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) October 29, 2020

Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts	**000-19319**	**04-3039129**
(State or other jurisdiction of incorporation)	*(Commission File Number)*	*(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 October 29, 2020, we issued a press release in which we reported our consolidated financial results for the three and nine months ended September 30, 2020. 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

Exhibit	Description of Document
99.1	Press Release Dated October 29, 2020.
104	Cover Page Interactive Data File — the cover page XBRL tags are embedded within the Inline XBRL document.

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: October 29, 2020

/s/ Michael Parini
Michael Parini
Executive Vice President, Chief Administrative, Legal and Business Development Officer

Vertex Reports Third-Quarter 2020 Financial Results

-Product revenues of $1.54 billion, a 62% increase compared to Q3 2019-

-Company raises revenue guidance; now expects 2020 product revenues of $6.0 to $6.2 billion-

BOSTON -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2020 and revised upward its full-year 2020 financial guidance for product revenues.

"This has been another very strong quarter for Vertex with execution across our CF business and continued earnings and revenue growth," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "We are pleased with our progress toward treating all people with cystic fibrosis highlighted by several recent milestones: the early approval and encouraging start of the launch of KAFTRIO in the EU, positive TRIKAFTA data in children ages 6-11, and continued expansion of our medicines' labels as with our recent approval of KALYDECO for infants as young as 4 months of age."

"As we extend our leadership in CF, we are also advancing a broad pipeline of innovative therapies," continued Dr. Kewalramani. "Our R&D strategy contemplates the high-risk nature of drug development and therefore includes a portfolio approach to each of our disease areas of interest. In AATD, while disappointed by the VX-814 outcome, we look forward to the VX-864 Phase 2 proof-of-concept data in the first half of 2021. Our pipeline spans multiple diseases, and multiple important clinical readouts are expected from now through the end of 2021, each of which we expect will hold transformative potential for patients and further growth for Vertex."

Third-Quarter 2020 Financial Highlights

	Three Months Ended September 30,		%
	2020	2019	Change
	(in millions, except per share amounts)		
Product revenues, net	$ 1,536	$ 950	62%
TRIKAFTA/KAFTRIO	$ 960	$ —	
SYMDEKO/SYMKEVI	$ 156	$ 404	
ORKAMBI	$ 226	$ 297	
KALYDECO	$ 194	$ 249	
GAAP Operating income	$ 672	$ 99	577%
Non-GAAP Operating income	$ 854	$ 403	112%
GAAP Net income	$ 667	$ 58	1,060%
Non-GAAP Net income	$ 697	$ 322	117%
GAAP Net income per share - diluted	$ 2.53	$ 0.22	1,050%
Non-GAAP Net income per share - diluted	$ 2.64	$ 1.23	115%

Product revenues increased 62% compared to the third 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 several significant reimbursement agreements.

GAAP and non-GAAP net income increased compared to the third quarter of 2019, largely driven by strong growth in total product revenues.

Cash, cash equivalents and marketable securities as of September 30, 2020 were $6.2 billion, an increase of approximately $2.3 billion compared to $3.8 billion as of December 31, 2019 driven by strong revenue and profitability.

Third-Quarter 2020 Expenses

	Three Months Ended September 30,	
	2020	2019
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 678	$ 716
Combined Non-GAAP R&D and SG&A expenses	$ 497	$ 416
GAAP R&D expense	$ 493	$ 556
Non-GAAP R&D expense	$ 350	$ 290
GAAP SG&A expense	$ 185	$ 160
Non-GAAP SG&A expense	$ 147	$ 127
GAAP income taxes	$ 78	$ 13
Non-GAAP income taxes	$ 155	$ 84
GAAP effective tax rate	11 %	19 %
Non-GAAP effective tax rate	18 %	21 %

Combined GAAP R&D and SG&A expenses decreased compared to the third quarter of 2019 due to a decrease in collaboration payments.

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

GAAP and Non-GAAP income taxes increased compared to the third 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 product revenues. The company also adjusted its expectation for combined GAAP R&D and SG&A expenses and non-GAAP effective tax rate. Vertex's guidance is summarized below:

	Current FY 2020	Previous FY 2020
TOTAL product revenues	$6.0 to 6.2 billion	$5.7 to 5.9 billion
Combined GAAP R&D and SG&A expenses (1)	$2.5 to 2.6 billion	$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 (1)	20% to 21%	21% to 22%

Key Business Highlights:

Cystic Fibrosis (CF) R&D pipeline:

Vertex expects to increase the number of CF patients eligible to take our medicines and thereby continue to grow our CF business. Important progress has been made in supporting the extension of the eligible patient population and expansion to additional geographies and age groups.

TRIKAFTA/KAFTRIO (elexacaftor, tezacaftor and ivacaftor)

- The European Commission granted marketing authorization for KAFTRIO to treat people with CF ages 12 years and older with one *F508del* mutation and one minimal function mutation or two *F508del* mutations.
- The European Medicines Agency (EMA) validated a Type II Variation Marketing Authorization Application (MAA) for KAFTRIO that will support future indication expansion of the EU label to people with CF who have one copy of the *F508del* mutation.
- Vertex reported positive Phase 3 data for the elexacaftor/tezacaftor/ivacaftor triple combination in children with CF ages 6-11 who have either two copies of the *F508del* mutation or one copy of the *F508del* mutation and one minimal function mutation. Vertex expects to file a supplemental New Drug Application (sNDA) with the U.S. Food and Drug Administration (FDA) in the fourth quarter of 2020.
- The FDA accepted three sNDAs for TRIKAFTA, SYMDEKO and KALYDECO. These regulatory submissions are intended to expand the labels of these drugs to include additional people with CF who have rare CFTR mutations.
- Vertex is initiating a Phase 3 study for the elexacaftor/tezacaftor/ivacaftor triple combination in children with CF ages 2-5 who have either two copies of the *F508del* mutation or one copy of the *F508del* mutation and one minimal function mutation.

SYMDEKO/SYMKEVI (tezacaftor and ivacaftor)

- Vertex announced that the EMA's Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for the label extension of SYMKEVI for the treatment of children with CF ages 6-11 with two *F508del* mutations or one *F508del* mutation and certain residual function mutations.

KALYDECO (ivacaftor)

- The FDA approved KALYDECO for use in infants with CF ages four months to less than six months old who have at least one mutation that is responsive to KALYDECO.
- Vertex announced that the EMA's CHMP adopted a positive opinion for the label extension of KALYDECO for the treatment of infants with CF ages 4 months to less than 6 months who have at least one mutation that is responsive to KALYDECO.

Genetic therapies

- Vertex and Moderna established a new collaboration aimed at the discovery and development of lipid nanoparticles (LNPs) and mRNAs that can deliver gene-editing therapies to cells in the lung for the treatment of CF.

R&D pipeline outside of CF:

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 are evaluating the use of an ex-vivo CRISPR gene-edited therapy for the treatment of transfusion-dependent beta-thalassemia (TDT) and sickle cell disease (SCD). This approach aims to edit a person’s hematopoietic stem cells to produce fetal hemoglobin in red blood cells, which has the potential to reduce or eliminate symptoms associated with disease.
- Vertex and CRISPR Therapeutics previously announced that, as of June, seven patients had been dosed across its two Phase 1/2 studies of the investigational CRISPR/Cas9 gene-editing therapy CTX001 and presented data at the European Hematology Association Congress from two TDT patients and one SCD patient. Additional patients have been enrolled and dosed in both TDT and

SCD studies and the company expects to report clinical data from more patients treated with CTX001 in addition to data from patients with longer follow-up in the fourth quarter.

- The EMA granted Priority Medicines (PRIME) designation to CTX001 for the treatment of severe SCD. CTX001 has also been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug, and Rare Pediatric Disease designations from the FDA and Orphan Drug Designation from the European Commission for both TDT and SCD.

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 is ongoing in a Phase 2 proof-of-concept study for the Z-AAT corrector, VX-864. Data from this study is expected in the first half of 2021.
- In October, Vertex discontinued development of VX-814 based on the safety and pharmacokinetic profile of VX-814 observed to date in the Phase 2 clinical study.

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 ongoing 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. Data from this study is expected in 2021.

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 has completed the required enabling nonclinical studies and manufacturing work to support the submission of an Investigational New Drug (IND) application to the U.S. FDA for the islet cells alone program in the fourth quarter of 2020.

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) increases or decreases in the fair value of contingent consideration, (v) acquisition-related costs and (vi) 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 should not be viewed as a substitute for the company’s GAAP results and are provided as a complement to results provided in accordance with GAAP. 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 that the company believes is helpful to an understanding of its ongoing business. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, 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’s calculation of non-GAAP financial measures likely differs from the calculations used by other companies. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

The company provides guidance regarding combined R&D and SG&A expenses and effective tax rate on a non-GAAP basis. The guidance regarding combined GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development activities. The company does not provide forward-looking reconciliations of these measures to the most directly comparable GAAP financial measures because it is unable, without unreasonable efforts, to calculate these GAAP measures with reasonable certainty.

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,	
	2020	**2019**	**2020**	**2019**
Revenues:				
Product revenues, net	$ 1,536,271	$ 949,828	$ 4,575,863	$ 2,747,461
Collaboration and royalty revenues	2,000	—	2,000	2,095
Total revenues	1,538,271	949,828	4,577,863	2,749,556
Costs and expenses:				
Cost of sales	186,182	131,914	533,199	362,746
Research and development expenses	493,497	555,948	1,362,953	1,274,529
Sales, general and administrative expenses	184,551	159,674	558,613	463,221
Change in fair value of contingent consideration	1,800	2,959	12,600	2,959
Total costs and expenses	866,030	850,495	2,467,365	2,103,455
Income from operations	672,241	99,333	2,110,498	646,101
Interest income	3,100	17,628	19,919	51,319
Interest expense	(13,856)	(14,548)	(41,863)	(44,253)
Other income (expense), net (2)	84,386	(31,747)	139,621	64,802
Income before provision for income taxes	745,871	70,666	2,228,175	717,969
Provision for income taxes	78,437	13,148	120,718	124,393
Net income	$ 667,434	$ 57,518	$ 2,107,457	$ 593,576
Net income per common share:				
Basic	$ 2.56	$ 0.22	$ 8.10	$ 2.32
Diluted	$ 2.53	$ 0.22	$ 7.98	$ 2.28
Shares used in per share calculations:				
Basic	260,392	256,946	260,313	256,289
Diluted	264,079	260,473	264,031	260,182

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,	
	2020	**2019**	**2020**	**2019**
GAAP net income	$ 667,434	$ 57,518	$ 2,107,457	$ 593,576
Stock-based compensation expense	99,539	85,420	332,434	268,898
(Increase) decrease in fair value of strategic investments (2)	(75,750)	31,216	(140,866)	(68,862)
Increase in fair value of contingent consideration (3)	1,800	2,959	12,600	2,959
Collaborative revenues and expenses (4)	78,050	203,777	141,300	262,286
Acquisition-related costs (5)	2,523	11,459	7,862	12,690
Total non-GAAP adjustments to pre-tax income	106,162	334,831	353,330	477,971
Tax adjustments (6)	(76,250)	(70,849)	(402,183)	(126,951)
Non-GAAP net income	$ 697,346	$ 321,500	$ 2,058,604	$ 944,596
Net income per diluted common share:				
GAAP	$ 2.53	$ 0.22	$ 7.98	$ 2.28
Non-GAAP	$ 2.64	$ 1.23	$ 7.80	$ 3.63
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	264,079	260,473	264,031	260,182

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,	
	2020	2019	2020	2019
GAAP total revenues	$ 1,538,271	$ 949,828	$ 4,577,863	$ 2,749,556
Collaborative revenues	(2,000)	—	(2,000)	(158)
Non-GAAP total revenues	$ 1,536,271	$ 949,828	$ 4,575,863	$ 2,749,398

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
GAAP cost of sales	$ 186,182	$ 131,914	$ 533,199	$ 362,746
Stock-based compensation expense	(1,250)	(1,337)	(3,998)	(4,178)
Non-GAAP cost of sales	$ 184,932	$ 130,577	$ 529,201	$ 358,568
GAAP research and development expenses	$ 493,497	$ 555,948	$ 1,362,953	$ 1,274,529
Stock-based compensation expense	(60,770)	(52,504)	(203,732)	(167,851)
Collaborative expenses (4)	(80,050)	(203,777)	(143,300)	(262,444)
Acquisition-related costs (5)	(2,523)	(10,122)	(7,409)	(10,122)
Non-GAAP research and development expenses	$ 350,154	$ 289,545	$ 1,008,512	$ 834,112
GAAP sales, general and administrative expenses	$ 184,551	$ 159,674	$ 558,613	$ 463,221
Stock-based compensation expense	(37,519)	(31,579)	(124,704)	(96,869)
Acquisition-related costs (5)	—	(1,337)	(453)	(2,568)
Non-GAAP sales, general and administrative expenses	$ 147,032	$ 126,758	$ 433,456	$ 363,784
Combined non-GAAP R&D and SG&A expenses	$ 497,186	$ 416,303	$ 1,441,968	$ 1,197,896

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
GAAP other income (expense), net	$ 84,386	$ (31,747)	$ 139,621	$ 64,802
(Increase) decrease in fair value of strategic investments (2)	(75,750)	31,216	(140,866)	(68,862)
Non-GAAP other income (expense), net	$ 8,636	$ (531)	$ (1,245)	$ (4,060)
GAAP provision for income taxes	$ 78,437	$ 13,148	$ 120,718	$ 124,393
Tax adjustments (6)	76,250	70,849	402,183	126,951
Non-GAAP provision for income taxes (7)	$ 154,687	$ 83,997	$ 522,901	$ 251,344

Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

	September 30, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 6,151,058	$ 3,808,294
Accounts receivable, net	791,917	633,518
Inventories	245,460	167,502
Property and equipment, net	920,913	745,080
Goodwill and intangible assets	1,402,158	1,402,158
Deferred tax assets	1,147,816	1,190,815
Other assets	642,311	371,098
Total assets	$ 11,301,633	$ 8,318,465
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 1,811,485	$ 1,204,522
Finance lease liabilities	587,772	577,371
Contingent consideration	189,100	176,500
Other liabilities	579,803	274,828
Shareholders' equity	8,133,473	6,085,244
Total liabilities and shareholders' equity	$ 11,301,633	$ 8,318,465
Common shares outstanding	260,174	258,993

Supplemental Income Tax Information

(in thousands, except percentages)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Components of provision for income taxes related to:				
Cash paid or accrued for income taxes	$ (2,249)	$ 12,961	$ 45,347	$ 22,953
Benefits from income taxes due to discrete tax items (5)	(60,244)	—	(297,599)	—
Provision for income taxes offset by net operating losses and credits (6)	140,930	187	372,970	101,440
GAAP provision for income taxes (7)	$ 78,437	$ 13,148	$ 120,718	$ 124,393
Cash paid or accrued for income taxes	$ (2,249)	$ 12,961	$ 45,347	$ 22,953
Adjustments to pre-tax income	16,006	70,849	104,584	126,951
Provision for income taxes offset by net operating losses and credits (6)	140,930	187	372,970	101,440
Non-GAAP provision for income taxes (7)	$ 154,687	$ 83,997	$ 522,901	$ 251,344
Effective tax rate reconciliation:				
GAAP effective tax rate	11 %	19 %	5 %	17 %
Impact of GAAP to Non-GAAP adjustments	7 %	2 %	15 %	4 %
Non-GAAP effective tax rate	18 %	21 %	20 %	21 %

Notes and Explanations

1: The company's increased combined GAAP R&D and SG&A expense guidance reflects the effect of a new collaboration agreement in the third quarter of 2020. The company's adjusted non-GAAP effective tax rate guidance includes a change in the utilization of certain tax assets.

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

3: During the three and nine months ended September 30, 2020 and 2019, the increase in the fair value of contingent consideration relates to potential payments to Exonics' former equity holders.

4: "Collaborative revenues and expenses" in the three and nine months ended September 30, 2020 and 2019 primarily related to collaborative upfront and milestone payments.

5: "Acquisition-related costs" in the three and nine months ended September 30, 2020 and 2019 related to costs associated with the company's acquisitions of Semma and Exonics.

6: In the three and nine months ended September 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 nine months ended September 30, 2020, "Tax adjustments" also included non-recurring discrete benefits to the company's provision for income taxes of approximately $60.2 million and $297.6 million, respectively, that the company excluded from its Non-GAAP measures.

7: The company records a provision for income taxes on its pre-tax income using an effective tax rate approximating statutory rates. Since the company released its valuation allowance on the majority of its net operating losses and other deferred tax assets as of December 31, 2018, its tax provision has included 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. 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. The company expects to utilize its remaining federal net operating losses in 2020. As a result, a larger portion of the company’s tax provision will represent a cash expense in future periods, subject to continued utilization of certain tax credits.

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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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 future label expansions, (iii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including collaborations with third parties, and (iv) expectations for the collaborations with Moderna. 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 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 in a timely manner, or at all, 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.

(VRTX-E)

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