

UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**  
**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): July 29, 2014

**VERTEX PHARMACEUTICALS INCORPORATED**

*(Exact name of registrant as specified in its charter)*

**MASSACHUSETTS**

*(State or other jurisdiction of incorporation)*

**000-19319**

*(Commission File Number)*

**04-3039129**

*(IRS Employer Identification No.)*

**50 Northern Avenue**

**Boston, Massachusetts 02210**

*(Address of principal executive offices) (Zip Code)*

**(617) 341-6100**

*(Registrant's telephone number, including area code)*

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**Item 2.02. Results of Operations and Financial Condition.**

On July 29, 2014, we issued a press release in which we reported our consolidated financial results for the quarter ended June 30, 2014. A copy of that press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The information set forth in Exhibit 99.1 shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.****(d) Exhibits**

| <u>Exhibit</u> | <u>Description of Document</u>     |
|----------------|------------------------------------|
| 99.1           | Press Release, dated July 29, 2014 |

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

### **VERTEX PHARMACEUTICALS INCORPORATED**

(Registrant)

Date: July 29, 2014

/s/ Kenneth L. Horton

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Kenneth L. Horton

Executive Vice President and Chief Legal Officer

## **Vertex Reports Second Quarter 2014 Financial Results and Provides Updates on Key Research and Development Programs**

**BOSTON** -- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2014. Vertex reported total second quarter 2014 revenues of \$138 million, including revenues of \$113 million from KALYDECO<sup>®</sup> (ivacaftor). The GAAP net loss for the second quarter of 2014 was \$(159) million, or \$(0.68) per share. The non-GAAP net loss for the second quarter of 2014 was \$(142) million, or \$(0.61) per share. As of June 30, 2014, Vertex had \$1.22 billion in cash, cash equivalents and marketable securities. In July 2014, Vertex entered into a credit agreement that provides for a secured loan of up to \$500 million, \$300 million of which Vertex received in July 2014, which adds to the company's cash balance. Vertex also reiterated its financial guidance for total 2014 non-GAAP revenues, 2014 KALYDECO revenues and non-GAAP operating expenses. The company also provided updates on its key research and development programs in cystic fibrosis (CF).

"As we enter the second half of the year, we continue to make significant progress toward achieving all of our key goals," commented Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Based on data generated throughout the first half of this year, we have increased confidence in our scientific approach to treating the underlying cause of CF and believe that we are on the right path to help the vast majority of people with this disease in the coming years. Importantly, with a strong financial platform, we are today well positioned to invest in our business to create future medicines and grow our revenues as we advance toward profitability."

## **Research and Development Updates**

Vertex today provided the following research and development updates:

### **KALYDECO<sup>®</sup> (ivacaftor)**

#### ***Global Availability of KALYDECO (ivacaftor)***

- KALYDECO is currently available to all eligible patients in the United States, England, Scotland, Northern Ireland, Wales, the Republic of Ireland, France, Germany, the Netherlands, Switzerland, Austria, Denmark, Sweden, Norway, Greece, Italy and Spain. In addition, Vertex signed a letter of intent with the pan-Canadian Pricing Alliance (pCPA) in the second quarter of 2014 to enable the public reimbursement of KALYDECO in Canada. Patients in the Canadian provinces of Ontario and Alberta are now able to receive KALYDECO under public reimbursement, and discussions are ongoing to add KALYDECO to drug programs in the remaining provinces and territories. In Australia, KALYDECO was approved in July 2013, but eligible patients are still not able to receive the medicine through public reimbursement. Vertex is awaiting a response from the Australian government to the company's proposal submitted in May that would allow all Australians with the G551D mutation ages 6 and older to receive KALYDECO and to stay on treatment once started. There are approximately 200 people age 6 years and older who have the G551D mutation in Australia.
- In February 2014, the U.S. Food and Drug Administration (FDA) approved a supplemental New Drug Application (sNDA) for KALYDECO for people with CF ages 6 and older who have one of eight additional mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. The eight additional mutations include: G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P and G1349D. In Canada, Vertex also recently received approval for the use of KALYDECO in people with CF who have these mutations, as well as the G970R mutation. In North America, approximately 150 people ages 6 and older have one of these additional mutations.

#### ***Additional Activities and Clinical Studies Aimed at Increasing the Number of People Eligible for Ivacaftor***

- **Gating Mutations in Europe:** In June, the European Committee for Medicinal Products for Human Use (CHMP) issued a positive opinion recommending the approval of KALYDECO for people with one of the eight additional mutations (G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P and G1349D). The CHMP's positive opinion will now be reviewed by the European Commission, which has the authority to approve medicines for the European Union (E.U.). The European Commission generally follows the recommendation of the CHMP and typically issues marketing

approval within three to four months. In Europe, approximately 250 people ages 6 and older have one of these additional mutations.

- **R117H Mutation:** Based on data from a Phase 3 study, Vertex submitted an sNDA in the U.S. in June and an MAA variation in the E.U. in July for the approval of KALYDECO for use in people ages 18 and older who have the R117H mutation. The Phase 3 study did not meet its primary endpoint of the absolute change from baseline in percent predicted forced expiratory volume in one second (ppFEV<sub>1</sub>), however a pre-specified subset analysis in patients 18 years of age and older showed statistically significant improvements in lung function and other key secondary endpoints. In North America, Europe and Australia, approximately 700 people with CF ages 18 and older have at least one copy of an R117H mutation.
- **Children Ages 2 to 5 with Gating Mutations:** A Phase 3 study of ivacaftor in children with CF ages 2 to 5 who have a gating mutation is complete, and data are expected in the third quarter of 2014 to support a potential NDA submission and MAA variation in the fourth quarter of 2014. The primary endpoint of this study is safety, and secondary endpoints include pharmacokinetics, change in sweat chloride and change in weight. In North America, Europe and Australia, approximately 300 children ages 2 to 5 have the gating mutations evaluated in this study.
- **Residual Function Study:** In June, Vertex announced data from a two-part proof-of-concept study of ivacaftor in 24 people with CF who had a residual function mutation. This study was the first to evaluate the use of ivacaftor in multiple residual function mutations, and based on the data from the study, Vertex plans to initiate a larger Phase 3 study in people with residual function mutations that will evaluate longer-duration treatment, pending discussions with regulatory authorities. In North America, Europe and Australia, more than 3,000 people ages 6 and older have non-R117H mutations that result in residual function.

### **Lumacaftor in Combination with Ivacaftor**

#### ***Planned Regulatory Submissions for People 12 and Older with Two Copies of the F508del Mutation***

- In June, Vertex announced data from the Phase 3 TRAFFIC and TRANSPORT studies evaluating lumacaftor (VX-809) in combination with ivacaftor in people with CF ages 12 and older who have two copies (homozygous) of the F508del mutation. Based on these data, Vertex is on track to submit an NDA and MAA for the combination therapy in people ages 12 and older who have two copies of the F508del mutation in the fourth quarter of 2014. In North America, Europe and Australia, there are more than 22,000 people ages 12 and older who have two copies of the F508del mutation.

## ***Orphan Drug Designation***

- Also in June, the U.S. FDA granted the combination of lumacaftor and ivacaftor Orphan Drug Designation. The FDA grants Orphan Drug Designation to medicines intended to treat fewer than 200,000 people in the U.S. The combination of lumacaftor and ivacaftor also recently received Orphan designation in Europe.

## ***Phase 2 Study in People with One Copy of the F508del Mutation (heterozygous)***

- Vertex today reported results from a Phase 2, 8-week exploratory study of lumacaftor in combination with ivacaftor in 125 people ages 12 and older who have one copy (heterozygous) of the F508del mutation and a second mutation that is not expected to respond to either ivacaftor or VX-809 alone. The study evaluated a twice daily (q12h) combination of VX-809 (400mg) and ivacaftor (250mg) compared to placebo. The primary endpoints were safety, tolerability and mean absolute change in ppFEV<sub>1</sub> from baseline at Day 56, and key secondary endpoints included absolute change in body mass index (BMI), absolute change in patient-reported respiratory symptoms as reported in the CF questionnaire-revised (CFQ-R) and absolute change in sweat chloride, among others.
- In the study, the within-group mean absolute change in ppFEV<sub>1</sub> at day 56 for the patients who received the combination regimen was -0.62 percentage points (p=0.4550) compared to -1.23 percentage points (p=0.1287) for those who received placebo. The mean absolute treatment difference was 0.61 percentage points (p=0.5978) at day 56. The study did not meet its primary efficacy endpoint. For patients who received the combination, the mean absolute improvement in CFQ-R at day 56 was +6.48 points (p=0.0131) versus placebo. Additionally, there was a -11.03 mmol/L (p<0.0001) decrease in sweat chloride at day 56 for those who received the combination compared to those who received placebo. There was no increase observed in body mass index (BMI).
- Safety results from this study were consistent with the Phase 3 TRAFFIC and TRANSPORT studies in people with two copies of the F508del mutation. The combination regimen was generally well tolerated. The most common adverse events, regardless of treatment group, were respiration abnormal, infective pulmonary exacerbation, cough, increased sputum and headache, and adverse events that occurred more frequently in patients who received the combination regimen than those who received placebo were generally respiratory in nature and included dyspnea and respiration abnormal, as well as gastroesophageal reflux. 6.5 percent of patients who received combination therapy discontinued treatment because of adverse events compared to 0.0 percent of those who received placebo.

## **VX-661 in Combination with Ivacaftor**

### ***Ongoing 12-Week Study in People with Two Copies of the F508del Mutation***

- A 12-week Phase 2 study of VX-661 in combination with ivacaftor in people with CF who have two copies of the F508del mutation is currently underway. The study is designed to evaluate safety, efficacy and pharmacokinetics to characterize VX-661 for further clinical development.

### ***Further Development for VX-661***

- Pending data from the ongoing 12-week study of VX-661 and ivacaftor and discussions with regulatory authorities, Vertex plans to evaluate multiple development pathways for VX-661 in combination with ivacaftor, including the potential evaluation of this combination in people with one F508del mutation and one mutation known to respond to ivacaftor and in people with two copies of the F508del mutation. Additionally, VX-661 and ivacaftor may be evaluated in combination with, or without, a next-generation corrector in people with one copy of the F508del mutation and a mutation that is not expected to respond to ivacaftor or a first-generation corrector alone.

## **Next-Generation Corrector Research**

- Vertex has multiple next-generation correctors in the lead-optimization stage of research and expects to begin clinical development of a next-generation corrector in 2015.



## Second Quarter 2014 Non-GAAP Financial Results

The second quarter 2014 non-GAAP financial results exclude stock-based compensation expense, transition and restructuring costs related to the relocation of our corporate headquarters, a one-time cash payment received related to a lease agreement, hepatitis C costs and revenue and other adjustments. The second quarter 2013 non-GAAP financial results exclude stock-based compensation expense, expenses related to Alios (HCV), an inventory charge, certain interest expenses related to the company's convertible senior subordinated notes due 2015 that were converted during the second quarter of 2013 and other adjustments.

**Total Non-GAAP Revenues:** Total non-GAAP revenues for the second quarter of 2014 were \$121.9 million, including \$113.1 million in net product revenues from KALYDECO and \$8.8 million from royalties and collaborative revenues. The components of total non-GAAP revenues for the second quarter of 2014 were:

|                               | Three Months Ended June 30, 2014 |                      |                   |
|-------------------------------|----------------------------------|----------------------|-------------------|
|                               | (in millions)                    |                      |                   |
|                               | GAAP revenues                    | HCV related revenues | Non-GAAP revenues |
| <b>Product revenues</b>       |                                  |                      |                   |
| KALYDECO revenues, net        | \$ 113.1                         | \$ —                 | \$ 113.1          |
| INCIVEK revenues, net         | 9.3                              | (9.3)                | —                 |
| Total product revenues, net   | \$ 122.4                         | \$ (9.3)             | \$ 113.1          |
| <b>Royalty revenues</b>       | 13.0                             | (5.7)                | 7.3               |
| <b>Collaborative revenues</b> | 3.0                              | (1.5)                | 1.5               |
| <b>Total revenues</b>         | <u>\$ 138.4</u>                  | <u>\$ (16.5)</u>     | <u>\$ 121.9</u>   |

- **Net Product Revenues from KALYDECO:** Vertex's second quarter 2014 net product revenues from KALYDECO were \$113.1 million compared to \$99.0 million for the second quarter of 2013. The increased revenues, compared to the second quarter of 2013, resulted primarily from KALYDECO label-expansion in the U.S. In the second half of 2014, further growth and achievement of the company's total 2014 net product revenue guidance for KALYDECO is dependent on completion of reimbursement discussions in Australia for eligible patients with the G551D mutation and on the potential further expansion of the KALYDECO label globally.
- **Collaborative Revenues:** Vertex recognized \$1.5 million in non-GAAP collaborative revenues in the second quarter of 2014. In June, the company entered into a licensing agreement with Janssen Pharmaceuticals, Inc. for the worldwide development and commercialization of VX-787. As part of the agreement, Vertex expects to receive an up-front payment of \$30 million from Janssen in the third quarter. Vertex expects to include all, or a portion of, this payment in its collaborative revenues in the second half of 2014.

**Non-GAAP Operating Expenses:** Total non-GAAP operating expenses for the second quarter of 2014 were \$237.4 million, compared to \$280.7 million for the second quarter of 2013. This reduction was primarily the result of prioritization of investment toward medicines for CF resulting in decreased R&D and SG&A expenses as follows:

- **Research and Development (R&D) Expenses:** Non-GAAP R&D expenses were \$179.5 million for the second quarter of 2014, compared to \$191.2 million in non-GAAP R&D expenses for the second quarter of 2013.
- **Sales, General and Administrative (SG&A) Expenses:** Non-GAAP SG&A expenses were \$57.9 million for the second quarter of 2014, compared to \$89.5 million in non-GAAP SG&A expenses for the second quarter of 2013.

**Non-GAAP Net Income (Loss) Attributable to Vertex:** Vertex's second quarter 2014 non-GAAP net loss was \$(142) million, or \$(0.61) per diluted share, compared to a non-GAAP net loss of \$(6.2) million, or \$(0.03) per diluted share, for the second quarter of 2013. The increased non-GAAP net loss for the second quarter of 2014 was primarily the result of a reduction in INCIVEK net product revenues and the removal of INCIVEK net product revenues from the company's non-GAAP financial results, partially offset by decreased operating expenses.

### **Cash Position at June 30, 2014**

**Cash Position:** As of June 30, 2014, Vertex had \$1.22 billion in cash, cash equivalents and marketable securities compared to \$1.47 billion in cash, cash equivalents and marketable securities as of December 31, 2013. In July 2014, Vertex entered into a credit agreement that provides for a secured loan of up to \$500 million, \$300 million of which Vertex received in July 2014.

### **2014 Financial Guidance**

*This section contains forward-looking guidance about the financial outlook for Vertex Pharmaceuticals.*

- Vertex today reiterated its financial guidance for total 2014 non-GAAP revenues, 2014 KALYDECO revenues and non-GAAP operating expenses:
  - **Total Revenues:** Vertex expects total non-GAAP revenues of \$520 to \$550 million.
    - **KALYDECO Net Revenues:** Vertex expects total 2014 KALYDECO net revenues of \$470 to \$500 million. Achieving this guidance depends on anticipated revenues

from Canada following the recently signed letter of intent to enable public reimbursement of KALYDECO for eligible patients with the G551D mutation, from Australia following the potential completion of reimbursement discussions in the second half of 2014, from Europe following the potential approval of KALYDECO for use in additional gating mutations and in the U.S. from the use of KALYDECO in additional mutations.

- **Non-GAAP Operating Expenses:** Vertex expects 2014 non-GAAP operating expenses to be in the range of \$890 to \$930 million.

### **Non-GAAP Financial Measures**

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial results exclude (i) in 2014, revenue and expenses related to hepatitis C, stock-based compensation expense, transition and restructuring costs related to the relocation of the company's corporate headquarters, a one-time cash payment received related to a lease agreement and other adjustments and (ii) in 2013, stock-based compensation expense, expenses related to Alios (HCV), the impairment of VX-222, an inventory charge, certain interest expenses related to the convertible notes due 2015 and other adjustments. These results are provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding its financial position. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally and to manage the company's business and to evaluate its performance. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

### **Second Quarter 2014 GAAP Financial Results**

**Total Revenues:** Total revenues for the second quarter of 2014 were \$138.4 million compared with \$310.8 million in total revenues for the second quarter of 2013. Second quarter 2014 revenues are comprised primarily of \$113.1 million in KALYDECO net revenues and an aggregate of \$25.3 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues. For the second quarter of 2013, Vertex reported \$99.0 million in net product revenues from KALYDECO and \$211.7 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues.

**Operating Costs and Expenses:** Total operating costs and expenses for the second quarter of 2014 were \$319.3 million, including certain charges of \$81.9 million, compared to \$367.7 million for the second quarter of 2013, including certain charges of \$87.0 million. GAAP operating costs and expenses include:

- **R&D Expenses:** R&D expenses were \$224.8 million for the second quarter of 2014, including \$45.3 million of certain charges, compared to \$222.5 million for the second quarter of 2013, including \$31.3 million of certain charges.
- **Sales, General and Administrative (SG&A) Expenses:** SG&A expenses were \$77.4 million for the second quarter of 2014, including \$19.6 million of certain charges, compared to \$106.5 million for the second quarter of 2013, including \$17.0 million of certain charges.

**Net Loss Attributable to Vertex:** Vertex's second quarter 2014 net loss was \$(159.4) million, or \$(0.68) per share, and includes net charges of \$17.7 million. Vertex's GAAP net loss for the second quarter of 2013 was \$(57.2) million, or \$(0.26) per share, including net charges of \$51.0 million.

**Vertex Pharmaceuticals Incorporated**  
**Second Quarter Results**  
**Condensed Consolidated Statements of Operations Data**  
(in thousands, except per share amounts)  
(unaudited)

|   | Three Months Ended<br>June 30, |                    | Six Months Ended<br>June 30, |                     |
|---|--------------------------------|--------------------|------------------------------|---------------------|
|   | 2014                           | 2013               | 2014                         | 2013                |
| <b>Revenues:</b>  |                                |                    |                              |                     |
| Product revenues, net   | \$ 122,319                     | \$ 254,789         | \$ 225,780                   | \$ 522,170          |
| Royalty revenues  | 13,015                         | 49,120             | 23,748                       | 92,693              |
| Collaborative revenues  | 3,087                          | 6,841              | 7,344                        | 24,255              |
| Total revenues  | <u>138,421</u>                 | <u>310,750</u>     | <u>256,872</u>               | <u>639,118</u>      |
| <b>Costs and expenses:</b>  |                                |                    |                              |                     |
| Cost of product revenues  | 9,655                          | 24,695             | 18,227                       | 55,650              |
| Royalty expenses  | 7,645                          | 13,236             | 14,549                       | 25,024              |
| Research and development expenses (R&D)                               | 224,780                        | 222,455            | 463,743                      | 440,550             |
| Sales, general and administrative expenses (SG&A)                     | 77,446                         | 106,521            | 151,658                      | 199,400             |
| Restructuring expenses  | (270)                          | 776                | 5,918                        | 815                 |
| Intangible asset impairment charge (Note 1)                           | —                              | —                  | —                            | 412,900             |
| Total costs and expenses  | <u>319,256</u>                 | <u>367,683</u>     | <u>654,095</u>               | <u>1,134,339</u>    |
| Loss from operations  | <u>(180,835)</u>               | <u>(56,933)</u>    | <u>(397,223)</u>             | <u>(495,221)</u>    |
| Interest expense, net   | (15,585)                       | (6,551)            | (31,302)                     | (10,016)            |
| Other income (expense), net (Note 2)                                  | 37,731                         | (27)               | 38,182                       | (1,214)             |
| Loss before provision for (benefit from) income taxes                 | <u>(158,689)</u>               | <u>(63,511)</u>    | <u>(390,343)</u>             | <u>(506,451)</u>    |
| Provision for (benefit from) income taxes (Note 1)                    | 693                            | (1,799)            | 1,496                        | (132,112)           |
| Net loss  | <u>(159,382)</u>               | <u>(61,712)</u>    | <u>(391,839)</u>             | <u>(374,339)</u>    |
| Net loss attributable to noncontrolling interest (Note 3)             | —                              | 4,547              | —                            | 9,158               |
| Net loss attributable to Vertex                                       | <u>\$ (159,382)</u>            | <u>\$ (57,165)</u> | <u>\$ (391,839)</u>          | <u>\$ (365,181)</u> |
| <b>Net loss per share attributable to Vertex common shareholders:</b> |                                |                    |                              |                     |
| Basic   | \$ (0.68)                      | \$ (0.26)          | \$ (1.68)                    | \$ (1.67)           |
| Diluted   | \$ (0.68)                      | \$ (0.26)          | \$ (1.68)                    | \$ (1.67)           |
| <b>Shares used in per share calculations:</b>                         |                                |                    |                              |                     |
| Basic   | 233,808                        | 222,053            | 233,353                      | 218,795             |
| Diluted   | 233,808                        | 222,053            | 233,353                      | 218,795             |

## Consolidated Revenues

(in millions)

(unaudited)

|                               | Three Months Ended |                   |                      |                       |                  |
|-------------------------------|--------------------|-------------------|----------------------|-----------------------|------------------|
|                               | June 30,<br>2014   | March 31,<br>2014 | December 31,<br>2013 | September 30,<br>2013 | June 30,<br>2013 |
| <b>Product revenues</b>       |                    |                   |                      |                       |                  |
| KALYDECO revenues, net        | \$ 113.1           | \$ 99.5           | \$ 109.5             | \$ 101.1              | \$ 99.0          |
| INCIVEK revenues, net         | 9.3                | 3.9               | 19.3                 | 85.6                  | 155.8            |
| Total product revenues, net   | 122.4              | 103.5             | 128.8                | 186.7                 | 254.8            |
| <b>Royalty revenues</b>       | 13.0               | 10.7              | 36.9                 | 27.0                  | 49.1             |
| <b>Collaborative revenues</b> | 3.0                | 4.3               | 185.4                | 8.0                   | 6.8              |
| <b>Total revenues</b>         | <b>\$ 138.4</b>    | <b>\$ 118.5</b>   | <b>\$ 351.2</b>      | <b>\$ 221.7</b>       | <b>\$ 310.8</b>  |

**Reconciliation of GAAP to Non-GAAP Financial Information-Second Quarter**  
(in thousands, except per share amounts)  
(unaudited)

**Three Months Ended June 30, 2014**

|   | GAAP         | Adjustments                               |  |                            |                            | Non-GAAP     |
|---|--------------|---|--|----------------------------|----------------------------|--------------|
|   |              | Stock-based compensation expense (Note 4) | Corporate headquarters relocation (Note 5) | HCV related costs (Note 6) | Other adjustments (Note 7) |              |
| Income (loss) from operations   | \$ (180,835) | \$ 42,444                                 | \$ 12,761                                  | \$ (2,323)                 | \$ 1,467                   | \$ (126,486) |
| Other income (expense), net   | 22,146       | —   | (36,685)                                   | —                          | —                          | (14,539)     |
| Income (loss) before provision for (benefit from) incomes taxes                         | (158,689)    | 42,444                                    | (23,924)                                   | (2,323)                    | 1,467                      | (141,025)    |
| Provision for (benefit from) income taxes   | 693          | —   | —  | —                          | —                          | 693          |
| Net income (loss)   | \$ (159,382) | \$ 42,444                                 | \$ (23,924)                                | \$ (2,323)                 | \$ 1,467                   | \$ (141,718) |
| Net income (loss) per diluted share attributable to Vertex common shareholders (Note 8) | \$ (0.68)    |   |  |                            |                            | \$ (0.61)    |

**Three Months Ended June 30, 2013**

|   | GAAP        | Adjustments                               |                            |                            |                            | Non-GAAP   |
|---|-------------|---|----------------------------|----------------------------|----------------------------|------------|
|   |             | Stock-based compensation expense (Note 4) | Alios transaction (Note 3) | HCV related costs (Note 6) | Other adjustments (Note 7) |            |
| Income (loss) from operations   | \$ (56,933) | \$ 41,263                                 | \$ 7,007                   | \$ 5,083                   | \$ 776                     | \$ (2,804) |
| Other income (expense), net   | (6,578)     | —   | (183)                      | —                          | 3,908                      | (2,853)    |
| Income (loss) before provision for (benefit from) incomes taxes                         | (63,511)    | 41,263                                    | 6,824                      | 5,083                      | 4,684                      | (5,657)    |
| Provision for (benefit from) income taxes   | (1,799)     | —   | 2,357                      | —                          | —                          | 558        |
| Net income (loss)   | (61,712)    | 41,263                                    | 4,467                      | 5,083                      | 4,684                      | (6,215)    |
| Net loss (income) attributable to noncontrolling interest (Alios)                       | 4,547       | —   | (4,547)                    | —                          | —                          | —          |
| Net income (loss) attributable to Vertex  | \$ (57,165) | \$ 41,263                                 | \$ (80)                    | \$ 5,083                   | \$ 4,684                   | \$ (6,215) |
| Net income (loss) per diluted share attributable to Vertex common shareholders (Note 8) | \$ (0.26)   |   |                            |                            |                            | \$ (0.03)  |

**Reconciliation of GAAP to Non-GAAP Financial Information-Second Quarter**  
(in thousands)  
(unaudited)

|  | <b>Three Months Ended June 30,</b> |                   |
|--|------------------------------------|-------------------|
|  | <b>2014</b>                        | <b>2013</b>       |
| GAAP total costs and expenses                              | \$ 319,256                         | \$ 367,683        |
| Adjustments:   |                                    |                   |
| Cost of product revenues and royalty expenses              | (17,300)                           | (37,931)          |
| Stock-based compensation expense (Note 4)                  | (42,444)                           | (41,263)          |
| Corporate headquarters relocation (Note 5)                 | (12,761)                           | —                 |
| HCV related costs (Note 6)                                 | (7,889)                            | —                 |
| Alios transaction (Note 3)                                 | —                                  | (7,007)           |
| Other adjustments (Note 7)                                 | (1,467)                            | (776)             |
| <b>Non-GAAP operating costs and expenses</b>               | <b>\$ 237,395</b>                  | <b>\$ 280,706</b> |
| <br>GAAP research and development expenses                 | <br>\$ 224,780                     | <br>\$ 222,455    |
| Adjustments:   |                                    |                   |
| Stock-based compensation expense (Note 4)                  | (27,253)                           | (25,700)          |
| Corporate headquarters relocation (Note 5)                 | (9,382)                            | —                 |
| HCV related costs (Note 6)                                 | (5,049)                            | —                 |
| Alios transaction (Note 3)                                 | —                                  | (5,566)           |
| Other adjustments (Note 7)                                 | (3,584)                            | —                 |
| <b>Non-GAAP research and development expenses</b>          | <b>\$ 179,512</b>                  | <b>\$ 191,189</b> |
| <br>GAAP sales, general and administrative expenses        | <br>\$ 77,446                      | <br>\$ 106,521    |
| Adjustments:   |                                    |                   |
| Stock-based compensation expense (Note 4)                  | (15,191)                           | (15,563)          |
| Corporate headquarters relocation (Note 5)                 | (1,706)                            | —                 |
| HCV related costs (Note 6)                                 | (2,666)                            | —                 |
| Alios transaction (Note 3)                                 | —                                  | (1,441)           |
| <b>Non-GAAP sales, general and administrative expenses</b> | <b>\$ 57,883</b>                   | <b>\$ 89,517</b>  |



**Reconciliation of GAAP to Non-GAAP Financial Information-Six Month**  
(in thousands, except per share amounts)  
(unaudited)

**Six Months Ended June 30, 2014**

|   | GAAP         | Adjustments                               |  |                            |                            | Non-GAAP     |
|---|--------------|---|--|----------------------------|----------------------------|--------------|
|   |              | Stock-based compensation expense (Note 4) | Corporate headquarters relocation (Note 5) | HCV related costs (Note 6) | Other adjustments (Note 7) |              |
| Income (loss) from operations   | \$ (397,223) | \$ 89,024                                 | \$ 32,370                                  | \$ 8,999                   | \$ 5,123                   | \$ (261,707) |
| Other income (expense), net   | 6,880        | —   | (36,685)                                   | —                          | —                          | (29,805)     |
| Income (loss) before provision for (benefit from) incomes taxes                         | (390,343)    | 89,024                                    | (4,315)                                    | 8,999                      | 5,123                      | (291,512)    |
| Provision for (benefit from) income taxes   | 1,496        | —   | —  | —                          | —                          | 1,496        |
| Net income (loss)   | \$ (391,839) | \$ 89,024                                 | \$ (4,315)                                 | \$ 8,999                   | \$ 5,123                   | \$ (293,008) |
| Net income (loss) per diluted share attributable to Vertex common shareholders (Note 8) | \$ (1.68)    |   |  |                            |                            | \$(1.26)     |

**Six Months Ended June 30, 2013**

|   | GAAP         | Adjustments                               |                            |                            |                            | Non-GAAP |
|---|--------------|---|----------------------------|----------------------------|----------------------------|----------|
|   |              | Stock-based compensation expense (Note 4) | Alios transaction (Note 3) | HCV related costs (Note 6) | Other adjustments (Note 7) |          |
| Income (loss) from operations   | \$ (495,221) | \$ 72,416                                 | \$ 12,296                  | \$ 417,983                 | \$ 815                     | \$ 8,289 |
| Other income (expense), net   | (11,230)     | —   | (175)                      | —                          | 3,908                      | (7,497)  |
| Income (loss) before provision for (benefit from) incomes taxes                         | (506,451)    | 72,416                                    | 12,121                     | 417,983                    | 4,723                      | 792      |
| Provision for (benefit from) income taxes   | (132,112)    | —   | 5,783                      | 127,586                    | —                          | 1,257    |
| Net income (loss)   | (374,339)    | 72,416                                    | 6,338                      | 290,397                    | 4,723                      | (465)    |
| Net loss (income) attributable to noncontrolling interest (Alios)                       | 9,158        | —   | (9,158)                    | —                          | —                          | —        |
| Net income (loss) attributable to Vertex  | \$ (365,181) | \$ 72,416                                 | \$ (2,820)                 | \$ 290,397                 | \$ 4,723                   | \$ (465) |
| Net income (loss) per diluted share attributable to Vertex common shareholders (Note 8) | \$ (1.67)    |   |                            |                            |                            | \$(0.00) |

**Reconciliation of GAAP to Non-GAAP Financial Information-Six Month**  
(in thousands)  
(unaudited)

|  | Six Months Ended June 30, |                   |
|--|---------------------------|-------------------|
|  | 2014                      | 2013              |
| GAAP total costs and expenses                              | \$ 654,095                | \$ 1,134,339      |
| Adjustments:   |                           |                   |
| Cost of product revenues and royalty expenses              | (32,776)                  | (80,674)          |
| Stock-based compensation expense (Note 4)                  | (89,024)                  | (72,416)          |
| Corporate headquarters relocation (Note 5)                 | (32,370)                  | —                 |
| HCV related costs (Note 6)                                 | (23,441)                  | —                 |
| Alios transaction (Note 3)                                 | —                         | (12,296)          |
| Other adjustments (Note 7)                                 | (5,123)                   | (413,715)         |
| <b>Non-GAAP operating costs and expenses</b>               | <b>\$ 471,361</b>         | <b>\$ 555,238</b> |
| <br>GAAP research and development expenses                 | <br>463,743               | <br>440,550       |
| Adjustments:   |                           |                   |
| Stock-based compensation expense (Note 4)                  | (60,153)                  | (44,973)          |
| Corporate headquarters relocation (Note 5)                 | (21,583)                  | —                 |
| HCV related costs (Note 6)                                 | (14,046)                  | —                 |
| Alios transaction (Note 3)                                 | —                         | (9,614)           |
| Other adjustments (Note 7)                                 | (6,909)                   | —                 |
| <b>Non-GAAP research and development expenses</b>          | <b>361,052</b>            | <b>385,963</b>    |
| <br>GAAP sales, general and administrative expenses        | <br>\$ 151,658            | <br>\$ 199,400    |
| Adjustments:   |                           |                   |
| Stock-based compensation expense (Note 4)                  | (28,871)                  | (27,443)          |
| Corporate headquarters relocation (Note 5)                 | (3,906)                   | —                 |
| HCV related costs (Note 6)                                 | (8,572)                   | —                 |
| Alios transaction (Note 3)                                 | —                         | (2,682)           |
| <b>Non-GAAP sales, general and administrative expenses</b> | <b>\$ 110,309</b>         | <b>\$ 169,275</b> |

**Condensed Consolidated Balance Sheets Data**  
(in thousands)  
(unaudited)

|   | <u>June 30, 2014</u> | <u>December 31, 2013</u> |
|---|----------------------|--------------------------|
| <b>Assets</b>                                     |                      |                          |
| Cash, cash equivalents and marketable securities  | \$ 1,219,161         | \$ 1,465,076             |
| Accounts receivable, net                          | 81,842               | 85,517                   |
| Inventories                                       | 11,982               | 14,147                   |
| Other current assets                              | 34,399               | 23,836                   |
| Restricted cash                                   | 129                  | 130                      |
| Property and equipment, net                       | 730,000              | 696,911                  |
| Goodwill  | 30,992               | 30,992                   |
| Other non-current assets                          | 9,315                | 2,432                    |
| <b>Total assets</b>                               | <u>\$ 2,117,820</u>  | <u>\$ 2,319,041</u>      |
| <b>Liabilities and Shareholders' Equity</b>       |                      |                          |
| Other liabilities                                 | \$ 388,020           | \$ 422,377               |
| Accrued restructuring expense                     | 18,984               | 28,353                   |
| Deferred revenues                                 | 65,279               | 70,969                   |
| Construction financing lease obligation           | 473,268              | 440,937                  |
| Shareholders' equity                              | 1,172,269            | 1,356,405                |
| <b>Total liabilities and shareholders' equity</b> | <u>\$ 2,117,820</u>  | <u>\$ 2,319,041</u>      |
| Common shares outstanding                         | 237,331              | 233,789                  |

**Note 1:** The company determined that the value of VX-222 had become impaired and that the fair value of VX-222 was zero as of March 31, 2013. This resulted in a \$412.9 million impairment charge in the six months ended June 30, 2013. In connection with this impairment charge, the company recorded a credit of \$127.6 million in its provision for income taxes.

**Note 2:** The company recorded the effect of a one-time cash payment received related to a lease agreement in Other income (expense), net during the second quarter of 2014.

**Note 3:** The company consolidated the financial statements of its collaborator Alios for the three and six months ended June 30, 2013. The company determined that it would no longer consolidate Alios as of December 31, 2013. Each reporting period that Vertex consolidated Alios, the company estimated the fair value of the contingent milestone payments and royalties payable by Vertex to Alios. Any increase in the fair value of these contingent milestone and royalty payments resulted in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis.

**Note 4:** Stock-based compensation expense in the three and six months ended June 30, 2014 includes the effect of the company's full career provision, which was effective for equity grants issued in February 2014, and results in partial or full acceleration of stock compensation expense for qualified grants.

**Note 5:** In the three and six months ended June 30, 2014, "Corporate headquarters relocation" primarily consists of (i) \$11.1 million and \$25.5 million transition costs related to the company's relocation, respectively, (ii) \$1.7 million and \$6.9 million in restructuring charges related to this relocation, respectively, and (iii) a \$36.7 million credit to record the impact of the one-time cash payment received discussed in Note 2 above.

**Note 6:** In the three and six months ended June 30, 2014, "HCV related costs" primarily consists of (i) \$9.3 million and \$13.2 million net product revenues related to INCIVEK, respectively, (ii) \$5.7 million and \$10.6 million royalty revenues related to INCIVO, respectively, and a corresponding amount of royalty expenses, (iii) \$3.6 million and \$11.2 million net charges related to post-restructuring HCV collaborative revenues and development costs, respectively, and (iv) \$2.7 million and \$8.6 million related to the 2014 pharma fee and commercial costs related to INCIVEK, respectively. In the three and six months ended June 30, 2013, HCV related costs consisted of (i) an inventory write-off related to INCIVEK of \$5.1 million recorded in the second quarter of 2013 and (ii) the first quarter of 2013 VX-222 impairment charge, net of tax discussed in Note 1 above.

**Note 7:** In the three and six months ended June 30, 2014, "Other adjustments" consists of (i) development cost associated with VX-509 of \$3.6 million and \$6.9 million, respectively, and (ii) restructuring credits related to a lease obligation of \$2.1 million and \$1.8 million, respectively. In the three and six months ended June 30, 2013, "Other adjustments" consists of (i) \$3.9 million of interest expense related to the 2015 Notes that were converted in the second quarter of 2013 and (ii) restructuring charges related to a lease obligation of \$0.8 million and \$0.8 million, respectively.

**Note 8:** Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 233,808,000 and 222,053,000 for the three months ended June 30, 2014 and 2013, respectively, and 233,353,000 and 218,795,000 for the six months ended June 30, 2014 and 2013, respectively.

## **INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO (ivacaftor)**

Ivacaftor (150 mg tablets) is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the CFTR gene.

In the United States, ivacaftor is also indicated for the treatment of CF in patients age 6 and older who have one of the following mutations in the CFTR gene: G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R. In Canada, ivacaftor is indicated for these same mutations and additionally for G970R.

Ivacaftor is not effective in patients with CF with 2 copies of the F508del mutation (F508del/F508del) in the CFTR gene. The safety and efficacy of ivacaftor in children with CF younger than 6 years of age have not been established.

Elevated liver enzymes (transaminases; ALT and AST) have been reported in patients receiving ivacaftor. It is recommended that ALT and AST be assessed prior to initiating ivacaftor, every 3 months during the first year of treatment, and annually thereafter. Patients who develop increased transaminase levels should be closely monitored until the abnormalities resolve. Dosing should be interrupted in patients with ALT or AST of greater than 5 times the upper limit of normal. Following resolution of transaminase elevations, consider the benefits and risks of resuming ivacaftor dosing.

Use of ivacaftor with medicines that are strong CYP3A inducers, such as the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort, substantially decreases exposure of ivacaftor and may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of ivacaftor must be adjusted when used concomitantly with strong and moderate CYP3A inhibitors or when used in patients with moderate or severe hepatic disease.

Ivacaftor can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with ivacaftor include headache; upper respiratory tract infection (the common cold), including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; and dizziness. These are not all the possible side effects of ivacaftor. A list of the adverse reactions can be found in the product labeling for each country where ivacaftor is approved. Patients should tell their healthcare providers about any side effect that bothers them or does not go away.

Please see KALYDECO U.S. Prescribing Information, EU Summary of Product Characteristics, Canadian Product Monograph, Australian Consumer Medicine Information and Product Information, Swiss Prescribing Information and Patient Information, and the New Zealand Datasheet and Consumer Medicine Information.

## **Indication and Important Safety Information for INCIVEK (telaprevir)**

INCIVEK® (telaprevir) is a prescription medicine used with the medicines peginterferon alfa and ribavirin to treat chronic (lasting a long time) hepatitis C genotype 1 infection in adults with stable liver problems, who have not been treated before or who have failed previous treatment. It is not known if INCIVEK is safe and effective in children under 18 years of age.

### **Important Safety Information**

INCIVEK® (telaprevir) should always be used in combination with peginterferon alfa and ribavirin. INCIVEK combination treatment may cause serious side effects including skin rash and serious skin reactions, anemia (low red blood cell count) that can be severe, and birth defects or death of an unborn baby.

Skin rashes are common with INCIVEK combination treatment. Sometimes these skin rashes and other skin reactions can become serious, require treatment in a hospital, and may lead to death. Patients should call their healthcare provider right away if they develop any skin changes or itching during treatment with INCIVEK. Their healthcare provider will decide if they need treatment or if they need to stop INCIVEK or any of their other medicines. Patients should not stop taking INCIVEK combination treatment without talking with their healthcare provider first.

Patients' healthcare providers will do blood tests regularly to check for anemia. If anemia is severe, the healthcare providers may tell them to stop taking INCIVEK.

INCIVEK combined with peginterferon alfa and ribavirin may cause birth defects or death of an unborn baby. Therefore, a patient should not take INCIVEK combination treatment if she is pregnant or may become pregnant, or if he is a man with a sexual partner who is pregnant. Females who can become pregnant and females whose male partner takes these medicines must have a negative pregnancy test before starting treatment, every month during treatment, and for 6 months after treatment ends. Patients must use two forms of effective birth control during treatment and for 6 months after all treatment has ended. These two forms of birth control should not contain hormones, as these may not work during treatment with INCIVEK.

INCIVEK and other medicines can affect each other and can also cause side effects that can be serious or life-threatening. There are certain medicines patients cannot take with INCIVEK combination treatment. Patients should tell their healthcare providers about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements.

The most common side effects of INCIVEK combination treatment include itching, nausea, diarrhea, vomiting, anal or rectal problems (including hemorrhoids, discomfort, burning or itching around or near the anus), taste changes and tiredness. There are other possible side effects of INCIVEK, and side effects associated with peginterferon alfa and ribavirin also apply to INCIVEK combination treatment. Patients should tell their healthcare provider about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information including Boxed Warning, and the Medication Guide for INCIVEK available at [www.INCIVEK.com](http://www.INCIVEK.com).

## **About Vertex**

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For four years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit [www.vrtx.com](http://www.vrtx.com).

## **Special Note Regarding Forward-looking Statements**

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Leiden's statements in the second paragraph of the press release, the information provided in the section captioned "2014 Financial Guidance," and the information provided regarding (i) the European Commission's review of the CHMP's positive opinion recommending the approval of KALYDECO for people with CF who have one of eight mutations; (ii) Vertex's sNDA in the U.S. and an MAA variation in Europe for people with CF ages 18 and older who have the R117H mutation; (iii) expectations about Vertex's Phase 3 study of ivacaftor in children with CF ages 2 to 5 who have a gating

mutation, and potential regulatory submissions based on the data from this study; (iv) plans to initiate a Phase 3 study in people with residual function mutations; (v) plans to submit regulatory applications for the approval of lumacaftor in combination with ivacaftor in the fourth quarter of 2014; (vi) planned further development of VX-661; and (vii) Vertex's next-generation corrector research program. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2014 revenues and financial results and its 2014 non-GAAP operating expenses may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds, that Vertex could experience unforeseen delays in submitting regulatory filings, that regulatory authorities may not approve, or approve on a timely basis, lumacaftor in combination with ivacaftor due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at [www.vrtx.com](http://www.vrtx.com). Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

### **Conference Call and Webcast**

The company will host a conference call and webcast today at 5:00 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at [www.vrtx.com](http://www.vrtx.com) in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website until August 31, 2014.

(VRTX-GEN)

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