Vertex Reports First-Quarter 2020 Financial Results

April 29, 2020

-Product revenues of $1.52 billion, a 77% increase compared to Q1 2019-

-Company raises revenue guidance; now expects 2020 CF revenues of $5.3 to $5.6 billion-

BOSTON--(BUSINESS WIRE)--Apr. 29, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2020 and revised upward its full-year 2020 financial guidance for total cystic fibrosis (CF) product revenues.

"The COVID-19 pandemic has presented unprecedented challenges to societies, communities and businesses around the world, and while these global challenges will continue for some time to come, I am very proud of how Vertex has responded to ensure that we continue to deliver on our mission for patients, keep our employees safe and achieve our business goals," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "Importantly, our business continues to grow, and throughout the first quarter, thousands of patients initiated treatment with our medicines worldwide. The U.S. launch of TRIKAFTA has been remarkable, with the majority of eligible patients having now initiated treatment with this medicine. This strong interest reflects TRIKAFTA's substantial benefits for patients and has resulted in significant increases in revenue to support continued investment in both our internal pipeline and business development efforts to support future growth. Looking ahead, we continue to be differentiated by our focus on serial innovation, investment in transformative medicines aimed at the underlying cause of disease, the breadth of our pipeline and capabilities and our financial strength."

First-Quarter 2020 Financial Highlights

	Three Months Ended March 31,		%
	2020	**2019**	**Change**
	(in millions, except per share amounts)		
Product revenues, net	$ 1,515	$ 857	77%
TRIKAFTA	$ 895	$ —	
SYMDEKO/SYMKEVI	$ 173	$ 320	
ORKAMBI	$ 234	$ 293	
KALYDECO	$ 213	$ 244	
GAAP Operating income	$ 720	$ 277	160%
Non-GAAP Operating income	$ 877	$ 377	133%
GAAP Net income	$ 603	$ 269	124%
Non-GAAP Net income	$ 674	$ 296	128%
GAAP Net income per share - diluted	$ 2.29	$ 1.03	122%

Non-GAAP Net income per share - diluted	$ 2.56	$ 1.14	125%

Total product revenues increased 77% compared to the first quarter of 2019, primarily driven by the uptake of TRIKAFTA in the U.S. and the uptake of our medicines outside the U.S. following the completion of key reimbursement agreements in 2019.

GAAP and Non-GAAP net income each increased more than 120% compared to the first quarter of 2019, largely driven by the strong growth in total product revenues.

Cash, cash equivalents and marketable securities as of March 31, 2020 were $4.2 billion, an increase of approximately $400 million compared to $3.8 billion as of December 31, 2019.

First-Quarter 2020 Expenses

	Three Months Ended March 31,	
	2020	**2019**
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 631	$ 487
Combined Non-GAAP R&D and SG&A expenses	$ 477	$ 388
GAAP R&D expense	$ 449	$ 339
Non-GAAP R&D expense	$ 337	$ 273
GAAP SG&A expense	$ 182	$ 147
Non-GAAP SG&A expense	$ 140	$ 114
GAAP income taxes	$ 55	$ 52
Non-GAAP income taxes	$ 184	$ 81
GAAP effective tax rate	8 %	16 %
Non-GAAP effective tax rate	21 %	21 %

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to the first quarter of 2019, 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 compared to the first quarter of 2019 primarily due to Vertex's increased operating 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 2020 Financial Guidance

Vertex today revised upward its guidance for full-year 2020 CF product revenues and reiterated its guidance for GAAP and non-GAAP combined R&D and SG&A expenses and for its non-GAAP effective tax rate, as summarized below:

	Current FY 2020	Previous FY 2020

TOTAL product revenues	$ 5.3 to 5.6 billion	$ 5.1 to 5.3 billion
Combined GAAP R&D and SG&A expenses	Unchanged	$ 2.4 to 2.55 billion
Combined Non-GAAP R&D and SG&A expenses	Unchanged	$ 1.95 to 2.0 billion
Non-GAAP effective tax rate	Unchanged	21% to 22%

Key Business Highlights:

Approved Medicines:

Supply Chain for Approved CF Medicines

- The global COVID-19 outbreak has not had any impact on the continuity of Vertex's supply chain for its approved medicines.
- Vertex remains highly confident in its ability to continue to supply all of its approved medicines to patients around the world.

TRIKAFTA (elexacaftor, tezacaftor and ivacaftor)

- Vertex has seen rapid uptake of TRIKAFTA across all groups of eligible patients following approval of this medicine by the U.S. Food and Drug Administration (FDA) in October 2019. The majority of the approximately 18,000 eligible patients have now initiated treatment with TRIKAFTA.
- In Europe, the Marketing Authorization Application for the elexacaftor, tezacaftor and ivacaftor triple combination in patients with at least one *F508del* mutation ages 12 and older continues to be under review with the European Medicines Agency (EMA).
- Vertex also recently submitted applications for approval of the elexacaftor, tezacaftor and ivacaftor triple combination for patients with at least one *F508del* mutation ages 12 and older in Australia and Switzerland.
- Vertex recently completed enrollment for a Phase 3 study evaluating the use of the elexacaftor, tezacaftor and ivacaftor triple combination regimen in children with CF ages 6 through 11 who have two copies of the *F508del* mutation or who have one *F508del* mutation and one minimal function mutation. Pending data from the study, Vertex plans to submit a supplemental New Drug Application (sNDA) to the U.S. FDA in the second half of 2020 for children ages 6-11 with at least one *F508del* mutation, followed by regulatory submissions in other countries.

KALYDECO (ivacaftor)

- Vertex recently completed the submission of an sNDA to the U.S. FDA and Type 2 variation to the EMA for the use of KALYDECO in infants ages four to less than six months. KALYDECO is currently approved for use in infants as young as six months of age in both geographies.

Development Pipeline:

Vertex continues to progress its expanding pipeline of programs in the clinic, which span various diseases, modalities and stages of development. To ensure patient safety and reduce the burden on the healthcare system at a time of critical need, Vertex has temporarily paused or delayed enrollment in certain studies.

Alpha-1 Antitrypsin (AAT) Deficiency:

- Vertex has temporarily paused screening and enrollment in the Phase 2 study of VX-814; however, the study remains active and Vertex continues to initiate new clinical trial sites to enable future patient enrollment.

Beta Thalassemia and Sickle Cell Disease

- Vertex and its partner CRISPR Therapeutics remain on track to provide additional data from the two ongoing Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 in patients with transfusion-dependent beta thalassemia and in patients with severe sickle cell disease in 2020. New data expected in 2020 include initial data from additional patients dosed in each of the Phase 1/2 studies and longer duration follow-up data for the first patients dosed in each study. Screening, enrollment and mobilization in these studies is ongoing; however, no additional patients are

scheduled to initiate conditioning or dosing at this time.

Focal Segmental Glomerulosclerosis (FSGS):

- Vertex recently initiated a Phase 2 proof-of-concept study of VX-147 in people with FSGS.
- The 13-week open-label Phase 2 study is designed to evaluate the reduction in proteinuria in people with FSGS after treatment with VX-147.

Type 1 Diabetes:

- Vertex continues to advance its cell therapy program for the treatment of type 1 diabetes and expects to initiate clinical development in patients in late 2020 or early 2021.

Investments in External Innovation

- Expanding Capabilities in Genetic Therapies: In April, Vertex entered into a collaboration with Affinia Therapeutics to gain access to a novel library of AAV capsids that will bolster Vertex's ongoing research and development work in genetic therapies. The goal of the collaboration will be to develop genetic therapies for people affected by Duchenne muscular dystrophy (DMD), myotonic dystrophy 1 (DM1) and CF.
- mRNA Therapies for CF: Based on promising preclinical data generated to date, Vertex and Moderna recently extended their research collaboration aimed at the discovery and development of mRNA therapeutics for the treatment of CF.

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 collaboration agreements, (iii) gains or losses related to the fair value of the company's strategic investments, (iv) acquisition-related costs and (v) other adjustments. The company's non-GAAP financial results also exclude from its provision for income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income described above and certain discrete items. 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,	
	2020	**2019**
Revenues:		
Product revenues, net	$ 1,515,107	$ 857,253
Collaboration and royalty revenues	—	1,182
Total revenues	1,515,107	858,435
Costs and expenses:		

Cost of sales	162,497	95,092
Research and development expenses	448,528	339,490
Sales, general and administrative expenses	182,258	147,045
Change in fair value of contingent consideration	1,600	—
Total costs and expenses	794,883	581,627
Income from operations	720,224	276,808
Interest income	12,576	15,615
Interest expense	(14,136)	(14,868)
Other (expense) income, net (1)	(61,130)	42,610
Income before provision for income taxes	657,534	320,165
Provision for income taxes	54,781	51,534
Net income	$ 602,753	$ 268,631
Net income per common share:		
Basic	$ 2.32	$ 1.05
Diluted	$ 2.29	$ 1.03
Shares used in per share calculations:		
Basic	259,815	255,695
Diluted	263,515	260,175

Reconciliation of GAAP to Non-GAAP Net Income

First-Quarter Results

(in thousands, except per share amounts)

(unaudited)

	Three Months Ended March 31,	
	2020	**2019**
GAAP net income	$ 602,753	$ 268,631
Stock-based compensation expense	115,706	93,791

Decrease (increase) in fair value of strategic investments (1)	44,870	(43,551)
Increase in fair value of contingent consideration (2)	1,600	—
Collaborative revenues and expenses (3)	36,250	6,351
Acquisition-related costs (4)	2,883	—
Total non-GAAP adjustments to pre-tax income	201,309	56,591
Tax adjustments (5)	(129,608)	(29,392)
Non-GAAP net income	$ 674,454	$ 295,830
Net income per diluted common share:		
GAAP	$ 2.29	$ 1.03
Non-GAAP	$ 2.56	$ 1.14
Shares used in diluted per share calculations:		
GAAP and Non-GAAP	263,515	260,175

Reconciliation of GAAP to Non-GAAP Revenues and Expenses

First-Quarter Results

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2020	**2019**
GAAP total revenues	$ 1,515,107	$ 858,435
Collaborative revenues	—	(141)
Non-GAAP total revenues	$ 1,515,107	$ 858,294

	Three Months Ended March 31,	
	2020	**2019**
GAAP cost of sales	$ 162,497	$ 95,092
Stock-based compensation expense	(1,361)	(1,338)

Non-GAAP cost of sales	$ 161,136	$ 93,754
GAAP research and development expenses	$ 448,528	$ 339,490
Stock-based compensation expense	(72,687)	(59,715)
Collaborative expenses (3)	(36,250)	(6,492)
Acquisition-related costs (4)	(2,678)	—
Non-GAAP research and development expenses	$ 336,913	$ 273,283
GAAP sales, general and administrative expenses	$ 182,258	$ 147,045
Stock-based compensation expense	(41,658)	(32,738)
Acquisition-related costs (4)	(205)	—
Non-GAAP sales, general and administrative expenses	$ 140,395	$ 114,307
Combined non-GAAP R&D and SG&A expenses	$ 477,308	$ 387,590

	Three Months Ended March 31,	
	2020	**2019**
GAAP other (expense) income, net	$ (61,130)	$ 42,610
Decrease (increase) in fair value of strategic investments (1)	44,870	(43,551)
Non-GAAP other expense, net	$ (16,260)	$ (941)
GAAP provision for income taxes	$ 54,781	$ 51,534
Tax adjustments (5)	129,608	29,392
Non-GAAP provision for income taxes (6)	$ 184,389	$ 80,926

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	March 31, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 4,190,396	$ 3,808,294
Accounts receivable, net	845,269	633,518
Inventories	187,087	167,502
Property and equipment, net	736,303	745,080
Goodwill and intangible assets	1,402,158	1,402,158
Deferred tax assets	1,147,705	1,190,815
Other assets	384,283	371,098
Total assets	$ 8,893,201	$ 8,318,465
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 1,358,974	$ 1,204,522
Finance lease liabilities	572,916	577,371
Contingent consideration	178,100	176,500
Other liabilities	321,557	274,828
Shareholders' equity	6,461,654	6,085,244
Total liabilities and shareholders' equity	$ 8,893,201	$ 8,318,465
Common shares outstanding	259,079	258,993

Supplemental Income Tax Information

(in thousands, except percentages)

(unaudited)

	Three Months Ended March 31,	
	2020	**2019**
Components of provision for income taxes related to:		

Cash paid or accrued for income taxes	$ 9,370	$ 4,778
Provision for income taxes offset by net operating losses	45,411	46,756
GAAP provision for income taxes (6)	$ 54,781	$ 51,534

Cash paid or accrued for income taxes	$ 9,370	$ 4,778
Tax adjustments (5)	129,608	29,392
Provision for income taxes offset by net operating losses	45,411	46,756
Non-GAAP provision for income taxes (6)	$ 184,389	$ 80,926

Effective tax rate reconciliation:

GAAP effective tax rate	8 %	16 %
Impact of GAAP to Non-GAAP adjustments	13 %	5 %
Non-GAAP effective tax rate	21 %	21 %

Notes and Explanations

1: "Other income, net" includes gains and losses related to changes in the fair value of the company's strategic investments.

2: During the three months ended March 31, 2020, the increase in the fair value of the contingent consideration relates to potential payments to Exonics' former equity holders.

3: "Collaborative revenues and expenses" in the three months ended March 31, 2020 and 2019 primarily related to collaborative milestone payments.

4: "Acquisition-related costs" in the three months ended March 31, 2020 related to costs associated with the company's acquisitions of Semma and Exonics. There were no comparable amounts during the three months ended March 31, 2019.

5: In the three months ended March 31, 2020 and 2019, "Tax adjustments" primarily related to the estimated income taxes related to non-GAAP adjustments to pre-tax income including (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation), (ii) increases or decreases in the fair value of the company's strategic investments and (iii) collaborative payments. In the three months ended March 31, 2020, "Tax adjustments" also included a non-recurring discrete benefit to the company's provision for income taxes of approximately $50 million that the company excluded from its Non-GAAP measures.

6: The company records a provision for income taxes on its pre-tax income using an effective tax rate approximating 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 a portion of its tax provision to represent a non-cash expense until its net operating losses have been fully utilized. As of December 31, 2019, the company's federal net operating losses and credits that were available to offset future pre-tax income were approximately $3.5 billion.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 10 consecutive years on Science magazine's Top

Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

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. Kewalramani's statements in this press release, the information provided regarding future financial performance, the section captioned "Full-Year 2020 Financial Guidance" and statements regarding (i) regulatory filings, (ii) the development plan and timelines for the company's drug candidates and (iii) the company's expectations regarding the effects COVID-19 will have on its business and operations. 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 risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2020 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 COVID-19 may have different or more significant impacts on the company's business or operations than the company currently expects, that data from the company's development programs may not support registration or further development of its potential medicines due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and subsequent 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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Investors:
Michael Partridge, 617-341-6108
or
Zach Barber, 617-341-6470
or
Brenda Eustace, 617-341-6187

Media:
617-341-6992
mediainfo@vrtx.com

Source: Vertex Pharmaceuticals Incorporated