Vertex Reports Second Quarter 2024 Financial Results

August 1, 2024

— Product revenue of $2.65 billion, a 6% increase compared to Q2 2023 —

— Company raises full year product revenue guidance to $10.65 to $10.85 billion —

— FDA accepted NDA for vanzacaftor triple in CF with Priority Review and PDUFA target action date of January 2, 2025; additionally, MAA submissions validated in EU and U.K. —

— FDA accepted NDA for suzetrigine (VX-548) for moderate-to-severe acute pain with Priority Review and PDUFA target action date of January 30, 2025 —

— Advancing broad and deep clinical pipeline with multiple milestones expected in H2:24 —

BOSTON--(BUSINESS WIRE)--Aug. 1, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2024, and raised its full year product revenue guidance to $10.65 to $10.85 billion.

“Vertex delivered another strong quarter of revenue growth coupled with outstanding execution across the business, and we are increasing our full year product revenue guidance,” said Reshma Kewalramani, M.D., Chief Executive Officer, and President of Vertex. “Our focus for the second half of the year remains on commercial execution in CF and the global launch of CASGEVY, readying for the upcoming potential launches of the vanzacaftor triple in CF and suzetrigine in acute pain, while rapidly advancing a robust pipeline that is poised to deliver value for patients and shareholders for the long term.”

Second Quarter 2024 Results

Product revenue increased 6% to $2.65 billion compared to the second quarter of 2023, primarily driven by the continued strong performance of TRIKAFTA®/KAFTRIO®, including in younger age groups. Net product revenue in the second quarter of 2024 increased 7% to $1.61 billion in the U.S. and increased 5% to $1.03 billion outside the U.S., compared to the second quarter of 2023.

Combined GAAP and Non-GAAP R&D and SG&A expenses were $1.3 billion and $978 million, respectively, compared to $1.0 billion and $928 million, respectively, in the second quarter of 2023. The increases were due to 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.

Acquired IPR&D expenses were $4.4 billion compared to $111 million in the second quarter of 2023 due to $4.4 billion of Acquired IPR&D (AIPR&D) expenses associated with Vertex’s acquisition of Alpine Immune Sciences.

GAAP and Non-GAAP effective tax rates were (6.0)% and (10.0)%, respectively, compared to 21.2% and 21.0%, respectively, for the second quarter of 2023, primarily due to the impact of non-deductible AIPR&D expenses, which drove Vertex’s pre-tax loss in the second quarter of 2024. Please refer to Note 1 for further details on Vertex’s GAAP to Non-GAAP tax adjustments.

GAAP and Non-GAAP net losses were $3.6 billion and $3.3 billion, respectively, compared to net income of $916 million and $1.0 billion for the second quarter of 2023, respectively, given the impact of the Alpine AIPR&D expense in the second quarter of 2024.

Cash, cash equivalents and total marketable securities as of June 30, 2024 were $10.2 billion, compared to $13.7 billion as of December 31, 2023. The reduction in Vertex’s cash, cash equivalent and marketable securities balance compared to December 31, 2023, was due to the cash consideration paid to acquire Alpine in the second quarter of 2024, partially offset by positive cash flows provided by other operating activities.

Full Year 2024 Financial Guidance

Vertex today raised its full year product revenue guidance from $10.55-$10.75 billion to $10.65-$10.85 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 now 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 and the inclusion of Alpine operating expenses for the remainder of 2024. Vertex now expects 2024 AIPR&D expenses of approximately $4.6 billion for the full year, including the Alpine acquisition-related charge in the second quarter of 2024.

Vertex’s updated financial guidance is summarized below:

	Current FY 2024	Previous FY 2024
Total product revenues	$10.65 to $10.85 billion	$10.55 to $10.75 billion

Combined GAAP R&D and SG&A expenses (2)	$5.0 to $5.2 billion	$4.8 to $5.0 billion*
Combined Non-GAAP R&D and SG&A expenses (2)	Unchanged	$4.2 to $4.3 billion*
AIPR&D expenses	$4.6 billion**	$0.125 billion
Non-GAAP effective tax rate	~100%***	20% to 21%

* Guidance ranges provided on May 6, 2024 included combined GAAP R&D, AIPR&D and SG&A expenses of $4.9-$5.1 billion and combined Non-GAAP R&D, AIPR&D and SG&A expenses of $4.3-$4.4 billion. Included in both ranges were approximately $125 million for AIPR&D expenses.

** 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:

- The U.S. Food and Drug Administration (FDA) accepted the New Drug Application (NDA) for the once-daily vanzacaftor triple in people with CF 6 years and older and assigned Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of January 2, 2025. Vertex also received validation of its vanzacaftor triple Marketing Authorization Application (MAA) submissions from the European Medicines Agency (EMA) in the European Union (EU), and the Medicines and Healthcare products Regulatory Agency (MHRA) in the U.K. Vertex has also completed regulatory submissions for the vanzacaftor triple in Canada, Australia, New Zealand and Switzerland.
- Vertex announced an extended long-term reimbursement agreement with NHS England providing access to KAFTRIO, SYMKEVI® and ORKAMBI® and continued access to KALYDECO® for all existing and future eligible CF patients in England. Vertex has entered into similar agreements in Wales, Northern Ireland and Scotland. The agreements, which also include access to any future license extensions of these medicines, are a result of the National Institute for Health and Care Excellence's (NICE) and the Scottish Medicines Consortium's (SMC) positive recommendations for Vertex's CFTR modulators.
- In May, the European Commission granted approval to 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 July, Health Canada granted approval to TRIKAFTA for the treatment of people with CF with 152 rare responsive mutations in the CFTR gene. This represents the first approval outside of the United States where the mutations were approved based on *in vitro* data.
- Vertex has submitted regulatory applications to the FDA and 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), and the Kingdom of Bahrain (Bahrain) for the treatment of both SCD and TDT, and launches are ongoing.*

- Vertex has completed regulatory submissions for CASGEVY for SCD and TDT in Switzerland and in Canada, where it received Priority Review.
- As of mid-July, Vertex has activated more than 35 authorized treatment centers (ATCs) globally and increasing numbers of patients across all regions have initiated cell collection.
- The French National Authority for Health (HAS) approved Vertex's request for the implementation of an early access program (EAP) for the use of CASGEVY in indicated patients with SCD. HAS previously approved the implementation of an EAP for CASGEVY in indicated patients with TDT in the first quarter of 2024.

- Vertex continues to generate data from CLIMB-111, CLIMB-121 and the long-term follow-up study of CASGEVY and presented positive long-term data at the 2024 Annual European Hematology Association (EHA) Congress in June. These long-term data from more than 100 patients dosed with CASGEVY, with the longest follow-up of more than five years, confirm the transformative, consistent, and durable clinical benefits of CASGEVY over time.

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 without the limitations of opioids and other currently available medicines.

- The FDA accepted the NDA submission for suzetrigine for the treatment of moderate-to-severe acute pain and granted Priority Review with a PDUFA target action date of January 30, 2025. Suzetrigine has already been granted FDA Fast Track and Breakthrough Therapy designations for the treatment of 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 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 next-in-class triple oral small molecule combination, in cystic fibrosis***
  - Vertex initiated 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 late last year, and the multiple ascending dose (MAD) portion of the study is ongoing. Vertex expects to complete the trial and share data from this study 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 to more than 150,000 people in the U.S. and Europe alone.

Acute Pain

- Vertex is enrolling and dosing a Phase 1 trial for an intravenous formulation of VX-993, a next-generation selective NaV1.8 pain signal inhibitor.
- Vertex is on track to initiate a Phase 2 study this quarter with an oral formulation of VX-993 for the treatment of moderate to severe acute pain following bunionectomy surgery.

Peripheral Neuropathic Pain (PNP)

- 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, this quarter. The pivotal program is designed with two identical randomized controlled trials of approximately 1,100 patients each, studying suzetrigine at a once-daily 70mg dose. The primary endpoint is the change from baseline in the weekly average of daily pain intensity on the numeric pain rating scale (NPRS) at week 12 compared to placebo. The study also includes an active comparator arm of pregabalin, and a key secondary endpoint is non-inferiority on change from baseline to week 12 in NPRS score versus pregabalin. The FDA has granted suzetrigine Breakthrough Therapy Designation in DPN.
- Vertex has completed enrollment in its Phase 2 study of suzetrigine in painful lumbosacral radiculopathy (LSR), a condition representing more than 40% of patients suffering from PNP. Vertex expects to share results from this study in late 2024.
- Vertex is on track to initiate a Phase 2 study this quarter with an oral formulation of VX-993 for the treatment of DPN.

Consistent with its commitment to serial innovation and leadership in pain, Vertex also 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 an

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, in which a 45 mg once-daily dose of inaxaplin is compared to placebo, on top of standard of care.
- The study is designed to have a pre-planned interim analysis at Week 48 evaluating estimated glomerular filtration rate (eGFR) slope, a measure of kidney function, 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.

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 IgA nephropathy, a serious progressive, autoimmune kidney disease that can lead to end-stage renal disease. IgAN is the most common form of glomerulonephritis worldwide, affecting approximately 130,000 people in the U.S. alone, and there are currently no approved therapies that target its underlying cause. Vertex is also studying povetacicept in other serious B cell-mediated diseases, including autoimmune kidney diseases and autoimmune cytopenias.

- Following successful end-of-phase 2 regulatory interactions, Vertex is on track to initiate the Phase 3 clinical trial of povetacicept in IgA nephropathy (the RAINIER study) this quarter.
- 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 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 Vertex to seek accelerated approval in the U.S. Final analysis will occur at two years of treatment, with a primary endpoint of total eGFR slope through Week 104.
- The RUBY3 (autoimmune kidney diseases) and RUBY4 (autoimmune cytopenias) Phase 2 basket trials are ongoing with data readouts from certain cohorts expected later this year and into 2025.

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:*
  - Vertex announced positive results from the ongoing Phase 1/2 study at the American Diabetes Association 84th Scientific Sessions Conference in June 2024.
    - All 12 patients who received the full dose of VX-880 as a single infusion demonstrated islet cell engraftment and glucose-responsive insulin production.
    - All of these patients achieved ADA-recommended HbA1c levels <7.0% and >70% time-in-range, and 11 of 12 patients reduced or eliminated use of exogenous insulin.
    - All three of the patients with at least 12 months of follow-up, and therefore evaluable for the primary endpoint, met the primary endpoint of elimination of severe hypoglycemic events (SHEs) with HbA1c <7.0%, and the secondary endpoint of insulin independence.
  - Based on these positive results, Vertex has expanded the Phase 1/2 study to include 37 total patients. Vertex has completed enrollment and dosing in the original Phase 1/2 17-patient study.
- *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 is underway and enrolling and dosing. 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.
- *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 VX-880 islet cells to protect the cells from the immune system. 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, which holds the potential to address the underlying cause of DM1.

- Vertex continues to enroll and dose patients in the global Phase 1/2 clinical trial for VX-670 in people with DM1 and expects to complete the single ascending dose (SAD) portion of the study by the end of 2024.
- Following completion of the SAD portion of the trial, Vertex will move into the MAD portion, where 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 is enrolling and dosing 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 variants in the PKD1 gene, which encodes the PC1 protein, estimated to be ~25,000 (or ~10%) of the overall patient population.

Alpha-1 Antitrypsin Deficiency (AATD)

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

- Based on Phase 1 biomarker analyses, Vertex has determined that VX-634 and VX-668, two investigational small molecule AAT correctors, would not deliver transformative efficacy for people with AATD. As such, Vertex has discontinued development of both molecules.
- Consistent with its portfolio approach to research and development, Vertex is using the learnings from VX-634, VX-668 and prior molecules to continue to optimize the small molecule corrector and other approaches in the preclinical research phase.

Investments in External Innovation

- In May, Vertex completed its previously announced acquisition of Alpine Immune Sciences for approximately $5.0 billion in cash, including Alpine’s lead asset povetacicept, resulting in an approximate $4.4 billion charge to AIPR&D expenses in the second quarter of 2024.

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 June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
Product revenues, net	$ 2,645.6	$ 2,493.2	$ 5,336.2	$ 4,868.0

Costs and expenses:				
Cost of sales	371.9	308.6	714.5	575.5
Research and development expenses	966.6	785.7	1,755.7	1,528.3
Acquired in-process research and development expenses	4,449.1	110.5	4,525.9	457.6
Selling, general and administrative expenses	372.2	262.6	714.9	503.7
Change in fair value of contingent consideration	0.5	(0.6)	0.4	(2.5)
Total costs and expenses	6,160.3	1,466.8	7,711.4	3,062.6
(Loss) income from operations	(3,514.7)	1,026.4	(2,375.2)	1,805.4
Interest income	156.5	144.7	337.7	267.3
Interest expense	(9.9)	(11.2)	(20.3)	(22.6)
Other (expense) income, net	(23.1)	1.6	(54.3)	2.9
(Loss) income before provision for income taxes	(3,391.2)	1,161.5	(2,112.1)	2,053.0
Provision for income taxes	202.4	245.8	381.9	437.5
Net (loss) income	$ (3,593.6)	$ 915.7	$ (2,494.0)	$ 1,615.5
Net (loss) income per common share:				
Basic	$ (13.92)	$ 3.55	$ (9.66)	$ 6.27
Diluted	$ (13.92)	$ 3.52	$ (9.66)	$ 6.21
Shares used in per share calculations:				
Basic	258.1	257.7	258.1	257.6
Diluted	258.1	260.4	258.1	260.3

Vertex Pharmaceuticals Incorporated

Product Revenues

(in millions)(unaudited)

Three Months Ended June 30,		**Six Months Ended June 30,**	
2024	**2023**	**2024**	**2023**

TRIKAFTA/KAFTRIO	$ 2,449.2	$ 2,240.4	$ 4,932.8	$ 4,337.1
Other CF products	196.4	252.8	403.4	530.9
Product revenues, net	$ 2,645.6	$ 2,493.2	$ 5,336.2	$ 4,868.0

Vertex Pharmaceuticals Incorporated

Reconciliation of GAAP to Non-GAAP Financial Information

(in millions, except percentages)(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
GAAP cost of sales	$ 371.9	$ 308.6	$ 714.5	$ 575.5
Stock-based compensation expense	(1.8)	(1.8)	(3.6)	(3.7)
Intangible asset amortization expense	(5.1)	—	(10.1)	—
Non-GAAP cost of sales	$ 365.0	$ 306.8	$ 700.8	$ 571.8
GAAP research and development expenses	$ 966.6	$ 785.7	$ 1,755.7	$ 1,528.3
Stock-based compensation expense	(97.1)	(74.5)	(216.5)	(150.8)
Acquisition-related costs (3)	(172.3)	(2.8)	(172.3)	(5.6)
Non-GAAP research and development expenses	$ 697.2	$ 708.4	$ 1,366.9	$ 1,371.9
GAAP selling, general and administrative expenses	$ 372.2	$ 262.6	$ 714.9	$ 503.7
Stock-based compensation expense	(55.3)	(43.0)	(126.0)	(87.2)
Acquisition-related costs (3)	(36.5)	—	(36.5)	—
Non-GAAP selling, general and administrative expenses	$ 280.4	$ 219.6	$ 552.4	$ 416.5
Combined non-GAAP R&D and SG&A expenses	$ 977.6	$ 928.0	$ 1,919.3	$ 1,788.4
GAAP other (expense) income, net	$ (23.1)	$ 1.6	$ (54.3)	$ 2.9

Decrease (increase) in fair value of strategic investments	12.7	0.4	39.7	(6.0)
Non-GAAP other (expense) income, net	$ (10.4)	$ 2.0	$ (14.6)	$ (3.1)
GAAP provision for income taxes	$ 202.4	$ 245.8	$ 381.9	$ 437.5
Tax adjustments (1)	98.2	23.6	179.8	46.3
Non-GAAP provision for income taxes	$ 300.6	$ 269.4	$ 561.7	$ 483.8
GAAP effective tax rate	(6.0)%	21.2%	(18.1)%	21.3%
Non-GAAP effective tax rate	(10.0)%	21.0%	(37.3)%	21.1%

Vertex Pharmaceuticals Incorporated

Reconciliation of GAAP to Non-GAAP Financial Information (continued)

(in millions, except per share amounts)(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
GAAP operating (loss) income	$ (3,514.7)	$ 1,026.4	$ (2,375.2)	$ 1,805.4
Stock-based compensation expense	154.2	119.3	346.1	241.7
Intangible asset amortization expense	5.1	—	10.1	—
Increase (decrease) in fair value of contingent consideration	0.5	(0.6)	0.4	(2.5)
Acquisition-related costs (3)	208.8	2.8	208.8	5.6
Non-GAAP operating (loss) income	$ (3,146.1)	$ 1,147.9	$ (1,809.8)	$ 2,050.2
GAAP net (loss) income	$ (3,593.6)	$ 915.7	$ (2,494.0)	$ 1,615.5
Stock-based compensation expense	154.2	119.3	346.1	241.7
Intangible asset amortization expense	5.1	—	10.1	—
Decrease (increase) in fair value of strategic investments	12.7	0.4	39.7	(6.0)

Increase (decrease) in fair value of contingent consideration	0.5	(0.6)	0.4	(2.5)
Acquisition-related costs (3)	208.8	2.8	208.8	5.6
Total non-GAAP adjustments to pre-tax (loss) income	381.3	121.9	605.1	238.8
Tax adjustments (1)	(98.2)	(23.6)	(179.8)	(46.3)
Non-GAAP net (loss) income	$ (3,310.5)	$ 1,014.0	$ (2,068.7)	$ 1,808.0
Net (loss) income per diluted common share:				
GAAP	$ (13.92)	$ 3.52	$ (9.66)	$ 6.21
Non-GAAP	$ (12.83)	$ 3.89	$ (8.02)	$ 6.95
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	258.1	260.4	258.1	260.3

Notes

1: In the three and six months ended June 30, 2024 and 2023, “Tax adjustments” included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income (loss) 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 three and six months ended June 30, 2024, “Acquisition-related costs” included primarily related to compensation expense associated with cash-settled unvested Alpine equity awards.

Vertex Pharmaceuticals Incorporated

Condensed Consolidated Balance Sheets

(in millions)(unaudited)

	June 30, 2024	**December 31, 2023**
Assets		
Cash, cash equivalents and marketable securities	$ 5,795.5	$ 11,218.3
Accounts receivable, net	1,656.1	1,563.4
Inventories	914.6	738.8

Prepaid expenses and other current assets	575.4	623.7
Total current assets	8,941.6	14,144.2
Property and equipment, net	1,200.9	1,159.3
Goodwill and intangible assets, net	1,925.5	1,927.9
Deferred tax assets	2,185.6	1,812.1
Operating lease assets	569.8	293.6
Long-term marketable securities	4,393.1	2,497.8
Other long-term assets	915.6	895.3
Total assets	$ 20,132.1	$ 22,730.2
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 3,267.9	$ 3,020.2
Other current liabilities	279.3	527.2
Total current liabilities	3,547.2	3,547.4
Long-term finance lease liabilities	346.6	376.1
Long-term operating lease liabilities	586.8	348.6
Other long-term liabilities	876.8	877.7
Shareholders' equity	14,774.7	17,580.4
Total liabilities and shareholders' equity	$ 20,132.1	$ 22,730.2
Common shares outstanding	258.0	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) expectations, plans, and status of the potential near-term commercial launch of the vanzacaftor triple, (iii) expectations regarding long-term reimbursement agreements and access to our CF medicines in Europe and the U.K., (iv) our beliefs regarding the anticipated benefits of CASGEVY, our expectations regarding the number of patients initiating cell collection, and our beliefs with respect to the long-term follow-up study of CASGEVY, (v) expectations regarding the potential benefits and commercial success of the product candidates in our pain program, including plans and status of the potential near-term commercial launch of suzetrigine for the treatment of moderate-to-severe acute pain, (vi) plans to continue to advance new oral small molecule combination therapies for the treatment of CF (vii) expectations for our VX-522 Phase 1/2 study, including the potential benefits of this nebulized mRNA therapy and expectations to complete the trial and share data in the first half of 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 in the U.S. and Europe, (ix) plans to initiate a Phase 2 study with an oral formulation of VX-993 for the treatment of acute pain during the third quarter of 2024, (x) expectations regarding the study design and primary endpoint for the Phase 3 pivotal program of suzetrigine in patients with DPN, including plans to initiate the study in the third quarter of 2024, expectations to share results from the Phase 2 study of suzetrigine in LSR in late 2024, expectations to initiate a Phase 2 study with an oral formulation of VX-993 for the treatment of DPN in the third quarter of 2024, and plans to continue to develop NaV1.8 and 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 to seek accelerated approval in the U.S., (xii) expectations with respect to povetacicept, including our beliefs about its potential benefits and therapeutic scope, plans to initiate a Phase 3 clinical trial in IgAN, study designs and our expectations that the interim analysis of this study may serve as the basis to seek accelerated approval in U.S., and expectations regarding the RUBY3 and RUBY4 Phase 2 basket trials, including our expectations on timing for data readouts, (xiii) expectations regarding our T1D programs, including the status of each our studies evaluating VX-880 and VX-264, (xiv) expectations for the potential benefits and clinical status of VX-670 for the treatment in people with DM1, including plans to complete the SAD portion of the trial by end of 2024, and move into the MAD portion of the trial (xv) expectations regarding our ADPKD program, including the potential benefits of VX-407 and our beliefs regarding the targeted patient population, and (xvi) expectations with respect to our plans for our AATD program. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 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 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 successfully profit from the acquisition of Alpine Immune Sciences, 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.

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 Second 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.

(VRTX-E)

View source version on businesswire.com: https://www.businesswire.com/news/home/20240801201006/en/

Vertex Contacts:
Investor Relations:
Susie Lisa, CFA, 617-341-6108
Manisha Pai, 617-961-1899
Miroslava Minkova, 617-341-6135

Media:
617-341-6992
mediainfo@vrtx.com

Source: Vertex Pharmaceuticals Incorporated