# Vertex Reports Fourth Quarter 2021 and Full Year Financial Results

January 26, 2022

*-Full year product revenues of $7.57 billion, a 22% increase compared to full year 2020-*

*-Company provides full year 2022 product revenue guidance of $8.4 to $8.6 billion-*

*- Advancing broad clinical pipeline across six disease areas; multiple programs in mid- and late-stage development with clinical readouts expected in 2022-*

BOSTON--(BUSINESS WIRE)--Jan. 26, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the fourth quarter and full year ended December 31, 2021 and provided full year 2022 financial guidance.

"In 2021, Vertex delivered exceptional financial performance, including 22% revenue growth, coupled with important progress across our business. We expanded our leadership in cystic fibrosis-- treating more patients than ever before and advancing our next-in-class triple regimen into pivotal studies. In addition, our pipeline beyond CF accelerated and delivered important clinical data in new disease areas," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "As we move into 2022, with multiple programs in mid- and late-stage development, there are important milestones ahead. With continued innovation in CF and progress across our pipeline, we are poised to serve many more patients and drive revenue and earnings growth in 2022 and many years into the future."

**Fourth Quarter and Full Year 2021 Financial Highlights**

| | Three Months Ended December 31, | | % | Twelve Months Ended December 31, | | % |
|---|---|---|---|---|---|---|
| | 2021 | 2020 | Change | 2021 | 2020 | Change |
| | (in millions, except per share amounts) | | | | | |
| **Product revenues, net** | $ 2,073 | $ 1,627 | 27% | $ 7,573 | $ 6,203 | 22% |
| **TRIKAFTA/KAFTRIO** | $ 1,693 | $ 1,091 | | $ 5,697 | $ 3,864 | |
| **SYMDEKO/SYMKEVI** | $ 80 | $ 128 | | $ 420 | $ 629 | |
| **ORKAMBI** | $ 147 | $ 215 | | $ 772 | $ 908 | |
| **KALYDECO** | $ 152 | $ 193 | | $ 684 | $ 803 | |
| **GAAP operating income** | $ 878 | $ 746 | 18% | $ 2,782 | $ 2,856 | (3)% |
| **Non-GAAP operating income** | $ 1,124 | $ 887 | 27% | $ 4,344 | $ 3,491 | 24% |
| **GAAP net income** | $ 770 | $ 604 | 27% | $ 2,342 | $ 2,712 | (14)% |
| **Non-GAAP net income** | $ 866 | $ 661 | 31% | $ 3,384 | $ 2,719 | 24% |
| **GAAP net income per share - diluted** | $ 3.00 | $ 2.30 | 30% | $ 9.01 | $ 10.29 | (12)% |
| **Non-GAAP net income per share - diluted** | $ 3.37 | $ 2.51 | 34% | $ 13.02 | $ 10.32 | 26% |

**Full Year 2021 Results**

**Product revenues** increased 22% to $7.57 billion compared to 2020, primarily driven by the launch of KAFTRIO in multiple countries internationally and the performance of TRIKAFTA in the U.S., including the launch of TRIKAFTA in children 6-11 years old in the U.S. Net product revenues in 2021 increased 10% to $5.29 billion in the U.S. and increased 66% to $2.29 billion outside the U.S., compared to 2020.

**GAAP net income** decreased compared to 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 2020, driven by strong product revenue growth.

**Cash, cash equivalents and marketable securities** as of December 31, 2021 were $7.5 billion, an increase of approximately $0.9 billion compared to December 31, 2020. The increase was primarily driven by strong operating cash flow partially offset by

repurchases of our common stock authorized under our share repurchase programs and the $900 million payment to CRISPR Therapeutics.

**Full Year 2021 Expenses**

| | Twelve Months Ended December 31, | |
|---|---|---|
| | 2021 | 2020 |
| | (in millions) | |
| **Combined GAAP R&D and SG&A expenses** | $ 3,891 | $ 2,600 |
| **Combined Non-GAAP R&D and SG&A expenses** | $ 2,332 | $ 1,981 |
| **GAAP R&D expenses** | $ 3,051 | $ 1,830 |
| **Non-GAAP R&D expense** | $ 1,658 | $ 1,372 |
| **GAAP SG&A expenses** | $ 840 | $ 770 |
| **Non-GAAP SG&A expense** | $ 673 | $ 609 |
| **GAAP income taxes (1)** | $ 388 | $ 405 |
| **Non-GAAP income taxes** | $ 891 | $ 721 |
| **GAAP effective tax rate (1)** | 14% | 13% |
| **Non-GAAP effective tax rate** | 21% | 21% |

**Combined GAAP R&D and SG&A expenses** increased compared to 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 2020, primarily due to the advancement and expansion of Vertex's pipeline and incremental investment to support the launches of Vertex's medicines globally.

**GAAP income taxes** decreased compared to 2020, primarily due to the income tax impact of the $900 million payment to CRISPR and the impact of discrete tax events in 2021 compared to 2020 (Note 1).

**Non-GAAP income taxes** increased compared to 2020, primarily due to Vertex's increased operating income.

**Fourth Quarter 2021 Results**

**Product revenues** increased 27% to $2.07 billion compared to the fourth quarter of 2020, primarily driven by the strong launches of KAFTRIO in Europe and the performance of TRIKAFTA in the U.S. Net product revenues in the fourth quarter of 2021 increased 16% to $1.39 billion in the U.S. and increased 61% to $679 million outside the U.S., compared to the fourth quarter of 2020.

**GAAP and Non-GAAP net income** increased compared to the fourth quarter of 2020, driven by strong product revenue growth.

**Fourth Quarter 2021 Expenses**

| | Three Months Ended December 31, | |
|---|---|---|
| | 2021 | 2020 |
| | (in millions) | |
| **Combined GAAP R&D and SG&A expenses** | $ 950 | $ 678 |
| **Combined Non-GAAP R&D and SG&A expenses** | $ 703 | $ 539 |
| **GAAP R&D expenses** | $ 694 | $ 467 |
| **Non-GAAP R&D expenses** | $ 493 | $ 364 |
| **GAAP SG&A expenses** | $ 255 | $ 212 |
| **Non-GAAP SG&A expenses** | $ 210 | $ 175 |
| **GAAP income taxes (1)** | $ 101 | $ 284 |
| **Non-GAAP income taxes** | $ 239 | $ 198 |
| **GAAP effective tax rate (1)** | 12% | 32% |
| **Non-GAAP effective tax rate** | 22% | 23% |

**Combined GAAP R&D and SG&A expenses** increased compared to the fourth quarter of 2020, primarily due to the advancement and expansion of Vertex's pipeline, incremental investment to support the launches of Vertex's medicines globally, increased collaborative payments related to our business development activities and increased stock-based compensation expenses.

**Combined Non-GAAP R&D and SG&A expenses** increased compared to the fourth quarter of 2020, primarily due to the advancement and expansion of Vertex's pipeline and incremental investment to support the launches of Vertex's medicines globally.

**GAAP income taxes** decreased compared to the fourth quarter of 2020, primarily due to the impact of discrete tax events recognized in the fourth quarter of 2021 (Note 1) and the income tax impact on sales of certain strategic investments in the fourth quarter of 2020.

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

**Full Year 2022 Financial Guidance**

Vertex today provided full year 2022 financial guidance. Vertex's product revenue guidance is primarily based on expectations for continued strong performance of TRIKAFTA in the U.S., and KAFTRIO outside the U.S. Vertex's product revenue guidance reflects management's expectations for approved products in countries where Vertex has already secured reimbursement.

Vertex's guidance is summarized below:

| | FY 2022 |
|---|---|
| **Product revenues** | $8.4 to $8.6 billion |
| **Combined GAAP R&D and SG&A expenses (2)** | $3.30 to $3.45 billion |
| **Combined Non-GAAP R&D and SG&A expenses (2)** | $2.70 to $2.75 billion |
| **Non-GAAP effective tax rate** | 21% to 22% |

**Key Business Highlights**

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

Vertex anticipates the number of CF patients treated with our medicines will continue to grow as the uptake of TRIKAFTA in the U.S. and the launches of KAFTRIO outside the U.S. continue, we enter into additional reimbursement agreements and achieve new approvals for the treatment of younger patients. Recent progress includes:

- In January 2022, the European Commission and the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) approved a label extension for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor, for the treatment of CF in children ages 6 through 11 years old who have at least one F508del mutation in the CF transmembrane conductance regulator (CFTR) gene. With these approvals, approximately 1,900 children will be newly eligible for KAFTRIO®.
- In the fourth quarter of 2021, we secured additional reimbursement approvals for KAFTRIO® (ivacaftor/tezacaftor /elexacaftor) in a combination regimen with ivacaftor for the treatment of CF for eligible patients, including national reimbursement agreements in Spain and the Netherlands. The agreements generally cover people with CF ages 12 years and older who have at least one copy of the F508del mutation and allow us to introduce KAFTRIO® in these markets.
- The Phase 3 study of ORKAMBI in patients 12 to 24 months of age met its primary endpoint. ORKAMBI was well tolerated in this patient population, and no new safety concerns were identified. Substantial improvements were seen in sweat chloride, the secondary endpoint of the study. Based on these data, Vertex intends to submit regulatory filings in the U.S. in Q1 and in Europe in Q2 2022.

TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in more than 20 countries.

***R&D pipeline***

Vertex is delivering on a diversified 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 continues to pursue next-in-class CFTR modulator therapies as well as new treatment options for the approximately 10% of patients who cannot benefit from CFTR modulators alone.

- Enrollment is underway in two Phase 3 global, randomized, double-blind, active-controlled clinical trials (SKYLINE 102 and SKYLINE 103) evaluating Vertex's new once-daily investigational triple combination of VX-121/ *tezacaftor*/VX-561 in patients with CF. The SKYLINE 102 and SKYLINE 103 trials are expected to include 950 patients in total and will compare the performance of VX-121/*tezacaftor*/VX-561 to TRIKAFTA. Enrollment in both trials is expected to be completed by late 2022 or early 2023.

- In collaboration with Moderna, Vertex is evaluating CF mRNA therapeutics designed to treat the underlying cause of CF by enabling cells in the lungs to produce functional CFTR protein for the treatment of the approximately 10% of CF patients who do not produce any CFTR protein. IND-enabling studies are underway, and we plan to submit an IND for this program in 2022.

***Beta Thalassemia and Sickle Cell Disease (SCD)***

The CTX001 program employs a non-viral *ex vivo* CRISPR gene-editing therapy, which is being developed as a potential functional cure for transfusion-dependent thalassemia (TDT) and severe sickle cell disease (SCD).

- Enrollment is complete in the ongoing Phase 3 clinical trials in TDT and SCD, with more than 70 patients dosed to date. Vertex anticipates submitting global regulatory filings for CTX001 in TDT and SCD in late 2022.

***APOL1-Mediated Kidney Disease (AMKD)***

Vertex is evaluating the potential of oral, small molecule inhibitors of APOL1 function to treat people with AMKD.

- In December, Vertex announced that, in a Phase 2 proof-of-concept (POC) study in patients with APOL1-mediated focal segmental glomerulosclerosis (FSGS), VX-147 achieved a statistically significant and clinically meaningful mean reduction of 47.6% in the urine protein to creatinine ratio (UPCR) at Week 13 compared to baseline, on top of standard of care. VX-147 was well tolerated, with no treatment discontinuations due to adverse events and no serious adverse events considered related to study drug. These results provided the first clinical evidence and POC that an oral small molecule APOL1 inhibitor can decrease proteinuria in patients with APOL1-mediated kidney disease. Vertex anticipates completing its end of Phase 2 meeting with regulators and initiating pivotal development of VX-147 in AMKD in the first quarter of 2022.

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

Vertex is evaluating cell therapies designed to replace insulin-producing islet cells that are destroyed in people with T1D with the goal of developing a potential functional cure for this disease.

- VX-880 is a stem cell-derived, fully differentiated islet replacement therapy, using standard immunosuppression to protect the implanted cells. VX-880 is being evaluated in a Phase 1/2 clinical trial for the treatment of T1D.

- In January, Vertex announced Day 150 data for the first T1D patient in the Phase 1/2 clinical trial, treated with a single infusion of VX-880 at half the target dose. These data demonstrated robust improvements in fasting C-peptide (levels increased to 404 pmol/L) and glycemic control, with HbA1c reaching 6.7% and daily exogenous insulin requirement at 2 units, providing evidence of a clinically meaningful therapeutic effect of this single treatment with VX-880.

- The VX-880 Phase 1/2 study is ongoing in the U.S. and Canada. Enrollment and dosing continues and Vertex expects to share data from more patients and report longer-term follow up in 2022.

- Vertex is pursuing additional programs in T1D, in which these stem cell-derived islets are encapsulated and implanted in an immunoprotective device or modified to produce hypoimmune stem cell islets. IND-enabling studies for the cells plus device program are underway, and we plan to submit an IND in 2022.

***Pain (NaV1.8)***

Vertex has discovered multiple selective small molecule inhibitors of NaV1.8 with the objective of creating a new class of medicines that have the potential to be highly effective for both acute and chronic pain, without the limitations of opioids and other existing pain medications.

- Vertex is conducting two Phase 2 dose ranging acute pain studies with VX-548, one following bunionectomy surgery and the other following abdominoplasty surgery. Vertex expects to obtain data from both studies in Q1 2022.

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

- Vertex plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022.

Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs, with additional assets in CF, SCD, Beta Thalassemia, AMKD, T1D, Pain, and AATD in earlier stages of development.

**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 upfront and milestones payments, including the $900 million upfront payment to CRISPR Therapeutics, and certain other business development activities, (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**
**Fourth Quarter and Full Year Results**
**Consolidated Statements of Operations**
(in millions, except per share amounts)
(unaudited)

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | **2021** | **2020** | **2021** | **2020** |
| Revenues: | | | | |
| Product revenues, net | $ 2,072.6 | $ 1,626.9 | $ 7,573.4 | $ 6,202.8 |
| Other revenues | — | 0.9 | 1.0 | 2.9 |
| Total revenues | 2,072.6 | 1,627.8 | 7,574.4 | 6,205.7 |
| Costs and expenses: | | | | |
| Cost of sales | 247.4 | 203.1 | 904.2 | 736.3 |
| Research and development expenses (3) | 694.3 | 466.6 | 3,051.1 | 1,829.5 |
| Selling, general and administrative expenses | 255.2 | 211.8 | 840.1 | 770.5 |
| Change in fair value of contingent consideration | (2.0) | 0.5 | (3.1) | 13.1 |
| Total costs and expenses | 1,194.9 | 882.0 | 4,792.3 | 3,349.4 |
| Income from operations | 877.7 | 745.8 | 2,782.1 | 2,856.3 |
| Interest income | 1.2 | 2.3 | 4.9 | 22.2 |
| Interest expense | (15.1) | (16.3) | (61.5) | (58.2) |
| Other income, net | 7.1 | 156.8 | 4.9 | 296.6 |
| Income before provision for income taxes | 870.9 | 888.6 | 2,730.4 | 3,116.9 |
| Provision for income taxes | 100.8 | 284.4 | 388.3 | 405.2 |
| Net income | $ 770.1 | $ 604.2 | $ 2,342.1 | $ 2,711.7 |
| | | | | |
| Net income per common share: | | | | |
| Basic | $ 3.02 | $ 2.32 | $ 9.09 | $ 10.44 |
| Diluted | $ 3.00 | $ 2.30 | $ 9.01 | $ 10.29 |
| Shares used in per share calculations: | | | | |
| Basic | 254.6 | 260.0 | 257.7 | 259.8 |
| Diluted | 257.0 | 263.1 | 259.9 | 263.4 |

**Reconciliation of GAAP to Non-GAAP Net Income and Operating Income**
**Fourth Quarter and Full Year Results**
(in millions, except per share amounts)
(unaudited)

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP net income** | $ 770.1 | $ 604.2 | $ 2,342.1 | $ 2,711.7 |
| Stock-based compensation expense | 118.6 | 97.0 | 441.4 | 429.5 |
| Increase in fair value of strategic investments (4) | (12.1) | (171.1) | (17.1) | (311.9) |
| (Decrease) increase in fair value of contingent consideration (5) | (2.0) | 0.5 | (3.1) | 13.1 |
| Collaborative revenues and expenses (6) | 126.5 | 40.4 | 1,112.3 | 181.7 |
| Acquisition-related costs (7) | 2.8 | 2.8 | 11.3 | 10.6 |
| Total non-GAAP adjustments to pre-tax income | 233.8 | (30.4) | 1,544.8 | 323.0 |
| Tax adjustments (1) | (138.0) | 86.7 | (502.8) | (315.5) |
| **Non-GAAP net income** | $ 865.9 | $ 660.5 | $ 3,384.1 | $ 2,719.2 |
| | | | | |
| Net income per diluted common share: | | | | |
| GAAP | $ 3.00 | $ 2.30 | $ 9.01 | $ 10.29 |
| Non-GAAP | $ 3.37 | $ 2.51 | $ 13.02 | $ 10.32 |
| Shares used in diluted per share calculations: | | | | |
| GAAP and Non-GAAP | 257.0 | 263.1 | 259.9 | 263.4 |

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP operating income** | $ 877.7 | $ 745.8 | $ 2,782.1 | $ 2,856.3 |
| Stock-based compensation expense | 118.6 | 97.0 | 441.4 | 429.5 |
| (Decrease) increase in fair value of contingent consideration (5) | (2.0) | 0.5 | (3.1) | 13.1 |
| Collaborative revenues and expenses (6) | 126.5 | 40.4 | 1,112.3 | 181.7 |
| Acquisition-related costs (7) | 2.8 | 2.8 | 11.3 | 10.6 |
| **Non-GAAP operating income** | $ 1,123.6 | $ 886.5 | $ 4,344.0 | $ 3,491.2 |

**Reconciliation of GAAP to Non-GAAP Revenues and Expenses**
**Fourth Quarter and Full Year Results**
(in millions)
(unaudited)

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP total revenues** | $ 2,072.6 | $ 1,627.8 | $ 7,574.4 | $ 6,205.7 |
| Collaborative revenues | — | (0.9) | (1.0) | (2.9) |
| **Non-GAAP total revenues** | $ 2,072.6 | $ 1,626.9 | $ 7,573.4 | $ 6,202.8 |

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| **GAAP cost of sales** | $ 247.4 | $ 203.1 | $ 904.2 | $ 736.3 |
| Stock-based compensation expense | (1.7) | (1.6) | (6.3) | (5.6) |
| **Non-GAAP cost of sales** | $ 245.7 | $ 201.5 | $ 897.9 | $ 730.7 |
| | | | | |
| **GAAP research and development expenses** | $ 694.3 | $ 466.6 | $ 3,051.1 | $ 1,829.5 |
| Stock-based compensation expense | (71.9) | (59.0) | (268.3) | (262.7) |
| Collaborative expenses (6) | (126.5) | (41.3) | (1,113.3) | (184.6) |
| Acquisition-related costs (7) | (2.8) | (2.8) | (11.3) | (10.2) |
| **Non-GAAP research and development expenses** | $ 493.1 | $ 363.5 | $ 1,658.2 | $ 1,372.0 |

| | | | | |
|---|---|---|---|---|
| **GAAP selling, general and administrative expenses** | $ 255.2 | $ 211.8 | $ 840.1 | $ 770.5 |
| Stock-based compensation expense | (45.0) | (36.4) | (166.8) | (161.2) |
| Acquisition-related costs (7) | — | — | — | (0.4) |
| **Non-GAAP selling, general and administrative expenses** | $ 210.2 | $ 175.4 | $ 673.3 | $ 608.9 |
| **Combined non-GAAP R&D and SG&A expenses** | $ 703.3 | $ 538.9 | $ 2,331.5 | $ 1,980.9 |

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | **2021** | **2020** | **2021** | **2020** |
| **GAAP other income, net** | $ 7.1 | $ 156.8 | $ 4.9 | $ 296.6 |
| Increase in fair value of strategic investments (4) | (12.1) | (171.1) | (17.1) | (311.9) |
| **Non-GAAP other expense, net** | $ (5.0) | $ (14.3) | $ (12.2) | $ (15.3) |
| **GAAP provision for income taxes** | $ 100.8 | $ 284.4 | $ 388.3 | $ 405.2 |
| Tax adjustments (1) | 138.0 | (86.7) | 502.8 | 315.5 |
| **Non-GAAP provision for income taxes** | $ 238.8 | $ 197.7 | $ 891.1 | $ 720.7 |
| **GAAP effective tax rate** | 12% | 32% | 14% | 13% |
| **Non-GAAP effective tax rate** | 22% | 23% | 21% | 21% |

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

| | December 31, 2021 | December 31, 2020 |
|---|---|---|
| **Assets** | | |
| Cash, cash equivalents and marketable securities | $ 7,524.9 | $ 6,658.9 |
| Accounts receivable, net | 1,136.8 | 885.4 |
| Inventories | 353.1 | 280.8 |
| Property and equipment, net | 1,094.1 | 958.5 |
| Goodwill and intangible assets | 1,402.2 | 1,402.2 |
| Deferred tax assets | 934.5 | 882.8 |
| Other assets | 986.9 | 683.2 |
| **Total assets** | $ 13,432.5 | $ 11,751.8 |
| **Liabilities and Shareholders' Equity** | | |
| Accounts payable and accrued expenses | $ 1,873.6 | $ 1,560.1 |
| Finance lease liabilities | 556.7 | 581.5 |
| Contingent consideration | 186.5 | 189.6 |
| Other liabilities | 715.7 | 733.8 |
| Shareholders' equity | 10,100.0 | 8,686.8 |
| **Total liabilities and shareholders' equity** | $ 13,432.5 | $ 11,751.8 |
| Common shares outstanding | 254.5 | 259.9 |

**Notes and Explanations**

**1:** In the three and twelve months ended December 31, 2021 and 2020, "Tax adjustments" included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income and non-recurring discrete benefits to the company's provision for income taxes. "Tax adjustments" in the three and twelve months ended December 31, 2021 included a $44 million discrete benefit resulting from the conclusion of an R&D tax credit study. The twelve months ended December 31, 2021 also included a $95 million discrete benefit related to an increase in the U.K.'s corporate tax rate from 19% to 25%, which was enacted in June 2021 and will become effective in April 2023. "Tax adjustments" in the twelve months ended December 31, 2020 included discrete benefits of: (i) $209 million related to the transfer of intellectual property rights to the company's U.K. entity, (ii) $50 million related to the write-off of a long-term intercompany receivable and (iii) $38 million related to an increase in the U.K.'s corporate tax rate from 17% to 19%, which was enacted and became effective in July 2020.

**2:** The difference between the company’s full year 2022 combined GAAP R&D and SG&A expenses and combined non-GAAP

R&D and SG&A expenses guidance relates primarily to $120 million to $170 million of collaborative milestone payments and certain other business development activities related to existing business development agreements and $440 million to $510 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.

**3:** "Research and development expenses" includes the $900 million upfront payment to CRISPR in the twelve months ended December 31, 2021.

**4:** "Other income, net" includes net 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 twelve months ended December 31, 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 twelve months ended December 31, 2021 and 2020 related to collaborative upfront and milestone payments and certain other business development activities. The company's $900 million upfront payment to CRISPR is included in "Collaborative revenues and expenses" during the twelve months ended December 31, 2021.

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

***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 disease. In addition, Vertex has a rapidly expanding pipeline of cell and genetic 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 12 consecutive years on Science magazine's Top Employers list, one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies, 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 2022 Financial Guidance" and statements regarding (i) anticipated regulatory filings, data availability, and timing thereof, (ii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including study designs, patient enrollment, data availability and timing thereof, (iii) anticipated global regulatory filings for CTX001 in late 2022, (iv) expectations for continued growth in the number of CF patients treated with our medicines, including the number of children newly eligible for KAFTRIO in Europe, uptake of and expanded access to the company’s medicines, additional reimbursement agreements, new approvals, and expansion of treatment options for the patients who cannot benefit from CFTR modulators, (v) expectations for our pain program, including our expectation to obtain data from the bunionectomy and abdominoplasty studies in the first quarter of 2022, (vi) plans to enroll and dose more patients with VX-880 and to report longer-term follow up on our VX-880 program in 2022, (vii) expectations for an IND submission for our T1D cells plus device program in 2022, (viii) plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022, (ix) expectations for our collaboration with Moderna to evaluate CF mRNA therapeutics, including our plans to submit an IND for this program in 2022, (x) our plans to complete end of Phase 2 meetings with regulators and initiation of VX-147 into pivotal development in AMKD in the first quarter of 2022, and (xi) our plans to submit regulatory filings for ORKAMBI in the U.S. and Europe. 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 2022 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 the company may not be able to submit the anticipated regulatory filings on the expected timeline, or at all, 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 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, or the scientific data presented. 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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