# Vertex Reports Second-Quarter 2021 Financial Results

July 29, 2021

*- Product revenues of $1.79 billion, an 18% increase compared to Q2 2020 -*

*- Company raises full-year 2021 guidance for product revenues to $7.2 to $7.4B -*

*- Phase 3 study of next-in-class triple combination for CF to begin in the second half of 2021; multiple additional clinical milestones across the pipeline expected in the next 6 to 9 months -*

BOSTON--(BUSINESS WIRE)--Jul. 29, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2021 and raised full-year 2021 guidance for product revenues to $7.2 to $7.4B.

"In the second quarter of 2021, we saw continued, significant growth and strong business performance in our cystic fibrosis franchise. We have now secured reimbursement agreements for the triple combination in more than 15 countries outside the U.S. and started expansion into younger age groups with the U.S. approval in patients 6 to 11 years of age last month. With these advancements, we are poised to reach more patients in 2021 than previously forecasted and are therefore raising our 2021 revenue guidance. Looking forward, we continue to see significant growth ahead in CF, with more than 30,000 CF patients who may benefit from the triple combination but who are not yet treated," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex.

"We also made important progress with our pipeline programs in the first half of this year. Our pipeline programs are advancing quickly, with five programs in mid- or late-stage clinical trials. We continue to see impressive clinical results with CTX001, our most advanced program outside of CF, in which we have now dosed more than 45 patients. Our pre-commercial efforts for this program are underway, as we prepare to serve patients with sickle cell disease and beta thalassemia and address the significant market opportunity. We look forward to multiple R&D milestones and data readouts in the coming 6 to 9 months," said Dr. Kewalramani.

**Second-Quarter 2021 Financial Highlights**

| | Three Months Ended June 30, | | % |
|---|---|---|---|
| | **2021** | **2020** | **Change** |
| | (in millions, except per share amounts) | | |
| **Product revenues, net** | $ 1,793 | $ 1,524 | 18% |
| **TRIKAFTA/KAFTRIO** | $ 1,256 | $ 918 | |
| **SYMDEKO/SYMKEVI** | $ 134 | $ 172 | |
| **ORKAMBI** | $ 221 | $ 232 | |
| **KALYDECO** | $ 183 | $ 203 | |
| **GAAP operating (loss) income** | $ (38) | $ 718 | N/A |
| **Non-GAAP operating income** | $ 1,029 | $ 874 | 18% |
| **GAAP net income** | $ 67 | $ 837 | (92)% |
| **Non-GAAP net income** | $ 811 | $ 687 | 18% |

| | | | | |
|---|---|---|---|---|
| **GAAP net income per share - diluted** | $ 0.26 | $ 3.18 | (92)% |
| **Non-GAAP net income per share - diluted** | $ 3.11 | $ 2.61 | 19% |

**Product revenues** increased 18% compared to the second quarter of 2020, primarily driven by the uptake of KAFTRIO in Europe and continued strong performance of TRIKAFTA in the U.S. Net product revenues in the second quarter of 2021 increased 4% to $1.26 billion in the U.S. and increased 71% to $536 million outside the U.S., compared to the second quarter of 2020.

**GAAP net income** decreased compared to the second quarter of 2020, primarily due to a $900 million payment in connection with the amendment of Vertex's collaboration with CRISPR Therapeutics that was recorded as a GAAP R&D expense in the second quarter of 2021.

**Non-GAAP net income** increased compared to the second quarter of 2020, largely driven by strong growth in product revenues.

**Cash, cash equivalents and marketable securities** were $6.71 billion, an increase of $49 million compared to $6.66 billion as of December 31, 2020, primarily driven by strong operating cash flow from Vertex's revenue growth and profitability, and offset by the $900 million payment to CRISPR and repurchases of our common stock authorized under our 2020 share repurchase program.

**Second-Quarter 2021 Expenses**

| | Three Months Ended June 30, | |
|---|---|---|
| | **2021** | **2020** |
| | (in millions) | |
| **Combined GAAP R&D and SG&A expenses** | $ 1,602 | $ 613 |
| **Combined Non-GAAP R&D and SG&A expenses** | $ 537 | $ 467 |
| **GAAP R&D expenses (1)** | $ 1,407 | $ 421 |
| **Non-GAAP R&D expenses** | $ 383 | $ 321 |
| **GAAP SG&A expenses** | $ 195 | $ 192 |
| **Non-GAAP SG&A expenses** | $ 154 | $ 146 |
| **GAAP income taxes (2)** | $ (111) | $ (13) |
| **Non-GAAP income taxes** | $ 201 | $ 184 |
| **GAAP effective tax rate (2)** | 251% | (2)% |
| **Non-GAAP effective tax rate** | 20% | 21% |

**Combined GAAP R&D and SG&A expenses** increased compared to the second quarter of 2020, primarily due to the $900 million payment to CRISPR in the second quarter of 2021.

**Combined Non-GAAP R&D and SG&A expenses** increased compared to the second 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** reflected an increased benefit compared to the second quarter of 2020, primarily due to the income tax impact of the $900 million payment to CRISPR partially offset by a decrease in discrete tax benefits. Please refer to Note 2 for further details.

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

**Full-Year 2021 Financial Guidance**

Vertex today increased its full-year 2021 product revenue guidance based on strong year-to-date performance and the expected impact of recent reimbursement agreements. Vertex's guidance is summarized below:

| | Current FY 2021 | Previous FY 2021 |
|---|---|---|
| **Product revenues** | $7.2 to 7.4 billion | $6.7 to 6.9 billion |
| **Combined GAAP R&D and SG&A expenses (3)** | Unchanged | $3.8 to 3.95 billion |
| **Combined Non-GAAP R&D and SG&A expenses (3)** | Unchanged | $2.25 to 2.3 billion |
| **Non-GAAP effective tax rate** | Unchanged | 21% to 22% |

**Key Business Highlights**

***Cystic Fibrosis (CF) Marketed Products***

Vertex anticipates that the number of CF patients treated with our medicines will continue to grow as we enter into additional reimbursement agreements, achieve new approvals for the treatment of younger patients, and expand treatment options for the approximately 10 percent of patients who do not benefit from CFTR modulators, all of which will lead to continued growth of our CF business in the years ahead.

In June, the U.S. FDA approved expansion of the indication of TRIKAFTA *(elexacaftor/tezacaftor/ivacaftor and ivacaftor)* to include children with CF ages 6 through 11 years. Health Canada granted Marketing Authorization for TRIKAFTA for people with CF ages 12 years and older who have at least one *F508del* mutation. TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in more than 15 countries outside the U.S., including Italy and France.

***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:

***Cystic Fibrosis***

- Vertex recently announced plans to initiate Phase 3 studies of the next-in-class, once-daily triple combination of VX-121, tezacaftor and VX-561 in the second half of 2021. Clinical and preclinical data suggest that this triple combination has the potential to provide enhanced benefit for people with CF who have the *F508del* mutation on at least one allele.
- The Phase 3 program will consist of two 48-week trials, which will evaluate the safety and efficacy of the new combination relative to TRIKAFTA in a total of 800 patients. Both studies will measure the regulatory-enabling endpoint of absolute change in ppFEV1, a measure of lung function, that will be analyzed for non-inferiority to TRIKAFTA. Both studies will also assess absolute change from baseline in ppFEV1 and sweat chloride for superiority to TRIKAFTA.

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

- Based on the compelling data generated with CTX001, in April, Vertex and CRISPR Therapeutics announced an amendment to their collaboration for CTX001. In connection with the completion of the transaction in June, Vertex made a $900 million upfront payment to CRISPR.
- Data from 22 patients with at least three months of follow-up after CTX001 infusion were presented at EHA in June and continued to build the profile of a one-time functional cure for patients with transfusion-dependent beta thalassemia (TDT) and severe sickle cell disease (SCD), showing consistent and durable benefit with longer term data from a larger population of patients.
- Enrollment and dosing are ongoing in the clinical studies for CTX001 and more than 45 patients have been dosed across the program to date. Vertex anticipates achieving target enrollment in both studies in the third quarter of 2021, with

regulatory filings possible in the next 18 to 24 months.

***APOL1-mediated Kidney Diseases***

- Vertex is evaluating the potential of inhibitors of APOL1 function to treat people with APOL1-mediated kidney diseases.
- Enrollment is ongoing in a Phase 2 proof-of-concept study designed to evaluate the reduction in proteinuria in people with APOL1-mediated focal segmental glomerulosclerosis (FSGS) following treatment with VX-147.
- Data from this study are expected in the second half of 2021.

***Pain***

- NaV1.8 is a genetically and pharmacologically validated novel target for the treatment of pain, and Vertex has previously demonstrated clinical proof-of-concept with a small molecule investigational treatment targeting NaV1.8 in multiple pain indications including acute pain, neuropathic pain and musculoskeletal pain. Vertex's approach is to selectively inhibit NaV1.8 using small molecules with the objective of creating a new class of medicines that have the potential to provide superior relief of acute pain without the limitations of opioids, including their addictive potential. VX-548 is the most recent molecule to enter clinical development from Vertex's portfolio of NaV1.8 inhibitors.
- Vertex announced in July that the VX-548 Phase 2 acute pain program has been initiated. The proof-of-concept trial for acute pain following bunionectomy surgery is open for enrollment, and the VX-548 trial following abdominoplasty surgery will commence in the coming weeks.
- Data from the bunionectomy trial are expected by early 2022.

***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 stem cell-derived, fully differentiated, insulin-producing islet cells 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.
- A Phase 1/2 clinical trial for VX-880, the islet cells alone program, is ongoing in people with T1D. The first patient in this study has been dosed, and initial data from this study are expected in 2022.

***Alpha-1 Antitrypsin (AAT) Deficiency***

- Vertex continues to evaluate small molecule correctors of zAAT protein to target the underlying cause of AATD, and thereby address both lung and liver manifestations of the disease.
- Vertex plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022.

**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, including the $900 million upfront payment to CRISPR Therapeutics, (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**
**Second-Quarter Results**
**Consolidated Statements of Operations**
(in thousands, except per share amounts)
(unaudited)

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Revenues: | | | | |
| Product revenues, net | $ 1,793,370 | $ 1,524,485 | $ 3,516,675 | $ 3,039,592 |
| Other revenues | — | — | 1,000 | — |
| Total revenues | 1,793,370 | 1,524,485 | 3,517,675 | 3,039,592 |
| Costs and expenses: | | | | |
| Cost of sales | 227,972 | 184,520 | 420,301 | 347,017 |
| Research and development expenses (1) | 1,407,090 | 420,928 | 1,863,063 | 869,456 |
| Selling, general and administrative expenses | 194,669 | 191,804 | 386,746 | 374,062 |
| Change in fair value of contingent consideration | 1,600 | 9,200 | (2,300) | 10,800 |
| Total costs and expenses | 1,831,331 | 806,452 | 2,667,810 | 1,601,335 |
| (Loss) income from operations | (37,961) | 718,033 | 849,865 | 1,438,257 |
| Interest income | 1,133 | 4,243 | 2,598 | 16,819 |
| Interest expense | (15,478) | (13,871) | (31,156) | (28,007) |
| Other income (expense), net | 8,051 | 116,365 | (44,602) | 55,235 |
| (Loss) income before (benefit from) provision for income taxes | (44,255) | 824,770 | 776,705 | 1,482,304 |
| (Benefit from) provision for income taxes | (111,179) | (12,500) | 56,643 | 42,281 |
| Net income | $ 66,924 | $ 837,270 | $ 720,062 | $ 1,440,023 |
| | | | | |
| Net income per common share: | | | | |
| Basic | $ 0.26 | $ 3.22 | $ 2.78 | $ 5.54 |
| Diluted | $ 0.26 | $ 3.18 | $ 2.75 | $ 5.46 |
| Shares used in per share calculations: | | | | |
| Basic | 258,988 | 259,637 | 259,179 | 260,013 |
| Diluted | 261,020 | 263,403 | 261,468 | 263,746 |

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | **2021** | **2020** | **2021** | **2020** |
| **GAAP net income** | $ 66,924 | $ 837,270 | $ 720,062 | $ 1,440,023 |
| Stock-based compensation expense | 104,622 | 117,189 | 219,796 | 232,895 |
| (Increase) decrease in fair value of strategic investments (4) | (10,609) | (109,986) | 41,686 | (65,116) |
| Increase (decrease) in fair value of contingent consideration (5) | 1,600 | 9,200 | (2,300) | 10,800 |
| Collaborative revenues and expenses (6) | 958,400 | 27,000 | 959,050 | 63,250 |
| Acquisition-related costs (7) | 2,820 | 2,456 | 5,640 | 5,339 |
| Total non-GAAP adjustments to pre-tax income | 1,056,833 | 45,859 | 1,223,872 | 247,168 |
| Tax adjustments (2) | (312,484) | (196,325) | (351,445) | (325,933) |
| **Non-GAAP net income** | $ 811,273 | $ 686,804 | $ 1,592,489 | $ 1,361,258 |
| | | | | |
| Net income per diluted common share: | | | | |
| GAAP | $ 0.26 | $ 3.18 | $ 2.75 | $ 5.46 |
| Non-GAAP | $ 3.11 | $ 2.61 | $ 6.09 | $ 5.16 |
| Shares used in diluted per share calculations: | | | | |
| GAAP and Non-GAAP | 261,020 | 263,403 | 261,468 | 263,746 |

| | Three Months Ended June 30, | Six Months Ended June 30, |
|---|---|---|

| | 2021 | 2020 | 2021 | 2020 |
|---|---|---|---|---|
| **GAAP operating (loss) income** | $ (37,961) | $ 718,033 | $ 849,865 | $ 1,438,257 |
| Stock-based compensation expense | 104,622 | 117,189 | 219,796 | 232,895 |
| Increase (decrease) in fair value of contingent consideration (5) | 1,600 | 9,200 | (2,300) | 10,800 |
| Collaborative revenues and expenses (6) | 958,400 | 27,000 | 959,050 | 63,250 |
| Acquisition-related costs (7) | 2,820 | 2,456 | 5,640 | 5,339 |
| **Non-GAAP operating income** | $ 1,029,481 | $ 873,878 | $ 2,032,051 | $ 1,750,541 |

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP total revenues** | $ 1,793,370 | $ 1,524,485 | $ 3,517,675 | $ 3,039,592 |
| Collaborative revenues | — | — | (1,000) | — |
| **Non-GAAP total revenues** | $ 1,793,370 | $ 1,524,485 | $ 3,516,675 | $ 3,039,592 |

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP cost of sales** | $ 227,972 | $ 184,520 | $ 420,301 | $ 347,017 |
| Stock-based compensation expense | (1,540) | (1,387) | (2,971) | (2,748) |
| **Non-GAAP cost of sales** | $ 226,432 | $ 183,133 | $ 417,330 | $ 344,269 |
| | | | | |
| **GAAP research and development expenses** | $ 1,407,090 | $ 420,928 | $ 1,863,063 | $ 869,456 |
| Stock-based compensation expense | (62,615) | (70,275) | (135,417) | (142,962) |
| Collaborative expenses (6) | (958,400) | (27,000) | (960,050) | (63,250) |

| | | | | |
|---|---|---|---|---|
| Acquisition-related costs (7) | (2,820) | (2,208) | (5,640) | (4,886) |
| **Non-GAAP research and development expenses** | $ 383,255 | $ 321,445 | $ 761,956 | $ 658,358 |
| **GAAP selling, general and administrative expenses** | $ 194,669 | $ 191,804 | $ 386,746 | $ 374,062 |
| Stock-based compensation expense | (40,467) | (45,527) | (81,408) | (87,185) |
| Acquisition-related costs (7) | — | (248) | — | (453) |
| **Non-GAAP selling, general and administrative expenses** | $ 154,202 | $ 146,029 | $ 305,338 | $ 286,424 |
| **Combined non-GAAP R&D and SG&A expenses** | $ 537,457 | $ 467,474 | $ 1,067,294 | $ 944,782 |

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | **2021** | **2020** | **2021** | **2020** |
| **GAAP other income (expense), net** | $ 8,051 | $ 116,365 | $ (44,602) | $ 55,235 |
| (Increase) decrease in fair value of strategic investments (4) | (10,609) | (109,986) | 41,686 | (65,116) |
| **Non-GAAP other (expense) income, net** | $ (2,558) | $ 6,379 | $ (2,916) | $ (9,881) |
| **GAAP (benefit from) provision for income taxes** | $ (111,179) | $ (12,500) | $ 56,643 | $ 42,281 |
| Tax adjustments (2) | 312,484 | 196,325 | 351,445 | 325,933 |
| **Non-GAAP provision for income taxes (8)** | $ 201,305 | $ 183,825 | $ 408,088 | $ 368,214 |
| **GAAP effective tax rate** | 251 % | (2 )% | 7 % | 3 % |
| **Non-GAAP effective tax rate (8)** | 20 % | 21 % | 20 % | 21 % |

**Condensed Consolidated Balance Sheets**
(in thousands)
(unaudited)

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| **Assets** | | |

| | | |
|---|---|---|
| Cash, cash equivalents and marketable securities | $ 6,707,993 | $ 6,658,897 |
| Accounts receivable, net | 929,142 | 885,352 |
| Inventories | 321,620 | 280,777 |
| Property and equipment, net | 1,021,233 | 958,534 |
| Goodwill and intangible assets | 1,402,158 | 1,402,158 |
| Deferred tax assets | 952,808 | 882,779 |
| Other assets | 886,732 | 683,311 |
| **Total assets** | $ 12,221,686 | $ 11,751,808 |
| | | |
| **Liabilities and Shareholders' Equity** | | |
| Accounts payable and accrued expenses | $ 1,610,090 | $ 1,560,110 |
| Finance lease liabilities | 569,752 | 581,476 |
| Contingent consideration | 187,300 | 189,600 |
| Other liabilities | 658,148 | 733,807 |
| Shareholders' equity | 9,196,396 | 8,686,815 |
| **Total liabilities and shareholders' equity** | $ 12,221,686 | $ 11,751,808 |
| | | |
| Common shares outstanding | 259,114 | 259,890 |

**Notes and Explanations**

**1:** "Research and development expenses" include the company's $900 million upfront payment to CRISPR in the three and six months ended June 30, 2021.

**2:** In the three and six months ended June 30, 2021 and 2020, "Tax adjustments" included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income (loss) and non-recurring discrete benefits to the company's provision for income taxes. The estimated income taxes related to non-GAAP adjustments to the company's pre-tax income (loss) included adjustments for (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation), (ii) changes in the fair value of the company's strategic investments and (iii) collaborative upfront and milestone payments and (iv) other adjustments. Also included in "Tax adjustments" was a $100 million discrete benefit related to an increase in the U.K.'s corporate tax rate in the three and six months ended June 30, 2021, a $187 million discrete benefit related to the transfer of intellectual property rights to the company's U.K. entity in the three months ended June 30, 2020 and a $50 million discrete benefit from the first quarter of 2020 related to the write-off of a long-term intercompany receivable, which was included in the six months ended June 30, 2020.

**3:** 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 collaborative upfront and milestone payments related to existing 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.

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

**5:** During the three and six months ended June 30, 2021 and 2020, the change in the fair value of contingent consideration relates to potential payments to Exonics Therapeutics' former equity holders.

**6:** "Collaborative revenues and expenses" in the three and six months ended June 30, 2021 and 2020 related to collaborative upfront and milestone payments, including the company's $900 million upfront payment to CRISPR in the three and six months ended June 30, 2021.

**7:** "Acquisition-related costs" in the three and six months ended June 30, 2021 and 2020 related to costs associated with the company's acquisition of Exonics Therapeutics in 2019.

**8:** 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 represents a cash tax payable, 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 availability, new approvals, and timing thereof, (ii) anticipated future label expansions, (iii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including study designs, clinical site activations, patient enrollment, data availability and timing thereof, (iv) expectations for the collaborations with CRISPR, including expectations regarding achievement of target enrollment in the CTX001 clinical studies, anticipated benefits of the collaborations, the potential of CTX001 to be a one-time functional cure for patients with TDT and SCD, and the possibility of regulatory filings in the next 18-24 months, (v) expectations for uptake of and expanded access to the company's medicines, including additional reimbursement agreements, (vi) expectations for continued growth in the number of CF patients treated with our medicines, including our belief that we will reach more patients in 2021 than previously forecasted, (vii) expectations for our pain program, including the creation of a new class of non-opioid medicines that have the potential to provide superior relief of acute pain, our plan to commence the abdominoplasty study in the coming weeks, and expectation for available data from the bunionectomy trial, (viii) expectations for our CF pipeline program, including the plans to initiate Phase 3 studies of the next-in-class, once-daily triple combination and the potential of this new triple combination to provide enhanced benefit for people with CF, and (ix) expectations for the availability of initial data from the VX-880 study. 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 data from preclinical testing or early clinical trials, especially if based on a limited number of patients, may not be indicative of final results or available on anticipated timelines, 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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Source: Vertex Pharmaceuticals Incorporated