Vertex Reports Second Quarter 2022 Financial Results

August 4, 2022

— Product revenues of $2.20 billion, a 22% increase compared to Q2 2021 —

— Company raises full year 2022 product revenue guidance to $8.6 to $8.8 billion —

— Recent exa-cel and VX-880 clinical data presentations demonstrate transformative potential for patients with sickle cell disease, beta thalassemia and type 1 diabetes—

— Multiple clinical programs entering or progressing through late-stage clinical development—

BOSTON--(BUSINESS WIRE)--Aug. 4, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2022 and updated its full year 2022 financial guidance.

“With sustained and growing leadership in CF, programs in five disease areas now entering or progressing through late-stage clinical development and the next wave of innovation beginning to enter the clinic later this year, Vertex has reached a new inflection point,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “As we reach more CF patients, we are poised to deliver significant, durable financial returns for years to come. In parallel, we are advancing a broad and deep clinical pipeline of potentially transformative medicines across multiple serious diseases, spanning small molecules, cell and genetic therapies, and we expect this diversified portfolio of medicines will serve many more patients and drive substantial growth in the future.”

Second Quarter 2022 Financial Highlights

	Three Months Ended June 30,		%
	2022	2021	Change
	(in millions, except per share amounts)		
Product revenues, net	$ 2,196	$ 1,793	22%
TRIKAFTA/KAFTRIO	$ 1,893	$ 1,256	
SYMDEKO/SYMKEVI	$ 43	$ 134	
ORKAMBI	$ 122	$ 221	
KALYDECO	$ 139	$ 183	
GAAP operating income (loss)	$ 1,106	$ (38)	
Non-GAAP operating income *	$ 1,187	$ 71	
GAAP net income	$ 810	$ 67	
Non-GAAP net income *	$ 930	$ 43	
GAAP net income per share - diluted	$ 3.13	$ 0.26	
Non-GAAP net income per share - diluted *	$ 3.60	$ 0.17	

*Starting in the first quarter of 2022, Vertex no longer excludes research and development charges resulting from upfront or contingent milestone payments in connection with collaborations, asset acquisitions and/or licensing of third-party intellectual property rights from its Non-GAAP financial measures. Non-GAAP financial measures for the second quarter of 2021 have been recast to reflect this change.

Product revenues increased 22% to $2.20 billion compared to the second quarter of 2021, primarily driven by the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued strong performance of TRIKAFTA in the U.S., including the June 2021 launch of TRIKAFTA in children 6-11 years old in the U.S. Net product revenues in the second quarter of 2022 increased 13% to $1.42 billion in the U.S. and increased 46% to $781 million outside the U.S., compared to the second quarter of 2021.

GAAP and Non-GAAP net income increased compared to the second quarter of 2021, primarily due to strong product revenue growth and a one-time $900 million payment in connection with the amendment of Vertex's collaboration with CRISPR

Therapeutics in the second quarter of 2021. The payment to CRISPR is included in acquired in-process research and development expenses ("Acquired IPR&D") in the second quarter of 2021.

Cash, cash equivalents and marketable securities as of June 30, 2022 were $9.3 billion, an increase of approximately $1.7 billion compared to December 31, 2021. The increase was primarily driven by strong revenue growth and operating cash flow.

Second Quarter 2022 Expenses

	Three Months Ended June 30,	
	2022	2021
	(in millions)	
Combined GAAP R&D, Acquired IPR&D and SG&A expenses	$ 877	$ 1,602
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses *	$ 750	$ 1,496
GAAP R&D expenses	$ 600	$ 449
Non-GAAP R&D expenses *	$ 515	$ 383
Acquired IPR&D *	$ 62	$ 958
GAAP SG&A expenses	$ 215	$ 195
Non-GAAP SG&A expenses	$ 173	$ 154
GAAP income taxes (1)	$ 214	$ (111)
Non-GAAP income taxes *	$ 259	$ 11
GAAP effective tax rate (1)	21%	251%
Non-GAAP effective tax rate	22%	20%

*Starting in the first quarter of 2022, Vertex no longer excludes research and development charges resulting from upfront or contingent milestone payments in connection with collaborations, asset acquisitions and/or licensing of third-party intellectual property rights from its Non-GAAP financial measures. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations. Non-GAAP financial measures for the second quarter of 2021 have been recast to reflect this change.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses decreased compared to the second quarter of 2021, primarily due to the one-time $900 million payment to CRISPR in the second quarter of 2021, partially offset by increased investment in support of multiple programs that have advanced in mid- and late-stage clinical development and the costs to support launches of Vertex's therapies globally.

GAAP and Non-GAAP income taxes increased compared to the second quarter of 2021, primarily due to the income tax impact of the $900 million payment to CRISPR in the second quarter of 2021. GAAP income taxes also increased due to a discrete tax benefit recorded in the second quarter of 2021. Please refer to Note 1 for further details.

Full Year 2022 Financial Guidance

Vertex is raising its full year 2022 product revenue guidance to $8.6 to $8.8 billion. The increase primarily reflects the robust uptake of KAFTRIO/TRIKAFTA in countries outside the U.S. where Vertex has recently achieved reimbursement. Vertex is also increasing full year 2022 combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expense guidance. The increase results from the advancement of the company's clinical pipeline, including a number of programs that have recently entered or are initiating late-stage development, and from incremental expenses related to recent business development activity.

Updated guidance is summarized below:

	Current FY 2022	Previous FY 2022
Product revenues	$8.6 to $8.8 billion	$8.4 to $8.6 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	$3.48 to $3.63 billion	$3.30 to $3.45 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2) *	$3.0 to $3.1 billion	$2.82 to $2.92 billion
Non-GAAP effective tax rate	Unchanged	21% to 22%

*Starting in the first quarter of 2022, Vertex no longer excludes research and development charges resulting from upfront or contingent milestone payments in connection with collaborations, asset acquisitions and/or licensing of third-party intellectual property rights from its Non-GAAP financial measures. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations.

Key Business Highlights

Cystic Fibrosis (CF) Marketed Products

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

- Vertex has completed the Phase 3 study of TRIKAFTA/KAFTRIO (elexacaftor/tezacaftor/ivacaftor and ivacaftor) in children 2 to 5 years old. Data from this study showed similarly compelling efficacy of TRIKAFTA/KAFTRIO in children 2 to 5 years old to other age groups and no new safety findings. The Company expects to present the results at a medical forum later in 2022. Vertex anticipates submitting global regulatory filings for TRIKAFTA/KAFTRIO in children 2 to 5 years old this year.
- At the European Cystic Fibrosis Society's (ECFS) European Cystic Fibrosis Conference in June, Vertex presented data from the U.S. CF Foundation Patient Registry (CFFPR) of more than 16,000 people treated with TRIKAFTA for an average of nine months, showing that treatment with TRIKAFTA was associated with improved lung function and reduced risk of pulmonary exacerbations compared to pre-TRIKAFTA baseline, as well as lower risks of lung transplant and death, compared to the historical 2019 U.S. CFFPR population. Vertex also presented data for TRIKAFTA demonstrating no loss in mean lung function in people with F/F and F/MF mutations over a two-year period, in contrast to declines seen in the matched controls.
- In April, Health Canada granted marketing authorization for TRIKAFTA in children 6 to 11 years of age. With this approval, approximately 500 children with CF became newly eligible for treatment with a CFTR modulator. Vertex recently signed a Letter of Intent (LOI) with the pan-Canadian Pharmaceutical Alliance (pCPA) paving the way for reimbursement of TRIKAFTA for this age group.
- Vertex has filed a Supplemental New Drug Application (sNDA) with the U.S. Food and Drug Administration (FDA) and a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) for the use of ORKAMBI in children 12 months to less than 24 months old. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target date of September 4, 2022.

TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in more than 25 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 summarized below.

Cystic Fibrosis

Vertex continues to pursue next-in-class, small molecule CFTR modulator therapies as well as new treatment options for the approximately 5,000 patients who cannot benefit from CFTR modulators.

- Vertex is conducting 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 12 years of age and older. The SKYLINE 102 and SKYLINE 103 trials will compare the efficacy and safety of VX-121/tezacaftor/VX-561 to TRIKAFTA. More than 250 sites across both studies are active and enrolling patients. Enrollment in both trials is expected to be completed in late 2022 or early 2023.
- In parallel to SKYLINE 102 and 103, Vertex has also initiated a study of VX-121/tezacaftor/VX-561 in children with CF 6 to 11 years of age.
- In collaboration with Moderna, Vertex is developing CFTR mRNA therapeutics designed to treat the underlying cause of CF by programming cells in the lungs to produce functional CFTR protein for the treatment of the approximately 5,000 people with CF who do not produce any CFTR protein. IND-enabling studies have been completed, and Vertex is on track to submit an IND for this program in 2H 2022.

Beta Thalassemia and Sickle Cell Disease

The exa-cel (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). Vertex is developing exa-cel in collaboration with CRISPR Therapeutics.

- In June, at the European Hematology Association (EHA) Congress, Vertex and CRISPR presented data from 75 patients (44 with TDT, 31 with SCD) from the CLIMB-111, CLIMB-121 and CLIMB-131 studies with follow-up ranging from 1.2 to 37.2 months after exa-cel infusion. All 31 patients with severe SCD, characterized by recurrent vaso-occlusive crises

(VOCs), were free of VOCs after exa-cel infusion through the duration of follow-up, with follow-up ranging from 2.0 to 32.3 months. Of the 44 patients with TDT, 42 were transfusion-free with follow-up ranging from 1.2 to 37.2 months. Two patients who were not yet transfusion-free had 75% and 89% reductions in transfusion volume, respectively. The safety profile was generally consistent with myeloablative conditioning with busulfan and autologous stem cell transplant.
- Two additional Phase 3 studies of exa-cel have been initiated in pediatric patients, one in TDT and a second in SCD.
- Vertex has completed discussions with the EMA and the Medicines and Healthcare products Regulatory Agency (MHRA) on the submission package for exa-cel and is on track to submit for regulatory approvals of exa-cel for SCD and TDT in Europe and the UK by the end of 2022. Discussions with the U.S. FDA are ongoing.

APOL1-Mediated Kidney Disease (AMKD)

Vertex has discovered multiple oral, small molecule inhibitors of APOL1, pioneering a new class of medicines that target an underlying genetic driver of kidney disease.

- In March, Vertex initiated pivotal development of inaxaplin (VX-147) in a single Phase 2/3 study in patients with two APOL1 mutations and proteinuric kidney disease.
- This Phase 2/3 adaptive study will first evaluate two doses of inaxaplin to select a dose for Phase 3 and subsequently evaluate the efficacy and safety of the single, selected dose in the Phase 3 portion of the study. The primary efficacy endpoint for the final analysis is eGFR slope in patients receiving the selected dose of inaxaplin compared to placebo at two years. The study is designed to have a pre-planned interim analysis at Week 48 evaluating eGFR slope, supported by a percent change from baseline in proteinuria in the inaxaplin arm versus placebo. If positive, the interim analysis may serve as the basis for Vertex to seek accelerated approval of inaxaplin in the U.S. for patients with AMKD. Enrollment is ongoing, with more than 30 sites active in the U.S.
- The U.S. FDA recently granted inaxaplin Breakthrough Therapy designation for APOL1-mediated focal segmental glomerulosclerosis (FSGS) and the EMA has also granted inaxaplin Priority Medicines (PRIME) designation for AMKD.

Pain (NaV1.8)

Vertex has discovered multiple selective small molecule inhibitors of NaV1.8 with the objective of creating a new class of pain medicines that have the potential to provide effective pain relief, without the limitations of opioids and other standard-of-care pain medicines.

- In March, Vertex reported positive data from two Phase 2 dose-ranging acute pain studies with VX-548, one following bunionectomy surgery and the other following abdominoplasty surgery. Both studies met their primary endpoint and established proof of concept for VX-548.
- Vertex has completed its end-of-phase 2 meeting with the FDA and has reached agreement on the design of the Phase 3 program in acute pain. The Phase 3 program will include two randomized, double-blind, placebo-controlled studies evaluating the efficacy and safety of VX-548 for moderate to severe acute pain following bunionectomy or abdominoplasty surgery. Each study will also include a hydrocodone bitartrate/acetaminophen (HB/APAP) treatment arm. A third single-arm study will evaluate treatment with VX-548 for up to 14 days in multiple other types of moderate to severe acute pain. Vertex expects to initiate this program in the fourth quarter of 2022.
- Vertex also intends to initiate a Phase 2 dose-ranging study of VX-548 in neuropathic pain by the end of 2022.
- The U.S. FDA has granted VX-548 Breakthrough Therapy designation for moderate to severe acute pain.

Type 1 Diabetes (T1D)

Vertex is evaluating cell therapies using stem cell-derived islets to replace the endogenous 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, used in combination with 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 June, at the American Diabetes Association (ADA) Scientific Sessions Conference, Vertex provided additional data on the two patients dosed at half the target dose in Part A of its VX-880 Phase 1/2 study. Vertex had previously reported that both patients had achieved glucose-responsive insulin production, improvements in glycemic control and reductions in exogenous insulin requirements. Additional data presented at ADA also showed significant increases in the blood glucose time-in-range compared to baseline, following treatment with VX-880. Patient 1 showed a blood glucose time-in-range increase from 40.1% at baseline to 99.9% at Day 270 and was insulin independent. Patient 2 showed a time-in-range increase from 35.9% at baseline to 51.9% at Day 150 with a 30% reduction in exogenous insulin use.
- The VX-880 Phase 1/2 clinical trial has resumed enrollment in the U.S. Part B of the study is open for enrollment at sites in the U.S. and Canada.
- Vertex is advancing additional programs in T1D, in which these same stem cell-derived islets are encapsulated and implanted in an immunoprotective device or modified to produce hypoimmune stem cell islets, with the goal of eliminating the need for immunosuppression.
- Vertex is on track to submit an IND for the cells plus device program in 2022.

Alpha-1 Antitrypsin (AAT) Deficiency

Vertex is working to address the underlying genetic cause of alpha-1 antitrypsin (AAT) deficiency by developing novel small molecule correctors of Z-AAT protein folding, with the goal of increasing the secretion of functional AAT into the blood and addressing both the lung and the liver aspects of AAT deficiency.

- Vertex is on track to advance one or more novel small molecule Z-AAT correctors into the clinic in 2022.

Duchenne Muscular Dystrophy (DMD)

Vertex is investigating a novel approach to treating DMD by delivering CRISPR/Cas9 gene-editing technology to muscle cells, with the goal of restoring near-full length dystrophin protein expression by targeting specific mutations in the dystrophin gene that cause the disease.

- IND-enabling studies for the first *in vivo* gene editing therapy for DMD are underway. Vertex anticipates submitting an IND in 2023.

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.

Investments in External Innovation

Consistent with its strategy to develop transformative medicines for serious diseases, Vertex recently entered into the following transactions:

- In July, Vertex entered into a definitive agreement to acquire ViaCyte, a privately held company focused on delivering novel stem cell-derived cell replacement therapies as a functional cure for type 1 diabetes, for $320 million in cash. The acquisition will provide Vertex with complementary assets, capabilities and technologies that could accelerate Vertex's existing T1D programs. Vertex anticipates the acquisition will close later this year.
- Also in July, Vertex entered into a research collaboration with Verve Therapeutics, focused on discovering and developing an *in vivo* gene editing program for a liver disease.
- In May, Vertex acquired Catalyst Biosciences' complement portfolio and related intellectual property for $60 million in cash, adding capabilities in the area of complement-mediated diseases.

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) gains or losses related to the fair value of the company's strategic investments, (iii) increases or decreases in the fair value of contingent consideration, (iv) acquisition-related costs, (v) an intangible asset impairment charge 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’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, Acquired IPR&D and SG&A expenses and effective tax rate on a non-GAAP basis. The guidance regarding combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expenses does not include estimates associated with any potential future business development transactions, including collaborations, asset acquisitions and/or licensing of third-party intellectual property rights. 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
Consolidated Statements of Operations
(in millions, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
Revenues:				
Product revenues, net	$ 2,196.2	$ 1,793.4	$ 4,293.7	$ 3,516.7

Other revenues	—	—	—	1.0
Total revenues	2,196.2	1,793.4	4,293.7	3,517.7
Costs and expenses:				
Cost of sales	261.8	228.0	507.6	420.3
Research and development expenses	600.1	448.7	1,201.2	903.0
Acquired in-process research and development expenses (3)	61.9	958.4	63.9	960.1
Selling, general and administrative expenses	215.3	194.6	430.5	386.7
Change in fair value of contingent consideration	(49.2)	1.6	(56.7)	(2.3)
Total costs and expenses	1,089.9	1,831.3	2,146.5	2,667.8
Income (loss) from operations	1,106.3	(37.9)	2,147.2	849.9
Interest income	10.8	1.1	12.4	2.6
Interest expense	(14.6)	(15.5)	(29.5)	(31.2)
Other (expense) income, net	(78.1)	8.1	(150.9)	(44.6)
Income (loss) before provision for (benefit from) income taxes	1,024.4	(44.2)	1,979.2	776.7
Provision for (benefit from) income taxes	213.9	(111.2)	406.6	56.6
Net income	$ 810.5	$ 67.0	$ 1,572.6	$ 720.1
Net income per common share:				
Basic	$ 3.17	$ 0.26	$ 6.15	$ 2.78
Diluted	$ 3.13	$ 0.26	$ 6.09	$ 2.75
Shares used in per share calculations:				
Basic	255.9	259.0	255.5	259.2
Diluted	258.7	261.0	258.3	261.5

Vertex Pharmaceuticals Incorporated
Reconciliation of GAAP to Non-GAAP Net Income and Operating Income
(in millions, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
GAAP net income	$ 810.5	$ 67.0	$ 1,572.6	$ 720.1
Stock-based compensation expense	113.9	104.6	244.2	219.8
Decrease (increase) in fair value of strategic investments (4)	84.2	(10.6)	159.8	41.7
(Decrease) increase in fair value of contingent consideration (5)	(49.2)	1.6	(56.7)	(2.3)
Intangible asset impairment charge (5)	13.0	—	13.0	—
Acquisition-related costs (6)	2.8	2.8	5.6	5.6
Total non-GAAP adjustments to pre-tax income *	164.7	98.4	365.9	264.8
Tax adjustments (1) *	(44.7)	(122.1)	(100.9)	(160.3)
Non-GAAP net income *	$ 930.5	$ 43.3	$ 1,837.6	$ 824.6
Net income per diluted common share:				
GAAP	$ 3.13	$ 0.26	$ 6.09	$ 2.75
Non-GAAP *	$ 3.60	$ 0.17	$ 7.11	$ 3.15
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	258.7	261.0	258.3	261.5

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
GAAP operating income (loss)	$ 1,106.3	$ (37.9)	$ 2,147.2	$ 849.9
Stock-based compensation expense	113.9	104.6	244.2	219.8
(Decrease) increase in fair value of contingent consideration (5)	(49.2)	1.6	(56.7)	(2.3)
Intangible asset impairment charge (5)	13.0	—	13.0	—
Acquisition-related costs (6)	2.8	2.8	5.6	5.6
Non-GAAP operating income *	$ 1,186.8	$ 71.1	$ 2,353.3	$ 1,073.0

Vertex Pharmaceuticals Incorporated
Reconciliation of GAAP to Non-GAAP Expenses
(in millions, except percentages)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
GAAP cost of sales	$ 261.8	$ 228.0	$ 507.6	$ 420.3
Stock-based compensation expense	(2.4)	(1.6)	(4.6)	(3.0)
Non-GAAP cost of sales	$ 259.4	$ 226.4	$ 503.0	$ 417.3
GAAP research and development expenses	$ 600.1	$ 448.7	$ 1,201.2	$ 903.0
Stock-based compensation expense	(69.5)	(62.6)	(149.9)	(135.4)
Intangible asset impairment charge (5)	(13.0)	—	(13.0)	—
Acquisition-related costs (6)	(2.8)	(2.8)	(5.6)	(5.6)
Non-GAAP research and development expenses *	$ 514.8	$ 383.3	$ 1,032.7	$ 762.0
Acquired in-process research and development expenses *	$ 61.9	$ 958.4	$ 63.9	$ 960.1
GAAP selling, general and administrative expenses	$ 215.3	$ 194.6	$ 430.5	$ 386.7
Stock-based compensation expense	(42.0)	(40.4)	(89.7)	(81.4)
Non-GAAP selling, general and administrative expenses	$ 173.3	$ 154.2	$ 340.8	$ 305.3
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses *	$ 750.0	$ 1,495.9	$ 1,437.4	$ 2,027.4
GAAP other (expense) income, net	$ (78.1)	$ 8.1	$ (150.9)	$ (44.6)
Decrease (increase) in fair value of strategic investments (4)	84.2	(10.6)	159.8	41.7
Non-GAAP other income (expense), net	$ 6.1	$ (2.5)	$ 8.9	$ (2.9)
GAAP provision for (benefit from) income taxes	$ 213.9	$ (111.2)	$ 406.6	$ 56.6
Tax adjustments (1) *	44.7	122.1	100.9	160.3
Non-GAAP provision for income taxes *	$ 258.6	$ 10.9	$ 507.5	$ 216.9
GAAP effective tax rate	21%	251%	21%	7%
Non-GAAP effective tax rate	22%	20%	22%	21%

*Starting in the first quarter of 2022, Vertex no longer excludes research and development charges resulting from upfront or contingent milestone payments in connection with collaborations, asset acquisitions and/or licensing of third-party intellectual property rights from its Non-GAAP financial measures. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations. Non-GAAP financial measures for the three and six months ended June 30, 2021 have been recast to reflect this change.

Vertex Pharmaceuticals Incorporated
Condensed Consolidated Balance Sheets
(in millions)
(unaudited)

	June 30, 2022	**December 31, 2021**
Assets		
Cash, cash equivalents and marketable securities	$ 9,253.4	$ 7,524.9
Accounts receivable, net	1,332.9	1,136.8
Inventories	367.7	353.1
Property and equipment, net	1,100.1	1,094.1
Goodwill and intangible assets	1,389.2	1,402.2
Deferred tax assets	1,143.8	934.5
Other assets	995.1	986.9

Total assets	$ 15,582.2	$ 13,432.5
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 2,317.5	$ 1,873.6
Finance lease liabilities	531.6	556.7
Contingent consideration	129.8	186.5
Other liabilities	669.8	715.7
Shareholders' equity	11,933.5	10,100.0
Total liabilities and shareholders' equity	$ 15,582.2	$ 13,432.5
Common shares outstanding	256.0	254.5

Notes and Explanations

1: In the three and six months ended June 30, 2022 and 2021, "Tax adjustments" included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income (loss) and excess tax benefits related to stock-based compensation. "Tax adjustments" in the three and six months ended June 30, 2021 also included a $100 million discrete tax 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.

2: The difference between the company's full year 2022 combined GAAP R&D, Acquired IPR&D and SG&A expenses and combined non-GAAP R&D, Acquired IPR&D and SG&A expenses guidance relates primarily to $445 million to $500 million of stock-based compensation expense. The guidance regarding combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expenses does not include estimates associated with any potential future business development transactions, including collaborations, asset acquisitions and/or licensing of third-party intellectual property rights.

3: Vertex classifies upfront, contingent milestone, and other payments pursuant to its business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions as "Acquired in-process research and development expenses" in its consolidated statements of operations. These amounts were previously classified as "Research and development expenses." To conform prior periods to current presentation, the company reclassified $958 million and $960 million from "Research and development expenses" to "Acquired in-process research and development expenses" for the three and six months ended June 30, 2021, respectively. In the three and six months ended June 30, 2021, "Acquired in-process research and development expenses" primarily related to the $900 million upfront payment to CRISPR.

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

5: In June 2022, the company revised the scope of certain acquired programs, resulting in a $13 million "Intangible asset impairment charge" and a decrease in the associated fair value of contingent consideration.

6: "Acquisition-related costs" in the three and six months ended June 30, 2022 and 2021 related to costs associated with the company's acquisition of Exonics.

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, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

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 and one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies. 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, as amended, 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 discussions and filings, data availability, and timing thereof, (ii) the expectations, development plans and anticipated timelines for the company's products and product candidates and pipeline programs, including study designs, patient enrollment, data availability and timing thereof, (iii) expectations for continued growth in the number of CF patients treated with our medicines, including the number of children newly eligible for TRIKAFTA, uptake of and expanded access to the company's medicines, additional reimbursement agreements, new approvals, including market authorizations and label extensions outside of the U.S., and expansion of treatment options for the patients who cannot benefit from CFTR modulators alone, (iv) expectations regarding our collaboration with Moderna to develop CF mRNA therapeutics, including our plans to submit an IND for this program in 2022, (v) expectations regarding two additional Phase 3 studies of exa-cel in pediatric patients and anticipated regulatory filings for exa-cel, (vi) our plans regarding our Phase 2/3 study of inaxaplin in AMKD, and our beliefs regarding anticipated results of the study and the possibility for accelerated approval in the U.S. and Europe, (vii) expectations regarding the potential benefits of our pain program and products, and plans for the advancement of VX-548 into a Phase 3 program in acute pain in the second half of 2022 and to initiate a Phase 2 study in neuropathic pain by the end of 2022, and the possibility for accelerated approval in the U.S., (viii) the potential benefits and safety of VX-880, and our plans to continue to progress the Phase 1/2 program for VX-880, (ix) our plans and expectations regarding our additional programs in T1D, including the completion of IND-enabling studies for the encapsulated islet cell program and anticipated regulatory filings in 2022, (x) plans to advance one or more novel small molecule Z-AAT correctors into the clinic in 2022, (xi) our plans regarding our DMD program, and (xii) our investments and expectations in external innovation, including collaborations and acquisitions. 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 external factors 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 (SEC) 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 at 4:30 p.m. ET. To access the call, please dial (877) 270-2148 (U.S.) or +1 (412) 902-6510 (International) and reference the "Vertex Pharmaceuticals Second Quarter 2022 Earnings Call".

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. To ensure a timely connection, it is recommended that participants 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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