



July 26, 2017

Vertex Reports Second-Quarter 2017 Financial Results

-Second-quarter 2017 cystic fibrosis product revenues of \$514 million; \$324 million for ORKAMBI and \$190 million for KALYDECO-

-Vertex reiterates 2017 guidance for ORKAMBI and KALYDECO product revenues; updates guidance for combined GAAP and non-GAAP R&D and SG&A expenses-

-Pipeline of investigational CF medicines continues to progress and expand to support goal of treating all people with CF-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2017. Vertex reiterated its full-year 2017 financial guidance for ORKAMBI[®] (lumacaftor/ivacaftor) and KALYDECO[®] (ivacaftor) net revenues and updated its guidance for combined GAAP and non-GAAP R&D and SG&A expenses. The company also reviewed its recent progress toward treating all people with CF, including the completion of an asset purchase agreement with Concert Pharmaceuticals for worldwide development and commercialization rights to CTP-656 and other assets related to the treatment of CF.

Key financial results include:

	Three Months Ended June 30,		% Change
	2017	2016	
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	\$ 324	\$ 245	32%
KALYDECO product revenues, net	\$ <u>190</u>	\$ <u>180</u>	5%
TOTAL CF product revenues, net	\$ <u>514</u>	\$ <u>426</u>	21%
GAAP net income (loss)	\$ 18	\$ (65)	n/a
GAAP net income (loss) per share - diluted	\$ 0.07	\$ (0.26)	n/a
Non-GAAP net income	\$ 99	\$ 58	71%
Non-GAAP net income per share - diluted	\$ 0.39	\$ 0.24	63%

"During the first half of 2017, Vertex has made significant progress throughout the business and in particular, across our CF development programs," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Our progress has been marked by the progression of multiple combination regimens that allow us to treat more people with CF today and to potentially treat up to 90% of patients with this disease in the future."

Financial Highlights

Revenues:

- | Total CF net product revenues were \$514.0 million compared to \$425.7 million for the second quarter of 2016.
- | Net product revenues from ORKAMBI were \$324.4 million compared to \$245.5 million for the second quarter of 2016. The increase in ORKAMBI revenues was primarily driven by the continued uptake in the medicine globally and additional uptake in people with CF ages 6 to 11 in the U.S., where approval was received in September 2016.
- | Net product revenues from KALYDECO were \$189.6 million compared to \$180.2 million for the second quarter of 2016.

Expenses:

- Combined GAAP R&D and SG&A expenses were \$416.7 million compared to \$382.7 million for the second quarter of 2016. Combined non-GAAP R&D and SG&A expenses were \$333.4 million compared to \$306.3 million for the second quarter of 2016.
- GAAP R&D expenses were \$289.5 million compared to \$271.0 million for the second quarter of 2016. Non-GAAP R&D expenses were \$240.5 million compared to \$217.7 million for the second quarter of 2016. The increase in combined GAAP and non-GAAP R&D expenses was primarily attributable to R&D expenses related to the clinical development of the company's four triple combination regimens for CF.
- GAAP SG&A expenses were \$127.2 million compared to \$111.7 million for the second quarter of 2016. Non-GAAP SG&A expenses were \$92.9 million compared to \$88.6 million for the second quarter of 2016. The increase in combined GAAP and non-GAAP SG&A expenses was primarily driven by commercial support for the launch and expansion of ORKAMBI globally.

Net Income (Loss) Attributable to Vertex:

- GAAP net income was \$18.0 million, or \$0.07 per diluted share, for the second quarter of 2017, compared to a net loss of \$(64.5) million, or \$(0.26) per diluted share, for the second quarter of 2016. Non-GAAP net income was \$98.9 million, or \$0.39 per diluted share, for the second quarter of 2017, compared to \$58.0 million, or \$0.24 per diluted share, for the second quarter of 2016. Second quarter 2017 non-GAAP net income growth was primarily driven by increased product revenues.

Cash Position:

- As of June 30, 2017, Vertex had \$1.67 billion in cash, cash equivalents and marketable securities compared to \$1.43 billion in cash, cash equivalents and marketable securities as of December 31, 2016.

2017 Financial Guidance:

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

- Total CF Product Revenues:** Vertex expects total 2017 CF product revenues of \$1.84 to \$2.07 billion, comprised of ORKAMBI and KALYDECO product revenues.
- ORKAMBI:** The company continues to expect total 2017 product revenues for ORKAMBI of \$1.1 to \$1.3 billion. This range includes an estimate of potential additional European revenues in 2017 that is largely dependent on which European countries complete reimbursement agreements in 2017 and when these agreements become effective.
- KALYDECO:** The company continues to expect total 2017 product revenues for KALYDECO of \$740 to \$770 million. This range includes the recent U.S. approval of KALYDECO for the use in people with CF ages 2 and older who have one of 23 residual function mutations.
- Combined Non-GAAP and GAAP R&D and SG&A Expenses:** Vertex today updated its total 2017 guidance for combined non-GAAP R&D and SG&A expenses to a range of \$1.33 to \$1.36 billion, compared to its previously announced guidance of \$1.25 to \$1.30 billion. The updated guidance reflects the progression of the company's CF portfolio, including acceleration of Phase 2 studies for VX-659 and VX-445, preparation for pivotal studies for its triple combination regimens, and investment to develop CTP-656 as part of future triple combination regimens. The company also updated its GAAP R&D and SG&A expenses to a range of \$1.79 to \$1.92 billion, compared to its previously announced guidance of \$1.55 to \$1.70 billion. The updated GAAP guidance also reflects \$160.0 million in R&D expense that Vertex expects to incur in the third quarter of 2017 related to the upfront payment for the rights to CTP-656 and other assets acquired from Concert Pharmaceuticals.

Business Highlights

Vertex today provided the following updates:

ORKAMBI

Continued progress toward label expansion and global reimbursement: Vertex continues to make progress toward the reimbursement of ORKAMBI for people with CF ages 12 and older who have two copies of the *F508del* mutation in

the European Union. ORKAMBI is now available for eligible patients in Austria, Denmark, Germany, Ireland, Italy and Luxembourg. Negotiations continue in a number of other countries where CF is prevalent, including France, the Netherlands and the United Kingdom, among others.

KALYDECO

KALYDECO label expansion for people ages 2 and older: The U.S. Food and Drug Administration (FDA) recently approved KALYDECO for the use in people with CF ages 2 and older who have one of 23 residual function mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. More than 900 people ages 2 and older in the U.S. have one of these mutations. In addition to these mutations added to the label, Vertex is continuing discussions with the FDA concerning the approval for 600 additional people who have other residual function mutations responsive to KALYDECO.

In the U.S., KALYDECO is now approved to treat people with CF ages 2 and older who have one of 33 mutations in the *CFTR* gene responsive to ivacaftor based on clinical and/or in vitro assay data.

TEZACAFTOR/IVACAFTOR

Regulatory submissions for people ages 12 and older: Based on Phase 3 data, Vertex has submitted a new drug application (NDA) to the FDA and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for the tezacaftor/ivacaftor combination therapy in people with CF ages 12 and older.

Orphan Drug Designation: On June 16, 2017, the FDA granted Orphan Drug Designation to tezacaftor in combination with ivacaftor. The FDA grants Orphan Drug Designation to medicines intended to treat fewer than 200,000 people in the U.S.

Phase 3 study in people with one copy of the *F508del* mutation and a second mutation that results in a gating defect: Vertex announced today that it has completed enrollment in a study evaluating the tezacaftor/ivacaftor combination in people with CF ages 12 and older with one copy of the *F508del* mutation and a second mutation that results in a gating effect in the *CFTR* protein that has been shown to be responsive to ivacaftor alone. Data from this study are expected in the second half of 2017.

TRIPLE COMBINATION REGIMENS

Positive Phase 1 and Phase 2 data from three different triple combination regimens: Vertex continues to evaluate four different next-generation correctors to be included in an investigational triple combination regimen with tezacaftor and ivacaftor.

On July 18, 2017, Vertex announced positive data from Phase 1 and Phase 2 studies of three different triple combination regimens in people with CF who have one *F508del* mutation and one minimal function mutation, as well as positive data in people with two copies of the *F508del* mutation.

Pending additional data from all four next-generation correctors, discussions with regulatory agencies and input from a Steering Committee of global CF experts, Vertex plans to initiate pivotal development of one or more triple combination regimens in the first half of 2018.

CTP-656

CTP-656 for potential use in future combination regimens: On July 25, 2017, Vertex and Concert Pharmaceuticals announced the completion of their previously announced asset purchase agreement. Vertex now has worldwide development and commercialization rights to CTP-656 and other assets related to the treatment of CF. Concert received \$160 million in cash upon closing and is eligible to receive up to \$90 million in additional milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France.

CTP-656 is an investigational cystic fibrosis transmembrane conductance regulator (*CFTR*) potentiator that has the potential to be used as part of future once-daily combination regimens of *CFTR* modulators that treat the underlying cause of cystic fibrosis.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP

financial results and guidance exclude (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements and consolidated variable interest entities and (iii) other adjustments. These results are provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally and to manage the company's business and to evaluate its performance. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. The company provides guidance regarding product revenues in accordance with GAAP and provides guidance regarding combined research and development and sales, general, and administrative expenses on both a GAAP and a non-GAAP basis. The guidance regarding GAAP research and development expenses and sales, general and administrative expenses does not include estimates regarding expenses associated with any potential business development activities, but includes \$160.0 million in R&D expense related to the upfront payment that Vertex expects to incur in the third quarter of 2017 for the rights to CTP-656 and other assets acquired from Concert Pharmaceuticals. 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)

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Revenues:				
Product revenues, net	\$ 513,988	\$ 425,651	\$ 994,610	\$ 820,061
Royalty revenues	2,861	5,282	4,412	8,878
Collaborative revenues (Note 1)	27,286	675	259,831	749
Total revenues	<u>544,135</u>	<u>431,608</u>	<u>1,258,853</u>	<u>829,688</u>
Costs and expenses:				
Cost of product revenues	70,535	44,154	116,777	93,943
Royalty expenses	670	1,098	1,416	1,958
Research and development expenses	289,451	271,008	563,014	526,868
Sales, general and administrative expenses	127,249	111,652	240,575	216,866
Restructuring expenses	3,523	343	13,522	1,030
Total costs and expenses	<u>491,428</u>	<u>428,255</u>	<u>935,304</u>	<u>840,665</u>
Income (loss) from operations	52,707	3,353	323,549	(10,977)
Interest expense, net	(14,664)	(20,155)	(31,429)	(40,853)
Other (expenses) income, net	(2,537)	(1,219)	(3,081)	3,192
Income (loss) from operations before provision for income taxes	35,506	(18,021)	289,039	(48,638)
Provision for income taxes	4,337	18,130	8,322	23,615
Net income (loss)	31,169	(36,151)	280,717	(72,253)
Income attributable to noncontrolling interest (Note 4)	(13,173)	(28,374)	(14,965)	(33,903)
Net income (loss) attributable to Vertex	<u>\$ 17,996</u>	<u>\$ (64,525)</u>	<u>\$ 265,752</u>	<u>\$ (106,156)</u>
Amounts per share attributable to Vertex common shareholders:				
Net income (loss):				
Basic	\$ 0.07	\$ (0.26)	\$ 1.08	\$ (0.43)
Diluted	\$ 0.07	\$ (0.26)	\$ 1.06	\$ (0.43)
Shares used in per share calculations:				
Basic	247,521	244,482	246,782	244,124
Diluted	251,635	244,482	250,199	244,124

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,	
	2017	2016	2017	2016
GAAP income (loss) attributable to Vertex	\$ 17,996	\$ (64,525)	\$ 265,752	\$ (106,156)
Stock-based compensation expense	72,582	61,942	141,564	117,414
Collaboration and transaction revenues and expenses (Note 2)	4,051	59,720	(222,249)	69,151
Other adjustments (Note 3)	4,268	835	15,236	(15)
Non-GAAP net income attributable to Vertex	\$ 98,897	\$ 57,972	\$ 200,303	\$ 80,394

Amounts per diluted share attributable to Vertex common shareholders:

GAAP	\$ 0.07	\$ (0.26)	\$ 1.06	\$ (0.43)
Non-GAAP	\$ 0.39	\$ 0.24	\$ 0.80	\$ 0.33

Shares used in diluted per share calculations:

GAAP	251,635	244,482	250,199	244,124
Non-GAAP	251,635	246,426	250,199	246,872

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,	
	2017	2016	2017	2016
GAAP total revenues	\$ 544,135	\$ 431,608	\$ 1,258,853	\$ 829,688
Collaboration and transaction revenues (Note 2)	(27,222)	(573)	(259,684)	(647)
Other adjustments (Note 3)	—	489	—	(362)
Non-GAAP total revenues	\$ 516,913	\$ 431,524	\$ 999,169	\$ 828,679

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
GAAP cost of product revenues and royalty expenses	\$ 71,205	\$ 45,252	\$ 118,193	\$ 95,901
Other adjustments (Note 3)	—	6	—	(133)
Non-GAAP cost of product revenues and royalty expenses	\$ 71,205	\$ 45,258	\$ 118,193	\$ 95,768
GAAP research and development expenses	\$ 289,451	\$ 271,008	\$ 563,014	\$ 526,868
Stock-based compensation expense	(43,832)	(40,640)	(88,669)	(75,088)
Collaboration and transaction expenses (Note 2)	(5,024)	(12,746)	(7,033)	(12,905)
Other adjustments (Note 3)	(136)	51	(272)	845
Non-GAAP research and development expenses	\$ 240,459	\$ 217,673	\$ 467,040	\$ 439,720
GAAP sales, general and administrative expenses	\$ 127,249	\$ 111,652	\$ 240,575	\$ 216,866
Stock-based compensation expense	(28,750)	(21,302)	(52,895)	(42,326)
Collaboration and transaction expenses (Note 2)	(4,984)	(1,698)	(6,988)	(2,241)
Other adjustments (Note 3)	(609)	(61)	(1,442)	(29)
Non-GAAP sales, general and administrative expenses	\$ 92,906	\$ 88,591	\$ 179,250	\$ 172,270
Combined non-GAAP R&D and SG&A expenses	\$ 333,365	\$ 306,264	\$ 646,290	\$ 611,990

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
GAAP interest expense, net and other expense, net	\$ (17,201)	\$ (21,374)	\$ (34,510)	\$ (37,661)
Collaboration and transaction expenses (Note 2)	(40)	(36)	(74)	177
Non-GAAP interest expense, net and other expense, net	\$ (17,241)	\$ (21,410)	\$ (34,584)	\$ (37,484)
GAAP provision for income taxes	\$ 4,337	\$ 18,130	\$ 8,322	\$ 23,615
Collaboration and transaction expenses (Note 2)	(8,132)	(17,510)	(8,523)	(20,572)
Non-GAAP (benefit from) provision for income taxes	\$ (3,795)	\$ 620	\$ (201)	\$ 3,043

Condensed Consolidated Balance Sheets Data

(in thousands)
(unaudited)

	June 30, 2017	December 31, 2016
Assets		
Cash, cash equivalents and marketable securities	\$ 1,668,650	\$ 1,434,557
Restricted cash and cash equivalents (VIE) (Note 4)	64,628	47,762
Accounts receivable, net	247,949	201,083
Inventories	92,263	77,604
Property and equipment, net	740,103	698,362
Intangible assets and goodwill	334,724	334,724
Other assets (Note 1)	137,277	102,695
Total assets	\$ 3,285,594	\$ 2,896,787
Liabilities and Shareholders' Equity		
Accounts payable and accruals	\$ 421,003	\$ 376,700
Other liabilities	335,169	260,984
Deferred tax liability	136,649	134,063
Construction financing lease obligation	525,542	486,849
Debt	—	300,000
Shareholders' equity (Note 4)	1,867,231	1,338,191
Total liabilities and shareholders' equity	\$ 3,285,594	\$ 2,896,787
Common shares outstanding	250,770	248,301

Note 1: In the six months ended June 30, 2017, collaborative revenues were primarily attributable to a \$230 million up-front payment earned from our collaboration with Merck KGaA, Darmstadt, Germany. During the first quarter of 2017, the company received \$193.6 million of the up-front payment and the remaining \$36.4 million was remitted to the German tax authorities. The company filed a refund application for the tax withholding and expects to receive the refund in approximately second half of 2017. The income tax receivable is included in Other assets at June 30, 2017. During the three and six months ended June 30, 2017, collaborative revenues includes \$20.0 million that one of the company's consolidated variable interest entities ("VIEs") received from a collaboration agreement with a third party.

Note 2: In the three and six months ended June 30, 2017 and 2016, "Collaboration and transaction revenues and expenses" primarily consisted of (i) revenues and operating costs and expenses attributable to the company's VIEs and (ii) changes in the fair value of contingent payments due to VIEs. In the three and six months ended June 30, 2017, "Collaboration and transaction revenues and expenses" included the \$20.0 million of collaborative revenue and related tax provision that one of the company's VIEs earned in the three months ended June 30, 2017 and also consisted of revenues and expenses associated with the company's oncology program including the company's collaboration with Merck KGaA, Darmstadt, Germany and transaction costs associated with the company's purchase agreement with Concert Pharmaceuticals. The company has not adjusted its prior year Reconciliation of GAAP to Non-GAAP Revenues and

Expenses for the three and six months ended June 30, 2016 for \$5.7 million and \$9.8 million, respectively, of operating expenses related to its oncology program.

Note 3: In the three and six months ended June 30, 2017, "Other adjustments" primarily consisted of restructuring charges related to the company's decision to consolidate its research activities into its Boston, Milton Park and San Diego locations and to close our research site in Canada. In the three and six months ended June 30, 2016, "Other adjustments" primarily consisted of revenues and operating costs and expenses related to HCV as well as restructuring charges related to the company's relocation from Cambridge to Boston, Massachusetts.

Note 4: The company consolidates the financial statements of two of its collaborators as VIEs as of June 30, 2017 and December 31, 2016. These VIEs are consolidated because Vertex has licensed the rights to develop the company's collaborators' most significant intellectual property assets. The company's interest and obligations with respect to these VIEs' assets and liabilities are limited to those accorded to the company in its collaboration agreements. Restricted cash and cash equivalents (VIE) reflects the VIEs' cash and cash equivalents, which Vertex does not have any interest in and which will not be used to fund the collaboration. Each reporting period Vertex estimates the fair value of the contingent payments by Vertex to these collaborators. Any increase in the fair value of these contingent payments results in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis. The fair value of contingent payments is evaluated each quarter and any change in the fair value is reflected in the company's statement of operations.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For seven years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Leiden's statements in the second paragraph of the press release, the information provided in the section captioned "2017 Financial Guidance" and statements regarding (i) the country-by-country reimbursement negotiations for ORKAMBI, (ii) the timing of regulatory applications, including MAAs and NDAs, (iii) the development plan and timelines for our product development candidates, including tezacaftor in combination with ivacaftor and our next-generation triple combination regimens and (iv) potential milestone payments pursuant to the Concert transaction. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2017 revenues and expenses may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast

The company will host a conference call and webcast today at 5:15 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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