



Vertex Reports Third-Quarter 2019 Financial Results

October 30, 2019

- Product revenues of \$950 million, a 21% increase compared to Q3 2018 -

- Continued progression of pipeline of investigational medicines in multiple diseases -

BOSTON--(BUSINESS WIRE)--Oct. 30, 2019-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2019 and reiterated its full-year 2019 total product revenue guidance.

"2019 has been a year of significant progress for Vertex across all parts of our business. With the historic approval of TRIKAFTA, we are now one step closer to providing treatment for up to 90% of all people with CF. We've also had tremendous success bringing our CF medicines to more patients globally with reimbursement agreements recently reached in England, Spain, Australia, and Scotland, and through label expansions to younger patients," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "The company also continues to successfully execute on our strategy of creating transformative medicines for serious diseases through serial innovation. The rapid progress of our pipeline is expected to yield proof-of-concept data in multiple diseases in 2020, which will position Vertex for continued growth in the years ahead."

Third-Quarter 2019 Financial Highlights

	Three Months Ended September 30, %		
	2019	2018	Change
	(in millions, except per share amounts)		
Total product revenues, net	\$ 950	\$ 783	21%
KALYDECO	\$ 249	\$ 246	
ORKAMBI	\$ 297	\$ 282	
SYMDEKO/SYMKEVI	\$ 404	\$ 255	
GAAP Operating income	\$ 99	\$ 206	(52)%
Non-GAAP Operating income	\$ 403	\$ 295	37%
GAAP Net income	\$ 58	\$ 129	(55)%
Non-GAAP Net income	\$ 322	\$ 282	14%
GAAP Net income per share - diluted	\$ 0.22	\$ 0.50	(56)%
Non-GAAP Net income per share - diluted	\$ 1.23	\$ 1.09	13%

Total product revenues increased 21% compared to the third quarter of 2018, primarily driven by the global uptake of SYMDEKO and SYMKEVI in patients ages 12 and older.

GAAP net income decreased compared to the third quarter of 2018, primarily driven by a \$175 million upfront payment as part of Vertex's recently expanded collaboration with CRISPR Therapeutics, and was partially offset by the strong growth in total product revenues.

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

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

Third-Quarter 2019 Expenses

	Three Months Ended September 30,	
	2019	2018
	(in millions)	
Combined GAAP R&D and SG&A expenses	\$ 716	\$ 468
Combined Non-GAAP R&D and SG&A expenses	\$ 416	\$ 379
GAAP R&D expense	\$ 556	\$ 331
Non-GAAP R&D expense	\$ 290	\$ 274
GAAP SG&A expense	\$ 160	\$ 137
Non-GAAP SG&A expense	\$ 127	\$ 105
GAAP income taxes	\$ 13	\$ 8
Non-GAAP income taxes	\$ 84	\$ 5

Combined GAAP R&D and SG&A expenses increased compared to the third quarter of 2018, primarily due to the \$175 million upfront payment to CRISPR Therapeutics.

Combined Non-GAAP R&D and SG&A expenses increased compared to the third 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 third 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 third 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.

Full-Year 2019 Financial Guidance

Vertex today reiterated its 2019 revenue guidance that was updated on October 21, 2019. A summary of the company's current financial expectations is below:

<u>Current FY 2019</u>	<u>Previous FY 2019</u>
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TOTAL product revenues Unchanged \$ 3.70 to 3.75 billion

Combined GAAP R&D and SG&A expenses \$ 2.35 to 2.45 billion \$ 2.25 to 2.40 billion

Combined Non-GAAP R&D and SG&A expenses Unchanged \$ 1.65 to 1.70 billion

Non-GAAP effective tax rate Unchanged 21% to 22%

Clinical Development

Cystic Fibrosis (CF):

- On October 21, 2019, the company announced that the U.S. Food and Drug Administration (FDA) approved TRIKAFTA (elixacaftor/tezacaftor/ivacaftor and ivacaftor) for the treatment of CF in people ages 12 years and older who have at least one *F508del* mutation. TRIKAFTA is Vertex's fourth breakthrough medicine approved to treat the underlying cause of CF.
- Enrollment is ongoing in a Phase 3 study evaluating the elixacaftor/tezacaftor/ivacaftor combination regimen in children with CF ages 6 to 11 years who have two *F508del* mutations and in children who have one *F508del* mutation and one minimal function mutation.
- Vertex continues to make progress toward gaining approval and reimbursement for its CF medicines globally. Recent highlights include:
 - Reimbursement for ORKAMBI and SYMDEKO for eligible patients in England, Spain, Australia, and Scotland.
 - CHMP positive opinion for KALYDECO in the European Union (EU) for infants as early as 6 months old.
 - A Marketing Authorization Application (MAA) submitted to the European Medicines Agency (EMA) for the elixacaftor/tezacaftor/ivacaftor combination regimen to treat people with CF ages 12 years and older.

Alpha-1 Antitrypsin (AAT) Program:

- **VX-814:** In the fourth quarter of 2019, Vertex plans to initiate a Phase 2 proof-of-concept study of its first oral small molecule corrector, VX-814, for the treatment of alpha-1 antitrypsin (AAT) deficiency. This study is expected to enroll approximately 50 patients with AAT deficiency who have two Z mutations. The study will evaluate multiple doses of VX-814 administered for 28 days compared to placebo. The primary endpoints will be the change in the level of functional AAT protein in the blood as well as safety and tolerability. Pending enrollment, Vertex expects to obtain data from this study in 2020.
- **VX-864:** A Phase 1 study is ongoing in healthy volunteers evaluating VX-864, the company's second investigational small molecule corrector for the treatment of AAT deficiency.

Sickle Cell Disease and Beta Thalassemia:

- Enrollment is ongoing in the Phase 1/2 studies evaluating the novel gene-editing therapy CTX001 for the treatment of severe sickle cell disease and beta thalassemia. Vertex and its partner CRISPR Therapeutics plan to provide the first clinical data from these studies in the fourth quarter of 2019. These data will include measurements of safety and efficacy for patients with beta thalassemia and sickle cell disease treated with CTX001.

APOL1-Mediated Kidney Diseases:

- A Phase 1 study in healthy volunteers evaluating VX-147 is expected to be complete in the fourth quarter of 2019. VX-147 is the company's first investigational oral small molecule medicine for the treatment of APOL1-mediated focal segmental glomerulosclerosis (FSGS) and other serious kidney diseases. Pending the results of the Phase 1 study, Vertex plans to initiate a Phase 2 proof-of-concept study in 2020 to evaluate the ability of VX-147 to reduce protein levels in the urine. Vertex is also advancing multiple other APOL1 inhibitors through preclinical development.

Pain:

- A Phase 1 study is ongoing in healthy volunteers evaluating the investigational NaV1.8 inhibitor VX-961 for the treatment of pain. VX-961 has been granted Fast Track Designation by the FDA.

Investments in External Innovation

Type 1 Diabetes:

- On October 10, 2019, Vertex completed its previously announced acquisition of Semma Therapeutics, a privately held biotechnology company pioneering the use of stem cell-derived human islets as a potentially curative treatment for type 1 diabetes.

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, (iv) acquisition-related costs and (v) 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
Third-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenues:				
Product revenues, net.....	\$ 949,828	\$ 782,511	\$ 2,747,461	\$ 2,170,152
Collaboration and royalty revenues.....	—	2,024	2,095	7,339
Total revenues.....	949,828	784,535	2,749,556	2,177,491
Costs and expenses:				
Cost of sales.....	131,914	111,255	362,746	287,250
Research and development expenses.....	555,948	330,510	1,274,529	978,595
Sales, general and administrative expenses.....	159,674	137,295	463,221	404,406
Change in fair value of contingent consideration.....	2,959	—	2,959	—

Restructuring income.....	—	(174)	—	(188)
Total costs and expenses.....	850,495	578,886	2,103,455	1,670,063
Income from operations.....	99,333	205,649	646,101	507,428
Interest income.....	17,628	10,543	51,319	24,381
Interest expense.....	(14,548)	(18,686)	(44,253)	(53,727)
Other (expense) income, net (1)	(31,747)	(60,995)	64,802	89,662
Income from operations before provision for income taxes.....	70,666	136,511	717,969	567,744
Provision for income taxes (2).....	13,148	8,055	124,393	5,737
Net income.....	57,518	128,456	593,576	562,007
Loss (income) attributable to noncontrolling interest (3).....	—	290	—	(15,638)
Net income attributable to Vertex.....	\$ 57,518	\$ 128,746	\$ 593,576	\$ 546,369

Amounts per share attributable to Vertex common shareholders:

Net income:

Basic.....	\$ 0.22	\$ 0.51	\$ 2.32	\$ 2.15
Diluted.....	\$ 0.22	\$ 0.50	\$ 2.28	\$ 2.11

Shares used in per share calculations:

Basic.....	256,946	254,905	256,289	254,096
Diluted.....	260,473	259,788	260,182	258,972

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

Three Months Ended September 30,		Nine Months Ended September 30,	
2019	2018	2019	2018

GAAP net income attributable to Vertex	\$ 57,518	\$ 128,746	\$ 593,576	\$ 546,369
Stock-based compensation expense.....	85,420	85,532	268,898	246,104
Decrease (increase) in fair value of strategic investments (1).....	31,216	61,159	(68,862)	(88,217)
Increase in fair value of contingent consideration (3).....	2,959	200	2,959	23,100
Collaborative revenues and expenses (4).....	203,777	1,779	262,286	4,777
Acquisition-related costs (5) and other adjustments.....	11,459	1,398	12,690	3,520
Total non-GAAP adjustments to pre-tax income.....	334,831	150,068	477,971	189,284
Estimated income taxes related to non-GAAP adjustments to pre-tax income (6).....	(70,849)	3,114	(126,951)	(13,715)
Non-GAAP net income attributable to Vertex	\$ 321,500	\$ 281,928	\$ 944,596	\$ 721,938

Amounts per diluted share attributable to Vertex common shareholders:

Net income:

GAAP.....	\$ 0.22	\$ 0.50	\$ 2.28	\$ 2.11
Non-GAAP.....	\$ 1.23	\$ 1.09	\$ 3.63	\$ 2.79

Shares used in diluted per share calculations:

GAAP and Non-GAAP.....	260,473	259,788	260,182	258,972
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Reconciliation of GAAP to Non-GAAP Revenues and Expenses
Third-Quarter Results
(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
GAAP total revenues	\$ 949,828	\$ 784,535	\$ 2,749,556	\$ 2,177,491
Collaborative revenues (4).....	—	(680)	(158)	(3,540)

	\$ 949,828	\$ 783,855	\$ 2,749,398	\$ 2,173,951
	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Non-GAAP total revenues				
GAAP cost of sales	\$ 131,914	\$ 111,255	\$ 362,746	\$ 287,250
Stock-based compensation expense.....	(1,337)	(1,259)	(4,178)	(3,263)
Non-GAAP cost of sales	\$ 130,577	\$ 109,996	\$ 358,568	\$ 283,987
GAAP research and development expenses	\$ 555,948	\$ 330,510	\$ 1,274,529	\$ 978,595
Stock-based compensation expense.....	(52,504)	(52,918)	(167,851)	(153,018)
Collaborative expenses (4).....	(203,777)	(2,619)	(262,444)	(8,079)
Acquisition-related costs (5) and other adjustments.....	(10,122)	(1,388)	(10,122)	(3,128)
Non-GAAP research and development expenses	\$ 289,545	\$ 273,585	\$ 834,112	\$ 814,370
GAAP sales, general and administrative expenses	\$ 159,674	\$ 137,295	\$ 463,221	\$ 404,406
Stock-based compensation expense.....	(31,579)	(31,355)	(96,869)	(89,823)
Collaborative expenses (4).....	—	(289)	—	(1,704)
Acquisition-related costs (5) and other adjustments.....	(1,337)	(184)	(2,568)	(580)
Non-GAAP sales, general and administrative expenses	\$ 126,758	\$ 105,467	\$ 363,784	\$ 312,299
Combined non-GAAP R&D and SG&A expenses	\$ 416,303	\$ 379,052	\$ 1,197,896	\$ 1,126,669

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
GAAP other (expense) income, net	\$ (31,747)	\$ (60,995)	\$ 64,802	\$ 89,662
Decrease (increase) in fair value of strategic investments (1).....	31,216	61,159	(68,862)	(88,217)
Non-GAAP other (expense) income, net	\$ (531)	\$ 164	\$ (4,060)	\$ 1,445
GAAP provision for income taxes	\$ 13,148	\$ 8,055	\$ 124,393	\$ 5,737
Estimated income taxes related to non-GAAP adjustments to pre-tax income (6).....	70,849	(3,193)	126,951	7,647
Non-GAAP provision for income taxes (2)	\$ 83,997	\$ 4,862	\$ 251,344	\$ 13,384

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	September 30, 2019	December 31, 2018
Assets		
Cash, cash equivalents and marketable securities.....	\$ 3,996,331	\$ 3,168,242
Accounts receivable, net.....	443,315	409,688
Inventories.....	162,306	124,360
Property and equipment, net.....	733,509	812,005
Goodwill.....	447,525	50,384
Deferred tax assets.....	1,415,511	1,499,672
Other assets.....	316,056	181,547
Total assets	\$ 7,514,553	\$ 6,245,898

Liabilities and Shareholders' Equity

Accounts payable and accrued expenses.....	\$	1,266,311	\$	1,069,886
Finance lease liabilities.....		578,624		596,638
Contingent consideration.....		175,000		—
Other liabilities.....		241,224		144,171
Shareholders' equity.....		5,253,394		4,435,203
Total liabilities and shareholders' equity.....	\$	7,514,553	\$	6,245,898
Common shares outstanding.....		257,265		255,172

Supplemental Income Tax Information

(in thousands, except percentages)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Components of provision for income taxes related to:				
Cash taxes paid or accrued for state and foreign income taxes.....	\$ 12,961	\$ 4,862	\$ 22,953	\$ 13,384
VIE income taxes (6).....	—	79	—	6,068
Provision for income taxes offset by net operating losses.....	187	3,114	101,440	(13,715)
GAAP provision for income taxes (2).....	\$ 13,148	\$ 8,055	\$ 124,393	\$ 5,737
Cash taxes paid or accrued for state and foreign income taxes.....	\$ 12,961	\$ 4,862	\$ 22,953	\$ 13,384

Estimated income taxes attributable to Vertex related to non-GAAP adjustments to pre-tax income (5).....	70,849	(3,114)	126,951	13,715
Provision for income taxes offset by net operating losses.....	187	3,114	101,440	(13,715)
Non-GAAP provision for income taxes (2).....	\$ 83,997	\$ 4,862	\$ 251,344	\$ 13,384

Effective tax rate reconciliation:

GAAP effective tax rate.....	19 %	6 %	17 %	1 %
Impact of GAAP to Non-GAAP adjustments.....	2 %	(4) %	4 %	1 %
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 nine months ended September 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 nine months ended September 30, 2018, the fair value of the contingent payments payable by Vertex to the VIE increased by \$23.1 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 and nine months ended September 30, 2019.

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

5: "Acquisition-related costs" in the three and nine months ended September 30, 2019 primarily related to costs associated with the company's acquisition of Exonics. There were no comparable amounts during the three and nine months ended September 30, 2018.

6: In the three and nine months ended September 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) increases or decreases in the fair value of the company's strategic investments and (iii) collaborative upfront payments. In the three and nine months ended September 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 four 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 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. 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 and reimbursement for CF medicines globally and (ii) the development plan and timelines for our product development candidates, including our next-generation triple combination regimen, CTX001, VX-814, VX-864, VX-147 and VX-961. 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 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 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.

(VRTX-E)

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