



July 27, 2016

## Vertex Reports Second Quarter 2016 Financial Results

-Second quarter 2016 cystic fibrosis product revenues of \$426 million; \$245 million for ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor) and \$180 million for KALYDECO<sup>®</sup> (ivacaftor)-

-Vertex reiterates 2016 guidance for ORKAMBI product revenues of \$1.0 to \$1.1 billion and KALYDECO product revenues of \$685 to \$705 million-

-Pipeline of investigational CF medicines continues to progress and expand with addition of recent Moderna mRNA collaboration-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 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<sup>®</sup> and KALYDECO<sup>®</sup> revenues and expenses. Key financial results include:

	<b>Three Months Ended June 30,</b>		
	<b>2016</b>	<b>2015</b>	<b>% Change</b>
	(in millions, except per share and percentage data)		
<b>ORKAMBI product revenues, net</b>	\$ 245	\$ —	N/A
<b>KALYDECO product revenues, net</b>	\$ 180	\$ 155	16%
<b>TOTAL CF product revenues, net</b>	\$ 426	\$ 155	175%
<b>GAAP net loss</b>	\$ (65)	\$ (189)	(66)%
<b>GAAP net loss per share</b>	\$ (0.26)	\$ (0.78)	(67)%
<b>Non-GAAP net income (loss)</b>	\$ 58	\$ (131)	N/A
<b>Non-GAAP net income (loss) per share</b>	\$ 0.24	\$ (0.54)	N/A

"Just over a year ago, we received FDA approval for ORKAMBI, marking the most significant step to date in our journey to develop new medicines for potentially all people with CF," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Today, approximately 27,000 people are eligible for a medicine to treat the cause of their CF, and we're making significant progress toward bringing ORKAMBI and KALYDECO to even more patients while also advancing our pipeline of other potential medicines to enhance the future treatment of CF."

Vertex today reviewed recent progress from across its CF program:

### ORKAMBI

**Supplemental New Drug Application for the treatment of children ages 6 to 11 accepted for Priority Review by the U.S. FDA:** In late May 2016, the U.S. Food and Drug Administration (FDA) granted Vertex's request for Priority Review of a supplemental New Drug Application (sNDA) for approval of ORKAMBI for children ages 6 through 11 who have two copies of the F508del mutation. The FDA set a target review date of September 30, 2016 for a decision on the sNDA. There are approximately 2,400 children ages 6 through 11 who have two copies of the F508del mutation in the U.S. The sNDA was based on data from an open label Phase 3 safety study of ORKAMBI. Data from this study were presented at the 39<sup>th</sup> European Cystic Fibrosis Society (ECFS) conference on June 10, 2016.

**Enrollment complete in Phase 3 study in children ages 6 to 11 to support approval in Europe:** Vertex has 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. The primary endpoint 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.

**Initiation of Phase 3 study of ORKAMBI in children ages 2 to 5:** Vertex recently initiated a Phase 3 study of ORKAMBI in children ages 2 to 5. Similar to the study of KALYDECO in children in this age group, the first part of the two-part study is evaluating pharmacokinetics and safety to inform dose selection for the second part of the study. The primary endpoint of the second part of the study is safety and tolerability, with multiple efficacy measurements as secondary endpoints.

## **KALYDECO**

**Regulatory filing for patients with residual function mutations:** In October 2015, Vertex submitted an sNDA for approval of KALYDECO for treatment of people with CF ages 2 and older who have one of 23 residual function mutations and received a Complete Response Letter on this sNDA in February 2016. There are approximately 1,500 people ages 2 and older in the U.S. who have one of the 23 residual function mutations included in the sNDA, and Vertex continues to pursue FDA approval of KALYDECO for these patients as soon as possible.

## **VX-661 in Combination with Ivacaftor**

**Data from Phase 3 study in people with two copies of F508del mutation expected in first half of 2017:** Vertex today announced that it expects to complete enrollment of a 24-week Phase 3 placebo-controlled study evaluating the investigational combination of VX-661 and ivacaftor in people ages 12 and older who have two copies of the F508del mutation in August 2016. Data from this study are expected in the first half of 2017. The remaining three Phase 3 studies of VX-661 in combination with ivacaftor are proceeding as outlined in the company's April 27, 2016 press release. Vertex plans to submit a New Drug Application (NDA) to the FDA for VX-661 in combination with ivacaftor in the second half of 2017, pending data from the Phase 3 program.

## **Next-Generation Correctors**

**Ongoing Phase 1 studies in healthy volunteers:** Vertex's two next-generation correctors known as VX-152 and VX-440 are being evaluated alone and as part of a triple combination with VX-661 and ivacaftor in ongoing Phase 1 studies in healthy volunteers. Pending data from the Phase 1 studies, the company expects to begin Phase 2 clinical development in people with CF to evaluate one or both of the next-generation correctors with VX-661 and ivacaftor in the second half of 2016.

## **New Collaboration to Advance Future Treatment of CF**

**Collaboration with Moderna Therapeutics focused on mRNA Therapeutics for CF:** In early July, Vertex entered into an exclusive research collaboration and licensing agreement with Moderna Therapeutics aimed at the discovery and development of messenger Ribosomal Nucleic Acid (mRNA) therapies for the treatment of CF. The collaboration will focus on the use of mRNA therapies to treat the underlying cause of CF by enabling cells in the lungs to produce functional copies of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which is known to be defective in people with CF. As part of the collaboration, Vertex made an up-front payment of \$20 million to Moderna as well as a \$20 million equity investment. The investment will provide Vertex with an ownership stake in Moderna. Vertex will also pay Moderna future development and regulatory milestones of up to \$275 million, including \$220 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales.

## **Second Quarter 2016 Financial Highlights**

### **Revenues:**

- | Net product revenues from ORKAMBI were \$245.5 million. ORKAMBI was launched in the U.S. in July 2015.
- | Net product revenues from KALYDECO were \$180.2 million, compared to \$154.9 million for the second quarter of 2015.

### **Expenses:**

- | GAAP operating expenses were \$428.3 million compared to \$337.2 million for the second quarter of 2015. Non-GAAP operating expenses (combined non-GAAP R&D and SG&A) were \$306.3 million compared to \$253.9 million for the second 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 \$271.0 million compared to \$223.9 million for the second quarter of 2015. Non-GAAP R&D expenses were \$217.7 million compared to \$181.9 million for the second quarter of 2015. The increases were primarily driven by increased investment to progress our portfolio of CF medicines.
- GAAP SG&A expenses were \$111.7 million compared to \$94.4 million for the second quarter of 2015. Non-GAAP SG&A expenses were \$88.6 million compared to \$72.0 million for the second 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 \$(64.5) million, or \$(0.26) per diluted share, compared to GAAP net loss of \$(188.8) million, or \$(0.78) per diluted share, for the second quarter of 2015. Non-GAAP net income was \$58.0 million, or \$0.24 per diluted share, compared to a non-GAAP net loss of \$(130.7) million, or \$(0.54) per diluted share, for the second quarter of 2015.

#### **Cash Position:**

- As of June 30, 2016, Vertex had \$1.07 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 June 30, 2016, Vertex had \$300 million outstanding from a credit agreement, repayable by the end of the third quarter of 2017.

#### **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 \$1.0 to \$1.1 billion. As of June 30, 2016, approximately 6,000 patients had initiated treatment with ORKAMBI in the U.S. In addition to revenues from the use of ORKAMBI in patients ages 12 and older in the U.S., the 2016 ORKAMBI guidance also reflects potential revenues from the anticipated use of ORKAMBI in the U.S. for the treatment of people ages 6 to 11 who have two copies of the *F508del* mutation in the fourth quarter of 2016, pending FDA approval, and revenues from sales of ORKAMBI outside the U.S., primarily in Germany.
- 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**  
**Second Quarter Results**  
**Consolidated Statements of Operations Data**  
(in thousands, except per share amounts)  
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Revenues:				
Product revenues, net	\$ 425,651	\$ 160,388	\$ 820,061	\$ 291,263
Royalty revenues	5,282	5,077	8,878	11,869
Collaborative revenues	675	611	749	1,453
Total revenues	431,608	166,076	829,688	304,585
Costs and expenses:				
Cost of product revenues (Note 1)	44,154	15,409	93,943	24,790
Royalty expenses	1,098	1,451	1,958	4,377
Research and development expenses	271,008	223,858	526,868	439,457
Sales, general and administrative expenses	111,652	94,394	216,866	180,254
Restructuring expenses (income)	343	2,128	1,030	(1,144)
Total costs and expenses	428,255	337,240	840,665	647,734
Income (loss) from operations	3,353	(171,164)	(10,977)	(343,149)
Interest expense, net	(20,155)	(21,111)	(40,853)	(42,418)
Other (expenses) income, net	(1,219)	1,414	3,192	(3,699)
Loss from operations before provision for income taxes	(18,021)	(190,861)	(48,638)	(389,266)
Provision for income taxes	18,130	30,131	23,615	30,430
Net loss	(36,151)	(220,992)	(72,253)	(419,696)
(Income) loss attributable to noncontrolling interest	(28,374)	32,144	(33,903)	32,242
Net loss attributable to Vertex	<u>\$ (64,525)</u>	<u>\$ (188,848)</u>	<u>\$ (106,156)</u>	<u>\$ (387,454)</u>

Amounts per share attributable to Vertex common shareholders:

Net loss:

Basic and diluted	\$ (0.26)	\$ (0.78)	\$ (0.43)	\$ (1.61)
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Shares used in per share calculations:

Basic and diluted	244,482	240,757	244,124	240,129
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**Reconciliation of GAAP to Non-GAAP Net Income/(Loss)**  
**Second Quarter Results**

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
<b>GAAP loss attributable to Vertex</b>	<u>\$ (64,525)</u>	<u>\$ (188,848)</u>	<u>\$ (106,156)</u>	<u>\$ (387,454)</u>
Stock-based compensation expense	61,942	63,261	117,414	120,645
Real estate restructuring costs and income (Note 2)	137	1,178	575	(2,400)
HCV related revenues and costs (Note 3)	627	(6,004)	(810)	(10,473)
Other adjustments (Notes 4 and 5)	59,791	(270)	69,371	623
<b>Non-GAAP net income (loss) attributable to Vertex</b>	<u>\$ 57,972</u>	<u>\$ (130,683)</u>	<u>\$ 80,394</u>	<u>\$ (279,059)</u>

Amounts per diluted share attributable to Vertex common shareholders:

GAAP	\$ (0.26)	\$ (0.78)	\$ (0.43)	\$ (1.61)
Non-GAAP	\$ 0.24	\$ (0.54)	\$ 0.33	\$ (1.16)

Shares used in diluted per share calculations:

GAAP	244,482	240,757	244,124	240,129
Non-GAAP	246,426	240,757	246,872	240,129

**Reconciliation of GAAP to Non-GAAP Revenues and Expenses  
Second Quarter Results**

(in thousands)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP total revenues</b>	\$ 431,608	\$ 166,076	\$ 829,688
HCV related revenues (Note 3)	489	(6,094)	(362)	(8,963)
Other adjustments (Note 4)	(573)	(74)	(647)	(274)
<b>Non-GAAP total revenues</b>	<u>\$ 431,524</u>	<u>\$ 159,908</u>	<u>\$ 828,679</u>	<u>\$ 295,348</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP cost of product revenues and royalty expenses</b>	\$ 45,252	\$ 16,860	\$ 95,901
HCV related costs (Note 3)	6	(371)	(133)	(1,968)
<b>Non-GAAP cost of product revenues and royalty expenses</b>	<u>\$ 45,258</u>	<u>\$ 16,489</u>	<u>\$ 95,768</u>	<u>\$ 27,199</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP research and development expenses</b>	\$ 271,008	\$ 223,858	\$ 526,868
Stock-based compensation expense	(40,640)	(41,632)	(75,088)	(79,849)
HCV related costs (Note 3)	51	512	877	1,000
Other adjustments (Note 4)	(12,746)	(827)	(12,937)	(1,520)
<b>Non-GAAP research and development expenses</b>	<u>\$ 217,673</u>	<u>\$ 181,911</u>	<u>\$ 439,720</u>	<u>\$ 359,088</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP sales, general and administrative expenses</b>	\$ 111,652	\$ 94,394	\$ 216,866
Stock-based compensation expense	(21,302)	(21,629)	(42,326)	(40,796)
HCV related costs (Note 3)	(61)	(54)	(29)	2,851
Other adjustments (Note 4)	(1,698)	(695)	(2,241)	(1,147)
<b>Non-GAAP sales, general and administrative expenses</b>	<u>\$ 88,591</u>	<u>\$ 72,016</u>	<u>\$ 172,270</u>	<u>\$ 141,162</u>
<b>Combined non-GAAP R&amp;D and SG&amp;A expenses</b>	<u>\$ 306,264</u>	<u>\$ 253,927</u>	<u>\$ 611,990</u>	<u>\$ 500,250</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP interest expense, net and other expense, net</b>	\$ (21,374)	\$ (19,697)	\$ (37,661)
Other adjustments (Note 4)	(36)	—	177	—
<b>Non-GAAP interest expense, net and other expense, net</b>	<u>\$ (21,410)</u>	<u>\$ (19,697)</u>	<u>\$ (37,484)</u>	<u>\$ (46,117)</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
	<b>GAAP provision for income taxes</b>	\$ 18,130	\$ 30,131	\$ 23,615
Other adjustments (Note 4)	(17,510)	(29,653)	(20,572)	(29,589)
<b>Non-GAAP provision for income taxes</b>	<u>\$ 620</u>	<u>\$ 478</u>	<u>\$ 3,043</u>	<u>\$ 841</u>

## Condensed Consolidated Balance Sheets Data

(in thousands)

(unaudited)

	<b>June 30, 2016</b>	<b>December 31, 2015</b>
<b>Assets</b>		
Cash, cash equivalents and marketable securities	\$ 1,071,436	\$ 1,042,462
Restricted cash and cash equivalents (VIE) (Note 5)	70,513	78,910
Accounts receivable, net	189,356	177,639
Inventories	66,589	57,207
Property and equipment, net	690,607	697,715
Intangible assets and goodwill	334,724	334,724
Other assets	125,757	109,930
<b>Total assets</b>	<b>\$ 2,548,982</b>	<b>\$ 2,498,587</b>
<b>Liabilities and Shareholders' Equity</b>		
Other liabilities	\$ 394,870	\$ 426,482
Deferred tax liability	132,810	110,439
Accrued restructuring expense	12,484	15,358
Deferred revenues	18,879	26,010
Capital leases	51,763	58,468
Fan Pier lease obligation	472,834	473,043
Senior secured term loan	296,497	295,159
Shareholders' equity	1,168,845	1,093,628
<b>Total liabilities and shareholders' equity</b>	<b>\$ 2,548,982</b>	<b>\$ 2,498,587</b>
Common shares outstanding	247,704	246,307

**Note 1 :** Cost of product revenues in the six months ended June 30, 2016 includes the second and final \$13.9 million commercial milestone that was earned by CFFT in the first quarter of 2016 related to sales of ORKAMBI.

**Note 2:** The company excludes restructuring expense (income) from its non-GAAP income (loss) attributable to Vertex. In the three and six months ended June 30, 2016 and 2015, "Real estate restructuring costs and income" consisted of restructuring charges related primarily to the company's relocation from Cambridge to Boston, Massachusetts.

**Note 3:** In the three and six months ended June 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 and six months ended June 30, 2016, "Other adjustments" was primarily attributable to a \$48.4 million and \$57.4 million, respectively, 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. "Other adjustments" also includes payments for 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 June 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.

**U.S. INDICATION AND IMPORTANT SAFETY INFORMATION FOR ORKAMBI<sup>®</sup> (lumacaftor/ivacaftor) TABLETS**

ORKAMBI is a combination of lumacaftor and ivacaftor indicated for the treatment of cystic fibrosis (CF) in patients age 12 years and older who are homozygous for the F508del mutation in the CFTR gene. The efficacy and safety of ORKAMBI have not been established in patients with CF other than those homozygous for the F508del mutation.

Worsening of liver function, including hepatic encephalopathy, in patients with advanced liver disease has been reported in some patients with CF while receiving ORKAMBI.

Serious adverse reactions related to elevated transaminases have been reported in patients with CF receiving ORKAMBI and, in some instances, associated with concomitant elevations in total serum bilirubin.

Respiratory events (e.g., chest discomfort, shortness of breath, and chest tightness) were observed more commonly in patients during initiation of ORKAMBI compared to those who received placebo. Clinical experience in patients with percent predicted FEV1 < 40 is limited, and additional monitoring of these patients is recommended during initiation of therapy.

Co-administration of ORKAMBI with sensitive CYP3A substrates or CYP3A substrates with a narrow therapeutic index is not recommended as ORKAMBI may reduce their effectiveness. ORKAMBI may substantially decrease hormonal contraceptive exposure, reducing their effectiveness and increasing the incidence of menstruation-associated adverse reactions. Co-administration with strong CYP3A inducers is not recommended as they may reduce the therapeutic effectiveness of ORKAMBI.

Abnormalities of the eye lens (cataracts) have been reported in pediatric patients treated with ivacaftor, a component of ORKAMBI.

Increased blood pressure has been observed in some patients treated with ORKAMBI. Blood pressure should be monitored periodically in all patients being treated with ORKAMBI.

The most common adverse reactions associated with ORKAMBI include shortness of breath, sore throat, nausea, diarrhea, upper respiratory tract infection, fatigue, chest tightness, increased blood creatinine phosphokinase, rash, flatulence, runny nose, and influenza.

Please see the [full prescribing information](#) for ORKAMBI.

## **U.S. INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO<sup>®</sup> (ivacaftor)**

KALYDECO is a cystic fibrosis transmembrane conductance regulatory (CFTR) potentiator indicated for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have one of the following mutations in the CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R or R117H.

KALYDECO is not effective in patients with CF with 2 copies of the F508del mutation (F508del/F508del) in the CFTR gene. The safety and efficacy of KALYDECO in children with CF younger than 2 years of age have not been studied. The use of KALYDECO in children under the age of 2 years is not recommended.

High liver enzymes (transaminases; ALT and AST) have been reported in patients with CF receiving KALYDECO.

Use of KALYDECO with medicines that are strong CYP3A inducers substantially decreases exposure of KALYDECO and may diminish effectiveness. Therefore, co-administration is not recommended. The dose of KALYDECO must be adjusted when used concomitantly with strong and moderate CYP3A inhibitors or when used in patients with moderate or severe hepatic disease.

Cases of non-congenital lens opacities/cataracts have been reported in pediatric patients treated with KALYDECO.

The most common side effects associated with KALYDECO include headache; upper respiratory tract infection (common cold), including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

Please see the [full prescribing information](#) for KALYDECO.

## **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](http://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, KALYDECO, VX-661, VX-152 and VX-440, (ii) the timing of regulatory applications, including NDAs, sNDAs and MAAs and the status of interactions with regulatory authorities and (iii) the collaboration with Moderna. 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](http://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](http://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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