Vertex Reports Full-Year and Fourth-Quarter 2018 Financial Results

February 5, 2019

- Full-year 2018 CF product revenues of $3.04 billion, a 40% increase compared to $2.17 billion in 2017; fourth-quarter 2018 CF product revenues of $868 million -

- Full-year 2018 GAAP operating income increased 415% to $635 million; non-GAAP operating income increased 97% to $1.11 billion -

- Company provides full-year 2019 financial guidance for total CF product revenues of $3.45 billion to $3.55 billion -

BOSTON--(BUSINESS WIRE)--Feb. 5, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the full year and fourth quarter ended December 31, 2018 and provided full-year 2019 financial guidance.

Fourth-Quarter and Full-Year 2018 Financial Highlights

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2018	2017	% Change	2018	2017	% Change
	(in millions, except per share and percentage data)					
TOTAL CF product revenues, net	$ 868	$ 621	40%	$ 3,038	$ 2,165	40%
GAAP						
Operating income	$ 128	$ 126	2%	$ 635	$ 123	415%
Benefit from (provision for) income taxes ^	$ 1,493	$ (10)		$ 1,487	$ 107	
Net income	$ 1,551	$ 101		$ 2,097	$ 263	
Net income per share - diluted	$ 5.97	$ 0.39		$ 8.09	$ 1.04	
Non-GAAP						
Operating income	$ 348	$ 184	90%	$ 1,112	$ 564	97%
Provision for income taxes	$ (3)	$ (13)		$ (16)	$ (7)	
Net income	$ 337	$ 158	113%	$ 1,059	$ 495	114%
Net income per share - diluted	$ 1.30	$ 0.61	113%	$ 4.08	$ 1.95	109%

^ GAAP net income includes a benefit from income taxes of approximately $1.5 billion in the fourth quarter and full year ended December 31, 2018 due to the release of Vertex's valuation allowance on the majority of its net operating losses and other deferred tax assets.

"Our achievements in 2018 were marked by a significant increase in the number of CF patients being treated with our approved medicines and by remarkable progress advancing our two triple combination regimens through late-stage development. We remain on track to submit a New Drug Application for a triple combination regimen no later than mid-2019," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Beyond CF, we have made tremendous advances with our research and development pipeline. We initiated clinical development for potential medicines to treat alpha-1 antitrypsin deficiency, sickle cell disease and beta thalassemia, provided multiple positive data readouts in pain, and progressed several other drug candidates into late preclinical development. Through our continued progress in treating CF and other serious diseases, we believe Vertex will continue to create revenue and earnings growth in 2019 and beyond."

Full-Year 2018 CF Net Product Revenues

	Twelve Months Ended December 31,	
	2018	2017
	(in millions)	
TOTAL CF product revenues, net	$ 3,038	$ 2,165
KALYDECO product revenues, net	$ 1,008	$ 845
ORKAMBI product revenues, net	$ 1,262	$ 1,321
SYMDEKO product revenues, net	$ 769	$ —

Total CF net product revenues increased 40% compared to the full-year of 2017, primarily driven by the rapid uptake of SYMDEKO in the U.S. and the full year impact of KALYDECO label expansions.

Full-Year 2018 R&D and SG&A Expenses

	Twelve Months Ended December 31,	
	2018	2017
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 1,974	$ 1,821
GAAP R&D expense	$ 1,416	$ 1,325
GAAP SG&A expense	$ 558	$ 496
Combined Non-GAAP R&D and SG&A expenses	$ 1,527	$ 1,335
Non-GAAP R&D expense	$ 1,089	$ 959
Non-GAAP SG&A expense	$ 437	$ 375

Combined GAAP and non-GAAP R&D and SG&A expenses increased compared to the full year of 2017, primarily due to the advancement of the company's portfolio of triple combination regimens for CF and investments to support the treatment of CF globally.

Full-Year 2018 Income Taxes

Vertex released the valuation allowance on the majority of its deferred tax assets in the fourth quarter of 2018 based on, among other things, its achievement of cumulative profitability over the last several years and its expectations regarding future profitability. The one-time release of this valuation allowance resulted in Vertex recording a GAAP income tax benefit of $1.5 billion in 2018 compared to a benefit of $107.3 million in 2017.

Vertex reported non-GAAP income taxexpenseof $16.4 million in 2018, which excludes the release of the valuation allowance, compared to income tax expense of $6.8 million in 2017.

Full-Year 2018 Net Income and Cash Position

	Twelve Months Ended December 31,	
	2018	2017
	(in millions)	
GAAP net income	$ 2,097	$ 263
Non-GAAP net income	$ 1,059	$ 495

	As of December 31,	
	2018	2017
	(in millions)	
Cash, cash equivalents and marketable securities	$ 3,168	$ 2,089

GAAP net income increased compared to the full year of 2017 largely driven by the release of the tax valuation allowance and strong growth in total CF product revenues.

Non-GAAP net income increased 114% compared to the full year of 2017 largely driven by the strong growth in total CF product revenues.

Cash, cash equivalents and marketable securities as of December 31, 2018 were approximately $3.2 billion, an increase of approximately $1.1 billion compared to $2.1 billion as of December 31, 2017.

Fourth-Quarter 2018 CF Net Product Revenues

	Three Months Ended December 31,	
	2018	2017
	(in millions)	
TOTAL CF product revenues, net	$ 868	$ 621
KALYDECO product revenues, net	$ 259	$ 256
ORKAMBI product revenues, net	$ 315	$ 365
SYMDEKO product revenues, net	$ 294	$ —

Total CF net product revenues increased 40% compared to the fourth quarter of 2017, primarily driven by the rapid uptake of SYMDEKO in the U.S.

Fourth-Quarter 2018 R&D and SG&A Expenses

	Three Months Ended December 31,	
	2018	2017
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 591	$ 441
GAAP R&D expense	$ 438	$ 307
GAAP SG&A expense	$ 153	$ 135
Combined Non-GAAP R&D and SG&A expenses	$ 400	$ 355
Non-GAAP R&D expense	$ 275	$ 249
Non-GAAP SG&A expense	$ 125	$ 106

Combined GAAP R&D and SG&A expenses increased compared to the fourth quarter of 2017 primarily due to $111.9 million of expenses related to strategic licensing agreements entered into in the fourth quarter of 2018 and the advancement of the company's portfolio of triple combination regimens for CF and investments to support the treatment of CF globally.

Combined non-GAAP R&D and SG&A expenses increased compared to the fourth quarter of 2017 primarily due to the advancement of the company's portfolio of triple combination regimens for CF and investments to support the treatment of CF globally.

Fourth-Quarter 2018 Income Taxes

Vertex released the valuation allowance on the majority of its deferred tax assets in the fourth quarter of 2018. The one-time release of this valuation allowance resulted in Vertex recording a non-cash GAAP income tax benefit of $1.5 billion in the fourth quarter of 2018 compared to income tax expense of $10.3 million in the fourth quarter of 2017.

Vertex reported non-GAAP income tax expense of $3.0 million in the fourth quarter of 2018, which excludes the release of the valuation allowance,

compared to income tax expense of $12.7 million in the fourth quarter of 2017.

Fourth-Quarter 2018 Net Income

	Three Months Ended December 31,	
	2018	2017
	(in millions)	
GAAP net income	$ 1,551	$ 101
Non-GAAP net income	$ 337	$ 158

GAAP net income increased compared to the fourth quarter of 2017 largely driven by the release of the tax valuation allowance and strong growth in total CF product revenues, partially offset by upfront payments on strategic licensing agreements.

Non-GAAP net income increased 113% compared to the fourth quarter of 2017 largely driven by the strong growth in total CF product revenues.

Full-Year 2019 Financial Guidance

Vertex today provided its full-year 2019 guidance as follows:

	FY 2019
TOTAL CF product revenues	$ 3.45 to 3.55 billion
Combined GAAP R&D and SG&A expenses	$ 2.00 to 2.15 billion
Combined Non-GAAP R&D and SG&A expenses	$ 1.65 to 1.70 billion
GAAP and Non-GAAP effective tax rate	21% - 22%

The company's total CF product revenue growth in 2019 is expected to be driven primarily by the full-year impact of the SYMDEKO launch, recently completed reimbursement agreements and label expansions for the company's CF medicines. The company's full-year 2019 revenue guidance reflects only markets where its CF medicines are currently reimbursed.

The company's combined GAAP and non-GAAP R&D and SG&A expense guidance reflects CF development efforts, incremental investment to support the potential launch of a triple combination regimen and investment to support the expansion of Vertex's pipeline into new disease areas.

Based on the release of the company's valuation allowance in the fourth quarter of 2018, Vertex will also begin recording a tax provision in 2019 and expects its full-year GAAP and non-GAAP tax rate to be between 21% and 22%. The vast majority of this tax provision will be a non-cash expense until the company fully utilizes its net operating losses.

Business Highlights

TRIPLE COMBINATION REGIMENS

Bringing triple combination regimens to people with CF as quickly as possible: On November 27, 2018, Vertex announced positive data from two Phase 3 studies evaluating VX-659 in triple combination with tezacaftor and ivacaftor in people with CF who have one *F508del* mutation and one minimal function mutation and in people who have two *F508del* mutations. Data are expected in the first quarter of 2019 from the two Phase 3 studies evaluating VX-445 in triple combination with tezacaftor and ivacaftor in people with CF who have one *F508del* mutation and one minimal function mutation and in people who have two *F508del* mutations.

Vertex will evaluate data from both the VX-659 and VX-445 triple combination programs to choose the best regimen to submit for potential regulatory approval. Together these data are expected to provide the basis for submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) no later than mid-2019.

APPROVED CF MEDICINES

SYMKEVI in the European Union: On November 1, 2018, Vertex announced that the European Commission granted Marketing Authorization for SYMKEVI (tezacaftor/ivacaftor) in a combination regimen with ivacaftor (KALYDECO) for the treatment of people with CF ages 12 and older who either have two copies of the *F508del* mutation or who have one copy of the *F508del* mutation and a copy of one of the following 14 mutations in which the CFTR protein shows residual activity: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G,* and *3849+10kbC→T*.

SYMKEVI is now reimbursed and available to all eligible patients in Germany, Ireland and Austria.

Establishing long-term reimbursement outside of the U.S.: During 2018, Vertex established important reimbursement agreements in several countries, including Australia, Sweden and Denmark. These reimbursement agreements allow CF patients access to medicines that treat the

underlying cause of their disease for the first time and provide a pathway to access and rapid reimbursement for future CF medicines. The company continues to work toward establishing pricing and reimbursement agreements in other countries outside of the U.S. to ensure all eligible patients have access to current and future CF medicines from Vertex as quickly as possible.

SYMDEKO receives rare pediatric disease priority review voucher: On January 29, 2019, the FDA granted Vertex a rare pediatric disease priority review voucher based on the February 12, 2018 U.S. approval of SYMDEKO for the treatment of people with CF ages 12 and older who have two copies of the *F508del* mutation or who have at least one mutation that is responsive to tezacaftor/ivacaftor. A rare pediatric disease priority review voucher entitles the voucher holder to priority review of other human drug applications and is not limited to applications for drugs to treat rare pediatric diseases. Rare pediatric disease priority review vouchers can be transferred, including by sale, from one sponsor to another.

Treating patients at younger ages with CFTR modulators: The company continues to make significant progress toward gaining approval for its CF medicines to be used earlier in the course of disease progression. Recent highlights include:

- **KALYDECO** was approved for children with CF ages 12 to <24 months in the EU in November 2018 and in Canada in January 2019.
- **ORKAMBI** was approved for children with CF ages 2 to 5 years old in Canada in December 2018 and in the EU in January 2019.
- In late 2018, sNDAs were submitted to the FDA for **ivacaftor** in infants ages 6 to <12 months and for **tezacaftor/ivacaftor** in children ages 6 through 11.

LATE-STAGE RESEARCH & CLINICAL DEVELOPMENT

Vertex continues to invest to discover and develop transformative medicines in other serious diseases. The company has a portfolio of potential medicines across a range of diseases, including:

Sickle Cell Disease & Beta-Thalassemia: Vertex and its partner CRISPR Therapeutics are developing the autologous gene-edited hematopoietic stem cell therapy CTX001 for the treatment of sickle cell disease and beta-thalassemia. On January 4, 2019, Vertex and CRISPR Therapeutics announced that the FDA granted Fast Track Designation for CTX001 for the treatment of sickle cell disease. Phase 1/2 trials in sickle cell disease and beta-thalassemia are currently underway.

***Selective NaV1.8 Inhibitors for the Treatment of Pain*:** Data are expected in the first half of 2019 from a Phase 2b dose-ranging study evaluating the NaV1.8 inhibitor VX-150 using an oral formulation in patients with acute pain following bunionectomy surgery. The study is designed to evaluate multiple oral doses of VX-150 to potentially support pivotal development in acute pain.

In December 2018, the company announced positive results from a Phase 2 study evaluating VX-150 for the treatment of pain in people with small fiber neuropathy. This study is the third positive proof-of-concept study of VX-150 and further validates the potential role of NaV1.8 inhibition in the treatment of pain.

Vertex is advancing multiple pain molecules through late-stage preclinical development and plans to initiate clinical development with the first of these molecules in 2019.

***Alpha-1 Antitrypsin (AAT) Program*:** In December 2018, Vertex initiated clinical development of its first potential medicine for alpha-1 antitrypsin deficiency, a genetic disorder that is caused by mutations in a single gene that result in life-shortening systemic complications, primarily in the lung and liver.

The company is advancing multiple other small molecule correctors of alpha-1 antitrypsin deficiency through preclinical development.

***Focal Segmental Glomerulosclerosis (FSGS)*:** Vertex has multiple candidates in preclinical development for the treatment of FSGS. The company's goal is to advance its first potential medicine for this disease into clinical development in 2019. FSGS is a rare disease that attacks the kidney's filtering units causing serious scarring that leads to permanent kidney damage. FSGS is a leading cause of nephrotic syndrome in children and kidney failure in adults.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial results and guidance exclude from Vertex's pre-tax net income (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements and asset acquisitions, (iii) revenues and expenses related to consolidated variable interest entities, including asset impairment charges and the effects of the deconsolidation of variable interest entities in 2017 and 2018 and (iv) other adjustments, including gains or losses related to the fair value of the company's strategic investments. The company's non-GAAP financial results also exclude from its provision for or benefit from income taxes (i) the estimated tax impact related to its non-GAAP adjustments to pre-tax net income described above as well as (ii) non-operating tax adjustments, which are not associated with Vertex's normal, recurring operations and include the release of the company's valuation allowance on the majority of its net operating losses and other deferred tax assets in the fourth quarter of 2018. These results are provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally and to manage the company's business and to evaluate its performance. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. The company provides guidance regarding product revenues in accordance with GAAP and provides guidance regarding combined research and development and sales, general, and administrative expenses and its anticipated income taxes as a percentage of pre-tax net income on both a GAAP and a non-GAAP basis. The guidance regarding GAAP research and development expenses and sales, general and administrative expenses does not include estimates associated with any potential future business development activities. The guidance regarding the GAAP effective tax rate is based on currently available information and could be lower than the current guidance of 21 to 22% due to actual value of equity exercises by employees in 2019, geographic mix of business and business development activities. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

Vertex Pharmaceuticals Incorporated
Full-Year and Fourth-Quarter Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
Revenues:				
Product revenues, net	$ 868,173	$ 621,228	$ 3,038,325	$ 2,165,480
Collaboration and royalty revenues (Note 1)	1,933	30,406	9,272	323,172
Total revenues	870,106	651,634	3,047,597	2,488,652
Costs and expenses:				
Cost of sales	122,289	84,052	409,539	275,119
Research and development expenses (Note 2)	437,881	306,664	1,416,476	1,324,625
Sales, general and administrative expenses	153,210	134,794	557,616	496,079
Restructuring expenses (income)	4	387	(184)	14,246
Intangible asset impairment charges (Note 3)	29,000	—	29,000	255,340
Total costs and expenses	742,384	525,897	2,412,447	2,365,409
Income from operations	127,722	125,737	635,150	123,243
Interest expense, net	(4,773)	(12,547)	(34,119)	(57,550)
Other expense, net (Note 4)(Note 5)	(90,452)	(748)	(790)	(81,382)
Income (loss) from operations before (benefit from) provision for income taxes	32,497	112,442	600,241	(15,689)
(Benefit from) provision for income taxes (Note 5)(Note 6)	(1,492,599)	10,257	(1,486,862)	(107,324)
Net income	1,525,096	102,185	2,087,103	91,635
Loss (income) attributable to noncontrolling interest (Note 5)	25,431	(1,501)	9,793	171,849
Net income attributable to Vertex	$ 1,550,527	$ 100,684	$ 2,096,896	$ 263,484
Amounts per share attributable to Vertex common shareholders:				
Net income:				
Basic	$ 6.08	$ 0.40	$ 8.24	$ 1.06
Diluted	$ 5.97	$ 0.39	$ 8.09	$ 1.04
Shares used in per share calculations:				
Basic	254,868	251,557	254,292	248,858
Diluted	259,812	256,804	259,185	253,225

Reconciliation of GAAP to Non-GAAP Net Income
Full-Year and Fourth-Quarter Results
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
GAAP net income attributable to Vertex	$ 1,550,527	$ 100,684	$ 2,096,896	$ 263,484
Stock-based compensation expense	78,943	75,402	325,047	290,736
Collaborative and transaction revenues and expenses (Note 7)	106,570	(19,177)	134,447	(76,607)
Other adjustments (Note 8)	86,702	941	2,005	16,947
Total non-GAAP adjustments to pre-tax net income attributable to Vertex	272,215	57,166	461,499	231,076
Non-operating tax adjustments (Note 9)	(1,485,873)	—	(1,499,588)	—
Non-GAAP net income attributable to Vertex	$ 336,869	$ 157,850	$ 1,058,807	$ 494,560
Amounts per diluted share attributable to Vertex common shareholders:				
Net income:				

GAAP	$ 5.97	$ 0.39	$ 8.09	$ 1.04
Non-GAAP	$ 1.30	$ 0.61	$ 4.08	$ 1.95
Shares used in diluted per share calculations:				
GAAP	259,812	256,804	259,185	253,225
Non-GAAP	259,812	256,804	259,185	253,225

Reconciliation of GAAP to Non-GAAP Revenues and Expenses
Full-Year and Fourth-Quarter Results
(in thousands)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
GAAP total revenues	$ 870,106	$ 651,634	$ 3,047,597	$ 2,488,652
Collaborative and transaction revenues (Note 7)	(663)	(29,006)	(4,203)	(314,981)
Non-GAAP total revenues	$ 869,443	$ 622,628	$ 3,043,394	$ 2,173,671

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
GAAP cost of sales	$ 122,289	$ 84,052	$ 409,539	$ 275,119
Stock-based compensation expense (Note 10)	(1,280)	—	(4,543)	—
Non-GAAP cost of sales	$ 121,009	$ 84,052	$ 404,996	$ 275,119
GAAP research and development expenses	$ 437,881	$ 306,664	$ 1,416,476	$ 1,324,625
Stock-based compensation expense	(50,094)	(47,045)	(203,112)	(181,900)
Collaborative and transaction expenses (Note 7)	(111,925)	(10,249)	(120,004)	(182,695)
Other adjustments (Note 8)	(877)	(136)	(4,005)	(544)
Non-GAAP research and development expenses	$ 274,985	$ 249,234	$ 1,089,355	$ 959,486
GAAP sales, general and administrative expenses	$ 153,210	$ 134,794	$ 557,616	$ 496,079
Stock-based compensation expense	(27,569)	(28,357)	(117,392)	(108,836)
Collaborative and transaction expenses (Note 7)	(438)	(515)	(2,142)	(9,775)
Other adjustments (Note 8)	(162)	(418)	(742)	(2,157)
Non-GAAP sales, general and administrative expenses	$ 125,041	$ 105,504	$ 437,340	$ 375,311
Combined non-GAAP R&D and SG&A expenses	$ 400,026	$ 354,738	$ 1,526,695	$ 1,334,797

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
GAAP interest expense, net and other expense, net	$ (95,225)	$ (13,295)	$ (34,909)	$ (138,932)
Collaborative and transaction expenses (Note 7)	1,037	(4)	965	76,503
Other adjustments (Note 8)	85,659	—	(2,558)	—
Non-GAAP interest expense, net and other expense, net	$ (8,529)	$ (13,299)	$ (36,502)	$ (62,429)
GAAP (benefit from) provision for income taxes	$ (1,492,599)	$ 10,257	$ (1,486,862)	$ (107,324)
Estimated income taxes related to non-GAAP adjustments to pre-tax income (Note 11)	9,736	2,432	3,668	114,090
Non-operating tax adjustments (Note 9)	1,485,873	—	1,499,588	—
Non-GAAP provision for income taxes	$ 3,010	$ 12,689	$ 16,394	$ 6,766

Condensed Consolidated Balance Sheets Data
(in thousands)
(unaudited)

	December 31, 2018	December 31, 2017
Assets		
Cash, cash equivalents and marketable securities	$ 3,168,242	$ 2,088,666
Accounts receivable, net	409,688	281,343
Inventories	124,360	111,830
Property and equipment, net	812,005	789,437
Intangible assets and goodwill (Note 3)	50,384	79,384
Deferred tax asset (Note 6)	1,499,672	834
Other assets	181,547	194,520
Total assets	$ 6,245,898	$ 3,546,014
Liabilities and Shareholders' Equity		
Accounts payable and accruals	$ 715,482	$ 517,955
Other liabilities	528,050	421,842
Construction financing lease obligation	567,163	563,911
Shareholders' equity	4,435,203	2,042,306
Total liabilities and shareholders' equity	$ 6,245,898	$ 3,546,014
Common shares outstanding	255,172	253,253

Note 1: In 2017, collaborative and royalty revenues were primarily attributable to (i) a $25.0 million milestone earned from the company's collaboration with Janssen Pharmaceuticals, Inc. in the fourth quarter of 2017, (ii) a $230.0 million upfront payment earned from the company's collaboration with Merck KGaA, Darmstadt, Germany recorded in the first quarter of 2017 and (iii) a total of $40.0 million that Parion Sciences Inc., a company that Vertex consolidated as a variable interest entity ("VIE"), earned from a collaboration agreement with a third party during the second and third quarters of 2017.

Note 2: In the three and twelve months ended December 31, 2018, the company's research and development expenses include $111.9 million of expenses related to strategic licensing agreements entered into in the fourth quarter of 2018, including license agreements with Merck KGaA, Darmstadt, Germany and Arbor Biotechnologies, Inc. In 2017, the company's research and development expenses included $160.0 million that it paid to acquire VX-561 from Concert Pharmaceuticals, Inc. in the third quarter of 2017.

Note 3: In the three and twelve months ended December 31, 2018, the company recorded a $29.0 million intangible asset impairment charge related to VX-210 that it licensed from BioAxone Biosciences, Inc. In the twelve months ended December 31, 2017, the company recorded a $255.3 million intangible asset impairment charge related to Parion's pulmonary ENaC platform.

Note 4: In accordance with ASU No. 2016-01, *Recognition and Measurement of Financial Assets and Financial Liabilities*, which became effective on January 1, 2018, the company recorded a loss of $85.7 million in the three months ended December 31, 2018 and a net gain of $2.6 million in the twelve months ended December 31 2018 to "Other expense, net", related to changes in the fair value of the company's strategic investments. Prior to the adoption of ASU 2016-01, changes in the fair value of the company's strategic investments were recorded to equity on the company's condensed consolidated balance sheets until the related gains and losses were realized; therefore, there were no comparable amounts in the three and twelve months ended December 31, 2017.

Note 5: In 2017 and 2018, the company consolidated the financial statements of BioAxone and Parion as VIEs for portions or all of the three and twelve months ended December 31, 2018 and 2017 because Vertex had licensed the rights to develop these collaborator's most significant intellectual property asset. Each reporting period Vertex estimated the fair value of the contingent payments payable by Vertex to BioAxone and Parion. Any increase in the fair value of these contingent payments resulted in a decrease in net income attributable to Vertex on a dollar-for-dollar basis. The fair value of contingent payments was evaluated each quarter and any change in the fair value was reflected in the company's statement of operations.

The company deconsolidated Parion as of September 30, 2017 and BioAxone as of December 31, 2018. The company's impairment of Parion's ENaC platform and subsequent deconsolidation of Parion in the third quarter of 2017 resulted in the impairment charge of $255.3 million and a total benefit from income taxes of $126.2 million attributable to noncontrolling interest. The total impact of the Parion transaction on a GAAP basis was a $198.7 million loss attributable to noncontrolling interest and a $7.1 million loss attributable to Vertex and had no impact on Vertex’s non-GAAP net income in the third quarter of 2017. The net impact attributable to Vertex resulting from the deconsolidation of BioAxone in the three and twelve months ended December 31, 2018 was not material.

Note 6: In the three and twelve months ended December 31, 2018, the company recorded a non-cash benefit from income taxes of approximately $1.5 billion related to the release of its valuation allowance on the majority of its net operating losses and other deferred tax assets based on, among other things, its achievement of cumulative profitability over the last several years and its expectations regarding future profitability. As a result, the company recorded deferred tax assets of $1.5 billion on its consolidated balance sheet as of December 31, 2018, which were previously subject to the valuation allowance. Starting in the first quarter of 2019, the company will record a provision for income taxes on its pre-tax net income using an estimated effective tax rate that is expected to approximate statutory rates. This provision will include a significant non-cash charge due to the company's ability to offset its pre-tax net income against previously accumulated net operating losses. The company expects its cash paid for income taxes to increase significantly once all of its net operating losses have been utilized to offset its pre-tax net income. As of December 31, 2018, the

company's federal net operating losses and credits were approximately $4.5 billion.

Note 7: "Collaborative and transaction revenues and expenses" in the three and twelve months ended December 31, 2018 were primarily related to the upfront payments described in Note 2 as well an increase of $5.4 million and a decrease of $17.7 million, respectively, in the fair value of contingent payments payable by Vertex to BioAxone. "Collaborative and transaction revenues and expenses" in the three and twelve months ended December 31, 2017 were primarily related to (i) the collaborative revenues described in Note 1, (ii) the Concert transaction described in Note 2, (iii) a $62.6 million decrease in the fair value of contingent payments payable by Vertex to its VIEs for the full-year and (iv) a charge attributable to Parion that was recorded to "Other Expense, Net" upon deconsolidation in the third quarter of 2017.

Note 8: In the three and twelve months ended December 31, 2018, "Other adjustments" primarily consisted of the changes in the fair value of the company's strategic investments described in Note 4. In the three and twelve months ended December 31, 2017, "Other adjustments" primarily consisted of restructuring charges related to the company's decision to consolidate its research activities into its Boston, Milton Park and San Diego locations and to close its research site in Canada.

Note 9: "Non-operating tax adjustments" includes portions of the company's provision for income taxes that are not associated with the company's normal, recurring operations. In the three and twelve months ended December 31, 2018, "Non-operating tax adjustments" includes the non-cash benefit from income taxes related to the release of the company's valuation allowance on the majority of its net operating losses and other deferred tax assets described in Note 6. Additionally, in the three months ended December 31, 2018, the company recorded a provision for income taxes related to stock-based compensation of $13.7 million on a GAAP basis representing the reversal of the net benefit from income taxes it recorded in the nine months ended September 30, 2018 related to stock-based compensation. Accordingly, these discrete items related to stock-based compensation had no effect on the company's GAAP annual provision for income taxes and the company excluded this amount from its Non-GAAP measures for the three months ended December 31, 2018.

Note 10: In the three and twelve months ended December 31, 2018, "Cost of sales" included $1.3 million and $4.5 million, respectively, in stock-based compensation expense. In the three and twelve months ended December 31, 2017, “Cost of sales” included $0.8 million and $2.5 million, respectively, in stock-based compensation expense. Beginning with the first quarter of 2018, the company began adjusting for the stock-based compensation expense recorded in “Cost of sales” in its reconciliation of "Non-GAAP net income attributable to Vertex" and "Non-GAAP cost of sales". In its Non-GAAP reconciliation, the company is not adjusting for the stock-based compensation expense recorded in “Cost of sales” for the three and twelve months ended December 31, 2017.

Note 11: In the three and twelve months ended December 31, 2018 and 2017, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" related to the company's VIEs' income taxes. In the three and twelve months ended December 31, 2017, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" primarily related to the benefit from income taxes recorded as a result of the impairment and subsequent deconsolidation of Parion described in Note 3.

KALYDECO® (ivacaftor) U.S. INDICATION AND IMPORTANT SAFETY INFORMATION

KALYDECO (ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 12 months and older who have at least one mutation in their CF gene that is responsive to KALYDECO. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if KALYDECO is safe and effective in children under 12 months of age.

Patients should not take KALYDECO if they are taking certain medicines or herbal supplements such as: the antibiotics rifampin or rifabutin; seizure medications such as phenobarbital, carbamazepine, or phenytoin; or St. John's wort.

Before taking KALYDECO, patients should tell their doctor if they: have liver or kidney problems; drink grapefruit juice, or eat grapefruit or Seville oranges; are pregnant or plan to become pregnant because it is not known if KALYDECO will harm an unborn baby; and are breastfeeding or planning to breastfeed because is not known if KALYDECO passes into breast milk.

KALYDECO may affect the way other medicines work, and other medicines may affect how KALYDECO works. Therefore the dose of KALYDECO may need to be adjusted when taken with certain medications. Patients should especially tell their doctor if they take antifungal medications such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

KALYDECO can cause dizziness in some people who take it. Patients should not drive a car, use machinery, or do anything that needs them to be alert until they know how KALYDECO affects them. **Patients should avoid** food containing grapefruit or Seville oranges while taking KALYDECO.

KALYDECO can cause serious side effects including:

High liver enzymes in the blood have been reported in patients receiving KALYDECO. The patient's doctor will do blood tests to check their liver before starting KALYDECO, every 3 months during the first year of taking KALYDECO, and every year while taking KALYDECO. For patients who have had high liver enzymes in the past, the doctor may do blood tests to check the liver more often. Patients should call their doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of their skin or the white part of their eyes; loss of appetite; nausea or vomiting; or dark, amber-colored urine.

Abnormality of the eye lens (cataract) has been noted in some children and adolescents receiving KALYDECO. The patient's doctor should perform eye examinations prior to and during treatment with KALYDECO to look for cataracts. The most common side effects include headache; upper respiratory tract infection (common cold), which includes sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO.

Please click here to see the full U.S. Prescribing Information for KALYDECO.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR ORKAMBI® (lumacaftor/ivacaftor)

ORKAMBI is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have two copies of the

F508del mutation (*F508del/F508del*) in their *CFTR* gene. ORKAMBI should only be used in these patients. It is not known if ORKAMBI is safe and effective in children under 2 years of age.

Patients should not take ORKAMBI if they are taking certain medicines or herbal supplements, such as: the antibiotics rifampin or rifabutin; the seizure medicines phenobarbital, carbamazepine, or phenytoin; the sedatives and anti-anxiety medicines triazolam or midazolam; the immunosuppressant medicines cyclosporine, everolimus, sirolimus, or tacrolimus; or St. John's wort.

Before taking ORKAMBI, patients should tell their doctor about all their medical conditions, including if they: have or have had liver problems; have kidney problems; have had an organ transplant; or are using birth control. Hormonal contraceptives, including oral, injectable, transdermal, or implantable forms should not be used as a method of birth control when taking ORKAMBI. Patients should tell their doctor if they are pregnant or plan to become pregnant (it is unknown if ORKAMBI will harm the unborn baby) or if they are breastfeeding or planning to breastfeed (it is unknown if ORKAMBI passes into breast milk).

ORKAMBI may affect the way other medicines work and other medicines may affect how ORKAMBI works. Therefore, the dose of ORKAMBI or other medicines may need to be adjusted when taken together. Patients should especially tell their doctor if they take: antifungal medicines such as ketoconazole, itraconazole, posaconazole, or voriconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

When taking ORKAMBI, patients should tell their doctor if they stop ORKAMBI for more than 1 week as the doctor may need to change the dose of ORKAMBI or other medicines the patient is taking.

ORKAMBI can cause serious side effects, including:

Worsening of liver function in people with severe liver disease. The worsening of liver function can be serious or cause death. Patients should talk to their doctor if they have been told they have liver disease as their doctor may need to adjust the dose of ORKAMBI.

High liver enzymes in the blood, which can be a sign of liver injury**.** The patient's doctor will do blood tests to check their liver before they start ORKAMBI, every three months during the first year of taking ORKAMBI, and annually thereafter. The patient should call the doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of the skin or the white part of the eyes; loss of appetite; nausea or vomiting; dark, amber-colored urine; or confusion.

Breathing problems such as shortness of breath or chest tightness in patients when starting ORKAMBI, especially in patients who have poor lung function. If a patient has poor lung function, their doctor may monitor them more closely when starting ORKAMBI.

An increase in blood pressure in some people receiving ORKAMBI. The patient's doctor should monitor their blood pressure during treatment with ORKAMBI.

Abnormality of the eye lens (cataract) in some children and adolescents receiving ORKAMBI. For children and adolescents, the patient's doctor should perform eye examinations before and during treatment with ORKAMBI to look for cataracts.

The most common side effects of ORKAMBI include: breathing problems, such as shortness of breath and chest tightness; nausea; diarrhea; fatigue; increase in a certain blood enzyme called creatinine phosphokinase; rash; gas; common cold, including sore throat, stuffy or runny nose; flu or flu-like symptoms; and irregular, missed, or abnormal periods (menses) and increase in the amount of menstrual bleeding.

Side effects seen in children are similar to those seen in adults and adolescents. Additional common side effects seen in children include: cough with sputum, stuffy nose, headache, stomach pain, and increase in sputum.

Please click here to see the full Prescribing Information for ORKAMBI.

U.S INDICATION AND IMPORTANT SAFETY INFORMATION FOR SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) tablets

SYMDEKO is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who have two copies of the *F508del* mutation, or who have at least one mutation in the CF gene that is responsive to treatment with SYMDEKO. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if SYMDEKO is safe and effective in children under 12 years of age.

Patients should not take SYMDEKO if they take certain medicines or herbal supplements such as: the antibiotics rifampin or rifabutin; seizure medicines such as phenobarbital, carbamazepine, or phenytoin; St. John's wort.

Before taking SYMDEKO, patients should tell their doctor if they: have or have had liver problems; have kidney problems; are pregnant or plan to become pregnant because it is not known if SYMDEKO will harm an unborn baby; are breastfeeding or planning to breastfeed because it is not known if SYMDEKO passes into breast milk.

SYMDEKO may affect the way other medicines work, and other medicines may affect how SYMDEKO works. Therefore, the dose of SYMDEKO may need to be adjusted when taken with certain medicines. Patients should especially tell their doctor if they take antifungal medicines such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

SYMDEKO may cause dizziness in some people who take it. Patients should not drive a car, use machinery, or do anything that requires alertness until they know how SYMDEKO affects them.

Patients should avoid food or drink that contains grapefruit or Seville oranges while they are taking SYMDEKO.

SYMDEKO can cause serious side effects, including:

High liver enzymes in the blood, which have been reported in people treated with SYMDEKO or treated with ivacaftor alone. The patient's doctor will do blood tests to check their liver before they start SYMDEKO, every 3 months during the first year of taking SYMDEKO, and every year while taking SYMDEKO. Patients should call their doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of the skin or the white part of the eyes; loss of appetite; nausea or vomiting; dark, amber-colored urine.

Abnormality of the eye lens (cataract) in some children and adolescents treated with SYMDEKO or with ivacaftor alone. If the patient is a child or adolescent, their doctor should perform eye examinations before and during treatment with SYMDEKO to look for cataracts.

The most common side effects of SYMDEKO include headache, nausea, sinus congestion, and dizziness.

These are not all the possible side effects of SYMDEKO.

Please click here to see the full U.S. Prescribing Information for SYMDEKO.

About Vertex
Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston'sInnovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for nine years in a row. For additional information and the latest updates from the company, please visit www.vrtx.com.

Special Note Regarding Forward-Looking Statements
This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Leiden's statements in this press release, the information provided regarding future financial performance, including in the section captioned "2019 Financial Guidance" and statements regarding (i) the timing and expected outcome of regulatory applications, including NDAs and MAAs and (ii) the development plan and timelines for our product development candidates, including our next-generation triple combination regimens, CTX001, VX-150 and the company's AAT correctors. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2019 CF net product revenues, expenses and effective tax rates may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast
The company will host a conference call and webcast today at 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

(VRTX-E)

View source version on businesswire.com: https://www.businesswire.com/news/home/20190205005917/en/

Source: Vertex Pharmaceuticals Incorporated

Vertex Contacts:
Investors:
Michael Partridge, 617-341-6108
or
Eric Rojas, 617-961-7205
or
Zach Barber, 617-341-6470

Media:
617-341-6992
mediainfo@vrtx.com