Vertex Reports Fourth Quarter and Full Year 2023 Financial Results

February 5, 2024

— Full year product revenue of $9.87 billion, an 11% increase compared to full year 2022 —

— Company provides full year 2024 product revenue guidance of $10.55 to $10.75 billion —

— CASGEVY™ approved in the U.S., Great Britain, the Kingdom of Saudi Arabia and Bahrain —

— Vertex on track to submit new drug applications (NDAs) to the FDA by mid-2024 for both VX-548 in Acute Pain and the Vanzacaftor Triple in CF —

— Broad and deep clinical-stage pipeline continues to advance across 10 disease areas —

BOSTON--(BUSINESS WIRE)--Feb. 5, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the fourth quarter and full year ended December 31, 2023 and provided full year 2024 financial guidance.

“2023 was a transformative year for Vertex as we continued our strong performance, including 11% revenue growth, combined with significant advancement across the business. We expanded our leadership in CF, diversified our commercial opportunity with CASGEVY regulatory approvals in multiple regions, and rapidly advanced a broad pipeline with multiple additional near-term potential launch opportunities in disease areas outside of CF,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “Our progress in 2023 lays the foundation for the anticipated regulatory submissions for the vanzacaftor triple and VX-548 by mid-2024 and sets us on a path to expand our business in CF and beyond, beginning with the commercialization of CASGEVY in multiple geographies.”

Fourth Quarter 2023 Results

Product revenue increased 9% to $2.52 billion compared to the fourth quarter of 2022, primarily driven in the U.S. by the continued performance of TRIKAFTA, including the uptake in children with CF 2 to 5 years of age, and in ex-U.S. markets by the continued strong uptake of TRIKAFTA/KAFTRIO, including label extensions in younger age groups. Net product revenue in the fourth quarter of 2023 increased 8% to $1.57 billion in the U.S. and increased 12% to $943 million outside the U.S., compared to the fourth 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 $984 million and $872 million, respectively, in the fourth 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 and the costs to support launches of Vertex's therapies globally. Combined GAAP R&D, Acquired IPR&D and SG&A expenses also included increased stock-based compensation expense compared to the fourth quarter of 2022.

GAAP effective tax rate was 15.6% compared to 24.0% for the fourth quarter of 2022 based on increased benefits from R&D tax credits and a lower provision from uncertain tax positions.

Non-GAAP effective tax rate was 16.3% compared to 18.5% for the fourth quarter of 2022 as a result of increased benefits from R&D tax credits for the current year. Please refer to Note 1 for further details on our GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net income increased by 18% and 12%, respectively, compared to the fourth quarter of 2022, primarily due to higher interest income and lower taxes.

Full Year 2023 Results

Product revenue increased 11% to $9.87 billion compared to 2022, primarily driven by the continued strong uptake of TRIKAFTA/KAFTRIO in ex-U.S. markets, including label extensions in younger age groups, and the continued performance of TRIKAFTA in the U.S, including the ongoing launch in children with CF 2 to 5 years of age. Net product revenue in 2023 increased 6% to $6.04 billion in the U.S. and increased 18% to $3.83 billion outside the U.S., compared to 2022.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses were $4.83 billion and $4.24 billion, respectively, compared to $3.60 billion and $3.07 billion, respectively, in 2022. The increases were due to increased investment in support of multiple programs that have advanced in mid- and late-stage clinical development, the costs to support launches of Vertex's therapies globally, and increased acquired IPR&D expenses.

GAAP effective tax rate was 17.4% compared to 21.5% in 2022, largely as a result of higher U.S. R&D tax credits for the current and prior years.

Non-GAAP effective tax rate was 19.4% compared to 20.8% in 2022, also largely as a result of higher U.S. R&D tax credits for the current year. Please refer to Note 1 for further details on our GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net income increased by 9% and 3%, respectively, compared to 2022, primarily due to higher interest income and lower taxes.

Cash, cash equivalents and total marketable securities as of December 31, 2023 were $13.7 billion, compared to $10.9 billion as of December 31, 2022. The increase was primarily due to income from operations that was driven by strong revenue growth, and interest income, partially offset by income tax payments and repurchases of our common stock pursuant to our share repurchase program.

Full Year 2024 Financial Guidance

Vertex today provided full year 2024 financial guidance. Vertex's product revenue guidance of $10.55 billion to $10.75 billion includes expectations for continued growth in CF as well as the launch of CASGEVY in approved indications and geographies. Vertex's combined Non-GAAP R&D, Acquired IPR&D and SG&A expense guidance of $4.3 billion to $4.4 billion includes expectations for continued investment in our multiple mid and late-stage clinical development programs, commercial and manufacturing capabilities, and approximately $125 million of upfront and milestone payments.

Vertex's financial guidance is summarized below:

	FY 2024
Total product revenues	$10.55 to $10.75 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	$4.9 to $5.1 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2)	$4.3 to $4.4 billion
Non-GAAP effective tax rate	20% to 21%

Key Business Highlights

Marketed Products

Cystic Fibrosis (CF) Portfolio

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 and anticipated progress includes:

- Updated estimates for the number of patients living with CF from ~88,000 to ~92,000 in the U.S., Europe, Australia, and Canada.
- The European Commission granted approval in the fourth quarter of 2023 for label extension of KAFTRIO in combination with ivacaftor in children with CF 2 to 5 years of age who have at least one F508del mutation in the CFTR gene. With this approval, approximately 1,200 children are newly eligible for a medicine that could treat the underlying cause of their disease. Vertex will continue to work with reimbursement authorities across the European Union to ensure access for all eligible patients.
- In the U.K., the Medicines and Healthcare Products Regulatory Agency (MHRA) approved the use of KAFTRIO in children with CF 2 to 5 years of age who have at least one F508del mutation in the CFTR gene. With this approval, approximately 200 children are newly eligible for a medicine that could treat the underlying cause of their disease. Because of the existing reimbursement agreement between Vertex and the National Health Service, children ages 2 years of age and above in the U.K. already have access to KAFTRIO.
- The European Medicines Agency (EMA) validated the Marketing Authorization Application (MAA) extension for KAFTRIO in combination with ivacaftor to include people with CF who have a rare mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive based on clinical and/or in vitro data, including the N1303K mutation. If approved, approximately 2,800 people with CF in the European Union ages 2 years of age and above would be newly eligible for treatment. Vertex plans to submit regulatory filings for the same mutations in Australia, Brazil, Canada, New Zealand and Switzerland. Vertex also plans to submit a subset of these mutations, including N1303K and non-canonical splice mutations, not currently included in the U.S. TRIKAFTA label to the U.S. FDA.

Sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT): CASGEVY

- Vertex received regulatory approvals for CASGEVY in the U.S., Great Britain, Bahrain, and the Kingdom of Saudi Arabia (KSA) for the treatment of both SCD and TDT.
- Vertex also received a positive opinion for CASGEVY for the treatment of both SCD and TDT from the EMA's Committee for Medicinal Products for Human Use (CHMP).

- The French National Authority for Health (HAS) approved Vertex's request for the implementation of an early access program (EAP) for the use of CASGEVY to treat people with transfusion-dependent beta thalassemia (TDT) from 12-35 years of age for whom hematopoietic stem cell (HSC) transplantation is appropriate and a human leukocyte antigen (HLA)-matched related HSC donor is not available. Vertex is also pursuing an EAP submission for SCD in France and expects to hear the outcome of this decision in the coming months.
- The regulatory submission for CASGEVY in both SCD and TDT is currently under review in Switzerland, with submission in Canada planned for the first half of 2024.
- Vertex updated estimates for the number of patients living with severe SCD, from ~25,000 patients to ~30,000 patients in the U.S. and Europe, with additional patients in KSA and Bahrain. Vertex also updated estimates for patients with TDT, from ~7,000 to ~5,000 patients in the U.S. and Europe, with additional patients in KSA and Bahrain.
- Vertex has activated 12 authorized treatment centers (ATCs) in the U.S. and three in the EU, with the ultimate goal of activating approximately 50 ATCs in the U.S. and 25 in Europe. Vertex has also activated one of two planned ATCs in KSA.
- Vertex recently signed an agreement with Synergie Medication Collective, a medication contracting organization founded by a group of Blue Cross and Blue Shield affiliated companies covering approximately 100 million people in the U.S., to provide access to CASGEVY.
- On January 30th, the Biden Administration, in partnership with Centers for Medicare and Medicaid Services (CMS) and the Department of Health and Human Services (HHS), announced the details of the Center for Medicare and Medicaid Innovation Cell and Gene Therapy (CGT) Access Demonstration Model. The demonstration model is structured as a voluntary negotiation with CMS to devise multi-state outcomes-based arrangements (OBAs) for SCD cell and genetic therapies that state Medicaid programs can participate in beginning in January 2025. In the meantime, Vertex is actively engaging with Medicaid states to secure immediate reimbursement and coverage for CASGEVY and believes the CGT Access Model may provide an additional important tool to help address longstanding inequities in care by facilitating access and funding for potentially curative therapies for the sickle cell community.

Potential Near-Term Launch Opportunities

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

- ***Vanzacaftor/tezacaftor/deutivacaftor, the next-in-class triple oral small molecule combination, in cystic fibrosis***
- In the fourth quarter, Vertex completed three pivotal studies evaluating the efficacy and safety of vanzacaftor/tezacaftor /deutivacaftor ("the vanza triple") relative to TRIKAFTA in people with CF ages 6 years of age and older (ages 12+ in SKYLINE 102 and 103 studies; ages 6-11 in the RIDGELINE study).
- Vertex today shared positive data from the three pivotal studies for the vanza triple, showing that the studies met the primary endpoint and all key secondary endpoints. Based on these data, Vertex intends to submit global regulatory filings by mid-2024 for the vanza triple, including a New Drug Application (NDA) to the U.S. FDA using a priority review voucher and MAAs to the EMA and Health Canada, for people with CF 6 years of age and older.
- ***VX-548 for the treatment of moderate to severe 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 across a variety of pain states, without the limitations of opioids and other currently available medicines.*
- In the fourth quarter of 2023, Vertex completed the pivotal program, which includes three Phase 3 trials: one randomized, controlled trial in abdominoplasty; one randomized, controlled trial in bunionectomy; and a single-arm safety and effectiveness trial.
- Vertex recently shared positive results from the three Phase 3 trials of VX-548 in acute pain. Based on these data, Vertex plans to submit an NDA for the treatment of moderate to severe acute pain to the U.S. FDA by mid-2024. VX-548 has Fast Track and Breakthrough Therapy designations in acute pain.

Select Clinical-Stage R&D Pipeline

Vertex is delivering on a diversified pipeline of potentially transformative medicines for serious diseases utilizing a range of modalities. Recent and anticipated progress for select programs in clinical development is summarized below.

Cystic Fibrosis

Vertex continues to pursue a nebulized mRNA therapy for the more than 5,000 patients who do not make CFTR protein and cannot benefit from CFTR modulators, as well as next-in-class, small molecule, oral CFTR modulators.

- Vertex completed dosing in the single ascending dose (SAD) portion of the Phase 1/2 study of VX-522 in people with CF.
    - Vertex initiated the multiple ascending dose (MAD) portion of the study; screening, enrollment and dosing are underway. Vertex expects to share data from this study in late 2024 or early 2025.

Sickle Cell Disease and Beta Thalassemia

- Vertex completed enrollment in two global Phase 3 studies of CASGEVY in people 5 to 11 years of age with SCD or TDT.
- Additionally, Vertex continues to work on preclinical assets for gentler conditioning for CASGEVY, which could broaden the eligible patient population to more than 150,000 people.

Peripheral Neuropathic Pain (PNP)

- In December 2023, Vertex shared positive Phase 2 results with VX-548 in diabetic peripheral neuropathy, a condition that affects ~2 million Americans.
- Vertex will meet with regulators in the first quarter of 2024 and anticipates advancing VX-548 into pivotal development thereafter.
- Vertex initiated a Phase 2 study of VX-548 in lumbosacral radiculopathy (LSR), another type of peripheral neuropathic pain and the largest patient segment (over 40%) within the PNP category with high unmet need and no approved therapies. Screening, enrollment and dosing are underway.
- Vertex anticipates initiating a Phase 2 study with an oral formulation of VX-993, a next-generation selective NaV1.8 inhibitor, for the treatment of PNP in 2024.

Acute Pain

- Vertex also anticipates initiating a Phase 2 study with an oral formulation of VX-993, a next-generation NaV1.8 inhibitor, for the treatment of moderate to severe acute pain in of 2024.
- Vertex anticipates completing IND-enabling studies and filing an IND for an intravenous formulation of VX-993 in 2024.
- Consistent with its commitment to serial innovation and leadership in pain, Vertex also continues to develop NaV1.7 inhibitors, both for stand-alone use and in combination with NaV1.8 inhibitors, for both acute and peripheral neuropathic pain.

APOL1-Mediated Kidney Disease (AMKD)

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

- Vertex completed enrollment in the Phase 2B dose-ranging portion of the pivotal program for inaxaplin, a single Phase 2/3 clinical trial in patients with AMKD.
- Vertex expects to select a dose for the Phase 3 portion and begin Phase 3 in the first quarter of 2024.

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 one-time functional cure for this disease.

- *VX-880, fully differentiated islet cells with standard immunosuppression:*
  - Completed enrollment in Parts A, B, and C of the Phase 1/2 study of VX-880, an allogeneic, stem cell-derived, fully differentiated, insulin-producing islet cell therapy, used in conjunction with standard immunosuppression, in people with T1D and impaired awareness of hypoglycemia and recurrent hypoglycemic events.
  - Vertex has placed the study on a protocol-specified pause, pending review of the totality of the data by the independent data monitoring committee and global regulators.
- *VX-264, fully differentiated islet cells encapsulated in an immunoprotective device:*
  - The clinical trial for VX-264, which encapsulates the same VX-880 cells in a novel device designed to eliminate the need for immunosuppressants, is a multi-part, Phase 1/2 study.
  - Vertex has completed Part A of the study.
  - Per protocol, the Independent Data Monitoring Committee reviewed the totality of the data from the patients dosed in Part A of the study and recommended advancement to Part B of the study, which has been initiated in multiple centers and countries.
- *Hypoimmune, edited fully differentiated islet 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 to cloak the cells from the immune system. This program is progressing through the research stage.

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) intangible asset amortization expense, (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, (vi) an intangible asset impairment charge and (vii) 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 December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**
Product revenues, net	$ 2,517.7	$ 2,302.7	$ 9,869.2	$ 8,930.7
Costs and expenses:				
Cost of sales	368.0	283.3	1,262.2	1,080.3
Research and development expenses	824.6	694.1	3,162.9	2,540.3
Acquired in-process research and development expenses	17.8	22.6	527.1	115.5
Selling, general and administrative expenses	369.1	267.4	1,136.6	944.7
Change in fair value of contingent consideration	(50.3)	1.8	(51.6)	(57.5)
Total costs and expenses	1,529.2	1,269.2	6,037.2	4,623.3
Income from operations	988.5	1,033.5	3,832.0	4,307.4
Interest income	179.5	86.0	614.7	144.6
Interest expense	(10.6)	(11.6)	(44.1)	(54.8)
Other expense, net	(9.8)	(31.1)	(22.8)	(164.8)
Income before provision for income taxes	1,147.6	1,076.8	4,379.8	4,232.4
Provision for income taxes	178.8	257.9	760.2	910.4
Net income	$ 968.8	$ 818.9	$ 3,619.6	$ 3,322.0
Net income per common share:				
Basic	$ 3.76	$ 3.19	$ 14.05	$ 12.97
Diluted	$ 3.71	$ 3.15	$ 13.89	$ 12.82
Shares used in per share calculations:				
Basic	257.7	256.9	257.7	256.1
Diluted	260.9	260.3	260.5	259.1

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**
TRIKAFTA/KAFTRIO	$ 2,333.3	$ 2,021.5	$ 8,944.7	$ 7,686.8
Other CF products	184.4	281.2	924.5	1,243.9
Product revenues, net	$ 2,517.7	$ 2,302.7	$ 9,869.2	$ 8,930.7

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**

GAAP cost of sales	$ 368.0	$ 283.3	$ 1,262.2	$ 1,080.3
Stock-based compensation expense	(2.1)	(2.4)	(7.5)	(9.4)
Intangible asset amortization expense	(1.7)	—	(1.7)	—
Non-GAAP cost of sales	$ 364.2	$ 280.9	$ 1,253.0	$ 1,070.9
GAAP research and development expenses	$ 824.6	$ 694.1	$ 3,162.9	$ 2,540.3
Stock-based compensation expense	(123.0)	(68.0)	(354.9)	(297.9)
Intangible asset impairment charge (3)	—	—	—	(13.0)
Acquisition-related costs (4)	(2.8)	(2.8)	(11.3)	(24.9)
Non-GAAP research and development expenses	$ 698.8	$ 623.3	$ 2,796.7	$ 2,204.5
Acquired in-process research and development expenses	$ 17.8	$ 22.6	$ 527.1	$ 115.5
GAAP selling, general and administrative expenses	$ 369.1	$ 267.4	$ 1,136.6	$ 944.7
Stock-based compensation expense	(83.5)	(41.1)	(218.8)	(184.0)
Acquisition-related costs (4)	—	(0.7)	—	(13.9)
Non-GAAP selling, general and administrative expenses	$ 285.6	$ 225.6	$ 917.8	$ 746.8
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses	$ 1,002.2	$ 871.5	$ 4,241.6	$ 3,066.8
GAAP other expense, net	$ (9.8)	$ (31.1)	$ (22.8)	$ (164.8)
Decrease in fair value of strategic investments	0.4	6.0	0.6	149.1
Non-GAAP other expense, net	$ (9.4)	$ (25.1)	$ (22.2)	$ (15.7)
GAAP provision for income taxes	$ 178.8	$ 257.9	$ 760.2	$ 910.4
Tax adjustments (1)	35.5	(36.3)	194.7	101.7
Non-GAAP provision for income taxes	$ 214.3	$ 221.6	$ 954.9	$ 1,012.1
GAAP effective tax rate	15.6%	24.0%	17.4%	21.5%
Non-GAAP effective tax rate	16.3%	18.5%	19.4%	20.8%

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**
GAAP operating income	$ 988.5	$ 1,033.5	$ 3,832.0	$ 4,307.4
Stock-based compensation expense	208.6	111.5	581.2	491.3
Intangible asset amortization expense	1.7	—	1.7	—
Decrease (increase) in fair value of contingent consideration (3)	(50.3)	1.8	(51.6)	(57.5)
Intangible asset impairment charge (3)	—	—	—	13.0
Acquisition-related costs (4)	2.8	3.5	11.3	38.8
Non-GAAP operating income	$ 1,151.3	$ 1,150.3	$ 4,374.6	$ 4,793.0

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**
GAAP net income	$ 968.8	$ 818.9	$ 3,619.6	$ 3,322.0
Stock-based compensation expense	208.6	111.5	581.2	491.3
Intangible asset amortization expense	1.7	—	1.7	—
Decrease in fair value of strategic investments	0.4	6.0	0.6	149.1

Decrease (increase) in fair value of contingent consideration (3)	(50.3)	1.8	(51.6)	(57.5)
Intangible asset impairment charge (3)	—	—	—	13.0
Acquisition-related costs (4)	2.8	3.5	11.3	38.8
Total non-GAAP adjustments to pre-tax income	163.2	122.8	543.2	634.7
Tax adjustments (1)	(35.5)	36.3	(194.7)	(101.7)
Non-GAAP net income	$ 1,096.5	$ 978.0	$ 3,968.1	$ 3,855.0
Net income per diluted common share:				
GAAP	$ 3.71	$ 3.15	$ 13.89	$ 12.82
Non-GAAP	$ 4.20	$ 3.76	$ 15.23	$ 14.88
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	260.9	260.3	260.5	259.1

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

	December 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 11,218.3	$ 10,778.5
Accounts receivable, net	1,563.4	1,442.2
Inventories	738.8	460.6
Prepaid expenses and other current assets	623.7	553.5
Total current assets	14,144.2	13,234.8
Property and equipment, net	1,159.3	1,108.4
Goodwill and intangible assets, net	1,927.9	1,691.6
Deferred tax assets	1,812.1	1,246.9
Operating lease assets	293.6	347.4
Long-term marketable securities	2,497.8	112.2
Other long-term assets	895.3	409.6
Total assets	$ 22,730.2	$ 18,150.9
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 3,020.2	$ 2,430.6
Other current liabilities	527.2	311.5
Total current liabilities	3,547.4	2,742.1
Long-term finance lease liabilities	376.1	430.8
Long-term operating lease liabilities	348.6	379.5
Other long-term liabilities	877.7	685.8
Shareholders' equity	17,580.4	13,912.7
Total liabilities and shareholders' equity	$ 22,730.2	$ 18,150.9
Common shares outstanding	257.7	257.0

Notes and Explanations

1: In the three and twelve months ended December 31, 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. “Tax adjustments” for the twelve months ended December 31, 2023 also included a $75 million discrete benefit related to prior tax years resulting from a R&D tax credit study that was completed during the third quarter of 2023. “Tax adjustments” for the twelve months ended December 31, 2022 included $60 million of net discrete tax expense related to our uncertain tax positions associated with intercompany transfer pricing matters partially offset by changes in our estimated prior-year tax liabilities.

2: The difference between the company’s full year 2024 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 $600 million to $700 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 December 31, 2023, the company determined that additional pre-clinical studies for DMD will be required, which resulted in a decrease in the associated fair value of contingent consideration. In the three months ended June 30, 2022, the company revised the scope of certain acquired programs, resulting in a decrease in the associated fair value of contingent consideration and a $13 million “Intangible asset impairment charge.”

4: "Acquisition-related costs" in the three and twelve months ended December 31, 2023 and 2022 related to costs associated with the company's acquisition of Exonics and ViaCyte.

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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 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 LinkedIn, Facebook, Instagram, YouTube and Twitter/X.

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 2024 Financial Guidance” and statements regarding (i) expectations for continued growth in the number treated with our CF medicines, including through new approvals and reimbursements for eligible younger children, our continued work with reimbursement authorities to ensure access for eligible patients, and expectations for expansion of treatment options for patients that have a rare mutation of the CFTR gene, including the N1303K and non-canonical splice mutations, (ii) expectations associated with the recent and anticipated regulatory approvals for CASGEVY, including plans to pursue additional EAPs outside of the U.S., plans to submit additional regulatory filings for CASGEVY, expectations for potential CASGEVY revenue, expectations around the plans to activate ATCs in the U.S., Europe and KSA, expectations associated with our agreement with Synergie Medication Collective, as well as our beliefs regarding the potential benefits of the CGT Access Model, (iii) expectations, plans, and status of the potential near-term commercial launch of vanzacaftor/tezacaftor/deutivacaftor in CF, including our plans to submit regulatory filings in the U.S., Europe and Canada in mid-2024 and plans to use a priority review voucher in the U.S., (iv) expectations, plans, and status of the potential near-term commercial launch of VX-548 for the treatment of moderate to severe acute pain, including expectations regarding the potential benefits of VX-548, and our plans to submit regulatory filings by mid-2024, (v) expectations, development plans, and anticipated timelines for the company's products, 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, (vi) expectations for the expansion of treatment options for patients who cannot benefit from CFTR modulators alone, our collaboration with Moderna to develop CFTR mRNA therapeutics and the status of the MAD portion of the study for VX-522 and expectations to share data from this study in late 2024 or early 2025, (vii) expectations regarding our SCD and TDT program and clinical trials including expectations that a gentler conditioning for CASGEVY could broaden the eligible patient population to more than 150,000 people, (viii) expectations regarding our PNP program, including expectations to meet with regulators and advance VX-548 for PNP into pivotal development and timing thereof, the status of our Phase 2 study of VX-548 in LSR, and our plans to initiate a Phase 2 study with an oral formulation of VX-993 in 2024, (ix) expectations regarding our acute pain program, including plans to initiate a Phase 2 study with an oral formulation of VX-993 in 2024, expectations around completing IND-enabling studies and filing an IND for an intravenous formulation of VX-993 in 2024, and plans to continue to develop NaV1.7 and NaV1.8 inhibitors for both acute pain and PNP, (x) expectations regarding the potential benefits of our AMKD program and plans to select a dose for the Phase 3 portion and begin the Phase 3 study of inaxaplin in the first quarter of 2024, and (xi) expectations regarding our T1D programs, including the potential benefits of our T1D programs that use stem-cell derived, fully differentiated islet cells, and expectations for the advancement of our T1D programs, including clinical trial designs and clinical progress. 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 2024 full year 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 adequate reimbursement or additional regulatory approval for CASGEVY on the expected timeline, or at all, that we are unable to successfully develop, obtain approval or commercialize VX-548 as a treatment for acute or neuropathic pain, 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 Fourth 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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