**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) April 29, 2021

# 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 April 29, 2021, we issued a press release in which we reported our consolidated financial results for the three months ended March 31, 2021. 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 April 29, 2021. |
| 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: April 29, 2021

/s/ Joy Liu
Joy Liu
Senior Vice President, General Counsel

# Vertex Reports First-Quarter 2021 Financial Results

*-Product revenues of $1.72 billion, a 14% increase compared to Q1 2020-*

*- Company advancing clinical programs in six additional diseases beyond cystic fibrosis-*

*-Multiple Phase 2 proof-of-concept study results expected in 2021 -*

**BOSTON** -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2021 and reiterated full-year 2021 guidance for product revenues.

"In CF, our goal is that all eligible patients have access to and can benefit from CFTR modulators. In the first quarter, we continued to make significant progress towards this goal, and in so doing again delivered strong revenue and earnings growth," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "Beyond CF, we have also seen continued significant progress across our broad pipeline, including advancement of VX-548 to Phase 2 in acute pain, initiation of the Phase 1/2 clinical trial with VX-880 in type 1 diabetes and completion of enrollment and dosing in our Phase 2 proof-of-concept study with the AAT corrector, VX-864. The recent amendment of our agreement with CRISPR Therapeutics for the CTX001 program further enhances our leadership position in cell and genetic therapies and we look forward to completing enrollment of our ongoing trials for CTX001 in sickle cell disease and beta thalassemia this year and bringing this first-in-class treatment to patients with these devastating diseases as soon as possible."

**First-Quarter 2021 Financial Highlights**

| | Three Months Ended March 31, | | % |
|---|---|---|---|
| | 2021 | 2020 | Change |
| | (in millions, except per share amounts) | | |
| **Product revenues, net** | $ 1,723 | $ 1,515 | 14% |
| **TRIKAFTA/KAFTRIO** | $ 1,193 | $ 895 | |
| **SYMDEKO/SYMKEVI** | $ 125 | $ 173 | |
| **ORKAMBI** | $ 219 | $ 234 | |
| **KALYDECO** | $ 186 | $ 213 | |
| **GAAP Operating income** | $ 888 | $ 720 | 23% |
| **Non-GAAP Operating income** | $ 1,003 | $ 877 | 14% |
| **GAAP Net income** | $ 653 | $ 603 | 8% |
| **Non-GAAP Net income** | $ 781 | $ 674 | 16% |
| **GAAP Net income per share - diluted** | $ 2.49 | $ 2.29 | 9% |
| **Non-GAAP Net income per share - diluted** | $ 2.98 | $ 2.56 | 16% |

**Product revenues** increased 14% compared to the first quarter of 2020, primarily driven by the uptake of KAFTRIO in Europe and continued performance of TRIKAFTA in the U.S. Net product revenues in the first quarter of 2021 increased 6% to $1.25 billion in the U.S. and increased 43% to $470 million outside the U.S., compared to the prior year.

**GAAP and non-GAAP net income** increased compared to the first quarter of 2020, largely driven by strong growth in total product revenues.

**Cash, cash equivalents and marketable securities** as of March 31, 2021 were $6.9 billion, an increase of $265 million compared to $6.7 billion as of December 31, 2020 primarily driven by strong revenue and profitability offset by the repurchase of our common stock authorized under our stock repurchase plan.

**First-Quarter 2021 Expenses**

| | Three Months Ended March 31, | |
|---|---|---|
| | 2021 | 2020 |
| | (in millions) | |
| **Combined GAAP R&D and SG&A expenses** | $ 648 | $ 631 |
| **Combined Non-GAAP R&D and SG&A expenses** | $ 530 | $ 477 |
| **GAAP R&D expenses** | $ 456 | $ 449 |
| **Non-GAAP R&D expenses** | $ 379 | $ 337 |
| **GAAP SG&A expenses** | $ 192 | $ 182 |
| **Non-GAAP SG&A expenses** | $ 151 | $ 140 |
| **GAAP income taxes (1)** | $ 168 | $ 55 |
| **Non-GAAP income taxes** | $ 207 | $ 184 |
| **GAAP effective tax rate** | 20% | 8% |
| **Non-GAAP effective tax rate (1)** | 21% | 21% |

**Combined GAAP and Non-GAAP R&D and SG&A expenses** increased compared to the first quarter of 2020, primarily due to the expansion of Vertex's pipeline in CF and other disease areas and incremental investment to support the global launches of Vertex's medicines.

**GAAP income taxes and the GAAP effective tax rate** increased compared to the first quarter of 2020 due a non-recurring discrete tax benefit recognized in the first quarter of 2020 and Vertex's increased operating income.

**Non-GAAP income taxes** increased compared to the first quarter of 2020 primarily due to Vertex's increased operating income.

**Full-Year 2021 Financial Guidance**

Vertex today reiterated its full-year 2021 financial guidance, except for its expectations for combined GAAP R&D and SG&A expenses, which increased by $900 million as a result of Vertex's amended collaboration with CRISPR announced in April. Vertex's guidance is summarized below:

| | Current FY 2021 | Previous FY 2021 |
|---|---|---|
| **Product revenues** | Unchanged | $6.7 to 6.9 billion |
| **Combined GAAP R&D and SG&A expenses (2)** | $3.8 to 3.95 billion | $2.9 to 3.05 billion |
| **Combined Non-GAAP R&D and SG&A expenses (2)** | Unchanged | $2.25 to 2.3 billion |
| **Non-GAAP effective tax rate** | Unchanged | 21% to 22% |

**Key Business Highlights**

***Cystic Fibrosis (CF)***

Vertex anticipates that achieving new approvals and entering into additional reimbursement agreements for our current CFTR modulators will increase the number of CF patients treated with our medicines and continue to grow our CF business in the years ahead.

***Key progress in 2021 includes:***

- New approval received for **TRIKAFTA** *(elexacaftor/tezacaftor/ivacaftor and ivacaftor)* in Australia for people with CF ages 12 years and older who have at least one *F508del* mutation.
- Post-marketing application filed with the European Medicines Agency (EMA) for the expanded indication of **KAFTRIO** *(elexacaftor/tezacaftor/ivacaftor and ivacaftor)* to include children with CF ages 6 through 11 years.
- **TRIKAFTA/KAFTRIO** is now approved and reimbursed or accessible in 12 countries outside the U.S., including Denmark, Germany, Ireland, Israel, Switzerland and the countries within the UK.

***R&D pipeline***

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

***Beta Thalassemia and Sickle Cell Disease***

- In April, Vertex and CRISPR Therapeutics amended their collaboration for the CTX001 programs in beta thalassemia and sickle cell disease. Under the terms of the revised agreement, Vertex will lead worldwide development, manufacturing and commercialization of CTX001. The revised agreement provides Vertex with 60% and CRISPR Therapeutics with 40% of program economics. At closing, CRISPR Therapeutics will receive a $900 million upfront payment with the potential for an additional $200 million milestone payment upon CTX001 regulatory approval.
- The CTX001 program employs a non-viral *ex vivo* CRISPR gene-editing 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 the diseases.
- Enrollment and dosing are ongoing in the clinical studies for CTX001 and more than 30 patients have now been dosed to date. Completion of enrollment in both studies is expected in 2021.

- In April, the European Medicines Agency (EMA) granted Priority Medicines Designation (PRIME) to CTX001 for TDT. The program has previously been granted Regenerative Medicine Advanced Therapy (RMAT), Fast Track, Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) for both TDT and SCD. CTX001 has also been granted PRIME designation for SCD and Orphan Drug Designation from EMA 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 systemic levels of functional AAT. Misfolded Z-AAT protein is the root cause of AAT deficiency and the Vertex small molecule corrector program targets both the liver and lung manifestations of the disease.
- Patients enrolled in the Phase 2 proof-of-concept study for the Z-AAT corrector, VX-864, have completed the 28-day dosing period. The study includes a 28-day safety follow-up period which is ongoing, and results are expected in the second quarter of 2021.

***APOL1-mediated Kidney Diseases***

- Vertex is evaluating the potential of inhibitors of APOL1 function in people with APOL1-mediated 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. Results from this study are expected in the second half of 2021.

***Type 1 Diabetes (T1D)***

- Vertex is evaluating a cell therapy designed to replace insulin-producing islet cells in people with T1D. Vertex is pursuing two programs for the transplant of these fully-differentiated 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.
- In March, the U.S. FDA granted Fast Track Designation and Vertex initiated a Phase 1/2 clinical trial for VX-880, the islet cells alone program, in people with T1D.

***Pain***

- Vertex is evaluating selective small molecule inhibitors of NaV1.8, a genetically validated, novel target for the treatment of pain, with the goal of preventing pain signals traveling from the sensory

nerves to the central nervous system. Vertex has previously demonstrated clinical proof-of-concept with a small molecule investigational treatment targeting NaV1.8, VX-150, in multiple pain indications including acute pain, neuropathic pain and musculoskeletal pain.

- VX-548, a selective NaV1.8 inhibitor, demonstrated favorable safety, tolerability and pharmacokinetic profiles in Phase 1 studies. In these studies, the molecule exhibited a favorable profile at doses considerably lower than those required with our previous NaV1.8 inhibitors.
- VX-548 is expected to advance into Phase 2 proof-of-concept studies for acute pain in the second half of 2021.

***Investments in External Innovation***

- In April, we entered into a research collaboration with Obsidian Therapeutics, Inc., or Obsidian, aimed at the discovery of novel therapies that regulate gene-editing for the treatment of serious diseases. This collaboration enables us to leverage Obsidian's cytoDRiVE® platform technology to discover gene-editing medicines whose therapeutic activity can be precisely controlled using small molecules.

**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 collaborative milestones and upfront payments, (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 guidance regarding its GAAP effective tax rate because it is unable to forecast with reasonable certainty the impact of excess tax benefits related to stock-based compensation and the possibility of certain discrete items, which could be material.

**Vertex Pharmaceuticals Incorporated**
**First-Quarter Results**
**Consolidated Statements of Operations**
(in thousands, except per share amounts)
(unaudited)

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| Revenues: | | |
| Product revenues, net | $ 1,723,305 | $ 1,515,107 |
| Other revenues | 1,000 | — |
| Total revenues | 1,724,305 | 1,515,107 |
| Costs and expenses: | | |
| Cost of sales | 192,329 | 162,497 |
| Research and development expenses | 455,973 | 448,528 |
| Sales, general and administrative expenses | 192,077 | 182,258 |
| Change in fair value of contingent consideration | (3,900) | 1,600 |
| Total costs and expenses | 836,479 | 794,883 |
| Income from operations | 887,826 | 720,224 |
| Interest income | 1,465 | 12,576 |
| Interest expense | (15,678) | (14,136) |
| Other expense, net (3) | (52,653) | (61,130) |
| Income before provision for income taxes | 820,960 | 657,534 |
| Provision for income taxes | 167,822 | 54,781 |
| Net income | $ 653,138 | $ 602,753 |
| | | |
| Net income per common share: | | |
| Basic | $ 2.52 | $ 2.32 |
| Diluted | $ 2.49 | $ 2.29 |
| Shares used in per share calculations: | | |
| Basic | 259,369 | 259,815 |
| Diluted | 261,916 | 263,515 |

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

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| **GAAP net income** | $ 653,138 | $ 602,753 |
| Stock-based compensation expense | 115,174 | 115,706 |
| Decrease in fair value of strategic investments (3) | 52,295 | 44,870 |
| (Decrease) increase in fair value of contingent consideration (4) | (3,900) | 1,600 |
| Collaborative revenues and expenses (5) | 650 | 36,250 |
| Acquisition-related costs (6) | 2,820 | 2,883 |
| Total non-GAAP adjustments to pre-tax income | 167,039 | 201,309 |
| Tax adjustments (1) | (38,961) | (129,608) |
| **Non-GAAP net income** | $ 781,216 | $ 674,454 |
| | | |
| Net income per diluted common share: | | |
| GAAP | $ 2.49 | $ 2.29 |
| Non-GAAP | $ 2.98 | $ 2.56 |
| Shares used in diluted per share calculations: | | |
| GAAP and Non-GAAP | 261,916 | 263,515 |

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| **GAAP operating income** | $ 887,826 | $ 720,224 |
| Stock-based compensation expense | 115,174 | 115,706 |
| (Decrease) increase in fair value of contingent consideration (4) | (3,900) | 1,600 |
| Collaborative revenues and expenses (5) | 650 | 36,250 |
| Acquisition-related costs (6) | 2,820 | 2,883 |
| **Non-GAAP operating income** | $ 1,002,570 | $ 876,663 |

**Reconciliation of GAAP to Non-GAAP Revenues and Expenses**
**First-Quarter Results**
(in thousands)
(unaudited)

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| **GAAP total revenues** | $ 1,724,305 | $ 1,515,107 |
| Collaborative revenues | (1,000) | — |
| **Non-GAAP total revenues** | $ 1,723,305 | $ 1,515,107 |

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| **GAAP cost of sales** | $ 192,329 | $ 162,497 |
| Stock-based compensation expense | (1,431) | (1,361) |
| **Non-GAAP cost of sales** | $ 190,898 | $ 161,136 |
| | | |
| **GAAP research and development expenses** | $ 455,973 | $ 448,528 |
| Stock-based compensation expense | (72,802) | (72,687) |
| Collaborative expenses (5) | (1,650) | (36,250) |
| Acquisition-related costs (6) | (2,820) | (2,678) |
| **Non-GAAP research and development expenses** | $ 378,701 | $ 336,913 |
| | | |
| **GAAP sales, general and administrative expenses** | $ 192,077 | $ 182,258 |
| Stock-based compensation expense | (40,941) | (41,658) |
| Acquisition-related costs (6) | — | (205) |
| **Non-GAAP sales, general and administrative expenses** | $ 151,136 | $ 140,395 |
| | | |
| **Combined non-GAAP R&D and SG&A expenses** | $ 529,837 | $ 477,308 |

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| **GAAP other expense, net** | $ (52,653) | $ (61,130) |
| Decrease in fair value of strategic investments (3) | 52,295 | 44,870 |
| **Non-GAAP other expense, net** | $ (358) | $ (16,260) |
| | | |
| **GAAP provision for income taxes** | $ 167,822 | $ 54,781 |
| Tax adjustments (1) | 38,961 | 129,608 |
| **Non-GAAP provision for income taxes (7)** | $ 206,783 | $ 184,389 |
| | | |
| **GAAP effective tax rate** | 20 % | 8 % |
| **Non-GAAP effective tax rate (7)** | 21 % | 21 % |

## Condensed Consolidated Balance Sheets

(in thousands)

(unaudited)

| | March 31, 2021 | December 31, 2020 |
|---|---|---|
| **Assets** | | |
| Cash, cash equivalents and marketable securities | $ 6,923,968 | $ 6,658,897 |
| Accounts receivable, net | 977,551 | 885,352 |
| Inventories | 298,863 | 280,777 |
| Property and equipment, net | 986,123 | 958,534 |
| Goodwill and intangible assets | 1,402,158 | 1,402,158 |
| Deferred tax assets | 815,890 | 882,779 |
| Other assets | 710,506 | 683,311 |
| **Total assets** | $ 12,115,059 | $ 11,751,808 |
| | | |
| **Liabilities and Shareholders' Equity** | | |
| Accounts payable and accrued expenses | $ 1,659,876 | $ 1,560,110 |
| Finance lease liabilities | 572,856 | 581,476 |
| Contingent consideration | 185,700 | 189,600 |
| Other liabilities | 716,373 | 733,807 |
| Shareholders' equity | 8,980,254 | 8,686,815 |
| **Total liabilities and shareholders' equity** | $ 12,115,059 | $ 11,751,808 |
| | | |
| Common shares outstanding | 258,829 | 259,890 |

**Notes and Explanations**

**1:** In the three months ended March 31, 2021 and 2020, "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) decreases in the fair value of the company's strategic investments and (iii) collaborative milestone payments. In the three months ended March 31, 2020, "Tax adjustments" also included a non-recurring discrete benefit to the company's provision for income taxes of $50.4 million, relating to the write-off of a long-term intercompany receivable, that the company excluded from its Non-GAAP measures.

**2:** The company's increased combined GAAP R&D and SG&A expenses guidance reflects the expected effect upon closing of the company's contemplated transaction with CRISPR, which was announced in April 2021. The difference between the company's full-year 2021 combined GAAP R&D and SG&A expenses and combined non-GAAP R&D and SG&A expenses guidance relates primarily to $1.12 billion to $1.17 billion of R&D expenses related to existing and contemplated collaboration agreements and $430 million to $455 million of stock-based compensation expense. The guidance regarding combined GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development activities other than the company's contemplated transaction with CRISPR.

**3:** "Other expense, net" includes net losses related to changes in the fair value of the company's strategic investments and from sales of certain investments.

**4:** During the three months ended March 31, 2021 and 2020, the change in the fair value of contingent consideration relates to potential payments to Exonics Therapeutics' former equity holders.

**5:** "Collaborative revenues and expenses" in the three months ended March 31, 2021 and 2020 primarily related to collaborative milestone payments.

**6:** "Acquisition-related costs" in the three months ended March 31, 2021 and 2020 related to costs associated with the company's acquisition of Exonics Therapeutics in 2019.

**7:** The company released its valuation allowance on the majority of its net operating losses and other deferred tax assets as of December 31, 2018. As of December 31, 2020, the company had utilized substantially all of its remaining federal net operating losses. As a result, a larger portion of the company's tax provision will represent a cash tax payable beginning in 2021, subject to continued utilization of certain tax credits.

***Note:*** *Amounts may not foot due to rounding.*

**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 and operations, the section captioned "Full-Year 2021 Financial Guidance" and statements regarding (i) anticipated regulatory filings, data submissions, and approvals, (ii) anticipated future label expansions, (iii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including clinical site activations, patient enrollment, and data availability, (iv) expectations for the collaborations with CRISPR, including expectations regarding completion of enrollment in the CTX001 clinical studies and anticipated benefits of the collaborations, (v) expectations for uptake of and expanded access to the company’s medicines, including additional reimbursement agreements, (vi) expectations for an increase in the number of CF patients treated with our medicines, (vii) expectations for VX-548, including our plans to advance VX-548 into Phase 2 proof-of-concept studies in the second half of 2021, (viii) expectations for the transactions contemplated by the amended collaboration with CRISPR, including satisfaction of closing conditions, antitrust clearance, anticipated upfront and milestone payments to CRISPR, and anticipated future activities of the parties, (ix) expected effect on our expenses upon the closing of the transaction contemplated by the amended collaboration with CRISPR, and (x) anticipated investment in internal and external innovation. 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 2021 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 the closing of the transaction contemplated by the amended collaboration with CRISPR may not occur in a timely manner, or at all, that data from preclinical testing or early clinical trials, especially if based on a limited number of patients, may not be indicative of

final results, that the company may not realize the anticipated benefits from our collaborations with third parties, 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 the heading “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 and on the SEC’s website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

**Conference Call and Webcast**

The company will host a conference call and webcast today at 5:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

(VRTX-E)

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