



October 25, 2016

Vertex Reports Third Quarter 2016 Financial Results

-Third quarter 2016 cystic fibrosis product revenues of \$410 million; \$234 million for ORKAMBI[®] (lumacaftor/ivacaftor) and \$176 million for KALYDECO[®] (ivacaftor)-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended September 30, 2016 and reviewed recent progress with its approved and investigational cystic fibrosis (CF) medicines. Vertex also reiterated its financial guidance for total 2016 ORKAMBI[®] and KALYDECO[®] revenues and expenses. Key financial results include:

	Three Months Ended September 30,		
	2016	2015	% Change
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	\$ 234	\$ 131	79%
KALYDECO product revenues, net	\$ 176	\$ 166	6%
TOTAL CF product revenues, net	\$ 410	\$ 297	38%
GAAP net loss	\$ (42)	\$ (95)	(56)%
GAAP net loss per share	\$ (0.17)	\$ (0.39)	(56)%
Non-GAAP net income (loss)	\$ 40	\$ (32)	N/A
Non-GAAP net income (loss) per share	\$ 0.16	\$ (0.13)	N/A

"Vertex continues to make significant progress with the key growth drivers for our business - increasing the number of people being treated with ORKAMBI and KALYDECO, expanding the number of people eligible for these medicines through label-expansions and developing new medicines to treat potentially all people with CF in the future," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Our progress toward treating more people with CF was marked by several important milestones in recent weeks, including the approval of ORKAMBI for children ages six to eleven in the U.S. and today's announcement regarding the advancement of our pipeline of next-generation correctors. Importantly, we're also continuing to generate important additional data about the long-term benefits of treating the underlying cause of CF with both ORKAMBI and KALYDECO."

Vertex today reviewed recent progress from across its CF program:

ORKAMBI

FDA approval of ORKAMBI for the treatment of children ages 6 to 11: On September 28, 2016 the U.S. Food and Drug Administration (FDA) approved ORKAMBI for the treatment of children ages 6 through 11 who have two copies of the F508del mutation. There are approximately 2,400 children ages 6 through 11 who have two copies of the F508del mutation in the U.S.

Data from Phase 3 efficacy study to support approval in children ages 6 to 11 in Europe expected by year-end: Vertex completed enrollment in a six-month Phase 3 efficacy study evaluating ORKAMBI in children ages 6 through 11 who have two copies of the F508del mutation and expects data from this study by the end of 2016. The primary endpoint of the study is the absolute change in lung clearance index. Pending data from the study, Vertex plans to submit a Marketing Authorization Application variation in the European Union in the first half of 2017. In Europe, there are approximately 3,400 children ages 6 through 11 who have two copies of the F508del mutation.

Tezacaftor (VX-661) in Combination with Ivacaftor

Enrollment complete in two Phase 3 studies of tezacaftor (VX-661); data expected in first half of 2017: Vertex

has now completed enrollment in two of three ongoing Phase 3 studies of the investigational combination of tezacaftor and ivacaftor. Enrollment is complete in the Phase 3 study in people ages 12 and older who have two copies of the F508del mutation and also in the Phase 3 study in people ages 12 and older who have one F508del mutation and one residual function mutation. Data from both studies are expected in the first half of 2017. The Phase 3 study of tezacaftor in combination with ivacaftor in people with one F508del mutation and one gating mutation is expected to complete enrollment in early 2017. Vertex plans to submit a New Drug Application (NDA) to the FDA for tezacaftor in combination with ivacaftor in the second half of 2017, pending data from the Phase 3 program.

Next-Generation Correctors

Planned initiation of Phase 2 studies in CF patients: In a separate press release issued today, Vertex announced that it plans to initiate two Phase 2 studies to evaluate the next-generation correctors VX-440 and VX-152 in triple combination regimens with tezacaftor (VX-661) and ivacaftor in people with cystic fibrosis (CF). Both studies are expected to start by the end of 2016. Additional details on the design of these studies were provided today in a separate press release.

Additional next-generation correctors moving into clinical development: Vertex also today announced that it plans to begin a Phase 1 study of VX-659, the company's third next-generation corrector, by the end of 2016 and to advance a fourth next-generation corrector into clinical development in 2017. Additional details were provided today in a separate press release.

Third Quarter 2016 Financial Highlights

Revenues:

- | Net product revenues from ORKAMBI were \$234.0 million compared to \$130.8 million for the third quarter of 2015. ORKAMBI was launched in the U.S. in July 2015.
- | Net product revenues from KALYDECO were \$175.6 million, compared to \$165.9 million for the third quarter of 2015.

Expenses:

- | GAAP operating expenses were \$435.5 million compared to \$379.8 million for the third quarter of 2015. Non-GAAP operating expenses (combined non-GAAP R&D and SG&A) were \$298.0 million compared to \$277.7 million for the third quarter of 2015. The increases were primarily driven by increased costs related to the progression of our CF pipeline and to increased investment in global commercial support for the launch of ORKAMBI.
- | GAAP R&D expenses were \$275.4 million compared to \$246.3 million for the third quarter of 2015. Non-GAAP R&D expenses were \$214.0 million compared to \$201.6 million for the third quarter of 2015. The increases were primarily driven by increased investment to progress our portfolio of CF medicines.
- | GAAP SG&A expenses were \$106.1 million compared to \$99.8 million for the third quarter of 2015. Non-GAAP SG&A expenses were \$84.0 million compared to \$76.1 million for the third 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 loss was \$(41.8) million, or \$(0.17) per diluted share, compared to GAAP net loss of \$(95.1) million, or \$(0.39) per diluted share, for the third quarter of 2015. Non-GAAP net income was \$40.1 million, or \$0.16 per diluted share, compared to a non-GAAP net loss of \$(31.9) million, or \$(0.13) per diluted share, for the third quarter of 2015.

Cash Position:

- | As of September 30, 2016, Vertex had \$1.13 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.
- | As of September 30, 2016, Vertex had \$300 million outstanding from a 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.

2016 Financial Guidance:

Vertex today reiterated its 2016 revenue guidance for ORKAMBI and KALYDECO. The company also reiterated guidance for its 2016 combined non-GAAP R&D and SG&A expenses. The guidance is summarized below:

- ▮ **ORKAMBI:** The company continues to expect total 2016 product revenues for ORKAMBI of \$950 to \$990 million.
- ▮ **KALYDECO:** The company continues to expect total 2016 product revenues for KALYDECO of \$685 to \$705 million. 2016 guidance for KALYDECO currently excludes any revenues related to the potential approval of KALYDECO for people in the U.S. who have residual function mutations.
- ▮ **Operating Expenses (Combined Non-GAAP R&D and SG&A Expenses):** Vertex continues to expect that its combined non-GAAP R&D and SG&A expenses in 2016 will be in the range of \$1.18 to \$1.23 billion. Vertex's expected non-GAAP R&D and SG&A expenses exclude stock-based compensation expense and certain other expenses.

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 non-GAAP research and development and sales, general, and administrative expenses. The company does not provide guidance regarding GAAP research and development and sales, general, and administrative expenses because of the difficulty of estimating stock-based compensation expenses, and predicting whether or not there will be additional expense items for which adjustments are appropriate. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

Vertex Pharmaceuticals Incorporated
Third Quarter Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2016	2015	2016	2015
Revenues:				
Product revenues, net	\$409,689	\$302,511	\$1,229,750	\$ 593,774
Royalty revenues	3,835	5,759	12,713	17,628
Collaborative revenues	259	1,546	1,008	2,999
Total revenues	<u>413,783</u>	<u>309,816</u>	<u>1,243,471</u>	<u>614,401</u>
Costs and expenses:				
Cost of product revenues (Note 1)	53,222	30,269	147,165	55,059
Royalty expenses	855	1,691	2,813	6,068
Research and development expenses	275,370	246,284	802,238	685,741
Sales, general and administrative expenses	106,055	99,772	322,921	280,026
Restructuring expenses	8	1,826	1,038	682
Total costs and expenses	<u>435,510</u>	<u>379,842</u>	<u>1,276,175</u>	<u>1,027,576</u>
Loss from operations	(21,727)	(70,026)	(32,704)	(413,175)
Interest expense, net	(20,140)	(21,134)	(60,993)	(63,552)
Other income (expenses), net	(167)	(1,326)	3,025	(5,025)
Loss from operations before provision for income taxes	<u>(42,034)</u>	<u>(92,486)</u>	<u>(90,672)</u>	<u>(481,752)</u>
Provision for income taxes	503	1,330	24,118	31,760
Net loss	<u>(42,537)</u>	<u>(93,816)</u>	<u>(114,790)</u>	<u>(513,512)</u>
Loss (income) attributable to noncontrolling interest	<u>696</u>	<u>(1,333)</u>	<u>(33,207)</u>	<u>30,909</u>

Net loss attributable to Vertex	<u>\$ (41,841)</u>	<u>\$ (95,149)</u>	<u>\$ (147,997)</u>	<u>\$ (482,603)</u>
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Amounts per share attributable to Vertex common shareholders:

Net loss:

Basic and diluted	\$ (0.17)	\$ (0.39)	\$ (0.61)	\$ (2.00)
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Shares used in per share calculations:

Basic and diluted	244,920	241,969	244,529	240,749
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Reconciliation of GAAP to Non-GAAP Net Income (Loss) Third Quarter Results

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
GAAP loss attributable to Vertex	<u>\$ (41,841)</u>	<u>\$ (95,149)</u>	<u>\$ (147,997)</u>	<u>\$ (482,603)</u>
Stock-based compensation expense	61,209	65,734	178,623	186,379
Real estate restructuring costs and income (Note 2)	121	214	696	(2,186)
HCV related revenues and costs (Note 3)	(2,448)	(7,734)	(3,257)	(18,207)
Other adjustments (Notes 4 and 5)	23,090	5,007	92,460	5,631
Non-GAAP net income (loss) attributable to Vertex	<u>\$ 40,131</u>	<u>\$ (31,928)</u>	<u>\$ 120,525</u>	<u>\$ (310,986)</u>

Amounts per diluted share attributable to Vertex common shareholders:

GAAP	\$ (0.17)	\$ (0.39)	\$ (0.61)	\$ (2.00)
Non-GAAP	\$ 0.16	\$ (0.13)	\$ 0.49	\$ (1.29)

Shares used in diluted per share calculations:

GAAP	244,920	241,969	244,529	240,749
Non-GAAP	248,009	241,969	247,433	240,749

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

(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
GAAP total revenues	<u>\$413,783</u>	<u>\$309,816</u>	<u>\$1,243,471</u>	<u>\$614,401</u>
HCV related revenues (Note 3)	(43)	(6,415)	(405)	(15,378)
Other adjustments (Note 4)	(203)	(1,105)	(850)	(1,379)
Non-GAAP total revenues	<u>\$413,537</u>	<u>\$302,296</u>	<u>\$1,242,216</u>	<u>\$597,644</u>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
GAAP cost of product revenues and royalty expenses	<u>\$ 54,077</u>	<u>\$ 31,960</u>	<u>\$ 149,978</u>	<u>\$ 61,127</u>
HCV related costs (Note 3)	16	1,546	(117)	(422)
Non-GAAP cost of product revenues and royalty expenses	<u>\$ 54,093</u>	<u>\$ 33,506</u>	<u>\$ 149,861</u>	<u>\$ 60,705</u>

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
GAAP research and development expenses	<u>\$275,370</u>	<u>\$246,284</u>	<u>\$ 802,238</u>	<u>\$685,741</u>
Stock-based compensation expense	(39,980)	(44,700)	(115,068)	(124,550)
HCV related costs (Note 3)	2,465	(294)	3,342	707
Other adjustments (Note 4)	(23,889)	298	(36,828)	(1,222)

Note 3: In the three and nine months ended September 30, 2016 and 2015, "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 September 30, 2016, "Other adjustments" was primarily attributable to payments for collaborations. In the nine months ended September 30, 2016, "Other adjustments" was primarily attributable to a \$58.5 million 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 and payments for collaborations and the acquisition of certain early stage assets.

Note 5: The company consolidates the financial statements of two of its collaborators as variable interest entities ("VIEs") as of September 30, 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.

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 of the following mutations in their CF gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N*, *S549R*, or *R117H*. KALYDECO is not for use in people with CF due to other mutations in the CF gene. KALYDECO is not effective in patients with CF with two copies of the *F508del* mutation (*F508del/F508del*) in the CF gene. 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 it 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/anti-anxiety medicines triazolam or midazolam; the immunosuppressant medicines everolimus, sirolimus, or tacrolimus; or St. John's wort.

Before taking ORKAMBI, patients should tell their doctor if they: have or have had liver problems; have kidney problems; have had an organ transplant; are using birth control (hormonal contraceptives, including oral, injectable, transdermal or implantable forms). Hormonal contraceptives 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. It is unknown if ORKAMBI causes dizziness. Patients should not drive a car, use machinery, or do anything requiring alertness until the patient knows how ORKAMBI affects them.

ORKAMBI can cause serious side effects including:

High liver enzymes in the blood, which can be a sign of liver injury, have been reported in patients receiving ORKAMBI. 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.

Respiratory events such as shortness of breath or chest tightness were observed in patients when starting ORKAMBI. If a patient has poor lung function, their doctor may monitor them more closely when starting ORKAMBI.

An increase in blood pressure has been seen in some patients treated with ORKAMBI. The patient's doctor should monitor their blood pressure during treatment with ORKAMBI.

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

The most common side effects of ORKAMBI include: shortness of breath and/or chest tightness; upper respiratory tract infection (common cold), including sore throat, stuffy or runny nose; gastrointestinal symptoms including nausea, diarrhea, or gas; rash; fatigue; flu or flu-like symptoms; increase in muscle enzyme levels; and irregular, missed, or abnormal menstrual periods and heavier bleeding.

Please click [here](#) to see the full Prescribing Information for ORKAMBI.

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 six 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 "2016 Financial Guidance" and statements regarding (i) the expected timing and clinical trial designs for ongoing and planned clinical studies of ORKAMBI, tezacaftor (VX-661), and the company's next-generation correctors, including VX-659, and (ii) the timing of regulatory applications, including NDA and MAAs. 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 2016 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)

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