



Vertex Reports First-Quarter 2019 Financial Results

April 30, 2019

- First-quarter 2019 product revenues of \$857 million, a 34% increase compared to \$638 million in 2018-

- First-quarter 2019 GAAP operating income increased 115% to \$277 million; non-GAAP operating income increased 81% to \$377 million -

- On track to choose best triple combination regimen in Q2 2019; NDA submission planned for Q3 2019 -

BOSTON--(BUSINESS WIRE)--Apr. 30, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2019 and reiterated full-year 2019 financial guidance.

"Our goal is to develop transformative medicines for all people with CF and other serious diseases and to ensure all eligible patients have access to these medicines as quickly as possible," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We have made significant progress toward achieving this goal by rapidly advancing our triple combination regimens through late-stage development, and we remain on track to submit a New Drug Application for one of these medicines in the third quarter of 2019. We also continue to advance our earlier-stage programs targeting AAT, pain, FSGS and sickle cell disease. In the first quarter, we again delivered strong revenue and earnings growth, which further enhances our ability to make significant investments in internal and external innovation."

First-Quarter 2019 Financial Highlights

	<u>Three Months Ended March 31,</u>		<u>%</u> <u>Change</u>
	<u>2019</u>	<u>2018</u>	
	(in millions, except per share amounts)		
Total product revenues, net	\$ 857	\$ 638	34%
KALYDECO	\$ 244	\$ 250	
ORKAMBI	\$ 293	\$ 354	
SYMDEKO/SYMKEVI	\$ 320	\$ 34	
GAAP Operating income	\$ 277	\$ 129	115%
Non-GAAP Operating income	\$ 377	\$ 208	81%
GAAP Net income	\$ 269	\$ 210	28%
Non-GAAP Net income	\$ 296	\$ 196	51%
GAAP Net income per share - diluted	\$ 1.03	\$ 0.81	27%
Non-GAAP Net income per share - diluted	\$ 1.14	\$ 0.76	50%

Total product revenues increased 34% compared to the first quarter of 2018, primarily driven by the uptake of SYMDEKO in the U.S. since launch.

GAAP and Non-GAAP net income increased compared to the first quarter of 2018, largely driven by the strong growth in total product revenues, and was partially offset by increases in operating expenses and income taxes.

Cash, cash equivalents and marketable securities as of March 31, 2019 were \$3.5 billion, an increase of approximately \$300 million compared to \$3.2 billion as of December 31, 2018.

First-Quarter 2019 Expenses

	<u>Three Months Ended March 31,</u>	
	<u>2019</u>	<u>2018</u>
	(in millions)	
Combined GAAP R&D and SG&A expenses	\$ 487	\$ 440
Combined Non-GAAP R&D and SG&A expenses	\$ 388	\$ 360
GAAP R&D expense	\$ 339	\$ 311
Non-GAAP R&D expense	\$ 273	\$ 260
GAAP SG&A expense	\$ 147	\$ 130
Non-GAAP SG&A expense	\$ 114	\$ 100
GAAP income taxes	\$ 52	\$ (13)

Non-GAAP income taxes \$ 81 \$ 3

Combined GAAP and non-GAAP R&D and SG&A expenses increased compared to the first quarter of 2018 primarily due to the incremental investment to support the global use of Vertex's medicines and the expansion of Vertex's pipeline in CF and other new disease areas.

GAAP and Non-GAAP income taxes increased significantly compared to the first quarter of 2018 due to Vertex's release of its valuation allowance on the majority of its deferred tax assets in the fourth quarter of 2018. GAAP and non-GAAP income taxes in the first quarter of 2019 include a provision for income taxes on Vertex's pre-tax income using an estimated effective tax rate approximating statutory rates. This provision for income taxes includes a significant non-cash charge due to Vertex's ability to offset its pre-tax income against previously benefited net operating losses. Vertex expects its cash paid for income taxes to increase significantly once all of its net operating losses have been utilized to offset its pre-tax income. Refer to "Supplemental Income Tax Information" for discussion of the cash versus non-cash components of Vertex's provision for income taxes.

Full-Year 2019 Financial Guidance

Vertex today reiterated its full-year 2019 guidance as follows:

	<u>FY 2019</u>
TOTAL product revenues	\$ 3.45 to 3.55 billion
Combined GAAP R&D and SG&A expenses	\$ 2.00 to 2.15 billion
Combined Non-GAAP R&D and SG&A expenses	\$ 1.65 to 1.70 billion
Non-GAAP effective tax rate	21% - 22%

The company's total product revenue growth in 2019 is expected to be driven primarily by the full-year impact of the SYMDEKO launch, reimbursement agreements reached in 2018 and label expansions for the company's CF medicines. The company's full-year 2019 revenue guidance reflects only markets where its CF medicines are currently reimbursed.

The company's combined GAAP and non-GAAP R&D and SG&A expense guidance reflects CF development efforts, incremental investment to support the potential launch of a triple combination regimen and investment to support the expansion of Vertex's pipeline into new disease areas.

In addition, based on the release of the company's valuation allowance in the fourth quarter of 2018, Vertex has also begun to record a tax provision in 2019 and expects its full-year non-GAAP tax rate to be between 21% and 22%. The vast majority of this tax provision will be a non-cash expense until the company fully utilizes its net operating losses.

Business Highlights

INVESTIGATIONAL CF MEDICINES

Bringing CF medicines to more people as quickly as possible:

- Final Phase 3 24-week data are expected in the second quarter of 2019 from the triple combination program. Vertex plans to utilize these data to choose the best triple combination regimen to submit for regulatory approvals globally. The company plans to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the third quarter of 2019 and a Marketing Authorization Application (MAA) in Europe in the fourth quarter of 2019 for either the VX-659 or VX-445 triple combination regimen in people with CF who have two *F508del* mutations and in people with CF who have one *F508del* mutation and one minimal function mutation.
- The company has initiated a Phase 2 dose-ranging study evaluating the once-daily potentiator VX-561 as a monotherapy as requested by the FDA. The study is designed to evaluate multiple doses of VX-561 to support potential Phase 3 development of VX-561 in a once-daily triple combination regimen.
- Vertex has initiated a Phase 2 study evaluating the next-generation corrector, VX-121, in combination with VX-561 and tezacaftor as a potential once-daily triple combination regimen.

APPROVED CF MEDICINES

Securing access for Vertex CF medicines:

- The company continues to work toward establishing pricing and reimbursement agreements in additional countries outside of the U.S. Highlights in 2019 thus far include:
 - Positive recommendation for SYMDEKO in Australia for ages 12+ from the Pharmaceutical Benefits Advisory Committee (PBAC).
 - Reimbursement for ORKAMBI in Sweden for ages 2 to 5.
 - Expanded pricing agreement for ORKAMBI in Germany to include children ages 6 through 11.

Treating patients at younger ages with CFTR modulators:

- Vertex continues to make significant progress toward gaining approval for its CF medicines for use earlier in the course of disease progression. Recent highlights include:

- Approval for KALYDECO in the U.S. for infants ages 6 to <12 months.
- Approval for KALYDECO in Canada for children ages 12 to <24 months.
- Approval for ORKAMBI in the EU for children ages 2 to 5 years old.
- Supplemental New Drug Application (sNDA) submitted in the U.S. for tezacaftor/ivacaftor in children ages 6 to 11 years old.

LATE-STAGE RESEARCH & CLINICAL DEVELOPMENT

Alpha-1 Antitrypsin (AAT) Program:

- The FDA has granted Fast Track Designation for VX-814, Vertex's first small molecule corrector for the treatment of alpha-1 antitrypsin (AAT) deficiency. The company initiated a Phase 1 study of VX-814 in December 2018.
- Vertex is advancing other small molecule correctors of AAT through late preclinical development and expects to begin clinical development of a second small molecule AAT corrector in 2019.

Sickle Cell Disease & Beta-Thalassemia:

- In February 2019, CRISPR and Vertex announced that the first patient had been treated with CTX001 in a Phase 1/2 clinical study of patients with TDT, marking the first company-sponsored use of a CRISPR/Cas9 therapy in a clinical trial.
- In April 2019, Vertex and its partner CRISPR Therapeutics announced that the FDA has granted Fast Track Designation for CTX001, an investigational, autologous, gene-edited hematopoietic stem cell therapy, for the treatment of transfusion-dependent beta thalassemia (TDT).
- The companies are also evaluating CTX001 for the treatment of sickle cell disease (SCD) and received Fast Track Designation for CTX001 from the FDA in January 2019 for SCD. The companies announced in February 2019 that the first patient had been enrolled in a Phase 1/2 clinical study of CTX001 in severe SCD in the U.S. and is expected to be infused with CTX001 in mid-2019.
- Enrollment in both Phase 1/2 studies of CTX001 in patients with TDT and in patients with severe SCD is ongoing.

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 from Vertex's pre-tax income (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements, asset acquisitions and consolidated variable interest entities and (iii) other adjustments, including gains or losses related to the fair value of the company's strategic investments. The company's non-GAAP financial results also exclude from its provision for or benefit from income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income described above. 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 non-GAAP basis. The company also provides guidance regarding its anticipated income taxes as a percentage of pre-tax income on a non-GAAP basis. The guidance regarding GAAP research and development expenses and sales, general and administrative expenses does not include estimates associated with any potential future business development activities. 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
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2019	2018
Revenues:		
Product revenues, net	\$ 857,253	\$ 637,729
Collaboration and royalty revenues	1,182	3,070
Total revenues	858,435	640,799
Costs and expenses:		
Cost of sales	95,092	71,613

Research and development expenses	339,490	310,553
Sales, general and administrative expenses	147,045	129,808
Restructuring income	—	(76)
Total costs and expenses	<u>581,627</u>	<u>511,898</u>
Income from operations	276,808	128,901
Interest income	15,615	5,789
Interest expense	(14,868)	(16,886)
Other income, net (1)	42,610	96,838
Income from operations before provision for (benefit from) income taxes	320,165	214,642
Provision for (benefit from) income taxes (2)	<u>51,534</u>	<u>(12,659)</u>
Net income	268,631	227,301
Income attributable to noncontrolling interest (3)	—	(17,038)
Net income attributable to Vertex	<u>\$ 268,631</u>	<u>\$ 210,263</u>

Amounts per share attributable to Vertex common shareholders:

Net income:

Basic	\$ 1.05	\$ 0.83
Diluted	\$ 1.03	\$ 0.81

Shares used in per share calculations:

Basic	255,695	253,231
Diluted	260,175	258,526

Reconciliation of GAAP to Non-GAAP Net Income First-Quarter Results

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

	Three Months Ended March 31,	
	2019	2018
GAAP net income attributable to Vertex	\$ 268,631	\$ 210,263
Stock-based compensation expense	93,791	78,136
Increase in fair value of contingent consideration payable to VIE (3)	—	24,000
Increase in fair value of strategic investments (1) and other adjustments (4)	<u>(37,200)</u>	<u>(94,616)</u>
Total non-GAAP adjustments to pre-tax income	56,591	7,520
Estimated income taxes related to non-GAAP adjustments to pre-tax income (5)	<u>(29,392)</u>	<u>(21,859)</u>
Non-GAAP net income attributable to Vertex	<u>\$ 295,830</u>	<u>\$ 195,924</u>

Amounts per diluted share attributable to Vertex common shareholders:

Net income:

GAAP	\$ 1.03	\$ 0.81
Non-GAAP	\$ 1.14	\$ 0.76

Shares used in diluted per share calculations:

GAAP and Non-GAAP	260,175	258,526
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Reconciliation of GAAP to Non-GAAP Revenues and Expenses First-Quarter Results

(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2019	2018
GAAP total revenues	\$ 858,435	\$ 640,799
Other adjustments (4)	<u>(141)</u>	<u>(1,919)</u>
Non-GAAP total revenues	<u>\$ 858,294</u>	<u>\$ 638,880</u>

GAAP cost of sales

	Three Months Ended March 31,	
	2019	2018
	\$ 95,092	\$ 71,613

Stock-based compensation expense	(1,338)	(813)
Non-GAAP cost of sales	<u>\$ 93,754</u>	<u>\$ 70,800</u>
GAAP research and development expenses	\$ 339,490	\$ 310,553
Stock-based compensation expense	(59,715)	(48,488)
Other adjustments (4)	(6,492)	(2,073)
Non-GAAP research and development expenses	<u>\$ 273,283</u>	<u>\$ 259,992</u>
GAAP sales, general and administrative expenses	\$ 147,045	\$ 129,808
Stock-based compensation expense	(32,738)	(28,835)
Other adjustments (4)	—	(1,329)
Non-GAAP sales, general and administrative expenses	<u>\$ 114,307</u>	<u>\$ 99,644</u>
Combined non-GAAP R&D and SG&A expenses	<u>\$ 387,590</u>	<u>\$ 359,636</u>

	Three Months Ended March 31,	
	2019	2018
GAAP other income, net	\$ 42,610	\$ 96,838
Increase in fair value of strategic investments (1)	(43,551)	(95,458)
Non-GAAP other (expense) income, net	<u>\$ (941)</u>	<u>\$ 1,380</u>
GAAP provision for (benefit from) income taxes	\$ 51,534	\$ (12,659)
Estimated income taxes related to non-GAAP adjustments to pre-tax income (5)	29,392	15,454
Non-GAAP provision for income taxes (2)	<u>\$ 80,926</u>	<u>\$ 2,795</u>

Condensed Consolidated Balance Sheets

(in thousands)
(unaudited)

	March 31, 2019	December 31, 2018
Assets		
Cash, cash equivalents and marketable securities	\$ 3,478,035	\$ 3,168,242
Accounts receivable, net	438,297	409,688
Inventories	136,698	124,360
Property and equipment, net	742,559	812,005
Goodwill	50,384	50,384
Deferred tax assets	1,467,518	1,499,672
Other assets	229,623	181,547
Total assets	<u>\$ 6,543,114</u>	<u>\$ 6,245,898</u>
Liabilities and Shareholders' Equity		
Accounts payable and accruals	\$ 615,007	\$ 715,482
Finance lease liabilities	596,106	596,639
Other liabilities	608,688	498,574
Shareholders' equity	4,723,313	4,435,203
Total liabilities and shareholders' equity	<u>\$ 6,543,114</u>	<u>\$ 6,245,898</u>
Common shares outstanding	256,351	255,172

Supplemental Income Tax Information

(in thousands, except percentages)
(unaudited)

	Three Months Ended March 31,	
	2019	2018
Components of provision for (benefit from) income taxes related to:		
Cash taxes paid or accrued for state and foreign income taxes	\$ 4,778	\$ 2,795

VIE provision for income taxes (5)	—	6,405
Provision for income taxes offset by net operating losses	46,756	(21,859)
GAAP provision for (benefit from) income taxes (2)	\$ 51,534	\$ (12,659)
Cash taxes paid or accrued for state and foreign income taxes	\$ 4,778	\$ 2,795
Estimated income taxes attributable to Vertex related to non-GAAP adjustments to pre-tax income (5)	29,392	21,859
Provision for income taxes offset by net operating losses	46,756	(21,859)
Non-GAAP provision for income taxes (2)	\$ 80,926	\$ 2,795

Effective tax rate reconciliation:

GAAP effective tax rate	16 %	(6)%
Impact of GAAP to Non-GAAP adjustments	5 %	7 %
Non-GAAP effective tax rate	21 %	1 %

Notes and Explanations

1: The company recorded gains of \$43.6 million and \$95.5 million in the three months ended March 31, 2019 and 2018, respectively, to "Other income, net," related to changes in the fair value of its strategic investments.

2: In the fourth quarter of 2018, the company recorded a non-cash benefit from income taxes of approximately \$1.5 billion related to the release of its valuation allowance on the majority of its net operating losses and other deferred tax assets. As a result, the company recorded deferred tax assets of \$1.5 billion on its consolidated balance sheet as of December 31, 2018, which were previously subject to its valuation allowance. Starting in the first quarter of 2019, the company began recording a provision for income taxes on its pre-tax income using an estimated effective tax rate that approximates statutory rates. The provision includes a significant non-cash charge due to the company's ability to offset its pre-tax income against previously benefited net operating losses. The company expects the majority of its tax provision to represent a non-cash expense until its net operating losses have been fully utilized. As of December 31, 2018, the company's federal net operating losses and credits that were available to offset future pre-tax income were approximately \$4.5 billion.

3: During the three months ended March 31, 2018, the company consolidated the financial statements of a variable interest entity, or VIE, because Vertex had licensed the rights to develop the VIE's most significant intellectual property asset. During the three months ended March 31, 2018, the fair value of the contingent payments payable by Vertex to the VIE increased by \$24.0 million. This increase was attributable to noncontrolling interest and resulted in a decrease in net income attributable to Vertex on a dollar-for-dollar basis. The company deconsolidated the VIE as of December 31, 2018; therefore, there were no comparable amounts during the three months ended March 31, 2019.

4: "Other adjustments" in the three months ended March 31, 2019 primarily related to collaborative milestone payments. "Other adjustments" in the three months ended March 31, 2018 primarily related to revenues and expenses attributable to our VIE's operations and collaboration revenues and payments including those related to the company's oncology collaboration with Merck KGaA, Darmstadt, Germany.

5: In the three months ended March 31, 2019, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" primarily related to (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation) and (ii) the increase in the fair value of the company's strategic investments. In the three months ended March 31, 2018, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" were related to a provision for income taxes attributable to the company's VIE and excess tax benefits related to stock-based compensation.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for nine years in a row. 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 this press release, the information provided regarding future financial performance, including in the section captioned "Full Year 2019 Financial Guidance" and statements regarding (i) the timing and expected outcome of regulatory applications, including NDAs and MAAs, (ii) the timing of receipt of final Phase 3 24 week data from the triple combination program and (iii) the development plan and timelines for our product development candidates, including our next-generation triple combination regimens, VX-561, VX-121, CTX001, VX-150 and the company's AAT correctors. 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 2019 CF net product revenues, expenses and effective tax rates may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast

The company will host a conference call and webcast today at 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

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