April 27, 2016

Vertex Reports First Quarter 2016 Financial Results

-First quarter 2016 cystic fibrosis product revenues of $394 million; $223 million for ORKAMBI® (lumacaftor/ivacaftor) and $171 million for KALYDECO® (ivacaftor)-

-Provides 2016 guidance for ORKAMBI product revenues of $1.0 to $1.1 billion; increases 2016 guidance for KALYDECO product revenues to $685 to $705 million-

-Provides update on approved CF medicines and pipeline of investigational medicines-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended March 31, 2016 and provided an update on its approved CF medicines and other investigational medicines. Vertex also provided financial guidance for 2016 ORKAMBI® net revenues and increased its prior guidance for 2016 KALYDECO® net revenues. Key financial results include:

	Three Months Ended March 31,		
	2016	2015	% Change
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	$ 223	$ —	N/A
KALYDECO product revenues, net	$ 171	$ 130	31%
TOTAL CF product revenues, net	$ 394	$ 130	202%
GAAP net loss	$ (42)	$ (199)	(79)%
GAAP net loss per share	$ (0.17)	$ (0.83)	(80)%
Non-GAAP net income (loss)	$ 22	$ (148)	N/A
Non-GAAP net income (loss) per share	$ 0.09	$ (0.62)	N/A

"2016 marks an important transition for Vertex following the launch of ORKAMBI. With recent approvals and label expansions, there are now approximately 27,000 people with CF eligible to take ORKAMBI or KALYDECO. The number of CF patients eligible for and initiating treatment is driving significant revenue growth for a second straight year, and we expect this trend to continue in 2017 and beyond," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "In addition, we believe our portfolio of approved and pipeline medicines has the potential to treat the vast majority of people with CF. We remain focused on investing to create transformative future medicines and generating continued earnings growth in the years ahead."

CF Medicines and Pipeline Update

Vertex today provided the following updates for ORKAMBI, KALYDECO and the company's progress toward developing new medicines with the goal of treating all people with CF:

ORKAMBI

Additional Regulatory Approvals Support Expansion Efforts: During the first quarter of 2016, Vertex received regulatory approval for ORKAMBI for the treatment of people with CF ages 12 and older who have two copies of the *F508del* mutation in Canada and Australia, where together there are approximately 2,500 people who are eligible for treatment with ORKAMBI. Vertex has now begun the reimbursement approval process in these countries.

Supplemental New Drug Application in Children Ages 6 to 11: In late March 2016, Vertex submitted a supplemental New Drug Application (sNDA) to the FDA for the approval of ORKAMBI for treatment of children with CF ages 6 to 11

who have two copies of the *F508del* mutation. The submission included a request for Priority Review, which if granted would shorten the FDA's anticipated review time from 10 to six months. There are approximately 2,400 children ages 6 to 11 who have two copies of the *F508del* mutation in the U.S.

Vertex has submitted data from an open label Phase 3 clinical safety study of ORKAMBI in children ages 6 to 11 who have two copies of the *F508del* mutation for presentation at the 39th European Cystic Fibrosis Society Conference (ECFS), June 8 - 11 in Basel, Switzerland.

Enrollment Complete in Phase 3 Study of Children Ages 6 to 11: Vertex has completed enrollment in a six-month Phase 3 efficacy study evaluating ORKAMBI in approximately 200 children ages 6 to 11 who have two copies of the *F508del* mutation. Pending data from the study, Vertex plans to submit a Marketing Authorization Application (MAA) variation in Europe in the first half of 2017 for approval of ORKAMBI for use in this age group. There are approximately 3,400 children ages 6 to 11 who have two copies of the *F508del* mutation in the European Union.

KALYDECO

Study in Children Under Two Years of Age: Vertex has initiated a Phase 3 clinical study of KALYDECO in children under 2 years of age to evaluate the effect of KALYDECO on markers of CF in young children. The study will utilize a weight-based dose of KALYDECO granules that can be mixed in soft foods or liquids. The study will enroll infants with one of the 10 mutations for which KALYDECO is currently approved.

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. The company is in ongoing discussions with the FDA regarding a Complete Response Letter it received 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.

Pipeline of Investigational Medicines for CF

VX-661 - *Broad Phase 3 program ongoing in multiple groups of people with CF*

Vertex provided the following updates on the Phase 3 studies of the investigational combination of VX-661 and ivacaftor in multiple different groups of people with CF who have at least one copy of the *F508del* mutation:

- Enrollment in the study in people with two copies of the *F508del* mutation is expected to be complete in mid-2016, and data from this Phase 3 study are expected in early 2017.
- Enrollment is ongoing in the Phase 3 study of VX-661 in combination with ivacaftor in patients with one copy of the *F508del* mutation and a second mutation that results in a gating defect. Vertex plans to complete enrollment of this study in late 2016 or early 2017.
- Vertex has revised its enrollment target for the Phase 3 study of VX-661 in combination with ivacaftor in patients with one copy of the *F508del* mutation and a second mutation that results in residual CFTR function. The original expectation was for up to 300 patients to enroll in this study. Vertex now plans to enroll approximately 200 patients. Enrollment is expected to be complete in the second half of 2016.
- Enrollment is complete in Part A of the study in people with one copy of the *F508del* mutation and a second mutation that results in minimal CFTR function. An interim futility analysis of efficacy data from Part A of this study is expected to be completed in the third quarter of 2016.

In addition to evaluating the efficacy of the combination regimen, these Phase 3 studies will also provide safety data on the combination of VX-661 and ivacaftor to support the planned development of a triple combination regimen that includes a next-generation corrector in combination with VX-661 and ivacaftor.

VX-371 - *Enrollment ongoing in Phase 2 study of VX-371 in combination with ORKAMBI*

Vertex today announced data from an exploratory Phase 2, 14-day study of its inhaled epithelial sodium channel (ENaC) inhibitor, VX-371 (P-1037), being developed in collaboration with Parion Sciences. The study dosed 142 people ages 12 and older with a confirmed diagnosis of CF. There was no restriction based on *CFTR* mutation. 136 people completed the study. Patients were not using any CFTR modulator therapy immediately before or during the study. The primary endpoint of the study was safety compared to placebo. Secondary endpoints evaluated the effect on mean absolute forced expiratory volume in one second (FEV_1) and patient-reported respiratory symptoms as reported in the CF questionnaire-revised (CFQ-R). The study met its primary safety endpoint, and safety data from the study showed

that VX-371 was generally well tolerated. There were no statistically significant changes in FEV_1 or CFQ-R for those who received VX-371.

The clinical safety data announced today provide support for the company's ongoing placebo-controlled Phase 2a study evaluating VX-371 in combination with ORKAMBI, both with and without the addition of hypertonic saline. This study is expected to enroll approximately 150 people with CF ages 12 and older who have two copies of the F508del mutation. The primary endpoints of the Phase 2a study are safety and mean absolute change from baseline in FEV_1 at day 28 compared to placebo.

In vitro, VX-371 showed a meaningful change in cilia beat frequency when VX-371 was used in combination with ORKAMBI in human bronchial epithelial cells with two copies of the F508del mutation, but did not show a meaningful change in cilia beat frequency when VX-371 was used alone.

Next-Generation Correctors - *Phase 1 studies in healthy volunteers progressing as planned*

In the fourth quarter of 2015, Vertex initiated clinical development of two next-generation correctors known as VX-152 and VX-440. Both 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 successful completion of the Phase 1 studies of VX-152 and VX-440, the company expects to begin Phase 2 proof-of-concept studies in combination with VX-661 and ivacaftor in the second half of 2016.

CRISPR Collaboration - *Gene editing collaboration focused on discovering potential treatments to address the mutations and genes known to cause and contribute to CF*

In October 2015, Vertex entered into a strategic research collaboration with CRISPR Therapeutics focused on the use of CRISPR's gene editing technology, known as CRISPR-Cas9, to discover and develop potential new treatments aimed at the underlying genetic causes of human disease. The collaboration will evaluate the use of CRISPR-Cas9 across multiple diseases where targets have been validated through human genetics. As part of the collaboration, Vertex and CRISPR will evaluate the use of CRISPR-Cas9 to potentially correct the mutations in the *CFTR* gene known to result in the defective protein that causes CF and to edit other genes that contribute to the disease.

Other Research and Development Programs

Beyond CF, Vertex is advancing research and development programs focused on the treatment of key mechanisms in serious diseases. The company today provided the following updates to its pipeline programs:

Oncology

VX-970: VX-970 is an inhibitor of ATR, a critical regulator of the DNA damage repair system. Vertex presented data from a Phase 1 trial of VX-970 in combination with cisplatin in patients with advanced solid tumors at the Annual Association for Cancer Research (AACR) meeting on April 17, 2016.

Vertex is currently conducting two Phase 1/2 studies that are enrolling specific cohorts of triple-negative breast cancer patients and non-small cell lung cancer patients. In these studies, VX-970 is being dosed in combination with commonly used DNA-damaging repair therapies.

Vertex has also entered into two cooperative research and development agreements (CRADAs) with the National Cancer Institute to support evaluation of VX-970 across other types of cancers. The CRADA enables NCI to conduct multiple clinical studies that will evaluate treatment with VX-970 in people with small cell lung, head and neck, bladder, ovarian and other cancers.

Pain

VX-150: Vertex is developing VX-150 as a potential medicine for the treatment of pain. VX-150 is designed to block pain signaling through inhibition of a sodium channel known as NaV 1.8. In the first quarter of 2016, Vertex initiated a six-week crossover Phase 2 proof-of-concept study of VX-150 in approximately 100 people with symptomatic osteoarthritis of the knee. Vertex expects to complete enrollment of this study in the second half of 2016.

Acute Spinal Cord Injury

VX-210: In the first quarter of 2016, Vertex initiated a randomized, double-blind, placebo controlled Phase 2b/3 study to evaluate the efficacy and safety of VX-210 in patients with certain acute cervical spinal cord injuries. Vertex is developing VX-210 as a potential medicine for acute spinal cord injury. VX-210 is designed to inhibit a protein known as Rho that blocks neural regeneration after injury.

First Quarter 2016 Financial Highlights

Revenues:

- Net product revenues from ORKAMBI were $223.1 million. ORKAMBI was launched in the U.S. in July 2015.
- Net product revenues from KALYDECO were $170.5 million, compared to $130.2 million for the first quarter of 2015.

Expenses:

- Non-GAAP research and development (R&D) expenses were $222.0 million compared to $177.2 million for the first quarter of 2015. The increase was primarily driven by increased investment to progress our portfolio of CF medicines. GAAP R&D expenses, including stock-based compensation expense, were $255.9 million compared to $215.6 million for the first quarter of 2015.
- Non-GAAP sales, general and administrative (SG&A) expenses were $83.7 million compared to $69.1 million for the first quarter of 2015. The increase was primarily driven by increased investment to support the global launch of ORKAMBI. GAAP SG&A expenses, including stock-based compensation expense, were $105.2 million compared to $85.9 million for the first quarter of 2015.

Net Income (Loss) Attributable to Vertex:

- Non-GAAP net income was $22.4 million, or $0.09 per diluted share, compared to a non-GAAP net loss of $148.4 million, or $0.62 per diluted share, for the first quarter of 2015. The GAAP net loss, including stock-based compensation expense, was $41.6 million, or $0.17 per diluted share, compared to Vertex's GAAP net loss of $198.6 million, or $0.83 per diluted share, for the first quarter of 2015.

Cash Position:

- As of March 31, 2016, Vertex had $1.03 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 March 31, 2016, Vertex had $300 million outstanding from a credit agreement, repayable by the end of the third quarter of 2017. The agreement allows for the facility to increase to up to $500 million.

2016 Financial Guidance:

Vertex today provided 2016 revenue guidance for ORKAMBI and increased 2016 revenue guidance for 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 anticipates total 2016 product revenues for ORKAMBI of $1.0 to $1.1 billion. This guidance is based on the company's understanding of treatment patterns from the launch of ORKAMBI to date, including:

- **Uptake:** Approximately 65% of the 8,500 eligible patients in the U.S. have initiated treatment as of March 31, 2016. Vertex continues to expect the vast majority of eligible patients ages 12 and older in the U.S. will initiate treatment by the end of 2016.
- **Persistence:** Of the patients who have started on treatment, approximately 15% discontinued treatment within the first three months of initiation. The company projects that the proportion of all patients who initiate and remain on treatment will stabilize at approximately 70% to 80%.
- **Compliance:** The overall compliance rate, which reflects the number of pills actually taken by a patient in a given month, is expected to be between 70% to 80%.

2016 ORKAMBI guidance also reflects potential revenues from the anticipated approval 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 second half of 2016 and revenues from sales of ORKAMBI outside the U.S., primarily in Germany.

KALYDECO: Vertex today increased its guidance for 2016 revenues of KALYDECO. The company now expects product revenues of $685 to $705 million. The prior range, provided on January 10, 2016, was for KALYDECO product revenues of $670 to $690 million for 2016.

The change in KALYDECO guidance reflects:

- A continued increase in the number of patients initiating treatment with KALYDECO globally
- A reduced impact from the VX-661 Phase 3 program

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, Excluding Cost of Revenues (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 exclude stock-based compensation expense, 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. 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,	
	2016	**2015**
Revenues:		
Product revenues, net	$ 394,410	$ 130,875
Royalty revenues	3,596	6,792
Collaborative revenues	74	842
Total revenues	398,080	138,509
Costs and expenses:		
Cost of product revenues (Note 1)	49,789	9,381
Royalty expenses	860	2,926
Research and development expenses	255,860	215,599
Sales, general and administrative expenses	105,214	85,860
Restructuring expenses (income)	687	(3,272)
Total costs and expenses	412,410	310,494
Loss from operations	(14,330)	(171,985)
Interest expense, net	(20,698)	(21,307)
Other income (expenses), net	4,411	(5,113)
Loss from operations before provision for income taxes	(30,617)	(198,405)
Provision for income taxes	5,485	299
Net loss	(36,102)	(198,704)
(Income) loss attributable to noncontrolling interest	(5,529)	98
Net loss attributable to Vertex	$ (41,631)	$ (198,606)

Amounts per share attributable to Vertex common shareholders:		
Net loss:		
Basic and diluted	$ (0.17)	$ (0.83)
Shares used in per share calculations:		
Basic and diluted	243,831	239,493

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

	Three Months Ended March 31,	
	2016	**2015**
GAAP loss attributable to Vertex	$ (41,631)	$ (198,606)
Stock-based compensation expense	55,472	57,384
Real estate restructuring costs and income (Note 2)	436	(3,567)
HCV related revenues and costs (Note 3)	(1,436)	(4,469)
Other adjustments (Notes 4 and 5)	9,581	882
Non-GAAP net income (loss) attributable to Vertex	$ 22,422	$ (148,376)
Amounts per diluted share attributable to Vertex common shareholders:		
GAAP	$ (0.17)	$ (0.83)
Non-GAAP	$ 0.09	$ (0.62)
Shares used in diluted per share calculations:		
GAAP	243,831	239,493
Non-GAAP	246,680	239,493

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

	Three Months Ended March 31,	
	2016	**2015**
GAAP total revenues	$ 398,080	$ 138,509
HCV related revenues (Note 3)	(851)	(2,869)
Other adjustments (Note 4)	(74)	(200)
Non-GAAP total revenues	$ 397,155	$ 135,440

	Three Months Ended March 31,	
	2016	**2015**
GAAP cost of product revenues and royalty expenses	$ 50,649	$ 12,307
HCV related costs (Note 3)	(139)	(1,596)
Non-GAAP cost of product revenues and royalty expenses	$ 50,510	$ 10,711
GAAP research and development expenses	$ 255,860	$ 215,599
Stock-based compensation expense	(34,448)	(38,217)
HCV related costs (Note 3)	826	488
Other adjustments (Note 4)	(192)	(696)
Non-GAAP research and development expenses	$ 222,046	$ 177,174
GAAP sales, general and administrative expenses	$ 105,214	$ 85,860

Stock-based compensation expense	(21,024)	(19,167)
HCV related costs (Note 3)	32	2,904
Other adjustments (Note 4)	(543)	(448)
Non-GAAP sales, general and administrative expenses	$ 83,679	$ 69,149
Combined non-GAAP R&D and SG&A expenses	$ 305,725	$ 246,323

	Three Months Ended March 31,	
	2016	**2015**
GAAP interest expense, net and other expense, net	$ (16,287)	$ (26,420)
Other adjustments (Note 4)	211	—
Non-GAAP interest expense, net and other expense, net	$ (16,076)	$ (26,420)
GAAP provision for income taxes	$ 5,485	$ 299
Other adjustments (Note 4)	(3,063)	63
Non-GAAP provision for income taxes	$ 2,422	$ 362

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

	March 31, 2016	**December 31, 2015**
Assets		
Cash, cash equivalents and marketable securities	$ 1,025,618	$ 1,042,462
Restricted cash and cash equivalents (VIE) (Note 5)	76,273	78,910
Accounts receivable, net	181,878	177,639
Inventories	63,200	57,207
Property and equipment, net	690,521	697,715
Intangible assets and goodwill	334,724	334,724
Other assets	115,692	109,930
Total assets	$ 2,487,906	$ 2,498,587
Liabilities and Shareholders' Equity		
Other liabilities	$ 397,379	$ 426,482
Deferred tax liability	112,259	110,439
Accrued restructuring expense	13,935	15,358
Deferred revenues	23,195	26,010
Capital leases	56,178	58,468
Fan Pier lease obligation	472,940	473,043
Senior secured term loan	295,822	295,159
Shareholders' equity	1,116,198	1,093,628
Total liabilities and shareholders' equity	$ 2,487,906	$ 2,498,587
Common shares outstanding	247,287	246,307

Note 1 : Cost of product revenues 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 from its non-GAAP income (loss) attributable to Vertex restructuring expense (income). In the three months ended March 31, 2016, "Real estate restructuring costs and income" consisted of restructuring charges, related to the company's relocation from Cambridge to Boston, Massachusetts. In the three months ended March 31, 2015, "Real estate restructuring costs and income" consisted of restructuring credits of $3.6 million primarily related to the company's relocation from Cambridge to Boston, Massachusetts.

Note 3: In the three months ended March 31, 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 March 31, 2016, "Other adjustments" was primarily attributable to changes in the fair value of contingent milestone payments and royalties payable by Vertex to two variable interest entities ("VIEs"). In the three months ended March 31, 2015, "Other adjustments" was primarily attributable to development costs associated with VX-509.

Note 5: The company consolidates the financial statements of two of its collaborators as VIEs as of March 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.

U.S. INDICATION AND IMPORTANT SAFETY INFORMATION FOR ORKAMBI® (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.

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® (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.

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) 2016 guidance for ORKAMBI and KALYDECO, (ii) the expected timing and clinical trial designs for ongoing and planned clinical studies of ORKAMBI, KALYDECO, VX-661 VX-371, VX-152, VX-440, VX-970, VX-150 and VX-210, (iii) Vertex's expectations regarding uptake, persistence and compliance with respect to ORKAMBI, (iv) the timing of regulatory applications, including sNDAs and MAAs and the status of interactions with regulatory authorities and (v) Vertex's plans to submit and/or present data at scientific conferences. 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 5:00 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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