Vertex Reports First Quarter 2023 Financial Results

May 1, 2023

— Product revenue of $2.37 billion, a 13% increase compared to Q1 2022 —

— Company reiterates full year 2023 financial guidance, including product revenue guidance of $9.55 to $9.7 billion —

— TRIKAFTA approved in U.S. for children with cystic fibrosis 2 to 5 years of age —

— Pipeline advancement continues with completion of rolling BLA submissions for exa-cel in the U.S.; multiple additional clinical milestones expected in 2023 —

BOSTON--(BUSINESS WIRE)--May 1, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2023 and reiterated full year 2023 financial guidance.

“Vertex delivered a strong start to 2023, with outstanding execution across our business. We continue to reach more patients globally with our cystic fibrosis medicines and progress our broad and diverse pipeline, most notably completing the rolling BLA submissions for exa-cel in the U.S.,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “Over the course of this year, we look forward to continuing to expand our leadership in CF; preparing for near-term launches, including exa-cel; and advancing multiple potentially transformative medicines through mid- and late-stage clinical trials.”

First Quarter 2023 Results

Product revenue increased 13% to $2.37 billion compared to the first 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. Net product revenue in the first quarter of 2023 increased 3% to $1.40 billion in the U.S. and increased 33% to $971 million outside the U.S., compared to the first quarter of 2022.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses were $1.3 billion and $1.2 billion, respectively, compared to $818 million and $687 million, respectively, in the first quarter of 2022. The increases were due to higher acquired IPR&D expenses, increased investment in support of multiple programs that have advanced in mid- and late-stage clinical development, and the costs to support launches of Vertex's therapies globally.

GAAP effective tax rate was 21.5% compared to 20.2% for the first quarter of 2022.

Non-GAAP effective tax rate was 21.3% compared to 21.5% for the first 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 decreased by 8% and 12%, respectively, compared to the first quarter of 2022, primarily driven by higher acquired IPR&D expenses, increased investment in our mid- and late-stage clinical pipeline, and the costs to support launches of Vertex’s therapies globally, partially offset by strong revenue growth and increased interest income.

Cash, cash equivalents and total marketable securities as of March 31, 2023 were $11.5 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 upfront 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 reiterated its full year 2023 financial guidance, including CF product revenue guidance of $9.55 to $9.7 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 continued uptake of KAFTRIO/TRIKAFTA in ages 6+ in countries outside the U.S., including those with recent reimbursement agreements. 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’s financial guidance is summarized below:

	Current FY 2023	Previous FY 2023

CF product revenues	Unchanged	$9.55 to $9.7 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$4.35 to $4.6 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$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:

- Vertex received approval from the U.S. Food and Drug Administration (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 newly eligible for TRIKAFTA. Vertex has also completed regulatory submissions with the European Medicines Agency (EMA), the Medicines and Healthcare products Regulatory Agency (MHRA) in the United Kingdom, Health Canada, and the Therapeutic Goods Administration (TGA) in Australia for the use of KAFTRIO/TRIKAFTA in children 2 to 5 years of age.
- Vertex received a positive opinion from the EMA Committee for Medicinal Products for Human Use (CHMP) for the use of ORKAMBI in children 1 to <2 years of age with two copies of the F508del mutation in the CFTR gene. If this label extension is approved by the European Commission, nearly 300 children with CF will be eligible for the first time for a medicine to treat the underlying cause of their disease.
- Vertex has also submitted an sNDA to the FDA and Marketing Authorization Applications (MAAs) to the EMA, MHRA, and Health Canada for the use of KALYDECO in children from 1 month to <4 months of age. The FDA granted Priority Review designation and assigned a PDUFA date of May 3, 2023.

Potential Near-Term Launch Opportunities

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

- *Exagamglogene autotemcel (exa-cel), formerly known as CTX001, in severe sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT):*
  - Vertex completed the rolling submission of its biologics licensing applications (BLAs) in the U.S. The BLAs include requests for Priority Review, which if granted, would shorten the FDA's review of the application to eight months from the time of submission versus a standard review timeline of 12 months. In the U.S., exa-cel has been granted Fast Track, Regenerative Medicine Advanced Therapy (RMAT), Orphan Drug and Rare Pediatric Disease designations.
  - In December 2022, Vertex completed regulatory submissions for exa-cel with the EMA and MHRA in the EU and the U.K., respectively. Both the EMA and the MHRA have validated the MAAs, indicating acceptance of the marketing applications and initiation of the review. 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 (ILAP) from the MHRA.
- *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. Vertex expects to complete the SKYLINE studies by the end of 2023. In parallel, Vertex has initiated a study of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age, known as the RIDGELINE study, and the company also expects to complete this study by the end of 2023.
- *VX-548 in acute pain:* Vertex continues to enroll the Phase 3 pivotal program for its lead compound, VX-548, for the treatment of moderate to severe acute pain and expects to complete the pivotal program in late 2023 or early 2024. VX-548 has been granted Breakthrough Therapy and Fast Track designations in the U.S. 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 has initiated a single

ascending dose (SAD) clinical trial for VX-522 in people with CF, which is active and enrolling patients at multiple sites. Vertex 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 for VX-522.

Beta Thalassemia and Sickle Cell Disease

Exa-cel is a non-viral *ex vivo* CRISPR gene-editing therapy, which is being developed as a potential functional cure for TDT and SCD. Vertex is developing exa-cel in collaboration with CRISPR Therapeutics.

- Dosing in the Phase 1/2/3 CLIMB-111 and CLIMB-121 studies continues, as does the CLIMB-131 long-term follow-up study in patients 12 years of age and older.
- Two additional Phase 3 studies of exa-cel continue to enroll patients 5 to 11 years of age with TDT or SCD.

Neuropathic Pain (NaV1.8)

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

- Vertex continues to enroll and dose patients in a Phase 2 dose-ranging study of VX-548 in patients with diabetic peripheral neuropathy, a common form of peripheral neuropathic pain.
- Vertex expects to complete this study in late 2023 or early 2024.

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.

- In March, the *New England Journal of Medicine* published results from preclinical studies and a Phase 2 study evaluating the efficacy and safety of inaxaplin (VX-147) on top of standard-of-care in people with focal segmental glomerulosclerosis (FSGS) and two APOL1 variants, a form of AMKD. The results from the Phase 2 study of inaxaplin demonstrated a statistically significant and clinically meaningful mean reduction in proteinuria of 47.6% at 13 weeks compared to baseline. Inaxaplin was generally well tolerated in the study.
- 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 has completed enrollment and dosing in Part B of the Phase 1/2 study of VX-880. Vertex expects to present updated clinical data, including data from more patients and with longer duration of follow-up from the VX-880 study at scientific congresses in 2023, including the American Diabetes Association Scientific Sessions in June. Next, Vertex intends to begin Part C of the study with concurrent dosing. In March 2023, VX-880 was granted PRIME designation by the EMA.
2. *VX-264, fully differentiated cells encapsulated in immunoprotective device:* The Investigational New Drug (IND) application in the U.S. and the Clinical Trial Application (CTA) in Canada for VX-264, the cells plus device program, have been cleared, and Vertex plans to begin enrollment and dosing in a Phase 1/2 clinical trial in the near term.
3. *Edited fully differentiated cells:* Vertex's hypoimmune cell research program continues to progress.

In addition, a Phase 1/2 study of VCTX-211, a hypoimmune cell program using ViaCyte cells that originated under the CRISPR Therapeutics and ViaCyte collaboration, is active and enrolling patients.

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 initiated 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. This Phase 2 trial continues to enroll and dose patients.
- 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.

Additional Earlier Stage R&D Programs

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

Vertex is also advancing preclinical assets in new disease areas, such as Duchenne muscular dystrophy (DMD) and myotonic dystrophy type 1 (DM1). Additionally, Vertex is working on preclinical molecules with the potential to expand our leadership in existing disease areas, including assets targeting gentler conditioning for exa-cel and NaV1.7 in pain.

Investments in External Innovation

Consistent with its strategy to develop transformative medicines for serious diseases, in the first quarter of 2023, Vertex announced a new licensing agreement for the use of CRISPR Therapeutics' gene editing technology, known as CRISPR/Cas9, to accelerate the development of Vertex's hypoimmune cell therapies for T1D. Under this agreement, Vertex made an upfront payment of $100 million to CRISPR in the first quarter of 2023.

In addition, the previously announced global research collaboration with Entrada Therapeutics, focused on therapeutics for DM1, has closed. Under the terms of the agreement, upon closing, Vertex made an upfront payment of approximately $225 million to Entrada, as well as an equity investment of approximately $25 million.

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, and (v) 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 March 31,	
	2023	**2022**
Product revenues, net	$ 2,374.8	$ 2,097.5
Costs and expenses:		
Cost of sales	266.9	245.8
Research and development expenses	742.6	601.1
Acquired in-process research and development expenses	347.1	2.0
Selling, general and administrative expenses	241.1	215.2
Change in fair value of contingent consideration	(1.9)	(7.5)
Total costs and expenses	1,595.8	1,056.6
Income from operations	779.0	1,040.9
Interest income	122.6	1.6
Interest expense	(11.4)	(14.9)
Other income (expense), net	1.3	(72.8)
Income before provision for income taxes	891.5	954.8
Provision for income taxes	191.7	192.7

Net income	$ 699.8	$ 762.1
Net income per common share:		
Basic	$ 2.72	$ 2.99
Diluted	$ 2.69	$ 2.96
Shares used in per share calculations:		
Basic	257.4	255.1
Diluted	260.3	257.9

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

	Three Months Ended March 31,	
	2023	**2022**
TRIKAFTA/KAFTRIO	$ 2,096.7	$ 1,761.6
Other CF products	278.1	335.9
Product revenues, net	$ 2,374.8	$ 2,097.5

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

	Three Months Ended March 31,	
	2023	**2022**
GAAP cost of sales	$ 266.9	$ 245.8
Stock-based compensation expense	(1.9)	(2.2)
Non-GAAP cost of sales	$ 265.0	$ 243.6
GAAP research and development expenses	$ 742.6	$ 601.1
Stock-based compensation expense	(76.3)	(80.4)
Acquisition-related costs (3)	(2.8)	(2.8)
Non-GAAP research and development expenses	$ 663.5	$ 517.9
Acquired in-process research and development expenses	$ 347.1	$ 2.0
GAAP selling, general and administrative expenses	$ 241.1	$ 215.2
Stock-based compensation expense	(44.2)	(47.7)
Non-GAAP selling, general and administrative expenses	$ 196.9	$ 167.5
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses	$ 1,207.5	$ 687.4
GAAP other income (expense), net	$ 1.3	$ (72.8)
(Increase) decrease in fair value of strategic investments	(6.4)	75.6
Non-GAAP other (expense) income, net	$ (5.1)	$ 2.8
GAAP provision for income taxes	$ 191.7	$ 192.7
Tax adjustments (1)	22.7	56.2
Non-GAAP provision for income taxes	$ 214.4	$ 248.9
GAAP effective tax rate	21.5%	20.2%
Non-GAAP effective tax rate	21.3%	21.5%

Vertex Pharmaceuticals Incorporated
Reconciliation of GAAP to Non-GAAP Financial Information (continued)

(in millions, except per share amounts)(unaudited)

	Three Months Ended March 31,	
	2023	2022
GAAP operating income	$ 779.0	$ 1,040.9
Stock-based compensation expense	122.4	130.3
Decrease in fair value of contingent consideration	(1.9)	(7.5)
Acquisition-related costs (3)	2.8	2.8
Non-GAAP operating income	$ 902.3	$ 1,166.5

	Three Months Ended March 31,	
	2023	2022
GAAP net income	$ 699.8	$ 762.1
Stock-based compensation expense	122.4	130.3
(Increase) decrease in fair value of strategic investments	(6.4)	75.6
Decrease in fair value of contingent consideration	(1.9)	(7.5)
Acquisition-related costs (3)	2.8	2.8
Total non-GAAP adjustments to pre-tax income	116.9	201.2
Tax adjustments (1)	(22.7)	(56.2)
Non-GAAP net income	$ 794.0	$ 907.1
Net income per diluted common share:		
GAAP	$ 2.69	$ 2.96
Non-GAAP	$ 3.05	$ 3.52
Shares used in diluted per share calculations:		
GAAP and Non-GAAP	260.3	257.9

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

	March 31, 2023	December 31, 2022
Assets		
Cash, cash equivalents and marketable securities	$ 10,414.1	$ 10,778.5
Accounts receivable, net	1,547.8	1,442.2
Inventories	535.1	460.6
Prepaid expenses and other current assets	468.7	553.5
Total current assets	12,965.7	13,234.8
Property and equipment, net	1,111.7	1,108.4
Goodwill and intangible assets	1,691.6	1,691.6
Deferred tax assets	1,359.9	1,246.9
Operating lease assets	336.3	347.4
Long-term marketable securities	1,081.5	112.2
Other long-term assets	427.5	409.6
Total assets	$ 18,974.2	$ 18,150.9
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 2,649.2	$ 2,430.6
Other current liabilities	377.0	311.5
Total current liabilities	3,026.2	2,742.1
Long-term finance lease liabilities	417.6	430.8
Long-term operating lease liabilities	371.6	379.5
Other long-term liabilities	726.5	685.8
Shareholders' equity	14,432.3	13,912.7
Total liabilities and shareholders' equity	$ 18,974.2	$ 18,150.9

Common shares outstanding	257.5	257.0

Notes and Explanations

1: In the three months ended March 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.

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 $440 million to $575 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: "Acquisition-related costs" in the three months ended March 31, 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, including expectations that nearly 300 children with CF will be eligible for a medicine for the first time if/when ORKAMBI is approved for children 1 to <2 years of age in Europe, 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, 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) recent and anticipated regulatory filings, and data availability, as well as expectations regarding timing and regulatory review thereof, (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 regarding the potential benefits of exa-cel as a functional cure for TDT and SCD, (vii) expectations regarding the potential benefits and objectives of our pain program and products, including the expectation to complete the Phase 2 dose-ranging study of VX-548 in peripheral neuropathic pain 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 regarding the potential benefits of our T1D program, including our plans to continue to progress the Phase 1/2 program of VX-880, including expectations to present updated clinical data from the study at a scientific congress in 2023 and to begin Part C of the study, as well as our plans regarding our additional programs in T1D, including plans to begin enrollment and dosing in a Phase 1/2 clinical trial for VX-264 in the near term, (x) our expectations regarding our goals and the potential benefits of our AAT deficiency program and plans to continue to advance VX-864 and VX-634 in clinical trials, (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, including our belief that the CRISPR/Cas9 technology will accelerate development of our hypoimmune cell therapies for T1D. 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 (877) 270-2148 (U.S.) or +1 (412) 902-6510 (International) and reference the “Vertex Pharmaceuticals First 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.

(VRTX-E)

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