Vertex Reports First Quarter 2022 Financial Results

May 5, 2022

— Product revenues of $2.10 billion, a 22% increase compared to Q1 2021 —

— Company reiterates full year 2022 product revenue guidance of $8.4 to $8.6 billion —

— Mid- and late-stage clinical pipeline now spans 6 disease areas —

BOSTON--(BUSINESS WIRE)--May 5, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2022 and reiterated full year 2022 product revenue guidance.

“Following upon our success in transforming the treatment of cystic fibrosis, Vertex's unique and differentiated R&D strategy continues to deliver with positive Phase 2 proof-of-concept studies in multiple disease areas, and another wave of therapies set to enter the clinic in the second half of this year," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "With yet another quarter of strong revenue performance characterized by 22% year-over-year growth, we are well positioned for continued innovation and sustained growth as we work to bring additional transformative medicines to more patients around the globe."

First Quarter 2022 Financial Highlights

	Three Months Ended March 31,		%
	2022	**2021**	**Change**
	(in millions, except per share amounts)		
Product revenues, net	$ 2,097	$ 1,723	22 %
TRIKAFTA/KAFTRIO	$ 1,762	$ 1,193	
SYMDEKO/SYMKEVI	$ 65	$ 125	
ORKAMBI	$ 132	$ 219	
KALYDECO	$ 139	$ 186	
GAAP operating income	$ 1,041	$ 888	17 %
Non-GAAP operating income *	$ 1,167	$ 1,002	16 %
GAAP net income	$ 762	$ 653	17 %
Non-GAAP net income *	$ 907	$ 781	16 %
GAAP net income per share - diluted	$ 2.96	$ 2.49	19 %
Non-GAAP net income per share - diluted *	$ 3.52	$ 2.98	18 %

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

Product revenues increased 22% to $2.10 billion compared to the first quarter of 2021, primarily driven by the strong launches of TRIKAFTA/KAFTRIO in multiple countries internationally and the strong performance of TRIKAFTA in the U.S., including the June 2021 launch of TRIKAFTA in children 6-11 years old in the U.S. Net product revenues in the first quarter of 2022 increased 9% to $1.37 billion in the U.S. and increased 55% to $729 million outside the U.S., compared to the first quarter of 2021.

GAAP and Non-GAAP net income increased compared to the first quarter of 2021, driven by strong product revenue growth.

Cash, cash equivalents and marketable securities as of March 31, 2022 were $8.2 billion, an increase of approximately $0.7 billion compared to December 31, 2021. The increase was primarily driven by strong revenue growth and operating cash flow.

First Quarter 2022 Expenses

	Three Months Ended March 31,	
	2022	**2021**
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 818	$ 648
Combined Non-GAAP R&D and SG&A expenses *	$ 687	$ 531
GAAP R&D expenses	$ 603	$ 456
Non-GAAP R&D expenses *	$ 520	$ 380
GAAP SG&A expenses	$ 215	$ 192
Non-GAAP SG&A expenses	$ 167	$ 151
GAAP income taxes (1)	$ 193	$ 168
Non-GAAP income taxes *	$ 249	$ 206
GAAP effective tax rate (1)	20%	20%
Non-GAAP effective tax rate	22%	21%

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

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to the first quarter of 2021, primarily due to the progression of multiple product candidates into mid- to late-stage clinical development and incremental investment to support the launches of Vertex's therapies globally.

GAAP and Non-GAAP income taxes increased compared to the first quarter of 2021, primarily due to Vertex's increased operating income.

Full Year 2022 Financial Guidance

Vertex today reiterated full year 2022 product revenue guidance. Vertex's guidance is summarized below:

	Current FY 2022	Previous FY 2022
Product revenues	Unchanged	$8.4 to $8.6 billion
Combined GAAP R&D and SG&A expenses (2)	Unchanged	$3.30 to $3.45 billion
Combined Non-GAAP R&D and SG&A expenses (2) *	$2.82 to $2.92 billion	$2.70 to $2.75 billion
Non-GAAP effective tax rate	Unchanged	21% to 22%

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

Key Business Highlights

Cystic Fibrosis (CF) Marketed Products

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

- Health Canada granted marketing authorization for TRIKAFTA in children 6 to 11 years of age. With this approval, approximately 500 children with CF are newly eligible for treatment with a CFTR modulator.
- Vertex signed a reimbursement agreement with the Australian Pharmaceutical Benefits Scheme for TRIKAFTA® (ivacaftor/tezacaftor/elexacaftor) for the treatment of patients with cystic fibrosis 12 years and older with at least one F508del mutation in the CF transmembrane conductance regulator (CFTR) gene. With this agreement, approximately 700 people in Australia will have access to a CFTR modulator therapy for the first time.
- Vertex completed enrollment in the Phase 3 study of TRIKAFTA/KAFTRIO in children 2 to 5 years old. The Company anticipates filing a supplementary new drug application (sNDA) with the FDA before the end of 2022.
- In March, Vertex filed an sNDA with the FDA for ORKAMBI for the use of ORKAMBI in children 12 months to less than 24 months old. Vertex intends to submit regulatory filings in Europe in Q2 2022.
- In January, the European Commission and the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) approved a label extension for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor, for the treatment of CF in children ages 6 through 11 years old who have at least one F508del mutation in the CF transmembrane conductance regulator (CFTR) gene. With these approvals, approximately 1,900 children are newly eligible for KAFTRIO®.
- In 2021, Vertex presented the first long-term follow-up data for TRIKAFTA, demonstrating no loss in mean lung function at 96 weeks, a first for any CFTR modulator to date in people with F/F and F/MF mutations. Vertex has now completed a comparison of long-term data in TRIKAFTA patients to matched untreated controls, and will present these data at an upcoming medical congress.

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

R&D pipeline

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

Cystic Fibrosis

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

- Vertex is conducting two Phase 3 global, randomized, double-blind, active-controlled clinical trials (SKYLINE 102 and SKYLINE 103) evaluating Vertex's new once-daily investigational triple combination of VX-121/ *tezacaftor*/VX-561 in patients

with CF. The SKYLINE 102 and SKYLINE 103 trials will compare the efficacy and safety of VX-121/*tezacaftor*/VX-561 to TRIKAFTA. More than 180 sites across both studies are open and enrolling, and enrollment in both trials is expected to be completed in late 2022 or early 2023.
- In collaboration with Moderna, Vertex is developing CF mRNA therapeutics designed to treat the underlying cause of CF by programming cells in the lungs to produce functional CFTR protein for the treatment of the approximately 5,000 people with CF who do not produce any CFTR protein. IND-enabling studies have been completed, and Vertex is on track to submit an IND for this program in 2H 2022.

Beta Thalassemia and Sickle Cell Disease (SCD)

The CTX001 program employs a non-viral *ex vivo* CRISPR gene-editing therapy, which is being developed as a potential functional cure for transfusion-dependent thalassemia (TDT) and severe sickle cell disease (SCD). Vertex is developing CTX001 in collaboration with CRISPR Therapeutics.

- Enrollment is complete in the ongoing Phase 3 clinical trials in TDT and SCD, with more than 75 patients dosed to date. Vertex anticipates presenting updated data from the clinical trials, with more patients and longer follow-up, at medical conferences in 2022.
- Two new Phase 3 studies of CTX001 were initiated in pediatric patients with TDT and SCD.
- Vertex plans to submit global regulatory filings for CTX001 in TDT and SCD in late 2022.

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, Vertex initiated pivotal development of VX-147 in a single Phase 2/3 study in patients with AMKD with two APOL1 mutations and proteinuric kidney disease.
- This Phase 2/3 adaptive study will first evaluate two doses of VX-147 to select a dose for Phase 3 and subsequently evaluate the efficacy and safety of the single, selected dose in the Phase 3 portion of the study. The primary efficacy endpoint for the final analysis is eGFR slope in patients receiving the VX-147 selected dose compared to placebo at two years. The study is designed to have a pre-planned interim analysis at Week 48 evaluating eGFR slope, supported by a percent change from baseline in proteinuria in the VX-147 arm versus placebo. If positive, the interim analysis may serve as the basis for Vertex to seek accelerated approval of VX-147 in the U.S. for patients with AMKD.

Pain (NaV1.8)

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

- In March, Vertex reported positive data from two Phase 2 dose ranging acute pain studies with VX-548, one following bunionectomy surgery and the other following abdominoplasty surgery. Both studies met their primary endpoint and established proof of concept for VX-548.
- Vertex plans to advance VX-548 into pivotal development in acute pain in the second half of 2022, following discussions with regulators.

Type 1 Diabetes (T1D)

Vertex is evaluating cell therapies using stem cell-derived islets to replace the endogenous insulin-producing islet cells that are destroyed in people with T1D with the goal of developing a potential functional cure for this disease.

- VX-880 is a stem cell-derived, fully differentiated islet replacement therapy, used in combination with standard immunosuppression to protect the implanted cells. VX-880 is being evaluated in a Phase 1/2 clinical trial for the treatment of T1D.
- This program has been placed on clinical hold in the U.S. by the FDA, based on their determination of insufficient information for dose escalation with the product. Vertex is working collaboratively and with urgency to understand and address the FDA's questions.
- Vertex previously announced:
    - The first patient, who received a half dose of VX-880, is insulin-independent with an HbA1C of 5.2% at Day 270.
    - The second patient, who also received a half dose of VX-880, demonstrated restoration of glucose-responsive insulin production and significant improvement in glycemic control with reductions in exogenous insulin requirements.
- Taken together, results in the first two patients establish proof-of-concept for VX-880 in the treatment of T1D. Per protocol, the Independent Data Monitoring Committee reviewed the totality of the safety data from the first two patients dosed and recommended advancement to Part B of the study, and treatment with the full target dose.
    - The third patient treated with VX-880 received the full target dose and has reached the Day 29 follow-up milestone.

- Across the program, VX-880 has been generally well tolerated to date. There have been no serious adverse events (SAEs) considered related to VX-880. The majority of adverse events (AEs) were mild or moderate in all patients treated to date. The safety profile was generally consistent with the immunosuppressive regimen used in the study and the perioperative period.
- Vertex is continuing to advance additional programs in T1D, in which these same stem cell-derived islets are encapsulated and implanted in an immunoprotective device or modified to produce hypoimmune stem cell islets with the goal of eliminating the need for immunosuppression.
- Vertex is on track to submit an IND for the cells plus device program in 2022.

Vertex expects to share additional data for VX-880 at medical conferences this year.

Alpha-1 Antitrypsin (AAT) Deficiency

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

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

Duchenne Muscular Dystrophy (DMD)

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

- Vertex has advanced its first *in vivo* gene editing therapy for DMD into IND-enabling studies.

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

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 and SG&A expenses and effective tax rate on a non-GAAP basis. The guidance regarding combined GAAP and non-GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development transactions, including collaborations, asset acquisitions and/or licensing of third-party intellectual property rights. The company does not provide guidance regarding its GAAP effective tax rate because it is unable to forecast with reasonable certainty the impact of excess tax benefits related to stock-based compensation and the possibility of certain discrete items, which could be material.

Vertex Pharmaceuticals Incorporated
Consolidated Statements of Operations
(in millions, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2022	**2021**
Revenues:		
Product revenues, net	$ 2,097.5	$ 1,723.3
Other revenues	—	1.0
Total revenues	2,097.5	1,724.3

Costs and expenses:		
Cost of sales	245.8	192.3
Research and development expenses	603.1	456.0
Selling, general and administrative expenses	215.2	192.1
Change in fair value of contingent consideration	(7.5)	(3.9)
Total costs and expenses	1,056.6	836.5
Income from operations	1,040.9	887.8
Interest income	1.6	1.5
Interest expense	(14.9)	(15.7)
Other expense, net	(72.8)	(52.7)
Income before provision for income taxes	954.8	820.9
Provision for income taxes	192.7	167.8
Net income	$ 762.1	$ 653.1
Net income per common share:		
Basic	$ 2.99	$ 2.52
Diluted	$ 2.96	$ 2.49
Shares used in per share calculations:		
Basic	255.1	259.4
Diluted	257.9	261.9

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

	Three Months Ended March 31,	
	2022	**2021**
GAAP net income	$ 762.1	$ 653.1
Stock-based compensation expense	130.3	115.2

Decrease in fair value of strategic investments (3)	75.6	52.3
Decrease in fair value of contingent consideration (4)	(7.5)	(3.9)
Acquisition-related costs (5)	2.8	2.8
Total non-GAAP adjustments to pre-tax income *	201.2	166.4
Tax adjustments (1) *	(56.2)	(38.2)
Non-GAAP net income *	$ 907.1	$ 781.3
Net income per diluted common share:		
GAAP	$ 2.96	$ 2.49
Non-GAAP *	$ 3.52	$ 2.98
Shares used in diluted per share calculations:		
GAAP and Non-GAAP	257.9	261.9

	Three Months Ended March 31,	
	2022	**2021**
GAAP operating income	$ 1,040.9	$ 887.8
Stock-based compensation expense	130.3	115.2
Decrease in fair value of contingent consideration (4)	(7.5)	(3.9)
Acquisition-related costs (5)	2.8	2.8
Non-GAAP operating income *	$ 1,166.5	$ 1,001.9

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

	Three Months Ended March 31,	
	2022	**2021**
GAAP cost of sales	$ 245.8	$ 192.3

Stock-based compensation expense	(2.2)	(1.4)
Non-GAAP cost of sales	$ 243.6	$ 190.9
GAAP research and development expenses	$ 603.1	$ 456.0
Stock-based compensation expense	(80.4)	(72.8)
Acquisition-related costs (5)	(2.8)	(2.8)
Non-GAAP research and development expenses *	$ 519.9	$ 380.4
GAAP selling, general and administrative expenses	$ 215.2	$ 192.1
Stock-based compensation expense	(47.7)	(41.0)
Non-GAAP selling, general and administrative expenses	$ 167.5	$ 151.1
Combined non-GAAP R&D and SG&A expenses *	$ 687.4	$ 531.5

	Three Months Ended March 31,	
	2022	**2021**
GAAP other expense, net	$ (72.8)	$ (52.7)
Decrease in fair value of strategic investments (3)	75.6	52.3
Non-GAAP other income (expense), net	$ 2.8	$ (0.4)
GAAP provision for income taxes	$ 192.7	$ 167.8
Tax adjustments (1) *	56.2	38.2
Non-GAAP provision for income taxes *	$ 248.9	$ 206.0
GAAP effective tax rate	20 %	20 %
Non-GAAP effective tax rate	22 %	21 %

* Starting in the first quarter of 2022, Vertex no longer excludes research and development charges resulting from upfront or contingent milestone payments in connection

with collaborations, asset acquisitions and/or licensing of third-party intellectual property rights from its Non-GAAP financial measures. Non-GAAP financial measures for the first quarter of 2021 have been recast to reflect this change.

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

	March 31, 2022	December 31, 2021
Assets		
Cash, cash equivalents and marketable securities	$ 8,238.1	$ 7,524.9
Accounts receivable, net	1,292.8	1,136.8
Inventories	338.9	353.1
Property and equipment, net	1,107.4	1,094.1
Goodwill and intangible assets	1,402.2	1,402.2
Deferred tax assets	945.5	934.5
Other assets	931.2	986.9
Total assets	$ 14,256.1	$ 13,432.5
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 1,894.1	$ 1,873.6
Finance lease liabilities	544.2	556.7
Contingent consideration	179.0	186.5
Other liabilities	731.8	715.7
Shareholders' equity	10,907.0	10,100.0
Total liabilities and shareholders' equity	$ 14,256.1	$ 13,432.5
Common shares outstanding	255.6	254.5

Notes and Explanations

1: In the three months ended March 31, 2022 and 2021, "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 2022 combined GAAP R&D and SG&A expenses and combined non-GAAP R&D and SG&A expenses guidance relates primarily to $440 million to $510 million of stock-based compensation expense. The guidance regarding combined GAAP and non-GAAP R&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: "Other expense, net" includes net gains and losses related to changes in the fair value of the company's strategic investments.

4: During the three months ended March 31, 2022 and 2021, the change in the fair value of contingent consideration relates to potential payments to Exonics Therapeutics' former equity holders.

5: "Acquisition-related costs" in the three months ended March 31, 2022 and 2021 related to costs associated with the company's acquisition of Exonics Therapeutics in 2019.

Note: *Amounts may not foot due to rounding.*

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney disease. In addition, Vertex has a rapidly expanding pipeline of cell and genetic therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 12 consecutive years on Science magazine's Top Employers list and one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, Dr. Kewalramani's statements in this press release, the information provided regarding future financial performance and operations, the section captioned "Full Year 2022 Financial Guidance" and statements regarding (i) anticipated regulatory discussions and filings, data availability, and timing thereof, (ii) the expectations, development plans and anticipated timelines for the company's products and product candidates and pipeline programs, including study designs, patient enrollment, data availability and timing thereof, (iii) expectations for continued growth in the number of CF patients treated with our medicines, including the number of children newly eligible for TRIKAFTA/KAFTRIO, uptake of and expanded access to the company's medicines, additional reimbursement agreements, new approvals, including market authorizations and label extensions outside of the U.S., and expansion of treatment options for the patients who cannot benefit from CFTR modulators, (iv) expectations regarding our collaboration with Moderna to develop CF mRNA therapeutics, including our plans to submit an IND for this program in 2022, (v) anticipated presentations of data and global regulatory filings for CTX001 in late 2022, (vi) expectations regarding the potential benefits of our pain program and products, and plans for the advancement of VX-548 into pivotal development in acute pain in the second half of 2022, (vii) the potential benefits and safety of VX-880, our plans and expectations regarding interactions with the FDA, including our ability to resume our Phase 1/2 program for VX-880 at U.S. sites, and our plans to continue to progress the Phase 1/2 program for VX-880, (viii) our plans and expectations regarding our additional programs in T1D, including the completion of IND-enabling studies for the encapsulated islet cell program and anticipated regulatory filings in 2022, (ix) plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022, (x) our plans regarding our Phase 2/3 study of VX-147 in AMKD, and our beliefs regarding anticipated results of the study and the possibility for accelerated approval in the U.S. and (xi) our plans regarding our DMD program. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2022 product revenues, expenses and effective tax rates may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that the company may not be able to submit the anticipated regulatory filings on the expected timeline, or at all, that external factors may have different or more significant impacts on the company's business or operations than the company currently expects, that data from preclinical testing or clinical trials, especially if based on a limited number of patients, may not be indicative of final results or available on anticipated timelines, that the company may not realize the anticipated benefits from our collaborations with third parties, that data from the company's development programs may not support registration or further development of its potential medicines in a timely manner, or at all, due to safety, efficacy or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission (SEC) and available through the company's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements, or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast

The company will host a conference call and webcast at 4:30 p.m. ET. To access the call, please dial (877) 270-2148 (U.S.) or +1 (412) 902-6510 (International) and reference the "Vertex Pharmaceuticals First Quarter 2022 Earnings Call".

The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section. To ensure a timely connection, it is recommended that participants register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

(VRTX-E)

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