January 31, 2018

Vertex Reports Full-Year and Fourth-Quarter 2017 Financial Results

-Total 2017 CF product revenues of $2.17 billion, a 29% increase compared to $1.68 billion in 2016; 2017 KALYDECO revenues of $845 million and 2017 ORKAMBI revenues of $1.32 billion-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the full year and fourth quarter ended December 31, 2017.

Key financial results include:

	Three Months Ended December 31,		%	Twelve Months Ended December 31,		%
	2017	2016	Change	2017	2016	Change
	(in millions, except per share and percentage data)					
ORKAMBI product revenues, net	$ 365	$ 277	32%	$ 1,321	$ 980	35%
KALYDECO product revenues, net	$ 256	$ 177	44%	$ 845	$ 703	20%
TOTAL CF product revenues, net	$ 621	$ 454	37%	$ 2,165	$ 1,683	29%
GAAP Collaborative revenues	$ 29	$ 1	n/a	$ 315	$ 2	n/a
GAAP net income (loss)	$ 101	$ 33	206%	$ 263	$ (112)	n/a
GAAP net income (loss) per share - diluted	$ 0.39	$ 0.13	200%	$ 1.04	$ (0.46)	n/a
Non-GAAP net income	$ 158	$ 88	80%	$ 495	$ 211	134%
Non-GAAP net income per share - diluted	$ 0.61	$ 0.35	74%	$ 1.95	$ 0.85	129%

"2017 was an outstanding year for Vertex as we made significant progress across all aspects of our business that moved us closer toward our goal of delivering new medicines that treat the underlying cause of CF for all people with the disease," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "As we look at 2018 and beyond, Vertex's scientific expertise and financial strength position us to advance key pipeline programs in CF, including our triple combination regimens, and to bring forward potential new medicines in multiple other serious diseases."

Full-Year 2017 Financial Highlights

Revenues:

- Total CF product revenues increased 29% to $2.17 billion from $1.68 billion for 2016.
- Net product revenues from ORKAMBI increased 35% to $1.32 billion from $979.6 million for 2016. The increase in ORKAMBI revenues was driven by the continued uptake in children with CF ages 6 to 11 in the U.S. and an increase in the number of patients being treated in European countries where ORKAMBI is currently reimbursed.
- Net product revenues from KALYDECO increased 20% to $844.6 million from $703.4 million for 2016. The increase in KALYDECO revenues was primarily driven by the rapid uptake among people ages 2 and older in the U.S. who have certain residual function mutations and continued growth in the number of patients being treated outside of the U.S. where KALYDECO is currently approved and reimbursed.
- GAAP collaborative revenues increased to $315.2 million, from $1.9 million for 2016. 2017 collaborative revenues include $230.0 million in upfront revenue from the out-licensing of four oncology programs to Merck KGaA, Darmstadt, Germany in January 2017.

Expenses:

- Combined GAAP R&D and SG&A expenses were $1.82 billion compared to $1.48 billion for 2016. Combined Non-GAAP R&D and SG&A were $1.33 billion compared to $1.20 billion for 2016.
- GAAP R&D expenses were $1.32 billion compared to $1.05 billion for 2016. The increase in GAAP R&D expenses was primarily due to an upfront payment of $160.0 million related to the acquisition of VX-561 (previously known as CTP-656), an investigational once-daily CFTR potentiator, from Concert Pharmaceuticals. Non-GAAP R&D expenses were $959.5 million compared to $857.8 million for 2016. The increase in non-GAAP R&D expenses was primarily attributable to the clinical development of the company's triple combination regimens for CF.
- GAAP SG&A expenses were $496.1 million compared to $432.8 million for 2016. Non-GAAP SG&A expenses were $375.3 million compared to $344.2 million for 2016. The increase in GAAP and non-GAAP SG&A expenses was driven by investments to support the treatment of patients with KALYDECO and ORKAMBI globally and additional investments to prepare for the U.S. launch of the tezacaftor/ivacaftor combination.

Net Income (Loss) Attributable to Vertex:

- GAAP net income was $263.5 million, or $1.04 per diluted share, compared to a 2016 GAAP net loss of $(112.1) million, or $(0.46) per diluted share. Non-GAAP net income was $494.6 million, or $1.95 per diluted share, compared to a 2016 non-GAAP net income of $211.2 million, or $0.85 per diluted share, for 2016. Full-year 2017 GAAP and non-GAAP net income growth was driven by increased CF product revenues.

Cash Position:

- As of December 31, 2017, Vertex had $2.09 billion in cash, cash equivalents and marketable securities after repayment of the $300 million balance of outstanding debt in the first quarter of 2017 from a revolving credit agreement, compared to $1.43 billion in cash, cash equivalents and marketable securities as of December 31, 2016.

Fourth-Quarter 2017 Financial Highlights

Revenues:

- Total CF net product revenues increased 37% to $621.2 million from $454.0 million for the fourth quarter of 2016.
- Net product revenues from ORKAMBI increased 32% to $365.4 million from $276.9 million for the fourth quarter of 2016.
- Net product revenues from KALYDECO increased 44% to $255.8 million from $177.1 million for the fourth quarter of 2016.
- GAAP collaborative revenues increased to $29.1 million from $0.9 million for 2016. Fourth-quarter 2017 collaborative revenues include a $25 million milestone payment from Janssen Pharmaceuticals, Inc. based on the initiation of a pivotal Phase 3 clinical trial of pimodivir (previously VX-787) for treatment in patients who are hospitalized or are outpatients at higher risk of influenza-related complications.

Expenses:

- Combined GAAP R&D and SG&A expenses were $441.5 million compared to $358.4 million for the fourth quarter of 2016. Combined non-GAAP R&D and SG&A expenses were $354.7 million compared to $295.0 million for the fourth quarter of 2016.
- GAAP R&D expenses were $306.7 million compared to $248.5 million for the fourth quarter of 2016. Non-GAAP R&D expenses were $249.2 million compared to $207.1 million for the fourth quarter of 2016.
- GAAP SG&A expenses were $134.8 million compared to $109.9 million for the fourth quarter of 2016. Non-GAAP SG&A expenses were $105.5 million compared to $87.9 million for the fourth quarter of 2016.

Net Income Attributable to Vertex:

- GAAP net income was $100.7 million, or $0.39 per diluted share, compared to $32.9 million, or $0.13 per diluted share, for the fourth quarter of 2016. Non-GAAP net income was $157.9 million, or $0.61 per diluted share, compared to $87.7 million, or $0.35 per diluted share, for the fourth quarter of 2016.

2018 Financial Guidance

Vertex today provided full-year 2018 guidance for combined GAAP and non-GAAP R&D and SG&A expenses, as summarized below:

- **Combined Non-GAAP and GAAP R&D and SG&A Expenses:** Vertex expects that its combined GAAP R&D and SG&A expense in 2018 will be in the range of $1.80 to $1.95 billion and combined non-GAAP R&D and SG&A expense will be in the range of $1.50 to $1.55 billion. The increase compared to 2017 primarily reflects ongoing and anticipated CF development efforts, including the investment for the preparation and commercial supply for up to two pivotal programs for its triple combination regimens, and the incremental investment to support the planned launch of the tezacaftor/ivacaftor combination.

Vertex plans to provide total CF product revenue guidance for the full year of 2018 upon the anticipated approval by the U.S. Food and Drug Administration (FDA) of the tezacaftor/ivacaftor combination, which has an action date of February 28, 2018.

Stock Repurchase Program

The company today announced that its Board of Directors has authorized a share repurchase program of up to $500 million of common stock through December 31, 2019. The repurchase program is expected to be executed over two years with the primary objective of reducing the impact of dilution from employee equity programs.

Purchases may be made through the open market or privately negotiated transactions and may be made pursuant to Rule 10b5-1 plans or other means as determined by Vertex's management and in accordance with the requirements of the Securities and Exchange Commission.

"In 2017, we achieved significant revenues, earnings and cash flow growth, and we expect this will continue as we increase the number of patients we treat with our CF medicines," said Ian Smith, Executive Vice President and Chief Operating Officer. "At the same time, we will continue our internal and external investments to advance our CF pipeline and the development of transformational medicines in other disease areas."

Business Highlights

ORKAMBI

On January 10, 2018, Vertex announced that the European Medicines Agency (EMA) has granted extension of the Marketing Authorization for ORKAMBI in people with CF who have two copies of the *F508del* mutation to include children ages 6 through 11. In Europe, there are approximately 3,400 children ages 6 through 11 with two copies of this mutation.

In the first quarter of 2018, Vertex plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) and Marketing Authorization Application (MAA) line extension to the EMA for the use of ORKAMBI in children ages 2 to 5 with CF who have two copies of the *F508del* mutation.

KALYDECO

On December 7, 2017, Vertex announced positive results from an open-label Phase 3 study evaluating the safety and tolerability of KALYDECO in infants ages 1 to 2 years who have one of 10 mutations for which KALYDECO is currently approved. The study met its primary endpoint of safety, showing that KALYDECO was generally well tolerated, and safety data were consistent with those seen in previous Phase 3 studies of KALYDECO in children ages 2 to 5 years and 6 to 11 years. There was also substantial improvement in sweat chloride, a secondary endpoint, as well as in multiple measures of pancreatic function.

Based on results from this study, Vertex expects to submit regulatory applications to the FDA and EMA in the first quarter of 2018.

TEZACAFTOR/IVACAFTOR

An NDA for the tezacaftor/ivacaftor combination treatment for people with CF ages 12 and older who have two copies of the *F508del* mutation or who have at least one residual function mutation that is responsive to tezacaftor/ivacaftor is currently under priority review by the FDA with an action date of February 28, 2018. The EMA has validated the MAA for the tezacaftor/ivacaftor combination and the company expects approval in the EU in the second half of 2018.

TRIPLE COMBINATION REGIMENS

In a separate press release today, Vertex announced the selection of two next-generation correctors, VX-659 and VX-445, to advance into Phase 3 development as part of two different triple combination regimens for people with CF. Upon the completion of regulatory discussions, the company plans to initiate a Phase 3 program in the first half of 2018 to evaluate VX-659 in triple combination with tezacaftor and ivacaftor. In addition, Vertex plans to initiate a Phase 3 program in mid-2018 to evaluate VX-445 in triple combination with tezacaftor and VX-561 as a once-daily regimen, pending additional data in the first half of 2018, including Phase 2 data on the combination of VX-445, tezacaftor and VX-561.

SICKLE CELL DISEASE & β-THALASSEMIA

On December 12, 2017, Vertex and CRISPR Therapeutics announced that the companies will co-develop and co-commercialize CTX001, an investigational gene editing treatment, as part of the companies' previously announced collaboration aimed at the discovery and development of new gene editing treatments that use the CRISPR/Cas9 technology. CTX001 represents the first gene-based treatment that Vertex exclusively licensed from CRISPR Therapeutics as part of the collaboration.

For CTX001, CRISPR and Vertex will equally share all research and development costs and profits worldwide. A Clinical Trial Application (CTA) was submitted in December 2017 for CTX001 to support the initiation of a Phase 1/2 trial in β-thalassemia in 2018 in Europe, and an Investigational New Drug (IND) Application is planned to support the initiation of a Phase 1/2 trial in sickle cell disease in 2018 in the U.S. Additional details on the trial designs will be provided upon study initiation.

INFLUENZA

During the fourth quarter of 2017, Vertex earned a $25 million milestone payment from Janssen Pharmaceuticals, Inc. (Janssen) based on the initiation of a pivotal Phase 3 clinical trial of pimodivir (JNJ-63623872) in combination with standard of care treatment in patients who are hospitalized or are outpatients at higher risk of influenza-related complications.

In June 2014, Vertex entered into a licensing agreement with Janssen for the worldwide development and commercialization of pimodivir, previously VX-787 discovered by Vertex. As part of the agreement, Vertex has the potential to receive development and commercial milestone payments as well as tiered royalties ranging from the high-single digits to mid-teens based on a percent of future net product sales.

The pimodivir development program receives funding support from the Biomedical Advanced Research and Development Authority (BARDA), part of the U.S. Department of Health and Human Services.

PAIN

In the first quarter of 2018, Vertex expects to obtain data from a Phase 2 proof-of-concept study evaluating VX-150, a selective NaV1.8 channel blocker, for the treatment of acute pain following bunionectomy surgery. An additional Phase 2 proof-of-concept study further evaluating VX-150 for the treatment of pain caused by small fiber neuropathy is ongoing.

ONGOING RESEARCH & DEVELOPMENT

Vertex has ongoing development programs for potential medicines aimed at other serious and life-threatening diseases, including VX-210 for the treatment of acute cervical spinal cord injury. In addition, Vertex is progressing additional internal research programs in sickle cell disease, alpha-1 antitrypsin disease, adrenoleukodystrophy, and polycystic kidney disease. The company expects to advance one or more research-stage drug candidates into clinical development in 2018.

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 (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 related income tax benefits and the effects of the deconsolidation of a variable interest entity and (iv) other adjustments. 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 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 regarding expenses associated with any potential future 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
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,	
	2017	2016	2017	2016
Revenues:				
Product revenues, net	$ 621,228	$ 453,882	$ 2,165,480	$ 1,683,632
Royalty revenues	1,345	3,887	7,988	16,600
Collaborative revenues (Note 1)	29,061	937	315,184	1,945
Total revenues	651,634	458,706	2,488,652	1,702,177
Costs and expenses:				
Cost of product revenues	83,712	59,646	272,675	206,811
Royalty expenses	340	836	2,444	3,649
Research and development expenses	306,664	248,452	1,324,625	1,047,690
Sales, general and administrative expenses	134,794	109,908	496,079	432,829
Restructuring expenses	387	224	14,246	1,262
Intangible asset impairment charge (Note 2)	—	—	255,340	—
Total costs and expenses	525,897	419,066	2,365,409	1,692,241
Income from operations	125,737	39,640	123,243	9,936
Interest expense, net	(12,547)	(20,439)	(57,550)	(81,432)
Other (expenses) income, net (Note 2)	(748)	1,105	(81,382)	4,130
Income (loss) from operations before provision for (benefit from) income taxes (Note 2)	112,442	20,306	(15,689)	(67,366)
Provision for (benefit from) income taxes (Note 2)	10,257	(7,453)	(107,324)	16,665
Net income (loss)	102,185	27,759	91,635	(84,031)
(Income) loss attributable to noncontrolling interest (Note 2)	(1,501)	5,186	171,849	(28,021)
Net income (loss) attributable to Vertex	$ 100,684	$ 32,945	$ 263,484	$ (112,052)
Amounts per share attributable to Vertex common shareholders:				
Net income (loss):				
Basic	$ 0.40	$ 0.13	$ 1.06	$ (0.46)
Diluted	$ 0.39	$ 0.13	$ 1.04	$ (0.46)
Shares used in per share calculations:				
Basic	251,557	245,454	248,858	244,685
Diluted	256,804	247,757	253,225	244,685

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
GAAP net income (loss) attributable to Vertex	$ 100,684	$ 32,945	$ 263,484	$ (112,052)
Stock-based compensation expense	75,402	59,082	290,736	237,705
Concert upfront and transaction expenses (Note 3)	—	—	165,057	—
Revenues and expenses related to VIEs (Note 2)	—	(4,500)	14,083	54,850
Other collaborative and transaction revenue and expenses (Note 4)	(19,177)	—	(255,747)	33,000
Other adjustments (Note 5)	941	145	16,947	(2,306)
Non-GAAP net income attributable to Vertex	$ 157,850	$ 87,672	$ 494,560	$ 211,197
Amounts per diluted share attributable to Vertex common shareholders:				
GAAP	$ 0.39	$ 0.13	$ 1.04	$ (0.46)
Non-GAAP	$ 0.61	$ 0.35	$ 1.95	$ 0.85
Shares used in diluted per share calculations:				
GAAP	256,804	247,757	253,225	244,685
Non-GAAP	256,804	247,757	253,225	247,276

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
GAAP total revenues	$651,634	$458,706	$2,488,652	$1,702,177
Revenues related to VIEs (Note 2)	(497)	(94)	(43,376)	(944)
Other collaborative and transaction revenue (Note 4)	(28,509)	—	(271,605)	—
Other adjustments (Note 5)	—	(121)	—	(526)
Non-GAAP total revenues	$622,628	$458,491	$2,173,671	$1,700,707

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
GAAP cost of product revenues and royalty expenses	$ 84,052	$ 60,482	$ 275,119	$ 210,460
Other adjustments (Note 5)	—	98	—	(19)
Non-GAAP cost of product revenues and royalty expenses	$ 84,052	$ 60,580	$ 275,119	$ 210,441
GAAP research and development expenses	$306,664	$248,452	$1,324,625	$1,047,690
Stock-based compensation expense	(47,045)	(38,383)	(181,900)	(153,451)
Concert upfront payment (Note 3)	—	—	(160,000)	—
Expenses related to VIEs (Note 2)	(967)	(2,971)	(7,729)	(6,762)
Other collaborative and transaction expenses (Note 4)	(9,282)	—	(14,966)	(33,000)
Other adjustments (Note 5)	(136)	(13)	(544)	3,293

Non-GAAP research and development expenses	$249,234	$207,085	$ 959,486	$ 857,770
GAAP sales, general and administrative expenses	$134,794	$109,908	$ 496,079	$ 432,829
Stock-based compensation expense	(28,357)	(20,699)	(108,836)	(84,254)
Concert transaction expenses (Note 3)	—	—	(5,057)	—
Expenses related to VIEs (Note 2)	(465)	(1,160)	(3,826)	(4,160)
Other collaborative and transaction expenses (Note 4)	(50)	—	(892)	—
Other adjustments (Note 5)	(418)	(127)	(2,157)	(232)
Non-GAAP sales, general and administrative expenses	$105,504	$ 87,922	$ 375,311	$ 344,183
Combined non-GAAP R&D and SG&A expenses	$354,738	$295,007	$1,334,797	$1,201,953

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
GAAP interest expense, net and other expense, net	$ (13,295)	$ (19,334)	$ (138,932)	$ (77,302)
(Income) expenses related to VIEs (Note 2)	(4)	(32)	76,503	108
Non-GAAP interest expense, net and other expense, net	$ (13,299)	$ (19,366)	$ (62,429)	$ (77,194)
GAAP provision for (benefit from) income taxes	$ 10,257	$ (7,453)	$ (107,324)	$ 16,665
Income taxes related to VIEs (Note 2)	2,432	3,320	114,090	(16,743)
Non-GAAP provision for (benefit from) income taxes	$ 12,689	$ (4,133)	$ 6,766	$ (78)

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

	December 31, 2017	**December 31, 2016**
Assets		
Cash, cash equivalents and marketable securities	$ 2,088,666	$ 1,434,557
Restricted cash and cash equivalents (VIE) (Note 2)	1,489	47,762
Accounts receivable, net	281,343	200,364
Inventories	111,830	77,604
Property and equipment, net	789,437	698,362
Intangible assets and goodwill (Note 2)	79,384	334,724
Other assets	193,865	103,414
Total assets	$ 3,546,014	$ 2,896,787
Liabilities and Shareholders' Equity		
Accounts payable and accruals	$ 517,955	$ 376,700
Other liabilities	415,501	260,984
Deferred tax liability (Note 2)	6,341	134,063
Construction financing lease obligation	563,911	486,849
Debt	—	300,000
Shareholders' equity	2,042,306	1,338,191
Total liabilities and shareholders' equity	$ 3,546,014	$ 2,896,787
Common shares outstanding	253,253	248,301

Note 1: In the three months ended December 31, 2017, collaborative revenues were primarily attributable to a $25.0 million milestone earned from our collaboration with Janssen Pharmaceuticals, Inc. During the twelve months ended December 31, 2017, collaborative revenues also include a $230.0 million up-front payment earned from our collaboration with Merck KGaA, Darmstadt, Germany and $40.0 million that one of the company's consolidated variable interest entities ("VIEs") received from a collaboration agreement with a third party.

Note 2: The company consolidated the financial statements of Parion as a VIE during 2016 and through September 30,

2017 and BioAxone Biosciences, Inc. as a VIE during 2016 and 2017. These VIEs were consolidated because Vertex has licensed the rights to develop the company's collaborators' most significant intellectual property assets. The company's interest and obligations with respect to these VIEs' assets and liabilities are limited to those accorded to the company in its collaboration agreements. "Restricted cash and cash equivalents (VIE)" reflects the VIEs' cash and cash equivalents, which Vertex does not have any interest in and which will not be used to fund the collaboration. Each reporting period Vertex estimates the fair value of the contingent payments by Vertex to these collaborators. Any increase in the fair value of these contingent payments results in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis. The fair value of contingent payments is evaluated each quarter and any change in the fair value is reflected in the company's statement of operations.

In the third quarter of 2017, the company determined that the value of Parion's pulmonary ENaC platform had become impaired and that the fair value of the intangible asset was zero as of September 30, 2017. Accordingly, an impairment charge of $255.3 million and a benefit from income taxes of $126.2 million resulting from this charge and subsequent deconsolidation of Parion attributable to noncontrolling interest was recorded in the third quarter of 2017. The total impact of this 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.

As of December 31, 2017, the company has a $29.0 million intangible asset related to its collaboration agreement with BioAxone Biosciences, Inc.

Note 3: In July 2017, the company completed the acquisition of VX-561 (formerly CTP-656) from Concert Pharmaceuticals, Inc. The company paid Concert $160.0 million in cash to acquire VX-561, which was recorded as a research and development expense in the twelve months ended December 31, 2017. The company also recorded $5.1 million in transaction costs that were recorded as sales, general and administrative expenses in the twelve months ended December 31, 2017.

Note 4: In the three months ended December 31, 2017, "Other collaboration and transaction revenues and expenses" were primarily attributable to the $25.0 million milestone earned from our collaboration with Janssen Pharmaceuticals, Inc. In the twelve months ended December 31, 2017, "Other collaboration and transaction revenues and expenses" also include revenues and expenses associated with the company's oncology program including the company's collaboration with Merck KGaA, Darmstadt, Germany which include the $230 million upfront payment earned pursuant to the collaboration. In the three and twelve months ended December 31, 2016, "Other collaboration and transaction revenues and expenses" primarily consisted of collaboration and asset acquisition payments for early-stage research assets. The company has not adjusted its prior year Reconciliation of GAAP to Non-GAAP Revenues and Expenses for the three and twelve months ended December 31, 2016 for $5.8 million and $20.7 million, respectively, of operating expenses related to its oncology program.

Note 5: In the 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 our research site in Canada. In the twelve months ended December 31, 2016, "Other adjustments" primarily consisted of revenues and operating costs and expenses related to HCV as well as restructuring charges related to the company's relocation from Cambridge to Boston, Massachusetts.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO® (ivacaftor)

KALYDECO (ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have 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 2 years 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 Prescribing Information for KALYDECO (ivacaftor).**

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

ORKAMBI is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 6 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 6 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 cyclosporin, 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/or chest tightness; nausea; diarrhea; gas; increase in a certain muscle enzyme called creatinine phosphokinase; common cold, including sore throat, stuffy or runny nose; fatigue; flu or flu-like symptoms; rash; 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.

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's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. 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 eight 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 and Mr. Smith's statements in this press release, the information provided in the sections captioned "2018 Financial Guidance" and "Stock Repurchase Program" 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 tezacaftor in combination with ivacaftor and our next-generation triple combination regimens. 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 2018 expenses 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)

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