Vertex's fourth quarter results were mixed with the company posting a wider-than-expected loss but beating top-line estimates. The company, which was previously a strong player in the hepatitis C virus market, has now shifted its focus to the cystic fibrosis (CF) market. CF drug, Kalydeco, is doing very well and Vertex is working on expanding the product's label. Vertex has some important regulatory events coming up. Approval for the Kalydeco – lumacaftor combination would provide access to a huge number of eligible patients and boost Kalydeco sales. However, we remain concerned about Vertex's dependence on just the CF franchise for growth.

SUMMARY DATA

<table>
<thead>
<tr>
<th>Risk Level *</th>
<th>Average</th>
</tr>
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<tbody>
<tr>
<td>Type of Stock</td>
<td>Large-Growth</td>
</tr>
<tr>
<td>Industry</td>
<td>Med-Biomed/Gene</td>
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<tr>
<td>Zacks Industry Rank *</td>
<td>76 out of 267</td>
</tr>
</tbody>
</table>

52-Week High | $129.79
52-Week Low | $62.44
One-Year Return (%) | 64.29
Beta | 0.32
Average Daily Volume (sh) | 1,494,781
Shares Outstanding (mil) | 242
Market Capitalization ($mil) | $31,409
Short Interest Ratio (days) | 1.98
Institutional Ownership (%) | 94
Insider Ownership (%) | 2

Annual Cash Dividend | $0.00
Dividend Yield (%) | 0.00

5-Yr. Historical Growth Rates
Sales (%) | 60.3
Earnings Per Share (%) | N/A
Dividend (%) | N/A

P/E using TTM EPS | N/A
P/E using 2015 Estimate | N/A
P/E using 2016 Estimate | 33.8

Zacks Rank *: Short Term
1 – 3 months outlook | 3 - Hold

* Definition / Disclosure on last page

ZACKS CONSENSUS ESTIMATES

Revenue Estimates
(In millions of $)

<table>
<thead>
<tr>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
<th>Q4</th>
<th>Year</th>
</tr>
</thead>
<tbody>
<tr>
<td>(Mar)</td>
<td>(Jun)</td>
<td>(Sep)</td>
<td>(Dec)</td>
<td>(Dec)</td>
</tr>
<tr>
<td>2013</td>
<td>328 A</td>
<td>311 A</td>
<td>222 A</td>
<td>169 A</td>
</tr>
<tr>
<td>2014</td>
<td>108 A</td>
<td>122 A</td>
<td>165 A</td>
<td>141 A</td>
</tr>
<tr>
<td>2015</td>
<td>141 E</td>
<td>150 E</td>
<td>274 E</td>
<td>476 E</td>
</tr>
<tr>
<td>2016</td>
<td>2,758 E</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Note: 4Q13 onwards revenues exclude HCV sales.

Earnings per Share Estimates
(EPS is operating earnings before non-recurring items, but including employee stock options expenses)

<table>
<thead>
<tr>
<th>Q1</th>
<th>Q2</th>
<th>Q3</th>
<th>Q4</th>
<th>Year</th>
</tr>
</thead>
<tbody>
<tr>
<td>(Mar)</td>
<td>(Jun)</td>
<td>(Sep)</td>
<td>(Dec)</td>
<td>(Dec)</td>
</tr>
<tr>
<td>2013</td>
<td>-$0.12 A</td>
<td>-$0.21 A</td>
<td>-$0.46 A</td>
<td>-$0.66 A</td>
</tr>
<tr>
<td>2014</td>
<td>-$0.85 A</td>
<td>-$0.79 A</td>
<td>-$0.56 A</td>
<td>-$0.73 A</td>
</tr>
<tr>
<td>2015</td>
<td>-$0.80 E</td>
<td>-$0.85 E</td>
<td>-$0.52 E</td>
<td>$0.28 E</td>
</tr>
<tr>
<td>2016</td>
<td>$3.84 E</td>
<td></td>
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</table>

Note: Excluding HCV figures from 4Q13.

Projected EPS Growth - Next 5 Years % | N/A
OVERVIEW

Boston, MA-based Vertex Pharmaceuticals, Inc. is focused on the discovery, development, and commercialization of small molecule drugs targeting serious diseases. The company’s main area of focus is cystic fibrosis (CF).

The company’s lead product is Kalydeco (ivacaftor), which is approved for the treatment of patients (six years and above) with CF who have specific genetic mutations in their cystic fibrosis transmembrane conductance regulator (CFTR) gene. Vertex is working on expanding Kalydeco’s label and is studying it in combination with other CF candidates (with lumacaftor and VX-661).

Earlier, Vertex had a strong presence in the hepatitis C virus (HCV) market. However, with the entry of new products, the company reduced its focus in this area and subsequently dropped its HCV programs.

While CF remains the main area of focus, Vertex has other early-stage development programs in its pipeline targeting oncology (VX-970 and VX-803 – phase I for advanced solid tumor), genetic diseases and neurology. But the company is seeking partners for some of these programs. VX-787, a phase II candidate for the treatment of influenza, has been out-licensed to Janssen.

The company recorded total revenues of $535.8 million in 2014, down 48%.

REASONS TO BUY

- **Kalydeco a Potential Blockbuster:** The 2012 FDA approval of CF drug, Kalydeco, was a major boost for Vertex. Kalydeco is performing well with 2014 sales coming in at $463.8 million, up 24.9%. The product should continue performing well with reimbursement in additional countries leading to higher sales. We are optimistic on Kalydeco’s prospects and are encouraged by the company’s efforts to expand the label. Kalydeco, which was initially approved for use as a monotherapy in CF patients (age ≥6 years) who have the G551D mutation, gained FDA and EU approval for eight additional mutations in 2014 – this means another 400 eligible patients (150 in the U.S. and 250 in the EU). Vertex is currently looking to get Kalydeco approved in additional patient populations. FDA approval for children aged 2-5 years with specific mutations in their CFTR gene should drive sales in the second half of the year (under review in the EU). About 300 children in the U.S. fall in this category. Vertex also gained FDA approval for patients with the R117H mutation (EU review ongoing) – it is estimated that there are about 700 patients with the R117H mutation in North America, Europe and Australia. Patients with the R117H mutation have already started treatment and there should be continued uptake in the coming months. Meanwhile, encouraging results from a proof-of-concept study in patients with residual CFTR function were out in Jun 2014. Vertex intends to conduct a larger phase III study in patients with residual CFTR function – more than 3,000 people (6 years and above) are estimated to have a non-R117H residual function mutation in North America, Europe and Australia.

Vertex expects the number of patients eligible for Kalydeco to increase from 3,100 in 2014 to more than 3,700 by year end. Sales should be driven by continued uptake for the eight additional mutations in the U.S., reimbursement for the G551D patients in Australia and Canada and approval for other additional gating type mutations. Kalydeco has blockbuster potential. The currently approved indication addresses a small percentage of the CF patient population – so there is ample scope for the company to expand the commercial potential of the drug. Kalydeco sales are expected in the range of $560 - $580 million in 2015.

- **Strong CF Pipeline:** The CF market represents huge commercial potential. It is a rare, life-threatening disease estimated to affect about 75,000 people in North America, Europe and Australia. Vertex enjoys a
strong position in this market being the first company to successfully develop a drug (Kalydeco) that treats the underlying cause of CF.

Apart from Kalydeco, Vertex has a couple of promising CF correctors in its pipeline – lumacaftor and VX-661. Both are being evaluated in combination with ivacaftor. While the lumacaftor-ivacaftor combination generated positive phase III data and has been filed for approval, the lumacaftor-VX-661 combination is in phase III development. The CF correctors could bring in multi-billion dollar sales for Vertex if developed successfully.

- **Kalydeco – Lumacaftor Combo Data Looks Solid**: In Jun 2014, Vertex presented highly-awaited pivotal phase III data from a couple of studies (TRAFFIC and TRANSPORT) evaluating Kalydeco in combination with lumacaftor in CF patients (age ≥12 years) who have two copies of the F508del mutation in the CFTR gene. This is the most common form of the disease. About 22,000 (including 8,500 in the U.S. and 12,000 in Europe) people (age ≥12 years) across North America, Europe and Australia have two copies of the F508del mutation. Vertex is currently seeking U.S. (response expected by Jul 5) and EU (response expected in the fourth quarter) approval for this combination. The combination has breakthrough therapy designation in the U.S. If approved, adoption should be rapid as this combination would be the first to address the underlying cause of CF in this particular patient population.

- **Positive on Incivo Royalty Monetization**: In Nov 2013, Vertex sold its product royalty rights to its HCV treatment, Incivo, to partner Janssen. Vertex received a cash payment of $152 million. This deal was in line with Vertex’s efforts to move away from its HCV franchise and focus on the cystic fibrosis franchise. With patients awaiting new treatments, Incivek/Incivo revenues were declining rapidly. In such a scenario, the monetization of Incivo royalties makes sense. The $152 million cash inflow has come in handy for the company’s CF pipeline development efforts. Vertex also out-licensed its experimental flu treatment, VX-787 to Janssen. This deal will also bring in upfront and milestone payments and royalties.

### REASONS TO SELL

- **Competing Therapies in Development**: The CF market has been attracting the interest of several companies like Novartis, Pfizer and Sanofi. These companies are pursuing the development of CFTR potentiators, CFTR correctors and candidates with other mechanisms of action that can address the underlying cause of CF. Even though Vertex enjoys a strong position in this market, the entry of additional competition would cut into revenues.

- **Banking on CF Franchise**: Although we are positive on Vertex’s decision to focus on the CF franchise, we remain concerned about the company’s dependence on just the CF franchise for growth. Vertex has several studies ongoing with its CF product candidates – pipeline and regulatory setbacks would have an adverse impact on the shares.

### RECENT NEWS

**Vertex Posts Wider Fourth Quarter Loss, Affirms Kalydeco View – Jan 28**

Vertex Pharmaceuticals Inc.’s (VRTX) fourth-quarter 2014 loss came in at $0.73 per share (including stock-based compensation expense), much wider than the year-ago loss of $0.66 and the Zacks Consensus Estimate of a loss of $0.61.

Excluding the impact of stock-based compensation expense, fourth quarter 2014 loss was $0.55 per share as against the year-ago loss of $0.56 per share.
Vertex Pharma reported revenues of $140.6 million in the fourth quarter of 2014 (excluding hepatitis C virus/HCV revenues and royalties). Revenues were above the Zacks Consensus Estimate of $138 million but below the year-ago revenues of $168.8 million.

Full year loss was $2.93 per share, well above the year-ago loss of $1.47 per share. Meanwhile, revenues declined 47.9% to $535.8 million.

**Kalydeco Driving Revenues**

Vertex's fourth-quarter revenues consisted of sales from cystic fibrosis (CF) product Kalydeco ($124.4 million) and royalties and collaborative revenues ($16.2 million).

Vertex reported a 13.7% increase in Kalydeco sales in the fourth quarter of 2014 from the year-ago period. Sales benefited from use for additional mutations in the U.S. and increased revenues from ex-U.S. markets. Kalydeco revenues were, however, down slightly (1.9%) on a sequential basis. The sequential decline was expected as there was some inventory stocking in the third quarter as well as delayed access to patients in Australia (an issue that has since been resolved).

Adjusted (including stock-based compensation expense) research and development (R&D) expenses decreased 10.6% to $201.4 million. Fourth-quarter 2014 adjusted (including stock-based compensation expense) selling, general and administrative (SG&A) expenses increased 7.6% to $77 million.

**Affirms 2015 Outlook**

Vertex confirmed its guidance for 2015. The company expects Kalydeco revenues of $560 to $580 million. This takes into account the assumption that eligible patients in Australia will use Kalydeco now that reimbursement discussions have been completed. There should be rapid uptake in Australia in the first half of 2015 where more than 200 children and adults with CF are expected to be eligible for treatment.

Moreover, the guidance includes the impact of the late 2014 FDA approval of Kalydeco for use in CF patients with the R117H mutation – patients have already started treatment and there should be continued uptake in the coming months. The guidance also assumes the completion of reimbursement discussions for gating mutations in certain European countries. Vertex has also taken into consideration the use of Kalydeco in children with CF ages 2 to 5 with the G551D or other gating mutations in the U.S. Approval for this indication should drive Kalydeco growth in the second half of the year.

Vertex expects the number of patients eligible for Kalydeco to increase from the current level of 3,100 to more than 3,700 by year end.

Operating expenses will go up as the company gears up for the potential launch of its Kalydeco-lumacaftor combination (FDA action date: Jul 5; EU approval could come in the fourth quarter) and a phase III program evaluating VX-661+Kalydeco. The company expects operating expenses (excluding operating expenses) in the range of $1.05 billion to $1.10 billion (R&D: $770 - $800 million and SG&A: $280 - $300 million).

**VALUATION**

Vertex's fourth-quarter 2014 loss was $0.73 per share, much wider than the year-ago loss of $0.66 and the Zacks Consensus Estimate of a loss of $0.61.
Vertex reported revenues of $140.6 million in the fourth quarter of 2014 (excluding hepatitis C virus/HCV revenues and royalties). Revenues were above the Zacks Consensus Estimate of $138 million but below the year-ago revenues of $168.8 million.

Kalydeco is doing very well and Vertex is working on expanding the product's label. Approval for the Kalydeco - lumacaftor combination would provide access to a huge number of eligible patients.

We maintain a Neutral recommendation on Vertex. While we believe Kalydeco has blockbuster potential, we remain concerned about the company's dependence on just the CF franchise for growth. Our $136 price target corresponds to a P/B multiple of 29.2.

### Key Indicators

<table>
<thead>
<tr>
<th>Vertex Pharmaceuticals Incorporated (VRTX)</th>
<th>P/E F1</th>
<th>P/E F2</th>
<th>Est. 5-Yr EPS Gr%</th>
<th>P/CF (TTM)</th>
<th>P/S (TTM)</th>
<th>P/E 5-Yr High (TTM)</th>
<th>P/E 5-Yr Low (TTM)</th>
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</thead>
<tbody>
<tr>
<td>Industry Average</td>
<td>47.4</td>
<td>52.4</td>
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<td>38.8</td>
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<tr>
<td>S&amp;P 500</td>
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<td>6.4</td>
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<td>Incyte Corporation (INCY)</td>
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<td>Emergent BioSolutions, Inc. (EBS)</td>
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<td>12.8</td>
<td>23.4</td>
<td>40.8</td>
<td>11.6</td>
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TTM is trailing 12 months; F1 is 2015 and F2 is 2016, CF is operating cash flow

<table>
<thead>
<tr>
<th>Vertex Pharmaceuticals Incorporated (VRTX)</th>
<th>P/B Last Qtr.</th>
<th>P/B 5-Yr High</th>
<th>P/B 5-Yr Low</th>
<th>ROE (TTM)</th>
<th>D/E Last Qtr.</th>
<th>Div Yield Last Qtr.</th>
<th>EV/EBITDA (TTM)</th>
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<tbody>
<tr>
<td>Industry Average</td>
<td>27.9</td>
<td>28.7</td>
<td>7.0</td>
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<tr>
<td>S&amp;P 500</td>
<td>13.4</td>
<td>13.4</td>
<td>13.4</td>
<td>-112.7</td>
<td>0.1</td>
<td>0.1</td>
<td>40.1</td>
</tr>
</tbody>
</table>

TTM is trailing 12 months; F1 is 2015 and F2 is 2016, CF is operating cash flow
Earnings Surprise and Estimate Revision History
DISCLOSURES & DEFINITIONS

The analysts contributing to this report do not hold any shares of VRTX. The EPS and revenue forecasts are the Zacks Consensus estimates. Additionally, the analysts contributing to this report certify that the views expressed herein accurately reflect the analysts' personal views as to the subject securities and issuers. Zacks certifies that no part of the analysts' compensation was, is, or will be, directly or indirectly, related to the specific recommendation or views expressed by the analyst in the report. Additional information on the securities mentioned in this report is available upon request. This report is based on data obtained from sources we believe to be reliable, but is not guaranteed as to accuracy and does not purport to be complete. Because of individual objectives, the report should not be construed as advice designed to meet the particular investment needs of any investor. Any opinions expressed herein are subject to change. This report is not to be construed as an offer or the solicitation of an offer to buy or sell the securities herein mentioned. Zacks or its officers, employees or customers may have a position long or short in the securities mentioned and buy or sell the securities from time to time. Zacks uses the following rating system for the securities it covers. **Outperform**: Zacks expects that the subject company will outperform the broader U.S. equity market over the next six to twelve months. **Neutral**: Zacks expects that the company will perform in line with the broader U.S. equity market over the next six to twelve months. **Underperform**: Zacks expects the company will underperform the broader U.S. Equity market over the next six to twelve months. The current distribution of Zacks Ratings is as follows on the 1130 companies covered: Outperform - 15.0%, Neutral - 75.2%, Underperform – 8.9%. Data is as of midnight on the business day immediately prior to this publication.

Our recommendation for each stock is closely linked to the **Zacks Rank**, which results from a proprietary quantitative model using trends in earnings estimate revisions. This model is proven most effective for judging the timeliness of a stock over the next 1 to 3 months. The model assigns each stock a rank from 1 through 5. Zacks Rank 1 = Strong Buy. Zacks Rank 2 = Buy. Zacks Rank 3 = Hold. Zacks Rank 4 = Sell. Zacks Rank 5 = Strong Sell. We also provide a **Zacks Industry Rank** for each company which provides an idea of the near-term attractiveness of a company's industry group. We have 264 industry groups in total. Thus, the Zacks Industry Rank is a number between 1 and 264. In terms of investment attractiveness, the higher the rank the better. Historically, the top half of the industries has outperformed the general market. In determining **Risk Level**, we rely on a proprietary quantitative model that divides the entire universe of stocks into five groups, based on each stock's historical price volatility. The first group has stocks with the lowest values and are deemed **Low Risk**, while the 5th group has the highest values and are designated **High Risk**. Designations of **Below-Average Risk, Average Risk, and Above-Average Risk** correspond to the second, third, and fourth groups of stocks, respectively.