
UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported) May 5, 2025

Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts

(State or other jurisdiction of incorporation)

000-19319

(Commission File Number)

04-3039129

(I.R.S. Employer Identification No.)

50 Northern Avenue

Boston, Massachusetts 02210

(Address of principal executive offices) (Zip Code)

(617) 341-6100

(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, \$0.01 Par Value Per Share	VRTX	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02. Results of Operations and Financial Condition.

On May 5, 2025, we issued a press release in which we reported our consolidated financial results for the three months ended March 31, 2025. A copy of that press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The information set forth in Exhibit 99.1 shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 9.01. Financial Statements and Exhibits.**(d) Exhibits**

<u>Exhibit</u>	<u>Description of Document</u>
99.1	Press Release Dated May 5, 2025.
104	Cover Page Interactive Data File — the cover page XBRL tags are embedded within the Inline XBRL document.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

VERTEX PHARMACEUTICALS INCORPORATED

(Registrant)

Date: May 5, 2025

/s/ Jonathan Biller

Jonathan Biller

Executive Vice President, Chief Legal Officer

Vertex Reports First Quarter 2025 Financial Results

— Total revenue of \$2.77 billion, a 3% increase compared to Q1 2024; raised the low end of total revenue guidance by \$100 million to a new range of \$11.85 to \$12 billion —

— Strong progress with CASGEVY[®], ALYFTREK[™] and JOURNAVX[™] launches —

— Povetacicept (pove) IgAN Phase 3 interim analysis (IA) cohort fully enrolled and zimislecel Phase 3 program to complete dosing this quarter, setting up potential filings in 2026; inaxaplin Phase 3 IA cohort on track to complete enrollment in the second half of 2025 —

— Four programs already in pivotal development, with pivotal study of pove in primary membranous nephropathy (pMN) to start this year —

BOSTON -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the first quarter ended March 31, 2025, and raised the low end of its total revenue guidance range by \$100 million, from \$11.75 billion to \$12 billion to a new range of \$11.85 billion to \$12 billion.

“Vertex delivered a strong start to 2025 with notable execution across the business as we grow and diversify the revenue base, progress multiple launches and advance the R&D pipeline. We continued to expand our leadership in CF and build global momentum for CASGEVY, and we launched JOURNAVX in moderate-to-severe acute pain,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “With multiple programs in pivotal development including povetacicept, which continues to make rapid progress in achieving its potential as a pipeline-in-a-product, and additional programs in early and mid-stage development, Vertex is poised to continue to deliver value for years to come.”

First Quarter 2025 Results

Total revenue increased 3% to \$2.77 billion compared to the first quarter of 2024, primarily driven by the continued performance of TRIKAFTA[®]/KAFTRIO[®] and an early contribution from the U.S. launch of ALYFTREK. In the U.S., total revenue increased 9% to \$1.66 billion due to continued strong patient demand and higher net realized pricing. Outside the U.S., total revenue decreased 5% to \$1.11 billion as strong patient demand in both established and newer markets was offset by the expected revenue decline in Russia, where Vertex is experiencing violation of its intellectual property rights. Vertex has strong intellectual property protection for its medicines around the world and believes this violation in Russia is a limited and isolated matter.

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 \$1.2 billion and \$1.0 billion, respectively, in the first quarter of 2024. The increases were primarily due to 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.

Intangible asset impairment charge of \$379.0 million associated with VX-264 (the “cells plus device” program) in patients with type 1 diabetes, which will not be advancing further in clinical development, was included in GAAP operating income in the first quarter of 2025.

GAAP effective tax rate was 11.5% compared to 14.0% for the first quarter of 2024.

Non-GAAP effective tax rate was 18.8% compared to 17.4% for the first quarter of 2024. The difference between the GAAP and non-GAAP effective tax rates was primarily due to tax benefits related to stock-based compensation.

GAAP net income was \$646 million compared to \$1.1 billion for the first quarter of 2024, as a result of increased operating expenses and the intangible asset impairment charge.

Non-GAAP net income was \$1.1 billion compared to \$1.2 billion in the first quarter of 2024, as a result of increased operating expenses.

Cash, cash equivalents and total marketable securities as of March 31, 2025, were \$11.4 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 program.

Full Year 2025 Financial Guidance

Vertex today raised the low end of its full-year 2025 revenue guidance range from \$11.75 to \$12 billion to \$11.85 billion to \$12 billion, which assumes continued growth in CF, including the launch of ALYFTREK; as well as continued uptake of CASGEVY in multiple regions; and early contributions from the launch of JOURNAVX. Vertex 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. This 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>FY 2025</u>
Total revenue	\$11.85 to \$12.0 billion	\$11.75 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

Cystic Fibrosis (CF) Portfolio

Marketed Products

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 95 percent of all people living with CF in markets with the highest prevalence and are approved for patients as young as one month old. ALYFTREK, the newest marketed CFTR modulator, is approved in the U.S. and the United Kingdom (U.K.) for the treatment of patients 6 years and older. Vertex anticipates the number of CF patients taking its medicines will continue to grow through new approvals and reimbursement, treatment of younger patients, increased survival, and expansion into additional geographies. Recent and anticipated progress includes:

- 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, is now approved in the U.S. and the U.K. Vertex is working with the National Health Service (NHS) in the U.K. to secure coverage for eligible patients.

- In April, the European Medical Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for ALYFTREK for the treatment of CF patients ages 6 years and older. European Commission approval is expected in the second half of 2025. Regulatory reviews are underway for ALYFTREK in Canada, Switzerland, Australia and New Zealand.
- Vertex secured European Commission approval for the label expansion of KAFTRIO in combination with ivacaftor for CF patients ages 2 years and older who have at least one non-class I mutation in the CFTR gene. With this approval, approximately 4,000 people in the European Union are newly eligible for a medicine that treats the underlying cause of their disease. Work to ensure access for all eligible patients is underway.

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

CASGEVY is a non-viral, ex vivo CRISPR/Cas9 gene-edited cell therapy for eligible patients with SCD or TDT that has been shown to reduce or eliminate vaso-occlusive crises (VOCs) for patients with SCD and transfusion requirements for patients with TDT. CASGEVY is approved in the U.S., Great Britain, the EU, the Kingdom of Saudi Arabia (KSA), the Kingdom of Bahrain (Bahrain), Canada, Switzerland and the United Arab Emirates (UAE) for the treatment of both SCD and TDT. Recent highlights include:

- In Europe, Vertex secured a formal reimbursement agreement with NHS England for SCD patients to access CASGEVY, following the agreement reached in August 2024 for eligible TDT patients to access CASGEVY. Vertex recently entered into a similar agreement in Wales for both eligible SCD and TDT patients. Following a positive assessment, Vertex also finalized national reimbursement in Austria. In the Middle East, following regulatory approval in the UAE, Vertex secured reimbursement in the majority of emirates.
- Since launch through May 1st, Vertex has activated more than 65 authorized treatment centers (ATCs) globally and approximately 90 patients have had their first cell collection.
- Vertex recently filed a manufacturing license submission with the FDA and expects to begin manufacturing CASGEVY in Portsmouth, NH, in the second half of 2025. This submission is part of the planned ramp of CASGEVY manufacturing capacity as demand for the therapy increases.

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. Vertex continues to advance a portfolio of

selective NaV1.8 and NaV1.7 pain signal inhibitors for stand-alone use or in combination, with potential to provide effective pain relief without the limitations of opioids and other available medicines.

- On January 30, 2025, the FDA approved JOURNAVX for the treatment of adults with moderate-to-severe acute pain, and it is now available and stocked at pharmacies across the U.S., including major national and regional retail pharmacy chains.
- Since JOURNAVX became available in early March through April 18th, more than 20,000 prescriptions have been written and filled across the hospital and retail settings in different acute pain conditions, consistent with JOURNAVX's broad label.
- As of May 1st, across commercial and government payers, approximately 94 million lives already have covered access to JOURNAVX, and approximately 42 million have unrestricted access (i.e., without the need for prior authorization or step edits). Vertex has reached a formal coverage agreement with a large national Pharmacy Benefit Manager (PBM) to make JOURNAVX available to their customers, representing approximately 22 million commercial lives. A total of ten state Medicaid plans are also providing unrestricted access to JOURNAVX and 20 more are currently evaluating their policies.
- More than 50 large healthcare systems have taken steps to initiate pharmacy and therapeutics (P&T) committee reviews of JOURNAVX, and some have already added it on formularies.
- Vertex recently initiated two Phase 4 studies in various moderate-to-severe acute pain conditions to provide additional data on the effectiveness and safety of JOURNAVX as part of real-world clinical practice, in both inpatient and outpatient settings.
- The Alternatives to Pain Act has been reintroduced in both the House and Senate of the new U.S. Congress, while nearly 35 U.S. states to date have passed or introduced new legislation in support of non-opioid options. Vertex also expects JOURNAVX to be added to the list of treatments eligible for an add-on payment under the NOPAIN Act, which became effective on January 1, 2025.

Select Clinical-Stage R&D Pipeline

Cystic Fibrosis

- Vertex is enrolling and dosing studies in younger age groups — a Phase 3 study of TRIKAFTA/KAFTRIO in 1- to 2-year-olds and a Phase 3 study of ALYFTREK in 2- to 5-year-olds — to expand the labels and enable earlier treatment of children with CF.
- 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. Vertex expects to advance the once-daily, next-generation 3.0 VX-828 combination into a clinical trial in people with CF this year.

- Vertex has implemented a temporary pause to the multiple ascending dose (MAD) portion of the Phase 1/2 study of VX-522, a nebulized CFTR mRNA therapy, in order to assess a tolerability issue.

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.
- Vertex continues to advance preclinical assets for gentler conditioning for CASGEVY, which could broaden the eligible patient population.

Acute Pain

- Vertex expects to complete the Phase 2 study for an oral formulation of VX-993 this quarter. VX-993 is a next-generation selective NaV1.8 pain signal inhibitor, for the treatment of moderate-to-severe acute pain following bunionectomy surgery. Vertex expects to report results from the Phase 2 study in the second half of 2025. VX-993 has Fast Track designation for moderate-to-severe acute pain.
- Vertex has completed the Phase 1 study of an intravenous formulation of VX-993 in healthy volunteers. The FDA has granted the intravenous formulation of VX-993 Fast Track designation for moderate-to-severe 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 FDA has granted suzetrigine Fast Track designation in PNP and Breakthrough Therapy designation in DPN.
- 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.

- *Zimislecel (VX-880), fully differentiated islet cells with standard immunosuppression.*

- Vertex continues to enroll and dose patients in the Phase 3 portion of the Phase 1/2/3 study of zimislecel in patients with T1D with severe hypoglycemic events and impaired awareness of hypoglycemia in the U.S., Canada, U.K., and EU. Vertex expects to complete enrollment and dosing of the pivotal study in the second quarter of 2025 and submit marketing applications to global regulators 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 will present updated clinical data from the 12 participants who received the full dose of zimislecel as a single infusion with over one year of follow-up in the Phase 1/2 portion of the study in an oral presentation at the American Diabetes Association 85th Scientific Sessions. Vertex will host an in-person investor event at the ADA conference on Friday, June 20, 2025 at 7:15 p.m. CT / 8:15 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.
- *Additional approaches:*
 - Vertex previously announced that it has discontinued development of the VX-264 (“cells plus device” program), as the Phase 1/2 study did not meet its efficacy endpoint.
 - Vertex is pursuing research-stage programs to evaluate additional approaches that could provide transformative benefit to people with T1D and reduce or eliminate the need for standard immunosuppressive regimens. These approaches include alternative immunosuppressives, 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 povetacicept, a dual antagonist of the BAFF and APRIL cytokines, which play key roles in the pathogenesis of multiple B cell-mediated diseases. Pove 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 has completed enrollment of the interim analysis cohort. 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. Studies to support the launch of povetacept for home administration are underway.

- Based on positive results of povetacept in primary membranous nephropathy (pMN) in the RUBY-3 study, Vertex has reached agreement with the FDA to advance povetacept into pivotal development for this disease. Vertex will initiate a single, adaptive Phase 2/3 trial of povetacept vs. standard-of-care this year. In the Phase 2 portion, both 80 mg and 240 mg of povetacept will be assessed and the selected dose will advance to Phase 3, where the primary endpoint of complete clinical remission at 72 weeks of treatment will be evaluated.
- Vertex plans to present updated data from the IgAN and pMN cohorts of the RUBY-3 study at upcoming medical congresses.
- Vertex expects to share data and next steps from other RUBY-3 renal diseases and the hematologic conditions in the RUBY-4 study later this year.

APOL1-Mediated Kidney Disease (AMKD)

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

- Vertex expects to complete enrollment in the interim analysis cohort of the Phase 3 portion of the AMPLITUDE trial of inaxaplin in the second half of 2025. 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 continues to enroll and dose patients in the AMPLIFIED Phase 2 study of inaxaplin in people with AMKD and diabetes or other co-morbidities.

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.

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 expects to advance VX-407 into a Phase 2 proof-of-concept study this year in patients with a subset of variants in the PKD1 gene, which encodes the PC1 protein, estimated to be up to ~30,000 (or ~10%) of the overall patient population.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial results and guidance exclude from Vertex's pre-tax income (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 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
(unaudited, in millions, except per share amounts)

	Three Months Ended March 31,	
	2025	2024
Revenues:		
Product revenues, net	\$ 2,760.2	\$ 2,690.6
Other revenues	10.0	—
Total revenues	<u>2,770.2</u>	<u>2,690.6</u>
Costs and expenses:		
Cost of sales	363.0	342.6
Research and development expenses	979.7	789.1
Acquired in-process research and development expenses	19.8	76.8
Selling, general and administrative expenses	396.4	342.7
Intangible asset impairment charge	379.0	—
Change in fair value of contingent consideration	2.2	(0.1)
Total costs and expenses	<u>2,140.1</u>	<u>1,551.1</u>
Income from operations	630.1	1,139.5
Interest income	120.9	181.2
Interest expense	(3.0)	(10.4)
Other expense, net	<u>(17.6)</u>	<u>(31.2)</u>
Income before provision for income taxes	730.4	1,279.1
Provision for income taxes	84.1	179.5
Net income	<u>\$ 646.3</u>	<u>\$ 1,099.6</u>
Net income per common share:		
Basic	\$ 2.52	\$ 4.26
Diluted	\$ 2.49	\$ 4.21
Shares used in per share calculations:		
Basic	256.9	258.2
Diluted	259.5	261.1

Vertex Pharmaceuticals Incorporated
Total Revenues
(unaudited, in millions)

	Three Months Ended March 31,	
	2025	2024
TRIKAFTA/KAFTRIO	\$ 2,535.5	\$ 2,483.6
ALYFTREK	53.9	—
Other product revenues (1)	170.8	207.0
Product revenues, net	2,760.2	2,690.6
Other revenues	10.0	—
Total revenues	\$ 2,770.2	\$ 2,690.6

1: In the first quarter of 2025, "Other product revenues" included \$14.2 million from CASGEVY, and an insignificant amount from JOURNAVX. In the first quarter of 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 March 31,	
	2025	2024
GAAP cost of sales	\$ 363.0	\$ 342.6
Stock-based compensation expense	(2.6)	(1.8)
Intangible asset amortization expense	(5.0)	(5.0)
Non-GAAP cost of sales	<u>\$ 355.4</u>	<u>\$ 335.8</u>
GAAP research and development expenses	\$ 979.7	\$ 789.1
Stock-based compensation expense	(100.1)	(119.4)
Intangible asset amortization expense	(0.6)	—
Non-GAAP research and development expenses	<u>\$ 879.0</u>	<u>\$ 669.7</u>
Acquired in-process research and development expenses	\$ 19.8	\$ 76.8
GAAP selling, general and administrative expenses	\$ 396.4	\$ 342.7
Stock-based compensation expense	(63.4)	(70.7)
Non-GAAP selling, general and administrative expenses	<u>\$ 333.0</u>	<u>\$ 272.0</u>
Combined non-GAAP R&D, AIPR&D and SG&A expenses	<u><u>\$ 1,231.8</u></u>	<u><u>\$ 1,018.5</u></u>
GAAP other expense, net	\$ (17.6)	\$ (31.2)
Decrease in fair value of strategic investments	15.0	27.0
Non-GAAP other expense, net	<u>\$ (2.6)</u>	<u>\$ (4.2)</u>
GAAP provision for income taxes	\$ 84.1	\$ 179.5
Tax adjustments (2)	160.1	81.6
Non-GAAP provision for income taxes	<u>\$ 244.2</u>	<u>\$ 261.1</u>
GAAP effective tax rate	11.5 %	14.0 %
Non-GAAP effective tax rate	18.8 %	17.4 %

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

	Three Months Ended March 31,	
	2025	2024
GAAP operating income	\$ 630.1	\$ 1,139.5
Stock-based compensation expense	166.1	191.9
Intangible asset impairment charge	379.0	—
Intangible asset amortization expense	5.6	5.0
Increase (decrease) in fair value of contingent consideration	2.2	(0.1)
Non-GAAP operating income	<u>\$ 1,183.0</u>	<u>\$ 1,336.3</u>
GAAP net income	\$ 646.3	\$ 1,099.6
Stock-based compensation expense	166.1	191.9
Intangible asset impairment charge	379.0	—
Intangible asset amortization expense	5.6	5.0
Decrease in fair value of strategic investments	15.0	27.0
Increase (decrease) in fair value of contingent consideration	2.2	(0.1)
Total non-GAAP adjustments to pre-tax income	<u>567.9</u>	<u>223.8</u>
Tax adjustments (2)	<u>(160.1)</u>	<u>(81.6)</u>
Non-GAAP net income	<u>\$ 1,054.1</u>	<u>\$ 1,241.8</u>
Net income per diluted common share:		
GAAP	\$ 2.49	\$ 4.21
Non-GAAP	\$ 4.06	\$ 4.76
Shares used in diluted per share calculations:		
GAAP and Non-GAAP	259.5	261.1

2: In the first quarter of 2025 and 2024, “Tax adjustments” included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income and excess tax benefits related to stock-based compensation.

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

	<u>March 31, 2025</u>	<u>December 31, 2024</u>
Assets		
Cash, cash equivalents and marketable securities	\$ 6,201.2	\$ 6,115.9
Accounts receivable, net	1,805.1	1,609.4
Inventories	1,359.7	1,205.4
Prepaid expenses and other current assets	642.8	665.7
Total current assets	<u>10,008.8</u>	<u>9,596.4</u>
Property and equipment, net	1,295.9	1,227.8
Goodwill and other intangible assets, net	1,529.2	1,913.9
Deferred tax assets	2,544.3	2,331.1
Operating lease assets	1,338.5	1,356.8
Long-term marketable securities	5,156.5	5,107.9
Other long-term assets	1,007.3	999.3
Total assets	<u>\$ 22,880.5</u>	<u>\$ 22,533.2</u>
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	\$ 3,396.8	\$ 3,201.6
Other current liabilities	386.4	363.0
Total current liabilities	<u>3,783.2</u>	<u>3,564.6</u>
Long-term operating lease liabilities	1,537.7	1,544.4
Long-term finance lease liabilities	111.4	112.8
Other long-term liabilities	951.9	901.8
Shareholders' equity	16,496.3	16,409.6
Total liabilities and shareholders' equity	<u>\$ 22,880.5</u>	<u>\$ 22,533.2</u>
Common shares outstanding	257.0	256.9

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. 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 securing coverage for ALYFTREK in the U.K., (iii) expectations regarding approval of ALYFTREK in the E.U. in the second half of 2025, (iv) beliefs regarding the anticipated benefits and commercial launch progress of CASGEVY, and with respect to increasing and initiating manufacturing activities for CASGEVY in the second half of 2025, (v) 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 commercial coverage of JOURNAVX will continue to expand throughout 2025, and expectations that JOURNAVX will be included on the list of treatments that qualify for add-on payment under the NOPAIN Act, (vi) expectations to expand the labels for TRIKAFTA/KAFTRIO and ALYFTREK and enable earlier treatment of children with CF, (vii) expectations to advance the VX-828 combination into people with CF this year, (viii) expectations for the VX-522 clinical trial, (ix) expectations regarding Vertex's SCD and TDT program, including with respect to gentler conditioning for CASGEVY broadening the eligible patient population, (x) plans with respect to the studies of the intravenous and oral formulation of VX-993 for the treatment of acute pain, including expectations to complete the Phase 2 study of the oral formulation of VX-993 in acute pain and expectations to report results from that study in the second half of 2025, (xi) expectations regarding the pivotal study evaluating zimislecel in T1D, including the

expectations to complete enrollment and dosing during the fiscal quarter and submit marketing applications to global regulators in 2026, expectations to present updated data in upcoming medical conferences, and expectations and plans to pursue additional approaches to standard immunosuppression regimens, (xii) expectations with respect to povetacicept, including beliefs about its potential benefits and therapeutic scope, study designs, expectations regarding the Phase 3 RAINIER study, including plans to file for potential accelerated approval in the U.S. in the first half of 2026, beliefs with respect to the RUBY-3 basket study and plans to present updated data from this study at upcoming medical congresses, expectations and plans with respect to advancing povetacicept into pivotal development in pMN, beliefs with respect to the RUBY-4 basket study and expectations to share data and next steps later this year, (xiii) expectations regarding the AMPLITUDE trial in AMKD, including expectations to complete enrollment in the interim analysis cohort in the second half of 2025 and, assuming a positive interim analysis, apply for potential accelerated approval in the U.S., (xiv) expectations for the potential benefits and clinical status of VX-670 for the treatment in people with DM1, and (xv) 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 2025. 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 First 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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