



January 25, 2017

Vertex Reports Full-Year and Fourth-Quarter 2016 Financial Results

-2016 total CF product revenues of \$1.68 billion compared to \$982 million in 2015; \$980 million for ORKAMBI and \$703 million for KALYDECO-

-Fourth-quarter 2016 total CF product revenues of \$454 million; \$277 million for ORKAMBI and \$177 million for KALYDECO-

-Company reiterates 2017 financial guidance for ORKAMBI product revenues of \$1.1 to \$1.3 billion and KALYDECO product revenues of \$690 to \$710 million-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the full-year and quarter-ended December 31, 2016. On January 8, 2017, Vertex provided a comprehensive update on its ongoing research and development programs in cystic fibrosis (CF). The company today provided an update on its non-CF pipeline. Vertex also reiterated its financial guidance provided on January 8, 2017 for total 2017 ORKAMBI[®] (lumacaftor/ivacaftor) and KALYDECO[®] (ivacaftor) revenues and combined Non-GAAP R&D and SG&A expenses. Key financial results include:

	Three Months Ended December 31,		%	Twelve Months Ended December 31,		%
	2016	2015		2016	2015	
	(in millions, except per share and percentage data)					
ORKAMBI product revenues, net	\$ 277	\$ 220		\$ 980	\$ 351	
KALYDECO product revenues, net	\$ 177	\$ 181		\$ 703	\$ 632	
TOTAL CF product revenues, net	\$ 454	\$ 401	13%	\$ 1,683	\$ 982	71%
GAAP net income (loss)	\$ 33	\$ (74)		\$ (112)	\$ (556)	
GAAP net income (loss) per share - diluted	\$ 0.13	\$ (0.30)		\$ (0.46)	\$ (2.31)	
Non-GAAP net income (loss)	\$ 88	\$ 44		\$ 211	\$ (267)	
Non-GAAP net income (loss) per share - diluted	\$ 0.35	\$ 0.18		\$ 0.85	\$ (1.11)	

"2016 was a very important year for Vertex. It was marked by significant CF revenue growth from approximately \$980 million in 2015 to approximately \$1.7 billion in 2016. Additionally, our progress toward treating more people with CF continued in 2016 with the approval of ORKAMBI for children ages six to eleven in the U.S., the advancement of two next-generation correctors into Phase 2 development, and identifying two additional next-generation correctors for Phase 1 development," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "As we enter 2017, we anticipate continued revenue and earnings growth and additional important progress toward our long-term goal of treating all people with CF."

Full-Year 2016 Financial Highlights

Revenues:

- ┆ Total CF revenues were \$1.68 billion compared to \$982.3 million for 2015.
- ┆ Net product revenues from ORKAMBI were \$979.6 million compared to \$350.7 million for 2015. ORKAMBI was launched in the U.S. in July 2015.
- ┆ Net product revenues from KALYDECO were \$703.4 million compared to \$631.7 million for 2015.

Expenses:

- | Combined GAAP R&D and SG&A expenses were \$1.48 billion compared to \$1.37 billion for 2015. Combined Non-GAAP R&D and SG&A were \$1.20 billion compared to \$1.06 billion for 2015.
- | GAAP R&D expenses were \$1.05 billion compared to \$995.9 million for 2015. Non-GAAP R&D expenses were \$857.8 million compared to \$764.5 million for 2015. The increased R&D expenses for the full-year 2016 were primarily the result of increased costs related to the progression of the company's CF pipeline.
- | GAAP SG&A expenses were \$432.8 million compared to \$376.6 million for 2015. Non-GAAP SG&A expenses were \$344.2 million compared to \$295.4 million for 2015. The increased SG&A expenses were primarily the result of increased investment to support the global launch of ORKAMBI.

Net Income (Loss) Attributable to Vertex:

- | GAAP net loss was \$(112.1) million, or \$(0.46) per diluted share, compared to Vertex's 2015 GAAP net loss of \$(556.3) million, or \$(2.31) per diluted share. Non-GAAP net income was \$211.2 million, or \$0.85 per diluted share, compared to a non-GAAP net loss of \$(267.3) million, or \$(1.11) per diluted share, for 2015. The decreased net loss on a GAAP basis and the change to net income on a non-GAAP basis was the result of a full year of ORKAMBI product revenues and increased KALYDECO product revenues, partially offset by increased operating expenses.

Fourth-Quarter 2016 Financial Highlights

Revenues:

- | Total CF product revenues were \$454.0 million compared to \$400.6 million for the fourth quarter of 2015.
- | Net product revenues from ORKAMBI were \$276.9 million compared to \$219.9 million for the fourth quarter of 2015.
- | Net product revenues from KALYDECO were \$177.1 million, compared to \$180.7 million for the fourth quarter of 2015.

Expenses:

- | Combined GAAP R&D and SG&A expenses were \$358.4 million compared to \$406.7 million for the fourth quarter of 2015. Combined non-GAAP R&D and SG&A expenses were \$295.0 million compared to \$281.9 million for the fourth quarter of 2015.
- | GAAP R&D expenses were \$248.5 million compared to \$310.2 million for the fourth quarter of 2015. Non-GAAP R&D expenses were \$207.1 million compared to \$203.8 million for the fourth quarter of 2015. The decrease in GAAP R&D expenses was primarily due to a one-time \$75 million upfront payment to CRISPR Therapeutics AG that was included in GAAP research expenses in the fourth quarter of 2015.
- | GAAP SG&A expenses were \$109.9 million compared to \$96.5 million for the fourth quarter of 2015. Non-GAAP SG&A expenses were \$87.9 million compared to \$78.1 million for the fourth quarter of 2015. The increases were primarily driven by increased investment to support the global launch of ORKAMBI.

Net Income (Loss) Attributable to Vertex:

- | GAAP net income was \$32.9 million, or \$0.13 per diluted share, compared to GAAP net loss of \$(73.7) million, or \$(0.30) per diluted share, for the fourth quarter of 2015. Non-GAAP net income was \$87.7 million, or \$0.35 per diluted share, compared to a non-GAAP net income of \$43.6 million, or \$0.18 per diluted share, for the fourth quarter of 2015.

Cash Position:

- | As of December 31, 2016, Vertex had \$1.43 billion in cash, cash equivalents and marketable securities compared to \$1.04 billion in cash, cash equivalents and marketable securities as of December 31, 2015. On January 11, 2017, Vertex entered into a licensing agreement with Merck KGaA, Darmstadt, Germany for four clinical and pre-clinical oncology programs. Under the agreement, Vertex expects to receive \$230 million in up-front payments in the first quarter of 2017 subject to the completion of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act.
- | As of December 31, 2016, Vertex had \$300 million outstanding from a revolving credit agreement, which was refinanced on October 13, 2016 to lower the company's interest expense. The \$300 million outstanding under the new credit agreement matures in the fourth quarter of 2021.

2017 Financial Guidance:

Vertex today reiterated its 2017 revenue guidance for ORKAMBI and KALYDECO. The company also reiterated guidance for its 2017 combined non-GAAP R&D and SG&A expenses and introduced guidance for 2017 combined GAAP R&D and SG&A expenses. The guidance is summarized below:

- 1 **ORKAMBI:** The company continues to expect total 2017 product revenues for ORKAMBI of \$1.1 to \$1.3 billion. This range includes an estimate of potential additional European revenues in 2017 that is largely dependent on which European countries complete reimbursement agreements in 2017 and when these agreements become effective. The company expects first-quarter 2017 ORKAMBI net product revenues to be similar to fourth-quarter 2016 ORKAMBI net product revenues.
- 1 **KALYDECO:** The company continues to expect total 2017 product revenues for KALYDECO of \$690 to \$710 million.
- 1 **Combined GAAP and Non-GAAP R&D and SG&A Expenses:** Vertex expects that its 2017 combined GAAP R&D and SG&A expenses will be in the range of \$1.55 to \$1.70 billion and non-GAAP R&D and SG&A expenses will be in the range of \$1.25 to \$1.30 billion. The increase as compared to 2016 primarily reflects increased costs related to ongoing and planned CF development efforts and in the global infrastructure to support ORKAMBI and KALYDECO.

Research and Development Program in Other Serious Diseases

On January 8, 2017, Vertex provided a comprehensive update on its ongoing research and development programs in CF. The company today provided the following information on its non-CF pipeline:

Oncology: Licensing Agreement with Merck KGaA, Darmstadt, Germany

On January 11, 2017, Vertex announced that it entered into a licensing agreement with Merck KGaA, Darmstadt, Germany for the worldwide development and commercialization of four promising research and development programs for the treatment of cancer. As part of the agreement, Merck KGaA, Darmstadt, Germany licensed two clinical-stage programs comprised of the compounds VX-970, VX-803 and VX-984, targeting DNA damage and repair, along with two additional novel research programs that include one immuno-oncology program and a program against a completely novel target.

Vertex will receive an upfront payment of \$230 million, in addition to royalties on future net sales. The collaboration, and the related \$230 million up-front payment, is subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act. Merck KGaA, Darmstadt, Germany will assume full responsibility for the development and commercialization of all the programs.

Pain: VX-150 Phase 2 Study in Osteoarthritis

Vertex today announced data from a recently completed Phase 2 randomized, double-blind, placebo-controlled, cross-over study of VX-150 in people with pain from osteoarthritis of the knee. Data from the proof-of-concept study showed statistically significant pain relief with VX-150 and support its further development for the potential treatment of a broad spectrum of pain conditions. VX-150 is a first-in-class oral inhibitor of the sodium channel 1.8 (Na_v 1.8). The 14-day study, which enrolled 124 people with at least moderate pain caused by osteoarthritis of the knee, met its primary endpoint of a change from study baseline in the Western Ontario and McMaster Universities Osteoarthritis Index (WOMAC) pain subscale score at Day 14 compared to placebo, with a treatment effect of -0.8 (p=0.0356). The WOMAC pain subscale index assesses pain across five activities on a scale of zero to four using patient-reported questionnaires. The frequency and severity of adverse events observed in the study were similar between the placebo and VX-150 treatment periods. Four patients discontinued treatment due to adverse events in the VX-150 treatment period compared to zero patients in the placebo treatment period.

Vertex plans to initiate additional Phase 2 proof-of-concept studies in the second half of 2017 in neuropathic and acute pain with a goal of further evaluating the potential role of VX-150 in the treatment of different pain conditions.

Vertex is also progressing additional early research programs in adrenoleukodystrophy, alpha-1 antitrypsin disease, sickle cell disease and polycystic kidney disease.

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 stock-based compensation expense, revenues and expenses related to consolidated variable interest entities, costs and credits related to the relocation of the company's corporate headquarters and hepatitis C-related revenues and costs and 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 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		Twelve Months Ended	
	December 31,		December 31,	
	2016	2015	2016	2015
Revenues:				
Product revenues, net	\$453,882	\$406,550	\$1,683,632	\$1,000,324
Royalty revenues	3,887	6,331	16,600	23,959
Collaborative revenues	937	5,054	1,945	8,053
Total revenues	<u>458,706</u>	<u>417,935</u>	<u>1,702,177</u>	<u>1,032,336</u>
Costs and expenses:				
Cost of product revenues (Note 1)	59,646	62,092	206,811	117,151
Royalty expenses	836	1,293	3,649	7,361
Research and development expenses	248,452	310,181	1,047,690	995,922
Sales, general and administrative expenses	109,908	96,549	432,829	376,575
Restructuring expenses	224	1,524	1,262	2,206
Total costs and expenses	<u>419,066</u>	<u>471,639</u>	<u>1,692,241</u>	<u>1,499,215</u>
Income (loss) from operations	39,640	(53,704)	9,936	(466,879)
Interest expense, net	(20,439)	(20,654)	(81,432)	(84,206)
Other income (expenses), net	1,105	(1,690)	4,130	(6,715)
Income (loss) from operations before provision for (benefit from) income taxes	20,306	(76,048)	(67,366)	(557,800)
Provision for (benefit from) income taxes	(7,453)	(1,379)	16,665	30,381
Net Income (loss)	<u>27,759</u>	<u>(74,669)</u>	<u>(84,031)</u>	<u>(588,181)</u>
(Income) loss attributable to noncontrolling interest	5,186	938	(28,021)	31,847
Net income (loss) attributable to Vertex	<u>\$ 32,945</u>	<u>\$ (73,731)</u>	<u>\$ (112,052)</u>	<u>\$ (556,334)</u>
Amounts per share attributable to Vertex common shareholders:				
Net income (loss):				
Basic	\$ 0.13	\$ (0.30)	\$ (0.46)	\$ (2.31)
Diluted	\$ 0.13	\$ (0.30)	\$ (0.46)	\$ (2.31)
Shares used in per share calculations:				
Basic	245,454	242,987	244,685	241,312
Diluted	247,757	242,987	244,685	241,312

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,	
	2016	2015	2016	2015
GAAP income (loss) attributable to Vertex	\$ 32,945	\$ (73,731)	\$(112,052)	\$(556,334)
Stock-based compensation expense	59,082	44,645	237,705	231,025
Real estate restructuring costs and income (Note 2)	201	454	896	(1,748)
HCV related revenues and costs (Note 3)	(79)	(5,510)	(3,338)	(23,716)
Other adjustments (Notes 4 and 5)	(4,477)	77,786	87,986	83,424
Non-GAAP net income (loss) attributable to Vertex	<u>\$ 87,672</u>	<u>\$ 43,644</u>	<u>\$ 211,197</u>	<u>\$(267,349)</u>

Amounts per diluted share attributable to Vertex common shareholders:

GAAP	\$ 0.13	\$ (0.30)	\$ (0.46)	\$ (2.31)
Non-GAAP	\$ 0.35	\$ 0.18	\$ 0.85	\$ (1.11)
Shares used in diluted per share calculations:				
GAAP	247,757	242,987	244,685	241,312
Non-GAAP	247,757	246,635	247,276	241,312

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,	
	2016	2015	2016	2015
GAAP total revenues	\$458,706	\$417,935	\$1,702,177	\$1,032,336
HCV related revenues (Note 3)	(121)	(6,071)	(526)	(21,449)
Other adjustments (Note 4)	(94)	(1,509)	(944)	(2,888)
Non-GAAP total revenues	<u>\$458,491</u>	<u>\$410,355</u>	<u>\$1,700,707</u>	<u>\$1,007,999</u>

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2016	2015	2016	2015
GAAP cost of product revenues and royalty expenses	\$ 60,482	\$ 63,385	\$ 210,460	\$ 124,512
HCV related costs (Note 3)	98	(209)	(19)	(631)
Non-GAAP cost of product revenues and royalty expenses	\$ 60,580	\$ 63,176	\$ 210,441	\$ 123,881
GAAP research and development expenses	\$248,452	\$310,181	\$1,047,690	\$ 995,922
Stock-based compensation expense	(38,383)	(28,405)	(153,451)	(152,955)
HCV related costs (Note 3)	(13)	(213)	3,330	493
Other adjustments (Note 4)	(2,971)	(77,762)	(39,799)	(78,984)
Non-GAAP research and development expenses	<u>\$207,085</u>	<u>\$203,801</u>	<u>\$ 857,770</u>	<u>\$ 764,476</u>
GAAP sales, general and administrative expenses	\$109,908	\$ 96,549	\$ 432,829	\$ 376,575
Stock-based compensation expense	(20,699)	(16,240)	(84,254)	(78,070)
HCV related costs (Note 3)	(127)	—	(232)	2,807
Other adjustments (Note 4)	(1,160)	(2,176)	(4,160)	(5,892)
Non-GAAP sales, general and administrative expenses	<u>\$ 87,922</u>	<u>\$ 78,133</u>	<u>\$ 344,183</u>	<u>\$ 295,420</u>
Combined non-GAAP R&D and SG&A expenses	<u>\$295,007</u>	<u>\$281,934</u>	<u>\$1,201,953</u>	<u>\$1,059,896</u>

Three Months Ended **Twelve Months Ended**

	<u>December 31,</u>		<u>December 31,</u>	
	<u>2016</u>	<u>2015</u>	<u>2016</u>	<u>2015</u>
GAAP interest expense, net and other expense, net	\$ (19,334)	\$ (22,344)	\$ (77,302)	\$ (90,921)
Other adjustments (Note 4)	(32)	—	108	—
Non-GAAP interest expense, net and other expense, net	<u>\$ (19,366)</u>	<u>\$ (22,344)</u>	<u>\$ (77,194)</u>	<u>\$ (90,921)</u>
GAAP provision for (benefit from) income taxes	\$ (7,453)	\$ (1,379)	\$ 16,665	\$ 30,381
Other adjustments (Note 4)	3,320	636	(16,743)	(29,731)
Non-GAAP (benefit from) provision for income taxes	<u>\$ (4,133)</u>	<u>\$ (743)</u>	<u>\$ (78)</u>	<u>\$ 650</u>

Condensed Consolidated Balance Sheets Data

(in thousands)

(unaudited)

	<u>December 31, 2016</u>	<u>December 31, 2015</u>
Assets		
Cash, cash equivalents and marketable securities	\$ 1,434,557	\$ 1,042,462
Restricted cash and cash equivalents (VIE) (Note 5)	47,762	78,910
Accounts receivable, net	201,083	173,838
Inventories	77,604	57,207
Property and equipment, net	698,362	697,715
Intangible assets and goodwill	334,724	334,724
Other assets	99,693	113,731
Total assets	<u>\$ 2,893,785</u>	<u>\$ 2,498,587</u>
Liabilities and Shareholders' Equity		
Other liabilities	\$ 562,691	\$ 426,482
Deferred tax liability	134,063	110,439
Accrued restructuring expense	7,954	15,358
Deferred revenues	12,637	26,010
Capital leases	54,402	58,468
Construction financing lease obligation	486,849	473,043
Debt	296,998	295,159
Shareholders' equity	1,338,191	1,093,628
Total liabilities and shareholders' equity	<u>\$ 2,893,785</u>	<u>\$ 2,498,587</u>
Common shares outstanding	248,301	246,307

Note 1 : The company's cost of product revenues includes \$13.9 million of expense in each of the first quarter of 2016 and the fourth quarter of 2015 related to commercial milestones paid to the CFFT.

Note 2: The company excludes restructuring expense from its non-GAAP income (loss) attributable to Vertex. "Real estate restructuring costs and income" consisted of restructuring charges related primarily to the company's relocation from Cambridge to Boston, Massachusetts.

Note 3: "HCV related revenues and costs" included net product revenues from INCIVEK, royalty revenues from INCIVO, HCV collaborative revenues and operating costs and expenses related to HCV. The Company withdrew INCIVEK from the market in the United States in 2014.

Note 4: In the three months ended December 31, 2016, "Other adjustments" was primarily attributable to a net decrease in the fair value of contingent milestone payments and royalties payable by Vertex to Parion and BioAxone due to changes in certain assumptions used in establishing the fair value including the discount rate and development timeline. In the twelve months ended December 31, 2016, "Other adjustments" of \$54.9 million was primarily attributable to an increase in the fair value of contingent milestone payments and royalties payable by Vertex to Parion due to the Phase 2 study meeting its primary safety endpoint as well as \$33.0 million of payments for collaborations and the acquisition of certain early stage assets. In the three and twelve months ended December 31, 2015, "Other adjustments" was primarily attributable to a \$75.0 million payment related to our collaboration with CRISPR Therapeutics AG that was recorded as research expense.

Note 5: The company consolidates the financial statements of two of its collaborators as VIEs as of December 31, 2016 and December 31, 2015. These VIEs are 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 with these collaborators. 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 milestone payments and royalties payable by Vertex to these collaborators. Any increase in the fair value of these contingent milestone and royalty 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 milestone and royalty payments is evaluated each quarter and any change in the fair value is reflected in the company's statement of operations.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For seven years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences. 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 the second paragraph of the press release, the information provided in the section captioned "2017 Financial Guidance" and statements regarding (i) the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act with respect to our licensing agreement with Merck KGaA and (ii) the development plan and timeline for VX-150. 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 2017 revenues and 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-GEN)

View source version on businesswire.com: <http://www.businesswire.com/news/home/20170125006143/en/>

Vertex Contacts:

Investors:

Michael Partridge, 617-341-6108

or

Eric Rojas, 617-961-7205

or

Zach Barber, 617-341-6470

or

Media:

617-341-6992

mediainfo@vrtx.com

Source: Vertex Pharmaceuticals Incorporated

News Provided by Acquire Media