



April 27, 2017

Vertex Reports First-Quarter 2017 Financial Results

-First-quarter 2017 cystic fibrosis product revenues of \$481 million; \$295 million for ORKAMBI and \$186 million for KALYDECO-

-Company reiterates 2017 guidance for ORKAMBI product revenues of \$1.1 to \$1.3 billion and increases 2017 guidance for KALYDECO product revenues to \$710 to \$730 million-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended March 31, 2017. Vertex also reiterated guidance for total 2017 ORKAMBI® (lumacaftor/ivacaftor) revenues and combined GAAP and non-GAAP R&D and SG&A expenses, and increased its total 2017 guidance for KALYDECO® (ivacaftor) revenues.

Key financial results include:

	Three Months Ended March 31,		% Change
	2017	2016	
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	\$ 295	\$ 223	32%
KALYDECO product revenues, net	\$ 186	\$ 171	9%
TOTAL CF product revenues, net	\$ 481	\$ 394	22%
GAAP Collaborative revenues, net	\$ 233	\$ —	
GAAP net income (loss)	\$ 248	\$ (42)	
GAAP net income (loss) per share - diluted	\$ 0.99	\$ (0.17)	
Non-GAAP net income	\$ 101	\$ 22	
Non-GAAP net income per share - diluted	\$ 0.41	\$ 0.09	

"During the quarter, our progress toward treating more people with CF was marked by several important milestones, including expanding the number of people being treated with our approved medicines and developing potential new medicines to treat all people with CF in the future," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We saw continued uptake of ORKAMBI in eligible children ages 6 to 11 in the U.S. and completed an MAA submission for the same group of patients in Europe. Additionally, we reported positive data from two Phase 3 studies of the tezacaftor/ivacaftor combination, which we believe will bring us closer to treating more people with the disease. We remain focused on advancing key CF programs, including our triple combination regimens, to support our goal of providing treatments for all people with CF."

Financial Highlights

Revenues:

- | Total CF product revenues were \$480.6 million compared to \$393.6 million for the first quarter of 2016.
- | Net product revenues from ORKAMBI were \$294.9 million compared to \$223.1 million for the first quarter of 2016.
- | Net product revenues from KALYDECO were \$185.7 million, compared to \$170.5 million for the first quarter of 2016. Approximately \$9.0 million of the first quarter 2017 revenues was attributable to one-time adjustments mainly related

to reimbursement agreements in Europe.

- Collaborative revenues were \$232.5 million, compared to \$0.1 million for the first quarter of 2016. First-quarter 2017 collaborative revenues include \$230.0 million in upfront revenue from the out-licensing of four oncology programs to Merck KGaA, Darmstadt, Germany.

Expenses:

- Combined GAAP R&D and SG&A expenses were \$386.9 million compared to \$361.1 million for the first quarter of 2016. Combined non-GAAP R&D and SG&A expenses were \$312.9 million compared to \$305.7 million for the first quarter of 2016.
- GAAP R&D expenses were \$273.6 million compared to \$255.9 million for the first quarter of 2016. Non-GAAP R&D expenses were \$226.6 million compared to \$222.0 million for the first quarter of 2016.
- GAAP SG&A expenses were \$113.3 million compared to \$105.2 million for the first quarter of 2016. Non-GAAP SG&A expenses were \$86.3 million compared to \$83.7 million for the first quarter of 2016.

Net Income (Loss) Attributable to Vertex:

- GAAP net income was \$247.8 million, or \$0.99 per diluted share, compared to a net loss of \$(41.6) million, or \$(0.17) per diluted share, for the first quarter of 2016. First-quarter 2017 GAAP net income includes \$230.0 million in upfront revenue from the out-licensing of four oncology programs to Merck KGaA, Darmstadt, Germany. Non-GAAP net income was \$101.4 million, or \$0.41 per diluted share, compared to \$22.4 million, or \$0.09 per diluted share, for the first quarter of 2016. First-quarter 2017 non-GAAP net income growth was driven by increased product revenues.

Cash Position:

- As of March 31, 2017, Vertex had \$1.41 billion in cash, cash equivalents and marketable securities compared to \$1.43 billion in cash, cash equivalents and marketable securities as of December 31, 2016.
- On January 11, 2017, Vertex entered into a licensing agreement with Merck KGaA, Darmstadt, Germany for four clinical and pre-clinical oncology programs. Vertex received the majority of the upfront payment related to the agreement in the first quarter of 2017. The company expects to receive the remaining portion of the upfront payment, which was remitted to the German tax authorities, by the end of 2017.
- As of December 31, 2016, Vertex had \$300 million outstanding under its senior secured revolving credit facility, which was repaid in full in the first quarter of 2017. The facility remains in place and provides the company with access to approximately \$800 million in borrowing capacity, subject to the lender's approval.

2017 Financial Guidance:

Vertex today reiterated its 2017 guidance for ORKAMBI revenues and combined GAAP and non-GAAP R&D and SG&A expenses, and increased its 2017 revenue guidance for KALYDECO:

- 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.
- KALYDECO:** The company today increased its total 2017 product revenue guidance for KALYDECO to \$710 to \$730 million. The increase was based on one-time reimbursement adjustments recognized in the first quarter and the strong underlying demand for the medicine. The prior range, first provided on January 8, 2017, was for total 2017 KALYDECO product revenues of \$690 to \$710 million.
- Combined GAAP and Non-GAAP R&D and SG&A Expenses:** Vertex continues to expect that its total 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.

CF Medicines and Pipeline Update

Vertex today provided the following updates on its CF program:

ORKAMBI

Submission for approval to treat children ages 6 through 11 in Europe: In March 2017, Vertex completed the

submission of a Marketing Authorization Application (MAA) line extension to the European Medicines Agency (EMA) for approval of ORKAMBI in children ages 6 through 11 with two copies of the *F508del* mutation. There are approximately 3,400 children ages 6 through 11 who have two copies of the *F508del* mutation in Europe.

TEZACAFTOR/IVACAFTOR

Positive Phase 3 data support regulatory submissions in Q3 2017: On March 28, 2017, Vertex announced positive data from two Phase 3 studies of the investigational tezacaftor/ivacaftor combination in people with CF ages 12 and older who have two copies of the *F508del* mutation and people who have one mutation that results in residual CFTR function and one *F508del* mutation. Based on these results, Vertex plans to submit a New Drug Application (NDA) to the FDA and an MAA to the EMA in the third quarter of 2017.

Phase 3 study in people with one copy of the *F508del* mutation and a second mutation that results in a gating defect: In the first half of 2017, Vertex expects to complete enrollment in a study evaluating the investigational tezacaftor/ivacaftor combination in people with CF ages 12 and older with one copy of the *F508del* mutation and a second mutation that results in a gating defect in the CFTR protein that has been shown to be responsive to ivacaftor alone. Data from this study are not expected to be part of the initial regulatory submissions planned for the tezacaftor/ivacaftor combination.

Phase 3 study in children ages 6 to 11 in the U.S.: A Phase 3 study of the investigational tezacaftor/ivacaftor combination in children with CF ages 6 through 11 in the U.S. is proceeding as planned. The study is evaluating the pharmacokinetics, safety and tolerability of the tezacaftor/ivacaftor combination in people with CF ages 6 through 11 with either two copies of the *F508del* mutation or those with one copy of the *F508del* mutation and one residual function or gating mutation.

TRIPLE COMBINATION REGIMENS

Vertex is currently evaluating four different next-generation correctors to be included in an investigational triple combination regimen with tezacaftor and ivacaftor.

VX-152: Vertex announced today that the second cohort of patients with two copies of the *F508del* mutation in the Phase 2 study of VX-152 in combination with tezacaftor and ivacaftor is being amended to evaluate four weeks of triple combination dosing. The study had initially planned to evaluate two weeks of dosing. The company expects to have data from this study in the second half of 2017.

VX-440: The Phase 2 study of VX-440 in people with CF is progressing as planned and the company expects to have data from this study in the second half of 2017.

VX-659: The Phase 1 study of VX-659 in healthy volunteers and people with CF is progressing as planned and the company expects to have data from this study in the second half of 2017.

VX-445: Dosing is underway in a Phase 1 study of VX-445 to evaluate single ascending doses, multiple ascending doses for 10 days and triple combination dosing with the tezacaftor/ivacaftor combination for 14 days in healthy volunteers. Vertex also plans to evaluate triple combination dosing with VX-445 in people with CF who have one copy of the *F508del* mutation and one copy of a mutation that results in minimal CFTR function. The company expects to have data from this study in early 2018.

OTHER BUSINESS

CTP-656 for potential use in future combination regimens: On March 6, 2017, Vertex signed an asset purchase agreement to acquire CTP-656 from Concert Pharmaceuticals. CTP-656 is an investigational CFTR potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of CF. As part of the agreement, Vertex will pay Concert at closing \$160 million in cash for all worldwide development and commercialization rights to CTP-656. If CTP-656 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France. The agreement is subject to approval by Concert's shareholders and clearance under the Hart-Scott-Rodino Antitrust Improvements Act, neither of which has been obtained yet.

Licensing agreement with Merck KGaA, Darmstadt, Germany for oncology assets: In the first quarter of 2017, the previously announced transaction to out-license four programs for the treatment of cancer to Merck KGaA, Darmstadt, Germany was completed. As part of the agreement announced on January 11, 2017, 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 novel target. During the first quarter of 2017, Vertex received the majority of the upfront payment related to the agreement.

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 consolidated variable interest entities and other collaboration agreements and (iii) 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
First-Quarter Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2017	2016
Revenues:		
Product revenues, net	\$ 480,622	\$ 394,410
Royalty revenues	1,551	3,596
Collaborative revenues (Note 1)	232,545	74
Total revenues	<u>714,718</u>	<u>398,080</u>
Costs and expenses:		
Cost of product revenues (Note 2)	46,242	49,789
Royalty expenses	746	860
Research and development expenses	273,563	255,860
Sales, general and administrative expenses	113,326	105,214
Restructuring expenses	9,999	687
Total costs and expenses	<u>443,876</u>	<u>412,410</u>
Income (loss) from operations	270,842	(14,330)
Interest expense, net	(16,765)	(20,698)
Other (expense) income, net	(544)	4,411
Income (loss) from operations before provision for income taxes	253,533	(30,617)
Provision for income taxes	3,985	5,485
Net income (loss)	249,548	(36,102)
Income attributable to noncontrolling interest (Note 5)	(1,792)	(5,529)
Net income (loss) attributable to Vertex	<u>\$ 247,756</u>	<u>\$ (41,631)</u>

Amounts per share attributable to Vertex common shareholders:

Net income (loss):		
Basic	\$ 1.01	\$ (0.17)
Diluted	\$ 0.99	\$ (0.17)

Shares used in per share calculations:

Basic	246,024	243,831
Diluted	248,700	243,831

Reconciliation of GAAP to Non-GAAP Net Income (Loss)
First-Quarter Results

(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2017	2016
GAAP income (loss) attributable to Vertex	\$ 247,756	\$ (41,631)
Stock-based compensation expense	68,982	55,472
Collaboration revenues and expenses (Note 3)	(226,300)	9,431
Other adjustments (Note 4)	10,968	(850)
Non-GAAP net income attributable to Vertex	\$ 101,406	\$ 22,422

Amounts per diluted share attributable to Vertex common shareholders:

GAAP	\$ 0.99	\$ (0.17)
Non-GAAP	\$ 0.41	\$ 0.09

Shares used in diluted per share calculations:

GAAP	248,700	243,831
Non-GAAP	248,700	246,680

Reconciliation of GAAP to Non-GAAP Revenues and Expenses
First-Quarter Results

(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2017	2016
GAAP total revenues	\$ 714,718	\$ 398,080
Collaboration revenues (Note 3)	(232,462)	(74)
Other adjustments (Note 4)	—	(851)
Non-GAAP total revenues	\$ 482,256	\$ 397,155

	Three Months Ended March 31,	
	2017	2016
GAAP cost of product revenues and royalty expenses	\$ 46,988	\$ 50,649
Other adjustments (Note 4)	—	(139)
Non-GAAP cost of product revenues and royalty expenses	\$ 46,988	\$ 50,510
GAAP research and development expenses	\$ 273,563	\$ 255,860
Stock-based compensation expense	(44,837)	(34,448)
Collaboration expenses (Note 3)	(2,009)	(159)
Other adjustments (Note 4)	(136)	793
Non-GAAP research and development expenses	\$ 226,581	\$ 222,046
GAAP sales, general and administrative expenses	\$ 113,326	\$ 105,214
Stock-based compensation expense	(24,145)	(21,024)
Collaboration expenses (Note 3)	(2,004)	(543)
Other adjustments (Note 4)	(833)	32
Non-GAAP sales, general and administrative expenses	\$ 86,344	\$ 83,679
Combined non-GAAP R&D and SG&A expenses	\$ 312,925	\$ 305,725

Three Months Ended March 31,

	<u>2017</u>	<u>2016</u>
GAAP interest expense, net and other expense, net	\$ (17,309)	\$ (16,287)
Collaboration expenses (Note 3)	(34)	211
Non-GAAP interest expense, net and other expense, net	\$ (17,343)	\$ (16,076)
GAAP provision for income taxes	\$ 3,985	\$ 5,485
Collaboration expenses (Note 3)	(391)	(3,063)
Non-GAAP provision for income taxes	\$ 3,594	\$ 2,422

Condensed Consolidated Balance Sheets Data

(in thousands)

(unaudited)

	<u>March 31, 2017</u>	<u>December 31, 2016</u>
Assets		
Cash, cash equivalents and marketable securities	\$ 1,408,782	\$ 1,434,557
Restricted cash and cash equivalents (VIE) (Note 5)	44,564	47,762
Accounts receivable, net	207,955	201,083
Inventories	82,020	77,604
Property and equipment, net	708,395	698,362
Intangible assets and goodwill	334,724	334,724
Other assets (Note 1)	160,263	102,695
Total assets	<u>\$ 2,946,703</u>	<u>\$ 2,896,787</u>
Liabilities and Shareholders' Equity		
Accounts payable and accruals	\$ 341,107	\$ 376,700
Other liabilities	288,120	260,984
Deferred tax liability	135,402	134,063
Construction financing lease obligation	499,281	486,849
Debt	—	300,000
Shareholders' equity (Note 5)	1,682,793	1,338,191
Total liabilities and shareholders' equity	<u>\$ 2,946,703</u>	<u>\$ 2,896,787</u>
Common shares outstanding	248,891	248,301

Note 1: In the three months ended March 31, 2017, collaborative revenues were primarily attributable to a \$230 million up-front payment earned from our collaboration with Merck KGaA, Darmstadt, Germany. During the first quarter of 2017, the company received \$193.6 million of the up-front payment and the remaining \$36.4 million was remitted to the German tax authorities. The company filed a refund application for the tax withholding and expects to receive the refund in approximately six months. The income tax receivable is included in other assets at March 31, 2017.

Note 2: The company's cost of product revenues includes \$13.9 million of expense in the first quarter of 2016 related to commercial milestones paid to the CFFT.

Note 3: In the three months ended March 31, 2017 and 2016, "Collaborations revenues and expenses" consisted of changes in the fair value of contingent payments due to variable interest entities ("VIEs"). In the three months ended March 31, 2017, "Collaboration revenues and expenses" also consisted of revenues and expenses associated with the company's oncology program including the company's collaboration with Merck KGaA, Darmstadt, Germany. The company has not adjusted its prior year Reconciliation of GAAP to Non-GAAP Revenues and Expenses for the three months ended March 31, 2016 for \$4.2 million of operating expenses related to its oncology program.

Note 4: In the three months ended March 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 three months ended March 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.

Note 5: The company consolidates the financial statements of two of its collaborators as VIEs as of March 31, 2017 and December 31, 2016. 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. 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.

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) clearance under the Hart-Scott-Rodino Antitrust Improvements Act with respect to our agreement with Concert and (ii) the development plan and timelines for our product development candidates. 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-E)

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