Vertex Pharmaceuticals [NASDAQ: VTRX]

Initiate at BUY | PT: $289

November 19, 2021

CIMG investment Research

Biotech star with dominant cystic fibrosis portfolio at a favorable valuation

Vertex is a pharmaceutical drug manufacturer that has a product portfolio of 4 drugs targeted to treat the underlying causes of cystic fibrosis. These are the only FDA approved drugs to treat the underlying causes of cystic fibrosis. They have a pipeline consisting of another 15+ opportunities to expand their reach into other orphan disease areas. Due to bad recent clinical trial results which have caused a decrease in price, CIMG has an opportunity to enter a superior business focused on saving lives.

Investment Thesis

Vertex has established a monopoly in advanced cystic fibrosis (CF) remedies that can address ~90% of the patient population. Their latest approval, Trikafta, has allowed VRTX to expand their treatable population, which has allowed them to set up for short- and long-term growth. Beyond their legacy CF portfolio, VRTX has a robust pipeline of drugs that are aiming to treat other rare disease areas. The pipe currently consists of 5 other strategic disease areas and 13 treatments moving through trials.

Cystic fibrosis portfolio has proven dominance with further uptake potential. Vertex currently has a monopoly of the cystic fibrosis treatment space with no current competitors. VRTX is currently treating about 50% of their projected treatable population. With Trikafta approval in 2019, VRTX now has the ability to treat ~90% of the treatable population. To expand their CF portfolio, VRTX has a strong pipeline candidate (Phase III) that will improve upon the efficacy of their CF drugs, in turn spiking revenue as we've seen historically in the past with their previous CF drugs. We are projecting strong short-term uptake due to Trikafta, and long- term uptake due to their pipeline candidate.

VRTX's robust pipeline with help them drive future growth. Vertex is beginning to search for their next growth driver beyond cystic fibrosis. VRTX has been able to use their strong cash flow from their CF franchise to invest into strategic R&D opportunities. VRTX pioneered the process of rational drug design and has ample experience in rare diseases due to their CF franchise. Targeted rare disease areas for VRTX include sickle cell disease, beta thalassemia, kidney diseases, pain, and type 1 diabetes. Each of these areas has promising phase II candidates, providing safety that VRTX has a diversified pipe that provides long-term growth.

History of Vertex Pharmaceuticals

Founded in 1989, Vertex pioneered the idea of rational drug design, which is designing drugs based on understanding the proteins they wanted to target inside the body. This is substantially different from conventional drug design in which most pharmaceutical companies practiced at the time. Conventional drug design took root in finding a chemical substance and testing, most likely on animals, to determine its effects. Two decades after its inception, Vertex introduced its first drug Incivek that treated hepatitis C. Immediately upon its release, the drug ballooned to more than $1 bn in sales, but competitors were quick to copy Vertex. By 2013 sales of the drug slowed as patients began to switch to the competitor's treatment (Gilead's Sovaldi), which cured at a higher rate and without as many side effects.

Vertex was forced to make a transition to other care areas and focused heavily on cystic fibrosis. CF is a disease in which a genetic mutation prevents a protein from functioning properly and causes frequent infections often in the lungs. In 2012, Vertex's Kalydeco was approved by the FDA, which was the first drug to treat the underlying cause of CF.

Healthcare

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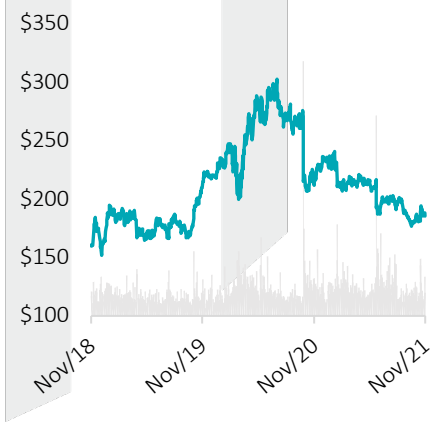
Figure 1: 3-Year Stock Chart & Volume



Price	$188
Dividend Yield	n/a
PT	$289
Upside	54%
52-Week Range	$243 - $176
Dil Shrs Outstand	258,988,000
Mkt Cap	$48bn
Ent Value	$42bn
LTM P/E	23x
LTM EV/EBITDA	15x
FY 2020 Revenue	$6.21bn
Operating Margin	46.0%
Net Margin	43.7%

success of Kalydeco has allowed Vertex to release three subsequent drugs that have strengthened the efficacy of the treatment. With four approved drugs, Vertex is effectively able to treat most genetic mutations that lead to CF. Vertex can treat 90% of the patient population and is researching solutions to reach the final 10%.

Expanding CF Franchise

Currently ~50% of the CF patient population is using one of Vertex's drugs, but there is more room to grow. In the short-term, the newest release, Trikafta, will be able to penetrate the market further. We are projecting ~13% more CF patients in the US will be under treatment by 2025.

Vertex has a phase III candidates in the pipeline that is looking promising. The new drug is likely to provide increased efficacy and be taken less often than the current options. We are projecting more patients will be willing to take the new drug after its release due to the benefits. In our projections, we have the penetration rate of Vertex's CF franchise to be reaching ~80% of patients by 2030.

Pipeline Beyond CF

While Vertex has seen success from its CF franchise, it is not time to start searching for new drugs to help grow the company into the future. Vertex spent more $11.8 bn on drug discovery and development from 2000 to 2019 to target other rare diseases as they believe this aligns with their strengths.

Sickle Cell Disease and Beta-Thalassemia

Vertex is teaming up with CRISPR on a one-time cure for sickle cell disease and beta-thalassemia. Due to the well-understood genetics of these diseases, Vertex is using a gene editing approach to find a cure. The most promising treatment is being evaluated for its safety and efficacy in phase I/II trials. The treatment has passed phase II proof of concept.

APOL1-Mediated Kidney Diseases

Mutations in the APOL1 gene can cause of kidney diseases such as focal segmental glomerulosclerosis (FSGS). Vertex aims to cure FSGS with a small molecule inhibitor off APOL1 function. Vertex's VX-147 is in phase II, and proof-of-concept results are expected in the next 3-6 months.

Pain

Current medicines addressing acute, neuropathic, and musculoskeletal pain are not strongly effective, have significant side effects, and have potential for addiction. VRTX is developing inhibitors of sodium channels embedded in sensory neurons that have shown proof-of-concept on the reduction of pain. These drugs are awaiting approval to move to phase III. Note: Pain treatments were not included in our valuation

Type I Diabetes

VRTX is investigating and advancing cell therapies aimed at treating an underlying cause of type 1 diabetes (T1D), which is the absence of insulin producing cells. The investigational approach is to replace the insulin producing cells that have been destroyed in people with T1D. VRTX is evaluating an approach where healthy cells would be transplanted and would require immunosuppression like an organ transplant. First data is expected to be released in the next 6 months as the treatment is in phase I/II.

Valuation

Our $289 price target for VRTX is based on a probability-adjusted discounted cash flow (DCF) model. We used an 8% discount rate and a long-term growth rate of 0% and projected out 10 years of cash flows. The core CF franchise of VRTX is contributing to the majority of the upside potential and represents ~$252 of the value on a per share basis. Additionally, we conservatively valued the pipeline of VRTX at ~$37 per share. This brings our valuation to $289 per share, representing ~54% upside potential to the current share price.

Figure 2: Rational vs Conventional Dev.

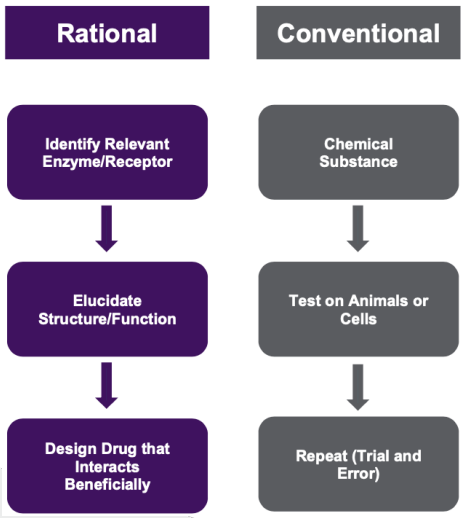


Figure 3: CF Patient Outcomes

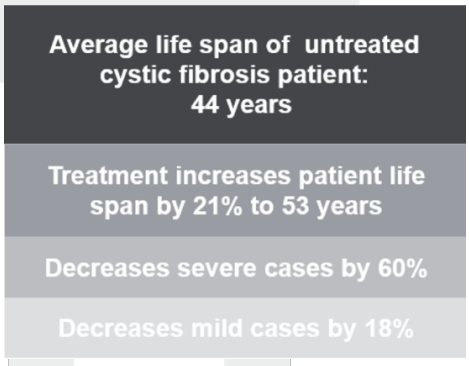


Figure 3: Small Molecule Addl. Info

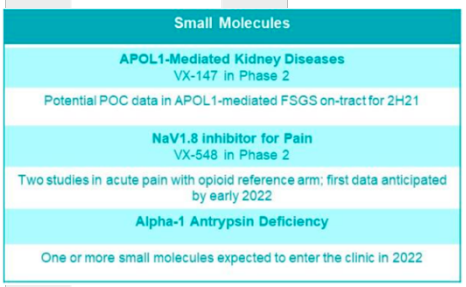
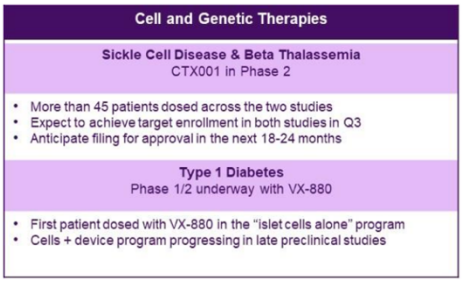


Figure 4: Cell & Genetic Therapies Addl. Info



Vertex Pharmaceutical Product Pipeline