Vertex Pharmaceuticals, Inc. (Nasdaq: VRTX)

Rating: Buy

Price Target: $500.00
Share Price: $375.63

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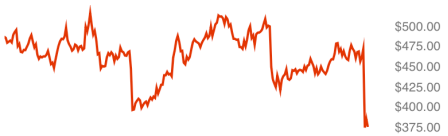
Company Data	
Average Daily Volume (M)	1.72
52-Week Range	372.35-519.88
Shares Outstanding (M)	256.40
Market Cap (B)	96.31
Enterprise Value (B)	85.81
Total Cash (B), mrq	6.38
Total Debt (B)	1.53
Total Debt to Cap	8.17%

GAAP Estimates			
FYE: Dec		2024A	2025E
EPS	Q1	4.21	2.49 A
	Q2	(13.92)	3.99 A
	Q3	4.01	4.21
	Q4	3.50	4.58
	FY	(2.08)	15.27
P/E		NM	24.6x
Rev	Q1	2,690.6	2,770.2
	Q2	2,645.6	2,964.7
	Q3	2,771.9	3,050.0
	Q4	2,912.0	3,230.0
	FY	11,020.1	12,014.9
EV/Sales		7.7x	7.1x

One-Year Performance Chart



As of August 7, 2025. Source: E*Trade.

August 8, 2025

Vertex Pharmaceuticals Reports Strong Second Quarter 2025 Earnings but Stock Slides After VX-993 Clinical Failure

Vertex Stock Falls on Discontinuation of VX-993 Program in Acute Pain; Diversification of Product Base Remains on Track for Potential New Approvals in 2026; Reiterate BUY Rating

Summary

- On August 4, 2025, Vertex reported consolidated financial results for the second quarter ended June 30, 2025, and reiterated full year 2025 guidance. Total revenue of $2,964.7 million increased 12.1% year-over-year, exceeding Kingswood Capital's estimate of $2,885.0 million by 2.8%. GAAP EPS of $3.99 for the quarter exceeded our forecast of $3.82 by 4.5%.

- Vertex left its FY 2025 guidance unchanged, predicting revenues in the $11.85-$12.00 billion range, 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%.

- Revenue guidance assumes continued growth in cystic fibrosis (CF), including the global launch of ALYFTREK™, continued uptake of CASGEVY® in multiple regions, and early contributions from the U.S. launch of JOURNAVX™. Combined GAAP R&D and SG&A expense guidance includes expectations for continued investment in multiple mid- and late-stage clinical development programs and commercial capabilities.

- 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, this week'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.

Q2 2025 Financial Results

- Total revenue in Q2 2025 increased 12.1% year-over-year to $2,964.7 million, primarily driven by the continued performance of cystic fibrosis therapies and early contributions from the three ongoing launches of ALYFTREK, CASGEVY, and JOURNAVX. In the U.S., total revenue compared to the second quarter of 2024 increased 14% (to $1.85 billion) due to continued strong patient demand. Outside the U.S., total revenue increased 8% (to $1.12 billion) due to strong performance across multiple geographies.

- Combined GAAP R&D and SG&A expenses were $1,403.0 million, compared to $1,338.8 million in the second quarter of 2024. The increase was 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.

- Acquired in-process R&D (AIPR&D) expenses were $2.2 million, compared to $4,449.1 million in the second quarter of 2024, when Vertex incurred a $4.4 billion expense in association with the acquisition of Alpine Immune Sciences.

- Q2 2025 GAAP net income was $1,032.9 million, compared to a net loss of $(3,593.6) million in the second quarter of 2024 resulting from the impact of the Alpine AIPR&D expense.

- Q2 2025 GAAP EPS was $3.99 on 258.9 million diluted shares outstanding, compared to $(13.92) on 258.1 million diluted shares outstanding in the second quarter of 2024.

- Vertex ended the quarter with $6,382.8 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 and income tax payments.

Commercial and Clinical Highlights

Cystic Fibrosis (CF)

- Vertex anticipates continued growth in cystic fibrosis through new approvals and reimbursement agreements, 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., the UK, Europe, and Canada. Vertex is working with reimbursement bodies across EU member states and Canada to secure access for all eligible patients as quickly as possible. ALYFTREK remains under regulatory review in Switzerland, Australia, and New Zealand.

- Label expansion efforts are underway for TRIKAFTA/KAFTRIO and ALYFTREK to include children 2 to 5 years of age. Phase 3 studies are ongoing to progress toward this goal.

- 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 currently being studied in healthy volunteers and is expected to move into a CF cohort later this year. The FDA's Independent Data Monitoring Committee endorsed restarting Vertex's Phase 1/2 clinical trial of VX-522, a nebulized CFTR mRNA therapy, which had been paused to assess a tolerability issue. Vertex anticipates resuming the multiple ascending dose (MAD) portion of the study in the near term.

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

- Vertex now has 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., Canada, Great Britain, the EU, Switzerland, the Kingdom of Saudi Arabia, the Kingdom of Bahrain, Qatar, and the United Arab Emirates. 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.

- Through June 30th, Vertex has expanded the number of authorized treatment centers to more than 75 globally, and approximately 115 patients across all regions have had their first cell collection, including 35 during Q2 2025. 29 patients have received infusions of CASGEVY, including 16 in Q2 2025. CASGEVY's contribution to Q2 2025 product revenue was $30.4 million, more than doubling from the prior quarter.

- Vertex secured access for eligible SCD and TDT patients through reimbursement agreements in ten countries, including Northern Ireland, Scotland, and Denmark. Additional agreements are being negotiated with reimbursement authorities globally.

- Vertex has completed enrollment of children 5 to 11 years of age with SCD or TDT in two global Phase 3 studies of CASGEVY and is on track to complete dosing in H2 2025.

Moderate-to-Severe Acute Pain and Peripheral Neuropathic Pain (PNP)

- Since JOURNAVX (suzetrigine), Vertex's first-in-class, selective, non-opioid NaV1.8 pain signal inhibitor, became available in early March 2025, more than 110,000 prescriptions have been written and filled across the hospital and retail settings in different acute pain conditions.

- As of mid-July, nearly 150 million individuals have covered access to JOURNAVX across commercial and government payers, compared to 94 million on May 1st. This includes coverage agreements with two of the three large national pharmacy benefit managers (PBMs) and unrestricted access within 16 state Medicaid plans, up from ten as of May 1st. Vertex expects access to JOURNAVX to broaden further throughout 2025.

- More than 50 of Vertex's targeted 150 large healthcare systems and more than 500 of Vertex's targeted 2,000 hospitals have added JOURNAVX to formularies, protocols, or order sets. Vertex has national group purchasing agreements in place with two of the largest group purchasing organizations in the U.S.

- Vertex completed its 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 acute pain following bunionectomy surgery. Despite being safe and well tolerated, VX-993 did not demonstrate statistically significant superiority compared to placebo, leading to the Company's decision to discontinue development of VX-993 as monotherapy in acute pain.

- Vertex continues to enroll and dose patients with diabetic peripheral neuropathy (DPN), a form of chronic pain, in a Phase 3 pivotal trial of suzetrigine. The FDA granted suzetrigine Fast Track designation in PNP and Breakthrough Therapy designation in DPN. Vertex intends to prioritize DPN as the first PNP indication and begin a second DPN Phase 3 study in the near term. The Company expects to complete enrollment in both Phase 3 studies by year-end 2026.

- Vertex continues to enroll and dose patients in a Phase 2 study for the oral formulation of VX-993 for the treatment of DPN.

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 patients with severe hypoglycemic events, with enrollment expected to be complete in the near term. 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 expects to 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.

- Zimislecel has been granted Regenerative Medicine Advanced Therapy (RMAT) and Fast Track designations from the U.S. FDA, Priority Medicines (PRIME) designation from the European Medicines Agency (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).

- In the Phase 1/2 portion of the Zimislecel T1D trial, 10 of 12 patients no longer required exogenous insulin at month 12, and all patients achieved the primary endpoint of elimination of severe hypoglycemic events from day 90 onwards, with HbA1c <7%.

- Povetacicept, a dual antagonist of the BAFF and APRIL cytokines, which play key roles in the pathogenesis of multiple B cell-driven diseases, 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. The global Phase 3 study of povetacicept in patients with IgAN, RAINIER, completed enrollment of the interim analysis cohort in the second quarter. 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. Vertex expects to complete enrollment in the full study this year. Studies to support the launch of povetacicept for at-home self-administration are also underway.

- Based on positive results of povetacicept in primary membranous nephropathy (pMN) in the Phase 2 RUBY-3 study, Vertex reached agreement with the FDA 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. Follow-on development priorities for povetacicept will be generalized myasthenia gravis (gMG) and warm autoimmune hemolytic anemia (wAIHA), with 175,000 and 35,000 potential patients, respectively, in the U.S. and Europe.

- Vertex is on track 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, which is also on track to complete enrollment by year-end.

- The U.S. Center for Medicare and Medicaid Services (CMS) recently updates their list of diagnostic codes to include new codes for AMKD. The new codes are expected to enable broader recognition and diagnosis of AMKD.

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 $15.27 and a P/E multiple of 32.7x.

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 A	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	2,964.7	3,050.0	3,230.0	12,014.9
Cost of Sales	1,262.2	342.6	371.9	392.6	423.4	1,530.5	363.0	407.5	442.3	468.4	1,681.1
Gross Profit	**8,607.0**	**2,348.0**	**2,273.7**	**2,379.3**	**2,488.6**	**9,489.6**	**2,407.2**	**2,557.2**	**2,607.8**	**2,761.7**	10,333.8
Operating Expenses:											
R&D	3,162.9	789.1	966.6	875.9	998.7	3,630.3	979.7	978.4	1,037.0	1,065.9	4,061.0
Acquired in-process R&D	527.1	76.8	4,449.1	15.0	87.5	4,628.4	19.8	2.2	25.0	25.0	72.0
SG&A	1,136.6	342.7	372.2	371.8	377.6	1,464.3	396.4	424.6	381.3	395.7	1,597.9
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	0.9	0.0	0.0	3.1
Total Operating Expenses	**4,775.0**	**1,208.5**	**5,788.4**	**1,263.0**	**1,462.6**	**9,722.5**	**1,777.1**	**1,406.1**	**1,443.3**	**1,486.6**	**6,113.0**
Operating Income	**3,832.0**	**1,139.5**	**-3,514.7**	**1,116.3**	**1,026.0**	**-232.9**	**630.1**	**1,151.1**	**1,164.5**	**1,275.1**	**4,220.8**
Interest Expense/Income, net	570.6	170.8	146.6	124.7	125.4	567.5	117.9	118.7	131.0	134.0	501.6
Other Income/Expense, net	-22.8	-31.2	-23.1	-16.9	-14.9	-86.1	-17.6	13.2	-	-	-4.4
Earnings Before Tax	**4,379.8**	**1,279.1**	**-3,391.2**	**1,224.1**	**1,136.5**	**248.5**	**730.4**	**1,283.0**	**1,295.5**	**1,409.1**	**4,718.0**
Income Tax Expense	760.2	179.5	202.4	178.7	223.5	784.1	84.1	250.1	207.3	225.5	766.9
Net Income	**3,619.6**	**1,099.6**	**-3,593.6**	**1,045.4**	**913.0**	**-535.6**	**646.3**	**1,032.9**	**1,088.2**	**1,183.6**	**3,951.0**
Weighted Average Basic Shares Outstanding	257.7	258.2	258.1	258.0	257.5	257.9	256.9	256.7	256.5	256.2	256.6
Weighted Average Diluted Shares Outstanding	260.5	261.1	258.1	261.0	260.5	257.9	259.5	258.9	258.5	258.3	258.8
Net Income per Share, GAAP, Basic	$14.05	$4.26	-$13.92	$4.05	$3.55	-$2.08	$2.52	$4.02	$4.24	$4.62	$15.40
Net Income per Share, GAAP, Diluted	$13.89	$4.21	-$13.92	$4.01	$3.50	-$2.08	$2.49	$3.99	$4.21	$4.58	$15.27
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

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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.

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Company-Specific Disclosures

Distribution of Ratings Kingswood Capital Partners, LLC			Investment Banking Services/Past 12 Months	
Rating	**Count**	**Percent**	**Count**	**Percent**
BUY	9	81.81	2	22.22
HOLD	1	9.09	0	0.00
SELL	0	0.00	0	0.00
NOT RATED	1	9.09	1	100.00

As of July 2025.

Kingswood Capital Partners has not received compensation from Vertex Pharmaceuticals, Inc. during the past 12 months. Kingswood is not currently engaged by Vertex Pharmaceuticals to provide investment banking or advisory services. The Research Analyst has a financial interest in VRTX stock.

Vertex Pharmaceuticals Rating History as of August 7, 2025

Source: E-Trade.

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