Vertex Reports Third Quarter 2024 Financial Results

November 4, 2024

— Product revenue of $2.77 billion, a 12% increase compared to Q3 2023 —

— Raising full-year product revenue guidance to $10.8 billion to $10.9 billion —

— Preparing for two potential near-term launches: vanzacaftor triple in CF and suzetrigine (VX-548) for moderate-to-severe acute pain —

— Pipeline progress continuing with three additional programs advancing to Phase 3: suzetrigine in DPN, povetacicept in IgAN and VX-880 in T1D —

BOSTON--(BUSINESS WIRE)--Nov. 4, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2024, and raised its full-year product revenue guidance to $10.8 billion to $10.9 billion.

“The third quarter marked another period of strong progress, with continued revenue growth and outstanding execution across the business, and we are again increasing our full-year product revenue guidance,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “Launch preparedness is well underway as we look forward to the potential approvals of the vanzacaftor triple for cystic fibrosis and suzetrigine, a new class of medicine for moderate-to-severe acute pain. With a broad and deep pipeline and three additional programs advancing to Phase 3 development in the last quarter alone, Vertex is well positioned for continued long-term growth.”

Third Quarter 2024 Results

Product revenue increased 12% to $2.77 billion compared to the third quarter of 2023, primarily driven by the continued strong performance of TRIKAFTA®/KAFTRIO®. Net product revenue in the third quarter of 2024 increased 10% to $1.71 billion in the U.S. and increased 14% to $1.06 billion outside the U.S., compared to the third quarter of 2023.

Combined GAAP and Non-GAAP R&D and SG&A expenses were $1.2 billion and $1.1 billion, respectively, compared to $1.1 billion and $942 million, respectively, in the third quarter of 2023. The increases were due to increased commercial investment to support launches of Vertex's therapies globally and continued investment in support of additional programs that have advanced to Phase 3 clinical development.

Acquired IPR&D (AIPR&D) expenses were $15 million compared to $52 million in the third quarter of 2023.

GAAP effective tax rate was 14.6% compared to 12.2% for the third quarter of 2023. Both periods included R&D tax credits for the current and prior years and excess tax benefits related to stock-based compensation.

Non-GAAP effective tax rate was 19.8% compared to 19.4% for the third quarter of 2023. Please refer to Note 1 for further details on Vertex’s GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net income were $1.0 billion and $1.1 billion, respectively, for each of the third quarters of 2024 and 2023. Increased product revenue was partially offset by increased R&D and SG&A expenses compared to the third quarter of 2023.

Cash, cash equivalents and total marketable securities as of September 30, 2024 were $11.2 billion, compared to $13.7 billion as of December 31, 2023. The reduction in Vertex’s cash, cash equivalents and marketable securities balance compared to December 31, 2023, was due to the cash consideration paid to acquire Alpine Immune Sciences in the second quarter of 2024 and repurchases of our common stock pursuant to our share repurchase program, partially offset by positive cash flows provided by other operating activities.

Full-Year 2024 Financial Guidance

Vertex today raised its full-year 2024 product revenue guidance from $10.65 billion to $10.85 billion to $10.8 billion to $10.9 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. Given the impact of the Alpine acquisition, for 2024, Vertex is providing guidance for both combined GAAP and Non-GAAP R&D and SG&A expenses and for AIPR&D expenses. Vertex continues to expect combined

Non-GAAP R&D and SG&A expenses to be in a range of $4.2 billion to $4.3 billion for the full year. This includes Vertex's expectations for continued investment in multiple mid- and late-stage clinical development programs and commercial and manufacturing capabilities. Vertex also continues to expect 2024 AIPR&D expenses of approximately $4.6 billion for the full year, including the Alpine acquisition-related charge of $4.4 billion in the second quarter of 2024.

Vertex's updated financial guidance is summarized below:

	Current FY 2024	Previous FY 2024
Total product revenue	$10.8 to $10.9 billion	$10.65 to $10.85 billion
Combined GAAP R&D and SG&A expenses (2)	Unchanged	$5.0 to $5.2 billion
Combined Non-GAAP R&D and SG&A expenses (2)	Unchanged	$4.2 to $4.3 billion
AIPR&D expenses	Unchanged	$4.6 billion*
Non-GAAP effective tax rate**	~90%	~100%

*Includes Alpine AIPR&D expense of $4.4 billion.
**Vertex's full-year Non-GAAP tax rate is impacted by the Alpine AIPR&D expense, which is non-deductible for tax.

Key Business Highlights

Marketed Products and Potential Near-Term Launch Opportunities

Cystic Fibrosis (CF) Portfolio

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

- As of the third quarter of 2024, KAFTRIO is now reimbursed in all 27 countries of the European Union.
- The U.S. Food and Drug Administration (FDA) has assigned the once-daily vanzacaftor triple in people with CF 6 years and older a Prescription Drug User Fee Act (PDUFA) target action date of January 2, 2025. The vanzacaftor triple was granted Priority Review by the FDA.
- Vertex has completed regulatory submissions for the vanzacaftor triple in the European Union (EU), the United Kingdom (U.K.), Canada, Australia, New Zealand and Switzerland, and reviews are underway.
- In July, Health Canada granted approval to TRIKAFTA for an additional 152 rare mutations in the CFTR gene.
- Vertex has submitted regulatory applications to the FDA and the European Medicines Agency (EMA) for TRIKAFTA/KAFTRIO for the treatment of people with CF and rare responsive mutations.

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 EU, the Kingdom of Saudi Arabia (KSA), the Kingdom of Bahrain (Bahrain), Canada and Switzerland for the treatment of both SCD and TDT, and launches are ongoing.*

- Vertex received regulatory approvals for CASGEVY for the treatment of patients 12 years of age and older with SCD or TDT in Switzerland and Canada.
- As of mid-October, Vertex has activated 45 authorized treatment centers (ATCs) globally and increasing numbers of patients across all regions have initiated cell collection.
- In the third quarter of 2024, Vertex's product revenue included revenue from the first patient infused with CASGEVY.
- Vertex announced a reimbursement agreement with NHS England for eligible patients with TDT to access CASGEVY. Vertex has also entered into commercial discussions with NHS England to secure access to CASGEVY for eligible patients with SCD.
- The Italian Medicines Agency approved Vertex's request for the implementation of an early access program (EAP), for the use of CASGEVY for the treatment of TDT and SCD.

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

Vertex has discovered and continues to advance 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 without the limitations of opioids and other currently available medicines.

- The FDA has assigned a PDUFA target action date of January 30, 2025, for suzetrigine for the treatment of moderate-to-severe acute pain. Suzetrigine was granted Priority Review by the FDA.

Select Clinical-Stage R&D Pipeline

Cystic Fibrosis

Vertex continues to pursue next-in-class, small molecule, oral CFTR modulators for the ~90% of people with CF 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 once-daily, next-in-class triple oral small molecule combination, in cystic fibrosis***
  - Vertex is enrolling and dosing a study 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.
- Consistent with its commitment to serial innovation and bringing as many patients as possible to normal levels of CFTR function, Vertex continues to advance new oral small molecule combination therapies through preclinical and clinical development. The most advanced next-wave CFTR modulators have completed, or are in the process of completing, Phase 1 clinical trials.
- ***VX-522, nebulized mRNA therapy***
  - Vertex completed the single ascending dose (SAD) portion of the Phase 1/2 study of VX-522 in people with CF in late 2023, and the multiple ascending dose (MAD) portion of the study is ongoing. Vertex expects to complete the trial and share data in the first half of 2025.

Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia

- Vertex has completed enrollment in two global Phase 3 studies of CASGEVY in children 5 to 11 years of age with SCD or TDT and the trials are ongoing.
- Vertex continues to work on preclinical assets for gentler conditioning for CASGEVY, which could broaden the eligible patient population.

Acute Pain

- Vertex has initiated a Phase 2 study for an oral formulation of VX-993, a next-generation selective NaV1.8 pain signal inhibitor, for the treatment of moderate-to-severe acute pain following bunionectomy surgery.
- Vertex continues to enroll and dose the Phase 1 trial for an intravenous formulation of VX-993.
- The FDA has granted Fast Track Designation to VX-993 in moderate-to-severe acute pain in both its oral and intravenous formulations.

Peripheral Neuropathic Pain (PNP)

- Vertex has initiated the Phase 3 pivotal program of suzetrigine in patients with painful diabetic peripheral neuropathy (DPN), a type of PNP that accounts for approximately 20% of patients suffering from PNP. The FDA has granted suzetrigine Breakthrough Therapy Designation in DPN.
- Vertex has completed the Phase 2 study of suzetrigine in painful lumbosacral radiculopathy (LSR), a condition representing more than 40% of patients suffering from PNP. Vertex continues to expect to share results from this study by the end of the year.
- Vertex has also initiated a Phase 2 study for the oral formulation of VX-993 for the treatment of DPN.

Consistent with its commitment to serial innovation and leadership in pain, Vertex continues to develop additional NaV1.8 inhibitors and NaV1.7 inhibitors, for stand-alone use or in combination, for the treatment of acute and peripheral neuropathic pain.

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 the underlying genetic driver of this kidney disease.

- Vertex continues to enroll and dose patients with AMKD in the Phase 3 portion of the global Phase 2/3 pivotal clinical trial of inaxaplin, in which a 45 mg once-daily dose of inaxaplin is compared to placebo, on top of standard of care.

IgA Nephropathy (IgAN) and Other B Cell-Mediated Diseases

Vertex is developing povetacicept, a dual inhibitor of the BAFF and APRIL pathways, as a potentially best-in-class approach to treat immunoglobulin A (IgA) nephropathy. Vertex is also studying povetacicept in other serious B cell-mediated diseases, including autoimmune kidney diseases, such as primary membranous nephropathy, and autoimmune cytopenias.

- Vertex has initiated the RAINIER study, the Phase 3 clinical trial of povetacicept in IgA nephropathy.
  - RAINIER is a global pivotal trial of povetacicept 80 mg vs. placebo on top of standard of care in approximately 480 people with IgAN. The study is designed to have a pre-planned interim analysis evaluating the change from baseline in urine protein creatinine ratio (UPCR) for the povetacicept arm versus placebo after a certain number of patients reach 36 weeks of treatment. If positive, the interim analysis may serve as the basis for accelerated approval in the U.S. Final analysis will occur at two years of treatment, with a primary endpoint of total estimated glomerular filtration rate (eGFR) slope through Week 104.
- Vertex presented updated data on 54 patients with IgAN from the RUBY-3 Phase 1b/2 basket study of povetacicept at the

American Society of Nephrology (ASN) Annual Meeting. Treatment with povetacicept 80 mg dosed subcutaneously every four weeks demonstrated a clinically meaningful decrease in proteinuria, with a mean 66% reduction from baseline in UPCR (n=8) at 48 weeks associated with stable renal function over 48 weeks as assessed by eGFR. By 48 weeks, 63% (5 out of 8) of study participants achieved clinical remission, defined as UPCR < 0.5 g/g, negative hematuria, and stable renal function (≤ 25% reduction in eGFR from baseline). Treatment with povetacicept 240 mg dosed subcutaneously every four weeks was associated with similar improvements in proteinuria, and stable renal function, and both doses have been well tolerated in patients with IgAN.

- Vertex also presented emerging data on povetacicept from RUBY-3 in patients with primary membranous nephropathy (pMN) at ASN. Treatment with povetacicept 80 mg dosed subcutaneously every four weeks demonstrated a mean 62% reduction from baseline in UPCR (n=3) at 24 weeks, associated with stable renal function. By week 24, 2/3 of patients (67%) had achieved partial clinical remission, defined as UPCR < 3.5 g/g and ≥50% reduction in UPCR from baseline. Povetacicept was also well-tolerated in these patients with pMN.
- Vertex is studying additional renal diseases in the RUBY-3 basket study and hematologic conditions in the RUBY-4 basket study, both of which are ongoing.

Type 1 Diabetes (T1D)

Vertex is evaluating stem cell-derived, fully differentiated islet cell therapies for patients suffering from T1D, with the goal of developing a potential one-time functional cure for this disease.

- *VX-880, fully differentiated islet cells with standard immunosuppression:*
  - Following successful end of Phase 2 meetings with the FDA, EMA and the U.K. Medicines and Healthcare products Regulatory Agency (MHRA), Vertex has reached agreement to advance VX-880 into pivotal development with the conversion of the ongoing Phase 1/2 study to a Phase 1/2/3 study.
  - The Phase 1/2/3 study will include a total of 50 patients infused with a single, target dose of VX-880. The primary endpoint is the proportion of patients with insulin independence and absence of severe hypoglycemic episodes (SHEs).
- *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 so that treatment with immunosuppressants is not required, is a global, multi-part, Phase 1/2 study.
  - Vertex has completed Part A of the study. As with the VX-880 study, patients in Part A receive a low dose with a stagger period between dosing.
  - Part B of the Phase 1/2 study is enrolling and dosing patients. In Part B, patients receive the full target dose with a stagger period between patients, and in Part C, patients will receive the full target dose with no stagger.
  - Vertex expects to share initial data from this study in 2025.
- *Hypoimmune, edited fully differentiated islet cells:*
  - Vertex's hypoimmune cell program involves editing the same stem cell-derived, fully differentiated VX-880 islet cells to protect the cells from the immune system, hence avoiding the need for immunosuppression. This research-stage program continues to make progress.

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, in-licensed from Entrada Therapeutics, is an oligonucleotide linked to a cyclic peptide to promote effective delivery into the cell and its nucleus and holds the potential to address the underlying cause of DM1.

- Vertex recently completed the single ascending dose (SAD) portion of the global Phase 1/2 clinical trial for VX-670 in people with DM1.
- Vertex has initiated the MAD portion of the Phase 1/2 study, in which both the safety and efficacy of VX-670 will be evaluated.

Autosomal Dominant Polycystic Kidney Disease (ADPKD)

Vertex is developing small molecule correctors that restore function to the variant polycystin 1 (PC1) protein, with the goal of addressing the underlying cause of ADPKD, the most common genetic kidney disease, affecting approximately 250,000 people in the U.S. and Europe.

- Vertex continues to enroll and dose its 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 variants in the PKD1 gene, which encodes the PC1 protein, estimated to be ~25,000 (or ~10%) of the overall patient population.

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 (loss) (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 (loss) described above and certain discrete items. These results should not be viewed as a substitute for the company's GAAP results and are provided as a complement to results provided in accordance with GAAP. Management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position that the company believes is helpful to an understanding of its ongoing business. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, to manage the company's business and to evaluate its performance. The company's calculation of non-GAAP financial measures likely differs from the calculations used by other companies. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
Product revenues, net	$ 2,771.9	$ 2,483.5	$ 8,108.1	$ 7,351.5
Costs and expenses:				
Cost of sales	392.6	318.7	1,107.1	894.2
Research and development expenses	875.9	810.0	2,631.6	2,338.3
Acquired in-process research and development expenses	15.0	51.7	4,540.9	509.3
Selling, general and administrative expenses	371.8	263.8	1,086.7	767.5
Change in fair value of contingent consideration	0.3	1.2	0.7	(1.3)
Total costs and expenses	1,655.6	1,445.4	9,367.0	4,508.0
Income (loss) from operations	1,116.3	1,038.1	(1,258.9)	2,843.5
Interest income	132.2	167.9	469.9	435.2
Interest expense	(7.5)	(10.9)	(27.8)	(33.5)
Other expense, net	(16.9)	(15.9)	(71.2)	(13.0)
Income (loss) before provision for income taxes	1,224.1	1,179.2	(888.0)	3,232.2
Provision for income taxes	178.7	143.9	560.6	581.4
Net income (loss)	$ 1,045.4	$ 1,035.3	$ (1,448.6)	$ 2,650.8
Net income (loss) per common share:				
Basic	$ 4.05	$ 4.01	$ (5.61)	$ 10.29
Diluted	$ 4.01	$ 3.97	$ (5.61)	$ 10.18
Shares used in per share calculations:				
Basic	258.0	258.0	258.1	257.7
Diluted	261.0	260.6	258.1	260.4

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
TRIKAFTA/KAFTRIO	$ 2,585.0	$ 2,274.3	$ 7,517.8	$ 6,611.4
Other product revenues	186.9	209.2	590.3	740.1
Product revenues, net	$ 2,771.9	$ 2,483.5	$ 8,108.1	$ 7,351.5

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
GAAP cost of sales	$ 392.6	$ 318.7	$ 1,107.1	$ 894.2
Stock-based compensation expense	(1.9)	(1.7)	(5.5)	(5.4)
Intangible asset amortization expense	(5.0)	—	(15.1)	—
Non-GAAP cost of sales	$ 385.7	$ 317.0	$ 1,086.5	$ 888.8
GAAP research and development expenses	$ 875.9	$ 810.0	$ 2,631.6	$ 2,338.3
Stock-based compensation expense	(111.0)	(81.1)	(327.5)	(231.9)
Intangible asset amortization expense	(0.9)	—	(0.9)	—
Acquisition-related costs (3)	—	(2.9)	(172.3)	(8.5)
Non-GAAP research and development expenses	$ 764.0	$ 726.0	$ 2,130.9	$ 2,097.9
GAAP selling, general and administrative expenses	$ 371.8	$ 263.8	$ 1,086.7	$ 767.5
Stock-based compensation expense	(71.7)	(48.1)	(197.7)	(135.3)
Acquisition-related costs (3)	—	—	(36.5)	—
Non-GAAP selling, general and administrative expenses	$ 300.1	$ 215.7	$ 852.5	$ 632.2
Combined non-GAAP R&D and SG&A expenses	$ 1,064.1	$ 941.7	$ 2,983.4	$ 2,730.1
GAAP other expense, net	$ (16.9)	$ (15.9)	$ (71.2)	$ (13.0)
Decrease in fair value of strategic investments	10.8	6.2	50.5	0.2
Non-GAAP other expense, net	$ (6.1)	$ (9.7)	$ (20.7)	$ (12.8)
GAAP provision for income taxes	$ 178.7	$ 143.9	$ 560.6	$ 581.4
Tax adjustments (1)	104.0	112.9	283.8	159.2
Non-GAAP provision for income taxes	$ 282.7	$ 256.8	$ 844.4	$ 740.6
GAAP effective tax rate	14.6%	12.2%	(63.1)%	18.0%
Non-GAAP effective tax rate	19.8%	19.4%	(1,038.6)%	20.5%

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
GAAP operating income (loss)	$ 1,116.3	$ 1,038.1	$ (1,258.9)	$ 2,843.5
Stock-based compensation expense	184.6	130.9	530.7	372.6
Intangible asset amortization expense	5.9	—	16.0	—
Increase (decrease) in fair value of contingent consideration	0.3	1.2	0.7	(1.3)
Acquisition-related costs (3)	—	2.9	208.8	8.5
Non-GAAP operating income (loss)	$ 1,307.1	$ 1,173.1	$ (502.7)	$ 3,223.3
GAAP net income (loss)	$ 1,045.4	$ 1,035.3	$ (1,448.6)	$ 2,650.8
Stock-based compensation expense	184.6	130.9	530.7	372.6
Intangible asset amortization expense	5.9	—	16.0	—
Decrease in fair value of strategic investments	10.8	6.2	50.5	0.2

Increase (decrease) in fair value of contingent consideration	0.3	1.2	0.7	(1.3)
Acquisition-related costs (3)	—	2.9	208.8	8.5
Total non-GAAP adjustments to pre-tax income (loss)	201.6	141.2	806.7	380.0
Tax adjustments (1)	(104.0)	(112.9)	(283.8)	(159.2)
Non-GAAP net income (loss)	$ 1,143.0	$ 1,063.6	$ (925.7)	$ 2,871.6
Net income (loss) per diluted common share:				
GAAP	$ 4.01	$ 3.97	$ (5.61)	$ 10.18
Non-GAAP	$ 4.38	$ 4.08	$ (3.59)	$ 11.03
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	261.0	260.6	258.1	260.4

Notes

1: In the three and nine months ended September 30, 2024 and 2023, “Tax adjustments” included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income (loss), discrete benefits related to prior tax years resulting from R&D tax credit studies that were completed in the third quarter of each year and excess tax benefits related to stock-based compensation.

2: The difference between the company’s full-year 2024 combined GAAP R&D and SG&A expenses and combined non-GAAP R&D and SG&A expenses guidance relates primarily to $600 million to $700 million of stock-based compensation expense and $209 million of compensation expense primarily related to cash-settled unvested Alpine equity awards.

3: In the nine months ended September 30, 2024, “Acquisition-related costs” were primarily related to compensation expense associated with cash-settled unvested Alpine equity awards.

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

	September 30, 2024	December 31, 2023
Assets		
Cash, cash equivalents and marketable securities	$ 6,524.5	$ 11,218.3
Accounts receivable, net	1,750.6	1,563.4
Inventories	1,079.8	738.8
Prepaid expenses and other current assets	449.2	623.7
Total current assets	9,804.1	14,144.2
Property and equipment, net	1,117.8	1,159.3
Goodwill and intangible assets, net	1,919.6	1,927.9
Deferred tax assets	2,308.9	1,812.1
Operating lease assets	1,396.1	293.6
Long-term marketable securities	4,703.5	2,497.8
Other long-term assets	990.2	895.3
Total assets	$ 22,240.2	$ 22,730.2
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 3,615.5	$ 3,020.2
Other current liabilities	357.6	527.2
Total current liabilities	3,973.1	3,547.4
Long-term finance lease liabilities	114.0	376.1
Long-term operating lease liabilities	1,588.9	348.6
Other long-term liabilities	933.3	877.7
Shareholders' equity	15,630.9	17,580.4
Total liabilities and shareholders' equity	$ 22,240.2	$ 22,730.2
Common shares outstanding	257.7	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 acute and neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, autosomal dominant polycystic kidney disease, 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) the beliefs regarding anticipated benefits of CASGEVY, expectations for increasing numbers of patients initiating cell collection, and expectations with respect to international access and reimbursement for CASGEVY, (iii) expectations regarding the potential benefits and commercial success of suzetrigine for the treatment of moderate-to-severe acute pain, including beliefs regarding the efficacy and safety of suzetrigine, and beliefs that suzetrigine has potential to provide effective pain relief without the limitations of opioids and other available medicines, (iv) expectations and status of the potential near-term commercial launch of the vanzacaftor triple, and plans to continue to advance new oral small molecule combination therapies for the treatment of CF, (v) expectations for VX-522, including the potential benefits of this nebulized mRNA therapy and expectations to complete the Phase 1/2 study and share data in the first half of 2025, (vi) expectations regarding the SCD and TDT program, including expectations that a gentler conditioning for CASGEVY could broaden the eligible patient population, (vii) plans with respect to the studies of the intravenous and oral formulation of VX-993 for the treatment of acute pain, (viii) expectations regarding PNP patient populations, expectations to share results from the Phase 2 study of suzetrigine in LSR by the end of the year, and plans to continue to develop NaV1.8 and NaV1.7 inhibitors for both acute pain and PNP, (ix) expectations regarding the potential benefits of the AMKD program, including plans for the global Phase 2/3 pivotal clinical trial evaluating inaxaplin in patients with AMKD, (x) expectations with respect to povetacicept, including beliefs about its potential benefits and therapeutic scope, study designs, expectations that the interim analysis of this study may serve as the basis to seek accelerated approval in U.S., and beliefs with respect to updated data from the RUBY3 Phase 1b/2 basket study, as presented at a recent medical conference, (xi) expectations regarding the T1D programs, including the status of each of the studies evaluating VX-880 and VX-264 and the expectations to share initial data from the VX-264 study in 2025, (xii) expectations for the potential benefits and clinical status of VX-670 for the treatment in people with DM1, and (xiii) expectations regarding the ADPKD program, including the potential benefits of VX-407 and beliefs regarding the targeted patient population. 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 for CASGEVY on the expected timeline, or at all, that we are unable to successfully obtain approval or commercialize suzetrigine as a treatment for acute pain or the vanzacaftor triple for CF, 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 the company's trials may be delayed, that the company may not realize the anticipated benefits from 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.

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 Third 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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