**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): October 23, 2017

# VERTEX PHARMACEUTICALS INCORPORATED

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

| **MASSACHUSETTS** | **000-19319** | **04-3039129** |
|---|---|---|
| *(State or other jurisdiction of incorporation)* | *(Commission File Number)* | *(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):

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company o

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. o

**Item 2.01. Completion or Disposition of Assets.**

As described in Item 2.06 below, we are deconsolidating Parion Sciences, Inc's ("Parion") statements of operations and balance sheet from our consolidated financial statements effective as of September 30, 2017. This deconsolidation is deemed a disposition of assets under applicable guidance. The deconsolidation does not involve a transaction with any other party, and no consideration was given or received. We will file pro-forma financial statements reflecting this deconsolidation on or before October 27, 2017.

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

On October 25, 2017, we issued a press release in which we reported our consolidated financial results for the three and nine months ended September 30, 2017. 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 2.06. Material Impairments.**

On October 23, 2017, we concluded that the intangible asset related to Parion's pulmonary ENaC platform had become fully impaired. This conclusion was based on, among other factors, the results of a Phase 2 clinical trial of VX-371, the lead investigational ENaC inhibitor that we licensed from Parion. We evaluated the fair value of our pulmonary ENaC platform intangible asset from the perspective of a market participant and concluded that the fair value of this asset was zero as of September 30, 2017. Accordingly, a $255.3 million impairment charge and a benefit from income taxes of $97.7 million was recorded in the third quarter of 2017. We do not expect that this impairment charge will result in future cash expenditures. We also determined the licensed asset is no longer the most significant activity of Parion and therefore we are no longer the primary beneficiary of the activities that are most significant to Parion. As a result, we deconsolidated Parion's financial results from our statements of operations from our consolidated financial statements as of September 30, 2017.

**Item 9.01. Financial Statements and Exhibits.**

**(d) Exhibits**

| Exhibit | Description of Document |
|---|---|
| 99.1 | Press Release, dated October 25, 2017 |

**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: October 25, 2017 /s/ Michael J. LaCascia

Michael J. LaCascia
Senior Vice President and General Counsel

# Vertex Reports Third-Quarter 2017 Financial Results

*-Third-quarter 2017 cystic fibrosis product revenues of $550 million, up 34% versus Q3 2016; $336 million for ORKAMBI and $213 million for KALYDECO-*

*-Company increases total 2017 CF product revenue guidance to $2.10 to $2.15 billion; increases ORKAMBI revenue guidance to $1.29 to $1.32 billion and KALYDECO revenue guidance to $810 to $830 million-*

*-Provides update on clinical development programs, including top-line results for three clinical studies in CF: Phase 3 ORKAMBI in ages 2 to 5; Phase 3 tezacaftor/ivacaftor combination in gating mutations; Phase 2 VX-371 + ORKAMBI in F508del homozygous patients-*

**BOSTON** -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2017. Vertex also increased its total 2017 CF product revenue guidance, including revenue guidance for ORKAMBI® (lumacaftor/ivacaftor) and KALYDECO® (ivacaftor), and reiterated its total 2017 combined GAAP and non-GAAP R&D and SG&A expense guidance.

In addition, the company today reported top-line results for three clinical studies in CF, including: a Phase 3 study of ORKAMBI in children with CF ages 2 to 5 who have two copies of the *F508del* mutation; a Phase 3 study of the tezacaftor/ivacaftor combination in people with CF with one copy of the *F508del* mutation and one copy of a gating mutation; and a Phase 2 study of the ENaC inhibitor VX-371 in combination with ORKAMBI in people with CF who have two copies of the *F508del* mutation.

Key financial results include:

| | Three Months Ended September 30, | | % |
|---|---|---|---|
| | **2017** | **2016** | **Change** |
| | (in millions, except per share and percentage data) | | |
| **ORKAMBI product revenues, net** | $ 336 | $ 234 | 44% |
| **KALYDECO product revenues, net** | $ 213 | $ 176 | 22% |
| **TOTAL CF product revenues, net** | $ 550 | $ 410 | 34% |
| | | | |
| **GAAP net loss** | $ (103) | $ (39) | n/a |
| **GAAP net loss per share - diluted** | $ (0.41) | $ (0.16) | n/a |
| | | | |
| **Non-GAAP net income** | $ 136 | $ 43 | 216% |
| **Non-GAAP net income per share - diluted** | $ 0.53 | $ 0.17 | 212% |

"Vertex has never been stronger than it is today with significant progress across all aspects of our business," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We are now treating more patients with our approved medicines than ever before, resulting in significant revenues and

earnings growth. We expect this financial trajectory to continue, driven by our pipeline of transformative CF medicines."

Dr. Leiden continued, "We look forward to continued progress in 2018 with the anticipated approval of our third CF medicine, and advancement into pivotal development of our portfolio of triple combination regimens, which have the potential to treat nearly all CF patients in the future."

**Financial Highlights**

**Revenues:**

- Total CF net product revenues were $549.6 million compared to $409.7 million for the third quarter of 2016.

- Net product revenues from ORKAMBI were $336.2 million compared to $234.0 million for the third quarter of 2016. The increase in ORKAMBI revenues was driven by a number of factors, including the continued uptake in children with CF ages 6 to 11 in the U.S. and the addition of revenues from European countries where ORKAMBI is currently reimbursed.

- Net product revenues from KALYDECO were $213.5 million compared to $175.6 million for the third quarter of 2016. The increase in KALYDECO revenues was driven by the approval and uptake among people ages 2 and older in the U.S. who have certain residual function mutations.

**Expenses:**

- Combined GAAP R&D and SG&A expenses were $575.7 million compared to $378.4 million for the third quarter of 2016. Combined non-GAAP R&D and SG&A expenses were $333.8 million compared to $295.0 million for the third quarter of 2016.

- GAAP R&D expenses were $454.9 million compared to $272.4 million for the third quarter of 2016. The increase in GAAP R&D expenses was primarily due to an upfront payment of $160.0 million related to the acquisition of VX-561 (previously known as CTP-656), an investigational once-daily CFTR potentiator, from Concert Pharmaceuticals. Non-GAAP R&D expenses were $243.2 million compared to $211.0 million for the third quarter of 2016. The increase in non-GAAP R&D expenses was primarily attributable to the clinical development of the company's triple combination regimens for CF.

- GAAP SG&A expenses were $120.7 million compared to $106.1 million for the third quarter of 2016. Non-GAAP SG&A expenses were $90.6 million compared to $84.0 million for the third quarter of 2016. The increase in GAAP and non-GAAP SG&A expenses was driven by the global support for KALYDECO and ORKAMBI.

**Net Income (Loss) Attributable to Vertex:**

- GAAP net loss was $(103.0) million, or $(0.41) per diluted share, for the third quarter of 2017, compared to a net loss of $(38.8) million, or $(0.16) per diluted share, for the third quarter of 2016. The GAAP net loss in the third quarter of 2017 was primarily due to an upfront payment of $160.0 million related to the acquisition of VX-561 from Concert Pharmaceuticals. Non-GAAP net income was $136.4 million, or $0.53 per diluted share, for the third quarter of 2017, compared to $43.1 million, or $0.17 per diluted share, for the third quarter of 2016. Third quarter 2017 non-GAAP net income growth was driven by increased CF product revenues.

**Intangible Asset Impairment:**

- Based upon Phase 2 data evaluating VX-371 in combination with ORKAMBI (reported below), Vertex concluded that the intangible asset had become fully impaired, and also resulted in the deconsolidation of Parion Sciences. This impairment caused a write down of the assets, including the intangible asset, related to Parion, offset by the benefit from income taxes and the reversal of non-controlling interest, which resulted in an increase in GAAP net loss of $7.1 million for the third quarter of 2017 and had no impact on non-GAAP net income.

**Cash Position:**

- As of September 30, 2017, Vertex had $1.81 billion in cash, cash equivalents and marketable securities compared to $1.43 billion in cash, cash equivalents and marketable securities as of December 31, 2016.

**2017 Financial Guidance:**

Vertex today increased its total 2017 CF product revenue guidance, including ORKAMBI and KALYDECO revenue guidance, and reiterated its combined GAAP and non-GAAP R&D and SG&A expense guidance:

- **Total CF Product Revenues:** Vertex now expects total 2017 CF product revenues of $2.10 to $2.15 billion, an increase from its previously announced guidance of $1.87 to $2.10 billion.

- **ORKAMBI:** The company now expects total 2017 product revenues for ORKAMBI of $1.29 to $1.32 billion, an increase from its previously announced guidance of $1.1 to $1.3 billion. The updated guidance reflects the strong underlying demand for ORKAMBI throughout the year among people with CF ages 6 and older in the U.S. and is based on estimates of potential revenues from countries where ORKAMBI is currently reimbursed. This guidance does not assume recognition of any ORKAMBI product revenues from France in 2017.

- **KALYDECO:** The company now expects total 2017 product revenues for KALYDECO of $810 to $830 million, an increase from its previously announced guidance of $770 to $800 million. The updated guidance reflects the rapid uptake among people with CF ages 2 and older in the U.S. who have certain residual function mutations, following recent label expansions for these patients.

- **Combined Non-GAAP and GAAP R&D and SG&A Expenses:** Vertex continues to expect that total 2017 combined GAAP R&D and SG&A expense will be in the range of $1.79 to $1.92 billion and combined non-GAAP R&D and SG&A expense will be in the range of $1.33 to $1.36 billion.

## Clinical Update

Vertex today provided updates on a number of its clinical development programs, including top-line results for three clinical studies in CF:

### KALYDECO

***Label expansion for people ages 2 and older with residual function mutations:*** On August 1, 2017, Vertex announced that the U.S. Food and Drug Administration (FDA) approved KALYDECO for more than 600 people with CF ages 2 and older who have one of five residual function mutations that result in a splicing defect in the *CFTR* gene. This approval followed the FDA's approval of KALYDECO in May 2017 for 23 other residual function mutations.

***Phase 3 study in children under two years of age:*** Vertex today announced that enrollment is complete in the 12 to 24-month age group of the Phase 3 study evaluating the safety of KALYDECO in children under 2 years of age with one of 10 gating and R117H mutations.

**ORKAMBI**

***Phase 3 results in children ages 2 to 5:*** Vertex today announced results from a 2-part, open-label Phase 3 study of ORKAMBI in 60 children ages 2 to 5 with CF who have two copies of the *F508del* mutation. The study met its primary endpoint of safety showing that ORKAMBI was generally well tolerated and there were no new safety concerns compared to prior studies of ORKAMBI in people ages 6 through 11. Secondary endpoints showed decreases in sweat chloride and improvements in nutritional status as measured by change in weight (weight-for-age z score) and body mass index (BMI-for-age z score).

Based on results from this study, Vertex expects to submit a New Drug Application (NDA) to the FDA and a Marketing Authorization Application (MAA) line extension to the European Medicines Agency (EMA) in the first quarter of 2018.

**TEZACAFTOR/IVACAFTOR**

***Regulatory submissions accepted for people ages 12 and older:*** On August 24, 2017, Vertex announced that the FDA granted Priority Review of the NDA for the use of the tezacaftor/ivacaftor combination treatment studied in people with CF ages 12 and older who have two copies of the *F508del* mutation or one F508del mutation and one residual function mutation that is responsive to tezacaftor/ivacaftor and set an action date of February 28, 2018.

Additionally, the EMA has validated the MAA for the tezacaftor/ivacaftor combination, confirming that the submission is complete. The company expects approval in the EU in the second half of 2018.

***Phase 3 results in people with one copy of the F508del mutation and one copy of a gating mutation:*** Vertex announced today top-line results from a Phase 3, randomized, double-blind, parallel-group study evaluating the addition of tezacaftor in people with CF ages 12 and older who were already receiving ivacaftor monotherapy and who have one copy of the *F508del* mutation and one copy of

a gating mutation. The study enrolled 151 CF patients throughout sites in the U.S., Canada, Australia and the EU.

The study did not meet its primary endpoint of absolute change in percent predicted forced expiratory volume in one second ($ppFEV_1$) from baseline through 8 weeks. For those receiving tezacaftor in addition to ivacaftor, $ppFEV_1$ improved 0.5 percentage points compared to 0.2 percentage points in those receiving placebo in addition to ivacaftor (p=0.5846). Safety data from the study showed that the addition of tezacaftor to ivacaftor was generally well tolerated and consistent to prior Phase 3 studies of the tezacaftor/ivacaftor combination. Key secondary endpoints were changes in sweat chloride and change in CFQ-R. Sweat Chloride decreased 5.8 mmol/L in those who received tezacaftor in addition to ivacaftor compared to placebo in addition to ivacaftor (p=0.0216). There was no change in CFQ-R compared to the placebo group.

Based on the results from this study, Vertex does not plan to seek regulatory approval for the tezacaftor/ivacaftor combination in people with CF ages 12 and older with one copy of the *F508del* mutation and one copy of a gating mutation, the vast majority of whom are today eligible for KALYDECO.

**TRIPLE COMBINATION REGIMENS**

Vertex continues to evaluate four different next-generation correctors to be included in an investigational triple combination regimen. The company expects to initiate pivotal development of up to two triple combination regimens in the first half of 2018 pending discussions with regulatory agencies and additional Phase 2 data for VX-152, VX-659 and VX-445, which are expected in early 2018.

Vertex recently amended its Phase 2 studies evaluating VX-659 and VX-445 to add additional cohorts of patients in order to evaluate each of these next-generation correctors in combination with tezacaftor and VX-561 as a potential once-daily triple combination regimen. VX-561 was acquired from Concert Pharmaceuticals in the third quarter of 2017. These additional 4-week study arms will evaluate once-daily triple combination dosing in people with CF who have one copy of the *F508del* mutation and one copy of a mutation that results in minimal CFTR function.

**ENaC**

***Phase 2 study of VX-371 in patients already receiving ORKAMBI:*** Vertex today announced results from a Phase 2, 28-day study of the inhaled epithelial sodium channel (ENaC) inhibitor, VX-371 (P-1037), being developed in collaboration with Parion Sciences. The study primarily evaluated VX-371 + hypertonic saline versus hypertonic saline alone in CF patients who were already receiving ORKAMBI and who continued to receive ORKAMBI throughout the study. The study dosed 142 CF patients ages 12 and older who are homozygous for the *F508del* mutation. The study did not meet its primary efficacy endpoint. In patients being treated with ORKAMBI, the addition of hypertonic saline resulted in a decrease in $ppFEV_1$ of 0.1 percentage points at Day 28. In patients being treated with ORKAMBI, the addition of VX-371 + hypertonic saline resulted in an increase in $ppFEV_1$ of 0.1 percentage points at Day 28. Safety data from the study showed that the addition of VX-371, with or without hypertonic saline, was generally well tolerated in patients already receiving ORKAMBI, and the safety profile was consistent with that observed in prior studies of VX-371 monotherapy. A Phase 2 study of VX-371 monotherapy in patients with primary ciliary dyskinesia (PCD) is ongoing.

**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 and guidance exclude (i) stock-based compensation expense, (ii) revenues and expenses related to business development transactions including collaboration agreements and asset acquisitions, (iii) revenues and expenses related to consolidated variable interest entities, including asset impairment charges and related income tax benefits and the effects of the deconsolidation of a variable interest entity and (iv) 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 the company's 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. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. The company provides guidance regarding product revenues in accordance with GAAP and provides guidance regarding combined research and development and sales, general, and administrative expenses on both a GAAP and a non-GAAP basis. The guidance regarding GAAP research and development expenses and sales, general and administrative expenses does not include estimates regarding expenses associated with any potential future business development activities. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

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

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2017** | **2016** | **2017** | **2016** |
| Revenues: | | | | |
| Product revenues, net | $ 549,642 | $ 409,689 | $ 1,544,252 | $ 1,229,750 |
| Royalty revenues | 2,231 | 3,835 | 6,643 | 12,713 |
| Collaborative revenues (Note 1) | 26,292 | 259 | 286,123 | 1,008 |
| Total revenues | 578,165 | 413,783 | 1,837,018 | 1,243,471 |
| Costs and expenses: | | | | |
| Cost of product revenues | 72,186 | 53,222 | 188,963 | 147,165 |
| Royalty expenses | 688 | 855 | 2,104 | 2,813 |
| Research and development expenses | 454,947 | 272,370 | 1,017,961 | 799,238 |
| Sales, general and administrative expenses | 120,710 | 106,055 | 361,285 | 322,921 |
| Restructuring expenses | 337 | 8 | 13,859 | 1,038 |
| Intangible asset impairment charge (Note 2) | 255,340 | — | 255,340 | — |
| Total costs and expenses | 904,208 | 432,510 | 1,839,512 | 1,273,175 |
| Loss from operations | (326,043) | (18,727) | (2,494) | (29,704) |
| Interest expense, net | (13,574) | (20,140) | (45,003) | (60,993) |
| Other (expenses) income, net (Note 2) | (77,553) | (167) | (80,634) | 3,025 |
| Loss from operations before (benefit from) provision for income taxes (Note 2) | (417,170) | (39,034) | (128,131) | (87,672) |
| (Benefit from) provision for income taxes (Note 2) | (125,903) | 503 | (117,581) | 24,118 |
| Net loss | (291,267) | (39,537) | (10,550) | (111,790) |
| Loss (income) attributable to noncontrolling interest (Note 2) | 188,315 | 696 | 173,350 | (33,207) |
| Net (loss) income attributable to Vertex | $ (102,952) | $ (38,841) | $ 162,800 | $ (144,997) |
| | | | | |
| Amounts per share attributable to Vertex common shareholders: | | | | |
| Net (loss) income: | | | | |
| Basic | $ (0.41) | $ (0.16) | $ 0.66 | $ (0.59) |
| Diluted | $ (0.41) | $ (0.16) | $ 0.64 | $ (0.59) |
| Shares used in per share calculations: | | | | |
| Basic | 250,268 | 244,920 | 247,963 | 244,529 |
| Diluted | 250,268 | 244,920 | 252,095 | 244,529 |

**Reconciliation of GAAP to Non-GAAP Net Income (Loss)**
**Third-Quarter Results**
(in thousands, except per share amounts)
(unaudited)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2017** | **2016** | **2017** | **2016** |
| **GAAP (loss) income attributable to Vertex** | $ (102,952) | $ (38,841) | $ 162,800 | $ (144,997) |
| Stock-based compensation expense | 73,770 | 61,209 | 215,334 | 178,623 |
| Concert upfront and transaction expenses (Note 3) | 160,962 | — | 165,057 | — |
| Revenues and expenses related to VIEs (Note 2) | 7,093 | 1,200 | 14,083 | 59,350 |
| Other collaborative and transaction revenue and expenses (Note 4) | (3,236) | 22,000 | (236,570) | 33,000 |
| Other adjustments (Note 5) | 770 | (2,437) | 16,006 | (2,451) |
| **Non-GAAP net income attributable to Vertex** | $ 136,407 | $ 43,131 | $ 336,710 | $ 123,525 |
| | | | | |
| Amounts per diluted share attributable to Vertex common shareholders: | | | | |
| GAAP | $ (0.41) | $ (0.16) | $ 0.64 | $ (0.59) |
| Non-GAAP | $ 0.53 | $ 0.17 | $ 1.33 | $ 0.50 |
| Shares used in diluted per share calculations: | | | | |
| GAAP | 250,268 | 244,920 | 252,095 | 244,529 |
| Non-GAAP | 255,792 | 248,009 | 252,095 | 247,433 |

**Reconciliation of GAAP to Non-GAAP Revenues and Expenses**
**Third-Quarter Results**
(in thousands)
(unaudited)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2017** | **2016** | **2017** | **2016** |
| **GAAP total revenues** | $ 578,165 | $ 413,783 | $ 1,837,018 | $ 1,243,471 |
| Revenues related to VIEs (Note 2) | (21,082) | (203) | (42,879) | (850) |
| Other collaborative and transaction revenue (Note 4) | (5,209) | — | (243,096) | — |
| Other adjustments (Note 5) | — | (43) | — | (405) |
| **Non-GAAP total revenues** | $ 551,874 | $ 413,537 | $ 1,551,043 | $ 1,242,216 |

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2017** | **2016** | **2017** | **2016** |
| **GAAP cost of product revenues and royalty expenses** | $ 72,874 | $ 54,077 | $ 191,067 | $ 149,978 |
| Other adjustments (Note 5) | — | 16 | — | (117) |
| **Non-GAAP cost of product revenues and royalty expenses** | $ 72,874 | $ 54,093 | $ 191,067 | $ 149,861 |
| | | | | |
| **GAAP research and development expenses** | $ 454,947 | $ 272,370 | $ 1,017,961 | $ 799,238 |
| Stock-based compensation expense | (46,186) | (39,980) | (134,855) | (115,068) |
| Concert upfront payment (Note 3) | (160,000) | — | (160,000) | — |
| Expenses related to VIEs (Note 2) | (3,548) | (1,885) | (6,762) | (3,791) |
| Other collaborative and transaction expenses (Note 4) | (1,865) | (22,000) | (5,684) | (33,000) |
| Other adjustments (Note 5) | (136) | 2,461 | (408) | 3,305 |
| **Non-GAAP research and development expenses** | $ 243,212 | $ 210,966 | $ 710,252 | $ 650,684 |
| | | | | |
| **GAAP sales, general and administrative expenses** | $ 120,710 | $ 106,055 | $ 361,285 | $ 322,921 |
| Stock-based compensation expense | (27,584) | (21,229) | (80,479) | (63,555) |
| Concert transaction expenses (Note 3) | (962) | — | (5,057) | — |
| Expenses related to VIEs (Note 2) | (1,201) | (758) | (3,361) | (2,999) |
| Other collaborative and transaction expenses (Note 4) | (109) | — | (842) | — |
| Other adjustments (Note 5) | (297) | (76) | (1,739) | (106) |
| **Non-GAAP sales, general and administrative expenses** | $ 90,557 | $ 83,992 | $ 269,807 | $ 256,261 |
| | | | | |
| **Combined non-GAAP R&D and SG&A expenses** | $ 333,769 | $ 294,958 | $ 980,059 | $ 906,945 |

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2017** | **2016** | **2017** | **2016** |
| **GAAP interest expense, net and other expense, net** | $ (91,127) | $ (20,307) | $ (125,637) | $ (57,968) |
| Expenses (income) related to VIEs (Note 2) | 76,581 | (36) | 76,507 | 138 |
| **Non-GAAP interest expense, net and other expense, net** | $ (14,546) | $ (20,343) | $ (49,130) | $ (57,830) |
| | | | | |
| **GAAP (benefit from) provision for income taxes** | $ (125,903) | $ 503 | $ (117,581) | $ 24,118 |
| Income taxes related to VIEs (Note 2) | 120,181 | 509 | 111,658 | (20,063) |
| **Non-GAAP (benefit from) provision for income taxes** | $ (5,722) | $ 1,012 | $ (5,923) | $ 4,055 |

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

| | September 30, 2017 | December 31, 2016 |
|---|---|---|
| **Assets** | | |
| Cash, cash equivalents and marketable securities | $ 1,812,248 | $ 1,434,557 |
| Restricted cash and cash equivalents (VIE) (Note 2) | 1,803 | 47,762 |
| Accounts receivable, net | 263,493 | 201,083 |
| Inventories | 98,192 | 77,604 |
| Property and equipment, net | 759,978 | 698,362 |
| Intangible assets and goodwill (Note 2) | 79,384 | 334,724 |
| Other assets | 183,227 | 102,695 |
| **Total assets** | $ 3,198,325 | $ 2,896,787 |
| | | |
| **Liabilities and Shareholders' Equity** | | |
| Accounts payable and accruals | $ 455,692 | $ 376,700 |
| Other liabilities | 381,167 | 260,984 |
| Deferred tax liability (Note 2) | 10,682 | 134,063 |
| Construction financing lease obligation | 547,540 | 486,849 |
| Debt | — | 300,000 |
| Shareholders' equity | 1,803,244 | 1,338,191 |
| **Total liabilities and shareholders' equity** | $ 3,198,325 | $ 2,896,787 |
| | | |
| Common shares outstanding | 252,683 | 248,301 |

**Note 1:** In the nine months ended September 30, 2017, collaborative revenues were primarily attributable to a $230 million up-front payment earned from our collaboration with Merck KGaA, Darmstadt, Germany. During the three and nine months ended September 30, 2017, collaborative revenues also includes $20.0 million and $40.0 million, respectively, that one of the company's consolidated variable interest entities ("VIEs") received from a collaboration agreement with a third party.

**Note 2:** The company consolidated the financial statements of two of its collaborators as VIEs during 2016 and through September 30, 2017. These VIEs were consolidated because Vertex has licensed the rights to develop the company's collaborators' most significant intellectual property assets. The company's interest and obligations with respect to these VIEs' assets and liabilities are limited to those accorded to the company in its collaboration agreements. "Restricted cash and cash equivalents (VIE)" reflects the VIEs' cash and cash equivalents, which Vertex does not have any interest in and which will not be used to fund the collaboration. Each reporting period Vertex estimates the fair value of the contingent payments by Vertex to these collaborators. Any increase in the fair value of these contingent payments results in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis. The fair value of contingent payments is evaluated each quarter and any change in the fair value is reflected in the company's statement of operations.

In the third quarter of 2017, the company determined that the value of Parion's pulmonary ENaC platform had become impaired and that the fair value of the intangible asset was zero as of September 30, 2017. Accordingly, an impairment charge of $255.3 million and a benefit from income taxes of $126.2 million resulting from this charge and subsequent deconsolidation of Parion attributable to noncontrolling interest was recorded in the third quarter of 2017. The total impact of this transaction on a GAAP basis was a $198.7 million loss attributable to noncontrolling interest and a $7.1 million loss attributable to Vertex and had no impact on Vertex's non-GAAP net income in the third quarter of 2017.

As of September 30, 2017, the company has a $29.0 million intangible asset related to its collaboration agreement with BioAxone Biosciences, Inc.

**Note 3:** In July 2017, the company completed the acquisition of VX-561 (formerly CTP-656) from Concert Pharmaceuticals, Inc. The company paid Concert $160.0 million in cash to acquire VX-561, which was recorded as a research and development expense in the three and nine months ended September 30, 2017.

**Note 4:** In the three and nine months ended September 30, 2017, "Other collaboration and transaction revenues and expenses" primarily consisted of revenues and expenses associated with the company's oncology program including the company's collaboration with Merck KGaA, Darmstadt, Germany including the $230 million upfront payment earned pursuant to the collaboration. In the three and nine months ended September 30, 2016, "Other collaboration and transaction revenues and expenses" primarily consisted of collaboration and asset acquisition payments for early-stage research assets. The company has not adjusted its prior year Reconciliation of GAAP to Non-GAAP Revenues and Expenses for the three and nine months ended September 30, 2016 for $5.0 million and $14.9 million, respectively, of operating expenses related to its oncology program.

**Note 5:** In the three and nine months ended September 30, 2017, "Other adjustments" primarily consisted of restructuring charges related to the company's decision to consolidate its research activities into its Boston, Milton Park and San Diego locations and to close our research site in Canada. In the three and nine months ended September 30, 2016, "Other adjustments" primarily consisted of revenues and operating costs and expenses related to HCV as well as restructuring charges related to the company's relocation from Cambridge to Boston, Massachusetts.

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

KALYDECO (ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 2 years and older who have one mutation in their CF gene that is responsive to KALYDECO. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if KALYDECO is safe and effective in children under 2 years of age.

**Patients should not take KALYDECO if they are taking certain medicines or herbal supplements such as:** the antibiotics rifampin or rifabutin; seizure medications such as phenobarbital, carbamazepine, or phenytoin; or St. John's wort.

**Before taking KALYDECO, patients should tell their doctor if they:** have liver or kidney problems; drink grapefruit juice, or eat grapefruit or Seville oranges; are pregnant or plan to become pregnant because it is not known if KALYDECO will harm an unborn baby; and are breastfeeding or planning to breastfeed because is not known if KALYDECO passes into breast milk.

**KALYDECO may affect the way other medicines work, and other medicines may affect how KALYDECO works.** Therefore the dose of KALYDECO may need to be adjusted when taken with certain medications. Patients should especially tell their doctor if they take antifungal medications such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

KALYDECO can cause dizziness in some people who take it. Patients should not drive a car, use machinery, or do anything that needs them to be alert until they know how KALYDECO affects them. Patients should avoid food containing grapefruit or Seville oranges while taking KALYDECO.

**KALYDECO can cause serious side effects including:**

**High liver enzymes in the blood have been reported in patients receiving KALYDECO.** The patient's doctor will do blood tests to check their liver before starting KALYDECO, every 3 months during the first year of taking KALYDECO, and every year while taking KALYDECO. For patients who have had high liver enzymes in the past, the doctor may do blood tests to check the liver more often. Patients should call their doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of their skin or the white part of their eyes; loss of appetite; nausea or vomiting; or dark, amber-colored urine.

Abnormality of the eye lens (cataract) has been noted in some children and adolescents receiving KALYDECO. The patient's doctor should perform eye examinations prior to and during treatment with KALYDECO to look for cataracts. The most common side effects include headache; upper respiratory tract infection (common cold), which includes sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO. **Please click here to see the full Prescribing Information for KALYDECO (ivacaftor).**

**INDICATION AND IMPORTANT SAFETY INFORMATION FOR ORKAMBI® (lumacaftor/ivacaftor) TABLETS**

ORKAMBI is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have two copies of the *F508del* mutation (*F508del/F508del*) in their CFTR gene. ORKAMBI should only be used in these patients. It is not known if ORKAMBI is safe and effective in children under 6 years of age.

**Patients should not take ORKAMBI if they are taking certain medicines or herbal supplements, such as**: the antibiotics rifampin or rifabutin; the seizure medicines phenobarbital, carbamazepine, or phenytoin; the sedatives/anti-anxiety medicines triazolam or midazolam; the immunosuppressant medicines everolimus, sirolimus, or tacrolimus; or St. John's wort.

**Before taking ORKAMBI, patients should tell their doctor if they:** have or have had liver problems; have kidney problems; have had an organ transplant; are using birth control (hormonal contraceptives, including oral, injectable, transdermal or implantable forms). Hormonal contraceptives should not be used as a method of birth control when taking ORKAMBI. Patients should tell their doctor if they are pregnant or plan to become pregnant (it is unknown if ORKAMBI will harm the unborn baby) or if they are breastfeeding or planning to breastfeed (it is unknown if ORKAMBI passes into breast milk).

ORKAMBI may affect the way other medicines work and other medicines may affect how ORKAMBI works. Therefore, the dose of ORKAMBI or other medicines may need to be adjusted when taken together. Patients should especially tell their doctor if they take: antifungal medicines such as ketoconazole, itraconazole, posaconazole, or voriconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

**When taking ORKAMBI, patients should** tell their doctor if they stop ORKAMBI for more than 1 week as the doctor may need to change the dose of ORKAMBI or other medicines the patient is taking. It is unknown if ORKAMBI causes dizziness. Patients should not drive a car, use machinery, or do anything requiring alertness until the patient knows how ORKAMBI affects them.

**ORKAMBI can cause serious side effects including:**

**High liver enzymes in the blood, which can be a sign of liver injury, have been reported in patients receiving ORKAMBI.** The patient's doctor will do blood tests to check their liver before they start ORKAMBI, every three months during the first year of taking ORKAMBI, and annually thereafter. The patient should call the doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of the skin or the white part of the eyes; loss of appetite; nausea or vomiting; dark, amber-colored urine; or confusion.

**Respiratory events such as shortness of breath or chest tightness were observed in patients when starting ORKAMBI.** If a patient has poor lung function, their doctor may monitor them more closely when starting ORKAMBI.

**An increase in blood pressure has been seen in some patients treated with ORKAMBI.** The patient's doctor should monitor their blood pressure during treatment with ORKAMBI.

**Abnormality of the eye lens (cataract) has been noted in some children and adolescents receiving ORKAMBI and ivacaftor, a component of ORKAMBI**. For children and adolescents, the patient's doctor should perform eye examinations prior to and during treatment with ORKAMBI to look for cataracts.

The most common side effects of ORKAMBI include: shortness of breath and/or chest tightness; upper respiratory tract infection (common cold), including sore throat, stuffy or runny nose; gastrointestinal symptoms including nausea, diarrhea, or gas; rash; fatigue; flu or flu-like symptoms; increase in muscle enzyme levels; and irregular, missed, or abnormal menstrual periods and heavier bleeding.

**Please click here to see the full Prescribing Information for ORKAMBI.**

**About Vertex**
Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston's Innovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for eight years in a row.

For additional information and the latest updates from the company, please visit 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 third paragraph of the press release, the information provided in the section captioned "2017 Financial Guidance" and statements regarding (i) the timing and expected outcome of regulatory applications, including NDAs, MAAs and MAA line extensions and (ii) the development plan and timelines for our product development candidates, including tezacaftor in combination with ivacaftor and our next-generation triple combination regimens. 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 2017 revenues and expenses may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that data from the company's development programs may not support registration or further development of its compounds 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. 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 4:30 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 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.

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