July 29, 2015

Vertex Reports Second Quarter 2015 Financial Results

-Second quarter 2015 total revenues of $166 million, including net product revenues of approximately $155 million for KALYDECO® (ivacaftor) in cystic fibrosis-

-Vertex increases guidance for total 2015 KALYDECO net revenues; now expects KALYDECO revenues of $575 to $590 million-

-ORKAMBI™ (lumacaftor/ivacaftor) launch underway following FDA approval on July 2-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2015. Vertex also increased its financial guidance for total 2015 KALYDECO® (ivacaftor) revenues and reiterated its prior guidance for non-GAAP operating expenses. Key financial results include:

	Three Months Ended June 30,		
	2015	2014	% Change
	(in millions, except per share and percentage data)		
KALYDECO product revenues, net	$ 154.9	$ 113.1	37%
GAAP net loss	$ (188.8)	$ (159.4)	18%
GAAP net loss per share	$ (0.78)	$ (0.68)	15%
Non-GAAP net loss	$ (130.7)	$ (141.7)	(8)%
Non-GAAP net loss per share	$ (0.54)	$ (0.61)	(11)%

"With the recent approval of ORKAMBI and continued label and geographic expansion for KALYDECO, we have made significant progress toward our goals of treating many more people with cystic fibrosis and positioning the company for long-term revenue and earnings growth," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "As we enter the second half of the year, we remain focused on advancing key pipeline programs in CF including VX-661, the ENaC inhibitor VX-371 (P-1037) and our next-generation correctors, and on bringing forward potential new medicines in multiple other diseases."

Vertex today provided the following updates:

ORKAMBI™ (lumacaftor/ivacaftor)

On July 2, 2015, the U.S. Food and Drug Administration (FDA) approved ORKAMBI (lumacaftor/ivacaftor) for the treatment of cystic fibrosis (CF) in people ages 12 and older with two copies of the F508del mutation. Patients have now begun to receive ORKAMBI. Outside of the U.S., Vertex has submitted ORKAMBI for regulatory approval in the European Union, Australia and Canada. A decision by the European Medicines Agency (EMA) is anticipated by the end of 2015. Reviews by Health Canada and Australia's Therapeutic Goods Administration (TGA) are ongoing.

Studies of Lumacaftor in Combination with Ivacaftor in Children Ages 6 to 11

Vertex is currently conducting two Phase 3 clinical studies of lumacaftor in combination with ivacaftor in children 6 to 11 years of age. The first study is evaluating lumacaftor in combination with ivacaftor in approximately 50 children in the U.S. to support the potential FDA approval in children ages 6 to 11. The primary endpoint of this six-month study is safety. This study is fully enrolled, and pending data, Vertex plans to submit a supplemental New Drug Application (sNDA) to the FDA in the first half of 2016. In Europe, an efficacy study is required to support approval in children ages 6 to 11, and Vertex recently initiated a study to evaluate lumacaftor in combination with ivacaftor in these patients to support potential approval in Europe. The six-month study will evaluate lumacaftor in combination with ivacaftor in approximately 200 children at sites in North America, Europe and Australia. The primary endpoint of the second study is the absolute change in lung clearance index.

KALYDECO® (ivacaftor)

Throughout the first half of 2015, Vertex completed reimbursement discussions in multiple key countries in Europe to enable patients with non-G551D gating mutations to receive KALYDECO. Vertex is currently awaiting a decision on its applications for European Union approval of KALYDECO for use in people ages 18 and older with the R117H mutation and in children ages 2 to 5 with one of nine gating mutations.

Phase 3 Studies Investigating VX-661 in Combination with Ivacaftor

Vertex has initiated four 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. The studies are evaluating VX-661 dosed as 100 mg once daily (QD) in combination with ivacaftor dosed as 150 mg every 12 hours (q12h). Additional details on these four studies are noted below:

- **Two Copies of the F508del Mutation:** In the first quarter of 2015, Vertex began a Phase 3 study to evaluate the combination of VX-661 and ivacaftor in people ages 12 and older who have two copies of the F508del mutation. Enrollment of approximately 500 patients in North America and Europe is ongoing.
- **One Copy of the F508del Mutation and a Second Mutation that Results in a Gating Defect in the CFTR Protein:** Vertex recently began a Phase 3 study to evaluate the combination of VX-661 and ivacaftor in people ages 12 and older who have one copy of the F508del mutation and a second mutation that results in a gating defect in the CFTR protein. Enrollment of approximately 200 patients in North America and Europe is ongoing.
- **One Copy of the F508del Mutation and a Second Mutation That Results in Residual CFTR Function:** Vertex recently began a Phase 3 study to evaluate the combination of VX-661 and ivacaftor in people ages 12 and older who have one copy of the F508del mutation and a second mutation that results in residual CFTR function. This study is also evaluating ivacaftor dosed without VX-661. Enrollment of approximately 300 patients in North America, Europe and Australia is ongoing.
- **One Copy of the F508del Mutation and A Second Mutation That Results in Minimal CFTR Function:** Vertex today announced the initiation of a Phase 3 study to evaluate the combination of VX-661 and ivacaftor in people ages 12 and older who have one copy of the F508del mutation and a second mutation that results in minimal CFTR function. The study will enroll approximately 150 patients, and expansion of the study to an additional approximately 150 patients will depend on an interim futility analysis of efficacy data from the initial 150 patients.

Development of Investigational VX-371 (P-1037)

In the second quarter, Vertex and Parion Sciences entered into a collaboration to develop investigational epithelial sodium channel (ENaC) inhibitors, including VX-371 (P-1037), for the potential treatment of CF and other pulmonary diseases. Parion is conducting an exploratory Phase 2a study of inhaled VX-371 in approximately 120 people with CF. The study is enrolling people with a confirmed diagnosis of CF and any CFTR mutation, including those who have mutations not expected to respond to ivacaftor alone. Data from this study are expected in mid-2016. In addition, Vertex plans to begin a Phase 2a study to evaluate whether the addition of VX-371 to the combination of lumacaftor and ivacaftor in people with CF who have two copies of the F508del mutation provides additional benefit as compared to the combination of lumacaftor and ivacaftor alone. This Phase 2a study is expected to begin in early 2016.

Second Quarter 2015 Non-GAAP Financial Results

The non-GAAP financial results for the second quarter 2015 and second quarter 2014 exclude stock-based compensation expense, costs related to the relocation of the company's corporate headquarters, hepatitis C-related revenues and costs and other adjustments.

Total Non-GAAP Revenues: Total non-GAAP revenues for the second quarter of 2015 were $159.9 million, including $154.9 million in net product revenues from KALYDECO and $5.0 million from royalty revenues.

- **Net Product Revenues from KALYDECO:** Vertex's second quarter 2015 net product revenues from KALYDECO were $154.9 million compared to $113.1 million for the second quarter of 2014. The increased KALYDECO net product revenues, compared to the second quarter of 2014, resulted primarily from additional people being treated with KALYDECO in both U.S. and ex-U.S. markets.

Non-GAAP Cost of Product Revenues and Royalty Expenses (COR): Total combined non-GAAP COR expenses for the second quarter of 2015 were $16.5 million, compared to $11.1 million for the second quarter of 2014.

Non-GAAP Research and Development (R&D) Expenses and Sales, General and Administrative (SG&A) Expenses: Total combined non-GAAP R&D and SG&A expenses for the second quarter of 2015 were $253.9 million, compared to $237.4 million for the second quarter of 2014. The components were:

- **R&D Expenses:** Non-GAAP R&D expenses were $181.9 million for the second quarter of 2015, compared to $179.5 million in non-GAAP R&D expenses for the second quarter of 2014. The R&D expenses for the second quarter of 2015 were similar to the second quarter of 2014 as a result of the completion of the Phase 3 program for the combination of lumacaftor and ivacaftor in the first half of 2014, offset by increased costs related to the initiation of the pivotal Phase 3 program for VX-661 in combination with ivacaftor in the first half of 2015.
- **SG&A Expenses:** Non-GAAP SG&A expenses were $72.0 million for the second quarter of 2015, compared to $57.9 million in non-GAAP SG&A expenses for the second quarter of 2014. This increase was primarily the result of increased investment in global commercial support for the planned launch of ORKAMBI.

Non-GAAP Net Loss Attributable to Vertex: Vertex's second quarter 2015 non-GAAP net loss was $130.7 million, or $0.54 per diluted share, compared to a non-GAAP net loss of $141.7 million, or $0.61 per diluted share, for the second quarter of 2014. The non-GAAP net loss for the second quarter of 2015 was similar to the second quarter of 2014 as a result of increased KALYDECO product revenues, offset by increased operating expenses and interest expense.

Cash Position at June 30, 2015

As of June 30, 2015, Vertex had $1.0 billion in cash, cash equivalents and marketable securities compared to $1.4 billion in cash, cash equivalents and marketable securities as of December 31, 2014. As of June 30, 2015, Vertex had $300 million outstanding from a credit agreement that provides for a secured loan of up to $500 million.

2015 Financial Guidance

This section contains forward-looking guidance about the financial outlook for Vertex.

Vertex today increased its financial guidance for total 2015 KALYDECO revenues and reiterated its guidance for non-GAAP operating expenses:

- **KALYDECO Net Revenues:** Vertex now expects KALYDECO net revenues of $575 to $590 million for 2015. The prior range, first provided on January 28, 2015, was for KALYDECO net revenues of $560 to $580 million for 2015.
- **Non-GAAP R&D and SG&A Expenses:** Vertex reiterated its guidance for combined non-GAAP R&D and SG&A expenses in 2015 of $1.05 to $1.10 billion.

Vertex's expected combined non-GAAP R&D and SG&A expenses exclude stock-based compensation expense and certain other expenses recorded in 2015.

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 including a one-time 2014 cash payment related to a lease agreement, 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.

Second Quarter 2015 GAAP Financial Results

Total Revenues: Total revenues for the second quarter of 2015 were $166.1 million compared with $138.4 million in total revenues for the second quarter of 2014. Second quarter 2015 revenues were comprised primarily of $154.9 million in KALYDECO net product revenues and an aggregate of $11.2 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues. For the second quarter of 2014, Vertex reported $113.1 million in net product revenues from KALYDECO and an aggregate of $25.4 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues.

Operating Costs and Expenses: Total operating costs and expenses for the second quarter of 2015 were $337.2 million,

including certain charges of $66.8 million, compared to $319.0 million for the second quarter of 2014, including certain charges of $70.5 million. GAAP operating costs and expenses included:

- **COR Expenses:** COR expenses were $16.9 million for the second quarter of 2015, including $0.4 million of certain charges, compared to $17.3 million for the second quarter of 2014, including $6.2 million of certain charges.
- **R&D Expenses:** R&D expenses were $223.9 million for the second quarter of 2015, including $41.9 million of certain charges, compared to $224.5 million for the second quarter of 2014, including $45.0 million of certain charges.
- **SG&A Expenses:** SG&A expenses were $94.4 million for the second quarter of 2015, including $22.4 million of certain charges, compared to $77.4 million for the second quarter of 2014, including $19.6 million of certain charges.

Net Loss Attributable to Vertex: Vertex's second quarter 2015 net loss was $188.8 million, or $0.78 per diluted share, including net charges of $58.2 million. Vertex's second quarter 2014 net loss was $159.4 million, or $0.68 per diluted share, including net charges of $17.7 million.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	**2014**	**2015**	**2014**
Revenues:				
Product revenues, net	$ 160,388	$ 122,319	$ 291,263	$ 225,780
Royalty revenues	5,077	13,015	11,869	23,748
Collaborative revenues	611	3,087	1,453	7,344
Total revenues	166,076	138,421	304,585	256,872
Costs and expenses:				
Cost of product revenues	15,409	9,655	24,790	18,227
Royalty expenses	1,451	7,645	4,377	14,549
Research and development expenses	223,858	224,487	439,457	463,104
Sales, general and administrative expenses	94,394	77,446	180,254	151,658
Restructuring expenses (income)	2,128	(270)	(1,144)	5,918
Total costs and expenses	337,240	318,963	647,734	653,456
Loss from operations	(171,164)	(180,542)	(343,149)	(396,584)
Interest expense, net	(21,111)	(15,585)	(42,418)	(31,302)
Other income (expenses), net	1,414	37,731	(3,699)	38,182
Loss from continuing operations before provision for income taxes	(190,861)	(158,396)	(389,266)	(389,704)
Provision for income taxes	30,131	693	30,430	1,496
Loss from continuing operations	(220,992)	(159,089)	(419,696)	(391,200)
Loss from discontinued operations, net of tax	—	(293)	—	(639)
Net loss	(220,992)	(159,382)	(419,696)	(391,839)
Loss attributable to noncontrolling interest	32,144	—	32,242	—
Net loss attributable to Vertex	$ (188,848)	$ (159,382)	$ (387,454)	$ (391,839)
Amounts attributable to Vertex:				
Loss from continuing operations	$ (188,848)	$ (159,089)	$ (387,454)	$ (391,200)
Loss from discontinued operations	—	(293)	—	(639)
Net loss attributable to Vertex	$ (188,848)	$ (159,382)	$ (387,454)	$ (391,839)
Amounts per share attributable to Vertex common shareholders:				
Net loss from continuing operations:				
Basic and diluted	$ (0.78)	$ (0.68)	$ (1.61)	$ (1.68)
Net loss:				

Basic and diluted	$ (0.78)	$ (0.68)	$ (1.61)	$ (1.68)
Shares used in per share calculations:				
Basic and diluted	240,757	233,808	240,129	233,353

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	**2014**	**2015**	**2014**
GAAP loss attributable to Vertex	$ (188,848)	$ (159,382)	$ (387,454)	$ (391,839)
Stock-based compensation expense	63,261	42,444	120,645	89,024
Real estate restructuring costs and income (Note 1)	1,178	(26,037)	(2,400)	(6,095)
HCV related revenues and costs (Note 2)	(6,004)	(2,327)	(10,473)	8,993
Other adjustments (Note 3)	(270)	3,584	623	6,909
Non-GAAP net loss attributable to Vertex	$ (130,683)	$ (141,718)	$ (279,059)	$ (293,008)
Amounts per diluted share attributable to Vertex common shareholders:				
GAAP	$ (0.78)	$ (0.68)	$ (1.61)	$ (1.68)
Non-GAAP	$ (0.54)	$ (0.61)	$ (1.16)	$ (1.26)
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	240,757	233,808	240,129	233,353

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,	
	2015	**2014**	**2015**	**2014**
GAAP total revenues	$ 166,076	$ 138,421	$ 304,585	$ 256,872
HCV related revenues (Note 2)	(6,094)	(16,445)	(8,963)	(26,715)
Other adjustments (Note 3)	(74)	—	(274)	—
Non-GAAP total revenues	$ 159,908	$ 121,976	$ 295,348	$ 230,157

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	**2014**	**2015**	**2014**
GAAP cost of product revenues and royalty expenses	$ 16,860	$ 17,300	$ 29,167	$ 32,776
HCV related costs (Note 2)	(371)	(6,233)	(1,968)	(12,273)
Non-GAAP cost of product revenues and royalty expenses	$ 16,489	$ 11,067	$ 27,199	$ 20,503
GAAP research and development expenses	$ 223,858	$ 224,487	$ 439,457	$ 463,104
Stock-based compensation expense	(41,632)	(27,253)	(79,849)	(60,153)
Real estate restructuring costs (Note 1)	—	(9,382)	—	(21,583)
HCV related costs (Note 2)	512	(4,756)	1,000	(13,407)
Other adjustments (Note 3)	(827)	(3,584)	(1,520)	(6,909)
Non-GAAP research and development expenses	$ 181,911	$ 179,512	$ 359,088	$ 361,052
GAAP sales, general and administrative expenses	$ 94,394	$ 77,446	$ 180,254	$ 151,658

Stock-based compensation expense	(21,629)	(15,191)	(40,796)	(28,871)
Real estate restructuring costs (Note 1)	—	(1,706)	—	(3,906)
HCV related costs (Note 2)	(54)	(2,666)	2,851	(8,572)
Other adjustments (Note 3)	(695)	—	(1,147)	—
Non-GAAP sales, general and administrative expenses	$ 72,016	$ 57,883	$ 141,162	$ 110,309
Combined Non-GAAP R&D and SG&A expenses	$ 253,927	$ 237,395	$ 500,250	$ 471,361

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	**2014**	**2015**	**2014**
GAAP interest expense, net and other income (expense), net	$ (19,697)	$ 22,146	$ (46,117)	$ 6,880
Real estate restructuring income (Note 1)	—	(36,685)	—	(36,685)
Non-GAAP interest expense, net and other income (expense), net	$ (19,697)	$ (14,539)	$ (46,117)	$ (29,805)
GAAP provision for income taxes	$ 30,131	$ 693	$ 30,430	$ 1,496
Other adjustments (Note 3)	(29,653)	—	(29,589)	—
Non-GAAP provision for income taxes	$ 478	$ 693	$ 841	$ 1,496

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

	June 30, 2015	**December 31, 2014**
Assets		
Cash, cash equivalents and marketable securities	$ 1,016,450	$ 1,387,106
Restricted cash and cash equivalents (VIE) (Note 4)	88,318	8,418
Accounts receivable, net	94,519	75,964
Inventories	42,113	30,848
Property and equipment, net	713,378	715,812
Intangible assets and goodwill	334,724	68,915
Other assets	84,571	47,616
Total assets	$ 2,374,073	$ 2,334,679
Liabilities and Shareholders' Equity		
Other liabilities	$ 333,185	$ 307,374
Deferred tax liability	112,413	15,044
Accrued restructuring expense	19,843	45,855
Deferred revenues	35,949	45,276
Capital leases	56,821	57,099
Fan Pier lease obligation	472,834	473,073
Senior secured term loan	294,812	294,775
Shareholders' equity	1,048,216	1,096,183
Total liabilities and shareholders' equity	$ 2,374,073	$ 2,334,679
Common shares outstanding	244,342	241,764

Note 1: In the three and six months ended June 30, 2015, "Real estate restructuring costs and income" consisted of restructuring charges and credits, respectively, related to the company's relocation from Cambridge to Boston, Massachusetts. In the three and six months ended June 30, 2014, "Real estate restructuring costs and income" consisted of (i) transition costs related to the company's relocation that were recorded as R&D and SG&A, (ii) restructuring credits and charges, respectively, related to this relocation and (iii) credits recorded to other (expense) income, net to record the effect of the one-time cash payment received related to a lease agreement in the second quarter of 2014.

Note 2: In the three and six months ended June 30, 2014 and 2015, "HCV related revenues and costs" included in the company's loss from continuing operations consisted of:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
	(in millions)			
Net product revenues from Incivek	$ 5.5	$ 9.3	$ 6.2	$ 13.2
Royalty revenues from Incivo	0.1	5.7	1.6	10.6
HCV collaborative revenues	0.5	1.5	1.2	2.9
COR expenses	(0.4)	(6.2)	(2.0)	(12.3)
R&D and SG&A credits (including pharma fee)	0.5	(7.4)	3.9	(22.0)
Restructuring expenses	(0.2)	(0.2)	(0.4)	(0.8)

Note 3: In each of the three and six months ended June 30, 2014 and 2015, "Other adjustments" consisted of development cost associated with VX-509. In addition, in the three and six months ended June 30, 2015, "Other adjustments" included amounts related to two variable interest entities ("VIEs").

Note 4: The company consolidates the financial statements of two of its collaborators as VIEs as of June 30, 2015 and consolidated a single VIE as of December 31, 2014. These VIEs are consolidated because Vertex has licensed the rights to develop the company's collaborator's 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.

Note 5: In each of the three and six months ended June 30, 2014 and 2015, the company excludes from its non-GAAP loss attributable to Vertex restructuring expense (income). In addition, in the three and six months ended June 30, 2014 discontinued operations related to the effect of the company's relationship with Alios are excluded from its non-GAAP loss attributable to Vertex.

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 five 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 "2015 Financial Guidance," and statements regarding the expected timing of potential approval of ORKAMBI in ex-U.S. markets and the expected timing and clinical trial designs of the (i) Phase 3 clinical studies of lumacaftor in combination with ivacaftor in children 6 to 11 years of age, (ii) Phase 3 program of VX-661 in combination with ivacaftor and (iii) Phase 2a clinical studies of VX-371 (P-1037). 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 2015 revenues and financial results and its 2015 non-GAAP operating expenses may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized), that regulatory authorities outside of the United States may not approve, or approve on a timely basis, ORKAMBI, 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.

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