Vertex Reports First Quarter 2024 Financial Results

May 6, 2024

— Product revenue of $2.69 billion, a 13% increase compared to Q1 2023; reiterated full year 2024 financial guidance, including product revenue guidance of $10.55 to $10.75 billion —

— Submitted NDA and MAA filings for vanzacaftor triple in CF to FDA and EMA, respectively —

— Initiated rolling NDA submission for suzetrigine (VX-548) in moderate-to-severe acute pain and on track to complete this quarter —

— Entered into agreement to acquire Alpine Immune Sciences, including povetacicept, a Phase 3-ready asset in IgA nephropathy and potential pipeline-in-a-product —

BOSTON--(BUSINESS WIRE)--May 6, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2024, and reiterated full year 2024 financial guidance.

"Vertex delivered a strong start to 2024 with 13 percent product revenue growth and outstanding execution across the business. This quarter, we continued to expand our leadership in CF including completion of the regulatory submissions for the vanzacaftor triple, advanced the global launch of CASGEVY ™, and initiated the rolling submission for suzetrigine in moderate-to-severe acute pain, while progressing our broad and deep pipeline of potentially transformative medicines," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "We also recently announced the acquisition of Alpine Immune Sciences, a compelling strategic fit. We look forward to welcoming the Alpine team and together accelerating the development of povetacicept, a potential best-in-class treatment for IgAN, and fully exploring povetacicept's pipeline-in-a-product potential."

First Quarter 2024 Results

Product revenue increased 13% to $2.69 billion compared to the first quarter of 2023, primarily driven in ex-U.S. markets by the continued strong uptake of TRIKAFTA®/KAFTRIO®, including label extensions in younger age groups, and in the U.S. by the continued performance of TRIKAFTA, including the uptake in children with CF 2 to 5 years of age. Net product revenue in the first quarter of 2024 increased 8% to $1.52 billion in the U.S. and increased 21% to $1.17 billion outside the U.S., compared to the first quarter of 2023.

Combined GAAP and Non-GAAP R&D, Acquired IPR&D and SG&A expenses were $1.2 billion and $1.0 billion, respectively, compared to $1.3 billion and $1.2 billion, respectively, in the first quarter of 2023. The decreases were due to lower Acquired IPR&D expenses partially offset by increased investments to support launches of Vertex's therapies globally and continued investment in support of multiple programs that have advanced in mid- and late-stage clinical development.

GAAP effective tax rate was 14.0% compared to 21.5% for the first quarter of 2023, primarily due to a discrete adjustment to Vertex's income tax reserves as well as tax benefits related to stock-based compensation.

Non-GAAP effective tax rate was 17.4% compared to 21.3% for the first quarter of 2023, primarily due to a discrete adjustment to Vertex's income tax reserves. Please refer to Note 1 for further details on Vertex's GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net income increased by 57% and 56%, respectively, compared to the first quarter of 2023, primarily due to higher product revenues and lower Acquired IPR&D expenses.

Cash, cash equivalents and total marketable securities as of March 31, 2024 were $14.6 billion, compared to $13.7 billion as of December 31, 2023. The increase was primarily due to cash from operations driven by strong revenue growth, partially offset by business development payments, and repurchases of Vertex's common stock.

Full Year 2024 Financial Guidance

Vertex today reiterated its full year 2024 financial guidance, including product revenue guidance of $10.55 to $10.75 billion. Vertex's product revenue guidance includes expectations for continued growth in CF as well as for the launch of CASGEVY in approved indications and geographies. Vertex's combined Non-GAAP R&D, Acquired IPR&D and SG&A expense guidance of $4.3 billion to $4.4 billion includes expectations for continued investment in multiple mid- and late-stage clinical development programs, commercial and manufacturing capabilities, and approximately $125 million of upfront and milestone payments. The recently announced acquisition of Alpine Immune Sciences is expected to close in the second quarter. Vertex does not anticipate adjusting

its guidance for Alpine's operating expenses, other than the potential impact of purchase accounting.

Vertex's financial guidance is summarized below:

	Current FY 2024	Previous FY 2024
Total product revenues	Unchanged	$10.55 to $10.75 billion
Combined GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$4.9 to $5.1 billion
Combined Non-GAAP R&D, Acquired IPR&D and SG&A expenses (2)	Unchanged	$4.3 to $4.4 billion
Non-GAAP effective tax rate	Unchanged	20% to 21%

Key Business Highlights

Marketed Products and Potential Near-Term Launch Opportunities

Cystic Fibrosis (CF) Portfolio

Vertex anticipates the number of CF patients taking our medicines will continue to grow through new approvals and reimbursement for the treatment of younger patients. Recent and anticipated progress includes:

- The European Commission has granted approval for KALYDECO for the treatment of infants with CF ages 1 month to less than 4 months with specific mutations in the CFTR gene. KALYDECO now represents the first and only medicine approved in Europe to treat the underlying cause of CF for this age group.
- In the first quarter, Vertex shared positive data from the pivotal studies for the next-generation triple combination of vanzacaftor/tezacaftor/deutivacaftor (the "vanzacaftor triple"), showing that the two randomized studies in patients 12 years and older met the primary endpoint and all key secondary endpoints, and the results in the single-arm study in children ages 6 to 11 were even more pronounced.
- Vertex has submitted regulatory marketing applications for the once-daily vanzacaftor triple in people with CF 6 years and older to the U.S. Food and Drug Administration (FDA), using a priority review voucher, and to the European Medicines Agency (EMA). Vertex intends to complete regulatory submissions to the MHRA in Great Britain, Health Canada, SwissMedic, the Australian Therapeutic Goods Administration (TGA) and the New Zealand Ministry of Health for people with CF 6 years of age and older later this year.

CASGEVY for the treatment of sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT):

CASGEVY is a non-viral, ex vivo *CRISPR/Cas9 gene-edited cell therapy for eligible patients with SCD or TDT that has been shown to reduce or eliminate vaso-occlusive crises (VOCs) for patients with SCD and transfusion requirements for patients with TDT.*

- CASGEVY is approved in the U.S., Great Britain, the European Union (EU), the Kingdom of Saudi Arabia (KSA), and the Kingdom of Bahrain (Bahrain) for the treatment of both SCD and TDT. Vertex has completed regulatory submissions for CASGEVY for SCD and TDT in Switzerland and Canada; the submission in Canada was granted priority review.
- As of mid-April, Vertex has activated more than 25 authorized treatment centers (ATCs) globally and multiple patients across all regions have initiated cell collection.
- Vertex has signed multiple agreements with both commercial and government health insurance providers in the U.S. to provide access to CASGEVY.
- Vertex has also secured reimbursed access for people with SCD or TDT in KSA and Bahrain, as well as for people with TDT in France through an early access program.

Suzetrigine (VX-548) for the treatment of moderate to severe acute pain:

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

- In the first quarter, Vertex shared positive results from the three Phase 3 trials of suzetrigine in moderate-to-severe acute pain.
- The FDA has granted a rolling New Drug Application (NDA) submission to suzetrigine in moderate-to-severe acute pain, and Vertex has started the rolling submission process. The submission is on track to be completed in the second quarter of 2024. Suzetrigine has also been granted FDA Fast Track and Breakthrough Therapy designations in moderate-to-severe acute pain.

Select Clinical-Stage R&D Pipeline

Cystic Fibrosis

Vertex continues to pursue next-in-class, small molecule, oral CFTR modulators for the ~90% of patients who may benefit from

such an approach, as well as a nebulized mRNA therapy for the more than 5,000 people with CF who do not make CFTR protein and cannot benefit from CFTR modulators.

- ***Vanzacaftor/tezacaftor/deutivacaftor, the next-in-class triple oral small molecule combination, in cystic fibrosis***
  - Vertex plans to initiate a new cohort in the Phase 3 study, RIDGELINE, in the second half of 2024 in children with cystic fibrosis ages 2 to 5 years who have at least one F508del mutation or a mutation responsive to triple combination CFTR modulators.
- ***Nebulized mRNA therapy:***
  - Vertex continues to enroll and dose patients in the multiple ascending dose (MAD) portion of the Phase 1/2 study of VX-522 in people with CF.
  - Vertex expects to share data from this study in late 2024 or early 2025.

Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia

- Vertex has completed enrollment in two global Phase 3 studies of CASGEVY in people 5 to 11 years of age with SCD or TDT.
- Vertex continues to work on preclinical assets for gentler conditioning for CASGEVY, which could broaden the eligible patient population to more than 150,000 people in the U.S. and Europe.

Acute Pain

- Vertex plans to initiate a Phase 2 study with an oral formulation of VX-993, a next-generation selective NaV1.8 inhibitor, for the treatment of moderate to severe acute pain in 2024.
- The FDA cleared the investigational new drug (IND) application for an intravenous formulation of VX-993 for the treatment of moderate to severe acute pain, and Vertex has initiated a Phase 1 trial.
- Consistent with its commitment to serial innovation and leadership in pain, Vertex also continues to develop NaV1.7 inhibitors, for stand-alone use or in combination with NaV1.8 inhibitors, for both acute and peripheral neuropathic pain.

Peripheral Neuropathic Pain (PNP)

- Following a successful end-of-Phase 2 meeting with the FDA, Vertex is on track to initiate the Phase 3 pivotal program of suzetrigine in patients with painful diabetic peripheral neuropathy (DPN), a type of PNP, in the second half of 2024. The FDA recently granted suzetrigine Breakthrough Therapy designation in this indication.
- Vertex continues to enroll and dose patients in its Phase 2 study of suzetrigine in painful lumbosacral radiculopathy (LSR), representing ~40% of the PNP category. Vertex is on track to complete enrollment in the Phase 2 study by the end of 2024.
- Vertex anticipates initiating a Phase 2 study with an oral formulation of VX-993 for the treatment of PNP in 2024.

APOL1-Mediated Kidney Disease (AMKD)

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

- Based on the totality of the unblinded data reviewed by the Independent Data Safety Monitoring Committee (IDMC), Vertex advanced into the Phase 3 portion of the global Phase 2/3 pivotal clinical trial in patients with AMKD, in which a 45 mg once-daily oral dose of inaxaplin will be compared to placebo, on top of standard of care.
- In addition, based on the IDMC review, the trial has been expanded to include adolescents 10 to 17 years of age with AMKD.
- 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 inaxaplin arm versus placebo. If positive, the interim analysis may serve as the basis for Vertex to seek accelerated approval in the U.S.

Type 1 Diabetes (T1D)

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

- *VX-880, fully differentiated islet cells with standard immunosuppression:*
  - Based on the totality of evidence reviewed by the IDMC, the Phase 1/2 study in people with T1D and impaired awareness of hypoglycemia and recurrent hypoglycemic events has resumed dosing.
  - Vertex has completed enrollment in Parts A, B, and C of the global 17-patient study and expects to complete dosing soon.
  - Vertex plans to present updated data from the ongoing Phase 1/2 study at the American Diabetes Association 84th Scientific Sessions Conference in June 2024.
- *VX-264, fully differentiated islet cells encapsulated in an immunoprotective device:*
  - The clinical trial for VX-264, which encapsulates the same VX-880 islet cells in a novel device designed to eliminate the need for immunosuppressants, is a multi-part, Phase 1/2 study.

    - Vertex has completed Part A of the study and initiated Part B.
- *Hypoimmune, edited fully differentiated islet cells:*
    - Vertex's hypoimmune cell program involves using CRISPR/Cas9 to gene edit the same stem cell-derived, fully differentiated islet cells used in the VX-880 and VX-264 programs to cloak the cells from the immune system. This program is progressing through the research stage.

Myotonic Dystrophy Type 1 (DM1)

Vertex is evaluating multiple approaches that target the underlying cause of DM1, the most prevalent muscular dystrophy in adults, with ~110,000 people living with the disease in the U.S. and Europe, and no approved therapies. Vertex's lead approach, VX-670, was in-licensed from Entrada Therapeutics in February 2023 and is an oligonucleotide connected to a cyclic peptide to promote effective delivery into cells, which holds the potential to address the underlying cause of DM1.

- The IND in the U.S. for VX-670 has cleared, as have the clinical trial applications (CTAs) in Canada, the U.K. and the EU, and the clinical trial notification (CTN) in Australia.
- Enrollment and dosing are underway.

Autosomal Dominant Polycystic Kidney Disease (ADPKD)

Vertex is developing small molecule correctors that restore function to PC1 missense variants, with the goal to address the underlying cause of ADPKD, the most common genetic kidney disease, affecting approximately 250,000 people in the U.S. and Europe.

- Vertex has initiated a Phase 1 clinical trial in healthy volunteers for VX-407, a first-in-class small molecule corrector that targets the underlying cause of ADPKD in patients with a subset of PKD1 variants, estimated at ~25,000 (or ~10 percent) of the overall ~250,000 ADPKD patient population.

Investments in External Innovation

- Vertex and Alpine Immune Sciences entered into a definitive agreement under which Vertex will acquire Alpine for approximately $4.9 billion in cash. Vertex has commenced a tender offer to purchase all of the outstanding shares of common stock of Alpine for $65 per share in cash. The transaction is anticipated to close in the second quarter of 2024. Alpine's lead molecule, povetacicept, is a highly potent and effective dual antagonist of BAFF (B cell activating factor) and APRIL (a proliferation-inducing ligand). Through Phase 2 development, povetacicept has shown potential best-in-class efficacy in IgA nephropathy (IgAN), a serious, progressive, autoimmune disease of the kidney that can lead to end-stage-renal disease. There are currently no approved therapies that target the underlying cause of IgAN, which is the most common cause of primary (idiopathic) glomerulonephritis worldwide, affecting approximately 130,000 people in the U.S. alone. Povetacicept is on track to enter Phase 3 clinical development in IgAN in the second half of 2024. Phase 1b/2 studies in autoimmune renal diseases and cytopenias are ongoing with data expected later this year.
- Vertex achieved a clinical milestone for VX-670 in DM1 in the first quarter of 2024, resulting in a $75 million milestone payable to Entrada.

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) intangible asset amortization expense, (iii) gains or losses related to the fair value of the company's strategic investments, (iv) increases or decreases in the fair value of contingent consideration, (v) acquisition-related costs, and (vi) other adjustments. The company's non-GAAP financial results also exclude from its provision for income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income described above and certain discrete items. These results should not be viewed as a substitute for the company's GAAP results and are provided as a complement to results provided in accordance with GAAP. Management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position that the company believes is helpful to an understanding of its ongoing business. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, to manage the company's business and to evaluate its performance. The company's calculation of non-GAAP financial measures likely differs from the calculations used by other companies. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

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

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

	Three Months Ended March 31,	
	2024	2023
Product revenues, net	$ 2,690.6	$ 2,374.8
Costs and expenses:		
Cost of sales	342.6	266.9
Research and development expenses	789.1	742.6
Acquired in-process research and development expenses	76.8	347.1
Selling, general and administrative expenses	342.7	241.1
Change in fair value of contingent consideration	(0.1)	(1.9)
Total costs and expenses	1,551.1	1,595.8
Income from operations	1,139.5	779.0
Interest income	181.2	122.6
Interest expense	(10.4)	(11.4)
Other (expense) income, net	(31.2)	1.3
Income before provision for income taxes	1,279.1	891.5
Provision for income taxes	179.5	191.7
Net income	$ 1,099.6	$ 699.8
Net income per common share:		
Basic	$ 4.26	$ 2.72
Diluted	$ 4.21	$ 2.69
Shares used in per share calculations:		
Basic	258.2	257.4
Diluted	261.1	260.3

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

	Three Months Ended March 31,	
	2024	2023
TRIKAFTA/KAFTRIO	$ 2,483.6	$ 2,096.7
Other CF products	207.0	278.1
Product revenues, net	$ 2,690.6	$ 2,374.8

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

	Three Months Ended March 31,	
	2024	2023
GAAP cost of sales	$ 342.6	$ 266.9
Stock-based compensation expense	(1.8)	(1.9)
Intangible asset amortization expense	(5.0)	—
Non-GAAP cost of sales	$ 335.8	$ 265.0
GAAP research and development expenses	$ 789.1	$ 742.6
Stock-based compensation expense	(119.4)	(76.3)
Acquisition-related costs	—	(2.8)
Non-GAAP research and development expenses	$ 669.7	$ 663.5
Acquired in-process research and development expenses	$ 76.8	$ 347.1
GAAP selling, general and administrative expenses	$ 342.7	$ 241.1

Stock-based compensation expense	(70.7)	(44.2)
Non-GAAP selling, general and administrative expenses	$ 272.0	$ 196.9
Combined non-GAAP R&D, Acquired IPR&D and SG&A expenses	$ 1,018.5	$ 1,207.5
GAAP other (expense) income, net	$ (31.2)	$ 1.3
Decrease (increase) in fair value of strategic investments	27.0	(6.4)
Non-GAAP other expense, net	$ (4.2)	$ (5.1)
GAAP provision for income taxes	$ 179.5	$ 191.7
Tax adjustments (1)	81.6	22.7
Non-GAAP provision for income taxes	$ 261.1	$ 214.4
GAAP effective tax rate	14.0%	21.5 %
Non-GAAP effective tax rate	17.4%	21.3 %

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

	Three Months Ended March 31,	
	2024	**2023**
GAAP operating income	$ 1,139.5	$ 779.0
Stock-based compensation expense	191.9	122.4
Intangible asset amortization expense	5.0	—
Decrease in fair value of contingent consideration	(0.1)	(1.9)
Acquisition-related costs	—	2.8
Non-GAAP operating income	$ 1,336.3	$ 902.3
GAAP net income	$ 1,099.6	$ 699.8
Stock-based compensation expense	191.9	122.4
Intangible asset amortization expense	5.0	—
Decrease (increase) in fair value of strategic investments	27.0	(6.4)
Decrease in fair value of contingent consideration	(0.1)	(1.9)
Acquisition-related costs	—	2.8
Total non-GAAP adjustments to pre-tax income	223.8	116.9
Tax adjustments (1)	(81.6)	(22.7)
Non-GAAP net income	$ 1,241.8	$ 794.0
Net income per diluted common share:		
GAAP	$ 4.21	$ 2.69
Non-GAAP	$ 4.76	$ 3.05
Shares used in diluted per share calculations:		
GAAP and Non-GAAP	261.1	260.3

Notes

1: In the three months ended March 31, 2024 and 2023, "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 2024 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 $600 million to $700 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.

Condensed Consolidated Balance Sheets
(in millions)(unaudited)

	March 31, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 10,171.3	$ 11,218.3
Accounts receivable, net	1,793.2	1,563.4
Inventories	813.1	738.8
Prepaid expenses and other current assets	511.1	623.7
Total current assets	13,288.7	14,144.2
Property and equipment, net	1,172.8	1,159.3
Goodwill and intangible assets, net	1,922.9	1,927.9
Deferred tax assets	1,963.0	1,812.1
Operating lease assets	312.9	293.6
Long-term marketable securities	4,381.4	2,497.8
Other long-term assets	875.7	895.3
Total assets	$ 23,917.4	$ 22,730.2
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 3,147.3	$ 3,020.2
Other current liabilities	648.6	527.2
Total current liabilities	3,795.9	3,547.4
Long-term finance lease liabilities	361.5	376.1
Long-term operating lease liabilities	359.8	348.6
Other long-term liabilities	853.6	877.7
Shareholders' equity	18,546.6	17,580.4
Total liabilities and shareholders' equity	$ 23,917.4	$ 22,730.2
Common shares outstanding	258.3	257.7

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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 consecutive years on Science magazine's Top Employers list and one of Fortune’s 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on LinkedIn, Facebook, Instagram, YouTube and Twitter/X.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements that are subject to risks, uncertainties and other factors. All statements other than statements of historical fact are statements that could be deemed forward-looking statements, including all statements regarding the intent, belief, or current expectation of Vertex and members of the Vertex senior management team. Forward-looking statements are not purely historical and may be accompanied by words such as “anticipates,” “may,” “forecasts,” “expects,” “intends,” “plans,” “potentially,” “believes,” “seeks,” “estimates,” and other words and terms of similar meaning. Such statements include, without limitation, Dr. Kewalramani's statements in this press release, the information provided regarding future financial performance and operations, the section captioned “Full Year 2024 Financial Guidance” and statements regarding (i) expectations for Vertex’s continued growth in CF, including through new approvals and reimbursements for the treatment of younger patients, (ii) expectations, plans, and status of the potential near-term commercial launch of the vanzacaftor triple, including our plans to submit regulatory filings in Great Britain, Canada, Switzerland, Australia and New Zealand in 2024, (iii) our beliefs regarding the anticipated benefits of CASGEVY, expectations around activation of ATCs, and plans to secure additional reimbursed access outside of the U.S., (iv) expectations regarding the potential benefits and commercial success of the product candidates in our pain program, (v) expectations, plans, and status of the potential near-term commercial launch of suzetrigine for the treatment of

moderate-to-severe acute pain, including our plans to complete regulatory submissions in the second quarter of 2024, (vi) plans to initiate a Phase 3 RIDGELINE study for the vanzacaftor triple in children with CF ages 2 to 5 in the second half of 2024, (vii) expectations for our VX-522 Phase 1/2 study, including the potential benefits of this nebulized mRNA therapy and expectations to share data in late 2024 or early 2025, (viii) expectations regarding our SCD and TDT program, including expectations that a gentler conditioning for CASGEVY could broaden the eligible patient population to more than 150,000 people, (ix) expectations to initiate the Phase 3 pivotal program of suzetrigine in patients with DPN in the second half of 2024, expectations to complete enrollment in the Phase 2 study of suzetrigine in LSR by the end of 2024, and expectations to initiate a Phase 2 study with an oral formulation of VX-993 for the treatment of PNP in 2024, (x) plans to initiate a Phase 2 study with an oral formulation of VX-993 for the treatment of acute pain in 2024, and plans to continue to develop NaV1.7 inhibitors for both acute pain and PNP, (xi) expectations regarding the potential benefits of our AMKD program, including plans for our global Phase 2/3 pivotal clinical trial evaluating inaxaplin in patients with AMKD, study designs and our expectations that the interim analysis of this study may serve as the basis for accelerated approval in the U.S., (xii) expectations regarding our T1D programs, the status of our Phase 1/2 study of VX-880, including expectations for completion of dosing and plans to present data at an upcoming medical conference, (xiii) the potential benefits and clinical status of VX-670 for the treatment in people with DM1, (xiv) expectations regarding our ADPKD program, including the potential benefits of VX-407 as a first-in-class small molecule corrector and our beliefs regarding the targeted patient population, and (xv) expectations, plans, and the anticipated timeline for the pending acquisition of Alpine Immune Sciences, Inc., including with respect to Alpine, and the therapeutic scope of and the potential benefits of povetacicept, our beliefs regarding povetacicept's target patient population, and our beliefs regarding the clinical progress and availability of clinical data from the current Alpine pipeline. 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 2024 full year product revenues, expenses and effective tax rates may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that the company may not be able to receive adequate reimbursement or additional regulatory approvals for CASGEVY on the expected timeline, or at all, that we are unable to successfully develop, obtain approval or commercialize suzetrigine as a treatment for acute or neuropathic pain, 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 Vertex may not be able to complete, successfully integrate, or profit from the acquisition of Alpine Immune Sciences, Inc., 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 that anticipated commercial launches may be delayed, if they occur at all. Forward-looking statements in this press release should be evaluated together with the many uncertainties that affect Vertex's business, particularly those risks listed under the heading "Risk Factors" and the other cautionary factors discussed in Vertex's periodic reports filed with the SEC, including Vertex's annual report on Form 10-K for the year ended December 31, 2023, and its quarterly reports on Form 10-Q and current reports on Form 8-K, all of which are 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.

Additional Information about the Acquisition and Where to Find It

The tender offer for the outstanding shares of common stock of Alpine Immune Sciences, Inc. referenced in this communication commenced on April 22, 2024. This communication is for informational purposes only and is neither an offer to purchase nor a solicitation of an offer to sell shares of Alpine Immune Sciences, Inc., nor is it a substitute for any tender offer materials that Vertex or Alpine Immune Sciences, Inc. have filed with the SEC. On April 22, 2024, when the tender offer commenced, Vertex filed with the SEC a Tender Offer Statement on Schedule TO which included an Offer to Purchase, a related Letter of Transmittal and certain other tender offer documents (together, the "Tender Offer Materials"), and Alpine Immune Sciences, Inc. filed with the SEC a Solicitation/Recommendation Statement on Schedule 14D-9 (the "Solicitation/Recommendation Statement") with respect to the tender offer. ALPINE IMMUNE SCIENCES, INC. SECURITY HOLDERS ARE URGED TO READ THE TENDER OFFER MATERIALS AND THE SOLICITATION/RECOMMENDATION STATEMENT BECAUSE THEY CONTAIN IMPORTANT INFORMATION WHICH SHOULD BE READ CAREFULLY BEFORE ANY DECISION IS MADE WITH RESPECT TO THE TENDER OFFER. The Tender Offer Materials and the Solicitation/Recommendation Statement are available for free at the SEC's website at www.sec.gov. Additional copies of the Tender Offer Materials can be obtained free of charge under the "Investors" section of Vertex's website at https://investors.vrtx.com/financial-information/sec-filings or by contacting Vertex by phone at (617) 341-6108, by email at Investorinfo@VRTX.com, or by directing requests for such materials to the information agent for the offer, which is named in the Tender Offer Materials. In addition to the Tender Offer Materials and the Solicitation/Recommendation Statement, Alpine Immune Sciences, Inc. and Vertex file periodic reports and other information with the SEC. Vertex's and Alpine Immune Sciences, Inc.'s filings with the SEC are also available for free to the public from commercial document-retrieval services, at the website maintained by the SEC at www.sec.gov, and their respective investor relations websites.

Conference Call and Webcast

The company will host a conference call and webcast at 4:30 p.m. ET. To access the call, please dial (833) 630-2124 (U.S.) or +1(412) 317-0651 (International) and reference the "Vertex Pharmaceuticals First Quarter 2024 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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