Vertex Reports Second Quarter 2025 Financial Results

August 4, 2025

— Total revenue of $2.96 billion, a 12% increase compared to Q2 2024; reiterated full year 2025 financial guidance, including revenue guidance of $11.85 to $12 billion —

— Continued strong execution of CASGEVY®, ALYFTREK® and JOURNAVX® launches —

— Rapid advancement of next wave of clinical programs through pivotal development, with suzetrigine in DPN, zimislecel in T1D, povetacicept in IgAN and pMN, and inaxaplin in AMKD —

—David Altshuler, M.D., PhD., Chief Scientific Officer (CSO), announces intent to retire August 1, 2026; as part of planned transition, Mark Bunnage, D.Phil., SVP of Global Research, to assume role of CSO effective February 1, 2026 —

BOSTON--(BUSINESS WIRE)--Aug. 4, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the second quarter ended June 30, 2025, and reiterated full year 2025 financial guidance.

"Vertex delivered a strong quarter of revenue growth with each of our three product launches — ALYFTREK, JOURNAVX, and CASGEVY — contributing, as well as continued advancement of our clinical programs," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "As we enter the second half of the year, we are focused on expanding leadership in cystic fibrosis, executing the launches, advancing the pipeline, and preparing for submissions and commercialization in additional disease areas." Dr. Kewalramani added, "I would like to express my deep gratitude to David for his exceptional scientific vision and patient impact over the last 13 years. As CSO, David has been at the helm through the discovery, development and approval of four CF medicines, our groundbreaking CRISPR/Cas9 gene-edited therapy, and our novel non-opioid pain medicine. As part of the planned transition, I am delighted that Mark Bunnage, current SVP and Head of Global Research, will be the next CSO. He is a world-class scientist with long tenure at the company who has contributed enormously to our R&D success, working alongside David since 2016 and heading up discovery research since March 2024. Mark is the ideal leader to drive the next wave of innovation."

Second Quarter 2025 Results

Total revenue increased 12% to $2.96 billion compared to the second quarter of 2024, primarily driven by the continued performance of cystic fibrosis (CF) therapies and early contributions from the three ongoing launches. In the U.S., total revenue increased 14% to $1.85 billion due to continued strong patient demand and favorable gross-to-net versus prior year. Outside the U.S., total revenue increased 8% to $1.12 billion due to strong performance across multiple geographies.

Combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expenses were $1.4 billion and $1.2 billion, respectively, compared to $5.8 billion and $5.4 billion, respectively, in the second quarter of 2024. The decreases were primarily due to $4.4 billion of Acquired IPR&D (AIPR&D) expenses associated with Vertex's acquisition of Alpine Immune Sciences incurred in the second quarter of 2024, partially offset by continued R&D investment in support of multiple mid- and late-stage clinical development programs and increased commercial investment to support the launch of JOURNAVX.

GAAP and non-GAAP effective tax rates were 19.5% and 19.4%, respectively, compared to (6.0)% and (10.0)%, respectively, for the second quarter of 2024. In the second quarter of 2024, Vertex reported a pre-tax loss, due to the impact of non-deductible AIPR&D expenses related to the Alpine acquisition.

GAAP and non-GAAP net income were $1.0 billion and $1.2 billion, respectively, compared to net losses of $3.6 billion and $3.3 billion, respectively, for the second quarter of 2024, 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, 2025, were $12.0 billion, compared to $11.2 billion as of December 31, 2024. The increase was primarily due to cash flows from operating activities, partially offset by repurchases of Vertex's common stock pursuant to its share repurchase programs and income tax payments.

Full Year 2025 Financial Guidance

Vertex today reiterated its full year 2025 financial guidance, including revenue guidance of $11.85 billion to $12 billion, which assumes continued growth in CF, including the global launch of ALYFTREK; continued uptake of CASGEVY in multiple regions; and early contributions from the U.S. launch of JOURNAVX. Vertex also reiterated its guidance for both combined GAAP and non-GAAP R&D, AIPR&D, and SG&A expenses, which includes expectations for continued investment in multiple mid- and late-stage clinical development programs and commercial capabilities, and AIPR&D expenses of approximately $100 million. In addition, Vertex reiterated non-GAAP effective tax rate guidance of 20.5-21.5%. Vertex does not currently expect a material change to its 2025 non-GAAP effective tax rate, following the enactment of the recent tax legislation under H.R.1. This financial guidance also includes an immaterial cost impact from tariffs in 2025 based on currently known tariff rates and regulations.

Vertex's financial guidance is summarized below:

	Current FY 2025	Previous FY 2025

Total revenue	Unchanged	$11.85 to $12.0 billion
Combined GAAP R&D, AIPR&D and SG&A expenses *	Unchanged	$5.55 to $5.7 billion
Combined non-GAAP R&D, AIPR&D and SG&A expenses *	Unchanged	$4.9 to $5.0 billion
Non-GAAP effective tax rate	Unchanged	20.5% to 21.5%

*The difference between the combined GAAP R&D, AIPR&D and SG&A expenses and the combined non-GAAP R&D, AIPR&D and SG&A expenses guidance relates primarily to $650 million to $700 million of stock-based compensation expense.

**Combined GAAP and Non-GAAP R&D, AIPR&D and SG&A expenses guidance includes approximately $100 million of AIPR&D expenses.

Key Business Highlights

Marketed Products
Cystic Fibrosis (CF) Portfolio
Vertex has worked for more than 20 years to discover and develop medicines to treat the underlying cause of CF. Vertex CFTR modulators can treat nearly 95 percent of all people living with CF in core markets and are approved for patients as young as one month old. ALYFTREK, the newest marketed CFTR modulator, is approved in the U.S., the United Kingdom (U.K.), Europe and Canada for the treatment of patients 6 years and older. Vertex anticipates that the number of CF patients taking its medicines will continue to grow through new approvals and reimbursement agreements, treatment of younger patients, increased survival, and expansion into additional geographies. Recent and anticipated progress includes:

- Vertex secured European Commission approval of ALYFTREK, the once-daily, next-in-class combination CFTR modulator, for the treatment of people with CF ages 6 years and older who have at least one F508del mutation or another responsive mutation in the CFTR gene. Eligible patients in Germany and Denmark already have access to ALYFTREK, and Vertex expects that eligible patients in Ireland will have access in the coming weeks. Vertex continues to work with reimbursement bodies across additional EU member states to ensure access for all eligible patients as quickly as possible. In addition, Vertex entered into a reimbursement agreement with the National Health Service (NHS) England for eligible CF patients to access ALYFTREK.
- In July, Vertex received approval from Health Canada for ALYFTREK for the treatment of people with CF ages 6 years and older who have at least one F508del mutation or another responsive mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. Vertex is working to secure reimbursement for eligible patients in Canada.
- Regulatory reviews are underway for ALYFTREK in Switzerland, Australia, and New Zealand.

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), Qatar, Canada, Switzerland and the United Arab Emirates (UAE) for the treatment of both SCD and TDT. In total, there are more than 60,000 eligible patients in these countries, including approximately 37,000 in North America and Europe and more than 23,000 in the Middle East. Recent highlights include:*

- Through reimbursement agreements, Vertex has secured access for eligible SCD and TDT patients in 10 countries. Countries with recent reimbursement agreements include Northern Ireland, Scotland and Denmark. Vertex continues to work with government and reimbursement authorities globally to secure access for eligible patients.
- Vertex has met its goal of activating more than 75 authorized treatment centers (ATCs) globally. Globally, since launch through June 30th, 2025, approximately 115 patients have had their first cell collection, and 29 patients have received infusions of CASGEVY, including 16 infused in the second quarter of 2025.

JOURNAVX (suzetrigine) for the treatment of moderate-to-severe acute pain
JOURNAVX is a first-in-class, oral, selective, non-opioid NaV1.8 pain signal inhibitor, approved in the U.S. for the treatment of moderate-to-severe acute pain.

- Since JOURNAVX became available at pharmacies in early March through mid-July more than 110,000 prescriptions for JOURNAVX have been written and filled across the hospital and retail settings in different acute pain conditions, consistent with JOURNAVX's broad label.

- As of mid-July, across commercial and government payers, nearly 150 million individuals already have covered access to JOURNAVX, representing almost half of U.S. covered lives. This includes formal coverage agreements with two of the three large national pharmacy benefit managers (PBMs) and unrestricted access within 16 state Medicaid plans. Vertex expects access to JOURNAVX to continue to expand over the course of 2025.
- More than 50 of Vertex's targeted 150 large healthcare systems and more than 500 individual hospitals of the 2,000 targeted institutions have added JOURNAVX to formularies, protocols or order sets. Vertex now has national group purchasing agreements in place with two of the largest group purchasing organizations in the U.S.

Select Clinical-Stage R&D Pipeline

Cystic Fibrosis

- Vertex is completing Phase 3 studies in younger age groups to expand the TRIKAFTA/KAFTRIO and ALYFTREK labels and enable earlier treatment of children with CF. Vertex recently completed enrollment in a global study of ALYFTREK in children 2 to 5 years of age.
- Vertex is on track to complete the healthy volunteer study of VX-828, the once-daily, next-generation 3.0 corrector, and advance the VX-828 combination into a CF cohort this year.
- The Independent Data Monitoring Committee (IDMC) has completed its review of VX-522, a nebulized CFTR mRNA therapy, and endorsed restart of the Phase 1/2 study. Vertex expects to resume dosing in the multiple ascending dose (MAD) portion of the study in the near term.

Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia

- Vertex has completed enrollment of children 5 to 11 years of age with SCD or TDT in two global Phase 3 studies of CASGEVY and is on track to complete dosing in the second half of 2025.
- Vertex continues to advance preclinical assets for gentler conditioning for CASGEVY, which could broaden the eligible patient population.

Acute Pain

- Vertex announced results from its Phase 2 placebo-controlled dose-ranging study evaluating the safety and efficacy of VX-993, an investigational selective NaV1.8 pain signal inhibitor, in treating acute pain after bunionectomy surgery. Treatment with VX-993 did not result in a statistically significant improvement on the primary endpoint of the time-weighted sum of the pain intensity difference from 0 to 48 hours (SPID48) compared to placebo. VX-993 was generally safe and well-tolerated. Based on these results, Vertex will not further advance VX-993 as monotherapy in acute pain.

Peripheral Neuropathic Pain (PNP)

- Vertex continues to enroll and dose patients with diabetic peripheral neuropathy (DPN) in a Phase 3 pivotal trial of suzetrigine. The U.S. Food and Drug Administration (FDA) has granted suzetrigine Fast Track designation in PNP and Breakthrough Therapy designation in DPN.
- As part of an End of Phase 2 discussion with the FDA, the Agency indicated they do not see a path to a broad PNP label at this time. As such, Vertex will not initiate a Phase 3 lumbosacral radiculopathy (LSR) study but instead will prioritize DPN as the first PNP indication and begin a second DPN Phase 3 study in the near term. Vertex expects to complete enrollment in both DPN Phase 3 studies by the end of next year. Vertex will continue to work with the FDA to expand the DPN indication over time to include additional neuropathic pain conditions and assess potential pathways to secure a broad PNP label.
- Vertex continues to enroll and dose patients in a Phase 2 study for the oral formulation of VX-993 for the treatment of DPN.

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.

- Vertex is on track to complete enrollment and dosing in the Phase 3 portion of the Phase 1/2/3 global study of zimislecel in patients with T1D with severe hypoglycemic events and impaired awareness of hypoglycemia in the near term. Vertex expects global regulatory submissions for zimislecel in 2026.
- Zimislecel has been granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. Food and Drug Administration, Priority Medicines (PRIME) designation from the EMA, and has secured an Innovation Passport under the Innovative Licensing and Access Pathway (ILAP) from the UK Medicines and Healthcare products Regulatory Agency (MHRA).
- Vertex announced positive results from the Phase 1/2 portion of the Phase 1/2/3 clinical trial of zimislecel in an oral presentation at the American Diabetes Association 85th Scientific Sessions and simultaneously published these data in the *New England Journal of Medicine.* All 12 participants who received the full dose in a single infusion and were followed

for at least one year:
    - Demonstrated engraftment with glucose-responsive endogenous C-peptide production, which was durable through the period of follow-up;
    - Achieved the ADA/EASD-recommended targets of HbA1c <7% and time in range of >70% and were free of SHEs from day 90 onwards;
    - Had a reduction in exogenous insulin use (mean reduction in daily insulin dose: 92%), and 10/12 (83%) no longer required exogenous insulin at Month 12; and
    - Achieved the Phase 1/2 primary endpoint of elimination of SHEs with HbA1c <7%.
- Vertex is pursuing research-stage programs to evaluate additional approaches that could provide transformative benefits to people with T1D and reduce or eliminate the need for standard immunosuppressive regimens. These approaches include improved immunosuppression, gene editing, and novel immunoprotection to encapsulate the islet cells.

IgA Nephropathy (IgAN), Primary Membranous Nephropathy (pMN) and Other B Cell-Driven Diseases
Vertex is developing povetacicept, a dual antagonist of the BAFF and APRIL cytokines, which play key roles in the pathogenesis of multiple B cell-driven diseases. Povetacicept represents a potentially best-in-class approach to treat IgAN and pMN and has pipeline-in-a-product potential.

- The global Phase 3 RAINIER trial of povetacicept in patients with IgAN completed enrollment of the interim analysis cohort in the second quarter. The interim analysis will be conducted once this cohort reaches 36 weeks of treatment, with the potential to file for Accelerated Approval in the U.S. in the first half of 2026, if results are supportive. Vertex expects to complete enrollment in the full study this year. Studies to support the launch of povetacicept for at-home self-administration are well underway.
- Based on the strength of the Phase 2 results in the RUBY-3 study, Vertex completed its End of Phase 2 meeting with the FDA and reached agreement with the Agency on the pivotal development program in pMN. Vertex is on track to initiate the Phase 2/3 trial of povetacicept in patients with pMN later this year.
- In addition, Vertex has prioritized generalized myasthenia gravis (gMG) and warm autoimmune hemolytic anemia (wAIHA) as the next potential indications for povetacicept. Other RUBY-3 and RUBY-4 indications have been deprioritized. In the U.S. and Europe alone, there are approximately: 300,000 people diagnosed with IgAN, 150,000 people diagnosed with pMN, 175,000 people diagnosed with gMG, and 35,000 people diagnosed with primary wAIHA.
- Vertex entered into an exclusive collaboration and license agreement in June with Ono Pharmaceutical Co., Ltd. for the development and commercialization of povetacicept in Japan and South Korea. This partnership further expands upon the geographic expansion momentum started through a partnership with Zai Lab announced this past January.

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 cause of this genetic kidney disease.

- Vertex is on track to complete enrollment in the interim analysis cohort of the AMPLITUDE Phase 2/3 trial of inaxaplin this year. Vertex will conduct the pre-planned interim analysis once this cohort has been treated for 48 weeks, with potential to file for accelerated approval in the U.S. if the results are supportive. The study has now met its minimum target for pediatric enrollment (ages 10-17) and continues to enroll both pediatric and adult patients.
- The U.S. Center for Medicare and Medicaid (CMS) recently updated their list of diagnostic codes, known as ICD-10-CM codes, to include new codes for APOL1-mediated kidney disease (AMKD). This represents significant progress for patients and physicians, as the new codes will enable broader recognition and diagnosis of AMKD.
- Vertex continues to enroll and dose patients in the AMPLIFIED Phase 2 study of inaxaplin in people with AMKD and diabetes or other co-morbidities and is on track to complete enrollment of the study by the end of 2025.

Myotonic Dystrophy Type 1 (DM1)
Vertex is evaluating multiple approaches that target the underlying cause of DM1. Vertex's lead approach, VX-670, is an oligonucleotide linked to a cyclic peptide, which holds the potential to address the underlying cause of DM1.

- Vertex continues to enroll and dose the MAD portion of the global Phase 1/2 clinical trial of VX-670 in people with DM1, which will assess both safety and efficacy. Vertex is on track to complete enrollment and dosing in the trial in the first half of 2026.

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.

- Vertex has completed the Phase 1 study of VX-407 in healthy volunteers.
- Vertex is on track to initiate a Phase 2 proof-of-concept study of VX-407 in the near term in patients with a subset of variants in the PKD1 gene, which encodes the PC1 protein, estimated to be up to ~30,000 (or up to ~10%) of the overall patient population.

Additional Earlier Stage R&D Programs

Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs, with additional assets in CF, SCD, TDT, pain, AMKD, T1D, DM1, and ADPKD in earlier stages of development. Additionally, Vertex is working on preclinical molecules with the potential to expand its leadership in existing disease areas, including assets targeting improved immunosuppression for zimislecel, gentler conditioning for CASGEVY, and inhibition of NaV1.7 in pain.

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, (vi) an intangible asset impairment charge, and (vii) other adjustments. The company's non-GAAP financial results also exclude from its provision for income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income (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, AIPR&D and SG&A expenses and effective tax rate on a non-GAAP basis. Unless otherwise noted, the guidance regarding combined R&D, AIPR&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.

Vertex Pharmaceuticals Incorporated

Consolidated Statements of Income (Loss)

(unaudited, in millions, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Product revenues, net	$ 2,944.0	$ 2,645.6	$ 5,704.2	$ 5,336.2
Other revenues	20.7	—	30.7	—
Total revenues	2,964.7	2,645.6	5,734.9	5,336.2
Costs and expenses:				
Cost of sales	407.5	371.9	770.5	714.5
Research and development expenses	978.4	966.6	1,958.1	1,755.7
Acquired in-process research and development expenses	2.2	4,449.1	22.0	4,525.9
Selling, general and administrative expenses	424.6	372.2	821.0	714.9
Intangible asset impairment charge	—	—	379.0	—
Change in fair value of contingent consideration	0.9	0.5	3.1	0.4

Total costs and expenses	1,813.6	6,160.3	3,953.7	7,711.4
Income (loss) from operations	1,151.1	(3,514.7)	1,781.2	(2,375.2)
Interest income	122.4	156.5	243.3	337.7
Interest expense	(3.7)	(9.9)	(6.7)	(20.3)
Other income (expense), net	13.2	(23.1)	(4.4)	(54.3)
Income (loss) before provision for income taxes	1,283.0	(3,391.2)	2,013.4	(2,112.1)
Provision for income taxes	250.1	202.4	334.2	381.9
Net income (loss)	$ 1,032.9	$ (3,593.6)	$ 1,679.2	$ (2,494.0)
Net income (loss) per common share:				
Basic	$ 4.02	$ (13.92)	$ 6.54	$ (9.66)
Diluted	$ 3.99	$ (13.92)	$ 6.48	$ (9.66)
Shares used in per share calculations:				
Basic	256.7	258.1	256.8	258.1
Diluted	258.9	258.1	259.2	258.1

Vertex Pharmaceuticals Incorporated

Total Revenues

(unaudited, in millions)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
TRIKAFTA/KAFTRIO	$ 2,551.1	$ 2,449.2	$ 5,086.6	$ 4,932.8
ALYFTREK	156.8	—	210.7	—
Other product revenues (1)	236.1	196.4	406.9	403.4
Product revenues, net	2,944.0	2,645.6	5,704.2	5,336.2
Other revenues	20.7	—	30.7	—

Total revenues	$ 2,964.7	$ 2,645.6	$ 5,734.9	$ 5,336.2

1: In the three and six months ended June 30, 2025, "Other product revenues" included $30.4 million and $44.6 million from CASGEVY, respectively, and $12.0 million and $13.3 million, respectively, from JOURNAVX. In the three and six months ended June 30, 2024, there were no revenues for these products. The remaining “Other product revenues” are related to KALYDECO, ORKAMBI, and SYMDEKO/SYMKEVI, our other CF products.

Vertex Pharmaceuticals Incorporated

Reconciliation of GAAP to Non-GAAP Financial Information

(unaudited, in millions, except percentages)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
GAAP cost of sales	$ 407.5	$ 371.9	$ 770.5	$ 714.5
Stock-based compensation expense	(2.5)	(1.8)	(5.1)	(3.6)
Intangible asset amortization expense	(5.1)	(5.1)	(10.1)	(10.1)
Non-GAAP cost of sales	$ 399.9	$ 365.0	$ 755.3	$ 700.8
GAAP research and development expenses	$ 978.4	$ 966.6	$ 1,958.1	$ 1,755.7
Stock-based compensation expense	(99.6)	(97.1)	(199.7)	(216.5)
Intangible asset amortization expense	(0.7)	—	(1.3)	—
Acquisition-related costs (2)	—	(172.3)	—	(172.3)
Non-GAAP research and development expenses	$ 878.1	$ 697.2	$ 1,757.1	$ 1,366.9
Acquired in-process research and development expenses	$ 2.2	$ 4,449.1	$ 22.0	$ 4,525.9
GAAP selling, general and administrative expenses	$ 424.6	$ 372.2	$ 821.0	$ 714.9
Stock-based compensation expense	(65.2)	(55.3)	(128.6)	(126.0)
Acquisition-related costs (2)	—	(36.5)	—	(36.5)
Non-GAAP selling, general and administrative expenses	$ 359.4	$ 280.4	$ 692.4	$ 552.4

Combined non-GAAP R&D, AIPR&D and SG&A expenses	$ 1,239.7	$ 5,426.7	$ 2,471.5	$ 6,445.2
GAAP other income (expense), net	$ 13.2	$ (23.1)	$ (4.4)	$ (54.3)
(Increase) decrease in fair value of strategic investments	(5.4)	12.7	9.6	39.7
Non-GAAP other income (expense), net	$ 7.8	$ (10.4)	$ 5.2	$ (14.6)
GAAP provision for income taxes	$ 250.1	$ 202.4	$ 334.2	$ 381.9
Tax adjustments (3)	32.1	98.2	192.2	179.8
Non-GAAP provision for income taxes	$ 282.2	$ 300.6	$ 526.4	$ 561.7
GAAP effective tax rate	19.5 %	(6.0)%	16.6 %	(18.1)%
Non-GAAP effective tax rate	19.4 %	(10.0)%	19.1 %	(37.3)%

Vertex Pharmaceuticals Incorporated

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

(unaudited, in millions, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	**2024**	**2025**	**2024**
GAAP operating income (loss)	$ 1,151.1	$ (3,514.7)	$ 1,781.2	$ (2,375.2)
Stock-based compensation expense	167.3	154.2	333.4	346.1
Intangible asset impairment charge	—	—	379.0	—
Intangible asset amortization expense	5.8	5.1	11.4	10.1
Increase in fair value of contingent consideration	0.9	0.5	3.1	0.4
Acquisition-related costs (2)	—	208.8	—	208.8
Non-GAAP operating income (loss)	$ 1,325.1	$ (3,146.1)	$ 2,508.1	$ (1,809.8)

GAAP net income (loss)	$ 1,032.9	$ (3,593.6)	$ 1,679.2	$ (2,494.0)
Stock-based compensation expense	167.3	154.2	333.4	346.1
Intangible asset impairment charge	—	—	379.0	—
Intangible asset amortization expense	5.8	5.1	11.4	10.1
(Increase) decrease in fair value of strategic investments	(5.4)	12.7	9.6	39.7
Increase in fair value of contingent consideration	0.9	0.5	3.1	0.4
Acquisition-related costs (2)	—	208.8	—	208.8
Total non-GAAP adjustments to pre-tax income	168.6	381.3	736.5	605.1
Tax adjustments (3)	(32.1)	(98.2)	(192.2)	(179.8)
Non-GAAP net income (loss)	$ 1,169.4	$ (3,310.5)	$ 2,223.5	$ (2,068.7)
Net income (loss) per diluted common share:				
GAAP	$ 3.99	$ (13.92)	$ 6.48	$ (9.66)
Non-GAAP	$ 4.52	$ (12.83)	$ 8.58	$ (8.02)
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	258.9	258.1	259.2	258.1

2: In the three and six months ended June 30, 2024, “Acquisition-related costs” were primarily related to compensation expense associated with cash-settled unvested Alpine equity awards.

3: In the three and six months ended June 30, 2025 and 2024, “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.

Vertex Pharmaceuticals Incorporated

Condensed Consolidated Balance Sheets

(unaudited, in millions)

	June 30, 2025	**December 31, 2024**
Assets		
Cash, cash equivalents and marketable securities	$ 6,382.8	$ 6,115.9

Accounts receivable, net	1,893.5	1,609.4
Inventories	1,499.3	1,205.4
Prepaid expenses and other current assets	652.3	665.7
Total current assets	10,427.9	9,596.4
Property and equipment, net	1,335.1	1,227.8
Goodwill and other intangible assets, net	1,523.5	1,913.9
Deferred tax assets	2,711.7	2,331.1
Operating lease assets	1,313.8	1,356.8
Long-term marketable securities	5,645.9	5,107.9
Other long-term assets	1,078.8	999.3
Total assets	$ 24,036.7	$ 22,533.2

Liabilities and Shareholders' Equity

Accounts payable and accrued expenses	$ 3,713.0	$ 3,201.6
Other current liabilities	425.4	363.0
Total current liabilities	4,138.4	3,564.6
Long-term operating lease liabilities	1,527.4	1,544.4
Other long-term liabilities	1,195.5	1,014.6
Shareholders' equity	17,175.4	16,409.6
Total liabilities and shareholders' equity	$ 24,036.7	$ 22,533.2

Common shares outstanding	256.3	256.9

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, type 1 diabetes, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, and myotonic dystrophy type 1.

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 2025 Financial Guidance” and statements regarding (i) expectations for Vertex’s continued growth in CF, including by increasing the number of CF patients taking its medicines through new approvals and reimbursements, treatment of younger patients, increased survival, and expansion into additional geographies, (ii) expectations regarding access to ALYFTREK in Ireland and other E.U member states, (iii) beliefs regarding the anticipated benefits, commercial launch progress, and access for CASGEVY, (iv) expectations regarding the potential benefits of JOURNAVX for the treatment of moderate-to-severe acute pain, including regarding the efficacy and safety of JOURNAVX, beliefs that JOURNAVX has potential to provide effective pain relief without the limitations of opioids and other available medicines, expectations with respect to the commercial launch progress and that access for JOURNAVX will continue to expand throughout 2025, (v) expectations to expand the labels for TRIKAFTA/KAFTRIO and ALYFTREK and enable earlier treatment of children with CF, (vi) expectations to complete the healthy volunteer study of VX-828 combination into people with CF and advance the VX-828 combination into a CF cohort this year, (vii) expectations to resume dosing in the MAD portion of the Phase 1/2 study of VX-522 in the near term, (viii) expectations regarding Vertex’s SCD and TDT program, including with respect to completing dosing in two Phase 3 studies of CASGEVY in the second half of 2025, and with respect to gentler conditioning for CASGEVY broadening the eligible patient population, (ix) our plans not to advance VX-993 as monotherapy in acute pain, (x) plans to begin a second DPN Phase 3 study, expectations to complete enrollment in both DPN Phase 3 studies by the end of next year, and expectations to work with the FDA to broaden the DPN indication over time and include additional PNP conditions, (xi) expectations for the Phase 2 study of the oral formulation of VX-993 in DPN, (xii) expectations regarding the pivotal study evaluating zimislecel in T1D, including the expectations to complete enrollment and dosing in the near term and submit global regulatory filings, and expectations and plans to pursue additional approaches to standard immunosuppression regimens, (xiii) expectations with respect to povetacicept, including beliefs about its potential benefits and therapeutic scope, study designs, and expectations regarding the Phase 3 RAINIER study, including plans to complete enrollment in the full study this year and apply for potential accelerated U.S. regulatory approval in 2026 if results are supportive, expectations and plans with respect to advancing povetacicept into pivotal development in pMN later this year, beliefs with respect to the RUBY-4 basket study, (xiv) expectations regarding the AMPLITUDE trial in AMKD, including expectations to complete enrollment in the interim analysis cohort in the second half of 2025 and apply for potential accelerated U.S. if results are supportive, (xv) beliefs regarding the potential benefits and clinical status of VX-670 for the treatment in people with DM1 and expectations to complete enrollment and dosing in the first half of 2026, and (xvi) expectations regarding the ADPKD program, including the potential benefits of VX-407 and expectations to advance VX-407 into a Phase 2 proof-of-concept study in the near term. 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 2025 full year 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 we may be unable to successfully commercialize ALYFTREK as a treatment for CF or JOURNAVX as a treatment for acute 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 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 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 2025 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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