Vertex Pharmaceuticals, Inc. (Nasdaq: VRTX)

Rating: Buy

Price Target: $500.00
Share Price: $462.58

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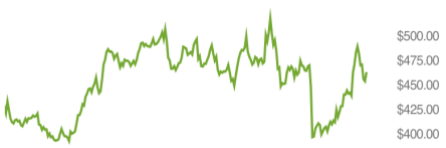
Company Data

Average Daily Volume (M)	1.67
52-Week Range	377.85-519.88
Shares Outstanding (M)	256.90
Market Cap (B)	118.84
Enterprise Value (B)	114.38
Total Cash (B), mrq	6.12
Total Debt (B)	1.66
Total Debt to Cap	9.17%

GAAP Estimates

FYE: Dec		2024EA	2025E
EPS	Q1	4.21	3.67
	Q2	(13.92)	3.69
	Q3	4.01	3.83
	Q4	3.50	4.04
	FY	(2.08)	15.23
P/E		NM	30.4x
Rev	Q1	2,690.6	2,850.0
	Q2	2,645.6	2,875.0
	Q3	2,771.9	3,000.0
	Q4	2,912.0	3,150.0
	FY	11,020.1	11,875.0
EV/Sales		10.4x	9.6x

One-Year Performance Chart



As of February 13, 2025. Source: E*Trade.

February 14, 2025

Vertex Pharmaceuticals Reports Fourth Quarter and Full Year 2024 Earnings

Earnings Miss Amid Strong Performance; Expectations for Continued Growth and Diversification of Product Base in 2025; Reiterate BUY Rating

Summary

- On February 10, 2025, Vertex reported consolidated financial results for the fourth quarter and full year ended December 31, 2024, and provided its FY2025 financial guidance. Product revenue of $2,912.0 million for the fourth quarter and of $11,020.1 million for the year ended December 31, 2024, exceeded Kingswood Capital estimates of $2,775.0 million and $10,883.1 million by 4.9% and 1.3%, respectively. However, GAAP EPS of $3.50 for the quarter and $(2.08) for FY 2024 fell 12.5% and 24.5% short of our forecast of $4.00 and $(1.57). These numbers reflect higher than anticipated R&D expenses and effective tax rates.

- Vertex issued FY 2025 revenue guidance of $11.75-12.0 billion, reflecting expectations for continued growth in cystic fibrosis, continued uptake of CASGEVY for sickle cell disease and transfusion-dependent beta thalassemia, as well as early contributions from the commercial launch of JOURNAVX for acute pain. The company predicted 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 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, APOL1-mediated kidney disease, IgA nephropathy (IgAN), and Type 1 diabetes.

- Sustained execution has helped Vertex achieve both a strong operating margin and cash position, allowing continued, significant investments in its pipeline and commercial capabilities. We reiterate our BUY rating and $500 price target on the stock in light of ongoing diversification of the company's revenue base, disease areas of focus, R&D pipeline, and operating geographies, which we expect will build long-term value for shareholders.

Q4 2025 Financial Results

- Product revenue was $2,912.0 million, a 16% increase over product revenue of $2,517.7 million for Q4 2023, primarily driven by the strong performance of TRIKAFTA/KAFTRIO. Revenue growth rates were 17% in the U.S. (to $1.84 billion) and 14% outside the U.S. (to $1.07 billion), compared to the fourth quarter of 2023.

- Combined GAAP R&D and SG&A expenses increased by 20% to $1,375.1 million, compared to $1,143.4 million in the fourth quarter of 2023, 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.

- Acquired in-process R&D (AIPR&D) expenses were $87.5 million, compared to $17.8 million in the fourth quarter of 2023.

- Q4 2024 GAAP net income was $913.0 million, compared to $968.8 million in the fourth quarter of 2023, as increased product revenue was more than offset by increased operating expenses, lower interest income, and increased tax expense compared to Q4 2023. The higher effective tax rate derived from reduced U.S. R&D tax credits.

- Q4 2024 GAAP EPS was $3.50 on 260.5 million diluted shares outstanding, compared to $3.71 on 260.9 million diluted shares outstanding in the fourth quarter of 2023.

FY 2024 Financial Results

- Product revenue for FY 2024 was $11,020.1 million, a 12% increase over FY 2023, primarily driven by the strong performance of TRIKAFTA/KAFTRIO. Revenue growth rates were 11% in the U.S. (to $6.68 billion) due to continued strong patient demand and higher net realized pricing, and 13% outside the U.S. (to $4.34 billion) due to strong patient demand in both established and newer markets.

- Combined GAAP R&D and SG&A expenses for FY 2024 were $5,094.6 million, compared to $4,299.5 million for FY 2023, due to increased commercial investment in support of global

product launches and continued R&D investments in support of clinical development programs that have advanced to Phase 3.

- FY 2024 acquired in-process R&D (AIPR&D) expenses were $4,628.4 million, compared to $527.1 million for FY 2023, reflecting Vertex's acquisition of Alpine Immune Sciences during 2024.

- FY 2024 GAAP net loss was ($535.6) million, compared to net income of $3,619.6 million for FY 2023, as strong operating results were offset by the impact of higher AIPR&D expenses related to the Alpine acquisition.

- FY 2024 GAAP EPS was ($2.08) on 257.9 million diluted shares outstanding, compared to $13.89 on 260.5 million diluted shares outstanding for FY 2023.

- Vertex ended the year with $6,115.9 million in cash, cash equivalents, and marketable securities, compared to $11,218.3 million as of 12/31/23. The decrease is primarily due to the $5.0 billion cash consideration paid to acquire Alpine Immune Sciences in the second quarter of 2024 and $1.2 billion spent on the repurchase of approximately 2.7 million shares of VRTX common stock under the company's share repurchase program, partially offset by positive cash flow from other operations.

FY 2025 Guidance

- Vertex issued FY 2025 revenue guidance of $11.75-12.00 billion, reflecting expectations for continued growth in cystic fibrosis, continued uptake of CASGEVY for sickle cell disease and transfusion-dependent beta thalassemia, as well as early contributions from the commercial launch of JOURNAVX for acute pain.

- The company predicted FY 2025 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%.

Commercial and Clinical Highlights

Cystic Fibrosis (CF)

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

- In December 2024, Vertex received FDA approval for the expanded use of its best-selling CF drug, TRIKAFTA/KAFTRIO. The drug is now approved in the U.S. for patients with a total of 272 cystic fibrosis transmembrane conductance regulator (CFTR) mutations.

- Vertex's ALYFTREK triple combination therapy for CF in patients six years and older received FDA approval in December 2024. ALYFTREK is under regulatory review in the United Kingdom, EU, Canada, Switzerland, Australia, and New Zealand.

- Vertex continues to develop next-generation, oral, small molecule CFTR modulators, as well as a nebulized mRNA therapy for CF patients who do not make CFTR protein and cannot benefit from CFTR modulators. Data from a Phase 1/2 clinical trial of VX-522, a nebulized CFTR mRNA therapy, is expected in the first half of 2025.

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.

- As of year-end 2024, Vertex has activated more than 50 authorized treatment centers globally and more than 50 patients across all regions have initiated cell collection. CASGEVY's contribution to 2024 product revenue was $10 million. Vertex expects the number of new patients to grow significantly throughout 2025.

- For label expansion purposes, Vertex has completed enrollment of children aged 5-11 years with SCD or TDT in two global Phase 3 studies of CASGEVY and expects to complete dosing of this age group in 2025.

Acute 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.

- Vertex is working to make JOURNAVX available through national retail pharmacies and pharmacy chains, with shipping expected to begin by the end of February 2025.

- A Phase 3 pivotal trial of suzetrigine in patients with painful diabetic peripheral neuropathy (DPN), a type of chronic peripheral neuropathic pain (PNP) that accounts for approximately 20% of patients suffering from PNP, is currently enrolling. The FDA has granted suzetrigine Breakthrough Therapy Designation in DPN.

- Vertex also plans to initiate a Phase 3 study of suzetrigine in painful lumbosacral radiculopathy (LSR, or sciatica) during 2025.

Other Programs

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

- Vertex is currently enrolling 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, comparing a once-daily dose of inaxaplin to placebo plus standard of care. A pre-planned interim analysis will be conducted after a certain number of patients reach 48 weeks of treatment. Enrollment in the interim analysis cohort is expected to complete in 2025. If positive, the interim analysis may serve as the basis for accelerated approval in the U.S.

- 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. The povetacicept Phase 3 study, RAINIER, is a global pivotal trial of povetacicept 80mg versus placebo on top of standard of care in approximately 480 patients with IgAN. A pre-planned interim analysis will be conducted after a certain number of patients reach 36 weeks of treatment, evaluating the change from baseline in the urine protein creatinine ratio for the povetacicept arm versus the placebo arm. Vertex expects to complete enrollment in this interim analysis cohort in 2025. If positive, the interim analysis may serve as the basis for accelerated approval in the U.S. Final analysis will occur at two years of treatment, with a primary endpoint of total estimated glomerular filtration rate slope through Week 104.

- Zimislecel (VX-880) advanced to Phase 3 testing in type 1 diabetes. 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 reached agreement with regulatory agencies in the U.S. and Europe to convert its ongoing Phase 1/2 study to a Phase 1/2/3 study, in which a total of 50 patients will be infused with a single target dose of VX-880. The primary endpoint is the proportion of patients with insulin independence and absence of severe hypoglycemic episodes. Vertex expects to complete enrollment and dosing of the pivotal study in 2025 and file for potential approval after patients have completed one year of insulin-free follow-up, assuming positive data.

Management Transition

- Vertex's Chief Operating Officer, Stuart Arbuckle, will be retiring on July 1, 2025. Charlie Wagner, Vertex's Chief Financial Officer, will be adding the COO responsibilities to his current role, while Duncan McKechnie, SVP and Head of North America Commercial, will assume the role of Chief Commercial Officer.

Transactions

- Vertex announced two transactions: (i) an exclusive collaboration and license agreement with Zai Lab for the development and commercialization off povetacicept in mainland China, Hong Kong, Macau, Taiwan, and Singapore, signed in January 2025, and (ii) a strategic collaboration with Orna Therapeutics for the use of Orna's lipid nanoparticle (LNP) technology to develop *in vivo* gene editing therapies for SCD and TDT. *In vivo* gene editing would avoid the complex process of collecting cells from a patient, manipulating them in a lab and re-implanting them, making it potentially more scalable and less invasive than the *ex vivo* gene editing process required with CASGEVY.

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™, its 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 begin accumulating shares of Vertex during the next 12-18 months. Our 12-month price target of $500.00 is based on 2025 projected earnings of $15.23 and a P/E multiple of 32.8x.

Vertex Pharmaceuticals Financial Forecast											
	FY 23 A	MAR 24 A	JUN 24 A	SEP 24 A	DEC 24 A	FY 24 A	MAR 25 E	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	2,850.0	2,875.0	3,000.0	3,150.0	11,875.0
Cost of Sales	1,262.2	342.6	371.9	392.6	423.4	1,530.5	413.3	416.9	435.0	456.8	1,721.9
Gross Profit	**8,607.0**	**2,348.0**	**2,273.7**	**2,379.3**	**2,488.6**	**9,489.6**	**2,436.8**	**2,458.1**	**2,565.0**	**2,693.3**	**10,153.1**
Operating Expenses:											
R&D	3,162.9	789.1	966.6	875.9	998.7	3,630.3	969.0	991.9	1,050.0	1,102.5	4,113.4
Acquired in-process R&D	527.1	76.8	4,449.1	15.0	87.5	4,628.4	25.0	25.0	25.0	25.0	100.0
SG&A	1,136.6	342.7	372.2	371.8	377.6	1,464.3	370.5	366.6	375.0	385.9	1,497.9
Change in Fair Value of Contingent Consideration	-51.6	-0.1	0.5	0.3	-1.2	-0.5	0.0	0.0	0.0	0.0	0.0
Total Operating Expenses	**4,775.0**	**1,208.5**	**5,788.4**	**1,263.0**	**1,462.6**	**9,722.5**	**1,364.5**	**1,383.4**	**1,450.0**	**1,513.4**	**5,711.3**
Operating Income	**3,832.0**	**1,139.5**	**-3,514.7**	**1,116.3**	**1,026.0**	**-232.9**	**1,072.3**	**1,074.7**	**1,115.0**	**1,179.9**	**4,441.8**
Interest Expense/Income, net	570.6	170.8	146.6	124.7	125.4	567.5	125.0	128.0	131.0	134.0	518.0
Other Income/Expense, net	-22.8	-31.2	-23.1	-16.9	-14.9	-86.1	-	-	-	-	-
Earnings Before Tax	**4,379.8**	**1,279.1**	**-3,391.2**	**1,224.1**	**1,136.5**	**248.5**	**1,197.3**	**1,202.7**	**1,246.0**	**1,313.9**	**4,959.8**
Income Tax Expense	760.2	179.5	202.4	178.7	223.5	784.1	251.4	252.6	261.7	275.9	1,041.6
Net Income	**3,619.6**	**1,099.6**	**-3,593.6**	**1,045.4**	**913.0**	**-535.6**	**945.8**	**950.1**	**984.3**	**1,038.0**	**3,918.3**
Weighted Average Basic Shares Outstanding	257.7	258.2	258.1	258.0	257.5	257.9	257.4	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	257.6	257.4	257.2	257.0	257.3
Net Income per Share, GAAP, Basic	$14.05	$4.26	-$13.92	$4.05	$3.55	-$2.08	$3.67	$3.69	$3.83	$4.04	$15.24
Net Income per Share, GAAP, Diluted	$13.89	$4.21	-$13.92	$4.01	$3.50	-$2.08	$3.67	$3.69	$3.83	$4.04	$15.23
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.

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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

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Rating	**Count**	**Percent**	**Count**	**Percent**
BUY	4	80.00	1	25.00
HOLD	0	0.00	0	0.00
SELL	0	0.00	0	0.00
NOT RATED	1	20.00	1	100.00

As of November 2024.

Vertex Pharmaceuticals Rating History as of February 13, 2025

Source: E-Trade.

Other Disclosures

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