Vertex Reports Third Quarter 2023 Financial Results

November 6, 2023

— Product revenue of $2.48 billion, a 6% increase compared to Q3 2022 —

—Company raises full year 2023 product revenue guidance to approximately $9.85 billion —

—U.S. FDA Advisory Committee meeting for exa-cel in SCD completed; PDUFA date for exa-cel in SCD is December 8, 2023 —

— Pipeline continues to advance: Phase 3 data in CF and acute pain expected in early 2024; Phase 2 data in neuropathic pain expected in late 2023 —

BOSTON--(BUSINESS WIRE)--Nov. 6, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2023, raised full year 2023 guidance for product revenue and reiterated full year 2023 guidance for operating expenses.

“Vertex has delivered another strong quarter across the business. We remain relentless in our commitment to reach more patients with our cystic fibrosis medicines, while preparing for the potential launch of exa-cel in multiple geographies,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “Our R&D pipeline continues to make remarkable progress and we have a milestone-rich period coming up, with multiple major, near-term milestones, including completion of the Phase 3 pivotal trials for the vanzacaftor triple in cystic fibrosis and VX-548 in acute pain, as well as the Phase 2 VX-548 study data read-out in diabetic peripheral neuropathy.”

Third Quarter 2023 Results

Product revenue increased 6% to $2.48 billion compared to the third quarter of 2022, primarily driven by the continued performance of TRIKAFTA in the U.S., including the launch in children with CF 2 to 5 years of age and continued strong uptake of TRIKAFTA/KAFTRIO in ex-U.S. markets with recently achieved reimbursements, as well as label extensions in younger age groups. Net product revenue in the third quarter of 2023 increased 7% to $1.55 billion in the U.S. and increased 6% to $929 million outside the U.S., compared to the third quarter of 2022.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses were $1.1 billion and $993 million, respectively, compared to $921 million and $758 million, respectively, in the third 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, the costs to support launches of Vertex's therapies globally, and increased acquired IPR&D expenses.

GAAP effective tax rate was 12.2% compared to 20.9% for the third quarter of 2022 as a result of increased R&D tax credits for the current and prior years.

Non-GAAP effective tax rate was 19.4% compared to 21.4% for the third quarter of 2022 as a result of increased 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 11% and 2%, respectively, compared to the third quarter of 2022. Strong revenue growth, higher interest income and lower income tax expense in the third quarter of 2023 were partially offset by increased investment in our mid- and late-stage clinical pipeline, the costs to support launches of Vertex’s therapies globally, and higher acquired IPR&D expenses.

Cash, cash equivalents and total marketable securities as of September 30, 2023 were $13.6 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 today raised its full year 2023 CF product revenue guidance to approximately $9.85 billion. Vertex’s CF product revenue guidance includes expectations in the U.S. for continued performance of TRIKAFTA in ages 6+ and the launch of TRIKAFTA in the 2-5 age group, as well as the continued uptake of TRIKAFTA/KAFTRIO in multiple countries internationally. This guidance continues to include an approximate 150-basis-point negative impact from changes in foreign currency rates, inclusive of our foreign exchange risk management program. Vertex is also reiterating its guidance for full year 2023 GAAP and non-GAAP

combined R&D, acquired IPR&D and SG&A expenses and updating its guidance for full year non-GAAP effective tax rate.

Vertex's financial guidance is summarized below:

	Current FY 2023	Previous FY 2023
CF product revenues	~$9.85 billion	$9.7 to $9.8 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$4.55 to $4.8 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$4.1 to $4.2 billion
Non-GAAP effective tax rate	20 to 21%	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 and anticipated progress includes:

- Health Canada granted market authorization for the use of TRIKAFTA in children with CF 2 to 5 years of age who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. With this approval, approximately 330 patients are now eligible for the first time for a medicine that treats the underlying cause of their disease. Vertex is currently working with government and private payers in Canada to support access for this new patient population as soon as possible.
- The European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for the label extension of KAFTRIO in children with CF 2 to 5 years of age who have at least one F508del mutation in the CFTR gene. If this label extension is approved by the European Commission, more than 1,200 children would be newly eligible for treatment.

Potential Near-Term Launch Opportunities

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

- ***Exagamglogene autotemcel (exa-cel) in sickle cell disease (SCD) and transfusion-dependent beta thalassemia (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 one-time functional cure for SCD and TDT.*
  - The FDA has assigned exa-cel 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.
  - Reviews of the filings for exa-cel with the EMA in the E.U. and the MHRA in the U.K. are well underway, with regulatory decisions expected in the coming months.
  - Vertex submitted a marketing authorization application for exa-cel to the Saudi Food and Drug Authority (SFDA). Exa-cel is the first investigational medicine to receive Breakthrough Designation from the SFDA, reflecting the high unmet need for patients with SCD and TDT in the Kingdom of Saudi Arabia.
  - Clinical data from the CLIMB-111 and CLIMB-121 Phase 1/2/3 studies in TDT and SCD, respectively, were accepted for oral presentation at the upcoming American Society of Hematology (ASH) Annual Meeting and Exposition. Additionally, five abstracts were accepted for poster presentation.

- ***Vanzacaftor/tezacaftor/deutivacaftor, the next-in-class triple combination, in cystic fibrosis****.*
  - Vertex remains on track to complete the pivotal SKYLINE 102 and SKYLINE 103 studies, which are evaluating the efficacy and safety of vanzacaftor/tezacaftor/deutivacaftor relative to TRIKAFTA in patients with CF 12 years of age and older, and the RIDGELINE study of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age, by the end of 2023.
  - Vertex expects to share the results of all three 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 across a variety of pain states, including acute pain, without the limitations of opioids and other currently available medicines.*
  - For its lead compound, VX-548, for the treatment of moderate to severe acute pain, Vertex has completed the randomized, controlled Phase 3 pivotal trial in abdominoplasty and continues to enroll the randomized, controlled Phase 3 trial in bunionectomy and a single-arm safety and effectiveness trial. Vertex remains on track to complete the pivotal program for acute pain in late 2023.
  - Vertex expects to share the results of all three studies in early 2024.

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 programs in clinical development is summarized below.

Cystic Fibrosis

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

- Vertex is enrolling patients in a single ascending dose (SAD) clinical trial for VX-522, the CFTR mRNA therapeutic that Vertex is developing in collaboration with Moderna and expects to complete the SAD and initiate a multiple ascending dose (MAD) study by the end of 2023.
- Consistent with its overall strategy, Vertex is advancing additional CFTR potentiators and correctors through clinical development with the goal of bringing more patients to carrier levels of CFTR function. Vertex takes a portfolio approach to all of its programs and is also advancing additional research-stage CFTR modulators and genetic therapies for CF.

Sickle Cell Disease and Beta Thalassemia

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

Acute and Neuropathic Pain

- The Phase 2, 12-week, dose-ranging study of VX-548 in patients with diabetic peripheral neuropathy (DPN), a common form of peripheral neuropathic pain, has completed. Vertex expects to share results in late 2023. DPN represents approximately 20 percent of the total peripheral neuropathic pain patient population.
- In addition, Vertex plans to initiate another Phase 2 study of VX-548 in peripheral neuropathic pain in late 2023. This study will evaluate the efficacy of VX-548 in patients with lumbosacral radiculopathy (LSR), a second type of peripheral neuropathic pain with high unmet need and no approved therapies. LSR represents over 40 percent of the total peripheral neuropathic pain patient population.
- Consistent with its overall strategy, Vertex takes a portfolio approach with all 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 enrollment in the Phase 2B dose-ranging portion of the study in 2023.

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. Vertex has three programs that use these fully differentiated cells.

- *VX-880, fully differentiated islet cells with standard immunosuppression:*
  - Vertex established proof-of-concept for VX-880 in 2022. More recently, Vertex presented positive, updated clinical data from the ongoing VX-880 Phase 1/2 study at the European Association for the Study of Diabetes (EASD) Annual Meeting in October.
  - The Phase 1/2 study is designed as a sequential, three-part clinical trial to evaluate the safety and efficacy of VX-880. Vertex has completed enrollment in Part C of the study.
- *VX-264, fully differentiated islet cells encapsulated in immunoprotective device:*
  - VX-264 uses the same stem cell-derived, fully differentiated islets from 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 studying 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.
- *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.

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 has discontinued development of VX-864, a first-generation AATD corrector, due to non-serious rash events in

some patients.
- Vertex continues to enroll and dose healthy volunteers with VX-634 and VX-668, the next-wave of investigational small molecule AAT correctors with significantly improved potency and drug-like properties compared with the first-generation AATD correctors.

Duchenne Muscular Dystrophy

Vertex is pursuing preclinical research in Duchenne muscular dystrophy (DMD), using innovative approaches to target the underlying cause of disease, with the goal of transforming the lives of these patients by restoring near-full-length dystrophin and muscle function.

- Based on pre-clinical data generated to date, Vertex has determined that additional *in vitro* and animal studies of the delivery system for its gene editing components will be required prior to advancing the program into clinical development.
- Consistent with its portfolio approach to research and development, Vertex is also using the learnings from its first-generation vectors to design next-generation delivery systems for *in vivo* gene editing in DMD.

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 September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Product revenues, net	$ 2,483.5	$ 2,334.3	$ 7,351.5	$ 6,628.0
Costs and expenses:				
Cost of sales	318.7	289.4	894.2	797.0
Research and development expenses	810.0	645.0	2,338.3	1,846.2
Acquired in-process research and development expenses	51.7	29.0	509.3	92.9
Selling, general and administrative expenses	263.8	246.8	767.5	677.3
Change in fair value of contingent consideration	1.2	(2.6)	(1.3)	(59.3)
Total costs and expenses	1,445.4	1,207.6	4,508.0	3,354.1
Income from operations	1,038.1	1,126.7	2,843.5	3,273.9
Interest income	167.9	46.2	435.2	58.6
Interest expense	(10.9)	(13.7)	(33.5)	(43.2)
Other (expense) income, net	(15.9)	17.2	(13.0)	(133.7)
Income before provision for income taxes	1,179.2	1,176.4	3,232.2	3,155.6
Provision for income taxes	143.9	245.9	581.4	652.5
Net income	$ 1,035.3	$ 930.5	$ 2,650.8	$ 2,503.1

Net income per common share:				
Basic	$ 4.01	$ 3.63	$ 10.29	$ 9.78
Diluted	$ 3.97	$ 3.59	$ 10.18	$ 9.68
Shares used in per share calculations:				
Basic	258.0	256.5	257.7	255.8
Diluted	260.6	259.5	260.4	258.7

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
TRIKAFTA/KAFTRIO	$ 2,274.3	$ 2,010.5	$ 6,611.4	$ 5,665.3
Other CF products	209.2	323.8	740.1	962.7
Product revenues, net	$ 2,483.5	$ 2,334.3	$ 7,351.5	$ 6,628.0

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
GAAP cost of sales	$ 318.7	$ 289.4	$ 894.2	$ 797.0
Stock-based compensation expense	(1.7)	(2.4)	(5.4)	(7.0)
Non-GAAP cost of sales	$ 317.0	$ 287.0	$ 888.8	$ 790.0
GAAP research and development expenses	$ 810.0	$ 645.0	$ 2,338.3	$ 1,846.2
Stock-based compensation expense	(81.1)	(80.0)	(231.9)	(229.9)
Intangible asset impairment charge (3)	—	—	—	(13.0)
Acquisition-related costs (4)	(2.9)	(16.5)	(8.5)	(22.1)
Non-GAAP research and development expenses	$ 726.0	$ 548.5	$ 2,097.9	$ 1,581.2
Acquired in-process research and development expenses	$ 51.7	$ 29.0	$ 509.3	$ 92.9
GAAP selling, general and administrative expenses	$ 263.8	$ 246.8	$ 767.5	$ 677.3
Stock-based compensation expense	(48.1)	(53.2)	(135.3)	(142.9)
Acquisition-related costs (4)	—	(13.2)	—	(13.2)
Non-GAAP selling, general and administrative expenses	$ 215.7	$ 180.4	$ 632.2	$ 521.2
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses	$ 993.4	$ 757.9	$ 3,239.4	$ 2,195.3
GAAP other (expense) income, net	$ (15.9)	$ 17.2	$ (13.0)	$ (133.7)
Decrease (increase) in fair value of strategic investments	6.2	(16.7)	0.2	143.1
Non-GAAP other (expense) income, net	$ (9.7)	$ 0.5	$ (12.8)	$ 9.4
GAAP provision for income taxes	$ 143.9	$ 245.9	$ 581.4	$ 652.5
Tax adjustments (1)	112.9	37.1	159.2	138.0
Non-GAAP provision for income taxes	$ 256.8	$ 283.0	$ 740.6	$ 790.5
GAAP effective tax rate	12.2%	20.9%	18.0%	20.7%
Non-GAAP effective tax rate	19.4%	21.4%	20.5%	21.6%

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
GAAP operating income	$ 1,038.1	$ 1,126.7	$ 2,843.5	$ 3,273.9
Stock-based compensation expense	130.9	135.6	372.6	379.8
Increase (decrease) in fair value of contingent consideration (3)	1.2	(2.6)	(1.3)	(59.3)
Intangible asset impairment charge (3)	—	—	—	13.0
Acquisition-related costs (4)	2.9	29.7	8.5	35.3
Non-GAAP operating income	$ 1,173.1	$ 1,289.4	$ 3,223.3	$ 3,642.7

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
GAAP net income	$ 1,035.3	$ 930.5	$ 2,650.8	$ 2,503.1
Stock-based compensation expense	130.9	135.6	372.6	379.8
Decrease (increase) in fair value of strategic investments	6.2	(16.7)	0.2	143.1
Increase (decrease) in fair value of contingent consideration (3)	1.2	(2.6)	(1.3)	(59.3)
Intangible asset impairment charge (3)	—	—	—	13.0
Acquisition-related costs (4)	2.9	29.7	8.5	35.3
Total non-GAAP adjustments to pre-tax income	141.2	146.0	380.0	511.9
Tax adjustments (1)	(112.9)	(37.1)	(159.2)	(138.0)
Non-GAAP net income	$ 1,063.6	$ 1,039.4	$ 2,871.6	$ 2,877.0
Net income per diluted common share:				
GAAP	$ 3.97	$ 3.59	$ 10.18	$ 9.68
Non-GAAP	$ 4.08	$ 4.01	$ 11.03	$ 11.12
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	260.6	259.5	260.4	258.7

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

	September 30, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 11,928.2	$ 10,778.5
Accounts receivable, net	1,538.7	1,442.2
Inventories	688.7	460.6
Prepaid expenses and other current assets	540.2	553.5
Total current assets	14,695.8	13,234.8
Property and equipment, net	1,124.0	1,108.4
Goodwill and intangible assets	1,691.6	1,691.6
Deferred tax assets	1,729.1	1,246.9
Operating lease assets	310.5	347.4
Long-term marketable securities	1,700.0	112.2
Other long-term assets	475.2	409.6
Total assets	$ 21,726.2	$ 18,150.9
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 3,283.2	$ 2,430.6
Other current liabilities	316.2	311.5
Total current liabilities	3,599.4	2,742.1
Long-term finance lease liabilities	390.3	430.8
Long-term operating lease liabilities	354.4	379.5
Other long-term liabilities	869.3	685.8

Shareholders' equity	16,512.8	13,912.7
Total liabilities and shareholders' equity	$ 21,726.2	$ 18,150.9
Common shares outstanding	257.8	257.0

Notes and Explanations

1: In the three and nine months ended September 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. "Tax adjustments" also included a $74 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.

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 nine months ended September 30, 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 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 therapies across a range of modalities 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.

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 and Latin America. 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 2023 Financial Guidance" and statements regarding (i) expectations for continued growth in the number of people eligible and treated with our CF medicines, including newly eligible younger children, 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 acute pain, (iv) the expectations related to our exa-cel regulatory filings and potential approvals in the U.S., E.U., U.K., and the Kingdom of Saudi Arabia, including anticipated timing of regulatory decisions, 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 to complete both SKYLINE studies and the RIDGELINE study by the end of 2023 and share the results of these studies in early 2024, (vi) 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 by the end of 2023, (vii) expectations regarding the potential benefits and objectives of our pain program and products, including 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 early 2024, expectations to share the results of the recently completed study of VX-548 in patients with DPN and plans to initiate a second Phase 2 study in patients with LSR, (viii) expectations regarding the potential benefits of our AMKD program, and plans regarding our Phase 2/3 study of inaxaplin, including expectations to complete enrollment in the Phase 2B dose-ranging portion of the study in 2023, (ix) 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, (x) our expectations regarding our goals and the potential benefits of our AAT deficiency program, plans to continue to advance VX-634 and VX-668 in clinical trials, (xi) our plans and expectations for our DMD programs,

and (xii) plans with respect to our additional earlier stage research and development programs. 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 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 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 Third 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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Vertex Contacts:
Investor Relations:
Susie Lisa, CFA, 617-341-6108
Manisha Pai, 617-961-1899
Miroslava Minkova, 617-341-6135

Media:
617-341-6992
mediainfo@vrtx.com

Source: Vertex Pharmaceuticals Incorporated