

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): November 7, 2017

IONIS PHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in Charter)

Delaware

(State or Other Jurisdiction of Incorporation)

000-19125

(Commission File No.)

33-0336973

(IRS Employer Identification No.)

2855 Gazelle Court
Carlsbad, CA 92010

(Address of Principal Executive Offices and Zip Code)

Registrant's telephone number, including area code: **(760) 931-9200**

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:

- 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))

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

Emerging growth company

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.

Item 2.02 Results of Operations and Financial Condition.

On November 7, 2017, Ionis Pharmaceuticals, Inc. (the “Company”) issued a press release announcing the Company’s financial results for the quarter ended September 30, 2017. In addition to disclosing results that are determined in accordance with Generally Accepted Accounting Principles (GAAP), the Company also discloses pro forma or non-GAAP results of operations, which are adjusted from GAAP to exclude non-cash compensation related to stock awards. The Company is presenting pro forma information excluding non-cash compensation because the Company believes it is useful for investors in assessing the Company’s operating results compared to the prior year. A copy of the release is furnished with this report as an exhibit pursuant to “Item 2.02. Results of Operations and Financial Condition” of Form 8-K in accordance with SEC Release Nos. 33-8216 and 34-47583.

The information in this Current Report on Form 8-K and the Exhibit attached hereto shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, regardless of any general incorporation language in such filing.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1 Press Release dated November 7, 2017.

SIGNATURE

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

IONIS PHARMACEUTICALS, INC.

Dated: November 7, 2017

By: /s/ B. Lynne Parshall

B. LYNNE PARSHALL
Chief Operating Officer

[99.1](#) Press Release dated November 7, 2017.



IONIS PHARMACEUTICALS REPORTS FINANCIAL RESULTS AND HIGHLIGHTS FOR THIRD QUARTER 2017

- CONTINUED IMPROVEMENT IN OPERATING RESULTS WITH YEAR TO DATE 2017 OPERATING INCOME OF \$26 MILLION AND PRO FORMA OPERATING INCOME OF \$90 MILLION
- STRENGTHENED FINANCIAL POSITION WITH CASH OF OVER \$1 BILLION

Conference Call and Webcast Tuesday, November 7, 11:30 a.m. ET at www.ionispharma.com

Carlsbad, Calif., November 7, 2017 – Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today reported GAAP operating income of \$13.9 million and \$26.2 million for the three and nine months ended September 30, 2017, respectively, compared to GAAP operating income of \$16.1 million and a GAAP operating loss of \$87.5 million for the same periods in 2016. Ionis also reported pro forma operating income of \$35.4 million and \$89.9 million for the three and nine months ended September 30, 2017, respectively, compared to pro forma operating income of \$33.7 million and a pro forma operating loss of \$30.5 million for the same periods in 2016. This quarter is Ionis' fifth consecutive quarter of pro forma operating income. The Company ended the third quarter with over \$1 billion in cash. Ionis is on track to meet its 2017 guidance.

"With sales of over \$520 million so far this year, SPINRAZA is on track to be one of the most successful rare disease drug launches in history. Demand for SPINRAZA remains strong in the U.S., with Biogen reporting a 75% increase in the number of patients on therapy in the third quarter compared to the second quarter. Notably, last month Biogen reported it had received hundreds of start forms for patients who were not yet on therapy, further demonstrating strong demand for SPINRAZA. SPINRAZA sales outside the U.S. were \$73 million in the third quarter, surpassing the initial launch in the U.S., in part due to the broad expanded access program Biogen is conducting globally, including in the EU. Biogen is now selling SPINRAZA in 11 European countries plus Japan, and on a named patient basis in the Middle East and Latin America," said B. Lynne Parshall, chief operating officer of Ionis Pharmaceuticals. "SPINRAZA also continues to gain recognition in prestigious publications such as *The New England Journal of Medicine* and *The Lancet*. Importantly, SPINRAZA was recently awarded the esteemed Prix Galien USA Award for the Best Biotechnology Product in 2017, which recognizes extraordinary achievement in scientific innovation that improves the state of human health."

"Inotersen is now under regulatory review in the U.S. and EU for the treatment of hereditary TTR amyloidosis (hATTR). The European Medicines Agency recently granted accelerated assessment to inotersen, which may reduce the standard review time. We are pleased to have met the last 2017 filing deadline for an EU accelerated assessment filing. In the Phase 3 NEURO-TTR study, inotersen-treated patients experienced significant benefit in their quality of life and in measures of neurological disease compared to placebo-treated patients, and 50% of inotersen-treated patients improved in their quality of life from baseline. We believe that the benefit seen with inotersen in the NEURO-TTR study, combined with its superior convenience, could make inotersen the treatment of choice for this patient population. We are making substantial progress in advancing inotersen to the market, and we are in advanced discussions with potential co-commercialization partners. Inotersen has the potential to contribute significantly to our commercial revenue beginning next year."

"Volanesorsen is now under review for marketing authorization in the U.S., EU, and Canada for the treatment of patients with familial chylomicronemia syndrome. Volanesorsen will be reviewed in the U.S. and EU under a standard review and we were pleased that Akcea received priority review in Canada. Akcea is well along in creating a global commercial organization to support a potential launch of volanesorsen next year. We believe volanesorsen also has the potential to contribute significantly to our commercial revenue beginning next year."

"We have also continued to advance our large, diverse pipeline of over 40 drugs. With continued growth in SPINRAZA sales, and inotersen and volanesorsen moving towards the market, we believe we are well on our way to becoming a multi-product, profitable, commercial company, delivering innovative medicines to patients in need," concluded Ms. Parshall.

| | Q1:17* | Q2:17* | Q3:17* |
|-----------------------------------------|-------------------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| SPINRAZA Sales | • \$47M | • \$203M | • \$271M |
| Current Approvals | • U.S. | • U.S., EU, Canada, Japan | • U.S., EU, Canada, Japan, Brazil, Switzerland |
| U.S. Administration Sites | • 88 | • 145 | • 180 |
| U.S. Site Start Submission Forms | • 203 | • 233 | • 250 |
| U.S. Insurance Coverage | <ul style="list-style-type: none"> • 100 commercial plans • 65 Medicaid plans | <ul style="list-style-type: none"> • 80% of commercially insured patients • 65% of Medicaid patients • Most cover all SMA types | <ul style="list-style-type: none"> • 85% of commercially insured patients • 80% of Medicaid patients • Most cover all SMA types |
| Patients in EAP | • 353 in total of which 306 in EU | • 600 in total of which 460 in EU | • 680 in total across 26 countries |
| Current Filings | • EU, Canada, Japan | • Australia, Brazil, Switzerland, Israel, South Korea | • Australia, Israel, South Korea, Argentina |

*As announced by Biogen in their earnings call for the relevant quarter

Financial Results

“We continued our strong financial performance in the third quarter of 2017. We reported pro forma operating income of \$90 million and pro forma net income of \$51 million for the first nine months of this year demonstrating our progress toward sustained profitability. Our results were driven by the more than \$335 million of revenue we earned in the first nine months of this year, including more than \$60 million of commercial revenue from SPINRAZA sales and \$270 million of R&D revenue. As expected, our operating expenses increased for the first nine months of 2017 compared to 2016 primarily due to higher commercialization expenses as we and Akcea prepare to launch inotersen and volanesorsen. Fees we owe under our SPINRAZA in-licensing agreements also contributed to the year over year increase. We received over \$625 million in cash during the first nine months of 2017, including payments from partners and the proceeds from Akcea’s IPO. We ended the quarter with a cash balance of more than \$1 billion,” said Elizabeth L. Hougen, chief financial officer of Ionis Pharmaceuticals.

“As we prepare for the launch of inotersen and Akcea prepares for the launch of volanesorsen, we are projecting an increase in our operating expenses for the fourth quarter of 2017 compared to the third quarter of 2017. Even with this increase in operating expenses, we remain on track to meet our financial guidance for 2017 of pro forma operating income in the mid \$50 million range and more than \$950 million in cash,” concluded Ms. Hougen.

All pro forma amounts referred to in this press release exclude non-cash compensation expense related to equity awards. Please refer to the reconciliation of pro forma and GAAP measures, which is provided later in this release.

Revenue

Ionis' revenue for the three and nine months ended September 30, 2017 was \$120.9 million and \$335.4 million, respectively, compared to \$110.9 million and \$186.3 million for the same periods in 2016. Ionis' revenue in the first nine months of 2017 consisted of the following:

Commercial Revenue:

- \$60 million from SPINRAZA royalties; and
- \$5 million from other licensing and royalty payments.

R&D Revenue:

- \$105 million from Biogen, including \$50 million for the EU approval of SPINRAZA, \$40 million for SPINRAZA pricing approval in Japan and \$15 million for validating two undisclosed neurological disease targets;
- \$67 million from Bayer primarily for the license of IONIS-FXI-L_{RX};
- \$86 million from the amortization of upfront fees; and
- \$12 million primarily from services Ionis performed for its partners.

Operating Expenses

Ionis' operating expenses for the three and nine months ended September 30, 2017 on a GAAP basis were \$107.0 million and \$309.1 million, respectively, and on a pro forma basis were \$85.5 million and \$245.5 million, respectively. These amounts compare to GAAP operating expenses of \$94.8 million and \$273.7 million and pro forma operating expenses of \$77.2 million and \$216.8 million for the same periods in 2016. Ionis' year to date operating expenses increased year over year principally due to higher SG&A expenses as the Company prepares to commercialize volanesorsen and inotersen next year. The Company's SG&A expenses also increased this year compared to last year because of fees it owes under its in-licensing agreements related to SPINRAZA. The Company is projecting an increase in its operating expenses for the fourth quarter of 2017 compared to the third quarter of 2017 primarily due to the cost of preparing for the launch of inotersen and volanesorsen.

Net Income (Loss)

Ionis reported a net loss of \$4.9 million for the three months ended September 30, 2017, compared to net income of \$7.4 million for the same period in 2016, on a GAAP basis. For the nine months ended September 30, 2017 and 2016, Ionis reported a net loss of \$12.6 million and \$112.4 million, respectively, on a GAAP basis. Ionis reported pro forma net income of \$16.6 million and \$51.0 million for the three and nine months ended September 30, 2017, respectively, compared to pro forma net income of \$24.9 million and a pro forma net loss of \$55.5 million for the same periods in 2016. Ionis' GAAP net loss improved for the nine months ended September 30, 2017 compared to the same period in 2016 primarily due to the addition of commercial revenue from SPINRAZA royalties and increased R&D revenue. Ionis also recorded two non-cash, non-recurring items in other expenses, which contributed to the Company's net loss for the three months ended September 30, 2017. These two items were the previously capitalized fair value of the potential premium Ionis would have received from Novartis if Akcea had not completed its IPO and the loss the Company recognized on the purchase of its primary R&D facility. This loss represents the difference between the amount Ionis previously recorded as a financing liability for the leased facility and the purchase price Ionis paid for the facility in July 2017.

Net Loss Attributable to Noncontrolling Interest in Akcea Therapeutics, Inc.

Akcea sold shares of its common stock to third parties in its IPO. From the closing of the IPO through the end of the third quarter of 2017, Ionis owned 68 percent of Akcea. The shares held by third parties represent an interest in Akcea's equity that Ionis does not control. However, because Ionis continues to maintain overall control of Akcea through its voting interest, Ionis reflects the assets, liabilities and results of operations of Akcea in Ionis' consolidated financial statements. Ionis reflects the noncontrolling interest attributable to other holders of Akcea's common stock in a separate line called "Net loss attributable to noncontrolling interest in Akcea" on Ionis' statement of operations. Ionis' net loss attributable to noncontrolling interest in Akcea for the third quarter of 2017 was \$3.9 million. Ionis also added a corresponding account in its stockholders' equity section on its balance sheet called "Noncontrolling interest in Akcea Therapeutics, Inc."

Net Income (Loss) Attributable to Ionis Pharmaceuticals, Inc. Common Stockholders

Ionis reported a net loss attributable to Ionis' common stockholders of \$1.0 million for the three months ended September 30, 2017, compared to net income attributable to Ionis' common stockholders of \$7.4 million for the same period in 2016. For the nine months ended September 30, 2017 and 2016, Ionis reported a net loss attributable to Ionis' common stockholders of \$8.7 million and \$112.4 million, respectively. For the three months ended September 30, 2017, basic and diluted net loss per share were \$0.00. For the three months ended September 30, 2016, basic and diluted net income per share were \$0.06. For the nine months ended September 30, 2017, basic and diluted net income per share were \$0.02. For the nine months ended September 30, 2016, basic and diluted net loss per share were \$0.93.

Balance Sheet

As of September 30, 2017, Ionis had cash, cash equivalents and short-term investments of \$1.0 billion compared to \$665.2 million at December 31, 2016. During the nine months ended September 30, 2017, Ionis received over \$470 million in payments from its partners, primarily from Novartis, Bayer, and Biogen. Additionally, Ionis' cash balance at September 30, 2017 included the proceeds from Akcea's IPO and Novartis' strategic investment in Akcea. Ionis' working capital was \$933.5 million at September 30, 2017 compared to \$664.1 million at December 31, 2016.

Refer to the condensed consolidating statement of operations and balance sheet contained in the financial tables of this earnings release for further information on Ionis' noncontrolling interest in Akcea.

Conference Call

At 11:30 a.m. Eastern Time today, November 7, 2017, Ionis will conduct a live webcast conference call to discuss this earnings release and related activities. Interested parties may listen to the call by dialing 877-443-5662 or access the webcast at www.ionispharma.com. A webcast replay will be available for a limited time at the same address.

ABOUT IONIS PHARMACEUTICALS, INC.

Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over three dozen drugs in development. SPINRAZA® (nusinersen) has been approved in global markets for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. Drugs that have successfully completed Phase 3 studies include inotersen, an antisense drug Ionis is developing to treat patients with hereditary TTR amyloidosis (hATTR), and volanesorsen, an antisense drug discovered by Ionis and co-developed by Ionis and Akcea Therapeutics to treat patients with either familial chylomicronemia syndrome or familial partial lipodystrophy. Akcea, an affiliate of Ionis, is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders. If approved, volanesorsen will be commercialized through Ionis' affiliate, Akcea. Inotersen filings for marketing approval have been submitted in the U.S. and EU. Volanesorsen filings for marketing approval have been submitted in the U.S., EU, and Canada. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis Pharmaceuticals' financial position and outlook, Ionis' business, the business of Akcea Therapeutics, Inc., and the therapeutic and commercial potential of Ionis' technologies and products in development, including SPINRAZA, inotersen and volanesorsen. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended December 31, 2016, and its most recent quarterly report on Form 10-Q, which are on file with the SEC. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, “Ionis,” “Company,” “we,” “our,” and “us” refers to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics™ is a trademark of Ionis Pharmaceuticals, Inc. SPINRAZA® is a registered trademark of Biogen.

Ionis Pharmaceuticals Investor and Media Contacts:

D. Wade Walke, Ph.D.

Vice President, Corporate Communications and Investor Relations

760-603-2741

Alissa Santa Maria

Assistant Director, Corporate Development

760-603-2643

Jennifer Capuzelo

Assistant Director, Corporate Communications and Investor Relations

760-603-2606

Ionis Pharmaceuticals’ Corporate and Drug Development Highlights

(Q3 2017 and subsequent activities)

Recent SPINRAZA Accomplishments:

- Biogen reported more than \$270 million from sales of SPINRAZA in the third quarter, bringing 2017 year-to-date sales to more than \$520 million.
- Ionis earned a \$40 million milestone payment from Biogen for SPINRAZA pricing approval in Japan.
- SPINRAZA was approved in Brazil and Switzerland, with additional approvals anticipated.

- Ionis and Biogen received the prestigious Prix Galien USA Award for the Best Biotechnology Product in 2017 for SPINRAZA.
- Results from the ENDEAR study of SPINRAZA in patients with infantile-onset SMA were published in *The New England Journal of Medicine*.
- Ionis and Biogen received the 2016-2017 Oligonucleotide Therapeutics Society Paper of the Year Award for the Phase 2 SPINRAZA publication in *The Lancet*.

Recent Corporate and Pipeline Accomplishments:

- Ionis filed for marketing authorization for inotersen in the U.S. and EU.
- Ionis presented data from the NEURO-TTR study of inotersen at the American Neurology Academy congress and European ATTR meeting demonstrating benefit compared to placebo in multiple measures of quality of life and disease severity, including both co-primary endpoints: the Norfolk quality of life score and mNIS+7.
- Ionis entered into a collaboration with Seventh Sense Biosystems to support the development of a novel push-button blood collection device to make blood testing more convenient and virtually painless, potentially enabling more convenient monitoring for patients being treated with inotersen and volanesorsen.
- AstraZeneca presented data from the Phase 1b/2 study of IONIS-STAT3-2.5_{Rx} in combination with its PD-L1 blocking antibody, Imfinzi (durvalumab) showing encouraging antitumor activity in patients with advanced solid tumors and recurrent metastatic head and neck cancer, with a safety and tolerability profile supporting continued development.
- Ionis initiated a Phase 1 study of IONIS-MAPT_{Rx} in patients with Alzheimer's disease and earned a \$10 million milestone payment from Biogen.

Recent Akcea Accomplishments:

- Akcea and Ionis filed for marketing authorization in the U.S., EU, and Canada for volanesorsen for the treatment of FCS.
- Volanesorsen was granted Priority Review in Canada.
- Akcea received Promising Innovative Medicine (PIM) designation for volanesorsen in the United Kingdom.
- Akcea announced positive results from the Phase 1/2 study of AKCEA-APOCIII-L_{Rx} in healthy volunteers and patients with elevated triglycerides.
- Akcea presented final results from the IN-FOCUS study demonstrating the significant burden of illness for patients with FCS at the National Organization for Rare Disorders (NORD) Summit.
- Akcea completed its initial public offering, including the underwriters' full exercise of their overallotment option generating over \$180 million in net proceeds.
- Akcea expanded the leadership team in the U.S. and established a global presence in Canada, United Kingdom, Germany, and France.

IONIS PHARMACEUTICALS, INC.
SELECTED FINANCIAL INFORMATION
Condensed Consolidated Statements of Operations
(In Thousands, Except Per Share Data)

| | Three months ended, September 30, | | Nine months ended, September 30, | |
|-----------------------------------------------------------------------------------|--------------------------------------|----------|-------------------------------------|--------------|
| | 2017 | 2016 | 2017 | 2016 |
| | (unaudited) | | (unaudited) | |
| Revenue: | | | | |
| Commercial revenue: | | | | |
| SPINRAZA royalties | \$ 32,890 | \$ - | \$ 60,467 | \$ - |
| Licensing and royalty revenue | 879 | 2,014 | 4,983 | 19,689 |
| Total commercial revenue | 33,769 | 2,014 | 65,450 | 19,689 |
| Research and development revenue under collaborative agreements | 87,142 | 108,913 | 269,917 | 166,583 |
| Total revenue | 120,911 | 110,927 | 335,367 | 186,272 |
| Expenses: | | | | |
| Research, development and patent expenses | 80,214 | 84,631 | 246,358 | 243,169 |
| Selling, general and administrative | 26,788 | 10,188 | 62,782 | 30,574 |
| Total operating expenses | 107,002 | 94,819 | 309,140 | 273,743 |
| Income (loss) from operations | 13,909 | 16,108 | 26,227 | (87,471) |
| Other income (expense): | | | | |
| Investment income | 2,811 | 989 | 7,504 | 3,912 |
| Interest expense | (10,825) | (9,746) | (33,966) | (28,861) |
| Loss on extinguishment of financing liability for leased facility | (7,689) | - | (7,689) | - |
| Other expenses | (2,141) | - | (3,528) | - |
| Income (loss) before income tax expense | (3,935) | 7,351 | (11,452) | (112,420) |
| Income tax expense | (961) | - | (1,184) | (1) |
| Net income (loss) | (4,896) | 7,351 | (12,636) | (112,421) |
| Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc. | 3,920 | - | 3,920 | - |
| Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders | \$ (976) | \$ 7,351 | \$ (8,716) | \$ (112,421) |
| Basic net income (loss) per share | \$ (0.00) | \$ 0.06 | \$ 0.02 | \$ (0.93) |
| Diluted net income (loss) per share | \$ (0.00) | \$ 0.06 | \$ 0.02 | \$ (0.93) |
| Shares used in computing basic net income (loss) per share | 124,370 | 120,989 | 123,746 | 120,795 |
| Shares used in computing diluted net income (loss) per share | 124,370 | 123,378 | 123,746 | 120,795 |

IONIS PHARMACEUTICALS, INC.
Reconciliation of GAAP to Pro Forma Basis:
Condensed Consolidated Operating Expenses, Income (Loss) From Operations, and Net Income (Loss)
(In Thousands)

| | Three months ended, September 30, | | Nine months ended, September 30, | |
|--------------------------------------------------------------------|--------------------------------------|------------------|-------------------------------------|--------------------|
| | 2017 | 2016 | 2017 | 2016 |
| | (unaudited) | | (unaudited) | |
| As reported operating expenses according to GAAP | \$ 107,002 | \$ 94,819 | \$ 309,140 | \$ 273,743 |
| Excluding compensation expense related to equity awards | (21,472) | (17,586) | (63,642) | (56,950) |
| Pro forma operating expenses | <u>\$ 85,530</u> | <u>\$ 77,233</u> | <u>\$ 245,498</u> | <u>\$ 216,793</u> |
| As reported income (loss) from operations according to GAAP | \$ 13,909 | \$ 16,108 | \$ 26,227 | \$ (87,471) |
| Excluding compensation expense related to equity awards | (21,472) | (17,586) | (63,642) | (56,950) |
| Pro forma income (loss) from operations | <u>\$ 35,381</u> | <u>\$ 33,694</u> | <u>\$ 89,869</u> | <u>\$ (30,521)</u> |
| As reported net income (loss) according to GAAP | \$ (4,896) | \$ 7,351 | \$ (12,636) | \$ (112,421) |
| Excluding compensation expense related to equity awards | (21,472) | (17,586) | (63,642) | (56,950) |
| Pro forma net income (loss) | <u>\$ 16,576</u> | <u>\$ 24,937</u> | <u>\$ 51,006</u> | <u>\$ (55,471)</u> |

Reconciliation of GAAP to Pro Forma Basis

As illustrated in the Selected Financial Information in this press release, pro forma operating expenses, pro forma income (loss) from operations, and pro forma net income (loss) were adjusted from GAAP to exclude compensation expense related to equity awards, which are non-cash. Ionis has regularly reported non-GAAP measures for operating results as pro forma results. These measures are provided as supplementary information and are not a substitute for financial measures calculated in accordance with GAAP. Ionis reports these pro forma results to better enable financial statement users to assess and compare its historical performance and project its future operating results and cash flows. Further, the presentation of Ionis' pro forma results is consistent with how Ionis' management internally evaluates the performance of its operations.

IONIS PHARMACEUTICALS, INC.
Condensed Consolidated Balance Sheets
(In Thousands)

| | September 30, 2017 <u>(unaudited)</u> | December 31, 2016 <u></u> |
|-----------------------------------------------------|---------------------------------------------|---------------------------------|
| Assets: | | |
| Cash, cash equivalents and short-term investments | \$ 1,010,808 | \$ 665,223 |
| Contracts receivable | 42,924 | 108,043 |
| Other current assets | 57,381 | 22,252 |
| Property, plant and equipment, net | 116,624 | 92,845 |
| Other assets | 24,824 | 24,104 |
| Total assets | <u>\$ 1,252,561</u> | <u>\$ 912,467</u> |
| Liabilities and stockholders' equity: | | |
| Other current liabilities | \$ 72,734 | \$ 82,504 |
| Current portion of deferred contract revenue | 104,913 | 51,280 |
| 1% convertible senior notes | 524,744 | 500,511 |
| Long-term obligations, less current portion | 72,846 | 87,409 |
| Long-term deferred contract revenue | 79,656 | 91,198 |
| Total Ionis stockholders' equity | 307,968 | 99,565 |
| Noncontrolling interest in Akcea Therapeutics, Inc. | 89,700 | - |
| Total stockholders' equity | <u>397,668</u> | <u>99,565</u> |
| Total liabilities and stockholders' equity | <u>\$ 1,252,561</u> | <u>\$ 912,467</u> |

IONIS PHARMACEUTICALS, INC.
SELECTED FINANCIAL INFORMATION
Condensed Consolidating Statement of Operations
(In Thousands)

Nine months ended,
September 30, 2017
(unaudited)

| | Ionis | Akcea | Eliminations | Ionis Consolidated |
|-----------------------------------------------------------------------------------|-----------|-------------|--------------|-----------------------|
| Revenue: | | | | |
| Commercial revenue: | | | | |
| SPINRAZA royalties | \$ 60,467 | \$ - | \$ - | \$ 60,467 |
| Licensing and royalty revenue | 4,983 | - | - | 4,983 |
| Total commercial revenue | 65,450 | - | - | 65,450 |
| Research and development revenue under collaborative agreements | 232,744 | \$ 37,173 | - | 269,917 |
| Intercompany revenue | 54,407 | - | (54,407) | - |
| Total revenue | 352,601 | 37,173 | (54,407) | 335,367 |
| Expenses: | | | | |
| Research, development and patent expenses | 199,934 | 100,921 | (54,497) | 246,358 |
| Selling, general and administrative | 42,819 | 19,963 | - | 62,782 |
| Total operating expenses | 242,753 | 120,884 | (54,497) | 309,140 |
| Income (loss) from operations | 109,848 | (83,711) | 90 | 26,227 |
| Other income (expense): | | | | |
| Investment income | 8,241 | 994 | (1,731) | 7,504 |
| Interest expense | (33,966) | (1,731) | 1,731 | (33,966) |
| Loss on extinguishment of financing liability for leased facility | (7,689) | - | - | (7,689) |
| Other expenses | (3,652) | 124 | - | (3,528) |
| Income (loss) before income tax expense | 72,782 | (84,324) | 90 | (11,452) |
| Income tax benefit (expense) | 882 | (2,066) | - | (1,184) |
| Net income (loss) | 73,664 | (86,390) | 90 | (12,636) |
| Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc. | - | - | 3,920 | 3,920 |
| Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders | \$ 73,664 | \$ (86,390) | \$ 4,010 | \$ (8,716) |

IONIS PHARMACEUTICALS, INC.
Condensed Consolidating Balance Sheet
(In Thousands)

September 30, 2017
(unaudited)

| | Ionis | Akcea | Eliminations | Ionis Consolidated |
|-----------------------------------------------------------|---------------------|-------------------|---------------------|-----------------------|
| Assets: | | | | |
| Cash, cash equivalents and short-term investments | \$ 724,677 | \$ 286,131 | \$ - | \$ 1,010,808 |
| Contracts receivable | 42,424 | 500 | - | 42,924 |
| Receivable from Akcea Therapeutics, Inc. | 9,501 | - | (9,501) | - |
| Other current assets | 56,132 | 1,249 | - | 57,381 |
| Property, plant and equipment, net | 116,520 | 104 | - | 116,624 |
| Other assets | 297,733 | 1,365 | (274,274) | 24,824 |
| Total assets | \$ 1,246,987 | \$ 289,349 | \$ (283,775) | \$ 1,252,561 |
| Liabilities and stockholders' equity: | | | | |
| Other current liabilities | \$ 62,822 | \$ 19,413 | \$ (9,501) | \$ 72,734 |
| Current portion of deferred contract revenue | 50,870 | 54,043 | - | 104,913 |
| 1% convertible senior notes | 524,744 | - | - | 524,744 |
| Long-term obligations, less current portion | 72,832 | 14 | - | 72,846 |
| Long-term deferred contract revenue | 63,319 | 18,035 | (1,698) | 79,656 |
| Total stockholders' equity before noncontrolling interest | 472,400 | 197,844 | (362,276) | 307,968 |
| Noncontrolling interest in Akcea Therapeutics, Inc. | - | - | 89,700 | 89,700 |
| Total stockholders' equity | 472,400 | 197,844 | (272,576) | 397,668 |
| Total liabilities and stockholders' equity | \$ 1,246,987 | \$ 289,349 | \$ (283,775) | \$ 1,252,561 |

###