

**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 6, 2018

**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 6, 2018, Ionis Pharmaceuticals, Inc. (the “Company”) issued a press release announcing the Company’s financial results for the quarter ended September 30, 2018. 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 6, 2018.

---

**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 6, 2018

By: /s/ Patrick R. O'Neil

---

**PATRICK R. O'NEIL**

Senior Vice President, Legal, General Counsel and Chief Compliance Officer

---

[99.1](#) Press Release dated November 6, 2018.

---



### **Ionis Reports Third Quarter 2018 Financial Results**

*Year-to-date revenues increased more than 15% compared to 2017, driven by increased SPINRAZA revenue*

*TEGSEDI launch underway in multiple markets*

*Conference call and webcast today, November 6, 2018, at 11:30 a.m. Eastern Time*

**CARLSBAD, Calif., November 6, 2018** – Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported financial results for the third quarter of 2018 and highlighted its recent business and pipeline successes.

“We enter the fourth quarter in a position of financial strength, driven by strong worldwide SPINRAZA sales. In addition, we achieved numerous successes during the quarter that advanced our pipeline, technology and business and contributed to our financial strength. In 2019 and beyond, we are positioned for continued growth bolstered by the addition of TEGSEDI commercial revenue,” said Stanley T. Croke, M.D., Ph.D., chairman of the board and chief executive officer of Ionis. “Beyond our commercial-stage drugs, we have a broad and growing pipeline of innovative programs we are advancing toward the market. We continue to advance our antisense technology, expanding its application to more diseases, both rare and common. An example of this is our LICA technology, which was further validated by the results from the Phase 2 study of AKCEA-APO(a)-LR<sub>x</sub> – our largest and longest study to date with a LICA drug – which demonstrated significant reductions in Lp(a) and an attractive safety profile. We also continue to expand our Ionis-owned pipeline and our existing relationships with partners. The successes we are achieving with our commercial-stage drugs and in advancing our pipeline have increased our financial strength and our ability to deliver innovative new drugs to patients in need.”

#### **Third Quarter 2018 Financial Highlights**

- *Revenues increased year-to-date by more than 15 percent compared to 2017*
    - o Total revenue was \$145 million and \$408 million for the third quarter and year-to-date 2018, respectively, compared to \$118 million and \$346 million for the same periods in 2017
    - o Commercial revenue from SPINRAZA for year-to-date 2018 was \$168 million, a nearly three-fold increase over year-to-date 2017
    - o Commercial revenue was 45 percent of Ionis’ total revenue in the first nine months of 2018 compared to less than 20 percent for the same period in 2017, reflecting Ionis’ transition to a commercial-stage company
-

- *On track for third consecutive year of pro forma operating profitability while investing in the launches of two drugs*
  - o GAAP operating results were a loss of \$19 million and \$72 million for the third quarter and year-to-date 2018, respectively, compared to operating income of \$11 million and \$37 million for the same periods in 2017
  - o Pro forma operating income was \$16 million and \$25 million for the third quarter and year-to-date 2018, respectively, compared to \$33 million and \$101 million for the same periods in 2017
  - o Operating expenses increased primarily due to higher SG&A expenses related to preparing to launch TEGSEDI and WAYLIVRA
- *Substantial cash position of \$2 billion enabling investment in commercial products and pipeline*
  - o The increase in Ionis' cash position was primarily due to the \$1 billion Ionis received from Biogen for the 2018 strategic neurology collaboration

“Our strong third quarter financial results put us on track for our third consecutive year of pro forma operating income. In the fourth quarter, we are excited to add TEGSEDI commercial sales to our growing SPINRAZA revenue and substantial base of R&D revenue, positioning us for significant revenue growth going forward,” said Elizabeth L. Hougen, chief financial officer of Ionis. “Importantly, we are in a strong financial position with sustainable profitability and \$2 billion in cash even as we support the launch of TEGSEDI in multiple markets and prepare for the launch of WAYLIVRA. Further, our financial strength coupled with our business strategy provides us with the flexibility to maximize the value of our robust antisense technology platform and innovative pipeline of drugs.”

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. Additionally, Ionis has labeled its prior period financial statements “as revised” to reflect the new revenue recognition accounting standard the Company adopted on January 1, 2018.

### **Business Highlights**

- *SPINRAZA – the first and only approved treatment for people with spinal muscular atrophy*
    - o SPINRAZA sales continued to grow in the third quarter, both in the U.S. and ex-U.S., with global sales of more than \$1 billion for year-to-date 2018, as reported by Biogen.
    - o Nearly 6,000 SMA patients were on SPINRAZA as of the third quarter.
    - o In the U.S., the number of adult patients on therapy grew by over 20 percent compared to the second quarter. Adult SMA patients, which represent the largest and most undertreated patient segment, accounted for more than 50 percent of start forms in the third quarter.
    - o Access outside the U.S. expanded with formal reimbursement in 28 markets and continued revenue growth in the EU, Asia Pacific and Latin America.
-

- *TEGSEDI™ (inotersen) – launched in multiple markets for the treatment of polyneuropathy of hereditary transthyretin amyloidosis (hATTR) in adult patients*
  - o TEGSEDI approved in the U.S., EU and Canada
  - o Commercial patients in Germany on TEGSEDI
  - o TEGSEDI prescriptions received in the U.S.
- *WAYLIVRA™ (volanesorsen) – under regulatory review for the treatment of people living with FCS*
  - o Preparing for launch in the EU following approval
  - o Planning to confirm a path forward in the U.S. and Canada

### **Pipeline and Business Progress**

- Ionis and Akcea reported positive top-line data from a Phase 2 study of AKCEA-APO(a)-L<sub>Rx</sub> in people with high levels of Lp(a) and established cardiovascular disease demonstrating robust target reductions and a favorable safety and tolerability profile
- Ionis and Roche entered a new collaboration to develop IONIS-FB-L<sub>Rx</sub> for the treatment of people with complement-mediated diseases. Ionis received a \$75 million upfront payment and will be eligible for development, regulatory and sales milestone payments and license fees of up to \$684 million plus royalties of up to 20 percent on commercial sales
- Positive Phase 1b/2 data for danvatirsen (IONIS-STAT3-2.5<sub>Rx</sub>) in combination with durvalumab were presented at the European Society for Medical Oncology (ESMO) 2018 Congress, demonstrating a response rate approximately double that of durvalumab alone, based on previous studies in patients with refractory head and neck cancer. Ionis earned a \$17.5 million milestone payment because AstraZeneca is advancing the program
- Ionis completed enrollment in a Phase 2b study of IONIS-FXI<sub>Rx</sub> in patients with end-stage renal disease on dialysis, with data planned for mid-2019
- Ionis or its partners initiated clinical studies with IONIS-GHR-L<sub>Rx</sub> (Phase 2), IONIS-C9<sub>Rx</sub> (Phase 1/2), IONIS-FXI-L<sub>Rx</sub> and IONIS-AZ4-2.5-L<sub>Rx</sub> (Phase 1)
- Ionis earned a \$10 million milestone payment from AstraZeneca for advancing an undisclosed oncology program into development
- Ionis appointed Dr. Michael Hayden and Mr. Peter N. Reikes to its Board of Directors

### **Key Upcoming Events**

- Potential approval and launch of WAYLIVRA in the EU
  - Pivotal program initiation for IONIS-HTT<sub>Rx</sub> in patients with Huntington's disease
  - Results from up to three Phase 2 studies and four Phase 1 studies
  - Initiations of up to three Phase 2 studies and two Phase 1 studies
-

## Revenue

Ionis' revenue in the three and nine months ended September 30, 2018 was \$145 million and \$408 million, respectively, compared to \$118 million and \$346 million for the same periods in 2017 and was comprised of the following (amounts in millions):

	Three months ended, September 30,		Nine months ended September 30,	
	2018	2017 (as revised)	2018	2017 (as revised)
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$ 70	\$ 33	\$ 168	\$ 60
Licensing and royalty revenue	13	2	14	6
Total commercial revenue	83	35	182	66
R&D Revenue:				
Amortization from upfront payments	31	23	92	70
Milestone payments	26	56	45	135
License fees	1	-	64	65
Other services	4	4	25	10
Total R&D revenue	62	83	226	280
Total revenue	\$ 145	\$ 118	\$ 408	\$ 346

The increase in revenue in the first nine months of 2018 compared to the same period in 2017 was primarily due to increasing commercial revenue from SPINRAZA royalties, which increased over 175 percent. Our R&D revenue for the first nine months of 2018 was significant and demonstrates our ability to generate sustainable revenue from our numerous partnerships.

R&D revenue from the amortization of upfront payments increased by \$22 million in the first nine months of 2018, compared to the same period in 2017 primarily due to Ionis' 2018 strategic neurology collaboration with Biogen, which started in the second quarter of 2018. In the fourth quarter of 2018, Ionis will add amortization revenue from its new collaboration with Roche to develop IONIS-FB-L<sub>Rx</sub>.

Ionis' 2018 R&D revenue from milestone payments was bolstered by two \$10 million milestone payments in the third quarter, one from Biogen and one from AstraZeneca, as Ionis' partnered programs advanced. In the same period of 2017, R&D revenue from milestone payments included \$90 million of milestone payments from Biogen for SPINRAZA approval in the EU and Japan. Already in the fourth quarter, Ionis has earned nearly \$30 million in milestone payments from AstraZeneca.

## **Operating Expenses**

Operating expenses for the three and nine months ended September 30, 2018 on a GAAP basis were \$164 million and \$480 million, respectively, and on a pro forma basis were \$129 million and \$383 million, respectively. These amounts compare to GAAP operating expenses for the three and nine months ended September 30, 2017 of \$107 million and \$309 million, respectively, and pro forma operating expenses of \$86 million and \$246 million, respectively. The increases in operating expenses were principally due to higher SG&A expenses as Akcea, Ionis' affiliate, prepared to commercialize TEGSEDI and WAYLIVRA. The Company's SG&A expenses also increased due to an increase in fees the Company owed under its in-licensing agreements related to SPINRAZA, as a result of increased SPINRAZA product sales.

## **Net Income (Loss) Attributable to Ionis Common Stockholders**

Ionis reported a net loss attributable to Ionis' common stockholders of \$5 million and \$46 million for the three and nine months ended September 30, 2018, respectively, compared to a net loss of \$3 million and net income of \$3 million for the same periods in 2017, all on a GAAP basis. On a pro forma basis, Ionis reported net income attributable to Ionis' common stockholders of \$30 million and \$51 million for the three and nine months ended September 30, 2018, respectively, compared \$19 million and \$67 million for the same periods in 2017.

At September 30, 2018, Ionis owned approximately 75 percent of Akcea. The shares of Akcea third parties own 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 owners 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 three and nine months ended September 30, 2018, was \$16 million and \$41 million, respectively. Ionis' net loss attributable to noncontrolling interest in Akcea for the three and nine months ended September 30, 2017, was \$5 million.

For the three months ended September 30, 2018 and 2017, basic and diluted net loss per share were \$0.03 and \$0.02, respectively. For the nine months ended September 30, 2018, basic and diluted net loss per share were each \$0.33. For the nine months ended September 30, 2017, basic and diluted net income per share were each \$0.13. All amounts are on a GAAP basis.

## **Balance Sheet**

As of September 30, 2018, Ionis had cash, cash equivalents and short-term investments of \$2 billion compared to \$1 billion at December 31, 2017. The increase in Ionis' cash, cash equivalents and short-term investments was primarily due to the \$1 billion Ionis received from Biogen for the 2018 strategic neurology collaboration.

---

## **Webcast and Conference Call**

Today, at 11:30 a.m. Eastern Time, 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](http://www.ionispharma.com). A webcast replay will be available for a limited time.

## **About Ionis Pharmaceuticals, Inc.**

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, proprietary antisense technology platform with the potential to treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs – such as children and adults with spinal muscular atrophy (SMA). We created SPINRAZA® (nusinersen)\* and are proud to have brought new hope to the SMA community by developing the first and only approved treatment for this disease.

Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 drugs with the potential to treat patients with cardiovascular disease, rare diseases, neurological diseases, infectious diseases and cancer. We created TEGSEDI™ (inotersen) the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy of hereditary transthyretin (TTR) amyloidosis (ATTR) in adult patients that our affiliate Akcea Therapeutics is commercializing. Together with Akcea, we are also bringing new medicines to patients with cardiometabolic lipid disorders.

To learn more about Ionis follow us on twitter @ionispharma or visit <http://ir.ionispharma.com/>.

\*Spinraza is marketed by Biogen.

---

## **Ionis' Forward-looking Statement**

This press release includes forward-looking statements regarding Ionis' business, financial guidance and the therapeutic and commercial potential of SPINRAZA, TEGSEDI (inotersen), WAYLIVRA (volanesorsen) and Ionis' technologies and products in development, including the business of Akcea Therapeutics, Inc., Ionis' majority owned affiliate. 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, 2017, and most recent Form 10-Q quarterly filing, 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 Akcea Therapeutics, Inc. TEGSEDI™ is a trademark of Akcea Therapeutics, Inc. WAYLIVRA™ is a trademark of Akcea Therapeutics, Inc. SPINRAZA® is a registered trademark of Biogen.

### **Ionis Pharmaceuticals Investor Contact:**

D. Wade Walke, Ph.D.  
Vice President, Investor Relations  
760-603-2741

### **Ionis Pharmaceuticals Media Contact:**

Roslyn Patterson  
Vice President, Corporate Communications  
760-603-2681

---

**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,	
	2018	2017	2018	2017
	(as revised)		(as revised)	
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$ 70,010	\$ 32,890	\$ 167,743	\$ 60,467
Licensing and royalty revenue	12,746	1,727	14,232	5,639
Total commercial revenue	82,756	34,617	181,975	66,106
Research and development revenue under collaborative agreements	62,639	83,697	225,584	280,281
Total revenue	145,395	118,314	407,559	346,387
Expenses:				
Research, development and patent	95,255	80,214	301,153	246,358
Selling, general and administrative	68,712	26,788	178,563	62,782
Total operating expenses	163,967	107,002	479,716	309,140
Income (loss) from operations	(18,572)	11,312	(72,157)	37,247
Other income (expense):				
Investment income	9,963	2,811	18,711	7,504
Interest expense	(11,282)	(10,825)	(33,332)	(33,966)
Loss on debt extinguishment	-	(7,689)	-	(7,689)
Other expenses	(22)	(2,141)	(145)	(3,528)
Income (loss) before income tax expense	(19,913)	(6,532)	(86,923)	(432)
Income tax expense	(452)	(961)	(824)	(1,184)
Net income (loss)	\$ (20,365)	\$ (7,493)	\$ (87,747)	\$ (1,616)
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	15,806	4,882	41,412	4,882
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$ (4,559)	\$ (2,611)	\$ (46,335)	\$ 3,266
Basic net income (loss) per share	\$ (0.03)	\$ (0.02)	\$ (0.33)	\$ 0.13
Diluted net income (loss) per share	\$ (0.03)	\$ (0.02)	\$ (0.33)	\$ 0.13
Shares used in computing basic net income (loss) per share	143,314	124,370	132,518	123,746
Shares used in computing diluted net income (loss) per share	143,314	124,370	132,518	125,858

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

Nine months ended,  
September 30, 2018  
(unaudited)

	Ionis	Akcea	Eliminations	Ionis Consolidated
<b>Revenue:</b>				
<b>Commercial revenue:</b>				
SPINRAZA royalties	\$ 167,743	\$ -	\$ -	\$ 167,743
Licensing and royalty revenue	9,432	12,000	(7,200)	14,232
Total commercial revenue	177,175	12,000	(7,200)	181,975
Research and development revenue under collaborative agreements	182,914	42,670	-	225,584
Intercompany revenue	49,937	-	(49,937)	-
Total revenue	410,026	54,670	(57,137)	407,559
<b>Expenses:</b>				
Research, development and patent expenses	209,690	96,808	(5,345)	301,153
Selling, general and administrative	69,394	116,620	(7,451)	178,563
Total operating expenses	279,084	213,428	(12,796)	479,716
Income (loss) from operations	130,942	(158,758)	(44,341)	(72,157)
<b>Other income (expense):</b>				
Investment income	14,625	4,086	-	18,711
Interest expense	(33,332)	-	-	(33,332)
Other expenses	-	(145)	-	(145)
Income (loss) before income tax expense	112,235	(154,817)	(44,341)	(86,923)
Income tax expense	(377)	(447)	-	(824)
Net income (loss)	\$ 111,858	\$ (155,264)	\$ (44,341)	\$ (87,747)
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	-	-	41,412	41,412
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$ 111,858	\$ (155,264)	\$ (2,929)	\$ (46,335)

**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,	
	2018	2017 (as revised)	2018	2017 (as revised)
	(unaudited)			
<b>As reported operating expenses according to GAAP</b>	\$ 163,967	\$ 107,002	\$ 479,716	\$ 309,140
Excluding compensation expense related to equity awards	<u>(34,883)</u>	<u>(21,472)</u>	<u>(97,210)</u>	<u>(63,642)</u>
<b>Pro forma operating expenses</b>	<u>\$ 129,084</u>	<u>\$ 85,530</u>	<u>\$ 382,506</u>	<u>\$ 245,498</u>
<b>As reported income (loss) from operations according to GAAP</b>	\$ (18,572)	\$ 11,312	\$ (72,157)	\$ 37,247
Excluding compensation expense related to equity awards	<u>(34,883)</u>	<u>(21,472)</u>	<u>(97,210)</u>	<u>(63,642)</u>
<b>Pro forma income from operations</b>	<u>\$ 16,311</u>	<u>\$ 32,784</u>	<u>\$ 25,053</u>	<u>\$ 100,889</u>
<b>As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	\$ (4,559)	\$ (2,611)	\$ (46,335)	\$ 3,266
Excluding compensation expense related to equity awards	<u>(34,883)</u>	<u>(21,472)</u>	<u>(97,210)</u>	<u>(63,642)</u>
<b>Pro forma net income attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	<u>\$ 30,324</u>	<u>\$ 18,861</u>	<u>\$ 50,875</u>	<u>\$ 66,908</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) (unaudited)

	<u>September 30,</u> 2018	<u>December 31,</u> 2017 (as revised)
<b>Assets:</b>		
Cash, cash equivalents and short-term investments	\$ 1,958,989	\$ 1,022,715
Contracts receivable	14,732	62,955
Other current assets	105,503	83,064
Property, plant and equipment, net	132,003	121,907
Other assets	36,686	32,133
Total assets	<u>\$ 2,247,913</u>	<u>\$ 1,322,774</u>
<b>Liabilities and stockholders' equity:</b>		
Other current liabilities	\$ 103,093	\$ 118,276
Current portion of deferred contract revenue	157,145	125,336
1% convertible senior notes	559,184	533,111
Long-term obligations, less current portion	64,963	72,745
Long-term deferred contract revenue	523,384	108,026
Total Ionis stockholders' equity	710,550	281,013
Noncontrolling interest in Akcea Therapeutics, Inc.	129,594	84,267
Total stockholders' equity	<u>840,144</u>	<u>365,280</u>
Total liabilities and stockholders' equity	<u>\$ 2,247,913</u>	<u>\$ 1,322,774</u>

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

September 30, 2018  
(unaudited)

	Ionis	Akcea	Eliminations	Ionis Consolidated
<b>Assets:</b>				
Cash, cash equivalents and short-term investments	\$ 1,638,674	\$ 320,315	\$ -	\$ 1,958,989
Contracts receivable	14,096	636	-	14,732
Receivable from Akcea Therapeutics, Inc.	23,888	-	(23,888)	-
Other current assets	99,952	8,397	(2,846)	105,503
Property, plant and equipment, net	126,246	5,757	-	132,003
Other assets	543,569	44,023	(550,906)	36,686
<b>Total assets</b>	<b>\$ 2,446,425</b>	<b>\$ 379,128</b>	<b>\$ (577,640)</b>	<b>\$ 2,247,913</b>
<b>Liabilities and stockholders' equity:</b>				
Other current liabilities	\$ 71,574	\$ 58,252	\$ (26,733)	\$ 103,093
Current portion of deferred contract revenue	124,798	32,347	-	157,145
1% convertible senior notes	559,184	-	-	559,184
Long-term obligations, less current portion	60,310	4,653	-	64,963
Long-term deferred contract revenue	522,619	2,464	(1,699)	523,384
Total stockholders' equity before noncontrolling interest	1,107,940	281,412	(678,802)	710,550
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	129,594	129,594
<b>Total stockholders' equity</b>	<b>1,107,940</b>	<b>281,412</b>	<b>(549,208)</b>	<b>840,144</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$ 2,446,425</b>	<b>\$ 379,128</b>	<b>\$ (577,640)</b>	<b>\$ 2,247,913</b>

SPINRAZA Q3 2017 – Q3 2018 Patient Dynamics

<b>U.S. Patient Dynamics*</b>	<b>Q3:17</b>	<b>Q4:17</b>	<b>Q1:18</b>	<b>Q2:18</b>	<b>Q3:18</b>
<b>Total patients</b>	1,230	1,640	1,910	2,160	2,410
<b>New patient starts</b>	520	420	290	270	260
<b>Average doses per patient</b>	1.8	1.5	1.2	1.1	1.1
<b>% Loading doses</b>	90%	75%	60%	45%	40%
<b>% Maintenance doses</b>	10%	25%	40%	55%	60%
<b>% Free doses</b>	20%	20%	20%	15%	15%

\*As reported by Biogen