UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) August 1, 2023

Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts	**000-19319**	**04-3039129**
(State or other jurisdiction of incorporation)	*(Commission File Number)*	*(I.R.S. Employer Identification No.)*

50 Northern Avenue
Boston, Massachusetts 02210
(Address of principal executive offices) (Zip Code)

(617) 341-6100
(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.01 Par Value Per Share	VRTX	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02. Results of Operations and Financial Condition.

On August 1, 2023, we issued a press release in which we reported our consolidated financial results for the three and six months ended June 30, 2023. A copy of that press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The information set forth in Exhibit 99.1 shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit	Description of Document
99.1	Press Release Dated August 1, 2023.
104	Cover Page Interactive Data File — the cover page XBRL tags are embedded within the Inline XBRL document.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

VERTEX PHARMACEUTICALS INCORPORATED
(Registrant)

Date: August 1, 2023

/s/ Jonathan Biller
Jonathan Biller
Executive Vice President, Chief Legal Officer

Vertex Reports Second Quarter 2023 Financial Results

— Product revenue of $2.49 billion, a 14% increase compared to Q2 2022 —

— Company raises full year 2023 product revenue guidance to $9.7 to $9.8 billion —

— FDA has accepted the exa-cel BLAs in both severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT); Priority Review granted for SCD with PDUFA date of December 8, 2023 —

— Pipeline continues to advance with multiple near-term clinical milestones —

BOSTON -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2023 and updated its full year 2023 financial guidance.

“The second quarter of 2023 marked another period of strong progress across our business. We are reaching more patients globally with our cystic fibrosis medicines, advancing our late-stage clinical programs and making rapid progress across our research and development pipeline of transformative medicines,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “In the second half of the year, we look forward to expanding our leadership in CF; continuing to prepare for several near-term potential launches, starting with exa-cel; and completing major Phase 3 trials including VX-548 in acute pain and the vanzacaftor triple in cystic fibrosis.”

Second Quarter 2023 Results

Product revenue increased 14% to $2.49 billion compared to the second quarter of 2022, primarily driven by the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued performance of TRIKAFTA in the U.S., including the launch in children with CF 2 to 5 years of age. Net product revenue in the second quarter of 2023 increased 7% to $1.51 billion in the U.S. and increased 26% to $985 million outside the U.S., compared to the second quarter of 2022.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses were $1.2 billion and $1.0 billion, respectively, compared to $877 million and $750 million, respectively, in the second quarter of 2022. The increases were due to increased investment in support of multiple programs that have advanced in mid- and late-stage clinical development, increased acquired IPR&D expenses, and the costs to support launches of Vertex's therapies globally.

GAAP effective tax rate was 21.2% compared to 20.9% for the second quarter of 2022.

Non-GAAP effective tax rate was 21.0% compared to 21.8% for the second quarter of 2022. Please refer to Note 1 for further details on our GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net income increased by 13% and 9%, respectively, compared to the second quarter of 2022, primarily driven by strong revenue growth and increased interest income partially offset by increased investment in our mid- and late-stage clinical pipeline, increased acquired IPR&D expenses, and the costs to support launches of Vertex's therapies globally.

Cash, cash equivalents and total marketable securities as of June 30, 2023 were $12.6 billion, compared to $10.9 billion as of December 31, 2022. The increase was primarily driven by strong revenue growth and operating cash flow, partially offset by our payments to Entrada Therapeutics, CRISPR Therapeutics and other collaboration partners, repurchases of our common stock pursuant to our share repurchase program, and income tax payments.

Full Year 2023 Financial Guidance

Vertex is raising its full year 2023 CF product revenue guidance to $9.7 to $9.8 billion, from $9.55 to $9.7 billion previously. The increase reflects the expected full-year impact of the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued performance of TRIKAFTA in the U.S. This guidance includes an approximately 150-basis-point negative impact from changes in foreign currency rates, inclusive of our foreign exchange risk management program. Vertex is also increasing full year 2023 combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expense guidance. The increase accounts for higher acquired IPR&D expenses incurred year-to-date, including a $70 million milestone payment to CRISPR Therapeutics.

Vertex's financial guidance is summarized below:

	Current FY 2023	Previous FY 2023
CF product revenues	$9.7 to $9.8 billion	$9.55 to $9.7 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	$4.55 to $4.8 billion	$4.35 to $4.6 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2)	$4.1 to $4.2 billion	$3.9 to $4.0 billion
Non-GAAP effective tax rate	Unchanged	21% to 22%

Key Business Highlights

Cystic Fibrosis (CF) Marketed Products

Vertex anticipates the number of CF patients taking our medicines will continue to grow, including through new approvals and reimbursement for the treatment of younger patients. Recent progress includes:

- Approval from the European Commission for the use of ORKAMBI in children with CF ages 1 to <2 years old who have two copies of the F508del mutation in the CFTR gene. With this approval, approximately 300 children with CF are eligible for the first time for a medicine that can treat the underlying cause of their disease.
- At the European Cystic Fibrosis Society's (ECFS) European Cystic Fibrosis Conference in June, Vertex presented interim results from the largest real-world study of TRIKAFTA/KAFTRIO, which showed sustained improvement in lung function, reduction in pulmonary exacerbations frequency and lower rates of lung transplant and death for people with CF.
- Approval from the U.S. Food and Drug Administration (FDA) for the use of KALYDECO in children with CF from 1 month to <4 months of age. This approval represents the first and only CFTR modulator approved for this age group. Vertex also submitted Marketing Authorization Applications (MAAs) to the European Medicines Agency (EMA), the Medicines and Healthcare products Regulatory Agency (MHRA) in the United Kingdom, and Health Canada for the use of KALYDECO in children with CF from 1 month to <4 months of age.
- Approval from the FDA for the use of TRIKAFTA in children 2 to 5 years of age with at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene or a mutation in the CFTR gene that is responsive to TRIKAFTA. With this approval, approximately 900 children are eligible for TRIKAFTA for the first time. Vertex also completed regulatory submissions with the EMA, the MHRA, Health Canada, and the Therapeutic Goods Administration in Australia for the use of KAFTRIO/TRIKAFTA in children 2 to 5 years of age.

Potential Near-Term Launch Opportunities

Vertex is preparing for the following near-term potential new product launches:

- ***Exagamglogene autotemcel (exa-cel) in SCD and TDT:*** *Exa-cel is a precise non-viral* ex vivo *CRISPR gene-editing therapy, which is being developed in collaboration with CRISPR Therapeutics as a potential functional cure for SCD and TDT.*
    - The FDA accepted the Biologics License Applications (BLAs) for exa-cel and assigned Prescription Drug User Fee Act (PDUFA) action dates of December 8, 2023, for SCD and March 30, 2024, for TDT. Exa-cel's BLA for SCD was granted Priority Review by the

FDA. The FDA has indicated that they plan to hold an advisory committee meeting for exa-cel. In the U.S., exa-cel has been granted Fast Track, Regenerative Medicine Advanced Therapy (RMAT), Orphan Drug and Rare Pediatric Disease designations.

  - As with the U.S. FDA, reviews of the regulatory filings for exa-cel with the EMA in the EU and the MHRA in the U.K. are well underway. Exa-cel has been granted Priority Medicines (PRIME) and Orphan Drug designation in the EU. In the U.K., exa-cel has been granted an Innovation Passport under the Innovative Licensing and Access Pathway from the MHRA.
  - At the 2023 Annual European Hematology Association (EHA) Congress in June, Vertex presented positive interim results from the pivotal trials of exa-cel in SCD and TDT as of a September 2022 data cut. Both trials met the primary and key secondary endpoints at pre-specified interim analyses, and the data continue to demonstrate transformative, consistent and durable benefit. The data presented at EHA were the basis of the EMA and MHRA regulatory filings for exa-cel. Vertex expects to present updated clinical data that served as the basis of the FDA filing at a future medical congress.
  - Dosing in the Phase 1/2/3 CLIMB-111 and CLIMB-121 studies is ongoing, and Vertex continues to enroll and follow patients in the CLIMB-131 long-term follow-up study.

- ***Vanzacaftor/tezacaftor/deutivacaftor, the next-in-class triple combination, in cystic fibrosis***.
  - In the fourth quarter of 2022, Vertex completed enrollment in the pivotal SKYLINE 102 and SKYLINE 103 trials, which evaluate the efficacy and safety of vanzacaftor/tezacaftor/deutivacaftor relative to TRIKAFTA in patients with CF 12 years of age and older. More recently, Vertex also completed enrollment in the RIDGELINE study of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age. Vertex expects to complete both the SKYLINE and RIDGELINE studies by the end of 2023 and share the results of these studies in early 2024.

- ***VX-548 in acute pain:*** *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 currently available medicines.*
  - Vertex continues to enroll the Phase 3 pivotal program, including two randomized controlled trials in abdominoplasty and bunionectomy and a single arm safety and

effectiveness trial, for its lead compound, VX-548, for the treatment of moderate to severe acute pain.

- Vertex expects to complete the pivotal program in late 2023 and share results from these studies in late 2023 or early 2024. In the U.S., VX-548 has been granted Breakthrough Therapy and Fast Track designations for moderate to severe acute pain.

R&D Pipeline

Vertex is delivering on a diversified pipeline of potentially transformative small molecule, mRNA, cell and genetic therapies aimed at serious diseases. Recent and anticipated progress for programs in clinical development is summarized below.

Cystic Fibrosis

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

- Vertex is developing VX-522, a CFTR mRNA therapeutic, in collaboration with Moderna. The goal of this therapy is to treat the underlying cause of CF by programming cells in the lungs to produce functional CFTR protein, and it is aimed at the treatment of the approximately 5,000 people with CF who do not produce any CFTR protein. Vertex is enrolling patients in a single ascending dose (SAD) clinical trial for VX-522, and the Company expects to complete the SAD and initiate a multiple ascending dose (MAD) study in 2023. In the U.S., the FDA has granted Fast Track designation to VX-522.
- Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs and is advancing additional CFTR modulators with the goal of bringing more patients to carrier levels of sweat chloride.

Beta Thalassemia and Sickle Cell Disease

- Two global Phase 3 studies of exa-cel continue to enroll and dose patients 5 to 11 years of age with TDT or SCD.
- Additionally, Vertex continues to work on preclinical assets for gentler conditioning for exa-cel, which could broaden the eligible patient population from 32,000 patients to more than 150,000.

Acute and Neuropathic Pain

- The Phase 2 dose-ranging study of VX-548 in patients with diabetic peripheral neuropathy, a common form of peripheral neuropathic pain, has been fully enrolled.

- Dosing in the Phase 2, 12-week VX-548 study continues, and Vertex expects to complete this study in late 2023 and share results in late 2023 or early 2024.
- Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs and is advancing additional NaV1.8 inhibitors as well as NaV1.7 inhibitors through research and earlier stages of development for pain.

APOL1-Mediated Kidney Disease (AMKD)

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

- Vertex continues to enroll and dose patients in the pivotal program for inaxaplin, a single Phase 2/3 clinical trial in patients with AMKD, and expects to complete the Phase 2B dose-ranging portion of the study in 2023.
- Inaxaplin was granted Breakthrough Therapy designation by the FDA for FSGS, as well as Orphan Drug and PRIME designations by the EMA for AMKD.

Type 1 Diabetes (T1D)

Vertex is evaluating cell therapies using stem-cell derived, fully differentiated, insulin-producing islet cells 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. Vertex has three programs that use these fully differentiated cells.

1. *VX-880, fully differentiated cells with standard immunosuppression:* Vertex established proof-of-concept for VX-880 in 2022. In June 2023, Vertex presented positive, updated clinical data from the ongoing VX-880 Phase 1/2 study at the American Diabetes Association Scientific Sessions (ADA). The Phase 1/2 study is designed as a sequential, multi-part clinical trial to evaluate the safety and efficacy of VX-880. In Part A, the first two patients received half the target dose of VX-880 cells. In Part B, patients received the full target dose with staggered dosing. Based on the results from Parts A and B, Vertex has initiated Part C of the study, with concurrent dosing at the full target dose, with trial sites currently active in the U.S., Canada, Norway, Switzerland, the Netherlands and France.

   In the data presented at ADA, all patients from Parts A and B of the study treated with VX-880 engrafted islet cells, produced endogenous insulin (C-peptide) and had improved glycemic control while reducing or eliminating insulin use. The two patients with at least one year of follow-up met the criteria for the primary endpoint of elimination of severe hypoglycemic events (SHEs) and HbA1C <7.0 and also achieved insulin independence.

2. *VX-264, fully differentiated islet cells encapsulated in immunoprotective device:* VX-264 uses the same stem cell-derived, fully differentiated islets used in the VX-880 program, which are encapsulated in a novel device designed to shield the cells from the body's immune system and obviate the need for immunosuppressive therapy. Vertex is enrolling and dosing patients with VX-264 in a Phase 1/2 clinical trial that is a sequential, multi-part study to evaluate the safety, tolerability and efficacy of VX-264. Part A of the study will dose patients with a partial dose of cells and a stagger between patients, and Part B will dose patients with a full target dose and a stagger between patients before moving to concurrent dosing in Part C. The study is enrolling patients in the U.S., Canada and the Netherlands, with additional global sites to be activated in the coming months. The first patient in Part A has been dosed.

3. *Edited fully differentiated cells:* Vertex's hypoimmune cell program involves using CRISPR/Cas9 to gene edit the same stem cell-derived, fully differentiated islets used in the VX-880 and VX-264 programs, in order to cloak the cells from the immune system. This is yet another possible path to eliminate the need for immunosuppressive therapy. This program is progressing through the research stage.

To further expand Vertex's capabilities in cell therapy manufacturing, in June 2023, Vertex and Lonza announced a strategic agreement to support the manufacture of Vertex's portfolio of investigational stem cell-derived, fully differentiated insulin-producing islet cell therapy. This agreement will help accelerate the development and commercialization of Vertex's potentially transformative cell therapy products for T1D.

Alpha-1 Antitrypsin 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 a 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 enrolling and dosing patients in a 48-week Phase 2 study of VX-864, a first-generation AAT corrector, to assess the impact of longer-term treatment on polymer clearance from the liver, as well as the resultant levels of functional AAT (fAAT) in the plasma. Vertex expects to complete enrollment in this study in 2023.

- Additionally, Vertex continues to enroll and dose healthy volunteers with VX-634, a follow-on small molecule AAT corrector. VX-634 is the first in a series of next-wave investigational molecules with significantly improved potency and drug-like properties compared to previous Vertex AAT correctors. Vertex expects to complete enrollment and dosing in this study in 2023.

Muscular Dystrophies

Vertex is also advancing preclinical assets in muscular dystrophies, including Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1).

Investments in External Innovation

As part of the collaboration with CRISPR Therapeutics on hypoimmune cells for T1D, Vertex achieved a research milestone in the second quarter of 2023, resulting in a $70 million milestone payment payable to CRISPR.

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. Unless otherwise noted, 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 Income
(in millions, except per share amounts)(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Product revenues, net	$ 2,493.2	$ 2,196.2	$ 4,868.0	$ 4,293.7
Costs and expenses:				
Cost of sales	308.6	261.8	575.5	507.6
Research and development expenses	785.7	600.1	1,528.3	1,201.2
Acquired in-process research and development expenses	110.5	61.9	457.6	63.9
Selling, general and administrative expenses	262.6	215.3	503.7	430.5
Change in fair value of contingent consideration	(0.6)	(49.2)	(2.5)	(56.7)
Total costs and expenses	1,466.8	1,089.9	3,062.6	2,146.5
Income from operations	1,026.4	1,106.3	1,805.4	2,147.2
Interest income	144.7	10.8	267.3	12.4
Interest expense	(11.2)	(14.6)	(22.6)	(29.5)
Other income (expense), net	1.6	(78.1)	2.9	(150.9)
Income before provision for income taxes	1,161.5	1,024.4	2,053.0	1,979.2
Provision for income taxes	245.8	213.9	437.5	406.6
Net income	$ 915.7	$ 810.5	$ 1,615.5	$ 1,572.6
Net income per common share:				
Basic	$ 3.55	$ 3.17	$ 6.27	$ 6.15
Diluted	$ 3.52	$ 3.13	$ 6.21	$ 6.09
Shares used in per share calculations:				
Basic	257.7	255.9	257.6	255.5
Diluted	260.4	258.7	260.3	258.3

Vertex Pharmaceuticals Incorporated
Product Revenues
(in millions)(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
TRIKAFTA/KAFTRIO	$ 2,240.4	$ 1,893.2	$ 4,337.1	$ 3,654.8
Other CF products	252.8	303.0	530.9	638.9
Product revenues, net	$ 2,493.2	$ 2,196.2	$ 4,868.0	$ 4,293.7

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
GAAP cost of sales	$ 308.6	$ 261.8	$ 575.5	$ 507.6
Stock-based compensation expense	(1.8)	(2.4)	(3.7)	(4.6)
Non-GAAP cost of sales	$ 306.8	$ 259.4	$ 571.8	$ 503.0
GAAP research and development expenses	$ 785.7	$ 600.1	$ 1,528.3	$ 1,201.2
Stock-based compensation expense	(74.5)	(69.5)	(150.8)	(149.9)
Intangible asset impairment charge (3)	—	(13.0)	—	(13.0)
Acquisition-related costs (4)	(2.8)	(2.8)	(5.6)	(5.6)
Non-GAAP research and development expenses	$ 708.4	$ 514.8	$ 1,371.9	$ 1,032.7
Acquired in-process research and development expenses	$ 110.5	$ 61.9	$ 457.6	$ 63.9
GAAP selling, general and administrative expenses	$ 262.6	$ 215.3	$ 503.7	$ 430.5
Stock-based compensation expense	(43.0)	(42.0)	(87.2)	(89.7)
Non-GAAP selling, general and administrative expenses	$ 219.6	$ 173.3	$ 416.5	$ 340.8
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses	$ 1,038.5	$ 750.0	$ 2,246.0	$ 1,437.4
GAAP other income (expense), net	$ 1.6	$ (78.1)	$ 2.9	$ (150.9)
Decrease (increase) in fair value of strategic investments	0.4	84.2	(6.0)	159.8
Non-GAAP other income (expense), net	$ 2.0	$ 6.1	$ (3.1)	$ 8.9
GAAP provision for income taxes	$ 245.8	$ 213.9	$ 437.5	$ 406.6
Tax adjustments (1)	23.6	44.7	46.3	100.9
Non-GAAP provision for income taxes	$ 269.4	$ 258.6	$ 483.8	$ 507.5
GAAP effective tax rate	21.2 %	20.9 %	21.3 %	20.5 %
Non-GAAP effective tax rate	21.0 %	21.8 %	21.1 %	21.6 %

Vertex Pharmaceuticals Incorporated
Reconciliation of GAAP to Non-GAAP Financial Information (continued)
(in millions, except per share amounts)(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
GAAP operating income	$ 1,026.4	$ 1,106.3	$ 1,805.4	$ 2,147.2
Stock-based compensation expense	119.3	113.9	241.7	244.2
Decrease in fair value of contingent consideration (3)	(0.6)	(49.2)	(2.5)	(56.7)
Intangible asset impairment charge (3)	—	13.0	—	13.0
Acquisition-related costs (4)	2.8	2.8	5.6	5.6
Non-GAAP operating income	$ 1,147.9	$ 1,186.8	$ 2,050.2	$ 2,353.3

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	**2022**	**2023**	**2022**
GAAP net income	$ 915.7	$ 810.5	$ 1,615.5	$ 1,572.6
Stock-based compensation expense	119.3	113.9	241.7	244.2
Decrease (increase) in fair value of strategic investments	0.4	84.2	(6.0)	159.8
Decrease in fair value of contingent consideration (3)	(0.6)	(49.2)	(2.5)	(56.7)
Intangible asset impairment charge (3)	—	13.0	—	13.0
Acquisition-related costs (4)	2.8	2.8	5.6	5.6
Total non-GAAP adjustments to pre-tax income	121.9	164.7	238.8	365.9
Tax adjustments (1)	(23.6)	(44.7)	(46.3)	(100.9)
Non-GAAP net income	$ 1,014.0	$ 930.5	$ 1,808.0	$ 1,837.6
Net income per diluted common share:				
GAAP	$ 3.52	$ 3.13	$ 6.21	$ 6.09
Non-GAAP	$ 3.89	$ 3.60	$ 6.95	$ 7.11
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	260.4	258.7	260.3	258.3

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

	June 30, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 11,236.3	$ 10,778.5
Accounts receivable, net	1,556.2	1,442.2
Inventories	603.5	460.6
Prepaid expenses and other current assets	476.9	553.5
Total current assets	13,872.9	13,234.8
Property and equipment, net	1,122.4	1,108.4
Goodwill and intangible assets	1,691.6	1,691.6
Deferred tax assets	1,538.0	1,246.9
Operating lease assets	324.3	347.4
Long-term marketable securities	1,357.3	112.2
Other long-term assets	442.7	409.6
Total assets	$ 20,349.2	$ 18,150.9
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 2,961.1	$ 2,430.6
Other current liabilities	391.0	311.5
Total current liabilities	3,352.1	2,742.1
Long-term finance lease liabilities	404.1	430.8
Long-term operating lease liabilities	363.5	379.5
Other long-term liabilities	759.3	685.8
Shareholders' equity	15,470.2	13,912.7
Total liabilities and shareholders' equity	$ 20,349.2	$ 18,150.9
Common shares outstanding	257.8	257.0

Notes and Explanations

1: In the three and six months ended June 30, 2023 and 2022, "Tax adjustments" included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income and excess tax benefits related to stock-based compensation.

2: The difference between the company's full year 2023 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 $475 million to $590 million of stock-based compensation expense. Unless otherwise noted, 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: In the three months ended June 30, 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.

4: "Acquisition-related costs" in the three and six months ended June 30, 2023 and 2022 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 clinical pipeline of investigational small molecule, mRNA, cell and genetic therapies (including gene editing) in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

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 13 consecutive years on Science magazine's Top Employers list and one of Fortune’s 100 Best Companies to Work For. 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 2023 Financial Guidance" and statements regarding (i) expectations for continued growth in the number of people eligible and treated with our CF medicines and expansion of treatment options for the patients who cannot benefit from CFTR modulators alone, (ii) the expectations, development plans and anticipated timelines for the company's products and product candidates and pipeline programs, including expectations for multiple additional near-term clinical milestones, study designs, patient enrollment, data availability, potential launches and timing thereof, (iii) the expectations, plans, and status of potential near-term product commercial launches, including those for exa-cel in SCD and TDT, vanzacaftor/tezacaftor/deutivacaftor in CF, and VX-548 in moderate to severe acute pain, (iv) the expectations related to our exa-cel regulatory filings, including the FDA’s plans to hold an advisory committee for exa-cel, our plans to present updated exa-cel clinical data at a future medical congress, expectations regarding the potential benefits of exa-cel as a functional cure for SCD and TDT, and our expectation that a gentler conditioning for exa-cel could broaden the eligible patient population for exa-cel, (v) expectations regarding our collaboration with Moderna to develop CFTR mRNA therapeutics, and plans to complete the single-ascending dose study and initiate the multiple-ascending dose study for VX-522 in 2023, (vi) expectations to complete both the SKYLINE and RIDGELINE studies by the end of 2023 and share the results of these studies in early 2024, (vii) expectations regarding the potential benefits and objectives of our pain program and products, expectations to complete the VX-548 pivotal program for the treatment of moderate to severe acute pain in late 2023 and share results from these studies in late 2023 or early 2024, and expectations to complete the VX-548 study in patients with diabetic peripheral neuropathy in late 2023 and share results in late 2023 or early 2024, (viii) expectations regarding the potential benefits of our AMKD program, and plans regarding our Phase 2/3 study of inaxaplin, including expectations to complete the Phase 2B dose-ranging portion of the study in 2023, (ix) expectations for the advancement of our T1D programs, including clinical trial designs and activation of additional global clinical sites, and expectations that the strategic agreement with Lonza will help accelerate the development and commercialization of our cell therapy products for T1D, (x) our expectations regarding

our goals and the potential benefits of our AAT deficiency program, plans to continue to advance VX-864 and VX-634 in clinical trials, including expectations to complete enrollment in the VX-864 study in 2023 and to complete enrollment and dosing in the VX-634 study in 2023, (xi) plans with respect to our additional earlier stage research and development programs, including preclinical assets in new disease areas such as DMD and DM1, and assets targeting gentler conditioning for exa-cel and NaV1.7 in pain, and (xii) expectations with respect to our investments in external innovation. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2023 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 receive regulatory approval for exa-cel 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 patient enrollment in our trials may be delayed, 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, that anticipated commercial launches may be delayed, if they occur at all, 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 (833) 630-2124 (U.S.) or +1(412) 317-0651 (International) and reference the “Vertex Pharmaceuticals Second Quarter 2023 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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