 2019 to March 2021 and as Vice President of Global Internal Audit from February 2017 to June 2019.

Prior to Boston Scientific Corporation, Ms. Ambrose served as an accountant at Ernst & Young LLP. She received her B.S. from the University of Virginia and is a Certified Public Accountant.

Item 6. Exhibits

Exhibit Number	Exhibit Description
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance - the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation
101.LAB	XBRL Taxonomy Extension Labels
101.PRE	XBRL Taxonomy Extension Presentation
101.DEF	XBRL Taxonomy Extension Definition
104	Cover Page Interactive Data File—the cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.

SIGNATURES

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

Vertex Pharmaceuticals Incorporated

April 30, 2021 By: /s/ Charles F. Wagner, Jr.

Charles F. Wagner, Jr.

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