

## Ionis reports first quarter 2019 financial results

May 9, 2019

**Revenues more than doubled compared to 2018, resulting in net income of \$84 million**  
**Nearly \$300 million in revenues driven by \$150 million license fee from Novartis and growing commercial revenues**  
**Conference call and webcast today, May 9, 2019, at 11:30 a.m. Eastern Time**

CARLSBAD, Calif., May 9, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported its financial results for the first quarter of 2019 and recent business highlights.

"Our strong first quarter results put us on track to achieve our 2019 goals. We added commercial revenue from the first full quarter of the TEGSEDI launch to our growing commercial revenue from SPINRAZA. WAYLIVRA is now our third antisense medicine approved in just over two years, and we look forward to launching in Europe next quarter through our affiliate, Akcea," said Stanley T. Crooke, M.D., Ph.D., chairman of the board and chief executive officer of Ionis. "This week, data presented from our medicines targeting Huntington's disease and SOD1-ALS once again demonstrate the potential for our antisense technology to provide benefit in disease measures for patients with serious and previously untreatable diseases. Both medicines are in Phase 3 clinical trials with potential to support rapid paths to patients. Novartis licensed our most advanced LICA medicine, AKCEA-APO(a)-LR<sub>x</sub>, targeting the millions of patients worldwide with Lp(a)-driven cardiovascular disease. Novartis' decision to advance AKCEA-APO(a)-LR<sub>x</sub> into a Phase 3 cardiovascular outcomes study further validates the potential of our rapidly expanding LICA pipeline to treat a broad range of diseases, including those for large patient populations. We plan to advance our next LICA medicine, AKCEA-TTR-LR<sub>x</sub> targeting TTR amyloidosis, into Phase 3 development in the second half of this year. We believe our accomplishments in the first quarter of 2019 position us for continued success. The power of our efficient technology and business strategy give us confidence that we can continue to deliver sustainable financial growth while aggressively investing in our commercial medicines and advancing our pipeline and our technology."

### First Quarter 2019 Financial Results and Highlights

- *Revenues more than doubled compared to Q1 2018*
  - Total revenue was \$297 million compared to \$144 million in Q1 2018.
  - Commercial revenue from SPINRAZA<sup>®</sup> (nusinersen) was \$60 million compared to \$41 million in Q1 2018.
  - TEGSEDI<sup>™</sup> (inotersen) product sales were \$7 million in its first full quarter on the market and \$9 million since launching in Q4 2018.
  - R&D revenue included \$150 million from Novartis for its license of AKCEA-APO(a)-LR<sub>x</sub> and \$35 million from Roche when it enrolled the first patient in the Phase 3 study of IONIS-HTT<sub>Rx</sub> (RG6042) in patients with Huntington's disease.
- *Achieved substantial operating income and net income*
  - Operating income and net income were \$121 million and \$84 million, respectively, compared to an operating loss and net loss of \$3 million and \$1 million, respectively, in Q1 2018, all on a GAAP basis.
  - Non-GAAP operating income and net income were \$167 million and \$126 million, respectively, compared to \$25 million for both non-GAAP operating income and net income in Q1 2018.
  - Operating expenses increased in the first quarter primarily due to Ionis' investment in commercializing TEGSEDI.
- *Substantial cash position grew to \$2.3 billion enabling aggressive investment broadly across Ionis' business*

"We achieved another quarter of strong financial performance with both operating income and net income in the first quarter of 2019, substantially outperforming the same quarter in 2018. Our revenues in the first quarter were composed of growing commercial revenues from SPINRAZA royalties and TEGSEDI product sales, on top of substantial R&D revenues, driven in large part by the one-time \$150 million license fee from Novartis for AKCEA-APO(a)-LR<sub>x</sub>. Looking ahead, we expect growing revenues this year from SPINRAZA, TEGSEDI and our partnered programs. And we also look forward to adding revenue following the EU launch of WAYLIVRA," said Elizabeth L. Hougen, chief financial officer of Ionis. "We are on track to achieve our 2019 financial guidance of net income and more than \$100 million in operating income, both on a non-GAAP basis. Our goal is to continue to be profitable while investing in our commercial products, our pipeline and our technology."

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

### Recent Business Highlights

- *SPINRAZA – the worldwide standard-of-care for the treatment of people with all forms of spinal muscular atrophy*
  - Biogen reported worldwide sales of SPINRAZA of \$518 million in the first quarter of 2019, a 42 percent increase compared to Q1 2018, driven primarily by increased penetration in existing markets, new country launches and continued uptake in the U.S. by children and adult patients.
  - There were more than 7,500 SMA patients from over 40 countries on SPINRAZA treatment at the end of the first quarter of 2019, including commercial patients and patients in the expanded access program and clinical trials.
  - SPINRAZA data from the ongoing NURTURE and SHINE open-label extension studies demonstrated continued durable efficacy and reinforced the safety profile of SPINRAZA in patients treated for up to 6 years, as presented by Biogen at the 2019 AAN Annual Meeting.
- *TEGSEDI – launch underway in multiple markets for the treatment of polyneuropathy of hereditary transthyretin amyloidosis (hATTR) in adult patients*

- o TEGSEDI product sales were \$7 million in its first full quarter on the market and \$9 million since launching in Q4 2018.
- o TEGSEDI received a positive Final Evaluation Document (FED) from the National Institute for Health and Care Excellence (NICE) authorizing reimbursement for the treatment of patients with polyneuropathy due to hATTR amyloidosis in England.
- o Data presented at AAN from the TEGSEDI NEURO-TTR open-label extension study demonstrated long-term efficacy and safety in patients with hATTR.
- **WAYLIVRA® (volanesorsen) – approved in the EU for the treatment of adults with genetically confirmed familial chylomicronemia syndrome (FCS) at high risk for pancreatitis**
  - o Akcea's preparations to launch in the EU are underway, beginning in Germany in Q3 2019.
  - o Launch in additional EU countries is planned in 2020.
  - o Earned a \$6 million milestone payment from PTC Therapeutics for the EU approval of WAYLIVRA.
- Roche presented nine-month data from the ongoing Phase 1/2 open-label extension study of IONIS-HTT<sub>Rx</sub> (RG6042) in patients with Huntington's disease at AAN, demonstrating continued and sustained reductions in mutant huntingtin protein with bi-monthly dosing.
  - o Based on these data, Roche amended the dosing regimen in the Phase 3 study of IONIS-HTT<sub>Rx</sub> (RG6042) in patients with Huntington's disease to replace the monthly dosing regimen with a tri-annual (every four months) dosing regimen.
- Biogen presented data from the Phase 1/2 study of tofersen (IONIS-SOD1<sub>Rx</sub>) in ALS patients with SOD-1 mutations (SOD1-ALS) at AAN, demonstrating benefit in clinical measures of ALS disease progression after three months of treatment.
  - o Tofersen is in a Phase 3 clinical study that could support a rapid path to patients.
  - o Biogen is collaborating with regulators to further define the scope of the clinical data package required to support registration.
- Ionis generated a \$7.5 million milestone payment for advancing a new target for an unidentified neurological disease under its 2018 strategic neurology collaboration with Biogen.
- Brett P. Monia, Ph.D., chief operating officer of Ionis was appointed to the Ionis board of directors.

#### Key Upcoming Data Events

- Open-label extension study of IONIS-HTT<sub>Rx</sub> (RG6042) in patients with Huntington's disease
- Phase 1/2 study of AKCEA-TTR-L<sub>Rx</sub> in healthy volunteers
- BROADEN study of WAYLIVRA in patients with familial partial lipodystrophy (FPL)
- Development program targeting FXI for the treatment of patients with clotting disorders
- Development program for the treatment of patients with HBV infection
- Phase 2 study of IONIS-GHR-L<sub>Rx</sub> in patients with acromegaly
- Phase 1 study of IONIS-ENAC-2.5<sub>Rx</sub> in healthy volunteers

#### Revenue

Ionis' revenue for the first quarter of 2019 was \$297 million compared to \$144 million for the same period in 2018 and was comprised of the following (amounts in millions):

	Three months ended, March 31,	
	2019	2018
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$60	\$41
TEGSEDI product sales, net	7	-
Licensing and royalty revenue	1	1
Total commercial revenue	68	42
R&D Revenue:		
Amortization from upfront payments	36	27
Milestone payments	40	7
License fees	150	62
Other services	3	6
Total R&D revenue	229	102
Total revenue	\$297	\$144

In the first quarter of 2019, Ionis significantly increased both commercial revenue and R&D revenue. Commercial revenue from SPINRAZA royalties increased more than 45 percent. The Company also added growing TEGSEDI product sales to its commercial revenue.

Ionis' R&D revenue substantially increased in the first quarter of 2019 due to the \$150 million and \$35 million the Company earned from Novartis and Roche, respectively.

In the second quarter of 2019, Alnylam announced it licensed Ionis' technology to Regeneron. Once the transaction closes, Ionis expects to earn \$20 million in sublicensing revenue.

### **Operating Expenses**

Operating expenses for the first quarter of 2019 on a GAAP basis were \$176 million and on a non-GAAP basis were \$130 million. These amounts compare to GAAP operating expenses for the first quarter of 2018 of \$147 million and non-GAAP operating expenses of \$119 million. The increase in operating expenses was principally due to Ionis' investments in the global launch of TEGSEDI.

### **Income Tax Expense**

Ionis recorded income tax expense of \$31 million for the three months ended March 31, 2019, compared to \$15,000 for the same period in 2018. The increase in its income tax expense was primarily due to Ionis' expectation that it will generate U.S. federal and state taxable income in 2019. Ionis' 2019 income tax expense has two components. The first component relates to federal income taxes. Ionis expects to utilize its deferred tax assets to offset its U.S. federal taxable income. The other component of Ionis' income tax expense relates to the estimated cash taxes it will pay for its state income taxes. Although Ionis is recording the expense for its state income taxes in 2019, Ionis will not have to make the majority of the payment for this liability until the first quarter of 2020.

### **Net (Income) Loss Attributable to Noncontrolling Interest in Akcea**

At March 31, 2019, Ionis owned approximately 76 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 income attributable to noncontrolling interest in Akcea for the first quarter of 2019 was \$6 million, compared to a net loss attributable to noncontrolling interest in Akcea of \$9 million for the same period in 2018.

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

Ionis reported net income attributable to Ionis' common stockholders of \$84 million for the first quarter of 2019 compared to a net loss of \$1 million for the same period in 2018, both on a GAAP basis. On a non-GAAP basis, Ionis reported net income attributable to Ionis' common stockholders of \$126 million for the first quarter of 2019 compared to \$25 million for the same period in 2018. The increase was primarily due to increases in revenue.

For the first quarter of 2019, basic and diluted net income per share were \$0.63 and \$0.62, respectively, compared to basic and diluted net loss per share of \$0.01 for the same period in 2018. All amounts are on a GAAP basis.

### **Balance Sheet**

Ionis added to its strong balance sheet, ending the first quarter of 2019 with cash, cash equivalents and short-term investments of \$2.3 billion, compared to \$2.1 billion at December 31, 2018.

### **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, drug discovery platform called antisense technology that can 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. We created the first and only approved treatment for children and adults with spinal muscular atrophy as well as the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy in adults with hereditary transthyretin amyloidosis. Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 novel medicines designed to treat a broad range of diseases including cardiovascular diseases, neurological diseases, infectious diseases, pulmonary diseases and cancer.

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 (nusinersen), TEGSEDI (inotersen) and 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, 2018, 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 <sup>TM</sup> is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics <sup>TM</sup> is a trademark of Akcea Therapeutics, Inc. TEGSEDI <sup>TM</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 Millions, Except Per Share Data)**

	Three months ended, March 31,	
	2019	2018
	(unaudited)	
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$60	\$41
TEGSEDI product sales, net	7	-
Licensing and royalty revenue	1	1
Total commercial revenue	68	42
Research and development revenue under collaborative agreements	229	102
Total revenue	297	144
Expenses:		
Cost of products sold	1	-
Research, development and patent	107	104
Selling, general and administrative	68	43
Total operating expenses	176	147
		(3)
Income (loss) from operations	121	
Other income (expense):		
Investment income	12	3
Interest expense	(12)	(10)
Income (loss) before income tax benefit	121	(10)
Income tax expense	(31)	-
Net income (loss)	\$90	\$(10)
Net (income) loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	(6)	9
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$84	\$(1)
Basic net income (loss) per share	\$0.63	\$(0.01)
Diluted net income (loss) per share	\$0.62	\$(0.01)
Shares used in computing basic net income (loss) per share	138,582	125,330
Shares used in computing diluted net income (loss) per share	141,537	125,330

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

	Three months ended, March 31, 2019 (unaudited)			
	Ionis			
	<u>Ionis</u>	<u>Akcea</u>	<u>Eliminations</u>	<u>Consolidated</u>
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$60	\$-	\$-	\$60
TEGSEDI product sales, net	-	7	-	7
Licensing and royalty revenue	1	-	-	1
Total commercial revenue	61	7	-	68
Research and development revenue under collaborative agreements	72	157	-	229
Intercompany revenue	88	-	(88)	-
Total revenue	221	164	(88)	297
Expenses:				

Cost of products sold	-	2	(1)	1
Research, development and patent expenses	82	100	(75)	107
Selling, general and administrative	23	45	-	68
Profit/ loss share for TEGSEDI commercialization activities	9	(9)	-	-
Total operating expenses	114	138	(76)	176
Income from operations	107	26	(12)	121
Other income (expense):				
Investment income	11	1	-	12
Interest expense	(12)	-	-	(12)
Income before income tax expense	106	27	(12)	121
Income tax expense	(31)	-	-	(31)
Net income	\$75	\$27	(12)	\$90
Net (income) loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	\$-	\$-	\$(6)	\$(6)
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$75	\$27	\$(18)	\$84

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

	Three months ended, March 31,	
	2019	2018
	(unaudited)	
<b>As reported research, development and patent expenses according to GAAP</b>	\$107	\$104
Excluding compensation expense related to equity awards	(25)	(20)
<b>Non-GAAP research, development and patent expenses</b>	<u>\$82</u>	<u>\$84</u>
<b>As reported selling, general and administrative expenses according to GAAP</b>	\$68	\$43
Excluding compensation expense related to equity awards	(21)	(8)
<b>Non-GAAP selling, general and administrative expenses</b>	<u>\$47</u>	<u>\$35</u>
<b>As reported operating expenses according to GAAP</b>	\$176	\$147
Excluding compensation expense related to equity awards	(46)	(28)
<b>Non-GAAP operating expenses</b>	<u>\$130</u>	<u>\$119</u>
<b>As reported income (loss) from operations according to GAAP</b>	\$121	\$(3)
Excluding compensation expense related to equity awards	(46)	(28)
<b>Non-GAAP income from operations</b>	<u>\$167</u>	<u>\$25</u>
<b>As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	\$84	\$(1)
Excluding compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	(42)	(26)
<b>Non-GAAP net income attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP</b>	<u>\$126</u>	<u>\$25</u>

**Reconciliation of GAAP to Non-GAAP Basis**

As illustrated in the Selected Financial Information in this press release, non-GAAP operating expenses, non-GAAP income (loss) from operations, and non-GAAP 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 non-GAAP results. These measures are provided as supplementary information and are not a substitute for financial measures calculated in accordance with GAAP. Ionis reports these non-GAAP 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' non-GAAP results is consistent with how Ionis' management internally evaluates the performance of its operations.

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

March 31, December 31,  
2019      2018

(unaudited)

Assets:

Cash, cash equivalents and short-term investments	\$2,254	\$2,084
Contracts receivable	10	13
Other current assets	110	111
Property, plant and equipment, net	134	132
Other assets	328	328
Total assets	<u>\$2,836</u>	<u>\$2,668</u>

Liabilities and stockholders' equity:

Other current liabilities	\$108	\$ 120
Current portion of deferred contract revenue	145	160
1% convertible senior notes	577	568
Long-term obligations, less current portion	76	65
Long-term deferred contract revenue	542	567
Total Ionis stockholders' equity	1,208	1,049
Noncontrolling interest in Akcea Therapeutics, Inc.	180	139
Total stockholders' equity	<u>1,388</u>	<u>1,188</u>
Total liabilities and stockholders' equity	<u>\$2,836</u>	<u>\$2,668</u>

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

March 31, 2019  
(unaudited)

				Ionis
	Ionis	Akcea	Eliminations	Consolidated

Assets:

Cash, cash equivalents and short-term investments	\$1,932	\$322	\$-	\$2,254
Contracts receivable	-	10	-	10
Other current assets	102	19	(11)	110
Property, plant and equipment, net	128	6	-	134
Other assets	950	102	(724)	328
Total assets	<u>\$3,112</u>	<u>\$459</u>	<u>\$(735)</u>	<u>\$2,836</u>

Liabilities and stockholders' equity:

Other current liabilities	\$87	\$32	\$(11)	\$108
Current portion of deferred contract revenue	123	22	-	145
1% convertible senior notes	577	-	-	577
Long-term obligations, less current portion	61	15	-	76
Long-term deferred contract revenue	542	2	(2)	542
Total stockholders' equity before noncontrolling interest	1,722	388	(902)	1,208
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	180	180
Total stockholders' equity	<u>1,722</u>	<u>388</u>	<u>(722)</u>	<u>1,388</u>
Total liabilities and stockholders' equity	<u>\$3,112</u>	<u>\$459</u>	<u>\$(735)</u>	<u>\$2,836</u>

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