Vertex Reports Full-Year and Fourth-Quarter 2020 Financial Results

February 1, 2021

-Full-year 2020 GAAP product revenues of $6.20 billion-

-Full-year 2020 non-GAAP product revenues of $6.20 billion, a 55% increase compared to full-year 2019-

-Company provides full-year 2021 product revenue guidance of $6.7 to $6.9 billion-

BOSTON--(BUSINESS WIRE)--Feb. 1, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the full year and fourth quarter ended December 31, 2020 and provided full-year 2021 financial guidance.

"Our achievements in 2020 were marked by a significant increase in the number of people treated with the triple combination in the U.S. and the EU. It was also a year marked by meaningful pipeline advancement. We now have clinical programs in seven disease areas, spanning multiple modalities including small molecules for alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases, gene editing for sickle cell disease and beta thalassemia, and cell therapy for type 1 diabetes," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "As we enter 2021, we look forward to treating more CF patients and reaching important R&D milestones in multiple additional diseases which will fuel our growth this year and for many years to come."

Fourth-Quarter and Full-Year 2020 Financial Highlights

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2020	2019	% Change	2020	2019	% Change
	(in millions, except per share amounts)					
GAAP Product revenues, net	$ 1,627	$ 1,413	15%	$ 6,203	$ 4,161	49%
Non-GAAP Product revenues, net (1)	$ 1,627	$ 1,257	29%	$ 6,203	$ 4,005	55%
GAAP Operating income	$ 746	$ 551	35%	$ 2,856	$ 1,198	139%
Non-GAAP Operating income	$ 887	$ 593	50%	$ 3,491	$ 1,786	95%
GAAP Net income	$ 604	$ 583	4%	$ 2,712	$ 1,177	130%
Non-GAAP Net income	$ 661	$ 444	49%	$ 2,719	$ 1,389	96%
GAAP Net income per share - diluted	$ 2.30	$ 2.23	3%	$ 10.29	$ 4.51	128%
Non-GAAP Net income per share - diluted	$ 2.51	$ 1.70	48%	$ 10.32	$ 5.33	94%

Full-Year 2020 Results

	Twelve Months Ended December 31,	
	2020	2019
	(in millions)	
GAAP Product revenues, net	$ 6,203	$ 4,161
Non-GAAP Product revenues, net (1)	$ 6,203	$ 4,005
TRIKAFTA/KAFTRIO	$ 3,864	$ 420
SYMDEKO/SYMKEVI	$ 629	$ 1,418
ORKAMBI	$ 908	$ 1,176
KALYDECO	$ 803	$ 991

GAAP and Non-GAAP product revenues increased 49% and 55%, respectively, compared to 2019, primarily driven by the uptake of TRIKAFTA in the U.S., KAFTRIO in Europe and our other medicines outside the U.S. following the completion of several significant reimbursement agreements. Net product revenues in 2020 were $4.8 billion in the U.S. and $1.4 billion outside the U.S.

GAAP and Non-GAAP net income increased compared to 2019, largely driven by strong growth in product revenues.

Cash, cash equivalents and marketable securities as of December 31, 2020 were $6.7 billion, an increase of approximately $2.9 billion compared to $3.8 billion as of December 31, 2019 driven by strong revenue and profitability.

Full-Year 2020 Expenses

	Twelve Months Ended December 31,	
	2020	2019
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 2,600	$ 2,413
Combined Non-GAAP R&D and SG&A expenses	$ 1,981	$ 1,694
GAAP R&D expense	$ 1,830	$ 1,755
Non-GAAP R&D expense	$ 1,372	$ 1,171
GAAP SG&A expense	$ 770	$ 658
Non-GAAP SG&A expense	$ 609	$ 523
GAAP income taxes	$ 405	$ 218
Non-GAAP income taxes	$ 721	$ 397
GAAP effective tax rate	13%	16%
Non-GAAP effective tax rate (2)	21%	22%

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to 2019, primarily due to the incremental investment to support the global use of Vertex's medicines and the expansion of Vertex's pipeline in CF and other disease areas.

GAAP and Non-GAAP income taxes increased compared to 2019 primarily due to Vertex's increased operating income. Refer to the "Supplemental Income Tax Information" section for discussion of the cash versus non-cash components of Vertex's provision for income taxes.

Fourth-Quarter 2020 Results

	Three Months Ended December 31,	
	2020	2019
	(in millions)	
GAAP Product revenues, net	$ 1,627	$ 1,413
Non-GAAP Product revenues, net (1)	$ 1,627	$ 1,257
TRIKAFTA/KAFTRIO	$ 1,091	$ 420
SYMDEKO/SYMKEVI	$ 128	$ 332
ORKAMBI	$ 215	$ 270
KALYDECO	$ 193	$ 236

GAAP and Non-GAAP product revenues increased 15% and 29%, respectively, compared to the fourth quarter of 2019, primarily driven by the uptake of TRIKAFTA in the U.S., KAFTRIO in Europe and our other medicines outside the U.S. following the completion of several significant reimbursement agreements.

GAAP and non-GAAP net income increased compared to the fourth quarter of 2019, largely driven by strong growth in total product revenues.

Fourth-Quarter 2020 Expenses

	Three Months Ended December 31,	
	2020	2019
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 678	$ 675
Combined Non-GAAP R&D and SG&A expenses	$ 539	$ 496
GAAP R&D expense	$ 467	$ 480
Non-GAAP R&D expense	$ 364	$ 337

GAAP SG&A expense	$	212	$	195
Non-GAAP SG&A expense	$	175	$	159
GAAP income taxes	$	284	$	94
Non-GAAP income taxes	$	198	$	145
GAAP effective tax rate		32%		14%
Non-GAAP effective tax rate (2)		23%		25%

Combined GAAP R&D and SG&A expenses were similar to the fourth quarter of 2019.

Combined Non-GAAP R&D and SG&A expenses increased compared to the fourth quarter of 2019, primarily due to the incremental investment to support the global use of Vertex's medicines and the expansion of Vertex's pipeline in CF and other disease areas.

GAAP and Non-GAAP income taxes increased compared to the fourth quarter of 2019 primarily due to Vertex's increased operating income. Refer to the "Supplemental Income Tax Information" section for discussion of the cash versus non-cash components of Vertex's provision for income taxes.

Full-Year 2021 Financial Guidance

Vertex today provided its full-year 2021 financial guidance. Product revenue guidance is primarily based on:

- The continued strong performance of TRIKAFTA in the U.S. and KAFTRIO in certain European countries
- The launch of medicines in the U.S. for rare mutations following approval in December 2020 and the approval of TRIKAFTA for children with CF ages 6-11 in the U.S. expected mid-year
- Countries where patients currently have access or reimbursement

Vertex's guidance is summarized below:

	FY 2021
Product revenues	$6.7 to 6.9 billion
Combined GAAP R&D and SG&A expenses (3)	$2.9 to 3.05 billion
Combined Non-GAAP R&D and SG&A expenses (3)	$2.25 to 2.3 billion
Non-GAAP effective tax rate	21% to 22%

Key Business Highlights

Cystic Fibrosis (CF) R&D pipeline

Vertex expects to increase the number of CF patients eligible to take our medicines and thereby continue to grow our CF business. Important progress has been made in increasing the number of eligible patients and expanding approval and access for our medicines to additional geographies and age groups.

TRIKAFTA/KAFTRIO (elexacaftor, tezacaftor and ivacaftor)

- The U.S. Food and Drug Administration (FDA) expanded the eligibility for TRIKAFTA to include people with CF ages 12 and older with certain mutations that are responsive to TRIKAFTA based on *in vitro* data. SYMDEKO and KALYDECO also received approvals to include additional responsive mutations in people with CF ages 6 and older and ages 4 months and older, respectively.
- Swissmedic, the Swiss Agency for Therapeutic Products, granted marketing authorization and a reimbursement agreement was reached for TRIKAFTA in Switzerland in people with CF ages 12 and older with two *F508del* mutations or one *F508del* mutation and one minimal function mutation.
- The U.S. FDA accepted a supplemental New Drug Application (sNDA) for TRIKAFTA for the treatment of children with CF ages 6 to 11 who have at least one *F508del* mutation or have certain mutations that are responsive to TRIKAFTA based on *in vitro* data. The FDA granted Priority Review of the sNDA and assigned a Prescription Drug User Fee Act (PDUFA) target action date of June 8, 2021.
- Health Canada accepted a New Drug Submission for Priority Review for TRIKAFTA for the treatment of people with CF ages 12 years and older.

SYMDEKO/SYMKEVI (tezacaftor and ivacaftor)

- The European Commission (EC) granted approval of the label extension for SYMKEVI to include people with CF ages 6 years and older with two copies of the *F508del* mutation or one copy of the *F508del* mutation and certain residual function mutations.

KALYDECO (ivacaftor)

- The EC granted approval of the label extension for KALYDECO to include the treatment of infants with CF ages 4 months and older who have *R117H* or certain gating mutations.

R&D pipeline outside of CF

Vertex continues to progress a broad pipeline of potentially transformative small molecule, cell and genetic therapies aimed at serious diseases. Recent and anticipated progress for key pipeline programs is noted below:

Beta Thalassemia and Sickle Cell Disease

- Vertex and its partner CRISPR Therapeutics are evaluating the use of an *ex vivo* CRISPR gene-edited therapy for the treatment of transfusion-dependent beta thalassemia (TDT) and sickle cell disease (SCD). This approach aims to edit a person's hematopoietic stem cells to produce fetal hemoglobin in red blood cells, which has the potential to reduce or eliminate symptoms associated with disease.
- Enrollment and dosing are ongoing in the clinical studies for CTX001. More than 20 patients have been dosed with CTX001 across both studies to date. Completion of enrollment in both studies is expected in 2021.

Alpha-1 Antitrypsin (AAT) Deficiency

- Vertex is evaluating multiple compounds with the potential to correct the misfolding of Z-AAT protein in the liver, in order to increase the levels of functional AAT in the blood. Misfolded Z-AAT protein is the root cause of AAT deficiency.
- Enrollment is ongoing in a Phase 2 proof-of-concept study for the Z-AAT corrector, VX-864. Data from this study is expected in the first half of 2021.

APOL1-mediated Kidney Diseases

- Vertex is evaluating the potential for inhibitors of APOL1 function to reduce proteinuria in people with serious kidney diseases, including focal segmental glomerulosclerosis (FSGS).
- Enrollment is ongoing in a Phase 2 proof-of-concept study designed to evaluate the reduction in proteinuria in people with APOL1-mediated FSGS after treatment with VX-147. Data from this study is expected in 2021.

Type 1 Diabetes (T1D)

- Vertex is developing a cell therapy designed to replace insulin-producing islet cells in people with T1D. Two opportunities exist for the transplant of these functional islets into patients: 1) transplantation of islet cells alone, using immunosuppression to protect the implanted cells and 2) implantation of the islet cells inside a novel immunoprotective device.
- The U.S. FDA cleared the Investigational New Drug Application (IND) for VX-880, the islet cells alone program. Vertex expects to initiate a Phase 1/2 clinical trial in the first half of 2021.

Investments in External Innovation

- Skyhawk Therapeutics and Vertex established a strategic collaboration to discover and develop novel small molecules that modulate RNA splicing for the treatment of serious diseases.

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) an adjustment to product revenues and related cost of sales to reflect the conclusion of the early access program for ORKAMBI in France in the fourth quarter of 2019, (iii) revenues and expenses related to collaboration agreements, (iv) gains or losses related to the fair value of the company's strategic investments, (v) increases or decreases in the fair value of contingent consideration, (vi) acquisition-related costs and (vii) other adjustments. The company's non-GAAP financial results also exclude from its provision for income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income described above and certain discrete items. These results should not be viewed as a substitute for the company's GAAP results and are provided as a complement to results provided in accordance with GAAP. Management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position that the company believes is helpful to an understanding of its ongoing business. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, to manage the company's business and to evaluate its performance. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. 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 R&D and SG&A expenses does not include estimates associated with any potential future business development activities. The company does not provide a 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
Fourth-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
Revenues:				
Product revenues, net	$1,626,920	$1,413,265	$6,202,783	$4,160,726
Collaboration and royalty revenues	900	—	2,900	2,095
Total revenues	1,627,820	1,413,265	6,205,683	4,162,821
Costs and expenses:				
Cost of sales	203,101	185,012	736,300	547,758
Research and development expenses	466,584	480,011	1,829,537	1,754,540
Sales, general and administrative expenses	211,843	195,277	770,456	658,498
Change in fair value of contingent consideration	500	1,500	13,100	4,459
Total costs and expenses	882,028	861,800	3,349,393	2,965,255
Income from operations	745,792	551,465	2,856,290	1,197,566
Interest income	2,320	12,359	22,239	63,678
Interest expense	(16,288)	(14,249)	(58,151)	(58,502)
Other income, net (4)	156,799	127,375	296,420	192,177
Income before provision for income taxes	888,623	676,950	3,116,798	1,394,919
Provision for income taxes	284,433	93,716	405,151	218,109
Net income	$ 604,190	$ 583,234	$2,711,647	$1,176,810
Net income per common share:				
Basic	$ 2.32	$ 2.26	$ 10.44	$ 4.58
Diluted	$ 2.30	$ 2.23	$ 10.29	$ 4.51
Shares used in per share calculations:				
Basic	260,038	258,003	259,841	256,728
Diluted	263,106	262,108	263,396	260,673

Reconciliation of GAAP to Non-GAAP Net Income
Fourth-Quarter Results
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
GAAP net income	$ 604,190	$ 583,234	$ 2,711,647	$ 1,176,810
Stock-based compensation expense	97,027	91,591	429,461	360,489
ORKAMBI Adjustment (1)	—	(140,854)	—	(140,854)
Increase in fair value of strategic investments (4)	(171,071)	(128,734)	(311,937)	(197,596)
Increase in fair value of contingent consideration (5)	500	1,500	13,100	4,459

Collaborative revenues and expenses (6)	40,400	56,057	181,700	318,343
Acquisition-related costs (7)	2,820	33,181	10,682	45,871
Total non-GAAP adjustments to pre-tax income	(30,324)	(87,259)	323,006	390,712
Tax adjustments (2)	86,728	(51,627)	(315,455)	(178,578)
Non-GAAP net income	$ 660,594	$ 444,348	$ 2,719,198	$ 1,388,944
Net income per diluted common share:				
GAAP	$ 2.30	$ 2.23	$ 10.29	$ 4.51
Non-GAAP	$ 2.51	$ 1.70	$ 10.32	$ 5.33
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	263,106	262,108	263,396	260,673

Reconciliation of GAAP to Non-GAAP Revenues and Expenses
Fourth-Quarter Results
(in thousands)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
GAAP total revenues	$ 1,627,820	$ 1,413,265	$ 6,205,683	$ 4,162,821
ORKAMBI Adjustment (1)	—	(155,773)	—	(155,773)
Collaborative revenues	(900)	—	(2,900)	(158)
Non-GAAP total revenues	$ 1,626,920	$ 1,257,492	$ 6,202,783	$ 4,006,890

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
GAAP cost of sales	$ 203,101	$ 185,012	$ 736,300	$ 547,758
ORKAMBI Adjustment (1)	—	(14,919)	—	(14,919)
Stock-based compensation expense	(1,581)	(1,397)	(5,579)	(5,575)
Non-GAAP cost of sales	$ 201,520	$ 168,696	$ 730,721	$ 527,264
GAAP research and development expenses	$ 466,584	$ 480,011	$ 1,829,537	$ 1,754,540
Stock-based compensation expense	(58,958)	(56,707)	(262,690)	(224,558)
Collaborative expenses (6)	(41,300)	(56,057)	(184,600)	(318,501)
Acquisition-related costs (7)	(2,820)	(30,461)	(10,229)	(40,583)
Non-GAAP research and development expenses	$ 363,506	$ 336,786	$ 1,372,018	$ 1,170,898
GAAP sales, general and administrative expenses	$ 211,843	$ 195,277	$ 770,456	$ 658,498
Stock-based compensation expense	(36,488)	(33,487)	(161,192)	(130,356)
Acquisition-related costs (7)	—	(2,720)	(453)	(5,288)
Non-GAAP sales, general and administrative expenses	$ 175,355	$ 159,070	$ 608,811	$ 522,854
Combined non-GAAP R&D and SG&A expenses	$ 538,861	$ 495,856	$ 1,980,829	$ 1,693,752

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
GAAP other income, net	$ 156,799	$ 127,375	$ 296,420	$ 192,177
Increase in fair value of strategic investments (4)	(171,071)	(128,734)	(311,937)	(197,596)
Non-GAAP other expense, net	$ (14,272)	$ (1,359)	$ (15,517)	$ (5,419)

GAAP provision for income taxes	$ 284,433	$ 93,716	$ 405,151	$ 218,109
Tax adjustments (2)	(86,728)	51,627	315,455	178,578
Non-GAAP provision for income taxes (8)	$ 197,705	$ 145,343	$ 720,606	$ 396,687

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	December 31, 2020	December 31, 2019
Assets		
Cash, cash equivalents and marketable securities	$ 6,658,897	$ 3,808,294
Accounts receivable, net	885,352	633,518
Inventories	280,777	167,502
Property and equipment, net	958,534	745,080
Goodwill and intangible assets	1,402,158	1,402,158
Deferred tax assets	882,779	1,190,815
Other assets	683,311	371,098
Total assets	$ 11,751,808	$ 8,318,465
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 1,560,110	$ 1,204,522
Finance lease liabilities	581,476	577,371
Contingent consideration	189,600	176,500
Other liabilities	733,807	274,828
Shareholders' equity	8,686,815	6,085,244
Total liabilities and shareholders' equity	$ 11,751,808	$ 8,318,465
Common shares outstanding	259,890	258,993

Supplemental Income Tax Information
(in thousands, except percentages)
(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019**
Components of provision for income taxes related to:				
Cash paid or accrued for income taxes	$ 122,975	$ 9,185	$ 168,322	$ 32,138
Benefits from income taxes due to discrete tax items (2)	10,034	—	(287,565)	—
Provision for income taxes offset by net operating losses and credits (8)	151,424	84,531	524,394	185,971
GAAP provision for income taxes (8)	$ 284,433	$ 93,716	$ 405,151	$ 218,109
Cash paid or accrued for income taxes	$ 122,975	$ 9,185	$ 168,322	$ 32,138
Adjustments to pre-tax income	(76,694)	51,627	27,890	178,578
Provision for income taxes offset by net operating losses and credits (8)	151,424	84,531	524,394	185,971
Non-GAAP provision for income taxes (8)	$ 197,705	$ 145,343	$ 720,606	$ 396,687
Effective tax rate reconciliation:				
GAAP effective tax rate	32%	14%	13%	16%
Impact of GAAP to Non-GAAP adjustments	(9)%	11%	8%	6%

Non-GAAP effective tax rate	23%	25%	21%	22%

Notes and Explanations

1: "ORKAMBI adjustment" in the company's Reconciliation of GAAP to Non-GAAP Net Income in the three and twelve months ended December 31, 2019 included an adjustment to net product revenues and cost of sales related to the conclusion of the early access program for ORKAMBI in France in the fourth quarter of 2019. The company had previously recognized a portion of net product revenues related to ORKAMBI distributed through the early access program in France. As a result, the company recognized an adjustment to increase net product revenues by $155.8 million and cost of sales by $14.9 million, which related to prior period shipments of ORKAMBI distributed through the early access program in France. The company excluded the adjustment to net product revenues and cost of sales from its Non-GAAP measures for the three and twelve months ended December 31, 2019.

2: In the three and twelve months ended December 31, 2020 and 2019, "Tax adjustments" primarily related to the estimated income taxes related to non-GAAP adjustments to pre-tax income including (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation), (ii) increases or decreases in the fair value of the company's strategic investments and (iii) collaborative payments. In the twelve months ended December 31, 2020, "Tax adjustments" also included non-recurring discrete benefits to the company's provision for income taxes, such as the transfer of intellectual property rights to the company's U.K. entity, of approximately $287.6 million that the company excluded from its Non-GAAP measures.

3: The difference between the company's full-year 2021 combined GAAP R&D and SG&A expenses and combined non-GAAP R&D and SG&A expenses guidance relates primarily to $430 million to $500 million of stock-based compensation expense and $200 million to $250 million of R&D expenses related to existing collaboration agreements. The guidance regarding combined GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development activities.

4: "Other income, net" includes gains related to changes in the fair value of the company's strategic investments and from sales of certain investments.

5: During the three and twelve months ended December 31, 2020 and 2019, the increase in the fair value of contingent consideration relates to potential payments to Exonics Therapeutics' former equity holders.

6: "Collaborative revenues and expenses" in the three and twelve months ended December 31, 2020 and 2019 primarily related to collaborative upfront and milestone payments.

7: "Acquisition-related costs" in the three and twelve months ended December 31, 2020 and 2019 related to costs associated with the company's acquisitions of Semma Therapeutics and Exonics.

8: The company records a provision for income taxes on its pre-tax income using an effective tax rate approximating statutory rates. Since the company released its valuation allowance on the majority of its net operating losses and other deferred tax assets as of December 31, 2018, its tax provision has included a significant non-cash charge due to the company's ability to offset its pre-tax income against previously benefited net operating losses and credits. As of December 31, 2019, the company had federal net operating losses and credits that were available to offset future pre-tax income. The company utilized substantially all of its remaining federal net operating losses in 2020. As a result, a larger portion of the company's tax provision will represent a cash expense in future periods, subject to continued utilization of certain tax credits.

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 diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. 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, 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 2021 Financial Guidance" and statements regarding (i) anticipated regulatory filings and data submissions, (ii) anticipated future label expansions, (iii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including collaborations with third parties, (iv) expectations for the collaborations with CRISPR, including expectations regarding completion of enrollment, (v) expectations for uptake of and expanded access to the company's medicines, including additional reimbursement agreements, and (vi) anticipated investment in internal and external innovation. 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 2021 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 COVID-19 may have different or more significant impacts on the company's business or operations than the company currently expects, data from preclinical testing or early clinical trials, especially if based on a limited number of patients, may not be indicative of final results, that the FDA may not approve VX-880 on a timely basis, or at all, 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 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. 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 today at 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). 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 under "Events and Presentations." To ensure a timely connection, it is recommended that users 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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