



Vertex Reports Second-Quarter 2020 Financial Results

July 30, 2020

-Product revenues of \$1.52 billion, a 62% increase compared to Q2 2019-

-Company raises revenue guidance; now expects 2020 CF revenues of \$5.7 to \$5.9 billion-

BOSTON--(BUSINESS WIRE)--Jul. 30, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2020 and revised upward its full-year 2020 financial guidance for total cystic fibrosis (CF) product revenues.

"This has been an exceptional first half for Vertex on all fronts and most importantly, in our efforts to bring our CF medicines to more people around the world. We have seen remarkable uptake of TRIKAFTA in the U.S., with the majority of eligible patients now taking this medicine; and in Europe, we secured a positive CHMP opinion earlier than expected and entered into a landmark expansion of our reimbursement agreement with NHS England that will give patients in England access to this medicine rapidly following European Commission approval," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "Additionally, despite the challenges of this unprecedented year, we have continued to make steady progress in our research programs and across our clinical development pipeline that will position us for continued growth into the future."

Second-Quarter 2020 Financial Highlights

	Three Months Ended June 30,		%
	2020	2019	
	(in millions, except per share amounts)		
Product revenues, net	\$ 1,524	\$ 940	62%
TRIKAFTA	\$ 918	\$ —	
SYMDEKO/SYMKEVI	\$ 172	\$ 362	
ORKAMBI	\$ 232	\$ 316	
KALYDECO	\$ 203	\$ 262	
GAAP Operating income	\$ 718	\$ 270	166%
Non-GAAP Operating income	\$ 874	\$ 413	112%
GAAP Net income	\$ 837	\$ 267	213%
Non-GAAP Net income	\$ 687	\$ 327	110%
GAAP Net income per share - diluted	\$ 3.18	\$ 1.03	209%
Non-GAAP Net income per share - diluted	\$ 2.61	\$ 1.26	107%

Total product revenues increased 62% compared to the second 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 increased 213% and 110%, respectively, compared to the second quarter of 2019, largely driven by the strong growth in total product revenues.

Cash, cash equivalents and marketable securities as of June 30, 2020 were \$5.5 billion, an increase of approximately \$1.6 billion compared to \$3.8 billion as of December 31, 2019.

Second-Quarter 2020 Expenses

Three Months Ended June 30,	
2020	2019
(in millions)	

Combined GAAP R&D and SG&A expenses	\$ 613	\$ 536
Combined Non-GAAP R&D and SG&A expenses	\$ 467	\$ 394
GAAP R&D expense	\$ 421	\$ 379
Non-GAAP R&D expense	\$ 321	\$ 271
GAAP SG&A expense	\$ 192	\$ 157
Non-GAAP SG&A expense	\$ 146	\$ 123
GAAP income taxes	\$ (13)	\$ 60
Non-GAAP income taxes	\$ 184	\$ 86
GAAP effective tax rate	(2)%	18%
Non-GAAP effective tax rate	21%	21%

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to the second 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 income taxes decreased compared to the second quarter of 2019. **Non-GAAP income taxes** increased compared to the second quarter of 2019 primarily due to Vertex's increased operating income. Refer to the "Supplemental Income Tax Information" section 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:

	<u>Current FY 2020</u>	<u>Previous FY 2020</u>
TOTAL product revenues	\$5.7 to 5.9 billion	\$5.3 to 5.6 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:

TRIKAFTA/KAFTRIO (elxacaftor, tezacaftor and ivacaftor)

- The majority of the approximately 18,000 eligible patients have initiated treatment with TRIKAFTA.
- In June, the European Medical Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for KAFTRIO for the treatment of European CF patients ages 12 and older with one *F508del* mutation and one minimal function mutation or two *F508del* mutations.
- In June, Vertex also announced the expansion of its reimbursement agreement with the National Health Service (NHS) England to include KAFTRIO, ahead of the medicine's anticipated approval by the European Commission. If approved, KAFTRIO will be available to people with CF in England ages 12 and older with one *F508del* mutation and one minimal function mutation or two *F508del* mutations.
- In July, Vertex announced positive Phase 3 study results for TRIKAFTA in people with CF ages 12 and older who have one copy of the *F508del* mutation and one gating or residual function mutation. This study was a post-marketing commitment and will be submitted to the U.S. FDA. In addition, the study data will be submitted to the EMA to support future indication expansion of the European Union (EU) label.
- Data from the Phase 3 study evaluating the use of the elxacaftor, tezacaftor and ivacaftor triple combination 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 is expected in the second half of 2020. Pending data from the study, Vertex will submit a supplemental New Drug Application (sNDA) to the U.S. FDA in the fourth quarter of 2020 for children ages 6 through 11 with at least one *F508del* mutation, followed by regulatory submissions in other countries.

SYMDEKO/SYMKEVI (tezacaftor and ivacaftor)

- The EMA review of the application for use of SYMKEVI in patients ages 6 through 11 in Europe is ongoing. If approved,

this will be the first CFTR modulator to treat patients ages 6 through 11 with residual function mutations in the EU.

KALYDECO (ivacaftor)

- In June, Vertex announced that the European Commission granted approval of the label extension for KALYDECO for the treatment of children and adolescents ages 6 months and older who have the *R117H* mutation.

Development Pipeline:

Vertex continues to progress a broad pipeline of potentially transformative small molecule, cell and genetic therapies aimed at serious diseases. Recent and anticipated progress for key pipeline programs is noted below:

Beta Thalassemia and Sickle Cell Disease:

- Vertex and its partner CRISPR Therapeutics provided new clinical data at the European Hematology Association (EHA) Congress from the two ongoing Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 in patients with transfusion-dependent beta thalassemia (TDT) and in patients with severe sickle cell disease (SCD). Data from two TDT patients demonstrated clinical proof-of-concept for CTX001 in this disease, and longer duration data from one SCD patient showed a durable effect on HbF levels and the patient was free of vaso-occlusive crises. Screening, enrollment and mobilization in these studies is ongoing; conditioning and dosing have been resumed following temporary COVID-19-related pauses in both studies. Vertex and CRISPR Therapeutics expect to report data from additional patients in the second half of 2020.

Alpha-1 Antitrypsin (AAT) Deficiency:

- Vertex is evaluating multiple compounds with the potential to correct the misfolding of Z-AAT protein in the liver, in order to increase the levels of functional AAT in the blood. Misfolded Z-AAT protein is the root cause of AAT deficiency.
- Enrollment and dosing have been re-initiated at some but not all sites following a temporary COVID-19-related pause in a Phase 2 proof-of-concept study designed to evaluate the levels of circulating, functional AAT protein after treatment with VX-814.
- A Phase 2 proof-of-concept study for a second Z-AAT corrector, VX-864, was initiated in July.

APOL1-mediated Kidney Diseases:

- Vertex is evaluating the potential for inhibitors of APOL1 function to reduce proteinuria in people with serious kidney diseases, including focal segmental glomerulosclerosis (FSGS).
- Enrollment is underway at multiple clinical trial sites in a Phase 2 proof-of-concept study designed to evaluate the reduction in proteinuria in people with APOL1-mediated FSGS after treatment with VX-147.

Type 1 Diabetes (T1D):

- Vertex is developing a cell therapy designed to replace insulin-producing islet cells in people with T1D. Two opportunities exist for the transplant of these functional islets into patients: 1) transplantation of islet cells alone, using immunosuppression to protect the implanted cells and 2) implantation of the islet cells inside a novel immunoprotective device.
- Vertex plans to submit an Investigational New Drug (IND) application to the U.S. FDA for the islet cells alone program in late 2020 to support evaluation of this potential therapy in patients with T1D.

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
Second-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues:				
Product revenues, net	\$ 1,524,485	\$ 940,380	\$ 3,039,592	\$ 1,797,633
Collaboration and royalty revenues	—	913	—	2,095
Total revenues	1,524,485	941,293	3,039,592	1,799,728
Costs and expenses:				
Cost of sales	184,520	135,740	347,017	230,832
Research and development expenses	420,928	379,091	869,456	718,581
Sales, general and administrative expenses	191,804	156,502	374,062	303,547
Change in fair value of contingent consideration	9,200	—	10,800	—
Total costs and expenses	806,452	671,333	1,601,335	1,252,960
Income from operations	718,033	269,960	1,438,257	546,768
Interest income	4,243	18,076	16,819	33,691
Interest expense	(13,871)	(14,837)	(28,007)	(29,705)
Other income, net (1)	116,365	53,939	55,235	96,549
Income before (benefit from) provision for income taxes	824,770	327,138	1,482,304	647,303
(Benefit from) provision for income taxes	(12,500)	59,711	42,281	111,245
Net income	<u>\$ 837,270</u>	<u>\$ 267,427</u>	<u>\$ 1,440,023</u>	<u>\$ 536,058</u>
Net income per common share:				
Basic	\$ 3.22	\$ 1.04	\$ 5.54	\$ 2.09
Diluted	\$ 3.18	\$ 1.03	\$ 5.46	\$ 2.06
Shares used in per share calculations:				
Basic	259,637	256,154	260,013	255,941
Diluted	263,403	259,822	263,746	260,015

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
GAAP net income	\$ 837,270	\$ 267,427	\$ 1,440,023	\$ 536,058
Stock-based compensation expense	117,189	89,687	232,895	183,478
Increase in fair value of strategic investments (1)	(109,986)	(56,527)	(65,116)	(100,078)
Increase in fair value of contingent consideration (2)	9,200	—	10,800	—
Collaborative revenues and expenses (3)	27,000	52,158	63,250	58,509
Acquisition-related costs (4)	2,456	1,231	5,339	1,231
Total non-GAAP adjustments to pre-tax income	45,859	86,549	247,168	143,140
Tax adjustments (5)	(196,325)	(26,710)	(325,933)	(56,102)

Non-GAAP net income	<u>\$ 686,804</u>	<u>\$ 327,266</u>	<u>\$ 1,361,258</u>	<u>\$ 623,096</u>
Net income per diluted common share:				
GAAP	\$ 3.18	\$ 1.03	\$ 5.46	\$ 2.06
Non-GAAP	\$ 2.61	\$ 1.26	\$ 5.16	\$ 2.40
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	263,403	259,822	263,746	260,015

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,	
	2020	2019	2020	2019
GAAP total revenues	\$ 1,524,485	\$ 941,293	\$ 3,039,592	\$ 1,799,728
Collaborative revenues	—	(17)	—	(158)
Non-GAAP total revenues	<u>\$ 1,524,485</u>	<u>\$ 941,276</u>	<u>\$ 3,039,592</u>	<u>\$ 1,799,570</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
GAAP cost of sales	\$ 184,520	\$ 135,740	\$ 347,017	\$ 230,832
Stock-based compensation expense	(1,387)	(1,503)	(2,748)	(2,841)
Non-GAAP cost of sales	<u>\$ 183,133</u>	<u>\$ 134,237</u>	<u>\$ 344,269</u>	<u>\$ 227,991</u>
GAAP research and development expenses	\$ 420,928	\$ 379,091	\$ 869,456	\$ 718,581
Stock-based compensation expense	(70,275)	(55,632)	(142,962)	(115,347)
Collaborative expenses (3)	(27,000)	(52,175)	(63,250)	(58,667)
Acquisition-related costs (4)	(2,208)	—	(4,886)	—
Non-GAAP research and development expenses	<u>\$ 321,445</u>	<u>\$ 271,284</u>	<u>\$ 658,358</u>	<u>\$ 544,567</u>
GAAP sales, general and administrative expenses	\$ 191,804	\$ 156,502	\$ 374,062	\$ 303,547
Stock-based compensation expense	(45,527)	(32,552)	(87,185)	(65,290)
Acquisition-related costs (4)	(248)	(1,231)	(453)	(1,231)
Non-GAAP sales, general and administrative expenses	<u>\$ 146,029</u>	<u>\$ 122,719</u>	<u>\$ 286,424</u>	<u>\$ 237,026</u>
Combined non-GAAP R&D and SG&A expenses	<u>\$ 467,474</u>	<u>\$ 394,003</u>	<u>\$ 944,782</u>	<u>\$ 781,593</u>
	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
GAAP other income, net	\$ 116,365	\$ 53,939	\$ 55,235	\$ 96,549
Increase in fair value of strategic investments (1)	(109,986)	(56,527)	(65,116)	(100,078)
Non-GAAP other income (expense), net	<u>\$ 6,379</u>	<u>\$ (2,588)</u>	<u>\$ (9,881)</u>	<u>\$ (3,529)</u>
GAAP (benefit from) provision for income taxes	\$ (12,500)	\$ 59,711	\$ 42,281	\$ 111,245
Tax adjustments (5)	196,325	26,710	325,933	56,102
Non-GAAP provision for income taxes (6)	<u>\$ 183,825</u>	<u>\$ 86,421</u>	<u>\$ 368,214</u>	<u>\$ 167,347</u>

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	June 30, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	\$ 5,450,769	\$ 3,808,294
Accounts receivable, net	791,768	633,518
Inventories	219,218	167,502
Property and equipment, net	728,357	745,080
Goodwill and intangible assets	1,402,158	1,402,158
Deferred tax assets	1,214,968	1,190,815
Other assets	409,129	371,098
Total assets	\$ 10,216,367	\$ 8,318,465
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	\$ 1,646,858	\$ 1,204,522
Finance lease liabilities	562,474	577,371
Contingent consideration	187,300	176,500
Other liabilities	300,493	274,828
Shareholders' equity	7,519,242	6,085,244
Total liabilities and shareholders' equity	\$ 10,216,367	\$ 8,318,465
Common shares outstanding	260,124	258,993

Supplemental Income Tax Information
(in thousands, except percentages)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Components of provision for (benefit from) income taxes related to:				
Cash paid or accrued for income taxes	\$ 38,226	\$ 5,214	\$ 47,596	\$ 9,992
Benefits from income taxes due to discrete tax items (5)	(187,000)	—	(237,355)	—
Provision for income taxes offset by net operating losses and credits (6)	136,274	54,497	232,040	101,253
GAAP (benefit from) provision for income taxes (6)	\$ (12,500)	\$ 59,711	\$ 42,281	\$ 111,245
Cash paid or accrued for income taxes	\$ 38,226	\$ 5,214	\$ 47,596	\$ 9,992
Adjustments to pre-tax income	9,325	26,710	88,578	56,102
Provision for income taxes offset by net operating losses and credits (6)	136,274	54,497	232,040	101,253
Non-GAAP provision for income taxes (6)	\$ 183,825	\$ 86,421	\$ 368,214	\$ 167,347

Effective tax rate reconciliation:

GAAP effective tax rate	(2)%	18%	3%	17%
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Impact of GAAP to Non-GAAP adjustments	23%	3%	18%	4%
Non-GAAP effective tax rate	21%	21%	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 and six months ended June 30, 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 and six months ended June 30, 2020 and 2019 primarily related to collaborative upfront and milestone payments.
- 4: "Acquisition-related costs" in the three and six months ended June 30, 2020 related to costs associated with the company's acquisitions of Semma and Exonics. There were no comparable amounts during the three and six months ended June 30, 2019.
- 5: In the three and six months ended June 30, 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 and six months ended June 30, 2020, "Tax adjustments" also included non-recurring discrete benefits to the company's provision for income taxes of approximately \$187 million and \$237 million, respectively, 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 and credits. The company expects a portion of its tax provision to represent a non-cash expense until its net operating losses and credits 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 and data submissions, (ii) anticipated regulatory approvals, including the anticipated KAFTRIO and SYMKEVI approvals, and future label expansions, and (iii) the development plan and timelines for the company's medicines, drug candidates and pipeline programs. 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 5:30 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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