

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) January 30, 2020

Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts

(State or other jurisdiction of incorporation)

000-19319

(Commission File Number)

04-3039129

(I.R.S. Employer Identification No.)

50 Northern Avenue

Boston, Massachusetts 02210

(Address of principal executive offices) (Zip Code)

(617) 341-6100

(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, \$0.01 Par Value Per Share	VRTX	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02. Results of Operations and Financial Condition.

On January 30, 2020, we issued a press release in which we reported our consolidated financial results for the three and twelve months ended December 31, 2019. A copy of that press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The information set forth in Exhibit 99.1 shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

<u>Exhibit</u>	<u>Description of Document</u>
99.1	Press Release, dated January 30, 2020

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

VERTEX PHARMACEUTICALS INCORPORATED

(Registrant)

Date: January 30, 2020

/s/ Michael Parini

Michael Parini

Executive Vice President, Chief Legal and Administrative Officer

Vertex Reports Full-Year and Fourth-Quarter 2019 Financial Results

- Full-year 2019 total GAAP product revenues of \$4.16 billion -

- Full-year 2019 total non-GAAP product revenues of \$4.00 billion, a 32% increase compared to the full-year 2018 -

- Company provides full-year 2020 total product revenue guidance of \$5.1 billion to \$5.3 billion -

BOSTON -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the full year and fourth quarter ended December 31, 2019 and provided full-year 2020 financial guidance.

"In 2019, our differentiated strategy of investing in serial scientific innovation to create transformative medicines delivered remarkable performance. This was marked by the early approval of TRIKAFTA, the rapid progression of our pipeline in additional diseases and continued financial growth as we continued to treat more patients with our medicines worldwide," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "This success is the culmination of many years of hard work to build a team, a pipeline and a company that we believe will discover, develop and commercialize transformative medicines for years to come."

Fourth-Quarter and Full-Year 2019 Financial Highlights

	Three Months Ended December 31,		%	Twelve Months Ended December 31,		%
	2019	2018		2019	2018	
	(in millions, except per share amounts)			(in millions, except per share amounts)		
GAAP Product revenues, net	\$ 1,413	\$ 868	63%	\$ 4,161	\$ 3,038	37%
Non-GAAP Product revenues, net[^]	\$ 1,257	\$ 868	45%	\$ 4,005	\$ 3,038	32%
GAAP Operating income	\$ 551	\$ 128	332%	\$ 1,198	\$ 635	89%
Non-GAAP Operating income	\$ 593	\$ 348	70%	\$ 1,786	\$ 1,112	61%
GAAP Net income ^{^^}	\$ 583	\$ 1,551		\$ 1,177	\$ 2,097	
Non-GAAP Net income	\$ 444	\$ 337	32%	\$ 1,389	\$ 1,059	31%
GAAP Net income per share - diluted ^{^^}	\$ 2.23	\$ 5.97		\$ 4.51	\$ 8.09	
Non-GAAP Net income per share - diluted	\$ 1.70	\$ 1.30	31%	\$ 5.33	\$ 4.08	31%

[^]Non-GAAP product revenues exclude \$155.8 million related to ORKAMBI in the fourth quarter and full year ended December 31, 2019. Please refer to Note 4 for further information.

^{^^} GAAP Net income and GAAP Net income per share - diluted included a benefit from income taxes of approximately \$1.5 billion in the fourth quarter and full year ended December 31, 2018 due to the release of Vertex's valuation allowance on the majority of its net operating losses and other deferred tax assets.

Full-Year 2019 Results

	Twelve Months Ended December 31,	
	2019	2018
	(in millions)	
Non-GAAP Product revenues, net	\$ 4,005	\$ 3,038
KALYDECO	\$ 991	\$ 1,008
ORKAMBI	\$ 1,176	\$ 1,262
SYMDEKO/SYMKEVI	\$ 1,418	\$ 769
TRIKAFTA	\$ 420	\$ —

Total GAAP and Non-GAAP product revenues increased 37% and 32%, respectively, compared to 2018, primarily driven by the global uptake of SYMDEKO and SYMKEVI in patients ages 12 and older, label expansions for the company's CF medicines globally, and the early approval and launch of TRIKAFTA in the U.S.

GAAP net income decreased compared to 2018, largely driven by the release of Vertex's tax valuation allowance in 2018.

Non-GAAP net income increased compared to 2018, driven by the strong growth in total product revenues.

Cash, cash equivalents and marketable securities as of December 31, 2019 were \$3.8 billion, an increase of approximately \$600 million compared to \$3.2 billion as of December 31, 2018.

Full-Year 2019 Expenses

	Twelve Months Ended December 31,	
	2019	2018
	(in millions)	
Combined GAAP R&D and SG&A expenses	\$ 2,413	\$ 1,974
Combined Non-GAAP R&D and SG&A expenses	\$ 1,694	\$ 1,527
GAAP R&D expense	\$ 1,755	\$ 1,416
Non-GAAP R&D expense	\$ 1,171	\$ 1,089
GAAP SG&A expense	\$ 658	\$ 558
Non-GAAP SG&A expense	\$ 523	\$ 437
GAAP income taxes	\$ 218	\$ (1,487)
Non-GAAP income taxes	\$ 397	\$ 16

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to 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. Combined GAAP R&D and SG&A expenses also increased compared to 2018 due to Vertex's business development activities.

GAAP and Non-GAAP income taxes increased significantly compared to 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 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.

Fourth-Quarter 2019 Results

	Three Months Ended December 31,	
	2019	2018
	(in millions)	
Non-GAAP Product revenues, net	\$ 1,257	\$ 868
KALYDECO	\$ 236	\$ 259
ORKAMBI	\$ 270	\$ 315
SYMDEKO/SYMKEVI	\$ 332	\$ 294
TRIKAFTA	\$ 420	\$ —

Total GAAP and Non-GAAP product revenues increased 63% and 45%, respectively, compared to the fourth quarter of 2018, primarily driven by the early approval and launch of TRIKAFTA in the U.S. and the global uptake of SYMDEKO and SYMKEVI in patients ages 12 and older.

GAAP net income decreased compared to the fourth quarter of 2018, largely driven by the release of Vertex's tax valuation allowance in the fourth quarter of 2018.

Non-GAAP net income increased compared to the fourth quarter of 2018, driven by the strong growth in total product revenues.

Fourth-Quarter 2019 Expenses

	Three Months Ended December 31,	
	2019	2018
	(in millions)	
Combined GAAP R&D and SG&A expenses	\$ 675	\$ 591
Combined Non-GAAP R&D and SG&A expenses	\$ 496	\$ 400
GAAP R&D expense	\$ 480	\$ 438
Non-GAAP R&D expense	\$ 337	\$ 275
GAAP SG&A expense	\$ 195	\$ 153
Non-GAAP SG&A expense	\$ 159	\$ 125
GAAP income taxes	\$ 94	\$ (1,493)
Non-GAAP income taxes	\$ 145	\$ 3

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to the fourth 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 fourth quarter of 2018 due to Vertex's release of its tax valuation allowance in the fourth quarter of 2018. Refer to "Full-Year 2019 Expenses" for further information. 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 provided its full-year 2020 financial guidance. A summary of the company's current financial expectations is below:

	FY 2020
TOTAL product revenues	\$5.1 to \$5.3 billion
Combined GAAP R&D and SG&A expenses	\$2.4 to \$2.55 billion
Combined Non-GAAP R&D and SG&A expenses	\$1.95 to \$2.0 billion
Non-GAAP effective tax rate	21% to 22%

"Entering 2020, Vertex has never been stronger," said Reshma Kewalramani, M.D., Executive Vice President and Chief Medical Officer. "We are well-positioned for both near- and long-term growth based on treating more people with our CF medicines and from other future medicines in diseases aligned with our strategy, including alpha-1 antitrypsin deficiency, APOL1-mediated kidney diseases, pain and severe hemoglobinopathies. Going forward, our financial strength will enable us to continue to significantly invest

in internal R&D and external innovation, which will provide access to new technologies, programs and expertise that will lead to further growth in the years ahead."

Clinical Development

Cystic Fibrosis (CF):

- On October 21, 2019, the company announced that the U.S. Food and Drug Administration (FDA) approved TRIKAFTA (elexacaftor/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.
- On October 31, 2019, the company announced that the European Medicines Agency (EMA) has validated the marketing authorization application (MAA) for the elexacaftor, tezacaftor and ivacaftor triple combination regimen.
- Enrollment is ongoing in a Phase 3 study evaluating the elexacaftor, tezacaftor and ivacaftor triple 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. Pending the results of the Phase 3 study, the company plans to submit a supplemental New Drug Application (sNDA) to the FDA in 2020 followed by additional submissions to other global regulatory agencies.

Alpha-1 Antitrypsin (AAT) Program:

- **VX-814:** Enrollment is ongoing in the Phase 2 proof-of-concept study evaluating VX-814, the company's first oral small molecule corrector for the treatment of alpha-1 antitrypsin (AAT) deficiency. 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:

- In November 2019, Vertex and its partner CRISPR Therapeutics announced positive, interim data from the first two patients with severe hemoglobinopathies - one patient with transfusion-dependent beta thalassemia and one patient with severe sickle cell disease - treated with the investigational CRISPR/Cas9 gene-editing therapy CTX001 in the ongoing Phase 1/2 clinical trials. Enrollment is ongoing in both trials and the companies expect to provide additional data for this program in 2020.

APOL1-Mediated Kidney Diseases:

- Vertex continues to advance a portfolio of molecules for the treatment of APOL1-mediated focal segmental glomerulosclerosis (FSGS) and other serious kidney diseases. In the fourth quarter of 2019, Vertex completed a Phase 1 study of its first investigational oral small molecule VX-147 in healthy volunteers. Vertex plans to initiate a Phase 2 proof-of-concept study in 2020 to evaluate the reduction in protein levels with VX-147.

Pain:

- The company announced today that it has discontinued Phase 1 development of VX-961. The company continues to advance a portfolio of NaV1.8 inhibitors into the clinic. As part of this strategy, Vertex plans to advance an additional molecule into clinical development in the first half of 2020.

Type 1 Diabetes:

- Vertex plans to advance its cell therapy program for the treatment of type 1 diabetes into clinical development in late 2020 or early 2021.

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) an adjustment to revenues and related cost of sales to reflect the conclusion of the early access program for ORKAMBI in France, (iii) revenues and expenses related to business development transactions including collaboration agreements, asset acquisitions and consolidated variable interest entities, (iv) gains or losses related to the fair value of the company's strategic investments, (v) acquisition-related costs and (vi) 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
Fourth-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Revenues:				
Product revenues, net	\$ 1,413,265	\$ 868,173	\$ 4,160,726	\$ 3,038,325
Collaboration and royalty revenues	—	1,933	2,095	9,272
Total revenues	1,413,265	870,106	4,162,821	3,047,597
Costs and expenses:				
Cost of sales	185,012	122,289	547,758	409,539
Research and development expenses	480,011	437,881	1,754,540	1,416,476
Sales, general and administrative expenses	195,277	153,210	658,498	557,616
Change in fair value of contingent consideration	1,500	—	4,459	—
Restructuring expense (income)	—	4	—	(184)
Intangible asset impairment charge	—	29,000	—	29,000
Total costs and expenses	861,800	742,384	2,965,255	2,412,447
Income from operations	551,465	127,722	1,197,566	635,150
Interest income	12,359	13,971	63,678	38,352
Interest expense	(14,249)	(18,744)	(58,502)	(72,471)
Other income (expense), net (1)	127,375	(90,452)	192,177	(790)
Income from operations before provision for (benefit from) income taxes	676,950	32,497	1,394,919	600,241
Provision for (benefit from) income taxes (2)	93,716	(1,492,599)	218,109	(1,486,862)
Net income	583,234	1,525,096	1,176,810	2,087,103
Loss attributable to noncontrolling interest (3)	—	25,431	—	9,793
Net income attributable to Vertex	\$ 583,234	\$ 1,550,527	\$ 1,176,810	\$ 2,096,896
Amounts per share attributable to Vertex common shareholders:				
Net income:				
Basic	\$ 2.26	\$ 6.08	\$ 4.58	\$ 8.24
Diluted	\$ 2.23	\$ 5.97	\$ 4.51	\$ 8.09
Shares used in per share calculations:				
Basic	258,003	254,868	256,728	254,292
Diluted	262,108	259,812	260,673	259,185

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

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP net income attributable to Vertex	\$ 583,234	\$ 1,550,527	\$ 1,176,810	\$ 2,096,896
Stock-based compensation expense	91,591	78,943	360,489	325,047
ORKAMBI adjustment (4)	(140,854)	—	(140,854)	—
(Increase) decrease in fair value of strategic investments (1)	(128,734)	85,659	(197,596)	(2,558)
Increase (decrease) in fair value of contingent consideration (3)	1,500	(5,370)	4,459	17,730
Collaborative revenues and expenses (5)	56,057	111,940	318,343	116,717
Acquisition-related costs (6) and other adjustments	33,181	1,043	45,871	4,563
Total non-GAAP adjustments to pre-tax income	(87,259)	272,215	390,712	461,499
Estimated income taxes related to non-GAAP adjustments to pre-tax income (7)	(51,627)	13,715	(178,578)	—
Benefit from income taxes due to release of valuation allowance (2)	—	(1,499,588)	—	(1,499,588)
Non-GAAP net income attributable to Vertex	\$ 444,348	\$ 336,869	\$ 1,388,944	\$ 1,058,807

Amounts per diluted share attributable to Vertex common shareholders:

Net income:				
GAAP	\$ 2.23	\$ 5.97	\$ 4.51	\$ 8.09
Non-GAAP	\$ 1.70	\$ 1.30	\$ 5.33	\$ 4.08

Shares used in diluted per share calculations:

GAAP and Non-GAAP	262,108	259,812	260,673	259,185
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Reconciliation of GAAP to Non-GAAP Revenues and Expenses

Fourth-Quarter Results

(in thousands)

(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP total revenues	\$ 1,413,265	\$ 870,106	\$ 4,162,821	\$ 3,047,597
ORKAMBI adjustment (4)	(155,773)	—	(155,773)	—
Collaborative revenues (5)	—	(663)	(158)	(4,203)
Non-GAAP total revenues	\$ 1,257,492	\$ 869,443	\$ 4,006,890	\$ 3,043,394
	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP cost of sales	\$ 185,012	\$ 122,289	\$ 547,758	\$ 409,539
ORKAMBI adjustment (4)	(14,919)	—	(14,919)	—
Stock-based compensation expense	(1,397)	(1,280)	(5,575)	(4,543)
Non-GAAP cost of sales	\$ 168,696	\$ 121,009	\$ 527,264	\$ 404,996
GAAP research and development expenses	\$ 480,011	\$ 437,881	\$ 1,754,540	\$ 1,416,476
Stock-based compensation expense	(56,707)	(50,094)	(224,558)	(203,112)
Collaborative expenses (5)	(56,057)	(111,925)	(318,501)	(120,004)
Acquisition-related costs (6) and other adjustments	(30,461)	(877)	(40,583)	(4,005)
Non-GAAP research and development expenses	\$ 336,786	\$ 274,985	\$ 1,170,898	\$ 1,089,355
GAAP sales, general and administrative expenses	\$ 195,277	\$ 153,210	\$ 658,498	\$ 557,616
Stock-based compensation expense	(33,487)	(27,569)	(130,356)	(117,392)
Collaborative expenses (5)	—	(438)	—	(2,142)
Acquisition-related costs (6) and other adjustments	(2,720)	(162)	(5,288)	(742)
Non-GAAP sales, general and administrative expenses	\$ 159,070	\$ 125,041	\$ 522,854	\$ 437,340
Combined non-GAAP R&D and SG&A expenses	\$ 495,856	\$ 400,026	\$ 1,693,752	\$ 1,526,695
	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
GAAP other income (expense), net	\$ 127,375	\$ (90,452)	\$ 192,177	\$ (790)
Collaborative expenses (4)	—	1,077	—	1,077
(Increase) decrease in fair value of strategic investments (1)	(128,734)	85,659	(197,596)	(2,558)
Non-GAAP other expense, net	\$ (1,359)	\$ (3,716)	\$ (5,419)	\$ (2,271)
GAAP provision for (benefit from) income taxes	\$ 93,716	\$ (1,492,599)	\$ 218,109	\$ (1,486,862)
Estimated income taxes related to non-GAAP adjustments to pre-tax income (7)	51,627	(3,979)	178,578	3,668
Benefit from income taxes due to release of valuation allowance (2)	—	1,499,588	—	1,499,588
Non-GAAP provision for income taxes (2)	\$ 145,343	\$ 3,010	\$ 396,687	\$ 16,394

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	December 31, 2019	December 31, 2018
Assets		
Cash, cash equivalents and marketable securities	\$ 3,808,294	\$ 3,168,242
Accounts receivable, net	633,518	409,688
Inventories	167,502	124,360
Property and equipment, net	745,080	812,005
Goodwill and intangible assets	1,402,158	50,384
Deferred tax assets	1,190,815	1,499,672
Other assets	371,098	181,547
Total assets	\$ 8,318,465	\$ 6,245,898
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	\$ 1,204,522	\$ 1,069,886
Finance lease liabilities	577,371	596,638
Contingent consideration	176,500	—
Other liabilities	274,828	144,171
Shareholders' equity	6,085,244	4,435,203
Total liabilities and shareholders' equity	\$ 8,318,465	\$ 6,245,898
Common shares outstanding	258,993	255,172

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018
Components of provision for income taxes related to:				
Cash taxes paid or accrued for state and foreign income taxes	\$ 9,185	\$ 3,010	\$ 32,138	\$ 16,394
Benefit from income taxes due to release of valuation allowance (2)	—	(1,499,588)	—	(1,499,588)
VIE income taxes (7)	—	(9,736)	—	(3,668)
Provision for income taxes offset by net operating losses	84,531	13,715	185,971	—
GAAP provision for (benefit from) income taxes (2)	\$ 93,716	\$ (1,492,599)	\$ 218,109	\$ (1,486,862)
Cash taxes paid or accrued for state and foreign income taxes	\$ 9,185	\$ 3,010	\$ 32,138	\$ 16,394
Estimated income taxes related to non-GAAP adjustments to pre-tax income (7)	51,627	(3,979)	178,578	3,668
Provision for income taxes offset by net operating losses	84,531	3,979	185,971	(3,668)
Non-GAAP provision for income taxes (2)	\$ 145,343	\$ 3,010	\$ 396,687	\$ 16,394
Effective tax rate reconciliation:				
GAAP effective tax rate	14%	**	16%	**
Impact of GAAP to Non-GAAP adjustments	11%	**	6%	**
Non-GAAP effective tax rate	25%	1%	22%	2%

** Not meaningful

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, 2019, the company's federal net operating losses and credits that were available to offset future pre-tax income were approximately \$3.5 billion.
- 3:** During the three and twelve months ended December 31, 2019, the increase in the fair value of the contingent consideration relates to payments potentially payable to Exonics' former equity holders. During the three and twelve months ended December 31, 2018, the company consolidated the financial statements of a variable interest entity, or VIE, resulting in changes in the fair value of contingent consideration.
- 4:** "ORKAMBI adjustment" in the three and twelve months ended December 31, 2019 includes an adjustment to net product revenues and cost of sales related to the conclusion of the early access program for ORKAMBI in France in the fourth quarter of 2019. The company had previously recognized a portion of net product revenues related to ORKAMBI distributed through the early access program in France. As a result, the company recognized an adjustment to increase net product revenues and cost of sales, which related to prior period shipments of ORKAMBI distributed through the early access program in France. The company has excluded the adjustment to net product revenues and cost of sales from its Non-GAAP measures for the three and twelve months ended December 31, 2019. Beginning in 2020, the company does not expect to adjust any revenues from its GAAP net product revenues.
- 5:** "Collaborative revenues and expenses" in the three and twelve months ended December 31, 2019 and 2018 primarily related to collaborative upfront and milestone payments. "Collaborative revenues and expenses" in the three and twelve months ended December 31, 2018 also included revenues and expenses attributable to our VIE's operations.
- 6:** "Acquisition-related costs" in the three and twelve months ended December 31, 2019 primarily related to costs associated with the company's acquisitions of Semma and Exonics. There were no comparable amounts during the three and twelve months ended December 31, 2018.
- 7:** In the three and twelve months ended December 31, 2019, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" primarily related to (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation), (ii) increases or decreases in the fair value of the company's strategic investments and (iii) collaborative upfront payments. In the three and twelve months ended December 31, 2018, "Estimated income taxes related to non-GAAP adjustments to pre-tax income" were related to a provision for income taxes attributable to the company's VIE and excess tax benefits related to stock-based compensation.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious 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 disease. 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. Leiden's statements in this press release, 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 (ii) the development plan and timelines for our drug candidates. 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 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 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

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