



Vertex Reports Second-Quarter 2019 Financial Results

July 31, 2019

- Product revenues of \$940 million, a 25% increase compared to 2018 -

- Company increases full-year 2019 total product revenue guidance to \$3.6 to \$3.7 billion -

- Company advancing programs in 5 additional diseases beyond cystic fibrosis -

BOSTON--(BUSINESS WIRE)--Jul. 31, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2019 and increased its full-year 2019 total product revenue guidance.

"We have made tremendous progress across our business in 2019 thus far. In CF, we submitted a New Drug Application to the FDA for our VX-445 triple combination regimen, which we believe has the potential to treat up to 90% of all CF patients in the future. We continue to focus on ensuring all eligible patients have access to our CF medicines as early as possible," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Additionally, we have rapidly grown our pipeline beyond CF, advancing seven new potential medicines across five disease areas, including beta thalassemia, sickle cell disease, alpha-1 antitrypsin deficiency, APOL1-mediated kidney diseases and pain. And through our expanded collaboration with CRISPR Therapeutics and acquisition of Exonics Therapeutics, we have now established a leading gene editing platform for the treatment of Duchenne Muscular Dystrophy and Myotonic Dystrophy Type 1."

Second-Quarter 2019 Financial Highlights

	Three Months Ended June 30,		%
	2019	2018	
	(in millions, except per share amounts)		
Total product revenues, net	\$ 940	\$ 750	25%
KALYDECO	\$ 262	\$ 253	
ORKAMBI	\$ 316	\$ 311	
SYMDEKO/SYMKEVI	\$ 362	\$ 186	
GAAP Operating income	\$ 270	\$ 173	56%
Non-GAAP Operating income	\$ 413	\$ 260	59%
GAAP Net income	\$ 267	\$ 207	29%
Non-GAAP Net income	\$ 327	\$ 244	34%
GAAP Net income per share - diluted	\$ 1.03	\$ 0.80	29%
Non-GAAP Net income per share - diluted	\$ 1.26	\$ 0.94	34%

Total product revenues increased 25% compared to the second quarter of 2018, primarily driven by the uptake of SYMDEKO in the U.S. and SYMKEVI in Germany.

GAAP net income increased compared to the second quarter of 2018, driven by the strong growth in total product revenues, and was partially offset by increases in operating expenses, including a \$50 million upfront payment as part of Vertex's recent collaboration with Kymera Therapeutics, and income taxes.

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

Cash, cash equivalents and marketable securities as of June 30, 2019 were \$4.0 billion, an increase of approximately \$800 million compared to \$3.2 billion as of December 31, 2018.

Second-Quarter 2019 Expenses

	Three Months Ended June 30,	
	2019	2018
	(in millions)	
Combined GAAP R&D and SG&A expenses	\$ 536	\$ 475
Combined Non-GAAP R&D and SG&A expenses	\$ 394	\$ 388
GAAP R&D expense	\$ 379	\$ 338
Non-GAAP R&D expense	\$ 271	\$ 281
GAAP SG&A expense	\$ 157	\$ 137
Non-GAAP SG&A expense	\$ 123	\$ 107
GAAP income taxes	\$ 60	\$ 10
Non-GAAP income taxes	\$ 86	\$ 6

Combined GAAP R&D and SG&A expenses increased compared to the second quarter of 2018 primarily due to the \$50 million upfront payment to Kymera Therapeutics.

Combined Non-GAAP R&D and SG&A expenses were similar to the second quarter of 2018.

GAAP and Non-GAAP income taxes increased significantly compared to the second 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 second 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. Refer to "Supplemental Income Tax Information" for discussion of the cash versus non-cash components of Vertex's provision for income taxes.

Share Repurchase Program

In order to reduce the impact of dilution from employee equity programs, the Board of Directors has authorized a share repurchase program of up to \$500 million of common stock through December 31, 2020.

The repurchase is expected to be executed from time to time, subject to general business and market conditions and other investment opportunities, through open market purchases or privately negotiated transactions, including through Rule 10b5-1 plans.

Full-Year 2019 Financial Guidance

Vertex today announced updates to its 2019 financial guidance as summarized below:

	<u>Current FY 2019</u>	<u>Previous FY 2019</u>
TOTAL product revenues	\$ 3.60 to 3.70 billion	\$ 3.45 to 3.55 billion
Combined GAAP R&D and SG&A expenses	\$ 2.25 to 2.40 billion	\$ 2.00 to 2.15 billion
Combined Non-GAAP R&D and SG&A expenses	Unchanged	\$ 1.65 to 1.70 billion
Non-GAAP effective tax rate	Unchanged	21% - 22%

The increase in total product revenue guidance is based on the strong product performance in the first half of 2019.

The company's revised combined GAAP R&D and SG&A expense guidance reflects upfront payments made to CRISPR Therapeutics and Kymera Therapeutics for transactions announced in the second quarter of 2019.

Business Highlights

CF CLINICAL DEVELOPMENT

- On July 22, 2019, the company announced that it submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for the triple combination of VX-445 (elexacaftor), tezacaftor and ivacaftor. A Marketing Authorization Application (MAA) submission to the European Medicines Agency (EMA) is planned for the fourth quarter of 2019.
- Enrollment is ongoing in a Phase 3 study evaluating the triple combination of elexacaftor, tezacaftor and ivacaftor in children ages 6 to 11 years.
- A Phase 2 dose-ranging study is ongoing to evaluate the once-daily potentiator VX-561 to support potential Phase 3 development of VX-561 in a once-daily triple combination regimen.
- A Phase 2 study is ongoing to evaluate the next-generation corrector, VX-121, in combination with VX-561 and tezacaftor as a potential once-daily triple combination regimen. VX-121 was granted Fast Track Designation by the FDA in the second quarter of 2019.
- 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 SYMDEKO in the U.S. for children ages 6 to 11 years; MAA submission in the EU planned for the second half of 2019
 - Approval for ORKAMBI in Australia for children ages 2 to 5 years
 - Approval for KALYDECO in Australia for children ages 12 to <24 months

CLINICAL DEVELOPMENT

Alpha-1 Antitrypsin (AAT) Program:

- Vertex has completed its evaluation of single and multiple doses of VX-814, the company's first investigational molecule for the treatment of alpha-1 antitrypsin (AAT) deficiency, in healthy volunteers. Based on the safety, tolerability and pharmacokinetic data from this study, the company plans to advance VX-814 into a Phase 2 dose-ranging study in patients with two Z mutations.
- The company expects to have clinical data from its AAT program in people who have two Z mutations in 2020.
- Vertex has also advanced a second investigational small molecule corrector, VX-864, into Phase 1 clinical development. Both VX-814 and VX-864 have received Fast Track Designation by the FDA.

Sickle Cell Disease and Beta Thalassemia:

- Vertex and its partner CRISPR Therapeutics have dosed the first patient in the Phase 1/2 clinical study of severe sickle cell disease using the novel gene-editing therapy CTX001. The first patient with beta thalassemia was dosed in the first quarter of this year.

APOL1-Mediated Kidney Diseases:

- Vertex has initiated a Phase 1 study evaluating VX-147, the company's first investigational oral small molecule medicine for

the treatment of APOL1-mediated focal segmental glomerulosclerosis (FSGS) and other serious kidney diseases. VX-147 is designed to inhibit APOL1 function, which is a causal genetic factor in FSGS and other proteinuric kidney diseases. Vertex is also advancing multiple other APOL1 inhibitors through preclinical development.

INVESTMENTS IN EXTERNAL INNOVATION

- On June 6, 2019, Vertex announced that it is expanding its collaboration with CRISPR Therapeutics and acquiring Exonics Therapeutics with the goal of developing novel therapies for Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1). Vertex completed the CRISPR and Exonics transactions in July 2019.
- On May 15, 2019, Vertex and Kymera Therapeutics entered into a four-year strategic research and development collaboration to develop small molecule protein degraders against multiple targets. The collaboration will leverage Kymera's expertise in targeted protein degradation and its proprietary Pegasus™ drug discovery platform and Vertex's scientific, clinical, and regulatory capabilities to accelerate the development of first-in-class medicines for people with serious diseases.

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, (iii) gains or losses related to the fair value of the company's strategic investments and (iv) other adjustments. 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
Second-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenues:				
Product revenues, net	\$ 940,380	\$ 749,912	\$ 1,797,633	\$ 1,387,641
Collaboration and royalty revenues	913	2,245	2,095	5,315
Total revenues	941,293	752,157	1,799,728	1,392,956
Costs and expenses:				
Cost of sales	135,740	104,382	230,832	175,995
Research and development expenses	379,091	337,532	718,581	648,085
Sales, general and administrative expenses	156,502	137,303	303,547	267,111

Restructuring expense (income)	—	62	—	(14)
Total costs and expenses	671,333	579,279	1,252,960	1,091,177
Income from operations	269,960	172,878	546,768	301,779
Interest income	18,076	8,049	33,691	13,838
Interest expense	(14,837)	(18,155)	(29,705)	(35,041)
Other income, net (1)	53,939	53,819	96,549	150,657
Income from operations before provision for (benefit from) income taxes	327,138	216,591	647,303	431,233
Provision for (benefit from) income taxes (2)	59,711	10,341	111,245	(2,318)
Net income	267,427	206,250	536,058	433,551
Loss (income) attributable to noncontrolling interest (3)	—	1,110	—	(15,928)
Net income attributable to Vertex	\$ 267,427	\$ 207,360	\$ 536,058	\$ 417,623

Amounts per share attributable to Vertex common shareholders:

Net income:

Basic	\$ 1.04	\$ 0.82	\$ 2.09	\$ 1.65
Diluted	\$ 1.03	\$ 0.80	\$ 2.06	\$ 1.61

Shares used in per share calculations:

Basic	256,154	254,135	255,941	253,685
Diluted	259,822	258,584	260,015	258,557

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,	
	2019	2018	2019	2018
GAAP net income attributable to Vertex	\$ 267,427	\$ 207,360	\$ 536,058	\$ 417,623
Stock-based compensation expense	89,687	82,436	183,478	160,572
Increase in fair value of strategic investments (1)	(56,527)	(53,918)	(100,078)	(149,376)

(Decrease) increase in fair value of contingent consideration payable to VIE (3)	—	(1,100)	—	22,900
Collaborative and transaction revenues and expenses (4) and other adjustments	53,389	4,278	59,740	5,120
Total non-GAAP adjustments to pre-tax income	86,549	31,696	143,140	39,216
Estimated income taxes related to non-GAAP adjustments to pre-tax income (5)	(26,710)	5,030	(56,102)	(16,829)
Non-GAAP net income attributable to Vertex	\$ 327,266	\$ 244,086	\$ 623,096	\$ 440,010

Amounts per diluted share attributable to Vertex common shareholders:

Net income:

GAAP	\$ 1.03	\$ 0.80	\$ 2.06	\$ 1.61
Non-GAAP	\$ 1.26	\$ 0.94	\$ 2.40	\$ 1.70

Shares used in diluted per share calculations:

GAAP and Non-GAAP	259,822	258,584	260,015	258,557
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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,	
	2019	2018	2019	2018
GAAP total revenues	\$ 941,293	\$ 752,157	\$ 1,799,728	\$ 1,392,956
Collaborative and transaction revenues (4)	(17)	(941)	(158)	(2,860)
Non-GAAP total revenues	\$ 941,276	\$ 751,216	\$ 1,799,570	\$ 1,390,096

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
GAAP cost of sales	\$ 135,740	\$ 104,382	\$ 230,832	\$ 175,995
Stock-based compensation expense	(1,503)	(1,191)	(2,841)	(2,004)
Non-GAAP cost of sales	\$ 134,237	\$ 103,191	\$ 227,991	\$ 173,991

GAAP research and development expenses	\$ 379,091	\$ 337,532	\$ 718,581	\$ 648,085
Stock-based compensation expense	(55,632)	(51,612)	(115,347)	(100,100)
Collaborative and transaction expenses (4) and other adjustments	(52,175)	(5,127)	(58,667)	(7,200)
Non-GAAP research and development expenses	\$ 271,284	\$ 280,793	\$ 544,567	\$ 540,785
GAAP sales, general and administrative expenses	\$ 156,502	\$ 137,303	\$ 303,547	\$ 267,111
Stock-based compensation expense	(32,552)	(29,633)	(65,290)	(58,468)
Collaborative and transaction expenses (4) and other adjustments	(1,231)	(482)	(1,231)	(1,811)
Non-GAAP sales, general and administrative expenses	\$ 122,719	\$ 107,188	\$ 237,026	\$ 206,832
Combined non-GAAP R&D and SG&A expenses	\$ 394,003	\$ 387,981	\$ 781,593	\$ 747,617

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
GAAP other income, net	\$ 53,939	\$ 53,819	\$ 96,549	\$ 150,657
Increase in fair value of strategic investments (1)	(56,527)	(53,918)	(100,078)	(149,376)
Non-GAAP other (expense) income, net	\$ (2,588)	\$ (99)	\$ (3,529)	\$ 1,281
GAAP provision for (benefit from) income taxes	\$ 59,711	\$ 10,341	\$ 111,245	\$ (2,318)
Estimated income taxes related to non-GAAP adjustments to pre-tax income (5)	26,710	(4,614)	56,102	10,840
Non-GAAP provision for income taxes (2)	\$ 86,421	\$ 5,727	\$ 167,347	\$ 8,522

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

June 30, 2019 December 31, 2018

Assets

Cash, cash equivalents and marketable securities \$ 3,951,222 \$ 3,168,242

Accounts receivable, net	464,900	409,688
Inventories	143,017	124,360
Property and equipment, net	731,131	812,005
Goodwill	50,384	50,384
Deferred tax assets	1,425,191	1,499,672
Other assets	267,626	181,547
Total assets	\$ 7,033,471	\$ 6,245,898

Liabilities and Shareholders' Equity

Accounts payable and accrued expenses	\$ 1,146,117	\$ 1,069,886
Finance lease liabilities	588,577	596,639
Other liabilities	228,812	144,170
Shareholders' equity	5,069,965	4,435,203
Total liabilities and shareholders' equity	\$ 7,033,471	\$ 6,245,898

Common shares outstanding	256,671	255,172
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Supplemental Income Tax Information (in thousands, except percentages) (unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Components of provision for (benefit from) income taxes related to:				
Cash taxes paid or accrued for state and foreign income taxes	\$ 5,214	\$ 5,727	\$ 9,992	\$ 8,522
VIE income taxes (5)	—	(416)	—	5,989
Provision for income taxes offset by net operating losses	54,497	5,030	101,253	(16,829)
GAAP provision for (benefit from) income taxes (2)	\$ 59,711	\$ 10,341	\$ 111,245	\$ (2,318)

Cash taxes paid or accrued for state and foreign income taxes	\$ 5,214	\$ 5,727	\$ 9,992	\$ 8,522
Estimated income taxes attributable to Vertex related to non-GAAP adjustments to pre-tax income (5)	26,710	(5,030)	56,102	16,829
Provision for income taxes offset by net operating losses	54,497	5,030	101,253	(16,829)
Non-GAAP provision for income taxes (2)	\$ 86,421	\$ 5,727	\$ 167,347	\$ 8,522

Effective tax rate reconciliation:

GAAP effective tax rate	18 %	5 %	17 %	(1) %
Impact of GAAP to Non-GAAP adjustments	3 %	(3) %	4 %	3 %
Non-GAAP effective tax rate	21 %	2 %	21 %	2 %

Notes and Explanations

1: The company records gains and losses related to changes in the fair value of its strategic investments to "Other income, net."

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 and six months ended June 30, 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 six months ended June 30, 2018, the fair value of the contingent payments payable by Vertex to the VIE increased by \$22.9 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 change in the fair value of the contingent payments payable by Vertex to the VIE decreased by \$1.1 million in the three months ended June 30, 2018. The company deconsolidated the VIE as of December 31, 2018; therefore, there were no comparable amounts during the three and six months ended June 30, 2019.

4: "Collaborative and transaction revenues and expenses" in the three and six months ended June 30, 2019 and 2018 primarily related to collaborative upfront and milestone payments. "Collaborative and transaction revenues and expenses" in the three and six months ended June 30, 2018 also included revenues and expenses attributable to our VIE's operations.

5: In the three and six months ended June 30, 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), (ii) the increase in the fair value of the company's strategic investments and (iii) collaborative upfront payments. In the three and six months ended June 30, 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 diseases. The company has three 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 medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

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 nine 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. 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 and (ii) the development plan and timelines for our product development candidates, including our next-generation triple combination regimen, VX-561, VX-121, CTX001, VX-814, VX-864 and VX-147. 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.

(VRTX-E)

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