

Ionis Reports First Quarter 2018 Financial Results

May 4, 2018

Total revenues increased by 25%, driven by SPINRAZA royalties
More than \$2 billion of cash expected upon closing of Biogen transaction
Expanded strategic neurology research collaboration with Biogen and invested in TEGSEDI™ commercialization through Akcea
Conference call and webcast today, May 4, 2018, at 11:30 a.m. Eastern Time

CARLSBAD, Calif., May 4, 2018 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported financial results for the first quarter of 2018 and highlighted its recent business and pipeline successes.



"We have successfully executed our commercialization strategy through the expansion of our strategic relationship with Biogen in neurological diseases and our commercialization of inotersen through our affiliate, Akcea. Our strategy is to create tailored commercial solutions for each of our drugs with the aim to maximize the commercial value of the drug and optimize our financial participation in this value. As our most recent collaboration with Biogen demonstrates, we have substantially increased the value of our antisense technology platform in neurological diseases over the last several years. The economics we achieved with our Bayer and Novartis partnerships show that we have done the same in multiple other therapeutic areas," said Stanley T. Croke, M.D., Ph.D., chairman of the board and chief executive officer. "Looking ahead, we are focused on launching TEGSEDI this year, assuming approval. As Akcea announced yesterday, the FDA decided they needed additional time to review some of our responses to their standard information requests and, therefore, has extended the review period for TEGSEDI. The new PDUFA date is October 6, 2018. We are working closely with the FDA to advance the review of our filing as quickly as possible. This year we also plan to launch WAYLIVRA, assuming approval. The commercialization of these two Ionis drugs will solidify Ionis as a multi-product, profitable company delivering innovative antisense medicines to patients in need."

First Quarter 2018 Financial Highlights

- *Revenues increased by 25%, driven by SPINRAZA royalties*
 - Total revenues were \$144 million, compared to \$116 million in Q1 2017
 - Commercial revenue from SPINRAZA royalties was \$41 million, compared to \$5 million in Q1 2017
 - R&D revenue included \$60 million in licensing fees for two drugs discovered by Ionis under its collaboration with AstraZeneca
 - Beginning in Q2 2018, Ionis' R&D revenue will include revenue from the amortization of the \$500 million technology access fee and equity premium related to Ionis' expanded strategic research collaboration with Biogen
- *GAAP operating and net loss near breakeven; on track for third consecutive year of pro forma operating profitability*
 - GAAP operating loss was \$3 million in Q1 2018, compared to GAAP operating income of \$19 million for the same period in 2017. Pro forma operating income was \$25 million in Q1 2018, compared to \$40 million for the same period in 2017
 - Operating expenses increased primarily due to higher SG&A expenses as Ionis prepares to commercialize TEGSEDI and WAYLIVRA this year
- *Cash will increase to more than \$2 billion, combining Ionis' first quarter cash balance of more than \$1 billion with \$1 billion expected upon closing of Ionis' expanded collaboration with Biogen*
 - During Q1 2018, Ionis received more than \$155 million in payments from partners

"In the first quarter, we made further progress toward our goal of being a multiproduct, profitable company. We ended the quarter with operating income of \$25 million and net income of \$27 million, both on a pro forma basis. Our strong first quarter results were driven by a 25% increase in revenue, primarily from substantial SPINRAZA royalties. Completing our seventh consecutive quarter of pro forma operating income keeps us on track for our third consecutive year of proforma operating income even while we prepare to launch two drugs. This strong performance is a direct result of our technology platform delivering high-value drugs combined with our business strategy, which aims to maximize the commercial potential of each of our drugs and optimize our participation in this value," said Elizabeth L. Hougen, chief financial officer of Ionis. "With the \$2 billion in cash we expect to have on the closing of the Biogen transaction, we have the financial strength to invest in opportunities that we believe will increase shareholder value, such as advancing and expanding our portfolio of drugs, retaining our drugs longer, and building a growing pipeline of Ionis-owned drugs that we commercialize ourselves through commercial affiliates."

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.

Business Highlights

- *Expanded strategic research collaboration with Biogen for neurological diseases – one of the largest research-stage collaborations ever*
 - \$1 billion upfront to Ionis, including \$625 million to purchase Ionis' stock at a 25% cash premium of \$125 million and a \$375 million upfront payment
 - Together, the cash premium and upfront payment represent a \$500 million technology access fee
 - Ionis is eligible to receive milestone payments and license fees up to \$270 million per drug and royalties up to 20% on net sales
 - Disease areas include dementia, neuromuscular diseases, movement disorders, ophthalmology, diseases of the inner ear, and neuropsychiatry
- *SPINRAZA[®] for SMA – one of the most successful orphan disease drug launches in history*
 - SPINRAZA[®], commercialized by Biogen, continued to generate growth with global revenues of \$364 million in Q1 2018
 - Increase of over 25% from last quarter in number of patients on SPINRAZA, including a 16% increase in number of patients treated in the U.S. and a more than 50% increase outside the US
 - Access expanding outside the U.S. with reimbursement in 24 countries; Biogen expects reimbursement in at least seven more countries by the end of 2018
 - Presented data from the SHINE open-label study at the American Academy of Neurology (AAN) annual meeting demonstrating continued benefit, improved motor function and mobility, and longer event-free survival for the most severely affected patients treated with SPINRAZA[®]
 - Presented data from the NURTURE study at the Muscular Dystrophy Association (MDA) Clinical Conference demonstrating continued benefit in motor function for infants, teens and young adults treated with SPINRAZA[®]
- *TEGSEDI (inotersen) for hereditary transthyretin amyloidosis (hATTR) – potential to transform the lives of people with hATTR; on-track to launch in 2018*
 - Invested in global commercialization of TEGSEDI by licensing TEGSEDI to Ionis' majority-owned affiliate, Akcea
 - Optimized Ionis' commercial participation with up to \$1.5 billion in milestone payments and a 60% profit share
 - Early access program enrolling in the U.S. and Europe
 - Global commercial organization staffed and focused on disease education; robust patient support program in place; supply chain in place and launch supplies ready to be labeled
 - Presented data from the Phase 3 NEURO-TTR study, the open label extension study and an investigator sponsored Phase 2 study at the International Symposium on Amyloidosis annual meeting and the AAN annual meeting
- *WAYLIVRA (volanesorsen) for FCS and FPL – potential first treatment for people with FCS; global on-track to launch in 2018*
 - Early access program enrolling in the U.S. and Europe
 - Global commercial organization staffed and focused on disease education; robust patient support program in place; supply chain in place and launch supplies ready to be labeled
 - Positive scientific opinion to initiate Early Access to Medicines Scheme (EAMS) by the UK's Medicines and Healthcare Products Regulatory Agency (MHRA), for the treatment of people with FCS
- *Collaboration with AstraZeneca for Cardiovascular, Renal and Metabolic Diseases*
 - Earned \$60 million for the license of second and third antisense drugs, IONIS-AZ5-2.5_{Rx} and IONIS-AZ6-2.5-_L_{Rx}, to treat a genetically associated form of kidney disease and nonalcoholic steatohepatitis (NASH), respectively, to AstraZeneca
 - As IONIS-AZ5-2.5_{Rx} and IONIS-AZ6-2.5-_L_{Rx} advance, Ionis may receive up to \$300 million for each drug in additional development and regulatory milestone payments, as well as tiered royalties on sales of each drug

Pipeline and Technology Progress

- Presented positive IONIS-HTT_{Rx} (RG6042) Phase 1/2 data in people with Huntington's disease (HD) at the annual CHDI HD conference. IONIS-HTT_{Rx} is the first drug in development to lower the disease-causing protein in people with HD
- Presented data at the AAN annual meeting that demonstrated broad potential of antisense drugs for neurological diseases with 14 presentations on Ionis' drugs to treat neurological diseases, including SMA, hATTR amyloidosis, Huntington's

disease, Alzheimer's disease, and ALS

- o Presented additional data from the Phase 1/2 study of IONIS-HTT_{Rx} that demonstrated correlations between reductions in mutant huntingtin (mHTT) and improvements in clinical measures of HD
- Published review paper titled, "RNA-targeted Therapeutics" in Cell Metabolism, authored by Stanley Crooke, M.D., Ph.D.; highlights antisense and other RNA-targeting therapeutics as important platforms for drug discovery across multiple diseases

"This year, we plan to launch two new promising drugs for rare diseases, TEGSEDI and WAYLIVRA. We look forward to adding commercial revenue from these drugs, assuming approval, to our growing revenue from SPINRAZA," said Brett P. Monia, Ph.D., chief operating officer and senior vice president of antisense drug discovery and translational medicine at Ionis Pharmaceuticals. "Our next set of commercial opportunities are on the horizon. We have multiple drugs that we or our partners plan to advance into pivotal studies in the next year or so, including IONIS-HTT_{Rx} for patients with Huntington's disease, IONIS-STAT3-2.5_{Rx} for patients with head and neck cancer and AKCEA-APO(a)-L_{Rx} in patients with high Lp(a) and risk of cardiovascular disease. Following closely behind these are drugs for rare diseases that have the potential to move quickly toward the market, including IONIS-GHR-L_{Rx} for patients with acromegaly and IONIS-TMPRSS6-L_{Rx} for patients with beta thalassemia."

Expected Events Through 2018

- Launch of TEGSEDI for people with hATTR, assuming approval
- Launch of WAYLIVRA for people with FCS, assuming approval
- Report results from six Phase 2 programs, including data from a study with AKCEA-APO(a)-L_{Rx} in people with high Lp(a) and AKCEA-ANGPTL3-L_{Rx} for people with rare hyperlipidemias
- Initiate up to nine new clinical studies, including a clinical study of AKCEA-TTR-L_{Rx} for hereditary and wild-type forms of ATTR

The recent Biogen transaction is subject to customary closing conditions, including the expiration of the applicable waiting period under the Hart Scott Rodino Antitrust Improvements Act of 1976 in the United States. Biogen and Ionis expect the deal to close in the second quarter of 2018.

Revenue

At the beginning of 2018, Ionis adopted the new revenue recognition accounting standard on a retrospective basis. Starting with Ionis' first quarter, all periods presented are shown using the new standard. Ionis has labeled its prior period financial statements "as revised" to indicate the change required under the accounting rules. Whenever Ionis refers to prior period results, they reflect the new accounting rules. This change did not have a significant impact on Ionis' previously reported revenue.

Ionis' revenue in the first quarter of 2018 was \$144.4 million, compared to \$115.8 million for the same period in 2017 and was comprised of the following (amounts in millions):

	Three months ended, March 31,	
	2018	2017
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$41	\$5
Licensing and royalty revenue	1	3
Total commercial revenue	42	8
R&D Revenue:		
License fees	62	65
Milestone payments	2	3
Amortization from:		
Upfront payments	27	20
Milestone payments	5	17
Other services	6	3

Total R&D revenue	102	108
	<hr/>	<hr/>
Total revenue	\$144	\$116
	<hr/>	<hr/>

License fees in the first quarter of 2018 were \$62 million primarily from AstraZeneca for the license of IONIS-AZ5-2.5_{Rx} and IONIS-AZ6-2.5-_{LRx}. The first quarter of 2017 included \$65 million in a license fee from Bayer for the license of IONIS-FXI-_{LRx}.

Operating Expenses

Operating expenses for the first quarter on a GAAP basis were \$147.7 million and on a pro forma basis were \$119.3 million compared to GAAP operating expenses of \$96.3 million and pro forma operating expenses of \$75.4 million for the same period in 2017. Operating expenses increased in Q1 2018, compared to 2017, principally due to higher SG&A expenses as Ionis and its affiliate Akcea prepare to commercialize WAYLIVRA and TEGSEDI. The Company's SG&A expenses also increased in Q1 2018 compared to Q1 2017 because of fees owed under its in-licensing agreements related to SPINRAZA, which increase as the Company's SPINRAZA revenue increases. R&D expenses accounted for a smaller portion of the increase in operating expenses. R&D expenses increased primarily from medical affairs expenses and manufacturing costs related to TEGSEDI for the planned launch.

Net Income (Loss)

Ionis reported a net loss of \$10.8 million for the first quarter of 2018, compared to net income of \$9.0 million for the same period in 2017, all according to GAAP. On a pro forma basis, Ionis reported net income of \$17.6 million for the first quarter of 2018, compared to net income of \$29.9 million for the same period in 2017. Ionis' GAAP net loss increased and its pro forma net income decreased in the first quarter of 2018 primarily due to increased operating expenses as Ionis prepares to commercialize TEGSEDI and WAYLIVRA.

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

Akcea sold shares of its common stock to third parties in its IPO in July 2017. From the closing of the IPO through the end of the first quarter in 2018, 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 first quarter of 2018 was \$9.4 million. Ionis also added a corresponding account in its stockholders' equity section on its balance sheet called "Noncontrolling interest in Akcea Therapeutics, Inc."

In April 2018, Ionis received 8 million shares of Akcea stock for the license of TEGSEDI and AKCEA-TTR-_{LRx} to Akcea and purchased an additional 10.7 million shares of Akcea stock for \$200 million, increasing Ionis' ownership percentage to approximately 75 percent. Ionis will reflect this increase in its ownership percentage in the second quarter of 2018.

Net Income (Loss) Attributable to Ionis Common Stockholders

Ionis reported a GAAP net loss attributable to Ionis' common stockholders of \$1.4 million for the first quarter of 2018, compared to GAAP net income of \$9.0 million for the same period in 2017. For the first quarter of 2018, basic and diluted net loss per share were \$0.01. For the first quarter of 2017, basic and diluted net income per share were \$0.07.

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. 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[®] (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 under regulatory review for marketing approval in the U.S., EU and Canada for the treatment of patients with hereditary ATTR amyloidosis. 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, an affiliate of Ionis focused on developing and commercializing drugs to treat patients with serious and rare diseases, will commercialize TEGSEDI and WAYLIVRA, if approved. 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 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 subsidiary. 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, which is 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™ 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, INC.
SELECTED FINANCIAL INFORMATION
Condensed Consolidated Statements of Operations
(In Thousands, Except Per Share Data)

	Three months ended, March 31,	
	2018	2017
	(as revised)	
	(unaudited)	
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$41,081	\$5,211
Licensing and royalty revenue	942	2,590
Total commercial revenue	42,023	7,801
Research and development revenue under collaborative agreements	102,396	107,999
Total revenue	144,419	115,800
Expenses:		
Research, development and patent expenses	104,067	82,638
Selling, general and administrative	43,653	13,677
Total operating expenses	147,720	96,315
Income (loss) from operations	(3,301)	19,485
Other income (expense):		
Investment income	3,610	2,280
Interest expense	(10,938)	(11,363)
Other expenses	(168)	(1,438)
Income (loss) before income tax expense	(10,797)	8,964
Income tax expense	(15)	-
Net income (loss)	\$(10,812)	\$8,964
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	\$9,392	-
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$(1,420)	\$8,964
Basic net income (loss) per share	\$(0.01)	\$0.07
Diluted net income (loss) per share	\$(0.01)	\$0.07
Shares used in computing basic net income (loss) per share	125,330	122,861
Shares used in computing diluted net income (loss) per share	125,330	124,972

IONIS PHARMACEUTICALS, INC.
SELECTED FINANCIAL INFORMATION
Condensed Consolidating Statement of Operations

(In Thousands)

Three months ended,
March 31, 2018
(unaudited)

	Ionis	Akcea	Eliminations	Ionis Consolidated
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$41,081	\$-	\$-	\$41,081
Licensing and royalty revenue	942	-	-	942
Total commercial revenue	42,023	-	-	42,023
Research and development revenue under collaborative agreements	85,288	17,108	-	102,396
Intercompany revenue	5,229	-	(5,229)	-
Total revenue	132,540	17,108	(5,229)	144,419
Expenses:				
Research, development and patent expenses	81,356	27,970	(5,259)	104,067
Selling, general and administrative	24,188	19,465	-	43,653
Total operating expenses	105,544	47,435	(5,259)	147,720
Income (loss) from operations	26,996	(30,327)	30	(3,301)
Other income (expense):				
Investment income	2,742	868	-	3,610
Interest expense	(10,938)	-	-	(10,938)
Other expenses	-	(168)	-	(168)
Income (loss) before income tax expense	18,800	(29,627)	30	(10,797)
Income tax expense	(15)	-	-	(15)
Net income (loss)	\$18,785	\$(29,627)	\$30	\$(10,812)
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	\$-	\$-	\$9,392	\$9,392
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$18,785	\$(29,627)	\$9,422	\$(1,420)

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, March 31,	
	2018	2017
	(as revised)	
	(unaudited)	
As reported operating expenses according to GAAP	\$147,720	\$96,315
Excluding compensation expense related to equity awards	(28,451)	(20,912)
Pro forma operating expenses	<u>\$119,269</u>	<u>\$75,403</u>
As reported income (loss) from operations according to GAAP	\$(3,301)	\$19,485
Excluding compensation expense related to equity awards	(28,451)	(20,912)
Pro forma income from operations	<u>\$25,150</u>	<u>\$40,397</u>

As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP \$(1,420) \$8,964

Excluding compensation expense related to equity awards

(28,451) (20,912)

Pro forma net income attributable to Ionis Pharmaceuticals, Inc. common stockholders

\$27,031 \$29,876

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)

	March 31, 2018	December 31, 2017
	(as revised)	
Assets:		
Cash, cash equivalents and short-term investments	\$1,035,301	\$1,022,715
Contracts receivable	36,858	62,955
Other current assets	71,124	83,064
Property, plant and equipment, net	123,188	121,907
Other assets	33,089	32,133
Total assets	<u>\$1,299,560</u>	<u>\$1,322,774</u>
Liabilities and stockholders' equity:		
Other current liabilities	\$92,516	\$118,276
Current portion of deferred contract revenue	120,127	125,336
1% convertible senior notes	541,635	533,111
Long-term obligations, less current portion	72,735	72,745
Long-term deferred contract revenue	85,446	108,026
Total Ionis stockholders' equity	301,391	281,013
Noncontrolling interest in Akcea Therapeutics, Inc.	85,710	84,267
Total stockholders' equity	<u>387,101</u>	<u>365,280</u>
Total liabilities and stockholders' equity	<u>\$1,299,560</u>	<u>\$1,322,774</u>

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

	March 31, 2018 (unaudited)			
	Ionis	Akcea	Eliminations	Ionis Consolidated
Assets:				
Cash, cash equivalents and short-term investments	\$790,366	\$244,935	\$-	\$1,035,301
Contracts receivable	36,858	-	-	36,858
Receivable from Akcea Therapeutics, Inc.	27,737	-	(27,737)	-
Other current assets	65,496	5,628	-	71,124
Property, plant and equipment, net	123,138	50	-	123,188
Other assets	305,449	1,853	(274,213)	33,089
Total assets	<u>\$1,349,044</u>	<u>\$252,466</u>	<u>\$(301,950)</u>	<u>\$1,299,560</u>
Liabilities and stockholders' equity:				
Other current liabilities	\$70,823	\$49,431	\$(27,738)	\$92,516
Current portion of deferred contract revenue	71,261	48,866	-	120,127
1% convertible senior notes	541,635	-	-	541,635
Long-term obligations, less current portion	72,725	10	-	72,735
Long-term deferred contract revenue	79,286	7,859	(1,699)	85,446
Total stockholders' equity before noncontrolling interest	513,314	146,300	(358,223)	301,391
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	85,710	85,710

Total stockholders' equity	513,314	146,300	(272,513)	387,101
Total liabilities and stockholders' equity	<u>\$1,349,044</u>	<u>\$252,466</u>	<u>\$(301,950)</u>	<u>\$1,299,560</u>

SPINRAZA Q1 2017 – Q1 2018 Patient Dynamics

U.S. Patient Dynamics*	Q1:17	Q2:17	Q3:17	Q4:17	Q1:18
Total patients	210	710	1,230	1,640	1,910
New patient starts	210	500	530	420	280
Average doses per patient	2.3	2.6	1.9	1.6	1.1
% Loading doses	100%	100%	90%	75%	60%
% Maintenance doses	0%	0%	10%	25%	40%
% Free doses	25%	20%	20%	20%	20%

*As announced by Biogen in their Q1:18 earnings call

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SOURCE Ionis Pharmaceuticals, Inc.

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