Vertex Pharmaceuticals, Inc. (Nasdaq: VRTX)

Rating: Buy

Price Target: $550.00
Share Price: $468.29

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December 12, 2024

Company Data	
Average Daily Volume (M)	1.16
52-Week Range	372.80-519.88
Shares Outstanding (M)	257.53
Market Cap (B)	120.84
Enterprise Value (B)	116.02
Total Cash (B), mrq	6.52
Total Debt (B), mrq	1.77
Total Debt to Cap	10.19%

GAAP Estimates				
FYE: Dec		2023A	2024E	2025E
EPS	Q1	2.69	4.21	3.86
	Q2	3.52	(13.92)	3.90
	Q3	3.97	4.01	3.96
	Q4	3.71	4.00	4.06
	FY	13.89	(1.57)	15.79
P/E		33.7x	NM	29.7x
Rev	Q1	2,374.8	2,690.6	2,750.0
	Q2	2,493.2	2,645.6	2,790.0
	Q3	2,483.5	2,771.9	2,845.0
	Q4	2,517.7	2,775.0	2,920.0
	FY	9,869.2	10,883.1	11,305.0
EV/Sales		11.8x	10.7x	10.3x

One-Year Performance Chart

As of December 11, 2024. Source: E*Trade.

Vertex Pharmaceuticals Provides CASGEVY Program Update at ASH

Transformative Benefits Prove Durable in Sickle-Cell Disease and Transfusion-Dependent Beta Thalassemia; Reiterate BUY Rating

Summary

- Vertex provided a program update on CASGEVY™, the first and to date only approved CRISPR/Cas9 gene-edited therapy, at the American Society of Hematology (ASH) Annual Meeting this week, revealing data from long-term follow-up of patients who participated in clinical trials.

- Transformative benefits of CASGEVY proved durable in both sickle cell disease (SCD) and transfusion-dependent beta thalassemia (TDT).

- CASGEVY's safety profile is consistent with busulfan conditioning and autologous hematopoietic stem cell transplant.

- Efforts to secure regulatory clearance in additional countries, improve patient access, and broaden reimbursement coverage are ongoing.

Clinical Data

- The longest follow up for both SCD and TDT patients exceeds five years, with a median of 33.2 months and 38.1 months, respectively. Evaluable patients were defined as those with at least 16 months of follow-up.

- In SCD, 39 out of 42 (93%) evaluable patients were free from vaso-occlusive crises for at least 12 consecutive months (VF12). The mean VF12 duration was 30.9 months; the maximum duration was 59.6 months.

- The three evaluable SCD patients who did not achieve VF12 had nonetheless experienced meaningful clinical benefit, reducing their rate of hospitalization by 91%, 71%, and 100%, respectively.

- In TDT, 53 out of 54 (98%) evaluable patients achieved transfusion-independence for at least 12 consecutive months with a weighted average hemoglobin of at least 9g/dL (TI12). The mean duration of transfusion independence was 34.5 months; the maximum duration was 64.1 months.

- The one evaluable TDT patient who had not achieved TI12 has been transfusion free for 8.2 months to date.

- Both SCD and TDT patients reported sustained and clinically meaningful improvements in quality of life and overall health status.

- CASGEVY's safety profile continues to be consistent with myeloablative conditioning with busulfan, a high-dose chemotherapy regimen that uses busulfan to kill bone marrow cells. The procedure destroys defective hematopoietic stem cells in the patient's bone marrow to eliminate stem cells with a mutated hemoglobin gene before the patient receives lab-edited stem cells with an intact hemoglobin gene via a hematopoietic stem cell transplant. Side effects from myeloablative conditioning with busulfan can include lowered white blood cell, red blood cell, and platelet counts, nausea and vomiting, and seizures, among others.

Commercial Landscape

- CASGEVY is approved for SCD and TDT in the U.S., European Union, Great Britain, Canada, Switzerland, Bahrain, and the Kingdom of Saudi Arabia.

- Vertex is preparing regulatory submissions in the United Arab Emirates and Kuwait.

- More than 45 authorized treatment centers have been activated globally to support the delivery of CASGEVY.

- More than 40 patients have a had a first cell collection for the purpose of gene editing.

- Vertex is continuing to work with reimbursement authorities to improve patient access to CASGEVY. In the U.S., Vertex secured an agreement with the Centers for Medicare & Medicaid Services to ensure broad and equitable access to CASGEVY.

- On September 24, 2024, Vertex and its manufacturing partner, Lonza, signed a long-term commercial supply agreement for CASGEVY for global supply of the therapy. Lonza will manufacture CASGEVY at its cGMP cell therapy manufacturing facility in Geleen, Netherlands. A second site in Portsmouth, NH, is expected to begin operations in 2025.

Our Analysis

- We perceive that the long-term clinical data available to date supports a role for CASGEVY as a functional cure for SCD and TDT.

- While broadening patient access to the therapy and lowering procedure cost remains a work in progress for the foreseeable future, we expect that the expertise of Vertex's commercial launch teams will increase CASGEVY's contribution to top line revenue in 2025.

- We reiterate our BUY rating on the stock, along with our $550 price target.

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, and TRIKAFTA, 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.

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, including having a 2023 P/E of 34.2x versus the competitor group median of 17.0x. The firm's 2023 enterprise value (EV)/Revenue was 11.1x versus its competitor group median of 6.6x. Vertex's 2023 EV/EBIT was 25.4x, versus its competitor group median of 15.6x. The company's 2023 gross margin was 87.2%, contrasted with its competitor group median of 74.2%, and its 2023 net margin was 36.7% against the competitor group median of 19.0%.

Combined with the above 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. Another near-term product launch opportunity is the vanzacaftor/tezacaftor/deutivacaftor triple oral small molecule combination for cystic fibrosis, which employs a similar mechanism of action as Vertex's blockbuster drug TRIKAFTA.

Vertex's non-opioid treatment for moderate to severe acute pain, Suzetrigine (VX-548), completed Phase 3 clinical trials in December 2023. It is now in regulatory review by the U.S. FDA. Non-opioid pain management is a significant, unmet medical need worldwide. It is possible that Suzetrigine could be approved within one calendar year, and capture a significant first mover share of the $80-90 billion global pain management market.

Based on our analysis of available clinical data, we expect regulatory approvals of the vanzacaftor triple combination and Suzetrigine in 2025, followed by accelerating revenue and EPS growth in 2026. We recommend investors begin accumulating shares of Vertex during the next 12-18 months. Our 12-month price target of $550.00 is based on next-twelve-months (NTM) projected earnings, not including one-time acquisition charges, of $15.50 and a P/E multiple of 35.5x.

Vertex Pharmaceuticals Financial Forecast											
	FY 23 A	MAR 24 A	JUN 24 A	SEP 24 A	DEC 24 E	FY 24 E	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	2,775.0	10,883.1	2,750.0	2,790.0	2,845.0	2,920.0	11,305.0
Cost of Sales	1,262.2	342.6	371.9	392.6	395.5	1,502.6	337.5	345.6	355.6	365.0	1,403.7
Gross Profit	**8,607.0**	**2,348.0**	**2,273.7**	**2,379.3**	**2,379.5**	**9,380.5**	**2,412.5**	**2,444.4**	**2,489.4**	**2,555.0**	**9,901.3**
Operating Expenses:											
R&D	3,162.9	789.1	966.6	875.9	888.0	3,519.6	935.0	948.6	967.3	992.8	3,843.7
Acquired in-process R&D	527.1	76.8	4,449.1	15.0	30.0	4,570.9	30.0	30.0	30.0	30.0	120.0
SG&A	1,136.6	342.7	372.2	371.8	374.6	1,461.3	357.5	362.7	369.9	379.6	1,469.7
Change in Fair Value of Contingent Consideration	-51.6	-0.1	0.5	0.3	0.5	1.2	-15.0	-15.0	-15.0	-15.0	-60.0
Total Operating Expenses	**4,775.0**	**1,208.5**	**5,788.4**	**1,263.0**	**1,293.1**	**9,553.0**	**1,307.5**	**1,326.3**	**1,352.2**	**1,387.4**	**5,373.4**
Operating Income	**3,832.0**	**1,139.5**	**-3,514.7**	**1,116.3**	**1,086.4**	**-172.5**	**1,105.0**	**1,118.1**	**1,137.3**	**1,167.6**	**4,528.0**
Interest Expense/Income, net	570.6	170.8	146.6	124.7	135.0	577.1	88.0	89.0	90.0	91.0	358.0
Other Income/Expense, net	-22.8	-31.2	-23.1	-16.9	-	-71.2	-	-	-	-	-
Earnings Before Tax	**4,379.8**	**1,279.1**	**-3,391.2**	**1,224.1**	**1,221.4**	**333.4**	**1,193.0**	**1,207.1**	**1,227.3**	**1,258.6**	**4,886.0**
Income Tax Expense	760.2	179.5	202.4	178.7	178.3	738.9	179.0	181.1	184.1	188.8	732.9
Net Income	**3,619.6**	**1,099.6**	**-3,593.6**	**1,045.4**	**1,043.1**	**-405.5**	**1,014.1**	**1,026.0**	**1,043.2**	**1,069.8**	**4,153.1**
Weighted Average Basic Shares Outstanding	257.7	258.2	258.1	258.0	257.9	258.0	259.8	260.0	260.4	260.7	260.2
Weighted Average Diluted Shares Outstanding	260.5	261.1	258.1	261.0	260.9	259.0	262.9	263.0	263.2	263.6	263.1
Net Income per Share, GAAP, Basic	$14.05	$4.26	-$13.92	$4.05	$4.04	-$1.57	$3.90	$3.95	$4.01	$4.10	$15.96
Net Income per Share, GAAP, Diluted	$13.89	$4.21	-$13.92	$4.01	$4.00	-$1.57	$3.86	$3.90	$3.96	$4.06	$15.79
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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SELL	0	0.00	0	0.00
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As of December 2024.

Vertex Pharmaceuticals Rating History as of December 11, 2024

Source: E-Trade.

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