Vertex Reports Third Quarter 2022 Financial Results

October 27, 2022

— Product revenue of $2.33 billion, an 18% increase compared to Q3 2021 —

— Company increases full year 2022 product revenue guidance to $8.8 billion to $8.9 billion —

— Significant progress in mid- and late-stage pipeline, with near-term commercialization opportunities:

Initiating global exa-cel regulatory submissions for Sickle Cell Disease and Beta Thalassemia in 2022

Initiated Phase 3 clinical studies of VX-548, a novel, selective NaV1.8 inhibitor, in acute pain —

BOSTON--(BUSINESS WIRE)--Oct. 27, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2022 and increased its full year 2022 revenue guidance.

"The third quarter marked another period of strong performance in the CF business and across the company," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "As we progress exa-cel towards global regulatory submission, initiate pivotal development of the acute pain program and continue the rapid advancement of multiple mid- and late-stage clinical programs, we are executing on our goal of serial innovation for patients, which will drive significant growth for the company for years to come."

Third Quarter 2022 Financial Highlights

	Three Months Ended September 30,		%
	2022	2021	Change
	(in millions, except per share amounts)		
Product revenues, net	$ 2,334	$ 1,984	18%
TRIKAFTA/KAFTRIO	$ 2,011	$ 1,556	
SYMDEKO/SYMKEVI	$ 38	$ 81	
ORKAMBI	$ 146	$ 185	
KALYDECO	$ 139	$ 162	
GAAP operating income	$ 1,127	$ 1,055	7%
Non-GAAP operating income *	$ 1,289	$ 1,162	11%
GAAP net income	$ 931	$ 852	9%
Non-GAAP net income *	$ 1,039	$ 912	14%
GAAP net income per share - diluted	$ 3.59	$ 3.28	9%
Non-GAAP net income per share - diluted *	$ 4.01	$ 3.51	14%

*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 third quarter of 2021 have been recast to reflect this change.

Product revenue increased 18% to $2.33 billion compared to the third quarter of 2021, primarily driven by the strong uptake of TRIKAFTA/KAFTRIO in multiple countries internationally and continued steady performance of TRIKAFTA in the U.S. Net product revenue in the third quarter of 2022 increased 5% to $1.46 billion in the U.S. and increased 46% to $879 million outside the U.S., compared to the third quarter of 2021.

GAAP and Non-GAAP net income increased by 9% and 14%, respectively, compared to the third quarter of 2021, primarily driven by strong revenue growth, partially offset by increased investment in our mid- and late-stage clinical pipeline.

Cash, cash equivalents and marketable securities as of September 30, 2022 were $9.8 billion, an increase of approximately

$2.2 billion compared to December 31, 2021. The year-to-date increase was primarily driven by strong revenue growth and operating cash flow, partially offset by income tax payments and our acquisition of ViaCyte.

Third Quarter 2022 Expenses

	Three Months Ended September 30,	
	2022	2021
	(in millions)	
Combined GAAP R&D, Acquired IPR&D and SG&A expenses	$ 921	$ 692
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses *	$ 758	$ 588
GAAP R&D expenses	$ 645	$ 467
Non-GAAP R&D expenses *	$ 549	$ 403
Acquired IPR&D *	$ 29	$ 27
GAAP SG&A expenses	$ 247	$ 198
Non-GAAP SG&A expenses	$ 180	$ 158
GAAP income taxes (1)	$ 246	$ 231
Non-GAAP income taxes *	$ 283	$ 232
GAAP effective tax rate (1)	21%	21%
Non-GAAP effective tax rate	21%	20%

*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. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations. Non-GAAP financial measures for the third quarter of 2021 have been recast to reflect this change.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses increased compared to the third quarter of 2021, due to increased investment in support of multiple programs that have advanced in mid- and late-stage clinical development and the costs to support launches of Vertex's therapies globally.

GAAP and Non-GAAP income taxes increased compared to the third quarter of 2021, primarily due to Vertex’s increased pre-tax income.

Full Year 2022 Financial Guidance

Vertex today increased its full year 2022 product revenue guidance to reflect the uptake of KAFTRIO/TRIKAFTA in countries outside the U.S. and continued performance of TRIKAFTA in the U.S. Vertex’s guidance is summarized below:

	Current FY 2022	Previous FY 2022
Product revenues	$8.8 to $8.9 billion	$8.6 to $8.8 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$3.48 to $3.63 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2) *	Unchanged	$3.0 to $3.1 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. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations.

Key Business Highlights

Cystic Fibrosis (CF) Marketed Products

Vertex anticipates the number of CF patients treated with our medicines will continue to grow, driven by the consistent performance of TRIKAFTA in the U.S., launches of KAFTRIO/TRIKAFTA outside the U.S., and additional reimbursement

agreements and new approvals for the treatment of younger patients. Recent and anticipated progress includes:

- U.S. regulatory approval for ORKAMBI in children with CF 12 months to less than 24 months of age.
- Reimbursement of KAFTRIO in Italy in children with CF 6 to 11 years of age.
- Upcoming presentation of full results from the TRIKAFTA/KAFTRIO Phase 3 study in children with CF 2 to 5 years of age at the North American Cystic Fibrosis Conference (NACFC) in November 2022. Vertex is on track to submit global filings for TRIKAFTA/KAFTRIO in children with CF 2 to 5 years of age before the end of 2022.
- On track to submit global filings for KALYDECO in children with CF from 1 month to less than 4 months of age before the end of 2022.

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.

- Vertex is conducting two Phase 3 global, randomized, double-blind, active-controlled clinical trials evaluating Vertex's new once-daily investigational triple combination of vanzacaftor/tezacaftor/deutivacaftor, formerly known as VX-121/tezacaftor /VX-561, in patients with CF 12 years of age and older. The SKYLINE 102 and SKYLINE 103 trials will compare the efficacy and safety of vanzacaftor/tezacaftor/deutivacaftor to TRIKAFTA. Enrollment in both trials is on track to be completed before the end of 2022.
- In parallel to SKYLINE 102 and SKYLINE 103, Vertex has also initiated a study of vanzacaftor/tezacaftor/deutivacaftor in children with CF 6 to 11 years of age, which is ongoing.
- In collaboration with Moderna, Vertex is developing a CFTR mRNA therapeutic 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 Q4 2022.

Beta Thalassemia and Sickle Cell Disease

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

- Vertex concluded discussions with the U.S. Food and Drug Administration (FDA), and the FDA granted exa-cel a rolling review, for which Vertex will submit its biologics licensing application (BLA) beginning in November 2022. Vertex expects to complete the submission by the end of Q1 2023. In the U.S., exa-cel has been granted Fast Track, Regenerative Medicine Advanced Therapy (RMAT), Rare Pediatric Disease and Orphan Drug designations.
- European/U.K. (EMA and MHRA) submissions remain on track for Q4 2022, and exa-cel has been granted EMA Priority Medicines (PRIME) designation in Europe and Orphan Drug designation in Europe and the U.K.
- Two additional Phase 3 studies of exa-cel in pediatric patients with TDT and SCD are ongoing.

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 standard-of-care pain medicines.

- Vertex reached agreement with the FDA on the Phase 3 pivotal program design for its lead compound, VX-548, for moderate to severe acute pain. The pivotal program has been initiated and will enroll a total of 2,000 patients with moderate to severe acute pain across two randomized controlled trials with treatment for 48 hours following bunionectomy or abdominoplasty surgery. An additional 250-patient single-arm study will evaluate the safety and effectiveness of VX-548 in multiple other types of moderate to severe acute pain with a treatment period of up to 14 days.
- VX-548 has been granted Fast Track and Breakthrough Therapy Designation in the U.S. for moderate to severe acute pain.
- Vertex also remains on track to initiate a Phase 2 study of VX-548 in neuropathic pain by year-end 2022.

APOL1-Mediated Kidney Disease (AMKD)

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

- Vertex continues to enroll the pivotal program for its lead compound, inaxaplin, formerly known as VX-147, in a single Phase 2/3 clinical trial in patients with AMKD.
- Inaxaplin was granted breakthrough therapy designation by the FDA for APOL1-mediated focal segmental

glomerulosclerosis (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 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.

- Proof of concept has been achieved in the VX-880 program, with the first two patients treated with half the targeted dose.
- Vertex continues to enroll patients in Part B of the Phase 1/2 study.
- Vertex remains on track to submit an IND for the cells plus device program in Q4 2022.

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 the goal of increasing the secretion of functional AAT into the blood and addressing both the lung and the liver aspects of AAT deficiency.

- Vertex recently initiated a first-in-human clinical trial for VX-634, a small molecule AAT corrector in healthy volunteers. 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, allowing potential exploration of the full dose response.
- Additionally, Vertex will soon initiate 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 levels of functional AAT (fAAT) in the plasma.

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.

- IND-enabling studies for the first *in vivo* gene editing therapy for DMD are underway. Vertex anticipates submitting an IND in 2023.

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

Investments in External Innovation

Consistent with its strategy to develop transformative medicines for serious diseases, on September 27, 2022, Vertex closed the acquisition of ViaCyte, a regenerative medicine company focused on delivering novel stem cell-derived cell replacement therapies as a potential functional cure for type 1 diabetes.

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. 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Revenues:				
Product revenues, net	$ 2,334.3	$ 1,984.1	$ 6,628.0	$ 5,500.8
Other revenues	—	—	—	1.0
Total revenues	2,334.3	1,984.1	6,628.0	5,501.8
Costs and expenses:				
Cost of sales	289.4	236.5	797.0	656.8
Research and development expenses	645.0	467.0	1,846.2	1,370.0
Acquired in-process research and development expenses (3)	29.0	26.7	92.9	986.8
Selling, general and administrative expenses	246.8	198.2	677.3	584.9
Change in fair value of contingent consideration	(2.6)	1.2	(59.3)	(1.1)
Total costs and expenses	1,207.6	929.6	3,354.1	3,597.4
Income from operations	1,126.7	1,054.5	3,273.9	1,904.4
Interest income	46.2	1.1	58.6	3.7
Interest expense	(13.7)	(15.2)	(43.2)	(46.4)
Other income (expense), net	17.2	42.4	(133.7)	(2.2)
Income before provision for income taxes	1,176.4	1,082.8	3,155.6	1,859.5
Provision for income taxes	245.9	230.9	652.5	287.5
Net income	$ 930.5	$ 851.9	$ 2,503.1	$ 1,572.0
Net income per common share:				
Basic	$ 3.63	$ 3.30	$ 9.78	$ 6.08
Diluted	$ 3.59	$ 3.28	$ 9.68	$ 6.03
Shares used in per share calculations:				
Basic	256.5	257.9	255.8	258.7
Diluted	259.5	259.7	258.7	260.9

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
GAAP net income	$ 930.5	$ 851.9	$ 2,503.1	$ 1,572.0
Stock-based compensation expense	135.6	103.0	379.8	322.8
(Increase) decrease in fair value of strategic investments(4)	(16.7)	(46.7)	143.1	(5.0)
(Decrease) increase in fair value of contingent consideration (5)	(2.6)	1.2	(59.3)	(1.1)
Intangible asset impairment charge (5)	—	—	13.0	—
Acquisition-related costs (6)	29.7	2.9	35.3	8.5
Total non-GAAP adjustments to pre-tax income *	146.0	60.4	511.9	325.2
Tax adjustments (1) *	(37.1)	(0.8)	(138.0)	(161.1)
Non-GAAP net income *	$ 1,039.4	$ 911.5	$ 2,877.0	$ 1,736.1
Net income per diluted common share:				
GAAP	$ 3.59	$ 3.28	$ 9.68	$ 6.03
Non-GAAP *	$ 4.01	$ 3.51	$ 11.12	$ 6.66
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	259.5	259.7	258.7	260.9

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
GAAP operating income	$ 1,126.7	$ 1,054.5	$ 3,273.9	$ 1,904.4
Stock-based compensation expense	135.6	103.0	379.8	322.8
(Decrease) increase in fair value of contingent consideration (5)	(2.6)	1.2	(59.3)	(1.1)
Intangible asset impairment charge (5)	—	—	13.0	—
Acquisition-related costs (6)	29.7	2.9	35.3	8.5
Non-GAAP operating income *	$ 1,289.4	$ 1,161.6	$ 3,642.7	$ 2,234.6

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
GAAP cost of sales	$ 289.4	$ 236.5	$ 797.0	$ 656.8
Stock-based compensation expense	(2.4)	(1.6)	(7.0)	(4.6)
Non-GAAP cost of sales	$ 287.0	$ 234.9	$ 790.0	$ 652.2
GAAP research and development expenses	$ 645.0	$ 467.0	$ 1,846.2	$ 1,370.0
Stock-based compensation expense	(80.0)	(61.0)	(229.9)	(196.4)
Intangible asset impairment charge (5)	—	—	(13.0)	—
Acquisition-related costs (6)	(16.5)	(2.9)	(22.1)	(8.5)
Non-GAAP research and development expenses *	$ 548.5	$ 403.1	$ 1,581.2	$ 1,165.1
Acquired in-process research and development expenses *	$ 29.0	$ 26.7	$ 92.9	$ 986.8
GAAP selling, general and administrative expenses	$ 246.8	$ 198.2	$ 677.3	$ 584.9
Stock-based compensation expense	(53.2)	(40.4)	(142.9)	(121.8)
Acquisition-related costs (6)	(13.2)	—	(13.2)	—
Non-GAAP selling, general and administrative expenses	$ 180.4	$ 157.8	$ 521.2	$ 463.1
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses *	$ 757.9	$ 587.6	$ 2,195.3	$ 2,615.0
GAAP other income (expense), net	$ 17.2	$ 42.4	$ (133.7)	$ (2.2)
(Increase) decrease in fair value of strategic investments(4)	(16.7)	(46.7)	143.1	(5.0)
Non-GAAP other income (expense), net	$ 0.5	$ (4.3)	$ 9.4	$ (7.2)
GAAP provision for income taxes	$ 245.9	$ 230.9	$ 652.5	$ 287.5
Tax adjustments (1) *	37.1	0.8	138.0	161.1
Non-GAAP provision for income taxes *	$ 283.0	$ 231.7	$ 790.5	$ 448.6
GAAP effective tax rate	21%	21%	21%	15%
Non-GAAP effective tax rate	21%	20%	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. These charges are included as "Acquired in-process research and development expenses," and were previously included in "Research and development expenses," in Vertex's consolidated statements of operations. Non-GAAP financial measures for the three and nine months ended September 30, 2021 have been recast to reflect this change.

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

	September 30, 2022	December 31, 2021
Assets		
Cash, cash equivalents and marketable securities	$ 9,770.7	$ 7,524.9
Accounts receivable, net	1,385.2	1,136.8
Inventories	388.2	353.1
Property and equipment, net	1,118.7	1,094.1
Goodwill and intangible assets	1,678.8	1,402.2
Deferred tax assets	1,162.7	934.5
Other assets	1,202.1	986.9
Total assets	$ 16,706.4	$ 13,432.5
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 2,391.3	$ 1,873.6
Finance lease liabilities	482.0	556.7
Contingent consideration	127.2	186.5
Other liabilities	676.3	715.7
Shareholders' equity	13,029.6	10,100.0
Total liabilities and shareholders' equity	$ 16,706.4	$ 13,432.5
Common shares outstanding	256.6	254.5

Notes and Explanations

1: In the three and nine months ended September 30, 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. "Tax adjustments" in the nine months ended September 30, 2021 also included a $100 million discrete tax benefit related to an increase in the U.K.'s corporate tax rate from 19% to 25%, which was enacted in June 2021 and will become effective in April 2023.

2: The difference between the company’s full year 2022 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 $460 million to $485 million of stock-based compensation expense. 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: Vertex classifies upfront, contingent milestone, and other payments pursuant to its business development transactions, including collaborations, licenses of third-party technologies, and asset acquisitions as “Acquired in-process research and development expenses” in its consolidated statements of operations. These amounts were previously classified as "Research and development expenses." To conform prior periods to current presentation, the company reclassified $27 million and $987 million from “Research and development expenses” to “Acquired in-process research and development expenses” for the three and nine months ended September 30, 2021, respectively. In the nine months ended September 30, 2021, "Acquired in-process research and development expenses" primarily related to the $900 million upfront payment to CRISPR.

4: "Other income (expense), net" includes net gains and losses related to changes in the fair value of the company's strategic investments.

5: In June 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.

6: "Acquisition-related costs" in the three and nine months ended September 30, 2022 and 2021 related to costs associated with the company's acquisitions 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 pipeline of investigational small molecule, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

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 Best Workplaces in Biotechnology and Pharmaceuticals and Best Workplaces for Women. 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 people eligible and treated with our CF medicines, including children newly eligible for our CF medicines, and expansion of treatment options for the patients who cannot benefit from CFTR modulators alone, (iv) expectations regarding our trials evaluating our once-daily investigational triple combination of vanzacaftor/tezacaftor/deutivacaftor, including study design and enrollment expectations, (v) expectations regarding our collaboration with Moderna to develop CFTR mRNA therapeutics, including our plans to submit an IND for this program in the fourth quarter of 2022, (vi) expectations regarding anticipated regulatory submissions in the U.S., Europe and the U.K. for exa-cel, including the timing of the regulatory submissions, and two additional Phase 3 studies of exa-cel in pediatric patients, (vii) expectations regarding the potential benefits of our pain program and products, plans for the advancement of VX-548 into a Phase 3 program in acute pain, including study designs and enrollment expectations, and plans to initiate a Phase 2 study in neuropathic pain by the end of 2022, (viii) expectations regarding the potential benefits of our AMKD program, plans regarding our Phase 2/3 study of inaxaplin in AMKD and enrollment expectations, (ix) the potential benefits and safety of VX-880, and our plans to continue to progress the Phase 1/2 program for VX-880, including patient enrollment, and our plans regarding our additional programs in T1D, including our expectations to submit an IND for the cells plus device program in the fourth quarter of 2022, (x) our expectations regarding the potential benefits of our AAT deficiency program and plans to advance VX-634 and VX-864 in clinical trials, (xi) our plans regarding our DMD program, including our expectations to submit an IND for this program in 2023, and (xii) our investments in external innovation, including expectations with regard to recent acquisitions. 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 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, 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 Third 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.

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