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VERTEX PHARMACEUTICALS INCORPORATED  
 FORM 10-Q  
 FOR THE QUARTER ENDED SEPTEMBER 30, 2018

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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” and “SYMKEPT” are registered trademarks of Vertex. Other brands, names and trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners.

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## 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		Nine Months Ended	
	September 30,		September 30,	
	2018	2017	2018	2017
Revenues:				
Product revenues, net	\$782,511	\$549,642	\$2,170,152	\$1,544,252
Royalty revenues	1,238	2,231	3,679	6,643
Collaborative revenues	786	26,292	3,660	286,123
Total revenues	784,535	578,165	2,177,491	1,837,018
Costs and expenses:				
Cost of sales	111,255	72,874	287,250	191,067
Research and development expenses	330,510	454,947	978,595	1,017,961
Sales, general and administrative expenses	137,295	120,710	404,406	361,285
Restructuring (income) expenses	(174 )	337	(188 )	13,859
Intangible asset impairment charge	—	255,340	—	255,340
Total costs and expenses	578,886	904,208	1,670,063	1,839,512
Income (loss) from operations	205,649	(326,043 )	507,428	(2,494 )
Interest expense, net	(8,143 )	(13,574 )	(29,346 )	(45,003 )
Other (expense) income, net	(60,995 )	(77,553 )	89,662	(80,634 )
Income (loss) before provision for (benefit from) income taxes	136,511	(417,170 )	567,744	(128,131 )
Provision for (benefit from) income taxes	8,055	(125,903 )	5,737	(117,581 )
Net income (loss)	128,456	(291,267 )	562,007	(10,550 )
Loss (income) attributable to noncontrolling interest	290	188,315	(15,638 )	173,350
Net income (loss) attributable to Vertex	\$128,746	\$(102,952)	\$546,369	\$162,800

## Amounts per share attributable to Vertex common shareholders:

## Net income (loss):

Basic \$0.51 \$(0.41 ) \$2.15 \$0.66

Diluted \$0.50 \$(0.41 ) \$2.11 \$0.64

## Shares used in per share calculations:

Basic 254,905 250,268 254,096 247,963

Diluted 259,788 250,268 258,972 252,095

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net income (loss)	\$ 128,456	\$(291,267)	\$ 562,007	\$(10,550 )
Changes in other comprehensive income (loss):				
Unrealized holding gains (losses) on marketable securities, net	224	5,961	137	(7,786 )
Unrealized gains (losses) on foreign currency forward contracts, net of tax of zero, \$0.9 million, \$0.5 million and \$2.9 million, respectively	4,029	(5,453 )	29,062	(27,379 )
Foreign currency translation adjustment	659	(3,884 )	6,800	(11,137 )
Total changes in other comprehensive income (loss)	4,912	(3,376 )	35,999	(46,302 )
Comprehensive income (loss)	133,368	(294,643 )	598,006	(56,852 )
Comprehensive loss (income) attributable to noncontrolling interest	290	188,315	(15,638 )	173,350
Comprehensive income (loss) attributable to Vertex	\$ 133,658	\$(106,328)	\$ 582,368	\$ 116,498

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

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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Balance Sheets

(unaudited)

(in thousands, except share and per share amounts)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	\$2,478,302	\$1,665,412
Marketable securities	577,583	423,254
Restricted cash and cash equivalents (VIE)	8,143	1,489
Accounts receivable, net	379,755	281,343
Inventories	124,150	111,830
Prepaid expenses and other current assets	118,678	165,635
Total current assets	3,686,611	2,648,963
Property and equipment, net	808,352	789,437
Intangible assets	29,000	29,000
Goodwill	50,384	50,384
Other assets	46,493	28,230
Total assets	\$4,620,840	\$3,546,014
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$92,149	\$73,994
Accrued expenses	504,240	443,961
Capital lease obligations, current portion	11,511	22,531
Early access sales accrual	324,251	232,401
Other liabilities, current portion	60,357	34,373
Total current liabilities	992,508	807,260
Capital lease obligations, excluding current portion	15,141	20,496
Deferred tax liability	9,414	6,341
Construction financing lease obligation, excluding current portion	561,389	563,406
Advance from collaborator, excluding current portion	81,610	78,431
Other liabilities, excluding current portion	26,297	27,774
Total liabilities	1,686,359	1,503,708
Commitments and contingencies		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding	—	—
Common stock, \$0.01 par value; 500,000,000 shares authorized, 255,610,812 and 253,253,362 shares issued and outstanding, respectively	2,551	2,512
Additional paid-in capital	7,443,263	7,157,362
Accumulated other comprehensive income (loss)	307	(11,572 )
Accumulated deficit	(4,540,005 )	(5,119,723 )
Total Vertex shareholders' equity	2,906,116	2,028,579
Noncontrolling interest	28,365	13,727
Total shareholders' equity	2,934,481	2,042,306
Total liabilities and shareholders' equity	\$4,620,840	\$3,546,014

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



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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Statements of Shareholders' Equity and Noncontrolling Interest

(unaudited)

(in thousands)

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Vertex Shareholders' Equity	Noncontrolling Interest	Total Shareholders' Equity
	Shares	Amount						
Balance at December 31, 2016	248,301	\$2,450	\$6,506,795	\$ 21,173	\$(5,373,836)	\$1,156,582	\$ 181,609	\$1,338,191
Cumulative effect adjustment for adoption of new accounting guidance	—	—	9,371	—	(9,371 )	—	—	—
Other comprehensive loss, net of tax	—	—	—	(46,302 )	—	(46,302 )	—	(46,302 )
Net income	—	—	—	—	162,800	162,800	(173,350 )	(10,550 )
Issuance of common stock under benefit plans	4,382	50	298,956	—	—	299,006	33	299,039
Stock-based compensation expense	—	—	218,991	—	—	218,991	—	218,991
VIE noncontrolling interest upon deconsolidation	—	—	—	—	—	—	3,910	3,910
Other VIE activity	—	—	—	—	—	—	(35 )	(35 )
Balance at September 30, 2017	252,683	\$2,500	\$7,034,113	\$(25,129 )	\$(5,220,407)	\$1,791,077	\$ 12,167	\$1,803,244
Balance at December 31, 2017	253,253	\$2,512	\$7,157,362	\$(11,572 )	\$(5,119,723)	\$2,028,579	\$ 13,727	\$2,042,306
Cumulative effect adjustment for adoption of new accounting guidance	—	—	—	(24,120 )	33,349	9,229	—	9,229
Other comprehensive income, net of tax	—	—	—	35,999	—	35,999	—	35,999



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Net income	—	—	—	—	546,369	546,369	15,638	562,007
Repurchases of common stock	(1,283 )	(13 )	(211,025 )	—	—	(211,038 )	—	(211,038 )
Issuance of common stock under benefit plans	3,641	52	250,368	—	—	250,420	—	250,420
Stock-based compensation expense	—	—	246,558	—	—	246,558	—	246,558
Other VIE activity	—	—	—	—	—	—	(1,000 )	(1,000 )
Balance at September 30, 2018	255,611	\$2,551	\$7,443,263	\$ 307	\$(4,540,005)	\$2,906,116	\$ 28,365	\$2,934,481

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

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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Statements of Cash Flows

(unaudited)

(in thousands)

	Nine Months Ended September 30,	
	2018	2017
Cash flows from operating activities:		
Net income (loss)	\$562,007	\$(10,550 )
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Stock-based compensation expense	246,104	216,990
Depreciation expense	53,594	44,965
Write-downs of inventories to net realizable value	13,089	11,138
Deferred income taxes	3,595	(113,969 )
Unrealized gain on equity securities	(88,217 )	—
Intangible asset impairment charge	—	255,340
Acquired in-process research and development	—	160,000
Deconsolidation of VIE	—	76,644
Other non-cash items, net	10,701	(2,841 )
Changes in operating assets and liabilities:		
Accounts receivable, net	(75,167 )	(60,165 )
Inventories	(24,461 )	(30,226 )
Prepaid expenses and other assets	31,797	(84,296 )
Accounts payable	23,023	6,925
Accrued expenses and other liabilities	199,643	147,476
Net cash provided by operating activities	955,708	617,431
Cash flows from investing activities:		
Purchases of available-for-sale debt securities	(329,367 )	(431,653 )
Maturities of available-for-sale debt securities	308,406	247,149
Expenditures for property and equipment	(79,803 )	(56,437 )
Decrease in “Restricted cash and cash equivalents (VIE)” due to deconsolidation	—	(61,602 )
Investment in equity securities	(60,490 )	—
Purchase of in-process research and development	—	(160,000 )
Net cash used in investing activities	(161,254 )	(462,543 )
Cash flows from financing activities:		
Issuances of common stock under benefit plans	245,754	298,205
Repurchase of common stock	(207,038 )	—
Payments on revolving credit facility	—	(300,000 )
Advance from collaborator	7,500	10,000
Payments on capital lease obligations	(19,792 )	(14,188 )
Proceeds from capital lease financing	3,417	—
Proceeds related to construction financing lease obligation	9,566	4,700
Payments on construction financing lease obligation	(4,866 )	(412 )
Repayments of advanced funding	(3,714 )	(3,132 )
Other financing activities	(1,000 )	—
Net cash provided by (used in) financing activities	29,827	(4,827 )
Effect of changes in exchange rates on cash	(4,756 )	5,001
Net increase in cash and cash equivalents	819,525	155,062
Cash, cash equivalents and restricted cash—beginning of period	1,667,526	1,231,707

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Cash, cash equivalents and restricted cash—end of period	\$2,487,051	\$1,386,769
Supplemental disclosure of cash flow information:		
Cash paid for interest	\$50,017	\$51,990
Cash paid for income taxes	\$10,316	\$4,154
Capitalization of costs related to construction financing lease obligation	\$3,389	\$33,827
Issuances of common stock from employee benefit plans receivable	\$5,509	\$868
Accrued share repurchase liability	\$4,000	\$—

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

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

A. Basis of Presentation and Accounting Policies

Basis of Presentation

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

The condensed consolidated financial statements reflect the operations of (i) the Company, (ii) its wholly-owned subsidiaries and (iii) consolidated variable interest entities (VIEs). All material intercompany balances and transactions have been eliminated. The Company operates in one segment, pharmaceuticals. As of September 30, 2017, the Company deconsolidated Parion Sciences, Inc. (“Parion”), a VIE the Company had consolidated since 2015. The Company's condensed consolidated statement of operations for the interim period ended September 30, 2018 excludes Parion. Please refer to Note B, “Collaborative Arrangements and Acquisitions,” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2017 that was filed with the Securities and Exchange Commission (the “SEC”) on February 15, 2018 (the “2017 Annual Report on Form 10-K”) for further information regarding the deconsolidation of Parion.

Certain information and footnote disclosures normally included in the Company’s 2017 Annual Report on Form 10-K have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the interim periods ended September 30, 2018 and 2017.

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, 2017, which are contained in the 2017 Annual Report on Form 10-K.

Use of Estimates

The preparation of condensed consolidated financial statements in accordance with 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. Significant estimates in these condensed consolidated financial statements have been made in connection with the calculation of revenues, inventories, research and development expenses, stock-based compensation expense, the fair value of intangible assets, goodwill, contingent consideration, noncontrolling interest, the consolidation and deconsolidation of VIEs, leases, the fair value of cash flow hedges, deferred tax asset valuation allowances and the provision for or benefit from income taxes. 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 Accounting Standards

Revenue Recognition

In 2014, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) No. 2014-09, Revenues from Contracts with Customers (Topic 606) (“ASC 606”). The new guidance became effective January 1, 2018. ASC 606 applies a more principles-based approach to recognizing revenue. Under ASC 606, revenue is recognized when a customer obtains control of promised goods or services in an amount that reflects the consideration that an entity expects to receive in exchange for those goods or services. In addition, the standard requires disclosure of the nature, amount, timing and uncertainty of revenue and cash flows arising from contracts with customers. The Company adopted ASC 606 on January 1, 2018 using the modified-retrospective adoption method for all contracts that were not completed as of the date of adoption. Under the modified-retrospective method, the Company recognized the cumulative effect of applying the standard within “Accumulated deficit” on its condensed consolidated

balance sheet as of January 1, 2018.

For all reporting periods, the Company has not disclosed the value of unsatisfied performance obligations for all product revenue contracts with an original expected length of one year or less, which is an optional exemption that is permitted under

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the adoption rules.

Based on the Company's review of existing customer contracts as of January 1, 2018, it concluded that the only significant impact that the adoption of ASC 606 had on its financial statements relates to shipments of ORKAMBI under early access programs in France. Prior to the adoption of ASC 606, the Company did not recognize revenue on the proceeds received from sales of ORKAMBI under early access programs in France because the price was not fixed or determinable based on the status of ongoing pricing discussions. As of January 1, 2018, the Company recorded a cumulative effect adjustment to its accumulated deficit of \$8.3 million related to the adoption of ASC 606, which primarily represented the Company's estimated amount of consideration it expects to retain related to these shipments that will not be subject to a significant reversal in amounts recognized, net of costs previously deferred related to these shipments. Please refer to Note B, "Revenue Recognition," for further information.

The Company concluded that the remaining \$6.9 million that was recorded as deferred revenue as of December 31, 2017 related to the Company's 2008 sale of its HIV protease inhibitor royalty stream is not subject to ASC 606 because it was initially accounted for pursuant to ASC 470, Debt, which is not under the scope of ASC 606. The Company will continue to recognize the payment received as royalty revenues over the expected life of the collaboration agreement with GlaxoSmithKline plc based on the units-of-revenue method.

The cumulative effect of applying ASC 606 to the Company's contracts with customers that were not completed as of January 1, 2018 was as follows:

	Balance as of December 31, 2017	Adjustments	Balance as of January 1, 2018
Assets	(in thousands)		
Accounts receivable, net	\$281,343	\$ 29,881	\$311,224
Inventories	111,830	(90 )	111,740
Prepaid expenses and other current assets	165,635	(17,166 )	148,469
Total assets	\$3,546,014	\$ 12,625	\$3,558,639
Liabilities and Shareholders' Equity			
Accrued expenses	\$443,961	\$ 8,586	\$452,547
Early access sales accrual	232,401	(7,273 )	225,128
Other liabilities, current portion	34,373	2,083	36,456
Accumulated other comprehensive loss	(11,572 )	949	(10,623 )
Accumulated deficit	(5,119,723 )	8,280	(5,111,443 )
Total liabilities and shareholders' equity	\$3,546,014	\$ 12,625	\$3,558,639

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The impact of adoption on the Company's condensed consolidated balance sheet as of September 30, 2018 was as follows:

	As of September 30, 2018		
	As Reported under ASC 606	Balances without Adoption of ASC 606	Effect of Change Higher/(Lower)
Assets	(in thousands)		
Accounts receivable, net	\$379,755	\$345,180	\$ 34,575
Inventories	124,150	124,245	(95 )
Prepaid expenses and other current assets	118,678	143,826	(25,148 )
Total assets	\$4,620,840	\$4,611,508	\$ 9,332
Liabilities and Shareholders' Equity			
Accrued expenses	\$504,240	\$515,880	\$ (11,640 )
Early access sales accrual	324,251	342,136	(17,885 )
Other liabilities, current portion	60,357	38,950	21,407
Accumulated other comprehensive income	307	186	121
Accumulated deficit	(4,540,005 )	(4,557,334 )	17,329
Total liabilities and shareholders' equity	\$4,620,840	\$4,611,508	\$ 9,332

The impact of adoption on the Company's condensed consolidated statement of operations for the three and nine months ended September 30, 2018 was as follows:

	Three Months Ended September 30, 2018		
	As Reported under ASC 606	Balances without Adoption of ASC 606	Effect of Change Higher/(Lower)
	(in thousands)		
Product revenues, net	\$782,511	\$777,117	\$ 5,394
Cost of sales	111,255	109,533	1,722
Income from operations	205,649	201,977	3,672
Net income attributable to Vertex	\$128,746	\$125,074	\$ 3,672

Amounts per share attributable to Vertex common shareholders:

Net income:			
Basic	\$0.51	\$0.49	\$ 0.02
Diluted	\$0.50	\$0.48	\$ 0.02
	Nine Months Ended September 30, 2018		
	As Reported under ASC 606	Balances without Adoption of ASC 606	Effect of Change Higher/(Lower)
	(in thousands)		
Product revenues, net	\$2,170,152	\$2,156,156	\$ 13,996

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Cost of sales	287,250	282,303	4,947
Income from operations	507,428	498,379	9,049
Net income attributable to Vertex	\$546,369	\$537,320	\$ 9,049

Amounts per share attributable to Vertex common shareholders:

Net income:

Basic	\$2.15	\$2.11	\$ 0.04
Diluted	\$2.11	\$2.07	\$ 0.04



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ASC 606 did not have an aggregate impact on the Company's net cash provided by operating activities, but resulted in offsetting changes in certain assets and liabilities presented within net cash provided by operating activities in the Company's condensed consolidated statement of cash flows.

Equity Investments

In 2016, the FASB issued ASU No. 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities ("ASU 2016-01"), which amended guidance related to the recording of financial assets and financial liabilities. Under the amended guidance, equity investments (except those accounted for under the equity method of accounting or those that result in consolidation of an investee) are measured at fair value with changes in fair value recognized in net income (loss). However, an entity has the option to measure equity investments without readily determinable fair values at (i) fair value or (ii) cost adjusted for changes in observable prices minus impairment. Changes in measurement under either alternative are recognized in net income (loss). The amended guidance became effective January 1, 2018 and required the modified-retrospective adoption approach. As of January 1, 2018, the Company held publicly traded equity investments and equity investments accounted for under the cost method. As a result, in the first quarter of 2018, the Company recorded a \$25.1 million cumulative effect adjustment to "Accumulated deficit" related to its publicly traded equity investments equal to the unrealized gain, net of tax, that was recorded in "Accumulated other comprehensive income (loss)" as of December 31, 2017. The adoption of ASU 2016-01 had no effect on the Company's equity investments accounted for under the cost method because the original cost basis of these investments was recorded on the Company's condensed consolidated balance sheet as of December 31, 2017. In the three and nine months ended September 30, 2018, the Company recorded expense of \$61.2 million and income of \$88.2 million, respectively, to "Other (expense) income, net," in its condensed consolidated statement of operations related to the change in fair value of its equity investments.

Stock-Based Compensation

In 2017, the FASB issued ASU 2017-09, Compensation — Stock Compensation (Topic 718) ("ASU 2017-09") related to the scope of stock option modification accounting to reduce diversity in practice and provide clarity regarding existing guidance. The new accounting guidance was effective January 1, 2018. The Company does not expect the adoption of ASU 2017-09 to have a significant effect on its condensed consolidated financial statements in future periods and had no effect in the three and nine months ended September 30, 2018.

Goodwill

In 2017, the FASB issued ASU 2017-04, Intangibles — Goodwill and Other (Topic 350) ("ASU 2017-04") related to measurements of goodwill. The amended guidance modifies the concept of impairment from the condition that exists when the carrying amount of goodwill exceeds its implied fair value to the condition that exists when the carrying amount of a reporting unit exceeds its fair value, which eliminates Step 2 from the goodwill impairment test. An entity would recognize an impairment charge for the amount by which the carrying amount exceeds the reporting unit's fair value; however, the loss recognized should not exceed the total amount of goodwill allocated to the related reporting unit. The new accounting guidance is required for annual or interim goodwill impairment tests conducted after January 1, 2020. The Company early adopted this new guidance and will utilize this approach for annual and interim goodwill impairment tests conducted after January 1, 2018. The Company does not expect the adoption of this guidance to have a significant effect on its condensed consolidated financial statements.

Intra-Entity Transfers

In 2016, the FASB issued ASU No. 2016-16, Intra-Entity Transfers of Assets Other Than Inventory ("ASU 2016-16"), which removes the previous exception in GAAP prohibiting an entity from recognizing current and deferred income tax expenses or benefits related to the transfer of assets, other than inventory, within the consolidated entity. The exception to defer the recognition of any tax impact on the transfer of inventory within the consolidated entity until it is sold to a third party remains unaffected. The amended guidance became effective January 1, 2018. In the first quarter of 2018, upon adoption of ASU 2016-16, the Company recorded a deferred tax asset and corresponding full valuation allowance of \$204.7 million equal to the unamortized cost of intellectual property rights transferred to the

United Kingdom in 2014 multiplied by an appropriate statutory rate. There was no cumulative effect adjustment to “Accumulated deficit” using the modified-retrospective adoption approach.

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#### Cash Flows - Restricted Cash

In 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230) Restricted Cash (“ASU 2016-18”), which requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash. Therefore, amounts described as restricted cash should be included with cash and cash equivalents when reconciling the beginning of period and end of period amounts shown on the statement of cash flows. The amended guidance became effective January 1, 2018 and is effective on a retrospective basis. 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 for the nine months ended September 30, 2018 and 2017 consisted of the following:

	Nine Months Ended September 30, 2018		Nine Months Ended September 30, 2017	
	Beginning of period	End of period	Beginning of period	End of period
	(in thousands)			
Cash and cash equivalents	\$1,665,412	\$2,478,302	\$1,183,945	\$1,384,966
Restricted cash and cash equivalents (VIE)	1,489	8,143	47,762	1,803
Prepaid expenses and other current assets	625	606	—	—
Cash, cash equivalents and restricted cash per statement of cash flows	\$1,667,526	\$2,487,051	\$1,231,707	\$1,386,769

The Company’s restricted cash is classified in “Prepaid expenses and other current assets” in its condensed consolidated balance sheets. The Company has recorded its VIE’s cash and cash equivalents as “Restricted cash and cash equivalents (VIE)” because (i) the Company does not have any interest in or control over BioAxone’s cash and cash equivalents and (ii) the Company’s agreement with BioAxone does not provide for BioAxone’s cash and cash equivalents to be used for the development of the asset that the Company licensed from BioAxone.

#### Recently Issued Accounting Standards

##### Internal-Use Software

In 2018, the FASB issued ASU 2018-15, Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer’s Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract (“ASU 2018-15”), which clarifies the accounting for implementation costs in cloud computing arrangements. The new guidance is effective for the Company on January 1, 2020. Early adoption is permitted. The Company currently is evaluating the impact the adoption of ASU 2018-15 will have on its condensed consolidated financial statements.

##### Fair Value Measurement

In 2018, the FASB issued ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement (“ASU 2018-13”), which modifies the disclosure requirements for fair value measurements. The new guidance is effective for the Company on January 1, 2020. Early adoption is permitted. The Company currently is evaluating the impact the adoption of ASU 2018-13 may have on its disclosures.

##### Derivatives and Hedging

In 2017, the FASB issued ASU 2017-12, Derivatives and Hedging (Topic 815) (“ASU 2017-12”), which helps simplify certain aspects of hedge accounting and enables entities to more accurately present their risk management activities in their financial statements. The new guidance is effective for the Company on January 1, 2019. The Company does not expect the adoption of ASU 2017-12 to have a significant effect on its condensed consolidated financial statements.

##### Leases

In 2016, the FASB issued ASU No. 2016-02, Leases (“ASU 2016-02”), which amends a number of aspects of lease accounting and requires entities to recognize on the balance sheet right-of-use assets and liabilities for leases with

lease terms of more than 12 months. ASU 2016-02 requires a modified-retrospective adoption approach. The new guidance is effective

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for the Company on January 1, 2019. The Company's project team has reviewed its portfolio of existing leases and current accounting policies to identify and assess the potential differences that would result from applying the requirements of the new standard. The Company anticipates that the amended guidance will result in the recognition of additional right of use assets and corresponding liabilities on its condensed consolidated balance sheets. As discussed in Note K, "Long-term Obligations," the Company currently applies build-to-suit accounting and is the deemed owner of its leased corporate headquarters in Boston and research site in San Diego, for which it is recognizing depreciation expense over the buildings' useful lives and imputed interest on the corresponding construction financing lease obligations. Under the amended guidance, the Company expects to account for these buildings as financing leases, resulting in increased depreciation expense over the respective lease terms, which are significantly shorter than the buildings' useful lives. The Company also expects a reduction in its imputed interest expense in the initial years of each financing lease term. The Company is in the process of quantifying the amount of financing and operating leases, corresponding liabilities and the cumulative effect adjustment to "Accumulated deficit" that will be recorded upon adoption of the amended guidance. The Company is also in the process of implementing appropriate changes to its controls to support lease accounting and related disclosures under the new standard. For a discussion of other recent accounting pronouncements please refer to Note A, "Nature of Business and Accounting Policies—Recent Accounting Pronouncements," in the 2017 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 the 2017 Annual Report on Form 10-K. The Company is disclosing changes in its accounting policies related to guidance that became effective January 1, 2018 in this Quarterly Report on Form 10-Q. Specifically, the Company has included below its updated revenue recognition policy pursuant to its adoption of ASC 606 in Note B, "Revenue Recognition," and its policy related to marketable and equity securities.

**Marketable and Equity Securities**

Effective January 1, 2018, the Company measures publicly traded corporate equity investments that have readily available prices at fair value, with changes in fair value recognized in "Other (expense) income, net," each reporting period.

Effective January 1, 2018, the Company records privately issued corporate equity investments that do not have readily determinable fair values at cost, and adjusts for changes in observable prices minus impairment. Each reporting period, the Company adjusts the carrying value of these investments if it observes that additional shares have been issued in an orderly transaction between market participants resulting in a price increase or decrease per share. Additionally, each reporting period the Company reviews these investments for impairment considering all available information to conclude whether an impairment exists. Changes in measurement for all corporate equity investments are recognized in "Other (expense) income, net."

**B. Revenue Recognition**

Pursuant to ASC 606, the Company recognizes revenue when a customer obtains control of promised goods or services. The Company records the amount of revenue that reflects the consideration that it expects to receive in exchange for those goods or services. The Company applies the following five-step model in order to determine this amount: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The Company only applies the five-step model to contracts when it is probable that it will collect the consideration to which it is entitled in exchange for the goods or services that it transfers to the customer. Once a contract is determined to be within the scope of ASC 606 at contract inception, the Company reviews the contract to determine which performance obligations it must deliver and which of these performance obligations are distinct. The Company

recognizes as revenue the amount of the transaction price that is allocated to each performance obligation when that performance obligation is satisfied or as it is satisfied. Generally, the Company's performance obligations are transferred to customers at a point in time, typically upon delivery.

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Product Revenues, Net

The Company sells its products principally to a limited number of specialty pharmacy and specialty distributors in the United States, which account for the largest portion of its total revenues, and makes international sales primarily to specialty distributors and retail chains, as well as hospitals and clinics, many of which are government-owned or supported (collectively, its “Customers”). The Company’s Customers in the United States subsequently resell the products to patients and health care providers. In accordance with ASC 606, the Company recognizes net revenues from product sales when the Customers obtain control of the Company’s products, which typically occurs upon delivery to the Customer. The Company’s payment terms are approximately 30 days in the United States and consistent with prevailing practice in international markets.

Revenues from product sales are recorded at the net sales price, or “transaction price,” which includes estimates of variable consideration that result from (a) trade allowances, which include invoice discounts for prompt payment and Customer fees, (b) government and private payor rebates, chargebacks, discounts and fees and (c) costs of co-pay assistance programs for patients, as well as other incentives for certain indirect customers. Reserves are established for the estimates of variable consideration based on the amounts earned or to be claimed on the related sales. The reserves are classified as reductions to “Accounts receivable, net” if payable to a Customer or “Accrued expenses” if payable to a third-party. Where appropriate, the Company utilizes the expected value method to determine the appropriate amount for estimates of variable consideration based on factors such as the Company’s historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. The amount of variable consideration that is included in the transaction price may be constrained and is included in net product revenues only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from the Company’s estimates. If actual results vary from the Company’s estimates, the Company adjusts these estimates, which would affect net product revenue and earnings in the period such variances become known.

Trade Allowances: The Company generally provides invoice discounts on product sales to its Customers for prompt payment and pays fees for distribution services, such as fees for certain data that Customers provide to the Company. The Company estimates that, based on its experience, its Customers will earn these discounts and fees, and deducts the full amount of these discounts and fees from its gross product revenues and accounts receivable at the time such revenues are recognized.

Rebates, Chargebacks, Discounts and Fees: The Company contracts with government agencies (its “Third-party Payors”) so that products will be eligible for purchase by, or partial or full reimbursement from, such Third-party Payors. The Company estimates the rebates, chargebacks, discounts and fees it will provide to Third-party Payors and deducts these estimated amounts from its gross product revenues at the time the revenues are recognized. For each product, the Company estimates the aggregate rebates, chargebacks and discounts that it will provide to Third-party Payors based upon (i) the Company’s contracts with these Third-party Payors, (ii) the government-mandated discounts and fees applicable to government-funded programs, (iii) information obtained from the Company’s Customers and other third-party data regarding the payor mix for such product and (iv) historical experience.

Other Incentives: Other incentives that the Company offers include co-pay mitigation rebates provided by the Company to commercially insured patients who have coverage and who reside in states that permit co-pay mitigation programs. Based upon the terms of the Company’s co-pay mitigation programs, the Company estimates average co-pay mitigation amounts for each of its products in order to establish appropriate accruals.

The Company makes significant estimates and judgments that materially affect its recognition of net product revenues. The Company adjusts its estimated rebates, chargebacks and discounts based on new information, including information regarding actual rebates, chargebacks and discounts for its products, as it becomes available. Claims by third-party payors for rebates, chargebacks and discounts frequently are submitted to the Company significantly after the related sales, potentially resulting in adjustments in the period in which the new information becomes known. The

Company's credits to revenue related to prior period sales have not been significant and primarily related to U.S. rebates, chargebacks and discounts.



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The Company excludes taxes collected from Customers relating to product sales and remitted to governmental authorities from revenues.

French Early Access Programs

Pursuant to ASC 605, Revenue Recognition, which was applicable until December 31, 2017, the Company only recognized revenues from product sales if it determined that the price was fixed or determinable at the time of delivery. If the Company determined that the price was not fixed or determinable, it deferred the recognition of revenues. If the Company was able to determine that the price was fixed or determinable, it recognized the net product revenues associated with the units.

The Company began distributing ORKAMBI through early access programs in France during the fourth quarter of 2015 and is engaged in ongoing pricing discussions regarding the final price for ORKAMBI in France. The Company's ORKAMBI net product revenues for 2017, 2016 and 2015 did not include any net product revenues from sales of ORKAMBI in France because the price was not fixed or determinable. The Company expects that the difference between the amounts collected based on the invoiced price and the final price for ORKAMBI in France will be returned to the French government.

As of September 30, 2018 and December 31, 2017, the Company's condensed consolidated balance sheets included \$324.3 million and \$232.4 million, respectively, classified as "Early access sales accrual" related to amounts collected in France as payment for shipments of ORKAMBI under the early access programs, which is considered to be a refund liability pursuant to ASC 606.

Upon adopting ASC 606 in the first quarter of 2018, the Company recorded an \$8.3 million cumulative effect adjustment to "Accumulated deficit" primarily related to shipments of ORKAMBI under early access programs in France. The Company determined the amount of the adjustment based upon (i) the status of pricing discussions in France upon adoption, (ii) the Company's estimate of the amount of consideration it expects to retain related to ORKAMBI sales in France that occurred on or prior to December 31, 2017 that will not be subject to a significant reversal in amounts recognized and (iii) recognition of costs previously deferred related to the ORKAMBI sales in France. For ORKAMBI sales in France that occurred after December 31, 2017 under the early access programs, the Company has recognized net product revenues based on the estimate of consideration it expects to retain that will not be subject to a significant reversal in amounts recognized.

If the Company's estimate regarding the amounts it will receive for ORKAMBI supplied pursuant to these early access programs changes, the Company will reflect the effect of the change in estimate in net product revenues in the period in which the change in estimate occurs and will include adjustments to all prior sales of ORKAMBI under the early access programs.

Collaborative Revenues

The Company recognizes collaborative revenues generated through collaborative research, development and/or commercialization agreements. The terms of these agreements typically include payment to the Company related to one or more of the following: nonrefundable, upfront license fees; development and commercial milestones; funding of research and/or development activities; and royalties on net sales of licensed products. Each type of payments results in collaborative revenues except for revenues from royalties on net sales of licensed products, which are classified as royalty revenues. Revenue is recognized upon satisfaction of a performance obligation by transferring control of a good or service to the collaborator.

For each collaborative research, development and/or commercialization agreement that results in revenue, the Company identifies all material performance obligations, which may include a license to intellectual property and know-how, research and development activities and/or transition activities. In order to determine the transaction price, in addition to any upfront payment, the Company estimates the amount of variable consideration at the outset of the contract either utilizing the expected value or most likely amount method, depending on the facts and circumstances relative to the contract. The Company constrains (reduces) the estimate of variable consideration such that it is probable that a significant reversal of previously recognized revenue will not occur throughout the life of the contract.

When determining if variable consideration should be constrained, management considers whether there are factors outside the Company's control that could result in a

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significant reversal of revenue. In making these assessments, the Company considers the likelihood and magnitude of a potential reversal of revenue. These estimates are re-assessed each reporting period as required.

Once the estimated transaction price is established, amounts are allocated to the performance obligations that have been identified. The transaction price is generally allocated to each separate performance obligation on a relative standalone selling price basis. In order to account for these agreements, the Company must develop assumptions that require judgment to determine the standalone selling price, which may include (i) the probability of obtaining marketing approval for the drug candidate, (ii) estimates regarding the timing of and the expected costs to develop and commercialize the drug candidate, (iii) estimates of future cash flows from potential product sales with respect to the drug candidate and (iv) appropriate discount and tax rates. Standalone selling prices used to perform the initial allocation are not updated after contract inception. The Company does not include a financing component to its estimated transaction price at contract inception unless it estimates that certain performance obligations will not be satisfied within one year.

**Upfront License Fees:** If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in an arrangement, the Company recognizes revenue from the related nonrefundable, upfront license fees based on the relative standalone selling price prescribed to the license compared to the total selling price of the arrangement. The revenue is recognized when the license is transferred to the collaborator and the collaborator is able to use and benefit from the license. For licenses that are not distinct from other obligations identified in the arrangement, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time. If the combined performance obligation is satisfied over time, the Company applies an appropriate method of measuring progress for purposes of recognizing revenue from nonrefundable, upfront license fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

**Development and Regulatory Milestone Payments:** Depending on facts and circumstances, the Company may conclude that it is appropriate to include certain milestones in the estimated transaction price or that it is appropriate to fully constrain the milestones. A milestone payment is included in the transaction price in the reporting period that the Company concludes that it is probable that recording revenue in the period will not result in a significant reversal in amounts recognized in future periods. The Company may record revenues from certain milestones in a reporting period before the milestone is achieved if the Company concludes that achievement of the milestone is probable and that recognition of revenue related to the milestone will not result in a significant reversal in amounts recognized in future periods. The Company records a corresponding contract asset when this conclusion is reached. Milestone payments that have not been included in the transaction price to date are fully constrained. These milestones remain fully constrained until the Company concludes that their achievement is probable and that recognition of the related revenue will not result in a significant reversal in amounts recognized in future periods. The Company re-evaluates the probability of achievement of such development milestones and any related constraint each reporting period and adjusts its estimate of the overall transaction price, including the amount of collaborative revenue that it has recorded, if necessary.

**Research and Development Activities/Transition Services:** If the Company is entitled to reimbursement from its collaborators for specified research and development expenses, the Company accounts for the related services that it provides as separate performance obligations if it determines that these services represent a material right. The Company also determines whether the reimbursement of research and development expenses should be accounted for as collaborative revenues or an offset to research and development expenses in accordance with provisions of gross or net revenue presentation. The Company recognizes the corresponding revenues or records the corresponding offset to research and development expenses as it satisfies the related performance obligations.

**Sales-based Milestone and Royalty Payments:** The Company's collaborators may be required to pay the Company sales-based milestones or royalties on future sales of commercial products. The Company recognizes revenues related

to sales-based milestone and royalties upon the later to occur of (i) achievement of the collaborator's underlying sales or (ii) satisfaction of any performance obligation(s) related to these sales, in each case assuming the license to the Company's intellectual property is deemed to be the predominant item to which the sales-based milestones and/or royalties relate.

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## Contract Liabilities

The following table summarizes changes in the Company's contract liabilities for the nine months ended September 30, 2018:

	Balance			
	at			
	January			Balance at
	1,	Additions	Deductions	September 30,
	2018			2018
	(ASC			
	606			
	adoption)			
	(in thousands)			

## Three Months Ended September 30, 2018

## Contract liabilities:

Other liabilities, current portion \$1,654 \$54,724 \$(16,608 ) \$ 39,770

The Company's contract liabilities relate to 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 pursuant to ASC 606. These contracts, which are classified as "Other liabilities, current portion," include upfront payments and fees. The Company defers a portion of the consideration received for shipments made up to the annual reimbursement limit, and 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.

During the nine months ended September 30, 2018, the Company did not recognize any revenues related to its contract liability balance as of January 1, 2018 or revenues related to performance obligations satisfied in previous periods.

## Disaggregation of Revenue

## Revenues by Product

Product revenues, net consisted of the following:

	Three Months Ended		Nine Months Ended	
	September 30,	September 30,	September 30,	September 30,
	2018 (as	2017 (as	2018 (as	2017 (as
	reported	reported	reported	reported
	under	under	under ASC	under ASC
	ASC	ASC 605)	606)	605)
	606)			
	(in thousands)			
KALYDECO	\$245,733	\$213,461	\$748,365	\$588,809
ORKAMBI	281,859	336,183	947,186	955,451
SYMDEKO	254,919	—	474,601	—
Other	—	(2 )	—	(8 )
Total product revenues, net	\$782,511	\$549,642	\$2,170,152	\$1,544,252

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## Revenues by Geographic Location

Net product revenues are attributed to countries based on the location of the Customer. Collaborative and royalty revenues are attributed to countries based on the location of the Company's subsidiary associated with the collaborative arrangement related to such revenues. Total revenues from external customers and collaborators by geographic region consisted of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018 (as reported under ASC 606)	2017 (as reported under ASC 605)	2018 (as reported under ASC 606)	2017 (as reported under ASC 605)
	(in thousands)			
United States	\$620,485	\$454,736	\$1,687,963	\$1,483,666
Outside of the United States				
Europe	132,876	94,057	400,685	278,407
Other	31,174	29,372	88,843	74,945
Total revenues outside of the United States	164,050	123,429	489,528	353,352
Total revenues	\$784,535	\$578,165	\$2,177,491	\$1,837,018

In the three and nine months ended September 30, 2018 and 2017, revenues attributable to Germany and the United Kingdom contributed the largest amounts to the Company's European revenues.

## C. Collaborative Arrangements and Acquisitions

## Cystic Fibrosis Foundation

The Company has a research, development and commercialization agreement that was originally entered into in 2004 with Cystic Fibrosis Foundation ("CFF"), as successor in interest to the Cystic Fibrosis Foundation Therapeutics, Inc. This agreement was most recently amended in 2016 (the "2016 Amendment"). Pursuant to the agreement, as amended, the Company has 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 VX-659 and VX-445, and tiered royalties ranging from single digits to sub-teens on any approved drugs 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 and SYMDEKO, sales are allocated equally to each of the active pharmaceutical ingredients in the combination product. The Company previously made certain commercial milestone payments under the agreement but there are no remaining commercial milestone payments payable by the Company to CFF pursuant to the agreement.

Pursuant to the 2016 Amendment, the Company received an upfront payment of \$75.0 million and is receiving development funding from CFF of up to \$6.0 million annually. The upfront payment plus any future development funding represent a form of financing pursuant to ASC 730, Research and Development, and thus the amounts are recorded as a liability on the condensed consolidated balance sheet, primarily reflected in "Advance from collaborator, excluding current portion". The liability is reduced over the estimated royalty term of the agreement. Reductions in the liability are reflected as an offset to "Cost of sales" and as "Interest expense, net".

The Company began marketing KALYDECO in 2012 and began marketing ORKAMBI in 2015. The Company received approval for SYMDEKO in the United States in February 2018 and the Company expects to obtain approval for SYMKEVI in the European Union in the fourth quarter of 2018. The Company has royalty obligations to CFF for ivacaftor, lumacaftor and tezacaftor until the expiration of patents covering those compounds. The Company has

patents in the United States and European Union covering the composition-of-matter of ivacaftor that expire in 2027 and 2025, respectively, subject to potential patent extension. The Company has patents in the United States and European Union covering the composition-of-matter of lumacaftor that expire in 2030 and 2026, respectively, subject to potential extension. The Company has patents in the United States and European Union covering the composition-of-matter of tezacaftor that expire in 2027 and 2028, respectively, subject to potential extension.

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CRISPR Therapeutics AG

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 has the exclusive right to license up to six CRISPR-Cas9-based targets, including targets for the potential treatment of sickle cell disease. In connection with the CRISPR Agreement, the Company made an upfront payment to CRISPR of \$75.0 million and a \$30.0 million investment in CRISPR pursuant to a convertible loan agreement that subsequently converted into common shares of CRISPR and was recorded on the Company’s condensed consolidated balance sheet. The Company has made several additional investments in CRISPR’s common shares, including investments totaling \$46.9 million in 2018. As of September 30, 2018, the Company recorded the fair value of its investment in CRISPR common shares of \$207.0 million in “Marketable securities” on its condensed consolidated balance sheet.

The Company funds all of the discovery activities conducted pursuant to the CRISPR Agreement. For targets that the Company elects to license, other than hemoglobinopathy treatments, the Company would lead all development and global commercialization activities. For each target that the Company elects to license, other than hemoglobinopathy targets, CRISPR has the potential to receive up to \$420.0 million in development, regulatory and commercial milestones and royalties on net product sales. As part of the collaboration, the Company and CRISPR share equally all development costs and potential worldwide revenues related to potential hemoglobinopathy treatments, including treatments for beta-thalassemia and sickle cell disease.

The Company may terminate the CRISPR Agreement upon 90 days’ notice to CRISPR prior to any product receiving marketing approval or upon 270 days’ notice after a product has received marketing approval. The CRISPR Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the CRISPR Agreement will continue in effect until the expiration of the Company’s payment obligations under the CRISPR Agreement.

In the fourth quarter of 2017, the Company entered into a co-development and co-commercialization agreement with CRISPR pursuant to the terms of the CRISPR Agreement, under which the Company and CRISPR will co-develop and co-commercialize CTX001 (the “CTX001 Co-Co Agreement”) for the treatment of hemoglobinopathy, including treatments for sickle cell disease and beta-thalassemia. The Company concluded that the CTX001 Co-Co Agreement is a cost-sharing arrangement, which results in the net impact of the arrangement being recorded within “Research and development expenses” in its condensed consolidated statements of operations. During the three and nine months ended September 30, 2018, the net expense related to the CTX001 Co-Co Agreement was \$5.2 million and \$14.1 million, respectively.

Merck KGaA

In January 2017, the Company entered into a strategic collaboration and license agreement (the “Merck KGaA Agreement”) with Merck KGaA, Darmstadt, Germany (“Merck KGaA”). Pursuant to the Merck KGaA Agreement, the Company granted Merck KGaA an exclusive worldwide license to research, develop and commercialize four oncology research and development programs. Under the Merck KGaA Agreement, the Company granted Merck KGaA exclusive, worldwide rights to two clinical-stage programs targeting DNA damage repair: its ataxia telangiectasia and Rad3-related protein inhibitor program, including VX-970 and VX-803, and its DNA-dependent protein kinase inhibitor program, including VX-984. In addition, the Company granted Merck KGaA exclusive, worldwide rights to two pre-clinical programs.

The Merck KGaA Agreement provided for an upfront payment from Merck KGaA to the Company of \$230.0 million. A portion of the upfront payment that was remitted to the German tax authorities in 2017 was refunded to the Company in February 2018. In addition to the upfront payment, the Company will receive tiered royalties on potential sales of licensed products, calculated as a percentage of net sales, that range from (i) mid-single digits to mid-twenties for clinical-stage programs and (ii) mid-single digits to high single digits for the pre-clinical research programs. Merck



KGaA has assumed full responsibility for development and commercialization costs for all programs. The Company evaluated the deliverables, primarily consisting of a license to the four programs and the obligation to complete certain fully-reimbursable research and development and transition activities as directed by Merck KGaA, pursuant to the Merck KGaA Agreement, under the multiple element arrangement accounting guidance that was applicable in 2017.

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The Company concluded that the license had stand-alone value from the research and development and transition activities based on the resources and know-how possessed by Merck KGaA, and thus concluded that there are two units of accounting in the arrangement. The Company determined the relative selling price of the units of accounting based on the Company's best estimate of selling price. The Company utilized key assumptions to determine the best estimate of selling price for the license, which included future potential net sales of licensed products, development timelines, reimbursement rates for personnel costs, discount rates, and estimated third-party development costs. The Company utilized a discounted cash flow model to determine its best estimate of selling price for the license and determined the best estimate of selling price for the research and development and transition activities based on what it would sell the services for separately. Given the significance of the best estimate of selling price for the license as compared to the best estimate of selling price for the research and development and transition services, reasonable changes in the assumptions used in the discounted cash flow model would not have a significant impact on the relative selling price allocation. Based on this analysis, the Company recognized the \$230.0 million upfront payment upon delivery of the license as well as research and development and transition activities during the first quarter of 2017. The Company records the reimbursement for the research and development and transition activities in its condensed consolidated statements of operations as collaborative revenue primarily due to the fact that it is the primary obligor in the arrangement. As of December 31, 2017, the Company's activities related to research and development and transition activities under the Merck KGaA Agreement were substantially complete.

Merck KGaA may terminate the Merck KGaA Agreement or any individual program by providing 90 days' notice, or, in the case of termination of a program with a product that has received marketing approval, 180 days' notice. The Merck KGaA Agreement also may be terminated by either party for a material breach by the other party, subject to notice and cure provisions. Unless earlier terminated, the Merck KGaA Agreement will continue in effect until the date on which the royalty term and all payment obligations with respect to all products in all countries have expired.

Variable Interest Entities (VIEs)

The Company has entered into several agreements pursuant to which it has licensed rights to certain drug candidates from third-party collaborators, resulting in the consolidation of the third parties' financial statements into the Company's condensed consolidated financial statements as VIEs. In order to account for the fair value of the contingent payments, which could consist of milestone, royalty and option payments, related to these collaborations under GAAP, the Company uses present-value models based on assumptions regarding the probability of achieving the relevant milestones, estimates regarding the timing of achieving the milestones, estimates of future product sales and the appropriate discount rates. The Company bases its estimates of the probability of achieving the relevant milestones on industry data for similar assets and its own experience. The discount rates used in the valuation model 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. Changes in these assumptions could have a material effect on the fair value of the contingent payments. The following collaborations have been reflected in the Company's financial statements as consolidated VIEs for portions or all of the periods presented:

Parion Sciences, Inc.

In 2015, the Company entered into a strategic collaboration and license agreement (the "Parion Agreement") with Parion to collaborate with Parion to develop investigational epithelial sodium channel ("ENaC") inhibitors, including VX-371 (formerly P-1037) and VX-551 (formerly P-1055), for the potential treatment of CF and all other pulmonary diseases. The Company is responsible for all costs, subject to certain exceptions, related to development and commercialization of the compounds.

Pursuant to the Parion Agreement, the Company has worldwide development and commercial rights to Parion's lead investigational ENaC inhibitors, VX-371 and VX-551, for the potential treatment of CF and all other pulmonary diseases and has the option to select additional compounds discovered in Parion's research program. To date Parion received \$85.0 million in upfront and milestone payments under the Parion Agreement. Parion has the potential to receive up to an additional (i) \$485.0 million in development and regulatory milestone payments for development of

ENaC inhibitors in CF, including \$360.0 million related to global filing and approval milestones, (ii) \$370.0 million in development and regulatory milestones for VX-371 and VX-551 in non-CF pulmonary indications and (iii) \$230.0 million in development and regulatory milestones should the Company elect to develop an additional ENaC inhibitor from Parion's research program. The

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Company agreed to pay Parion tiered royalties that range from the low double digits to mid-teens as a percentage of potential sales of licensed products.

The Company may terminate the Parion Agreement upon 90 days' notice to Parion prior to any licensed product receiving marketing approval or upon 180 days' notice after a licensed product has received marketing approval. If the Company experiences a change of control prior to the initiation of the first Phase 3 clinical trial for a licensed product, Parion may terminate the Parion Agreement upon 30 days' notice, subject to the Company's right to receive specified royalties on any subsequent commercialization of licensed products. The Parion Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the Parion Agreement will continue in effect until the expiration of the Company's royalty obligations, which expire on a country-by-country basis on the later of (i) the date the last-to-expire patent covering a licensed product expires or (ii) ten years after the first commercial sale in the country.

Following execution of the Parion Agreement, the Company determined that it had a variable interest in Parion via the Parion Agreement, and that the variable interest represented a variable interest in Parion as a whole because the fair value of the ENaC inhibitors represented more than half of the total fair value of Parion's assets. The Company also concluded that it was the primary beneficiary as it had the power to direct the activities that most significantly affect the economic performance of Parion and that it had the obligation to absorb losses and right to receive benefits that potentially could be significant to Parion. Accordingly, the Company consolidated Parion's financial statements beginning in June 2015. Notwithstanding the applicable accounting treatment, the Company's interests in Parion have been and continue to be limited to those accorded to the Company in the Parion Agreement.

As of September 30, 2017, the Company determined that the fair value of Parion's pulmonary ENaC platform had declined significantly based on data received in September 2017 from a Phase 2 clinical trial of VX-371 that did not meet its primary efficacy endpoint. After evaluating the results of the clinical trial and based on the decrease in the fair value of Parion's pulmonary ENaC platform relative to Parion's other activities, the Company determined that it was no longer the primary beneficiary of Parion as it no longer had the power to direct the significant activities of Parion. Accordingly, the Company deconsolidated Parion as of September 30, 2017. Please refer to Note B, "Collaborative Arrangements and Acquisitions," in the 2017 Annual Report on Form 10-K for further information regarding the deconsolidation of Parion.

BioAxone Biosciences, Inc.

In 2014, the Company entered into a license and collaboration agreement (the "BioAxone Agreement") with BioAxone Biosciences, Inc. ("BioAxone"), which resulted in the consolidation of BioAxone as a VIE beginning on October 1, 2014. The Company recorded an in-process research and development intangible asset of \$29.0 million for VX-210 and a corresponding deferred tax liability of \$11.3 million attributable to BioAxone. The Company made an initial payment to BioAxone of \$10.0 million in 2014. In the first quarter of 2018, the Company's option to purchase BioAxone expired and the Company paid a \$10.0 million license continuation fee to BioAxone. As of September 30, 2018, BioAxone had the potential to receive up to \$80.0 million in milestones, including development and regulatory milestone payments. As of September 30, 2018, the Company continues to conclude that it is the primary beneficiary of BioAxone and continues to consolidate BioAxone as a VIE.

As further discussed in Note P, "Subsequent Event," in the fourth quarter of 2018, the Company will determine whether it continues to be the primary beneficiary of BioAxone and should continue to consolidate BioAxone as a VIE based on its October 2018 announcement that it will terminate its Phase 2b clinical trial of VX-210.

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## Aggregate VIE Financial Information

An aggregate summary of net income attributable to noncontrolling interest related to the Company's VIEs for the three and nine months ended September 30, 2018 and 2017 is as follows:

	Three Months Ended September 30, 2018		Nine Months Ended September 30, 2017	
	2018	2017	2018	2017
	(in thousands)			
Loss attributable to noncontrolling interest before provision for (benefit from) income taxes and changes in fair value of contingent payments	\$411	\$238,946	\$1,394	\$222,448
Provision for (benefit from) income taxes	79	(120,181)	6,068	(111,658)
(Increase) decrease in fair value of contingent payments	(200)	69,550	(23,100)	62,560
Net loss (income) attributable to noncontrolling interest	\$290	\$188,315	\$(15,638)	\$173,350

The increase in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments for the nine months ended September 30, 2018 was primarily due to the expiration of the Company's option to purchase BioAxone in the first quarter of 2018 that increased the probability of a \$10.0 million license continuation fee for VX-210 (which was ultimately paid in the first quarter of 2018) and the probability that additional milestone and royalty payments related to the BioAxone Agreement would be paid. The increase in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments for the three months ended September 30, 2018 was primarily due to changes in the time value of money. The decreases in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments for the three and nine months ended September 30, 2017 were primarily due to the decrease in the fair value of Parion's pulmonary ENaC platform described above. The fair value of the contingent payments payable by the Company to BioAxone was \$32.0 million and \$18.9 million as of September 30, 2018 and December 31, 2017, respectively. During the three and nine months ended September 30, 2018 and 2017, the (increases) and decreases in the fair value of the contingent payments related to the Company's VIEs were as follows:

	Three Months Ended September 30, 2018	Nine Months Ended September 30, 2017
	(in thousands)	
Parion	\$—	\$69,550
BioAxone	(200)	(23,100)

Significant amounts related to the Company's consolidation of BioAxone as a VIE included in the Company's condensed consolidated balance sheets as of the dates set forth in the table below were as follows:

	September 30, 2018	December 31, 2017
	(in thousands)	
Restricted cash and cash equivalents (VIE)	\$8,143	\$1,489
Intangible assets	29,000	29,000
Deferred tax liability	9,414	4,756
Noncontrolling interest	28,365	13,727

The Company has recorded BioAxone's cash and cash equivalents as "Restricted cash and cash equivalents (VIE)" because (i) the Company does not have any interest in or control over BioAxone's cash and cash equivalents and (ii) the Company's agreement with BioAxone does not provide for BioAxone's cash and cash equivalents to be used for the development of the asset that the Company licensed from BioAxone. Assets recorded as a result of consolidating BioAxone's financial condition into the Company's balance sheets do not represent additional assets that could be used to satisfy claims against the Company's general assets.

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Other Collaborations

The Company has entered into various agreements pursuant to which it collaborates with third parties, including inlicensing and outlicensing arrangements. Although the Company does not consider any of these arrangements to be material, the most notable of these arrangements are described below.

Moderna Therapeutics, Inc.

In 2016, the Company entered into a strategic collaboration and licensing agreement (the “Moderna Agreement”) with Moderna Therapeutics, Inc. (“Moderna”), pursuant to which the parties are seeking to identify and develop messenger Ribonucleic Acid (“mRNA”) therapeutics for the treatment of CF. In connection with the Moderna Agreement, the Company made an upfront payment to Moderna of \$20.0 million and a \$20.0 million investment in Moderna pursuant to a convertible promissory note that converted into preferred stock in 2016. Moderna has the potential to receive future development and regulatory milestones of up to \$275.0 million, including \$220.0 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales.

Under the terms of the Moderna Agreement, Moderna leads discovery efforts and the Company leads all preclinical, development and commercialization activities associated with the advancement of mRNA Therapeutics that result from this collaboration and will fund all expenses related to the collaboration.

The Company may terminate the Moderna Agreement by providing advance notice to Moderna, with the required length of notice dependent on whether any product developed under the Moderna Agreement has received marketing approval. The Moderna Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the Moderna Agreement will continue in effect until the expiration of the Company’s payment obligations under the Moderna Agreement.

Janssen Pharmaceuticals, Inc.

In 2014, the Company entered into an agreement (the “Janssen Agreement”) with Janssen Pharmaceuticals, Inc. (“Janssen”). Pursuant to the agreement, Janssen has an exclusive worldwide license to develop and commercialize certain drug candidates for the treatment of influenza, including pimodivir (formerly VX-787). The Company received non-refundable payments of \$35.0 million from Janssen in 2014 and recognized a \$25.0 million milestone in the fourth quarter of 2017. The milestone, which was achieved based on the Phase 3 clinical trial Janssen initiated in the fourth quarter of 2017, was collected in the first quarter of 2018. The Company has the potential to receive additional regulatory and commercial milestone payments as well as royalties on future product sales, if any. Janssen is responsible for costs related to the development and commercialization of the compounds. Janssen may terminate the Janssen Agreement, subject to certain exceptions, upon six months’ notice.

Asset Acquisition

Concert Pharmaceuticals

In July 2017, the Company acquired certain CF assets including VX-561 (formerly CTP-656) (the “Concert Assets”) from Concert Pharmaceuticals Inc. (“Concert”) pursuant to an asset purchase agreement that was entered into in March 2017 (the “Concert Agreement”). VX-561 is an investigational CFTR potentiator that has the potential to be used as part of combination regimens of CFTR modulators to treat CF. Pursuant to the Concert Agreement, Vertex paid Concert \$160.0 million in cash for the Concert Assets. If VX-561 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90.0 million in milestones based on regulatory approval in the United States and reimbursement in the United Kingdom, Germany or France. The Company determined that substantially all of the fair value of the Concert Agreement was attributable to a single in-process research and development asset, VX-561, which did not constitute a business. The Company concluded that it did not have any alternative future use for the acquired in-process research and development asset. Thus, the Company recorded the \$160.0 million upfront payment to “Research and Development Expenses” in the third quarter of 2017. The total cost of the transaction was \$165.1 million including \$5.1 million of transaction costs that were recorded to “Sales, general and administrative expenses”. If the Company achieves regulatory





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approval and reimbursement milestones, the Company will record the value of the milestone as an intangible asset and will begin amortizing the asset in cost of sales in the period that the relevant milestone is achieved.

**D. Earnings Per Share**

Basic net income (loss) per share attributable to Vertex common shareholders is based upon the weighted-average number of common shares outstanding during the period, excluding restricted stock, restricted stock units and performance-based restricted stock units, or “PSUs,” that have been issued but are not yet vested. Diluted net income per share attributable to Vertex common shareholders 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 (loss) per share for the periods ended:

	Three Months Ended September 30, 2018		Nine Months Ended September 30, 2018	
	2017	2018	2017	2018
	(in thousands, except per share amounts)			
Basic net income (loss) attributable to Vertex per common share calculation:				
Net income (loss) attributable to Vertex common shareholders	\$128,746	\$(102,952)	\$546,369	\$162,800
Less: Undistributed earnings allocated to participating securities	(14)	—	(161)	(203)
Net income (loss) attributable to Vertex common shareholders—basic	\$128,732	\$(102,952)	\$546,208	\$162,597
Basic weighted-average common shares outstanding	254,905	250,268	254,096	247,963
Basic net income (loss) attributable to Vertex per common share	\$0.51	\$(0.41)	\$2.15	\$0.66
Diluted net income (loss) attributable to Vertex per common share calculation:				
Net income (loss) attributable to Vertex common shareholders	\$128,746	\$(102,952)	\$546,369	\$162,800
Less: Undistributed earnings allocated to participating securities	(13)	—	(158)	(200)
Net income (loss) attributable to Vertex common shareholders—diluted	\$128,733	\$(102,952)	\$546,211	\$162,600
Weighted-average shares used to compute basic net income (loss) per common share	254,905	250,268	254,096	247,963
Effect of potentially dilutive securities:				
Stock options	2,962	—	2,990	2,700
Restricted stock and restricted stock units (including PSUs)	1,896	—	1,866	1,204
Employee stock purchase program	25	—	20	228
Weighted-average shares used to compute diluted net income (loss) per common share	259,788	250,268	258,972	252,095
Diluted net income (loss) attributable to Vertex per common share	\$0.50	\$(0.41)	\$2.11	\$0.64

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The Company did not include the securities in the following table in the computation of the net income (loss) per share attributable to Vertex common shareholders calculations because the effect would have been anti-dilutive during each period:

	Three Months Ended September 30, 2018		Nine Months Ended September 30, 2017	
Stock options	2,572	10,278	2,152	3,904
Unvested restricted stock and restricted stock units (including PSUs)	1	4,241	4	281

E. Fair Value Measurements

The fair value of the Company's financial assets and liabilities reflects the Company's estimate of amounts that it would have received in connection with the sale of the assets or paid in connection with the transfer of the liabilities in an orderly transaction between market participants at the measurement date. In connection with measuring the fair value of its assets and liabilities, the Company seeks to maximize the use of observable inputs (market data obtained from sources independent from the Company) and to minimize the use of unobservable inputs (the Company's assumptions about how market participants would price assets and liabilities). The following fair value hierarchy is used to classify assets and liabilities based on the observable inputs and unobservable inputs used in order to value the assets and liabilities:

Quoted prices in active markets for identical assets or liabilities. An active market for an asset or liability is a Level 1: market in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis.

Observable inputs other than Level 1 inputs. Examples of Level 2 inputs include quoted prices in active Level 2: markets for similar assets or liabilities and quoted prices for identical assets or liabilities in markets that are not active.

Unobservable inputs based on the Company's assessment of the assumptions that market participants would use in pricing the asset or liability. Level 3:

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. As of September 30, 2018, the Company's investments were primarily in money market funds, U.S. Treasury securities, government-sponsored enterprise securities, corporate equity securities, corporate debt securities and commercial paper. 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.

As of September 30, 2018, all of the Company's financial assets and liabilities that were subject to fair value measurements were valued using observable inputs. The Company's financial assets valued based on Level 1 inputs consisted of money market funds, U.S. Treasury securities, government-sponsored enterprise securities and corporate equity securities. The Company's financial assets and liabilities valued based on Level 2 inputs consisted of corporate debt securities and commercial paper, which consisted of investments in highly-rated investment-grade corporations, and foreign currency forward contracts with reputable and creditworthy counterparties. During the three and nine months ended September 30, 2018 and 2017, the Company did not record any other-than-temporary impairment charges related to its financial assets.



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The following table sets forth the Company's financial assets and liabilities (excluding VIE cash and cash equivalents) subject to fair value measurements:

	Fair Value Measurements as of September 30, 2018			
	Total	Fair Value Hierarchy		
		Level 1	Level 2	Level 3
	(in thousands)			
Financial instruments carried at fair value (asset position):				
Cash equivalents:				
Money market funds	\$ 1,070,166	\$ 1,070,166	\$—	\$ —
U.S. Treasury securities	5,979	5,979	—	—
Government-sponsored enterprise securities	2,498	2,498	—	—
Corporate debt securities	7,572	—	7,572	—
Commercial paper	33,352	—	33,352	—
Marketable securities:				
Corporate equity securities	207,036	207,036	—	—
U.S. Treasury securities	6,043	6,043	—	—
Government-sponsored enterprise securities	6,462	6,462	—	—
Corporate debt securities	218,082	—	218,082	—
Commercial paper	139,960	—	139,960	—
Prepaid and other current assets:				
Foreign currency forward contracts	13,903	—	13,903	—
Other assets:				
Foreign currency forward contracts	713	—	713	—
Total financial assets	\$ 1,711,766	\$ 1,298,184	\$ 413,582	\$ —
Financial instruments carried at fair value (liability position):				
Other liabilities, current portion:				
Foreign currency forward contracts	\$(505)	) \$—	\$(505)	) \$ —
Other liabilities, excluding current portion:				
Foreign currency forward contracts	(66)	) —	(66)	) —
Total financial liabilities	\$(571)	) \$—	\$(571)	) \$ —

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	Fair Value Measurements as of December 31, 2017			
	Fair Value Hierarchy			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Financial instruments carried at fair value (asset position):				
Cash equivalents:				
Money market funds	\$614,951	\$614,951	\$—	\$ —
Government-sponsored enterprise securities	12,678	12,678	—	—
Commercial paper	57,357	—	57,357	—
Marketable securities:				
Corporate equity securities	74,821	74,821	—	—
Government-sponsored enterprise securities	2,303	2,303	—	—
Corporate debt securities	265,867	—	265,867	—
Commercial paper	80,263	—	80,263	—
Prepaid and other current assets:				
Foreign currency forward contracts	13	—	13	—
Total financial assets	\$1,108,253	\$704,753	\$403,500	\$ —
Financial instruments carried at fair value (liability position):				
Other liabilities, current portion:				
Foreign currency forward contracts	\$(13,642 )	\$—	\$(13,642 )	\$ —
Other liabilities, excluding current portion:				
Foreign currency forward contracts	(866 )	—	(866 )	—
Total financial liabilities	\$(14,508 )	\$—	\$(14,508 )	\$ —
Please refer to Note F, “Marketable Securities and Equity Investments,” for the carrying amount and related unrealized gains (losses) by type of the Company’s financial assets and liabilities.				
The Company’s VIE invested in cash equivalents consisting of U.S. Treasury securities of \$5.0 million as of September 30, 2018, which are valued based on Level 1 inputs. These cash equivalents are not included in the table above. The Company’s noncontrolling interest related to the Company’s VIE includes the fair value of the contingent payments, which could consist of milestone, royalty and option payments, which are valued based on Level 3 inputs. Please refer to Note C, “Collaborative Arrangements and Acquisitions,” for further information.				
<b>F. Marketable Securities and Equity Investments</b>				
Pursuant to the adoption of ASU 2016-01 on January 1, 2018, the Company began recording changes in the fair value of its investments in corporate equity securities (except those accounted for under the equity method of accounting or those that result in consolidation of an investee) to “Other (expense) income, net” in the Company’s condensed consolidated statements of operations. Prior to its adoption of ASU 2016-01, the Company recorded changes in the fair value of its investments in corporate equity securities to “Accumulated other comprehensive income (loss)” on its condensed consolidated balance sheet until the related gains or losses were realized. The Company continues to record unrealized gains (losses) on available-for-sale debt securities as a component of accumulated other comprehensive income (loss) until such gains and losses are realized.				

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A summary of the Company's cash equivalents and marketable securities is shown below:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
(in thousands)				
As of September 30, 2018				
Cash equivalents:				
Money market funds	\$1,070,166	\$—	\$—	\$1,070,166
U.S. Treasury securities	5,980	—	(1)	5,979
Government-sponsored enterprise securities	2,498	—	—	2,498
Corporate debt securities	7,573	—	(1)	7,572
Commercial paper	33,361	—	(9)	33,352
Total cash equivalents	1,119,578	—	(11)	1,119,567
Marketable securities:				
U.S. Treasury securities (matures within 1 year)	6,044	—	(1)	6,043
Government-sponsored enterprise securities (matures within 1 year)	4,982	—	—	4,982
Government-sponsored enterprise securities (matures after 1 year through 5 years)	1,481	—	(1)	1,480
Corporate debt securities (matures within 1 year)	179,738	5	(319)	179,424
Corporate debt securities (matures after 1 year through 5 years)	38,702	—	(44)	38,658
Commercial paper (matures within 1 year)	140,046	—	(86)	139,960
Total marketable debt securities	370,993	5	(451)	370,547
Corporate equity securities	90,133	116,903	—	207,036
Total marketable securities	\$461,126	\$116,908	\$ (451)	\$577,583

As of December 31, 2017

Cash equivalents:				
Money market funds	\$614,951	\$—	\$—	\$614,951
Government-sponsored enterprise securities	12,679	—	(1)	12,678
Commercial paper	57,371	—	(14)	57,357
Total cash equivalents	685,001	—	(15)	684,986
Marketable securities:				
Government-sponsored enterprise securities (matures within 1 year)	2,304	—	(1)	2,303
Corporate debt securities (matures within 1 year)	215,639	—	(363)	215,276
Corporate debt securities (matures after 1 year through 5 years)	50,697	—	(106)	50,591
Commercial paper (matures within 1 year)	80,372	—	(109)	80,263
Total marketable debt securities	349,012	—	(579)	348,433
Available-for-sale corporate equity securities	43,213	31,608	—	74,821
Total marketable securities	\$392,225	\$31,608	\$ (579)	\$423,254

Available-for-sale debt securities were recorded in the Company's condensed consolidated balance sheets as follows:

	As of September 30, 2018	As of December 31, 2017
(in thousands)		
Cash and cash equivalents	\$1,119,567	\$684,986
Marketable securities	370,547	348,433

Total \$1,490,114 \$1,033,419

The Company has a limited number of available-for-sale debt securities in insignificant loss positions as of September 30, 2018, which it does not intend to sell and has concluded it will not be required to sell before recovery of the

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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 and nine months ended September 30, 2018 and 2017.

The Company maintains strategic investments separately from the investment policy that governs its other cash, cash equivalents and marketable securities. During the three and nine months ended September 30, 2018, the Company recorded an unrealized loss of \$61.2 million and an unrealized gain of \$88.2 million, respectively, related to its investments in corporate equity securities, as follows:

During the three and nine months ended September 30, 2018, an unrealized loss of \$61.2 million and an unrealized gain of \$85.3 million, respectively, related to its equity investment in CRISPR, a publicly traded company. The CRISPR common stock held by the Company has a readily determinable fair value that is recorded in "Marketable securities" on the Company's condensed consolidated balance sheets. In 2018, the Company has purchased an additional \$46.9 million of CRISPR's common shares.

During the three and nine months ended September 30, 2018, unrealized gains of zero and \$2.9 million, respectively, related to its equity investments in privately held companies without readily determinable fair values for their stocks. As of September 30, 2018, 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 \$36.7 million.

G. Accumulated Other Comprehensive  
Income (Loss)

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

	Unrealized Holding Gains (Losses), Net of Tax				
	Foreign Currency Translation Adjustment	On Available- Debt Securities	On Equity Securities	On Foreign Currency Forward Contracts	Total
	(in thousands)				
Balance at December 31, 2017	\$(21,031)	\$(594)	\$25,069	\$(15,016)	\$(11,572)
Other comprehensive income before reclassifications	6,800	137	—	21,102	28,039
Amounts reclassified from accumulated other comprehensive income (loss)	—	—	—	7,960	7,960
Net current period other comprehensive income	\$6,800	\$137	\$—	\$29,062	\$35,999
Amounts reclassified to accumulated deficit pursuant to adoption of new accounting standard	949	—	(25,069)	—	(24,120)
Balance at September 30, 2018	\$(13,282)	\$(457)	\$—	\$14,046	\$307
	Unrealized Holding Gains (Losses), Net of Tax				
	Foreign Currency Translation Adjustment	On Available- Debt Securities	On Equity Securities	On Foreign Currency Forward Contracts	Total
	(in thousands)				
Balance at December 31, 2016	\$(7,862)	\$(10)	\$17,531	\$11,514	\$21,173
Other comprehensive loss before reclassifications	(11,137)	(170)	(7,616)	(25,981)	(44,904)
	—	—	—	(1,398)	(1,398)



Amounts reclassified from accumulated other comprehensive  
income (loss)

Net current period other comprehensive loss	\$ (11,137)	\$ (170)	\$ (7,616 )	\$ (27,379)	\$ (46,302)
Balance at September 30, 2017	\$ (18,999)	\$ (180)	\$ 9,915	\$ (15,865)	\$ (25,129)

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**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 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 September 30, 2018, all hedges were determined to be highly effective, and the Company has not recorded any ineffectiveness related to its hedging program since its inception.

The Company considers the impact of its counterparties' credit risk on the fair value of the foreign currency forward contracts. As of September 30, 2018 and December 31, 2017, credit risk did not change the fair value of the Company's foreign currency forward contracts.

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

	As of September 30, 2018	As of December 31, 2017
Foreign Currency	(in thousands)	
Euro	\$324,753	\$257,230
British pound sterling	74,494	77,481
Canadian dollar	37,098	—
Australian dollar	31,279	30,501
Total foreign currency forward contracts	\$467,624	\$365,212

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

The Company also enters into foreign exchange forward contracts with contractual maturities of less than one month 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 GAAP. The Company recognizes realized gains and losses for such contracts in "Other (expense) income, net" in its condensed consolidated statements of operations each period. As of September 30, 2018, the notional amount of foreign exchange contracts where hedge accounting under GAAP is not applied was \$115.8 million.

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During the three and nine months ended September 30, 2018 and 2017, the Company recognized the following related to foreign currency forward contracts in its condensed consolidated statements of operations:

	Three Months Ended September 30, 2018		Nine Months Ended September 30, 2017	
	2018	2017	2018	2017
	(in thousands)			
Designated as hedging instruments - Reclassified from AOCI				
Product revenues, net	\$1,721	\$(2,461)	\$(7,438)	\$4,291
Not designated as hedging instruments				
Other (expense) income, net	\$782	\$4,135	\$(1,832)	\$12,958

The following table summarizes the fair value of the Company's outstanding foreign currency forward contracts included on its condensed consolidated balance sheets:

As of September 30, 2018

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Designated as hedging instruments			
Prepaid expenses and other current assets	\$13,903	Other liabilities, current portion	\$(505)
Other assets	713	Other liabilities, excluding current portion	(66 )
Total assets	\$14,616	Total liabilities	\$(571)

As of December 31, 2017

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Designated as hedging instruments			
Prepaid expenses and other current assets	\$ 13	Other liabilities, current portion	\$(13,642)
Other assets	—	Other liabilities, excluding current portion	(866 )
Total assets	\$ 13	Total liabilities	\$(14,508)

As of September 30, 2018, the Company expects amounts that are related to foreign exchange forward contracts designated as cash flow hedges under GAAP recorded in "Prepaid expenses and other current assets" and "Other liabilities, current portion" 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 GAAP on the Company's condensed consolidated balance sheets:

As of September 30, 2018

	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
Foreign currency forward contracts (in thousands)					
Total assets	\$14,616	\$	—\$14,616	\$(571 )	\$14,045
Total liabilities	\$(571 )	\$	—\$(571 )	\$571	\$—



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	As of December 31, 2017				
	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
Foreign currency forward contracts (in thousands)					
Total assets	\$13	\$	—\$13	\$ (13 )	\$—
Total liabilities	\$(14,508)	\$	—\$(14,508)	\$ 13	\$(14,495)

## I. Inventories

Inventories consisted of the following:

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Raw materials	\$ 13,726	\$ 20,924
Work-in-process	77,408	74,237
Finished goods	33,016	16,669
Total	\$ 124,150	\$ 111,830

## J. Goodwill

As of September 30, 2018 and December 31, 2017, goodwill of \$50.4 million was recorded on the Company's condensed consolidated balance sheet.

## K. Long-term Obligations

## Construction Financing Lease Obligation

As a result of the Company being involved in the construction of several of its leased buildings in Boston and San Diego, the Company was deemed for accounting purposes to be the owner of these buildings during their construction periods and recorded project construction costs incurred by its landlords. Upon completion of these buildings, the Company determined that the underlying leases did not meet the criteria for "sale-leaseback" treatment. Accordingly, the Company depreciates the lease assets and records interest expense associated with the financing obligations for these buildings. The Company bifurcates the lease payments pursuant to these leases into (i) a portion that is allocated to the buildings and (ii) a portion that is allocated to the land on which the buildings were constructed. The portion of the lease obligations allocated to the land is treated as an operating lease.

## Fan Pier Leases

In 2011, the Company entered into two lease agreements, pursuant to which the Company leases approximately 1.1 million square feet of office and laboratory space in two buildings (the "Fan Pier Buildings") at Fan Pier in Boston, Massachusetts (the "Fan Pier Leases"). The Company commenced lease payments in December 2013, and will make lease payments pursuant to the Fan Pier Leases through December 2028. The Company has an option to extend the term of the Fan Pier Leases for an additional ten years.

## San Diego Lease

In December 2015, the Company entered into a lease agreement for 3215 Merryfield Row, San Diego, California with ARE-SD Region No. 23, LLC (the "San Diego Building"). Pursuant to this agreement, the Company agreed to lease approximately 170,000 square feet of office and laboratory space in San Diego, California ("San Diego Lease") for a term of 16 years. Base rent payments will commence in the second quarter of 2019. Pursuant to the San Diego Lease, during the initial 16-year term, the Company will pay an average of approximately \$10.2 million per year in aggregate rent, excluding operating expenses. The Company has the option to extend the lease term for up to two additional five-year terms.



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In the second quarter of 2018, the Company determined that the San Diego lease did not meet the criteria for “sale-leaseback” treatment when construction of the San Diego Building was completed. The Company based this determination on, among other things, its continuing involvement with the property in the form of non-recourse financing to the lessor. Accordingly, the Company began depreciating the asset and incurring interest expense related to the financing obligation during the second quarter of 2018. The portion of the lease obligations allocated to the land is treated as an operating lease that commenced in the fourth quarter of 2016.

Property and equipment, net and the carrying value of the Company’s construction financing lease obligation (including current and non-current portions and excluding interest that will be imputed over the course of the Company’s underlying lease agreements for these buildings) related to the Fan Pier Buildings and the San Diego Building were as follows:

	September	December
	30, 2018	31, 2017
	(in thousands)	
Property and equipment, net		
Fan Pier Buildings	\$466,185	\$475,725
San Diego Building	\$114,301	\$94,602

Construction financing lease obligation

Fan Pier Buildings	\$471,654	\$472,070
San Diego Building	\$94,088	\$87,392

Revolving Credit Facility

In October 2016, the Company entered into a Credit Agreement (the “Credit Agreement”) with Bank of America, N.A., as administrative agent and the lenders referred to therein. The Credit Agreement provides for a \$500.0 million revolving facility, \$300.0 million of which was drawn at closing (the “Loans”) and was repaid in February 2017. The Credit Agreement also provides that, subject to satisfaction of certain conditions, the Company may request that the borrowing capacity under the Credit Agreement be increased by an additional \$300.0 million. The Credit Agreement matures on October 13, 2021.

The Loans will bear interest, at the Company’s option, at either a base rate or a Eurodollar rate, in each case plus an applicable margin. Under the Credit Agreement, the applicable margins on base rate loans range from 0.75% to 1.50% and the applicable margins on Eurodollar loans range from 1.75% to 2.50%, in each case based on the Company’s consolidated leverage ratio (the ratio of the Company’s total consolidated debt to the Company’s trailing twelve-month EBITDA).

The Loans are guaranteed by certain of the Company’s domestic subsidiaries and secured by substantially all of the Company’s assets and the assets of the Company’s domestic subsidiaries (excluding intellectual property, owned and leased real property and certain other excluded property) and by the equity interests of the Company’s subsidiaries, subject to certain exceptions. Under the terms of the Credit Agreement, the Company must maintain, subject to certain limited exceptions, a consolidated leverage ratio of 3.00 to 1.00 and consolidated EBITDA of at least \$200.0 million, in each case measured on a quarterly basis.

The Credit Agreement contains customary representations and warranties and usual and customary affirmative and negative covenants. The Credit Agreement also contains customary events of default. In the case of a continuing event of default, the administrative agent would be entitled to exercise various remedies, including the acceleration of amounts due under outstanding loans.

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## L. Stock-based Compensation Expense and Share Repurchase

## Stock-based compensation expense

During the three and nine months ended September 30, 2018 and 2017, the Company recognized the following stock-based compensation expense:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
	(in thousands)			
Stock-based compensation expense by type of award:				
Stock options	\$27,234	\$25,969	\$81,880	\$80,865
Restricted stock and restricted stock units (including PSUs)	55,482	46,737	157,397	131,388
ESPP share issuances	2,725	2,428	7,281	6,738
Stock-based compensation expense related to inventories	91	(678)	(454)	(2,001)
Total stock-based compensation included in costs and expenses	\$85,532	\$74,456	\$246,104	\$216,990

## Stock-based compensation expense by line item:

Cost of sales	\$1,259	\$686	\$3,263	\$1,656
Research and development expenses	52,918	46,186	153,018	134,855
Sales, general and administrative expenses	31,355	27,584	89,823	80,479
Total stock-based compensation included in costs and expenses	\$85,532	\$74,456	\$246,104	\$216,990

The following table sets forth the Company's unrecognized stock-based compensation expense as of September 30, 2018, by type of award and the weighted-average period over which that expense is expected to be recognized:

Type of award:	As of September 30, 2018	
	Unrecognized Expense (in thousands)	Weighted-average Recognition Period (in years)
Stock options	\$189,045	2.77
Restricted stock and restricted stock units (including PSUs)	\$388,909	2.71
ESPP share issuances	\$3,051	0.47



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The following table summarizes information about stock options outstanding and exercisable as of September 30, 2018:

Range of Exercise Prices	Options Outstanding			Options Exercisable	
	Number Outstanding	Weighted-average Remaining Contractual Life	Weighted-average Exercise Price	Number Exercisable	Weighted-average Exercise Price
	(in thousands)	(in years)	(per share)	(in thousands)	(per share)
\$29.07–\$40.00	550	1.36	\$ 34.89	550	\$ 34.89
\$40.01–\$60.00	495	3.71	\$ 49.82	495	\$ 49.82
\$60.01–\$80.00	556	5.51	\$ 74.96	545	\$ 74.94
\$80.01–\$100.00	3,017	7.46	\$ 89.18	1,228	\$ 89.88
\$100.01–\$120.00	725	6.36	\$ 109.30	550	\$ 109.19
\$120.01–\$140.00	880	6.89	\$ 130.25	615	\$ 130.15
\$140.01–\$160.00	1,445	9.33	\$ 155.52	198	\$ 155.20
\$160.01–\$180.00	545	8.77	\$ 162.94	128	\$ 162.94
\$180.01–\$181.60	732	9.80	\$ 181.60	4	\$ 181.60
Total	8,945	7.18	\$ 111.22	4,313	\$ 89.83

#### Share repurchase program

The Board of Directors approved a share repurchase program, pursuant to which the Company is authorized to repurchase up to \$500.0 million of its common stock between February 1, 2018 and December 31, 2019. Under the share repurchase program, the Company is authorized to purchase shares from time to time through open market or privately negotiated transactions. Such purchases may be made pursuant to Rule 10b5-1 plans or other means as determined by the Company's management and in accordance with the requirements of the SEC.

During the nine months ended September 30, 2018, the Company repurchased 1,282,683 shares of its common stock under the share repurchase program for an aggregate of \$211.0 million (of which \$4.0 million was accrued as of September 30, 2018), including commissions and fees. The Company expects to fund further repurchases of its common stock through a combination of cash on hand and cash generated by operations.

#### M. Income Taxes

The Company is subject to U.S. federal, state, and foreign income taxes. For the three and nine months ended September 30, 2018, the Company recorded provisions for income taxes of \$8.1 million and \$5.7 million, respectively. The provision for income taxes for the three months ended September 30, 2018 related primarily to \$4.9 million for the Company's U.S. state and foreign taxes and \$3.1 million for the reversing effect of discrete items associated with stock-based compensation. The provision for income taxes for the nine months ended September 30, 2018 included \$13.4 million for the Company's U.S. state and foreign taxes and \$6.1 million for BioAxone's income taxes partially offset by a benefit of \$13.7 million from discrete items related to stock-based compensation. The Company has no liability for taxes payable by BioAxone and the income tax provision and related liability have been allocated to noncontrolling interest. For the three and nine months ended September 30, 2017, the Company recorded benefits from income taxes of \$125.9 million and \$117.6 million, respectively, which included \$120.2 million and \$111.7 million, respectively, related to the Company's VIEs' income tax provision. The VIEs' benefit from income taxes during the three and nine months ended September 30, 2017 related primarily to the impairment of Parion's pulmonary ENaC platform and decrease in the fair value of the contingent payments payable by the Company to Parion.

As of September 30, 2018 and December 31, 2017, the Company has unrecognized tax benefits of \$6.8 million and \$3.8 million, respectively. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of September 30, 2018, no interest and penalties have been accrued. The Company does not

expect that its unrecognized tax benefits will materially increase within the next twelve months. The Company did not recognize any material interest or penalties related to uncertain tax positions as of September 30, 2018 and December 31, 2017.

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The Company maintains a valuation allowance on the majority of its net operating losses and other deferred tax assets. Accordingly, the Company has not reported any benefits from income taxes relating to the remaining net operating losses and income tax credit carryforwards that will be utilized in future periods in those jurisdictions in which a valuation allowance is recorded.

On a periodic basis, the Company reassesses the valuation allowance on its deferred income tax assets, weighing positive and negative evidence to assess the recoverability of the deferred tax assets. In 2017, the Company reassessed the valuation allowance and considered negative evidence, including its cumulative losses over the three years ended December 31, 2017, and positive evidence, including its income during the year ended December 31, 2017. After assessing both the negative and positive evidence, the Company concluded that it should maintain the valuation allowance on its net operating losses and the majority of its other deferred tax assets as of December 31, 2017. The Company may release all or a portion of the valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, the Company's level of profitability, revenue growth, clinical program progression and expectations regarding future profitability. The Company's total deferred tax asset balance subject to the valuation allowance was approximately \$1.6 billion at December 31, 2017.

As described in Note A, "Basis of Presentation and Accounting Policies," the Company adopted amended guidance on the recognition of the deferred tax effects of intra-entity transfers of assets other than inventory, effective January 1, 2018. In connection with the adoption of this new standard, the Company recorded a deferred tax asset and corresponding full valuation allowance of \$204.7 million equal to the unamortized cost of intellectual property rights transferred to the United Kingdom in 2014 multiplied by an appropriate statutory rate. As a result, there was no cumulative effect adjustment to accumulated deficit using the modified-retrospective adoption approach.

In December 2017, the SEC staff issued SAB 118 to address the application of GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of H.R.1. The Company has recognized the provisional tax impacts related to deemed repatriated earnings and the revaluation of deferred tax assets and liabilities and included these amounts in its consolidated financial statements for the year ended December 31, 2017. The Company has an accumulated deficit from its foreign operations and does not have an associated liability from the repatriation tax on accumulated earnings in H.R.1. The ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, additional analysis, changes in interpretations and assumptions the Company has made, additional regulatory guidance that may be issued, and actions the Company may take as a result of H.R.1. The Company's accounting treatment is expected to be complete in the fourth quarter, which is one year from the enactment of H.R.1.

As of September 30, 2018, foreign earnings, which were not significant, have been retained indefinitely by foreign subsidiary companies for 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 2014, except where the Company has net operating losses or tax credit carryforwards that originate before 2014. The Company currently is under examination in Canada for 2011 through 2013, Germany for 2012 through 2015 and Italy for 2015 and 2016. No adjustments have been reported. The Company is not under examination by any other jurisdictions for any tax year.

**N. Restructuring Liabilities**

The Company has adopted several plans to restructure its facilities and operations for which it has incurred restructuring expenses. During the three and nine months ended September 30, 2018, the Company's restructuring expenses were not significant. During the nine months ended September 30, 2017, the Company's restructuring expenses primarily related to its decision to consolidate its research activities into its Boston, Milton Park and San

Diego locations commencing in February 2017. The Company closed its research site in Canada as a result of this decision affecting approximately 70 positions. As of September 30, 2018, the restructuring liability associated with this event was not material to the Company's condensed

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consolidated balance sheet. The Company does not anticipate any significant additional charges related to this restructuring event in the future.

O. Commitments and Contingencies

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 September 30, 2018 or December 31, 2017.

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P. Subsequent Event

In October 2018, the Company announced it will stop clinical development of VX-210 and terminate the Phase 2b clinical trial of VX-210 based on the recommendation of the clinical trial's Data Safety Monitoring Board and the Company's review of interim data the Company received in October 2018. The Company is in the process of determining its next steps with respect to the BioAxone Agreement. As a result of this decision, the Company expects to recognize an impairment charge in the fourth quarter of 2018 related to its VX-210 in-process research and development intangible asset of approximately \$29.0 million, which will be partially offset by a related benefit from income taxes. The Company also will assess whether it continues to be the primary beneficiary of BioAxone and should continue to consolidate BioAxone as a VIE and decrease the fair value of contingent payments payable by the Company to BioAxone in the fourth quarter of 2018.

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## 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 serious diseases. Our business is focused on developing and commercializing therapies for the treatment of cystic fibrosis, or CF, and advancing our research and development programs in other diseases. Our marketed products are KALYDECO (ivacaftor), ORKAMBI (lumacaftor in combination with ivacaftor) and SYMDEKO (tezacaftor in combination with ivacaftor), which are collectively approved to treat approximately 45% of the 75,000 CF patients in North America, Europe and Australia.

## Cystic Fibrosis

## Current Medicines

KALYDECO is approved for the treatment of approximately 6,000 CF patients who have the G551D mutation or other specified mutations in their cystic fibrosis transmembrane conductance regulator, or CFTR, gene. ORKAMBI is approved as a treatment for approximately 29,000 patients who have two copies of the F508del mutation, or F508del homozygous, in their CFTR gene. SYMDEKO was approved by the United States Food and Drug Administration, or FDA, in February 2018 for the treatment of patients with CF twelve years of age and older who are F508del homozygous or who have at least one mutation that is responsive to tezacaftor/ivacaftor and by Health Canada in June 2018 for the treatment of patients with CF twelve years of age and older who are F508del homozygous or who have other specified mutations in their CFTR gene. In July 2018, the European Medicines Agency, or EMA, adopted a positive opinion for tezacaftor in combination with ivacaftor, which we plan to market under the brand name SYMKEVI, and we expect to obtain approval for SYMKEVI in the European Union in the fourth quarter of 2018. SYMDEKO provides, and SYMKEVI, if approved, will provide an additional treatment option to certain CF patients who were already eligible for either KALYDECO or ORKAMBI. We use the brand name of a product only when we refer to the product that has been approved and with respect to the indications on the approved label. Otherwise, we use the scientific (or generic) name of the applicable compound or compounds comprising a drug candidate.

We continuously seek to increase the number of patients eligible to receive our medicines through label expansions. In the third quarter of 2018, we obtained approval from the FDA for KALYDECO for patients 12 to 24 months of age and ORKAMBI for patients 2 to 5 years of age. Activities in support of our label expansion efforts include:

In October 2018, we announced positive data from a Phase 3 clinical trial evaluating tezacaftor in combination with ivacaftor in patients with CF six to eleven years of age who are F508del homozygous or who have one copy of the F508del mutation and one residual function mutation. The clinical trial met its primary safety endpoint, and safety data showed that the combination was generally well tolerated. We plan to submit a supplemental new drug application, or sNDA, to the FDA in late 2018. To support potential approval in the European Union, an eight-week Phase 3 clinical trial is ongoing to evaluate tezacaftor in combination with ivacaftor in approximately 65 children six to eleven years of age. The primary endpoint of the clinical trial is the absolute change in the lung clearance index.

In October 2018, we announced positive data from a Phase 3 clinical trial of ivacaftor in patients with CF six to twelve months of age. We expect to submit an sNDA to the FDA and a line extension to the EMA in late 2018.

## Next-generation CFTR Corrector Compounds

We are conducting Phase 3 clinical trials for two next-generation corrector compounds, VX-659 and VX-445, as part of separate triple combination regimens with tezacaftor and ivacaftor. Each of the VX-659 and VX-445 Phase 3 development programs is comprised of two clinical trials. The first clinical trial in each program is enrolling approximately 360 patients with CF twelve years of age and older who have one copy of the F508del mutation in their CFTR gene and a second mutation that results in minimal CFTR function, whom we refer to as F508del/Min patients. The primary efficacy endpoint of the first clinical trial in each program is the mean absolute change from baseline in percent predicted forced expiratory volume in one second, or ppFEV1, at week four of treatment with the triple combination regimen versus placebo. The second clinical trial in each program will enroll approximately 100 F508del homozygous patients twelve years of age and older. The primary efficacy endpoint of the second clinical trial in each program is the mean absolute change from baseline in ppFEV1 at week four of treatment with the triple combination regimen compared to tezacaftor in combination with ivacaftor. We have completed enrollment in the Phase 3 clinical program for VX-659 and expect to complete enrollment of the Phase 3 clinical program for VX-445 in the fourth

quarter of 2018. We expect data from the VX-659 program in late 2018 and from the VX-445 program in the first quarter of 2019. Based on the anticipated completion of enrollment for the Phase 3 programs, we expect to submit an NDA to the FDA for F508del/Min patients no later than mid-2019. We have also recently initiated Phase 3 clinical trials for both VX-659 and VX-445 in F508del/Min and F508del homozygous patients who are six to 11 years of age.



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We believe the triple combination regimens we are evaluating could provide benefits to all CF patients who have at least one F508del mutation in their CFTR gene (approximately 90% of all CF patients). If approved, this would include (i) the first treatment option that treats the underlying cause of CF for F508del/Min patients, and (ii) an additional treatment option for patients with CF who are eligible for KALYDECO, ORKAMBI and/or SYMDEKO. In the second quarter of 2018, we announced positive clinical data from (i) a Phase 2 clinical trial arm evaluating VX-561 in combination with ivacaftor and VX-659 and (ii) a Phase 2 clinical trial arm evaluating VX-561 in combination with ivacaftor and VX-445. Based on recent feedback from the FDA, we plan to initiate a Phase 2 clinical trial in the first half of 2019 evaluating VX-561 as a monotherapy in patients with CF who have a gating mutation.

### Research and Development

We have a number of ongoing research and development programs in other diseases that we are conducting independently or in collaboration with third parties. We recently completed enrollment in a Phase 2 proof-of-concept clinical trial evaluating VX-150 for the treatment of pain caused by small fiber neuropathy and expect data from this clinical trial in early 2019. We plan to initiate a Phase 2b dose-ranging clinical trial to evaluate VX-150 in patients with acute pain following bunionectomy surgery. The clinical trial is designed to evaluate multiple oral doses of VX-150 to potentially support pivotal development in acute pain.

We are co-developing CTX001, an investigational gene editing treatment, for the treatment of beta-thalassemia and sickle cell disease, with CRISPR Therapeutics AG, or CRISPR.

In October 2018, the FDA lifted a clinical hold on the investigational new drug application, or IND, for CTX001 for the treatment of sickle cell disease that was submitted earlier this year. We and CRISPR expect to initiate the clinical trial in sickle cell disease by the end of 2018. The first two patients in the clinical trial will be dosed sequentially and, pending data from these initial two patients, subsequent patients can be dosed concurrently.

Enrollment of a Phase 1/2 clinical trial evaluating CTX001 in patients with beta-thalassemia is open at multiple clinical trial sites in Europe. The first patient in this Phase 1/2 clinical trial of CTX001 has been enrolled. This Phase 1/2 clinical trial is designed to assess the safety and efficacy of CTX001 in adult transfusion-dependent non beta zero/beta zero patients with beta-thalassemia. Similar to the clinical trial in sickle cell patients, the first two patients in the clinical trial will be dosed sequentially and, pending data from these initial two patients, subsequent patients can be dosed concurrently.

We plan to continue investing in our research programs and fostering scientific innovation in order to identify and develop transformative medicines for people with serious diseases. In addition to continuing our research in cystic fibrosis, pain and hemoglobinopathies, our current internal research programs include programs targeting alpha-1 antitrypsin deficiency, or AAT, focal segmental glomerulosclerosis and polycystic kidney disease. We plan to initiate clinical development of our first molecule in AAT by the end of 2018.

To supplement our internal research programs, we seek to 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. 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.

### 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 and can take 10 to 15 years or more. 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. Because 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 abrupt 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.

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If we believe that data from a completed registration program support approval of a drug candidate, we submit an NDA to the FDA requesting approval to market the drug candidate in the United States and seek analogous approvals from comparable regulatory authorities in jurisdictions outside the United States. 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 make it illegal for a prescription drug manufacturer to solicit, offer, receive or pay any remuneration to induce the referral of business, including the purchase or prescription of a particular drug that is reimbursed by a state or federal 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 that govern our international business practices with respect to payments to government officials. 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 covered 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 United States and ex-U.S. markets. In the United States, we have worked successfully with third party payors in order to promptly obtain appropriate levels of reimbursement for our CF medicines. We 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 in order to provide continued access to our current medicine.

In Europe and other ex-U.S. markets, we seek government reimbursement for our medicines on a country-by-country basis, because in many foreign countries patients are unable to access prescription pharmaceutical products that are not reimbursed by their governments. Typically, we need to obtain country-by-country reimbursement for each new medicine and each label expansion for a current medicine. We successfully obtained reimbursement for KALYDECO in each significant ex-U.S. market within two years of approval. Since we obtained approval for ORKAMBI in 2015, we have experienced significant challenges in obtaining reimbursement for ORKAMBI in ex-U.S. markets. We continue to discuss potential reimbursement for ORKAMBI in the United Kingdom and France, which represent significant potential markets for our CF medicines. We have reached pricing and reimbursement agreements for ORKAMBI in a number of countries, including Australia, Denmark, Germany, Ireland, Sweden and Italy. We have innovative reimbursement arrangements in place in certain ex-U.S. jurisdictions, including arrangements in Ireland, Denmark and Australia that provide a pathway to access and rapid reimbursement for certain future CF medicines.

### Collaboration Arrangements and Strategic Investments

#### 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:

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- CRISPR, pursuant to which we are collaborating on the discovery and development of potential new treatments aimed at the underlying genetic causes of human diseases using CRISPR-Cas9 gene editing technology;
- Moderna Therapeutics, Inc., or Moderna, pursuant to which we are seeking to identify and develop messenger ribonucleic acid, or mRNA therapeutics for the treatment of CF;
- BioAxone, pursuant to which we have been evaluating VX-210 as a potential treatment for patients who have spinal cord injuries; and
- Parion Sciences, Inc., or Parion, pursuant to which we are developing epithelial sodium channel, or ENaC, inhibitors for the treatment of pulmonary diseases.

Generally, when we in-license a technology or drug candidate, we make upfront payments to the collaborator, assume the costs of the program and agree to make contingent payments, which could consist of milestone, royalty and option payments. Depending on many factors, including the structure of the collaboration, the significance of the drug candidate that we license to the collaborator's operations and the other activities in which our collaborators are engaged, the accounting for these transactions can vary significantly. For example, the upfront payments and expenses incurred in connection with our CRISPR and Moderna collaborations are being expensed as research expenses because the collaboration represents a small portion of each of these collaborator's overall business. CRISPR and Moderna's activities unrelated to our collaborations have no effect on our consolidated financial statements. Parion and BioAxone have historically been accounted for as variable interest entities, or VIEs, and historically have been included in our consolidated financial statements due to: (i) the significance of the respective licensed programs to Parion and BioAxone as a whole, (ii) our power to control the significant activities under each collaboration, and (iii) our obligation to absorb losses and right to receive benefits that potentially could be significant. As of September 30, 2017, we determined that the above conditions were no longer satisfied with respect to Parion following the results of a Phase 2 clinical trial of VX-371 that did not meet its primary efficacy endpoint. As a result, we deconsolidated Parion from our consolidated financial statements as of September 30, 2017. BioAxone continues to be accounted for as a VIE and remains included in our consolidated financial statements as of September 30, 2018. However, based on interim data from a Phase 2b clinical trial of VX-210, the clinical trial's data monitoring safety board, or DSMB, has recommended that we stop the clinical trial early due to futility. There were no safety concerns noted in the DSMB's review of the data. Based on the DSMB's recommendation and our review of the interim data, we have decided to stop clinical development of VX-210 and to terminate the Phase 2b clinical trial of VX-210. As a result of this decision, we expect to recognize a charge in the fourth quarter of 2018 attributable to noncontrolling interest related to the impairment of our VX-210 intangible asset of approximately \$29.0 million, which will be partially offset by a related benefit from income taxes. We will also assess whether we continue to be the primary beneficiary of BioAxone and should continue to consolidate BioAxone's financial statements in the fourth quarter of 2018.

A collaborator that we account for as a VIE may engage in activities unrelated to our collaboration. The revenues and expenses unrelated to the programs we in-license from our VIEs have historically been immaterial to our consolidated financial statements. With respect to each of Parion, prior to its deconsolidation as of September 30, 2017, and BioAxone, the activities unrelated to our collaborations with these entities have represented approximately 2% or less of our total revenues and total expenses on an annual basis. As a result of the deconsolidation of Parion, we expect these amounts to decrease in future periods. For any consolidated VIEs, we evaluate the fair value of the contingent payments payable by us on a quarterly basis. Changes in the fair value of these contingent future payments affect net income attributable to Vertex on a dollar-for-dollar basis, with increases in the fair value of contingent payments payable by us to a VIE resulting in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) and decreases in the fair value of contingent payments payable by us to a VIE resulting in an increase in net income attributable to Vertex (or decrease in net loss attributable to Vertex). For additional information regarding our VIEs see Note C, "Collaborative Arrangements and Acquisitions," and our critical accounting policies in our 2017 Annual Report on Form 10-K.

**Out-License Agreements**

We also have out-licensed internally developed programs to collaborators who are leading the development of these programs. These outlicense arrangements include our collaboration agreements with:

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Janssen Pharmaceuticals, Inc., or Janssen, Inc., which is developing pimodivir (formerly VX-787) for the treatment of influenza; and

Merck KGaA, which licensed four 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

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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/or royalty revenues resulting from these programs.

Strategic Investments

In connection with our business development activities, we have periodically made equity investments in our collaborators. As of September 30, 2018 and December 31, 2017, we held strategic equity investments in CRISPR, a public company, and certain private companies, and we may make additional strategic equity investments in the future. While we invest the majority of our cash, cash equivalents and marketable securities in instruments with low risk 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.

Until December 31, 2017, changes in the fair value of these strategic investments were reflected on our balance sheet, but did not affect our net income until the related gains or losses were realized. As a result of new accounting guidance, effective January 1, 2018, any changes in the fair value of equity investments with readily determinable fair values (including publicly traded securities such as CRISPR) are recorded to other income (expense), net in our condensed consolidated statement of operations. For equity investments without readily determinable fair values (including private equity investments such as Moderna), each reporting period we are required to re-evaluate the carrying value of the investment, which may result in other income (expense).

In the third quarter and nine months ended September 30, 2018, we recorded within other income (expense), net an unrealized loss of \$61.2 million and an unrealized gain of \$85.3 million, respectively, related to changes in the fair value of our investment in CRISPR, which are included in net income attributable to Vertex. To the extent that we continue to hold strategic investments and in particular strategic investments in publicly traded companies, we will record on a quarterly basis other income (expense) related to these strategic investments. Due to 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 under GAAP on a quarterly and/or annual basis.

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## RESULTS OF OPERATIONS

	Three Months Ended		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	September 30, 2018	2017	\$	%	September 30, 2018	2017	\$	%
	(in thousands)				(in thousands)			
Revenues	\$784,535	\$578,165	\$206,370	36 %	\$2,177,491	\$1,837,018	\$340,473	19 %
Operating costs and expenses	578,886	904,208	(325,322 )	(36 )%	1,670,063	1,839,512	(169,449 )	(9 )%
Other items, net	(76,903 )	223,091	(299,994 )	n/a	38,941	165,294	(126,353 )	(76 )%
Net income (loss) attributable to Vertex	\$128,746	\$(102,952)	\$231,698	n/a	\$546,369	\$162,800	\$383,569	236 %
Net income (loss) per diluted share attributable to Vertex common shareholders	\$0.50	\$(0.41 )			\$2.11	\$0.64		
Diluted shares used in per share calculations	259,788	250,268			258,972	252,095		

## Net Income (Loss) Attributable to Vertex

Net income attributable to Vertex was \$128.7 million in the third quarter of 2018 as compared to net loss attributable to Vertex of \$103.0 million in the third quarter of 2017. Net income attributable to Vertex in the third quarter of 2018 reflected increases in CF net product revenues of \$232.9 million as compared to the third quarter of 2017. The decrease in operating costs and expenses in the third quarter of 2018 as compared to the third quarter of 2017 primarily reflected a one-time \$255.3 million impairment charge related to Parion's pulmonary ENaC platform recorded in the third quarter of 2017 and a one-time \$160.0 million payment to Concert in the third quarter of 2017 in connection with our acquisition of VX-561. There are not comparable expenses for either expense in the third quarter of 2018. The decrease in operating costs and expenses in the third quarter of 2018 as compared to the third quarter of 2017 was partially offset by increases in cost of sales and sales, general and administrative expenses.

Net income attributable to Vertex was \$546.4 million in the nine months ended September 30, 2018 as compared to net income attributable to Vertex of \$162.8 million in the nine months ended September 30, 2017. Our total revenues increased in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017, primarily due to a \$625.9 million increase in CF net product revenues, partially offset by \$230.0 million in one-time collaborative revenues recorded in the first quarter of 2017 related to an upfront payment from Merck KGaA. The decrease in operating costs and expenses in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017 primarily reflected a one-time \$255.3 million impairment charge related to Parion's pulmonary ENaC platform recorded in the third quarter of 2017 and a one-time \$160.0 million payment to Concert in the third quarter of 2017 in connection with our acquisition of VX-561. There are not comparable expenses for either expense in the nine months ended September 30, 2018. The decrease in operating costs and expenses in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017 was partially offset by increases in cost of sales and sales, general and administrative expenses.

Other items, net, in the third quarter and nine months ended September 30, 2018 primarily related to an unrealized loss of \$61.2 million and an unrealized gain of \$85.3 million, respectively, associated with changes in the fair value of our investment in CRISPR. These unrealized gains and losses are included in other items, net due to new accounting guidance that became effective on January 1, 2018. Other items, net, in the third quarter and nine months ended September 30, 2017 primarily related to a benefit from income taxes resulting from the impairment of Parion's pulmonary ENaC platform charge and subsequent deconsolidation of Parion attributable to noncontrolling interest that was recorded in the third quarter of 2017.

Diluted Net Income (Loss) Per Share Attributable to Vertex Common Shareholders



Diluted net income per share attributable to Vertex common shareholders was \$0.50 in the third quarter of 2018 as compared to diluted net loss per share attributable to Vertex common shareholders of \$0.41 in the third quarter of 2017.

Diluted net income per share attributable to Vertex common shareholders was \$2.11 in the nine months ended September 30, 2018 as compared to diluted net income per share attributable to Vertex common shareholders of \$0.64 in the nine months ended September 30, 2017.

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## Revenues

	Three Months				Nine Months Ended			
	Ended September		Increase/(Decrease)		September 30,		Increase/(Decrease)	
	2018	2017	\$	%	2018	2017	\$	%
	(in thousands)				(in thousands)			
Product revenues, net	\$782,511	\$549,642	\$232,869	42 %	\$2,170,152	\$1,544,252	\$625,900	41 %
Royalty revenues	1,238	2,231	(993)	(45)%	3,679	6,643	(2,964)	(45)%
Collaborative revenues	786	26,292	(25,506)	(97)%	3,660	286,123	(282,463)	(99)%
Total revenues	\$784,535	\$578,165	\$206,370	36 %	\$2,177,491	\$1,837,018	\$340,473	19 %

## Product Revenues, Net

	Three Months				Nine Months Ended			
	Ended September		Increase/(Decrease)		September 30,		Increase/(Decrease)	
	2018	2017	\$	%	2018	2017	\$	%
	(in thousands)				(in thousands)			
KALYDECO	\$245,733	\$213,461	\$32,272	15 %	\$748,365	\$588,809	\$159,556	27 %
ORKAMBI	281,859	336,183	(54,324)	(16)%	947,186	955,451	(8,265)	(1)%
SYMDEKO	254,919	—	254,919	n/a	474,601	—	474,601	n/a
Total CF product revenues, net	\$782,511	\$549,644	\$232,867	42 %	\$2,170,152	\$1,544,260	\$625,892	41 %

In the third quarter and nine months ended September 30, 2018, our total CF net product revenues increased by \$232.9 million and \$625.9 million, respectively, as compared to the third quarter and nine months ended September 30, 2017. The increase in total CF net product revenues was due to net product revenues from sales of SYMDEKO, which was approved by the FDA in February 2018, and increased KALYDECO net product revenues partially offset by decreased ORKAMBI net product revenues. We believe that our total CF net product revenues will continue to increase in the fourth quarter of 2018, largely driven by continued growth of SYMDEKO.

SYMDEKO net product revenues were \$254.9 million and \$474.6 million in the third quarter and nine months ended September 30, 2018, respectively. We expect SYMDEKO net product revenues to continue to increase in the fourth quarter of 2018 as additional patients initiate treatment. We believe that these additional patients will be comprised of both patients who were not previously receiving treatment with one of our CF medicines and patients who are switching from either ORKAMBI or KALYDECO to SYMDEKO. We expect the European Union to approve tezacaftor in combination with ivacaftor, which will be sold under the brand name SYMKEVI in ex-U.S. markets, in the fourth quarter of 2018, but even if approved, we do not expect to recognize significant net product revenues from sales of SYMKEVI during 2018.

KALYDECO net product revenues increased in the third quarter and nine months ended September 30, 2018 as compared to the third quarter and nine months ended September 30, 2017, primarily due to additional patients being treated with KALYDECO as we completed reimbursement discussions in various ex-U.S. jurisdictions and as we increased the number of patients eligible to receive KALYDECO through label expansions. In the third quarter and nine months ended September 30, 2018, we recognized \$90.9 million and \$268.7 million, respectively, in ex-U.S. KALYDECO net product revenues as compared to \$80.3 million and \$242.5 million in the third quarter and nine months ended September 30, 2017, respectively. We expect that net product revenues from sales of KALYDECO for the fourth quarter of 2018 will be similar to KALYDECO net product revenues from the third quarter of 2018. The approval of SYMDEKO has had, and we expect it will continue to have, a negative effect on the U.S. net product revenues from ORKAMBI as patients switch from ORKAMBI to SYMDEKO. In the third quarter of 2018, ORKAMBI U.S. net product revenues decreased by \$26.6 million as compared to the second quarter of 2018 and by \$73.4 million as compared to the first quarter of 2018. Global ORKAMBI net product revenues decreased by \$54.3 million and \$8.3 million in the third quarter and nine months ended September 30, 2018, respectively, as compared to the third quarter and nine months ended September 30, 2017, due to a decrease in U.S. net product revenues, partially offset by an increase in ex-U.S. ORKAMBI net product revenues. In the third quarter and nine months ended

September 30, 2018, we recognized approximately \$73.0 million and \$220.5 million, respectively, in ex-U.S. ORKAMBI net product revenues as compared to \$43.1 million and \$110.9 million in the third quarter and nine months ended September 30, 2017, respectively.

Our condensed consolidated balance sheet includes \$324.3 million collected as of September 30, 2018 in France related to ORKAMBI supplied under early access programs at the invoiced price. Pursuant to revenue recognition guidance that became effective under GAAP on January 1, 2018, we have recognized limited net product revenues to date on sales of ORKAMBI in France due to ongoing pricing discussions regarding the reimbursement rate for ORKAMBI. Please refer to Note A, "Basis of

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Presentation and Accounting Policies,” for a discussion of the application of the new revenue recognition guidance and to Note B, “Revenue Recognition,” for a discussion of our accounting treatment for our early access programs for ORKAMBI in France.

**Royalty Revenues**

Our royalty revenues were \$1.2 million and \$3.7 million in the third quarter and nine months ended September 30, 2018, respectively, as compared to \$2.2 million and \$6.6 million in the third quarter and nine months ended September 30, 2017, respectively. Our royalty revenues consist of revenues related to a cash payment we received in 2008 when we sold our rights to certain HIV royalties. We expect to continue to record royalty revenues related to this payment through the first half of 2019. Other future royalty revenues will be dependent on if, and when, our collaborators, including Janssen, Inc. and Merck KGaA, are able to successfully develop drug candidates that we have outlicensed to them.

**Collaborative Revenues**

Our collaborative revenues were \$0.8 million and \$3.7 million in the third quarter and nine months ended September 30, 2018, respectively, as compared to \$26.3 million and \$286.1 million in the third quarter and nine months ended September 30, 2017, respectively. The decrease in our collaborative revenues during the third quarter of 2018 as compared to the third quarter of 2017 was primarily due to a \$20.0 million milestone payment earned by Parion in the third quarter of 2017 pursuant to a license agreement Parion entered into with a third party. The milestone payment was included in our condensed consolidated financial statements even though we are not a party to such license agreement and have no economic interest in either the license or the milestone payment because during that period we were consolidating Parion as a VIE. Parion was deconsolidated as a VIE as of September 30, 2017 and payments received by Parion pursuant to this license agreement after this date are not recognized by us as collaborative revenues. The decrease in our collaborative revenues during the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017 was primarily due to revenue recognized related to the one-time \$230.0 million upfront payment earned in the first quarter of 2017 from Merck KGaA and \$40 million in upfront and milestone payments received by Parion in 2017 pursuant to its license agreement with a third party. Our collaborative revenues have historically fluctuated significantly from one period to another and may continue to fluctuate in the future.

**Operating Costs and Expenses**

	Three Months Ended		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)		
	September 30, 2018	September 30, 2017	\$	%	September 30, 2018	September 30, 2017	\$	%	
	(in thousands)				(in thousands)				
Cost of sales	\$ 111,255	\$ 72,874	\$ 38,381	53 %	\$ 287,250	\$ 191,067	\$ 96,183	50 %	
Research and development expenses	330,510	454,947	(124,437 )	(27 )%	978,595	1,017,961	(39,366 )	(4 )%	
Sales, general and administrative expenses	137,295	120,710	16,585	14 %	404,406	361,285	43,121	12 %	
Restructuring (income) expenses	(174 )	337	(511 )	n/a	(188 )	13,859	(14,047 )	n/a	
Intangible asset impairment charge	—	255,340	(255,340 )	(100)%	—	255,340	(255,340 )	(100)%	
Total costs and expenses	\$ 578,886	\$ 904,208	\$ (325,322)	(36 )%	\$ 1,670,063	\$ 1,839,512	\$ (169,449)	(9 )%	
Cost of Sales									

Our cost of sales primarily consists of the cost of producing inventories that corresponded to product revenues for the reporting period, plus the third-party royalties payable on our net sales of our products. Cost of sales also includes a small subroyalty payable to a third party related to royalty revenues that we historically classified under “Royalty Expenses.” Pursuant to our agreement with Cystic Fibrosis Foundation, our tiered third-party royalties on sales of SYMDEKO, KALYDECO and ORKAMBI, calculated as a percentage of net sales, range from the single digits to the sub-teens. As a result of the tiered royalty rate, which resets annually, our cost of sales as a percentage of CF net

product revenues are lower at the beginning of each calendar year.

Our cost of sales have been increasing primarily because of increased net product revenues. In the fourth quarter of 2018, we expect our cost of sales as a percentage of total CF net product revenues to be similar to the cost of sales as a percentage of total CF net product revenues in the third quarter of 2018.

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## Research and Development Expenses

	Three Months		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	Ended September 30, 2018	2017	\$	%	September 30, 2018	2017	\$	%
	(in thousands)				(in thousands)			
Research expenses	\$83,701	\$76,131	\$7,570	10 %	\$247,656	\$226,409	\$21,247	9 %
Development expenses	246,809	378,816	(132,007 )	(35)%	730,939	791,552	(60,613 )	(8)%
Total research and development expenses	\$330,510	\$454,947	\$(124,437 )	(27)%	\$978,595	\$1,017,961	\$(39,366 )	(4)%

Our research and development expenses include internal and external costs incurred for research and development of our drugs and drug candidates. We do not assign our internal costs, such as salary and benefits, stock-based compensation expense, laboratory supplies and other direct expenses and infrastructure costs, to individual 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 2015, we have incurred \$4.3 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 2017 and the nine months ended September 30, 2018, 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. We expect approval for ivacaftor in combination with tezacaftor in the European Union in the fourth quarter of 2018. If we obtain positive results from our ongoing Phase 3 clinical trials, we plan to submit an NDA to the FDA for a triple combination regimen with VX-659 or VX-445 no later than mid-2019. We cannot make a meaningful estimate when, if ever, our other clinical development programs will generate revenues and cash flows.

## Research Expenses

	Three Months		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	Ended September 30, 2018	2017	\$	%	September 30, 2018	2017	\$	%
	(in thousands)				(in thousands)			
Research Expenses:								
Salary and benefits	\$21,745	\$20,445	\$1,300	6 %	\$67,078	\$61,486	\$5,592	9 %
Stock-based compensation expense	16,462	15,641	821	5 %	47,503	44,366	3,137	7 %
Laboratory supplies and other direct expenses	12,312	10,791	1,521	14 %	37,327	33,980	3,347	10 %
Outsourced services	9,236	10,230	(994 )	(10)%	28,082	29,644	(1,562 )	(5)%

Collaboration and asset acquisition payments	1,791	425	1,366	321	%	4,350	425	3,925	924	%
Infrastructure costs	22,155	18,599	3,556	19	%	63,316	56,508	6,808	12	%
Total research expenses	\$83,701	\$76,131	\$7,570	10	%	\$247,656	\$226,409	\$21,247	9	%

We maintain a substantial investment in research activities. Our research expenses increased by 10% in the third quarter of 2018 as compared to the third quarter of 2017 and increased by 9% in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017. We expect to continue to invest in our research programs with a focus on identifying drug candidates with the goal of creating transformative medicines for serious diseases.

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## Development Expenses

	Three Months		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	Ended September				September 30,			
	2018	2017	\$	%	2018	2017	\$	%
	(in thousands)				(in thousands)			
Development Expenses:								
Salary and benefits	\$56,417	\$54,125	\$2,292	4 %	\$167,208	\$156,759	\$10,449	7 %
Stock-based compensation expense	36,456	30,545	5,911	19 %	105,515	90,489	15,026	17 %
Laboratory supplies and other direct expenses	23,571	10,828	12,743	118 %	60,083	34,171	25,912	76 %
Outsourced services	81,321	89,637	(8,316 )	(9 )%	259,499	251,677	7,822	3 %
Collaboration and asset acquisition payments	—	160,000	(160,000 )	(100)%	250	160,250	(160,000 )	(100)%
Drug supply costs	12,301	3,151	9,150	290 %	32,014	6,143	25,871	421 %
Infrastructure costs	36,743	30,530	6,213	20 %	106,370	92,063	14,307	16 %
Total development expenses	\$246,809	\$378,816	\$(132,007)	(35 )%	\$730,939	\$791,552	\$(60,613 )	(8 )%

Our development expenses decreased by 35% in the third quarter of 2018 as compared to the third quarter of 2017 and decreased by 8% in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017, primarily due the \$160.0 million payment to Concert in connection with the acquisition of VX-561 in the third quarter of 2017, partially offset by increased costs associated with ongoing clinical trials, including trials involving our next-generation CFTR corrector compounds that we are evaluating as part of triple combination treatment regimens.

## Sales, General and Administrative Expenses

	Three Months		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	Ended September				September 30,			
	2018	2017	\$	%	2018	2017	\$	%
	(in thousands)				(in thousands)			
Sales, general and administrative expenses	\$137,295	\$120,710	\$16,585	14 %	\$404,406	\$361,285	\$43,121	12 %

Sales, general and administrative expenses increased by 14% in the third quarter of 2018 as compared to the third quarter of 2017 and increased by 12% in the nine months ended September 30, 2018 as compared to the nine months ended September 30, 2017, primarily due to increased global support for KALYDECO and ORKAMBI and costs related to the launch of SYMDEKO in the United States.

## Restructuring (Income) Expenses

We recorded restructuring credits of \$174 thousand in the third quarter of 2018 and restructuring credits of \$188 thousand in the nine months ended September 30, 2018, as compared to restructuring expenses of \$0.3 million and \$13.9 million in the third quarter and nine months ended September 30, 2017, respectively. The restructuring expenses in the third quarter and nine months ended September 30, 2017 primarily related to our decision to consolidate our research activities into our Boston, Milton Park and San Diego locations and to close our research site in Canada.

## Intangible Asset Impairment Charge

In the third quarter of 2017, we recorded a \$255.3 million impairment charge related to Parion's pulmonary ENaC platform that we licensed from Parion in 2015 and a benefit from income taxes of \$97.7 million related to this impairment charge attributable to Parion. There were no corresponding intangible asset impairment charges in the three or nine months ended September 30, 2018.

## Other Items, Net

## Interest Expense, Net



Our interest expense, net relates primarily to interest expenses associated with certain of our real estate leases and outstanding debt, if any, partially offset by interest income from the investment of our cash equivalents and marketable securities. Net interest expense was \$8.1 million in the third quarter of 2018 as compared to \$13.6 million in the third quarter of 2017. The decrease in net interest expense in the third quarter of 2018 was primarily due to an increase in our interest

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income resulting from an increase in our cash equivalents and marketable securities, partially offset by additional interest expense associated with our lease in San Diego, California that we began incurring in the second quarter of 2018.

Interest expense, net was \$29.3 million in the nine months ended September 30, 2018 as compared to \$45.0 million in the nine months ended September 30, 2017. The decrease in net interest expense in the nine months ended September 30, 2018 was primarily due to an increase in our interest income resulting from an increase in our cash equivalents and marketable securities and a decrease in interest expense due to the repayment of \$300.0 million outstanding under our revolving credit facility in February 2017, partially offset by additional interest expense associated with our lease in San Diego, California.

For the fourth quarter of 2018, we expect to incur approximately \$17 million in imputed interest expenses related to our real estate leases. We expect our imputed interest expenses related to our real estate leases to decrease in 2019 as compared to 2018 based on updated guidance related to aspects of lease accounting that becomes effective in 2019. In addition to the updated accounting guidance, our future net interest expense will also be dependent on whether, and to what extent, we reborrow amounts under our credit facility and the amount of and prevailing market interest rates on our outstanding cash equivalents and marketable securities.

#### Other Income (Expense), Net

Other income (expense), net was an expense of \$61.0 million and income of \$89.7 million in the third quarter and nine months ended September 30, 2018, respectively, as compared to expenses of \$77.6 million and \$80.6 million in the third quarter and nine months ended September 30, 2017, respectively. Other expense, net in the third quarter of 2018 was primarily related to a \$61.2 million decrease in the fair value of our equity investment in CRISPR. Other income, net in the nine months ended September 30, 2018 was primarily related to an \$85.3 million increase in the fair value of our equity investment in CRISPR. Accounting guidance, which became effective January 1, 2018, requires that changes in the fair value of our equity investments are recorded in other income (expense), net in our condensed consolidated statement of operations. In prior periods, changes in the fair value of our equity investments were not reflected in our condensed consolidated statement of operations and a gain or loss was only recognized upon a sale of the underlying securities. As a result of the new guidance, our other income (expense), net will fluctuate based on increases or decreases in the fair value of our strategic investments.

Other expense, net in the third quarter and nine months ended September 30, 2017 was primarily related to the deconsolidation of Parion.

#### Income Taxes

In the third quarter of 2018, we recorded a provision for income taxes of \$8.1 million as compared to a benefit from income taxes of \$125.9 million in the third quarter of 2017. The provision for income taxes in the third quarter of 2018 was primarily due to \$4.9 million related to our U.S. state and foreign taxes and \$3.1 million related to the reversing effect of discrete items associated with stock-based compensation. The benefit from income taxes in the third quarter of 2017 related primarily to \$126.2 million attributable to noncontrolling interest as a result of our impairment of Parion's pulmonary ENaC platform and a decrease in the fair value of the contingent payments payable by us to Parion.

In the nine months ended September 30, 2018, we recorded a provision for income taxes of \$5.7 million as compared to a benefit from income taxes of \$117.6 million in the nine months ended September 30, 2017. The provision for income taxes in the nine months ended September 30, 2018 was primarily due to \$13.4 million related to our U.S. state and foreign taxes and \$6.1 million of income taxes attributable to BioAxone's income taxes partially offset by a benefit of \$13.7 million related to discrete items associated with stock-based compensation. The benefit from income taxes in the nine months ended September 30, 2017 related primarily to the amount recorded in the third quarter of 2017 for Parion's income taxes described above.

Since the adoption of new accounting guidance in 2017, we have been recording discrete tax adjustments associated with stock-based compensation on a quarterly basis. As noted above, we have recorded a net benefit of \$13.7 million related to these discrete items in the nine months ended September 30, 2018. In the fourth quarter of 2018, we expect the net benefit from income taxes recorded in the first three quarters of 2018 related to these adjustments to reverse, resulting in these discrete items having no effect on our annual provision for income taxes.

As discussed in Note M, "Income Taxes," we continue to maintain a valuation allowance on the majority of our net operating losses and other deferred tax assets. Due to this valuation allowance, we did not record a significant provision for income taxes in the third quarter and nine months ended September 30, 2018 and 2017. We are profitable from a U.S. federal income tax perspective and have used a portion of our net operating losses to offset this income since becoming profitable. We may release all or a portion of the valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and amount of such release, continue to be subject to, among other things, our level of profitability,

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our revenue growth, the progress of our clinical programs and expectations regarding future profitability. In the period of the release of the valuation allowance, we will recognize a significant non-cash credit to net income and we will reflect a deferred tax asset, which is currently subject to the valuation allowance, on our condensed consolidated balance sheet. Following the release, we expect to continue to utilize our net operating losses to offset income, but would begin recording a significant provision for income taxes reflecting the utilization of the deferred tax assets. The majority of this provision for income taxes would be a non-cash expense until our net operating losses are fully utilized. Our total deferred tax asset balance subject to the valuation allowance was approximately \$1.6 billion at December 31, 2017.

## Noncontrolling Interest (VIEs)

The net (income) loss attributable to noncontrolling interest (VIEs) recorded on our condensed consolidated statements of operations reflects our VIE's net (income) loss for the reporting period, adjusted for any changes in the noncontrolling interest holders' claim to net assets, including contingent milestone, royalty and option payments. A summary of net (income) loss attributable to noncontrolling interest related to our VIEs for the third quarter and nine months ended of 2018 and 2017 is as follows:

	Three Months Ended September 30, 2018 2017		Nine Months Ended September 30, 2018 2017	
	(in thousands)			
Loss attributable to noncontrolling interest before provision for (benefit from) income taxes and changes in fair value of contingent payments	\$411	\$238,946	\$1,394	\$222,448
Provision for (benefit from) income taxes	79	(120,181)	6,068	(111,658)
(Increase) decrease in fair value of contingent payments	(200)	69,550	(23,100)	62,560
Net loss (income) attributable to noncontrolling interest	\$290	\$188,315	\$(15,638)	\$173,350

The net income attributable to noncontrolling interest in the nine months ended September 30, 2018 was primarily due to an increase in the fair value of contingent payments related to the expiration of our option to purchase BioAxone that increased the probability of a \$10.0 million license continuation fee for VX-210 that was ultimately paid in the first quarter of 2018 and the probability that additional milestone and royalty payments related to the BioAxone Agreement would be paid, partially offset by a provision for income taxes related to this increase. The net income attributable to noncontrolling interest in the third quarter and nine months ended September 30, 2017 was primarily related to the \$255.3 million impairment charge related to Parion's pulmonary ENaC platform, a decrease in fair value of the contingent payments payable by us to Parion of \$69.6 million and benefit from income taxes of \$126.2 million related to these charges.

## LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of September 30, 2018 and December 31, 2017:

	September 30, 2018	December 31, 2017	Increase/(Decrease)	
	(in thousands)		\$	%
Cash, cash equivalents and marketable securities	\$3,055,885	\$2,088,666	\$ 967,219	46 %
Working Capital				
Total current assets	3,686,611	2,648,963	1,037,648	39 %
Total current liabilities	992,508	807,260	185,248	23 %
Total working capital	\$2,694,103	\$1,841,703	\$ 852,400	46 %

As of September 30, 2018, we had cash, cash equivalents and marketable securities of \$3.1 billion, which represented an increase of \$967 million from \$2.1 billion as of December 31, 2017. In the nine months ended September 30, 2018, our cash, cash equivalents and marketable securities balance increased due to cash receipts from product sales, an \$85.3 million increase in the fair value of our investment in CRISPR based on an increase in the price of CRISPR's

common stock and \$245.8 million of cash received from issuances of common stock under our employee benefit plans partially offset by cash expenditures to fund our operations and \$207.0 million of cash used to repurchase shares of our common stock. We expect that our future cash flows will be substantially dependent on CF product sales. As of September 30, 2018, total working capital was \$2.7 billion, which represented an increase of \$852 million from \$1.8 billion as of December 31, 2017. The most significant items that increased total working capital in the nine months

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ended September 30, 2018 were \$955.7 million of cash provided by operations, an \$85.3 million increase in the fair value of our investment in CRISPR based on an increase in the price of CRISPR's common stock and \$245.8 million of cash received from issuances of common stock under our employee benefit plans partially offset by \$207.0 million of cash used to repurchase shares of our common stock and expenditures for property and equipment of \$79.8 million as well as other expenditures.

### Sources of Liquidity

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 are receiving cash flows from sales of SYMDEKO, KALYDECO and ORKAMBI in the United States and ORKAMBI and KALYDECO from ex-U.S. markets. We expect approval for SYMKEVI in the European Union in the fourth quarter of 2018. Future net product revenues for ORKAMBI and, if approved, SYMKEVI, from ex-U.S. markets will be dependent on, among other things, the timing of and our ability to complete reimbursement discussions in European countries.

We may borrow up to \$500.0 million pursuant to a revolving credit facility that we entered into in October 2016. We may repay and reborrow amounts under the revolving credit agreement without penalty. Subject to certain conditions, we may request that the borrowing capacity under this credit agreement be increased by an additional \$300.0 million. In the nine months ended September 30, 2018, we received significant proceeds from the issuance of common stock under our employee benefit plans, but the amount and timing of future proceeds from employee benefits plans is uncertain. In the nine months ended September 30, 2018, the value of our strategic investment in CRISPR increased significantly but the future value of this strategic investment is uncertain. Other possible sources of future liquidity include strategic collaborative agreements that include research and/or development funding, commercial debt, public and private offerings of our equity and debt securities, development milestones and royalties on sales of products, software and equipment leases, 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.

### Future Capital Requirements

We incur substantial operating expenses to conduct research and development activities and to operate our organization. We have substantial facility and capital lease obligations, including leases for two buildings in Boston, Massachusetts that continue through 2028 and a lease in San Diego, California that continues through 2034. As of September 30, 2018, we have accrued approximately \$324.3 million from ORKAMBI early access programs in France. We expect we will be required to repay a portion of the collected amounts to the French government based on the difference between the invoiced price of ORKAMBI and the final price for ORKAMBI in France once we conclude our ongoing pricing discussions with the French government. To the extent we borrow amounts under the credit agreement we entered into in October 2016, we would be required to repay any outstanding principal amounts in 2021.

In addition, we have entered into certain collaboration agreements with third parties that include the funding of certain research, development and commercialization efforts with the potential for future milestone and royalty payments by us upon the achievement of pre-established developmental and regulatory targets and/or commercial targets and we may enter into additional business development transactions, including acquisitions, collaborations and equity investments, that require additional capital.

Our board of directors also has authorized a share repurchase program to repurchase up to \$500.0 million of shares of our common stock through December 31, 2019. As of September 30, 2018, \$289.0 million remained available to fund repurchases under the share repurchase program.

We expect that cash flows from SYMDEKO, KALYDECO, ORKAMBI and SYMKEVI, if approved in ex-U.S. markets, together with our current cash, cash equivalents and marketable securities will be sufficient to fund our operations for at least the next twelve months. The adequacy of our available funds to meet our future operating and capital requirements will depend on many factors, including the amounts of future revenues generated by SYMDEKO, KALYDECO, ORKAMBI and SYMKEVI, if approved in ex-U.S. markets, 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.



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### 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, 2017, which was filed with the Securities and Exchange Commission, or SEC, on February 15, 2018. There have been no material changes from the contractual commitments and obligations previously disclosed in that Annual Report on Form 10-K.

### CRITICAL ACCOUNTING POLICIES AND ESTIMATES

Our discussion and analysis of our financial condition and results of operations is based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate. During the nine months ended September 30, 2018, 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, 2017, which we filed with the SEC on February 15, 2018, except as set forth below:

#### Product Revenues - Early Access Programs

We began distributing ORKAMBI in France in 2015 through early access programs and are engaged in ongoing pricing discussions regarding the final price for ORKAMBI in France. We did not recognize any revenues from these product sales through December 31, 2017 based on accounting guidance in effect during this time because the price was not fixed or determinable. As of September 30, 2018 and December 31, 2017, our condensed consolidated balance sheet includes \$324.3 million and \$232.4 million, respectively, under the caption “Early access sales accrual” related to amounts accrued in France as payment for shipments of ORKAMBI under the early access programs. We expect to return the difference between the amounts collected based on the invoiced price and the final price for ORKAMBI in France to the French government.

Upon adopting ASC 606 in the first quarter of 2018, we recorded an \$8.3 million cumulative effect adjustment to “Accumulated deficit” primarily related to shipments of ORKAMBI under early access programs in France. We determined the amount of the adjustment based upon (i) the status of pricing discussions in France upon adoption and (ii) our estimate of the amount of consideration we expect to retain related to ORKAMBI sales in France that occurred on or prior to December 31, 2017 that will not be subject to a significant reversal in amounts recognized. For ORKAMBI sales in France that occurred after December 31, 2017 under the early access programs, we have recognized net product revenues based on the estimate of consideration we expect to retain that will not be subject to a significant reversal in amounts recognized. If our estimate regarding the amounts we will receive for ORKAMBI supplied pursuant to these early access programs changes, we will reflect the effect of the change in estimate in net product revenues in the period in which the change in estimate occurs and will include adjustments to all prior sales of ORKAMBI under the early access programs. Depending on the final price of ORKAMBI and because the current estimate is based on the amount that will not be subject to a significant reversal in amounts recognized this adjustment could be material. For more information regarding the new guidance please see Note B, “Revenue Recognition.”

### RECENT ACCOUNTING PRONOUNCEMENTS

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



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

As part of our investment portfolio, we own financial instruments that are sensitive to market risks. The investment portfolio is used to preserve our capital until it is required to fund operations, including our research and development activities. None of these market risk-sensitive instruments are held for trading purposes. We do not have derivative financial instruments in our investment portfolio.

#### Interest Rate Risk

We invest our cash in a variety of financial instruments, principally securities issued by the U.S. government and its agencies, investment-grade corporate bonds and commercial paper, and money market funds. These investments are denominated in U.S. Dollars. All of our interest-bearing securities are subject to interest rate risk and could decline in value if interest rates fluctuate. Substantially all of our investment portfolio consists of marketable securities with active secondary or resale markets to help ensure portfolio liquidity, and we have implemented guidelines limiting the term-to-maturity of our

investment instruments. Due to the conservative nature of these instruments, we do not believe that we have a material exposure to interest rate risk. If interest rates were to increase or decrease by 1%, the fair value of our investment portfolio would increase or decrease by an immaterial amount.

In October 2016, we entered into a credit agreement. Loans under the credit agreement bear interest, at our option, at either a base rate or a Eurodollar rate, in each case plus an applicable margin. The applicable margin on base rate loans ranges from 0.75% to 1.50% and the applicable margin on Eurodollar loans ranges from 1.75% to 2.50%, in each case, based on our consolidated leverage ratio (as defined in the credit agreement). We do not believe that changes in interest rates related to the credit agreement would have a material effect on our financial statements. As of September 30, 2018, we had no principal or interest outstanding. A portion of our interest expense, net in 2018 will be dependent on whether, and to what extent, we reborrow amounts under the existing facility.

#### Foreign Exchange Market Risk

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Euro and British Pound against the U.S. Dollar. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, payables and accruals and inventories. Both positive and negative effects to our net revenues from international product sales from movements in exchange rates are partially mitigated by the natural, opposite effect that exchange rates have on our international operating costs and expenses.

We have a foreign currency management program with the objective of reducing the effect of exchange rate fluctuations on our operating results and forecasted revenues and expenses denominated in foreign currencies. We currently have cash flow hedges for the Euro, British Pound, Canadian Dollar and Australian Dollar related to a portion of our forecasted product revenues that qualify for hedge accounting treatment under U.S. GAAP. We do not seek hedge accounting treatment for our foreign currency forward contracts related to monetary assets and liabilities that impact our operating results. As of September 30, 2018, we held foreign exchange forward contracts that were designated as cash flow hedges with notional amounts totaling \$467.6 million and had a net fair value of \$14.0 million recorded on our condensed consolidated balance sheet.

Although not predictive in nature, we believe a hypothetical 10% threshold reflects a reasonably possible near-term change in exchange rates. Assuming that the September 30, 2018 exchange rates were to change by a hypothetical 10%, the fair value recorded on our condensed consolidated balance sheet related to our foreign exchange forward contracts that were designated as cash flow hedges as of September 30, 2018 would change by approximately \$46.8 million. However, since these contracts hedge a specific portion of our forecasted product revenues denominated in certain foreign currencies, any change in the fair value of these contracts is recorded in "Accumulated other comprehensive income (loss)" on our condensed consolidated balance sheet and is reclassified to earnings in the same periods during which the underlying product revenues affect earnings. Therefore, any change in the fair value of these contracts that would result from a hypothetical 10% change in exchange rates would be entirely offset by the change in value associated with the underlying hedged product revenues resulting in no impact on our future anticipated earnings and cash flows with respect to the hedged portion of our forecasted product revenues.

### Item 4. Controls and Procedures

#### Evaluation of Disclosure Controls and Procedures

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, have concluded that, based on such evaluation, as of September 30, 2018 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

#### Changes in Internal Controls Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended September 30, 2018 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 Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the SEC on February 15, 2018. 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 or incorporate a number of forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, including statements regarding:

- our expectations regarding the amount of, timing of and trends with respect to our revenues, costs and expenses and other gains and losses, including those related to CF net product revenues, the potential release of the valuation allowance on our deferred income tax assets and the anticipated effect of lease accounting changes that will become effective in 2019;

- our expectations regarding clinical trials, development timelines, timing of our receipt of data from our ongoing and planned clinical trials and regulatory authority filings and submissions for ivacaftor, lumacaftor, tezacaftor, VX-659, VX-445, VX-561, VX-150 and CTX001 and the potential approval of SYMKEVI in the European Union;

- our ability to obtain reimbursement for ORKAMBI in ex-U.S. markets and our ability to otherwise successfully market KALYDECO, ORKAMBI and SYMDEKO/SYMKEVI 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 and drug candidates, including ivacaftor, lumacaftor, tezacaftor, VX-659, VX-445, VX-561, VX-150 and CTX001, 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 for further investigation, clinical trials or potential use as a treatment;

- our plan to continue investing in our research and development programs and our strategy to develop our drug candidates, alone or with third party-collaborators;

- the establishment, development and maintenance of collaborative relationships;

- potential business development activities;

- potential fluctuations in foreign currency exchange rates;

- 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.

Any or all of our forward-looking statements in this Quarterly Report on Form 10-Q may turn out to be wrong. They can be affected by inaccurate assumptions or by known or unknown risks and uncertainties. Many factors mentioned in this Quarterly Report on Form 10-Q will be important in determining future results. Consequently, no forward-looking statement can be guaranteed. Actual future results may vary materially from expected results. We also provide a cautionary discussion of risks and uncertainties under “Risk Factors” in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the SEC on February 15, 2018. These are factors and uncertainties that we think could cause our actual results to differ materially from expected results. Other factors and uncertainties besides those listed there could also adversely affect us.

Without limiting the foregoing, the words “believes,” “anticipates,” “plans,” “intends,” “expects” and similar expressions are intended to identify forward-looking statements. There are a number of factors and uncertainties that could cause actual events or results to differ materially from those indicated by such forward-looking statements, many of which are beyond our control. In addition, the forward-looking statements contained herein represent our estimate only as of the date of this filing and should not be relied upon as representing our estimate as of any subsequent date. While we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so to reflect actual results, changes in assumptions or changes in other factors affecting such forward-looking statements.

## Item 2. Unregistered Sales of Equity Securities and Use of Proceeds Issuer Repurchases of Equity Securities

The table set forth below shows all repurchases of securities by us during the three months ended September 30, 2018:

Period	Total Number of Shares Purchased (1)	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs (2)	Approximate Dollar Value of Shares that May Yet be Purchased Under the Plans or Programs (2)
July 1, 2018 to July 31, 2018	49,836	\$160.51	46,009	372,982,925
August 1, 2018 to August 31, 2018	263,154	\$174.79	259,459	326,985,627
September 1, 2018 to September 30, 2018	213,961	\$177.59	209,664	288,988,241
<b>Total</b>	<b>526,951</b>	<b>\$174.58</b>	<b>515,132</b>	<b>288,988,241</b>

(1) Consists of 515,132 shares repurchased pursuant to our share repurchase program (described in footnote 2 below) at an average price per share of \$178.58 and 11,819 restricted shares repurchased for \$0.01 per share from our employees pursuant to our equity plans. While we have restricted shares that are continuing to vest under our equity plans that are subject to repurchase rights upon termination of service, we have transitioned our equity program to

granting restricted stock units. Unvested restricted stock units are forfeited upon termination of service and do not result in an issuer repurchase that would be reflected in this table.

(2)Our Board of Directors has approved a share repurchase program pursuant to which we are authorized to repurchase up to \$500.0 million of our common stock by December 31, 2019; the program was announced on January 31, 2018. Under the share repurchase program, we are authorized to purchase shares from time to time through open market or privately negotiated transactions and such purchases may be 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. The approximate dollar value of shares that may yet be repurchased is based

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solely on shares that may be repurchased under the share repurchase program and excludes any shares that may be repurchased under our employee equity programs.

Item 6. Exhibits

Exhibit Number	Exhibit Description
10.1	<u>2006 Stock and Option Plan, as amended*</u>
10.2	<u>Amended and Restated 2013 Stock and Option Plan.*</u>
31.1	<u>Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u>
31.2	<u>Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u>
32.1	<u>Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.</u>
101.INS	XBRL Instance
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

\* Management contract, compensatory plan or agreement.

**SIGNATURES**

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

Vertex Pharmaceuticals Incorporated

October 25, 2018 By: /s/ Thomas Graney

Thomas Graney

Senior Vice President and Chief Financial Officer

(principal financial officer and

duly authorized officer)