



Vertex Reports Third Quarter 2025 Financial Results

November 3, 2025

— Total revenue of \$3.08 billion, an 11% increase compared to Q3 2024 —

— Refined full year financial guidance: total revenue guidance now \$11.9 to \$12.0 billion and total combined non-GAAP R&D, AIPR&D, and SG&A expense guidance now \$5.0 to \$5.1 billion —

— R&D pipeline continues to make progress: five programs in pivotal development and povetacept Phase 3 IgAN trial full enrollment complete; on track to submit first module of povetacept IgAN BLA to FDA by end of 2025 —

BOSTON--(BUSINESS WIRE)--Nov. 3, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2025, and refined full year 2025 financial guidance.

"Vertex delivered strong results across the board in the third quarter, extending our leadership in CF, continuing to build global momentum for CASGEVY, and advancing the launch of JOURNAVX in acute pain," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "We also delivered strong progress across the R&D pipeline, with completion of enrollment in the Phase 3 study of povetacept in IgAN, initiation of the Phase 2/3 study of povetacept in primary membranous nephropathy, as well as advancement of several programs in research and earlier-stage clinical development. For the remainder of 2025, we are focused on executing the ongoing launches, initiating the povetacept BLA submission in IgAN for potential U.S. accelerated approval, advancing the pipeline, and preparing for new launches in additional disease areas."

Third Quarter 2025 Results

Total revenue increased 11% to \$3.08 billion compared to the third 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 15% to \$1.98 billion due to continued strong CF patient demand, including for ALYFTREK; contributions from CASGEVY and JOURNAVX; and favorable net pricing in CF versus the prior year. Outside the U.S., total revenue increased 4% to \$1.10 billion due to solid performance across multiple geographies.

Combined GAAP and non-GAAP R&D, Acquired IPR&D and SG&A expenses were \$1.5 billion and \$1.3 billion, respectively, compared to \$1.3 billion and \$1.1 billion, respectively, in the third quarter of 2024. The increases were primarily due to increased R&D investment in support of multiple mid- and late-stage clinical development programs, increased commercial investment to support the launch of JOURNAVX in acute pain, and higher AIPR&D.

GAAP and non-GAAP effective tax rates were 16.6% and 17.6%, respectively, compared to 14.6% and 19.8%, respectively, for the third quarter of 2024. In each of the third quarters of 2025 and 2024, the tax rate benefited from increased R&D tax credits.

GAAP and non-GAAP net income were \$1.1 billion and \$1.2 billion, respectively, compared to \$1.0 billion and \$1.1 billion, respectively, for the third quarter of 2024, primarily driven by increased product revenue partially offset by increased operating expenses.

Cash, cash equivalents, and total marketable securities as of September 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.

Full Year 2025 Financial Guidance

Vertex today refined its full year 2025 revenue guidance with total revenue now expected to be \$11.9 to \$12.0 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 is also revising full year 2025 guidance for combined GAAP and non-GAAP operating expenses. Combined GAAP and non-GAAP R&D, AIPR&D, and SG&A expenses are now expected to be approximately \$5.65 to \$5.8 billion and \$5.0 to \$5.1 billion, respectively. This revised outlook accounts for acceleration of povetacept programs and increased investment in sales and marketing initiatives for JOURNAVX in acute pain. In addition, Vertex lowered its prior non-GAAP effective tax rate guidance of 20.5% to 21.5% to a new range of 17% to 18% to incorporate several one-time tax benefits. These include one-time tax benefits recognized in the third quarter of 2025 from Alpine-related R&D tax credits, as well as anticipated recognition in the fourth quarter of 2025 of previously deferred tax benefits. 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:

	<u>Current FY 2025</u>	<u>Previous FY 2025</u>
Total revenue	\$11.9 to \$12.0 billion	\$11.85 to \$12.0 billion
Combined GAAP R&D, AIPR&D and SG&A expenses *	\$5.65 to \$5.8 billion	\$5.55 to \$5.7 billion
Combined non-GAAP R&D, AIPR&D and SG&A expenses *	\$5.0 to \$5.1 billion	\$4.9 to \$5.0 billion
Non-GAAP effective tax rate	17% to 18%	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.), the European Union (EU), Canada, New Zealand, and Switzerland 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 progress includes:

- The U.S. launch of ALYFTREK is progressing well across all eligible patient groups, and launches outside the U.S. are showing a strong start in multiple markets.
- Vertex received approval from the European Commission and Health Canada in July and from Medsafe New Zealand and Swissmedic in October 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. Regulatory review is underway for ALYFTREK in Australia.
- Eligible patients in England, Ireland, Germany, Denmark and Northern Ireland currently have reimbursed access to ALYFTREK, and Vertex is working to secure access for eligible patients in additional countries.
- In October at the North American Cystic Fibrosis Conference (NACFC), Vertex presented multiple abstracts on ALYFTREK as well as the association between lower sweat chloride with CFTR modulator use and improved clinical outcomes. ALYFTREK has shown greater reductions in sweat chloride compared to TRIKAFTA in Phase 3 trials, suggesting that ALYFTREK has the potential for even greater improvements in quality of life and other health-related outcomes.

CASGEVY for the treatment of severe 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:

- In September, Vertex announced a reimbursement agreement for patients with SCD and TDT in Italy. Italy has the largest population of people living with TDT in Europe, with approximately 5,000 people 12 years and older with TDT and around 2,300 with SCD.
- Globally, since launch through September 30th, 2025, approximately 165 people with SCD or TDT have had their first cell collection for CASGEVY, including 50 people in the third quarter of 2025; and 39 people with SCD or TDT have received infusions of CASGEVY, including 10 people infused in the third quarter of 2025.

JOURNAVX (suzetrigine) for the treatment of moderate-to-severe acute pain

JOURNAVX is a first-in-class, oral, selective, non-opioid Na_v1.8 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-October more than 300,000 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-October, across commercial and government payers, more than 170 million individuals already have covered

access to JOURNAVX, representing more than half of U.S. covered lives. This includes formal coverage with two of the three large national pharmacy benefit managers (PBMs) and unrestricted access within 19 state Medicaid plans. Vertex expects access to JOURNAVX to continue to expand over the remainder of 2025 and into 2026.

- Approximately 90 of Vertex's targeted 150 large healthcare systems and more than 750 individual hospitals of the 2,000 targeted institutions have added JOURNAVX to formularies, protocols or order sets.

Select Clinical-Stage Pipeline Programs

Cystic Fibrosis

- In October, Vertex completed the pivotal study of TRIKAFTA in children 12 months to <24 months of age. The data showed that TRIKAFTA was generally safe and well tolerated, consistent with the established safety profile. Treatment with TRIKAFTA in this age group resulted in rapid, robust, and clinically meaningful improvement in the secondary endpoint of sweat chloride reduction, with a mean reduction of 71.8 mmol/L through Week 24. Vertex expects to submit for approval with global regulators in the first half of 2026.
- Vertex recently completed enrollment in the global study of ALYFTREK in children 2 to 5 years of age. In this pivotal study, approximately 65 children will receive ALYFTREK for 24 weeks. The primary endpoint is safety and tolerability, and secondary endpoints include absolute changes in sweat chloride. Vertex expects to share results from this trial in the first half of 2026.
- Vertex has advanced VX-828, the once-daily, next-generation 3.0 corrector, into a cohort of patients with CF.
- Vertex has resumed dosing in the multiple ascending dose (MAD) portion of the Phase 1/2 study of VX-522, a nebulized CFTR mRNA therapy, for the approximately 5,000 patients who cannot benefit from CFTR modulators.

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 this quarter. Vertex will share emerging data from these studies in an oral presentation at the American Society of Hematology (ASH) annual meeting on December 6th, 2025.

Peripheral Neuropathic Pain (PNP)

- Vertex previously initiated the first Phase 3 trial evaluating suzetrigine in diabetic peripheral neuropathy (DPN), a form of PNP, and is on track to start the second Phase 3 study later this month. Vertex expects to complete enrollment in both Phase 3 studies by the end of 2026.
- Vertex continues to enroll and dose patients in a Phase 2 study for the oral formulation of VX-993 for the treatment of painful 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 has completed enrollment in the Phase 1/2/3 study of zimislecel in people with T1D. Vertex has temporarily postponed completion of dosing in the study, pending an internal manufacturing analysis.
- 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 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-Mediated Diseases

Vertex is developing povetacept for multiple diseases. Povetacept is a dual inhibitor of the BAFF and APRIL cytokines, which play key roles in the pathogenesis of multiple B cell-mediated diseases. Povetacept represents a potentially best-in-class approach to control B cell activity in IgAN and pMN and has pipeline-in-a-product potential.

- The U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) and a rolling review to the BLA submission for povetacept for the treatment of IgAN. Vertex has completed enrollment of the interim analysis cohort for potential accelerated approval in the U.S. and has completed the studies to support the launch of povetacept for monthly, at-home self-administration with an autoinjector. Vertex is on track to submit the first module of the IgAN BLA to the FDA before the end of 2025 and plans to complete the full BLA submission in the first half of 2026 for potential accelerated approval in the U.S. Vertex has now also completed full enrollment of the Phase 3 study in IgAN.
- Vertex recently initiated the Phase 2/3 pivotal study of povetacept in patients with pMN. The FDA recently granted Fast Track designation for povetacept in pMN.
- Vertex will have an oral presentation of updated Phase 1/2 data on povetacept in IgAN and pMN at the American Society of Nephrology (ASN) Kidney Week. Vertex will host an in-person investor event at the ASN conference on Saturday,

November 8, 2025, at 7:00 p.m. CT / 8:00 p.m. ET. The investor event will also be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section.

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.

- In September, Vertex completed enrollment in the interim analysis cohort of the AMPLITUDE Phase 2/3 trial of inaxaplin. 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.
- Vertex is on track to complete enrollment in the AMPLIFIED Phase 2 study of inaxaplin by year end 2025. AMPLIFIED is a study of people with AMKD with moderate proteinuria, and people with AMKD and Type 2 diabetes — populations not being studied in the AMPLITUDE trial.

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 recently initiated AGLOW, a Phase 2 proof-of-concept study of VX-407 in patients with a subset of variants in the *PKD1* gene, which encodes the PC1 protein, estimated to be up to approximately 30,000 (or up to approximately 10%) of the overall patient population living with ADPKD. AGLOW is a 24-patient single-arm study that will evaluate the effect of VX-407 on height-adjusted total kidney volume (htTKV).

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 promote effective delivery into cells and address the causal biology 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.

Additional Earlier Stage R&D Programs

Consistent with its overall strategy, Vertex takes a portfolio approach to all of its programs, with additional assets or approaches 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 Na_v1.7 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenues:				
Product revenues, net	\$ 3,076.4	\$ 2,771.9	\$ 8,780.6	\$ 8,108.1
Other revenues	—	—	30.7	—
Total revenues	3,076.4	2,771.9	8,811.3	8,108.1
Costs and expenses:				
Cost of sales	414.8	392.6	1,185.3	1,107.1
Research and development expenses	977.7	875.9	2,935.8	2,631.6
Acquired in-process research and development expenses	54.5	15.0	76.5	4,540.9
Selling, general and administrative expenses	445.1	371.8	1,266.1	1,086.7
Intangible asset impairment charge	—	—	379.0	—
Change in fair value of contingent consideration	(1.9)	0.3	1.2	0.7
Total costs and expenses	1,890.2	1,655.6	5,843.9	9,367.0
Income (loss) from operations	1,186.2	1,116.3	2,967.4	(1,258.9)
Interest income	125.7	132.2	369.0	469.9
Interest expense	(3.3)	(7.5)	(10.0)	(27.8)
Other expense, net	(9.8)	(16.9)	(14.2)	(71.2)
Income (loss) before provision for income taxes	1,298.8	1,224.1	3,312.2	(888.0)
Provision for income taxes	215.9	178.7	550.1	560.6
Net income (loss)	\$ 1,082.9	\$ 1,045.4	\$ 2,762.1	\$ (1,448.6)
Net income (loss) per common share:				
Basic	\$ 4.24	\$ 4.05	\$ 10.77	\$ (5.61)
Diluted	\$ 4.20	\$ 4.01	\$ 10.68	\$ (5.61)
Shares used in per share calculations:				
Basic	255.6	258.0	256.4	258.1
Diluted	257.6	261.0	258.6	258.1

Vertex Pharmaceuticals Incorporated

Total Revenues

(unaudited, in millions)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
TRIKAFTA/KAFTRIO	\$ 2,653.6	\$ 2,585.0	\$ 7,740.2	\$ 7,517.8
ALYFTREK	247.0	—	457.7	—
Other product revenues (1)	175.8	186.9	582.7	590.3
Product revenues, net	3,076.4	2,771.9	8,780.6	8,108.1
Other revenues	—	—	30.7	—
Total revenues	\$ 3,076.4	\$ 2,771.9	\$ 8,811.3	\$ 8,108.1

1: In the three and nine months ended September 30, 2025, "Other product revenues" included \$16.9 million and \$61.5 million from CASGEVY, respectively, and \$19.6 million and \$32.9 million, respectively, from JOURNAVX. In the three and nine months ended September 30, 2024, "Other product revenues" included \$2.0 million from CASGEVY and no revenues from JOURNAVX. 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP cost of sales	\$ 414.8	\$ 392.6	\$ 1,185.3	\$ 1,107.1
Stock-based compensation expense	(2.8)	(1.9)	(7.9)	(5.5)
Intangible asset amortization expense	(5.0)	(5.0)	(15.1)	(15.1)
Non-GAAP cost of sales	\$ 407.0	\$ 385.7	\$ 1,162.3	\$ 1,086.5
GAAP research and development expenses	\$ 977.7	\$ 875.9	\$ 2,935.8	\$ 2,631.6
Stock-based compensation expense	(116.0)	(111.0)	(315.7)	(327.5)
Intangible asset amortization expense	(0.6)	(0.9)	(1.9)	(0.9)
Acquisition-related costs (2)	—	—	—	(172.3)
Non-GAAP research and development expenses	\$ 861.1	\$ 764.0	\$ 2,618.2	\$ 2,130.9
Acquired in-process research and development expenses	\$ 54.5	\$ 15.0	\$ 76.5	\$ 4,540.9
GAAP selling, general and administrative expenses	\$ 445.1	\$ 371.8	\$ 1,266.1	\$ 1,086.7
Stock-based compensation expense	(76.1)	(71.7)	(204.7)	(197.7)
Acquisition-related costs (2)	—	—	—	(36.5)
Non-GAAP selling, general and administrative expenses	\$ 369.0	\$ 300.1	\$ 1,061.4	\$ 852.5
Combined non-GAAP R&D, AIPR&D and SG&A expenses	\$ 1,284.6	\$ 1,079.1	\$ 3,756.1	\$ 7,524.3
GAAP other expense, net	\$ (9.8)	\$ (16.9)	\$ (14.2)	\$ (71.2)
Decrease in fair value of strategic investments	2.6	10.8	12.2	50.5
Non-GAAP other expense, net	\$ (7.2)	\$ (6.1)	\$ (2.0)	\$ (20.7)
GAAP provision for income taxes	\$ 215.9	\$ 178.7	\$ 550.1	\$ 560.6
Tax adjustments (3)	47.8	104.0	240.0	283.8
Non-GAAP provision for income taxes	\$ 263.7	\$ 282.7	\$ 790.1	\$ 844.4
GAAP effective tax rate	16.6%	14.6%	16.6%	(63.1)%
Non-GAAP effective tax rate	17.6%	19.8%	18.6%	(1,038.6)%

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
GAAP operating income (loss)	\$ 1,186.2	\$ 1,116.3	\$ 2,967.4	\$ (1,258.9)
Stock-based compensation expense	194.9	184.6	528.3	530.7
Intangible asset impairment charge	—	—	379.0	—
Intangible asset amortization expense	5.6	5.9	17.0	16.0
(Decrease) increase in fair value of contingent consideration	(1.9)	0.3	1.2	0.7
Acquisition-related costs (2)	—	—	—	208.8
Non-GAAP operating income (loss)	\$ 1,384.8	\$ 1,307.1	\$ 3,892.9	\$ (502.7)
GAAP net income (loss)	\$ 1,082.9	\$ 1,045.4	\$ 2,762.1	\$ (1,448.6)
Stock-based compensation expense	194.9	184.6	528.3	530.7

Intangible asset impairment charge	—	—	379.0	—
Intangible asset amortization expense	5.6	5.9	17.0	16.0
Decrease in fair value of strategic investments	2.6	10.8	12.2	50.5
(Decrease) increase in fair value of contingent consideration	(1.9)	0.3	1.2	0.7
Acquisition-related costs (2)	—	—	—	208.8
Total non-GAAP adjustments to pre-tax income	201.2	201.6	937.7	806.7
Tax adjustments (3)	(47.8)	(104.0)	(240.0)	(283.8)
Non-GAAP net income (loss)	\$ 1,236.3	\$ 1,143.0	\$ 3,459.8	\$ (925.7)

Net income (loss) per diluted common share:

GAAP	\$ 4.20	\$ 4.01	\$ 10.68	\$ (5.61)
Non-GAAP	\$ 4.80	\$ 4.38	\$ 13.38	\$ (3.59)

Shares used in diluted per share calculations:

GAAP and Non-GAAP	257.6	261.0	258.6	258.1
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2: 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.

3: In the three and nine months ended September 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. “Tax adjustments” for the three and nine months ended September 30, 2024 also included a discrete benefit related to prior tax years resulting from a R&D tax credit study that was completed in the third quarter of 2024.

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

	<u>September 30, 2025</u>	<u>December 31, 2024</u>
Assets		
Cash, cash equivalents and marketable securities	\$ 6,287.0	\$ 6,115.9
Accounts receivable, net	1,946.4	1,609.4
Inventories	1,626.8	1,205.4
Prepaid expenses and other current assets	709.4	665.7
Total current assets	<u>10,569.6</u>	<u>9,596.4</u>
Property and equipment, net	1,425.1	1,227.8
Goodwill and other intangible assets, net	1,517.8	1,913.9
Deferred tax assets	2,937.2	2,331.1
Operating lease assets	1,591.8	1,356.8
Long-term marketable securities	5,722.8	5,107.9
Other long-term assets	1,098.0	999.3
Total assets	<u>\$ 24,862.3</u>	<u>\$ 22,533.2</u>
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	\$ 4,033.8	\$ 3,201.6
Other current liabilities	441.5	363.0
Total current liabilities	<u>4,475.3</u>	<u>3,564.6</u>
Long-term operating lease liabilities	1,834.8	1,544.4
Other long-term liabilities	1,233.4	1,014.6
Shareholders' equity	17,318.8	16,409.6
Total liabilities and shareholders' equity	<u>\$ 24,862.3</u>	<u>\$ 22,533.2</u>
Common shares outstanding	254.0	256.9

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-

dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. 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 neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes, 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 16 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 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) the eligible patient population for Vertex's CFTR modulators and expectations for continued growth in CF, including by increasing the number of CF patients taking its medicines through new approvals and reimbursement agreements, treatment of younger patients, increased survival, and expansion into additional geographies, (ii) beliefs regarding the clinical benefits, commercial launch progress, and access in additional countries for ALYFTREK, (iii) beliefs regarding the anticipated benefits, commercial launch progress, eligible patient population, and access to CASGEVY, (iv) expectations regarding the potential benefits and commercial launch progress for JOURNAVX, including expectations with respect to expanding access to JOURNAVX over the remainder of 2025 and into 2026, (v) expectations to expand the labels for TRIKAFTA/KAFTRIO and ALYFTREK and enable earlier treatment of children with CF, including expectations to submit regulatory approval applications for TRIKAFTA/KAFTRIO in children 12 to <24 months of age with global regulators in the first half of 2026 and to share results from the ALYFTREK clinical trial in children 2 to 5 years of age in the first half of 2026, (vi) expectations and plans with respect to the clinical trials evaluating VX-828 and VX-522, (vii) expectations regarding Vertex's SCD and TDT program, including with respect to completing dosing in two Phase 3 studies of CASGEVY in the fourth quarter of 2025 and to share data from these studies in December 2025, (viii) plans to start the second Phase 3 DPN study in November and to complete enrollment in both Phase 3 studies evaluating suzetrigine for the treatment of DPN, (ix) expectations for the Phase 2 study of the oral formulation of VX-993 in DPN, (x) expectations regarding the clinical benefits and goals for zimislecel in T1D, and expectations and plans with respect to additional approaches that could provide transformative benefits to people with T1D and reduce or eliminate the need for standard immunosuppression regimens, (xi) expectations with respect to povetacept, including beliefs about its potential benefits and therapeutic scope, its potential to be a best-in-class approach to control B cell activity in IgAN and pMN, and its potential to be a pipeline-in-a-product, expectations regarding BLA submissions for IgAN, including with respect to submitting the first module to the FDA before the end of 2025 and completing the full submission in the first half of 2026, expectations with respect to the ongoing trials in pMN, and plans to present updated Phase 2 data for IgAN and pMN at an upcoming medical conference, (xii) expectations regarding the AMPLITUDE Phase 2/3 trial of inaxaplin in AMKD, including expectations to conduct the pre-planned interim analysis and apply for potential accelerated U.S. if results are supportive, and expectations to complete enrollment in the AMPLIFIED Phase 2 study of inaxaplin by year-end, (xiii) expectations regarding the ADPKD program and the Phase 2 proof-of-concept study of VX-407, and (xiv) 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. 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 further successfully commercialize ALYFTREK as a treatment for CF, JOURNAVX as a treatment for acute pain, and CASGEVY as a treatment for SCD and TDT, 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 Third 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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