Vertex Pharmaceuticals, Inc. (Nasdaq: VRTX)

Rating: Buy

Price Target: $500.00
Share Price: $450.03

Karen Sterling, PhD, CFA
ksterling@kingswoodus.com

Company Data	
Average Daily Volume (M)	1.52
52-Week Range	377.85-519.88
Shares Outstanding (M)	257.08
Market Cap (B)	115.69
Enterprise Value (B)	105.99
Total Cash (B), mrq	6.20
Total Debt (B)	1.65
Total Debt to Cap	9.09%

GAAP Estimates			
FYE: Dec		2024EA	2025E
EPS	Q1	4.21	2.49 A
	Q2	(13.92)	3.82
	Q3	4.01	4.18
	Q4	3.50	4.55
	FY	(2.08)	15.03
P/E		NM	29.9x
Rev	Q1	2,690.6	2,770.2
	Q2	2,645.6	2,885.0
	Q3	2,771.9	3,050.0
	Q4	2,912.0	3,230.0
	FY	11,020.1	11,935.2
EV/Sales		9.6x	8.9x

One-Year Performance Chart

As of May 6, 2025. Source: E*Trade.

May 7, 2025

Vertex Pharmaceuticals Reports First Quarter 2025 Earnings

Vertex Misses Street Expectations but Delivers Solid Start to 2025; Expectations for Continued Growth and Diversification of Product Base; Reiterate BUY Rating

Summary

- On May 5, 2025, Vertex reported consolidated financial results for the first quarter ended March 31, 2025, and adjusted its FY 2025 financial guidance. Total revenue of $2,770.2 million increased 3% year-over-year, missing Kingswood Capital's estimate of $2,850.0 million by 2.8%. GAAP EPS of $2.49 for the quarter fell 32.2% short of our forecast of $3.67 due to a one-time intangible asset impairment charge of $379.0 million.

- Vertex adjusted its FY 2025 revenue guidance from $11.75-12.00 billion to $11.85-$12.00 billion, reflecting expectations for continued growth in cystic fibrosis, including the launch of ALYFTREK; continued uptake of CASGEVY for sickle cell disease and transfusion-dependent beta thalassemia in multiple regions; as well as early contributions from the commercial launch of JOURNAVX for acute pain. The company maintained its prior guidance for combined GAAP R&D and SG&A expenses of $5.55-$5.70 billion (including approximately $100 million of acquired in-process R&D [AIPR&D] expenses), as well as a non-GAAP effective tax rate of 20.5%-21.5%.

- Vertex reported progress with its ongoing CASGEVY®, ALYFTREK™, and JOURNAVX™ launches in sickle cell disease/beta-thalassemia, cystic fibrosis, and acute pain indications, respectively.

- Vertex also continues efforts to diversify its product base by advancing multiple development programs into late-stage clinical trials and toward commercialization. Phase 3 trials are ongoing in chronic pain, Type 1 diabetes, IgA nephropathy (IgAN), and APOL1-mediated kidney disease, which, if successful, could set up potential filings for FDA regulatory approval in 2026.

- Sustained execution has helped Vertex achieve both a strong operating margin and cash position, allowing continued, significant investments in its pipeline and commercial capabilities. In our view, yesterday's selloff of VRTX shares will prove to be a temporary blip on the stock chart and presents a buying opportunity, while Vertex's long-term prospects remain strong. We reiterate our BUY rating and maintain our $500 price target on the stock. We expect that ongoing diversification of the company's revenue base, disease areas of focus, R&D pipeline, and operating geographies will build long-term value for shareholders.

Q1 2025 Financial Results

- Total revenue in Q1 2025 increased 3% year-over-year to $2,770.2 million, primarily driven by the strong performance of TRIKAFTA/KAFTRIO and an early contribution from the U.S. launch of ALYFTREK. Total revenue compared to the first quarter of 2024 increased 9% in the U.S. (to $1.66 billion) due to strong patient demand and higher net realized pricing. Outside the U.S., total revenue decreased 5% (to $1.11 billion), as strong patient demand was offset by revenue decline in Russia, where Vertex is experiencing a violation of its intellectual property rights. Management believes this violation to be a limited and isolated matter.

- Combined GAAP R&D and SG&A expenses increased by 15.5% to $1,395.9 million, compared to $1,208.6 million in the first quarter of 2024, due to costs associated with global product launches and continued R&D investments in support of clinical development programs that have advanced to Phase 3.

- An intangible asset impairment charge of $379.0 million was associated with the discontinuation of VX-264 development, the "cells plus device" program in patients with type 1 diabetes.

- Acquired in-process R&D (AIPR&D) expenses were $19.8 million, compared to $76.8 million in the first quarter of 2024.

- Q1 2025 GAAP net income was $646.3 million, compared to $1.1 billion in the first quarter of 2024, because of increased operating expenses and the intangible asset impairment charge.

- Q1 2025 GAAP EPS was $2.49 on 259.5 million diluted shares outstanding, compared to $4.21 on 261.1 million diluted shares outstanding in the first quarter of 2024.

- Vertex ended the quarter with $6,201.2 million in cash, cash equivalents, and marketable securities, compared to $6,115.9 million as of December 31, 2024. The increase was primarily

due to cash flows from operating activities, partially offset by repurchases of VRTX common stock under the company's share repurchase program.

Commercial and Clinical Highlights

Cystic Fibrosis (CF)

- Vertex anticipates continued growth in cystic fibrosis through new approvals and reimbursement, treatment of younger patients, patients living longer, and expansion into additional geographies.

- Vertex's ALYFTREK triple combination therapy for CF in patients six years and older is approved in the U.S. and the UK. ALYFTREK is under regulatory review in the EU, Canada, Switzerland, Australia, and New Zealand. European Commission (EC) approval is expected in the second half of 2025, following the issuance of a positive opinion by the European Medical Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) in April 2025.

- In April 2025, Vertex secured EC approval for the label expansion of KAFTRIO in combination with ivacaftor for CF patients 2 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene, expanding the potential patient pool in the EU by approximately 4,000 people.

- Vertex continues to advance next-generation, oral, small molecule combination therapies through preclinical and clinical development. VX-828, a once-daily, next-generation 3.0 combination therapy is expected to enter its first clinical trial during 2025. Vertex temporarily paused its Phase 1/2 clinical trial of VX-522, a nebulized CFTR mRNA therapy, to assess a tolerability issue.

Sickle Cell Disease (SCD) and Transfusion-Dependent Beta-Thalassemia (TDT)

- Vertex received regulatory approvals for CASGEVY, its non-viral, ex vivo CRISPR/Cas9 gene-edited cell therapy for the treatment of patients 12 years and older with SCD or TDT in the U.S., Great Britain, the EU, the Kingdom of Saudi Arabia, the Kingdom of Bahrain, Canada, Switzerland, and the United Arab Emirates.

- Through May 1st, Vertex has expanded the number of authorized treatment centers to more than 65 globally, and approximately 90 patients across all regions have had their first cell collection. CASGEVY's contribution to Q1 2025 product revenue was $14.2 million.

- Vertex filed a manufacturing license submission with the FDA and expects to begin manufacturing CASGEVY in its Portsmouth, NH, manufacturing facility during 2H 2025.

- Vertex completed enrollment of children aged 5 to 11 with SCD or TDT in two Phase 3 studies of CASGEVY. The company also continues to advance preclinical assets for gentler conditioning for CASGEVY, which may broaden the eligible patient population.

Moderate-to-Severe Acute Pain and Peripheral Neuropathic Pain

- On January 30, 2025, Vertex's JOURNAVX (suzetrigine), a first-in-class, selective, non-opioid NaV1.8 pain signal inhibitor, received FDA approval for the treatment of adults with moderate-to-severe acute pain.

- JOURNAVX is now available at pharmacies across the U.S., including major national and regional retail pharmacy chains.

- Since JOURNAVX became available in early March 2025, more than 20,000 prescriptions have been written and filled in different acute pain conditions.

- As of May 1st, approximately 94 million lives have covered access to JOURNAVX across commercial and government payers, though only 42 million (<45%) have unrestricted access (i.e., without the need for prior authorization or step edits). A total of ten state Medicaid plans are providing unrestricted access to JOURNAVX and an additional 20 state Medicaid plans are currently evaluating their policies.

- Vertex recently initiated two Phase 4 studies of JOURNAVX in various moderate-to-severe acute pain conditions to provide additional data on the effectiveness and safety 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 have passed or introduced new legislation in support of non-opioid pain treatment options. Vertex 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.

- Vertex expects to complete a Phase 2 study for an oral formulation of VX-993, its next-generation selective NaV1.8 pain signal inhibitor for the treatment of moderate-to-severe pain following bunionectomy surgery, during Q2 2025.

- The FDA granted Fast Track designation for moderate-to-severe pain for both the oral and IV formulations of VX-993.

- Vertex continues to enroll and dose patients with diabetic peripheral neuropathy (DPN), a form of chronic pain, in two studies: a Phase 3 pivotal trial of suzetrigine and a Phase 2 study for the oral formulation of VX-993.

Other Programs

- In addition to its chronic pain studies, Vertex is advancing Phase 3 development programs in Type 1 diabetes (T1D), IgA nephropathy (IgAN), and APOL1-mediated kidney disease (AMKD).

- Zimislecel (VX-880) advanced to the pivotal Phase 3 portion of a Phase 1/2/3 study in T1D. The therapy is being developed as a potential one-time functional cure for type 1 diabetes and entails delivering stem-cell-derived, fully differentiated islet cells in combination with standard immunosuppression. Vertex continues to enroll and dose patients at study sites in the U.S., Canada, UK, and EU, expecting to complete enrollment during Q2 2025 and submit marketing applications to global regulators in 2026, after patients have completed one year of insulin-free follow-up. The primary endpoint is the proportion of patients with insulin independence and absence of severe hypoglycemic episodes.

- Povetacicept advanced to Phase 3 testing in immunoglobulin A nephropathy (IgAN), a disease of the kidney and the immune system in which the glomeruli of the kidney are inflamed. A global Phase 3 study of povetacicept in patients with IgAN, RAINIER, has completed enrollment of the interim analysis cohort. A pre-planned 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. during 1H 2026 if results are supportive. Based on positive results of povetacicept in primary membranous nephropathy (pMN) in the RUBY-3 study, Vertex announced plans to advance povetacicept into pivotal development for this disease during 2025. Povetacicept represents a possible best-in-class approach to treating IgAN and pMN and has pipeline-in-a-product potential.

- Vertex expects to complete enrollment of the interim analysis cohort of patients with primary AMKD in the Phase 3 portion of the AMPLITUDE global Phase 2/3 pivotal trial of inaxaplin, a small molecule inhibitor of APOL1 function, by year-end 2025. A pre-planned interim analysis will be conducted once this cohort has been treated for 48 weeks. If positive, the interim analysis may serve as the basis for accelerated approval in the U.S. Vertex also continues to enroll and dose patients in the AMPLIFIED Phase 2 study of inaxaplin in people with AMKD and diabetes or other co-morbidities.

Company Description

Founded in 1989, and headquartered in Boston, MA, and London, U.K., Vertex Pharmaceuticals, Inc., is a global biotechnology company focused on discovering, developing, manufacturing, and commercializing small molecule treatments for chronic and genetic diseases, for which there is acute medical need.

The firm's initial focus, and subject of ongoing research and development, is cystic fibrosis (CF), a genetic disease affecting the lungs and other organs. The Vertex portfolio of marketed CF medicines includes KALYDECO, ORKAMBI, SYMDEKO, TRIKAFTA, and ALYFTREK which improve lung function and reduce hospitalizations in CF patients.

The company has also received regulatory approval for CASGEVY™, the first CRISPR/Cas9 gene editing therapy that treats the underlying causes of two chronic, life-shortening diseases: sickle cell disease and transfusion-dependent beta thalassemia. In addition, JOURNAVX™, Vertex's first-in-class, non-addictive, non-opioid oral pain drug, received FDA approval in January 2025 for use in adults with moderate to severe acute pain.

Vertex has a robust clinical pipeline of investigational therapies for other debilitating diseases and conditions, including non-opioid treatment of acute and chronic pain, Type 1 diabetes, alpha-1 antitrypsin deficiency, kidney disease, Duchenne muscular dystrophy, and myotonic dystrophy type 1.

The company has research and development sites and commercial offices in the United States, Canada, Latin America, the Middle East, Japan, Australia, and several European countries.

Valuation

As an established company in the cystic fibrosis space, Vertex leads its competitors across a spectrum of solvency, valuation, and profitability metrics. Vertex's rich valuation is, in our opinion, justified by its formidable cash resources, deep and broadening pipeline, historical successes in clinical trials, and the marketing of its drugs in key strategic regions.

We believe that additional shareholder value will be unlocked as Vertex's portfolio of approved products broadens. One example is the company's global launch of and reimbursement coverage for CASGEVY™, the company's gene therapy for SCD and beta thalassemia.

Vertex's non-opioid treatment for moderate to severe acute pain, JOURNAVX™ (suzetrigine), approved in January 2025, represents the first new class of pain medicine to receive U.S. FDA clearance in over 20 years, with blockbuster peak-sales potential.

As a result of Vertex's ongoing global commercial launches of newly approved therapies, we expect accelerating revenue and EPS growth in H2 2025 and beyond. We recommend investors continue accumulating shares of Vertex over the next 12 months. Our $500.00 price target is based on 2025 projected earnings of $15.03 and a P/E multiple of 33.3x.

Vertex Pharmaceuticals Financial Forecast											
	FY 23 A	MAR 24 A	JUN 24 A	SEP 24 A	DEC 24 A	FY 24 A	MAR 25 A	JUN 25 E	SEP 25 E	DEC 25 E	FY 25 E
Revenue	9,869.2	2,690.6	2,645.6	2,771.9	2,912.0	11,020.1	2,770.2	2,885.0	3,050.0	3,230.0	11,935.2
Cost of Sales	1,262.2	342.6	371.9	392.6	423.4	1,530.5	363.0	418.3	442.3	468.4	1,691.9
Gross Profit	**8,607.0**	**2,348.0**	**2,273.7**	**2,379.3**	**2,488.6**	**9,489.6**	**2,407.2**	**2,466.7**	**2,607.8**	**2,761.7**	10,243.3
Operating Expenses:											
R&D	3,162.9	789.1	966.6	875.9	998.7	3,630.3	979.7	995.3	1,037.0	1,065.9	4,077.9
Acquired in-process R&D	527.1	76.8	4,449.1	15.0	87.5	4,628.4	19.8	25.0	25.0	25.0	94.8
SG&A	1,136.6	342.7	372.2	371.8	377.6	1,464.3	396.4	389.5	381.3	395.7	1,562.8
Intangible Asset Impairment Charge		0.0					379.0				379.0
Change in Fair Value of Contingent Consideration	-51.6	-0.1	0.5	0.3	-1.2	-0.5	2.2	0.0	0.0	0.0	2.2
Total Operating Expenses	**4,775.0**	**1,208.5**	**5,788.4**	**1,263.0**	**1,462.6**	**9,722.5**	**1,777.1**	**1,409.8**	**1,443.3**	**1,486.6**	**6,116.7**
Operating Income	**3,832.0**	**1,139.5**	**-3,514.7**	**1,116.3**	**1,026.0**	**-232.9**	**630.1**	**1,056.9**	**1,164.5**	**1,275.1**	**4,126.6**
Interest Expense/Income, net	570.6	170.8	146.6	124.7	125.4	567.5	117.9	128.0	131.0	134.0	510.9
Other Income/Expense, net	-22.8	-31.2	-23.1	-16.9	-14.9	-86.1	-17.6	-	-	-	-17.6
Earnings Before Tax	**4,379.8**	**1,279.1**	**-3,391.2**	**1,224.1**	**1,136.5**	**248.5**	**730.4**	**1,184.9**	**1,295.5**	**1,409.1**	**4,619.9**
Income Tax Expense	760.2	179.5	202.4	178.7	223.5	784.1	84.1	189.6	207.3	225.5	706.4
Net Income	**3,619.6**	**1,099.6**	**-3,593.6**	**1,045.4**	**913.0**	**-535.6**	**646.3**	**995.3**	**1,088.2**	**1,183.6**	**3,913.4**
Weighted Average Basic Shares Outstanding	257.7	258.2	258.1	258.0	257.5	257.9	256.9	257.2	257.0	256.8	257.1
Weighted Average Diluted Shares Outstanding	260.5	261.1	258.1	261.0	260.5	257.9	259.5	260.4	260.2	260.0	260.3
Net Income per Share, GAAP, Basic	$14.05	$4.26	-$13.92	$4.05	$3.55	-$2.08	$2.52	$3.87	$4.23	$4.61	$15.22
Net Income per Share, GAAP, Diluted	$13.89	$4.21	-$13.92	$4.01	$3.50	-$2.08	$2.49	$3.82	$4.18	$4.55	$15.03
All figures in millions of U.S. Dollar except per share items.											

Sources: Capital IQ, Kingswood Capital Partners Estimates.

Risks to Our Price Target

Clinical trial setbacks: The success of Vertex's drug development pipeline depends on the outcomes of clinical trials, which are subject to risks and uncertainties, including unexpected safety issues, serious side effects, or lack of efficacy.

Regulatory: Vertex may face delays caused by, or rejections from regulatory agencies of its applications for regulatory clearance and marketing authorizations, which in turn would delay or prevent commercialization.

Pricing and reimbursement: Payers such as medical insurers or government-underwritten health systems may limit patient access, or decline to reimburse Vertex's therapies if Vertex's prices are deemed too high, or the efficacy of its therapies are deemed insufficient.

Dependence on a few key products: Vertex's revenue is heavily dependent on its cystic fibrosis products, and any setbacks or competition in this area could have a significant impact on the company's financial performance.

Intellectual property challenges: Vertex's patents may be challenged by competitors, declared invalid by the Patent Trial and Appeal Board, or in court, which could lead to loss of intellectual property or formulary exclusivity, and increased competition.

Competition: Vertex operates in a highly competitive industry, and its competitors may develop equivalent or superior therapies, thereby eroding its market share and revenue.

Supply chain disruptions: Vertex relies on a complex global supply chain to manufacture its therapies, and disruptions or quality issues could impact production, sales, as well as clinical trial timelines.

DISCLOSURES

Analyst Certification

The Research Analyst(s) denoted by an "AC" on the cover of this report certifies (or, where multiple Research Analysts are primarily responsible for this report, the Research Analyst denoted by an "AC" on the cover or within the document individually certifies, with respect to each security or issuer that the Research Analyst covers in this research) that: (1) all of the views expressed in this report accurately reflect the Research Analyst's personal views about any and all of the subject securities or issuers; and (2) no part of any of the Research Analyst's compensation was, is, or will be directly or indirectly related to the specific recommendations or views expressed by the Research Analyst(s) in this report.

I, Karen Sterling, certify that (1) the views expressed in this report accurately reflect my own views about any and all of the subject companies and securities; and (2) no part of my compensation was, is, or will be directly or indirectly related to the specific recommendations or views expressed by me in this report.

Explanation of Research Ratings (As of January 1, 2025), Designations and Analyst(s) Coverage Universe:

Kingswood Capital Partners, LLC uses the following rating system:

Buy - Buy-rated stocks are expected to have a total return of at least 15% over the following 12 months and are the most attractive stocks in the sector coverage area.

Hold - We believe this stock will perform in line with the average return of others in its industry over the following 12 months.

Sell - Sell-rated stocks are expected to have a negative total return of at least 15% over the following 12 months and are the least attractive stocks in the sector coverage area.

Not Rated (NR) – Kingswood Capital Partners, LLC DOES NOT cover this stock and therefore DOES NOT have forecasts, projections, target price, and recommendation on the shares of this company.

Company-Specific Disclosures

Distribution of Ratings Kingswood Capital Partners, LLC				
			Investment Banking Services/Past 12 Months	
Rating	**Count**	**Percent**	**Count**	**Percent**
BUY	6	75.00	2	33.33
HOLD	1	12.50	0	0.00
SELL	0	0.00	0	0.00
NOT RATED	1	12.50	1	100.00

As of April 2025.

Vertex Pharmaceuticals Rating History as of May 6, 2025

Source: E-Trade.

Other Disclosures

This report has been prepared by Kingswood Capital Partners, LLC. It does not constitute an offer or solicitation of any transaction in any securities referred to herein. Any recommendation contained in this report may not be suitable for all investors. Although the information contained herein has been obtained from recognized services, issuer reports or communications, or other services and sources believed to be reliable, its accuracy or completeness cannot be guaranteed. This report may contain links

to third-party websites, and Kingswood Capital Partners, LLC is not responsible for their content or any linked content contained therein. Such content is not part of this report and is not incorporated by reference into this report. The inclusion of a link in this report does not imply any endorsement by or affiliation with Kingswood Capital Partners, LLC; access to these links is at your own risk. Any opinions, estimates or projections expressed herein may assume some economic, industry and political considerations and constitute current opinions, at the time of issuance, that are subject to change. Any quoted price is as of the last trading session unless otherwise noted. Foreign currency rates of exchange may adversely affect the value, price or income of any security or financial instrument mentioned in this report. Investors in such securities and instruments, including ADRs, effectively assume currency risk. This information is being furnished to you for informational purposes only, and on the condition that it will not form a primary basis for any investment decision. Investors must make their own determination of the appropriateness of an investment in any securities referred to herein based on the applicable legal, tax and accounting considerations and their own investment strategies. By virtue of this publication, neither the Firm nor any of its employees shall be responsible for any investment decision.