

# Ionis Reports Second Quarter 2018 Financial Results

August 7, 2018

**Year-to-date revenues increased 15%, driven by increased SPINRAZA revenue  
TEGSEDI approved and on track to launch in the EU  
Positive FDA Advisory Committee vote recommending approval of WAYLIVRA  
Conference call and webcast today, August 7, 2018, at 11:30 a.m. Eastern Time**

CARLSBAD, Calif., Aug. 7, 2018 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported financial results for the second quarter of 2018 and highlighted its recent business and pipeline successes.



"With the approval of TEGSEDI in Europe, Ionis is entering a new chapter as a multi-product, sustainably profitable company. We anticipate the approval and launch of TEGSEDI and WAYLIVRA in multiple markets this year," said Stanley T. Crooke, M.D., Ph.D., chairman of the board and chief executive officer of Ionis. "Adding TEGSEDI and WAYLIVRA product sales to growing revenues from SPINRAZA positions Ionis for continued growth. In addition, we expect at least three of our drugs to advance into pivotal trials by the end of 2019, representing our next wave of near-term commercial opportunities. Importantly, we have achieved these successes while generating operating profits due to the combination of our efficient technology platform and business strategy."

## Second Quarter 2018 Financial Highlights

- *Revenues increased by 15 percent*
  - For the second quarter and year-to-date 2018 revenue was \$118 million and \$262 million, compared to \$112 million and \$228 million for the same periods in 2017
  - Commercial revenue from SPINRAZA for year-to-date 2018 was \$98 million, a three-fold increase over year-to-date 2017
  - Commercial revenue was more than 35 percent of Ionis' total revenue in the first half of 2018 compared to less than 15 percent for the same period in 2017, reflecting Ionis' transition to a commercial company
- *On track for third consecutive year of pro forma operating profitability while investing in the launch of two drugs*
  - GAAP operating results were a loss of \$50 million and \$54 million for the second quarter and year-to-date 2018, respectively, compared to income of \$6 million and \$26 million for the same periods in 2017
  - Pro forma operating results were a loss of \$16 million and income of \$9 million for the second quarter and year-to-date 2018, respectively, compared to income of \$28 million and \$68 million for the same periods in 2017
  - Operating expenses increased primarily due to higher SG&A expenses related to the commercialization of TEGSEDI and WAYLIVRA
- *Substantial cash position of \$2 billion enabling investment in commercial products and pipeline*
  - During the first half of 2018, Ionis received more than \$1.2 billion in payments from partners, including \$1 billion from Ionis' expanded collaboration with Biogen

"Our strong financial results were driven by a more than three-fold increase in commercial revenue from SPINRAZA compared to last year. Looking ahead to the second half of this year, we expect to continue to strengthen our financial performance as we add product sales from TEGSEDI and potentially WAYLIVRA to our growing SPINRAZA royalties. We also have the potential to earn numerous milestone payments from our partnered programs. In addition, we will have two full quarters of amortization from our expanded Biogen collaboration, providing further revenue growth," said Elizabeth L. Hougen, chief financial officer of Ionis. "We are on track to achieve our third consecutive year of pro forma operating income even as we prepare to launch two new drugs this year. In addition, we expect to end 2018 with more than \$1.8 billion in cash."

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

- **TEGSEDI™ (inotersen) – approved in the EU for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR)**
  - On track for post-summer launch in the EU

- On track for approval and launch in the U.S. in 2018
  - License agreement with PTC Therapeutics accelerates access to TEGSEDI in Latin America
  - Results from the TEGSEDI pivotal study published in the *New England Journal of Medicine*
  - Akcea's commercial organization staffed; patient support program and supply chain in place
- **WAYLIVRA™ (volanesorsen) – potential first treatment for people with FCS**
    - U.S. FDA Division of Metabolism and Endocrinology Products Advisory Committee voted in favor of approving WAYLIVRA
    - On track for approval and launch in the U.S. and EU in 2018
    - License agreement with PTC Therapeutics accelerates access to WAYLIVRA in Latin America
    - Akcea's commercial organization staffed; patient support program and supply chain in place
- **SPINRAZA – the first and only approved treatment for people with spinal muscular atrophy**
    - SPINRAZA, commercialized by Biogen, continues to generate growth, with global sales of \$423 million in the second quarter of 2018, a 250 percent increase from the second quarter of 2017
    - 10 percent of adults with SMA in the U.S. are currently on SPINRAZA treatment, a 20 percent increase from last quarter. Adult patients represent 60 percent of the U.S. SMA patient population
    - More than 5,000 people with SMA are now on SPINRAZA, representing a 28 percent increase from last quarter
    - Access outside the U.S. is expanding with reimbursement in 24 countries; Biogen expects reimbursement in at least four more countries by the end of 2018

#### Pipeline Progress

- European Union granted PRIME designation to IONIS-HTT<sub>Rx</sub> (RG6042), potentially providing accelerated assessment for the treatment of people with Huntington's disease
- European Medicines Agency granted Orphan Drug Designation to IONIS-MAPT<sub>Rx</sub> for the treatment of people with frontotemporal dementia
- Ionis earned a \$7.5 million milestone payment when the FDA approved Achaogen's ZEMDRI™ (plazomicin) for the treatment of people with complicated urinary tract infections.

#### Key Upcoming Events

- TEGSEDI EU launch
- TEGSEDI U.S. approval and launch
- WAYLIVRA U.S. and EU approval and launch
- Pivotal study of IONIS-HTT<sub>Rx</sub> in patients with Huntington's disease initiation by Roche
- Results from a Phase 2 clinical study of AKCEA-APO(a)-L<sub>Rx</sub> in patients with high Lp(a) and cardiovascular disease
- Results from a Phase 1/2 study of IONIS-SOD1<sub>Rx</sub> in patients with ALS and mutations in SOD1
- Phase 1 clinical study of IONIS-AZ4-2.5-L<sub>Rx</sub>, Ionis' first Generation 2.5 LICA drug to enter clinical development

#### Revenue

Ionis' revenue in the three and six months ended June 30, 2018 was \$117.7 million and \$262.2 million, respectively, compared to \$112.3 million and \$228.1 million for the same periods in 2017 and was comprised of the following (amounts in millions):

	Three months ended, June 30,		Six months ended, June 30,	
	2018	2017	2018	2017
Revenue:	(as revised)		(as revised)	
Commercial revenue:				
SPINRAZA royalties	\$57	\$23	\$98	\$28
Licensing and royalty revenue	-	1	1	4
Total commercial revenue	57	24	99	32
R&D Revenue:				
Amortization from upfront payments	34	26	61	47

Milestone payments	12	59	18	78
License fees	1	1	63	65
Other services	14	2	21	6
Total R&D revenue	61	88	163	196
Total revenue	\$118	\$112	\$262	\$228

The increase in revenue in the first half of 2018 compared to the same period in 2017 was primarily due to the increase in commercial revenue from SPINRAZA royalties, which increased over 250%. Revenue from the amortization of upfront payments increased due to Ionis' expanded strategic neurology collaboration with Biogen. Ionis expects that the quarterly amortization from this collaboration will be nearly \$14 million beginning in the third quarter. In the second quarter and year-to-date 2017, revenue included the \$50 million milestone payment from Biogen for SPINRAZA approval in the EU. License fees in the first half of 2018 were \$63 million, primarily from AstraZeneca for the license of IONIS-AZ5-2.5<sub>Rx</sub> and IONIS-AZ6-2.5-<sub>L<sub>Rx</sub></sub> compared to \$65 million in 2017, primarily from Bayer for the license of IONIS-FXI-<sub>L<sub>Rx</sub></sub>.

### Operating Expenses

Operating expenses for the three and six months ended June 30, 2018 on a GAAP basis were \$168.0 million and \$315.7 million, respectively, and on a pro forma basis were \$134.2 million and \$253.4 million, respectively. These amounts compare to GAAP operating expenses for the three and six months ended June 30, 2017 of \$105.8 million and \$202.1 million, respectively, and pro forma operating expenses of \$84.6 million and \$160.0 million, respectively. Operating expenses increased in the first half of 2018, compared to the same period in 2017, principally due to higher SG&A expenses as Akcea, Ionis' commercial affiliate, prepares to commercialize TEGSEDI and WAYLIVRA. The Company's SG&A expenses also increased in the first half of 2018 compared to the first half of 2017 due to an increase in fees the Company owed under its in-licensing agreements related to SPINRAZA. R&D expenses accounted for a smaller portion of the increase in operating expenses. R&D expenses increased primarily from medical affairs expenses related to the planned launch of TEGSEDI and WAYLIVRA.

### Net Income (Loss)

Ionis reported a net loss of \$56.6 million and \$67.4 million for the three and six months ended June 30, 2018, respectively, compared to a net loss of \$3.1 million and net income of \$5.9 million for the same periods in 2017, all according to GAAP. On a pro forma basis, Ionis reported a net loss of \$22.7 million and \$5.1 million for the three and six months ended June 30, 2018, respectively, compared to net income of \$18.2 million and \$48.0 million for the same periods in 2017. Ionis' GAAP net loss increased in the first half of 2018 primarily due to increased operating expenses related to the commercialization of TEGSEDI and WAYLIVRA.

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

From the closing of Akcea's IPO in July 2017 through mid-April 2018, Ionis owned 68 percent of Akcea. In April 2018, Ionis received an additional 18.7 million shares of Akcea's stock from the license of TEGSEDI and AKCEA-TTR-<sub>L<sub>Rx</sub></sub> to Akcea, increasing Ionis' ownership percentage to approximately 75 percent. Ionis' second quarter and year-to-date 2018 financial results reflect this increased ownership. 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, 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 three and six months ended June 30, 2018, was \$16.2 million and \$25.6 million, respectively. 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 Common Stockholders

Ionis reported a net loss attributable to Ionis' common stockholders of \$40.4 million and \$41.8 million for the three and six months ended June 30, 2018, respectively, compared to a net loss of \$3.1 million and net income of \$5.9 million for the same periods in 2017. For the three months ended June 30, 2018 and 2017, basic and diluted net loss per share were \$0.29 and \$0.02, respectively. For the six months ended June 30, 2018, basic and diluted net loss per share were each \$0.30. For the six months ended June 30, 2017, basic and diluted net income per share were each \$0.05. All amounts are on a GAAP basis.

### Balance Sheet

As of June 30, 2018, Ionis had cash, cash equivalents and short-term investments of \$2.0 billion compared to \$1.0 billion at December 31, 2017. During the first half of 2018, Ionis received over \$1.2 billion in payments from its partners, primarily from Biogen.

### 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.

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 45 drugs in development. SPINRAZA<sup>®</sup> (nusinersen) has been approved in global markets for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. TEGSEDI (inotersen) and WAYLIVRA (volanesorsen) are two antisense drugs that Ionis discovered and successfully advanced through Phase 3 studies. TEGSEDI is approved in the E.U. for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR, and is currently under regulatory review in the U.S. and Canada. WAYLIVRA is under regulatory review for marketing approval in the U.S., EU and Canada for the treatment of patients with familial chylomicronemia syndrome, or FCS. WAYLIVRA is also in a Phase 3 study in patients with familial partial lipodystrophy, or FPL. Akcea Therapeutics, an affiliate of Ionis focused on developing and commercializing drugs to treat patients with serious and rare diseases, will be responsible for commercializing TEGSEDI and WAYLIVRA. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at [www.ionispharma.com](http://www.ionispharma.com).

#### Ionis' Forward-looking Statement

This press release includes forward-looking statements regarding 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 this 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<sup>™</sup> is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics<sup>™</sup> is a trademark of Akcea Therapeutics, Inc. TEGSEDI<sup>™</sup> is a trademark of Akcea Therapeutics, Inc. WAYLIVRA<sup>™</sup> is a trademark of Akcea Therapeutics, Inc. SPINRAZA<sup>®</sup> is a registered trademark of Biogen.

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

	Three months ended, June 30,		Six months ended, June 30,	
	2018	2017	2018	2017
	(as revised)		(as revised)	
	(unaudited)			
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$56,653	\$22,366	\$97,734	\$27,577
Licensing and royalty revenue	545	1,322	1,487	3,912
Total commercial revenue	57,198	23,688	99,221	31,489
Research and development revenue under collaborative agreements	60,549	88,585	162,944	196,584
Total revenue	117,747	112,273	262,165	228,073
Expenses:				
Research, development and patent	101,830	83,506	205,897	166,144
Selling, general and administrative	66,198	22,317	109,851	35,994
Total operating expenses	168,028	105,823	315,748	202,138
Income (loss) from operations	(50,281)	6,450	(53,583)	25,935
Other income (expense):				
Investment income	5,134	2,465	8,748	4,744
Interest expense	(11,113)	(11,778)	(22,051)	(23,141)

Other expenses	45	-	(123)	(1,438)
Income (loss) before income tax expense	(56,215)	(2,863)	(67,009)	6,100
Income tax expense	(358)	(222)	(372)	(222)
Net income (loss)	<u>\$(56,573)</u>	<u>\$(3,085)</u>	<u>\$(67,381)</u>	<u>\$5,878</u>
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc. Inc.	16,215	-	25,606	-
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	<u>\$(40,358)</u>	<u>\$(3,085)</u>	<u>\$(41,775)</u>	<u>\$5,878</u>
Basic net income (loss) per share	<u>\$ (0.29)</u>	<u>\$ (0.02)</u>	<u>\$ (0.30)</u>	<u>\$ 0.05</u>
Diluted net income (loss) per share	<u>\$ (0.29)</u>	<u>\$ (0.02)</u>	<u>\$ (0.30)</u>	<u>\$ 0.05</u>
Shares used in computing basic net income (loss) per share	<u>128,712</u>	<u>123,989</u>	<u>127,030</u>	<u>123,428</u>
Shares used in computing diluted net income (loss) per share	<u>128,712</u>	<u>123,989</u>	<u>127,030</u>	<u>125,511</u>

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

	Six months ended, June 30, 2018 (unaudited)			
	Ionis	Akcea	Eliminations	Ionis Consolidated
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$97,734	\$-	\$-	\$97,734
Licensing and royalty revenue	1,487	-	-	1,487
Total commercial revenue	99,221	-	-	99,221
Research and development revenue under collaborative agreements	127,515	35,429	-	162,944
Intercompany revenue	5,229	-	(5,229)	-
Total revenue	<u>231,965</u>	<u>35,429</u>	<u>(5,229)</u>	<u>262,165</u>
Expenses:				
Research, development and patent expenses	143,770	67,427	(5,300)	205,897
Selling, general and administrative	47,649	61,752	450	109,851
Total operating expenses	<u>191,419</u>	<u>129,179</u>	<u>(4,850)</u>	<u>315,748</u>
Income (loss) from operations	40,546	(93,750)	(379)	(53,583)
Other income (expense):				
Investment income	6,334	2,414	-	8,748
Interest expense	(22,051)	-	-	(22,051)
Other expenses	-	(123)	-	(123)
Income (loss) before income tax expense	<u>24,829</u>	<u>(91,459)</u>	<u>(379)</u>	<u>(67,009)</u>
Income tax expense	(158)	(214)	-	(372)
Net income (loss)	<u>24,671</u>	<u>(91,673)</u>	<u>(379)</u>	<u>\$(67,381)</u>
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc. Inc.	-	-	25,606	\$25,606

Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders \$24,671 \$(91,673) \$(25,227) \$(41,775)

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

	Three months ended, June 30, 2018	2017	Six months ended, June 30, 2018	2017
	(as revised)	(unaudited)	(as revised)	(as revised)
<b>As reported operating expenses according to GAAP</b>	\$168,028	\$105,823	\$315,748	\$202,138
Excluding compensation expense related to equity awards	<u>(33,876)</u>	<u>(21,258)</u>	<u>(62,327)</u>	<u>(42,170)</u>
<b>Pro forma operating expenses</b>	<u>\$134,152</u>	<u>\$84,565</u>	<u>\$253,421</u>	<u>\$159,968</u>
<b>As reported income (loss) from operations according to GAAP</b>	\$(50,281)	\$6,450	\$(53,583)	\$25,935
Excluding compensation expense related to equity awards	<u>(33,876)</u>	<u>(21,258)</u>	<u>(62,327)</u>	<u>(42,170)</u>
<b>Pro forma income (loss) from operations</b>	<u>\$(16,405)</u>	<u>\$27,708</u>	<u>\$8,744</u>	<u>\$68,105</u>
<b>As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	\$(40,358)	\$(3,085)	\$(41,775)	\$5,878
Excluding compensation expense related to equity awards	<u>(33,876)</u>	<u>(21,258)</u>	<u>(62,327)</u>	<u>(42,170)</u>
<b>Pro forma net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	<u>\$(6,482)</u>	<u>\$18,173</u>	<u>\$20,552</u>	<u>\$48,048</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)**

	June 30, 2018	December 31, 2017
	(as revised)	
<b>Assets:</b>		
Cash, cash equivalents and short-term investments	\$1,980,450	\$1,022,715
Contracts receivable	16,761	62,955
Other current assets	86,061	83,064
Property, plant and equipment, net	127,940	121,907
Other assets	<u>36,103</u>	<u>32,133</u>
<b>Total assets</b>	<u>\$2,247,315</u>	<u>\$1,322,774</u>
<b>Liabilities and stockholders' equity:</b>		
Other current liabilities	\$88,030	\$118,276
Current portion of deferred contract revenue	160,589	125,336
1% convertible senior notes	550,328	533,111
Long-term obligations, less current portion	74,346	72,745
Long-term deferred contract revenue	556,586	108,026
Total Ionis stockholders' equity	696,255	281,013
Noncontrolling interest in Akcea Therapeutics, Inc.	<u>121,181</u>	<u>84,267</u>
<b>Total stockholders' equity</b>	<u>817,436</u>	<u>365,280</u>
<b>Total liabilities and stockholders' equity</b>	<u>\$2,247,315</u>	<u>\$1,322,774</u>

**Condensed Consolidating Balance Sheet  
(In Thousands)**

June 30, 2018  
(unaudited)

				Ionis
	Ionis	Akcea	Eliminations	Consolidated
<b>Assets:</b>				
Cash, cash equivalents and short-term investments	\$1,598,599	\$381,851	\$-	\$1,980,450
Contracts receivable	13,466	3,295	-	16,761
Receivable from Akcea Therapeutics, Inc.	27,137	-	(27,137)	-
Other current assets	82,486	3,575	-	86,061
Property, plant and equipment, net	126,417	1,523	-	127,940
Other assets	502,993	4,761	(471,651)	36,103
<b>Total assets</b>	<b>\$2,351,098</b>	<b>\$395,005</b>	<b>\$(498,788)</b>	<b>\$2,247,315</b>
<b>Liabilities and stockholders' equity:</b>				
Other current liabilities	\$53,669	\$61,498	\$(27,137)	\$88,030
Current portion of deferred contract revenue	129,583	35,713	(4,708)	160,589
1% convertible senior notes	550,328	-	-	550,328
Long-term obligations, less current portion	72,795	1,551	-	74,346
Long-term deferred contract revenue	552,445	5,839	(1,698)	556,586
Total stockholders' equity before noncontrolling interest				
Interest	992,278	290,404	(586,427)	696,255
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	121,181	121,181
<b>Total stockholders' equity</b>	<b>992,278</b>	<b>290,404</b>	<b>(465,246)</b>	<b>817,436</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$2,351,098</b>	<b>\$395,005</b>	<b>\$(498,788)</b>	<b>\$2,247,315</b>

SPINRAZA Q2 2017 – Q2 2018 Patient Dynamics

<b>U.S. Patient Dynamics*</b>	<b>Q2:17</b>	<b>Q3:17</b>	<b>Q4:17</b>	<b>Q1:18</b>	<b>Q2:18</b>
<b>Total patients</b>	710	1,230	1,640	1,910	2,160
<b>New patient starts</b>	500	520	420	290	270
<b>Average doses per patient</b>	2.6	1.9	1.6	1.1	1.1
<b>% Loading doses</b>	100%	90%	75%	60%	45%
<b>% Maintenance doses</b>	0%	10%	25%	40%	55%
<b>% Free doses</b>	20%	20%	20%	20%	15%

\*As announced by Biogen in their Q2:18 Earnings call

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SOURCE Ionis Pharmaceuticals, Inc.

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