

Ionis Reports Second Quarter 2019 Financial Results

August 7, 2019

Q2 2019 year-to-date revenues increased more than 75% to \$461 million
SPINRAZA sales surpass \$1 billion in the first half of 2019
Ionis on track for third consecutive year of net income
Conference call and webcast today, August 7, 2019, at 11:30 a.m. Eastern Time

CARLSBAD, Calif., Aug. 7, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported its financial results for the second quarter and year-to-date 2019 and recent business highlights.

"We enter the second half of 2019 in a position of substantial financial strength driven by revenue growth of more than 75 percent. SPINRAZA's blockbuster performance, with over \$1 billion in net sales in the first half, contributed significantly to our strong financial results. There are approximately 8,400 patients on SPINRAZA, an increase of approximately 12 percent compared to last quarter. Product sales from TEGSEDI's second full quarter on the market also contributed to our strong first half results. Additionally, WAYLIVRA is on track to launch in Europe this quarter. With three medicines commercialized in the last three years, an advancing technology and a pipeline of over 40 medicines, we are positioned to continue delivering value to patients and shareholders through this year and beyond," said Stanley T. Crooke, M.D., Ph.D., chairman of the board and chief executive officer of Ionis.

"Our strong year-to-date financial results were driven by continued revenue growth across both commercial and R&D revenues. These results put us on track to meet or potentially improve upon our 2019 guidance. We are also on track to achieve our fourth consecutive year of operating income and our third consecutive year of net income, both on a non-GAAP basis. We believe our ability to be profitable while investing in commercial activities, fully exploiting our pipeline and advancing our technology, clearly sets us apart from our peers," said Elizabeth L. Hougen, chief financial officer of Ionis.

Year-to-Date 2019 Financial Results and Highlights

- Year-to-date revenues increased more than 75 percent
 - Commercial revenue from SPINRAZA® (nusinersen) royalties increased more than 30 percent to \$130 million for the first half of 2019 compared to 2018.
 - TEGSEDI® (inotersen) product sales were \$17 million in the first half of 2019, reflecting a more than 40 percent increase in the second quarter compared to the first quarter.
 - R&D revenue nearly doubled in the first half of 2019 compared to 2018.
- Significant net income for the first half of 2019 puts Ionis on track for its third consecutive year of net income
 - Operating income and net income significantly improved to \$103 million and \$84 million, respectively, in the first half of 2019 compared to 2018.
 - Non-GAAP operating income increased by more than 20-fold in the first half of 2019 compared to 2018.
 - Non-GAAP net income increased by approximately 10-fold in the first half of 2019 compared to 2018.
- Maintained substantial cash position of \$2.3 billion while aggressively investing broadly across Ionis' business

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.

"In addition to our commercial medicines, we have a broad and advancing pipeline of potentially transformational medicines we are moving toward the market. In our late-stage pipeline, our medicines targeting Huntington's disease and SOD1-ALS are progressing in Phase 3 studies, both with the potential to support registration. Our LICA medicines targeting Lp(a)-driven cardiovascular disease and TTR amyloidosis are on track to initiate Phase 3 programs this year. Positive results from our HBV program, our advancing FXI program, which is nearing completion, and the initiation of multiple Phase 2 studies demonstrate the potential value in our mid-stage pipeline. All of these potentially transformational medicines demonstrate the ability of our technology to address a broad range of diseases from rare diseases to those affecting millions of patients," said Brett P. Monia, chief operating officer at Ionis.

Recent Business Highlights

- SPINRAZA – a worldwide foundation-of-care for the treatment of patients of all ages with spinal muscular atrophy (SMA)
 - Worldwide sales of SPINRAZA increased by nearly 30 percent to \$1 billion in the first half of 2019 compared to 2018.
 - Patients on SPINRAZA treatment increased by approximately 12 percent, compared to last quarter, to approximately 8,400 patients across global commercial, clinical and expanded access settings.
 - Biogen believes the global SMA opportunity to be significantly greater than previous estimates, with over 45,000 patients in markets where Biogen has a direct presence.
- TEGSEDI – launch underway in multiple markets for the treatment of polyneuropathy of hereditary transthyretin amyloidosis (hATTR) in adult patients
 - Product sales were \$17 million in the first half of 2019 and \$10 million in the second quarter of 2019.
 - On track to launch in England this month following a positive reimbursement recommendation from NICE.
 - Launch in additional EU countries planned; launch in Latin America planned with PTC Therapeutics.
- WAYLIVRA® (volanesorsen) – the only medicine approved in the EU for the treatment of adults with genetically confirmed

familial chylomicronemia syndrome (FCS) at high risk for pancreatitis

- On track to launch in the EU this month, beginning in Germany, with additional EU country launches planned in 2020.
- Encouraging discussions continued with the U.S. Food and Drug Administration to clarify a path forward in the U.S.
- Received \$6 million milestone payment from PTC Therapeutics for the EU approval of WAYLIVRA in the second quarter.
- Achieved the primary endpoint of a statistically significant reduction in triglyceride levels and an important secondary endpoint of a statistically significant reduction in liver fat with good safety and tolerability in patients with familial partial lipodystrophy (FPL) in the BROADEN study.
- Ionis achieved positive results in its program to treat patients with hepatitis B viral infection as reported by GSK.
- Ionis initiated a Phase 2 study of IONIS-FB-L_{Rx} in patients with geographic atrophy secondary to age-related macular degeneration.
- Ionis initiated a Phase 2 study of IONIS-PKK-L_{Rx} in patients with hereditary angioedema.
- [Ionis appointed Joan E. Herman, MBA, MS, president and chief executive officer of Herman and Associates, LLC to the Ionis board of directors.](#)

Key Upcoming Events

- Ionis and Akcea plan to initiate the Phase 3 program for AKCEA-TTR-L_{Rx} in patients with TTR amyloidosis before the end of 2019.
- Novartis plans to initiate the Phase 3 HORIZON cardiovascular outcomes study of AKCEA-APO(a)-L_{Rx} (TQJ230) in patients with elevated Lp(a)-driven cardiovascular disease before the end of 2019.
- Ionis and Akcea plan to present data from the Phase 1/2 study of AKCEA-TTR-L_{Rx} in healthy volunteers in September 2019 at the European ATTR Amyloidosis meeting and at the Heart Failure Society of America.
- Roche plans to present data from the open label extension portion of the Phase 1/2 study of IONIS-HTT_{Rx} (RG6042) in patients with Huntington's disease.
- Ionis plans to initiate a Phase 2 study of IONIS-FB-L_{Rx} in a second indication under Ionis' collaboration with Roche to develop medicines for the treatment of complement-mediated diseases.
- Ionis and its partners plan to report data from the HBV and FXI clinical programs at future medical conferences.
- Ionis and Akcea plan to report top line results from Phase 2 studies of AKCEA-ANGPTL3-L_{Rx} and AKCEA-APOCIII-L_{Rx} in 1H 2020.

Revenue

Ionis' revenue in the three and six months ended June 30, 2019 was \$164 million and \$461 million, respectively, compared to \$118 million and \$262 million for the same periods in 2018 and was comprised of the following (amounts in millions):

	Three months ended, June 30,		Six months ended, June 30,	
	2019	2018	2019	2018
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$71	\$57	\$130	\$98
TEGSEDI product sales, net	10	-	17	-
Licensing and royalty revenue	7	-	9	1
Total commercial revenue	88	57	156	99
R&D Revenue:				
Amortization from upfront payments	41	34	75	61
Milestone payments	12	12	52	18
License fees	22	1	173	63
Other services	1	14	5	21
Total R&D revenue	76	61	305	163
Total revenue	\$164	\$118	\$461	\$262

For the first half of 2019, Ionis significantly increased both commercial revenue and R&D revenue compared to the same period in 2018. Commercial revenue from SPINRAZA royalties increased over 30 percent primarily due to increased SPINRAZA product sales. TEGSEDI product sales increased more than 40 percent over the first quarter of this year and were another important source of commercial revenue for Ionis.

Ionis' R&D revenue substantially increased in the first half of 2019 compared to the same period in 2018 primarily due to the following:

- \$150 million the Company earned from Novartis when Novartis licensed AKCEA-APO(a)-L_{Rx};
- \$35 million the Company earned from Roche when Roche enrolled the first patient in the Phase 3 study of IONIS-HTT_{Rx} in patients with Huntington's disease; and
- \$20 million the Company earned from Alnylam when Alnylam licensed Ionis' technology to Regeneron.

Operating Expenses

Operating expenses for the three and six months ended June 30, 2019 on a GAAP basis were \$183 million and \$358 million, respectively, and on a non-GAAP basis were \$141 million and \$271 million, respectively. These amounts compare to GAAP operating expenses for the three and six months ended June 30, 2018 of \$168 million and \$316 million, respectively, and non-GAAP operating expenses of \$134 million and \$253 million, respectively. The increase in operating expenses was principally due to Ionis' investment in the global launch of TEGSEDI and preparing to launch WAYLIVRA in the EU.

Income Tax Expense

Ionis recorded an income tax benefit of \$7 million for the three months ended June 30, 2019 and income tax expense of \$24 million for the six months ended June 30, 2019. Ionis' income tax expense in the first half of this year was primarily due to Ionis' expectation that it will generate U.S. federal and state taxable income in 2019.

Net Loss Attributable to Noncontrolling Interest in Akcea

At June 30, 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 loss attributable to noncontrolling interest in Akcea for the three and six months ended June 30, 2019 was \$9 million and \$3 million, respectively, compared to \$16 million and \$25 million for the three and six months ended June 30, 2018, respectively.

Net Income (Loss) Attributable to Ionis Common Stockholders

On a GAAP basis, Ionis reported net loss and net income attributable to Ionis' common stockholders of \$1 million and \$84 million for the three and six months ended June 30, 2019, respectively, compared to a net loss of \$40 million and \$42 million for the same periods in 2018. On a non-GAAP basis, Ionis reported net income attributable to Ionis' common stockholders of \$38 million for the three months ended June 30, 2019, compared to a net loss attributable to Ionis' common stockholders of \$10 million for the same period in 2018. For the six months ended June 30, 2019, Ionis reported net income attributable to Ionis' common stockholders of \$162 million, compared to \$15 million for the same period in 2018, all on a non-GAAP basis. The increase was primarily due to increases in revenue.

For the three months ended June 30, 2019, basic and diluted net loss per share were \$0.01, compared to \$0.29 for the same period in 2018. For the six months ended June 30, 2019, basic and diluted net income per share were \$0.62 and \$0.61, respectively, compared to basic and diluted net loss per share of \$0.30 for the same period in 2018. All amounts are on a GAAP basis.

Balance Sheet

Ionis maintained its strong balance sheet, ending the second 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. 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 visit www.ionispharma.com or follow us on twitter @ionispharma.

*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, and the 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 registered trademark of Akcea Therapeutics, Inc. TEGSEDI[®] is a registered trademark of Akcea Therapeutics, Inc. WAYLIVRA[®] is a registered trademark of Akcea Therapeutics, Inc. SPINRAZA[®] 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, June 30,		Six months ended, June 30,	
	2019	2018	2019	2018
	(unaudited)			
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$71	\$57	\$130	\$98
TEGSEDI product sales, net	10	-	17	-
Licensing and royalty revenue	7	-	9	1
Total commercial revenue	88	57	156	99
Research and development revenue under collaborative agreements	76	61	305	163
Total revenue	164	118	461	262
Expenses:				
Cost of products sold	1	-	2	-
Research, development and patent	106	102	213	206
Selling, general and administrative	76	66	143	110
Total operating expenses	183	168	358	316
Income (loss) from operations	(19)	(50)	103	(54)
Other income (expense):				
Investment income	14	5	25	9
Interest expense	(12)	(11)	(23)	(22)
Income (loss) before income tax benefit (expense)	(17)	(56)	105	(67)
Income tax benefit (expense)	7	-	(24)	-
Net income (loss)	\$(10)	\$(56)	\$81	\$(67)
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	9	16	3	25
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$(1)	\$(40)	\$84	\$(42)
Basic net income (loss) per share	\$(0.01)	\$(0.29)	\$0.62	\$(0.30)
Diluted net income (loss) per share	\$(0.01)	\$(0.29)	\$0.61	\$(0.30)
Shares used in computing basic net income (loss) per share	140	129	139	127
Shares used in computing diluted net income (loss) per share	140	129	142	127

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

	Six months ended, June 30, 2019 (unaudited)			
	Ionis	Akcea	Eliminations	Ionis Consolidated
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$130	\$ -	\$ -	\$130
TEGSEDI product sales, net	-	17	-	17
Licensing and royalty revenue	4	5	-	9
Total commercial revenue	134	22	-	156
Research and development revenue under collaborative agreements	137	168	-	305
Intercompany revenue	91	-	(91)	-
Total revenue	362	190	(91)	461
Expenses:				
Cost of products sold	-	8	(6)	2
Research, development and patent expenses	168	120	(75)	213
Selling, general and administrative	48	95	-	143

Profit/ loss share for TEGSEDI commercialization activities	20	(20)	-	-
Total operating expenses	236	203	(81)	358
Income (loss) from operations	126	(13)	(10)	103
Other income (expense):				
Investment income	22	3	-	25
Interest expense	(23)	-	-	(23)
Income before income tax expense	125	(10)	(10)	105
Income tax expense	(24)	-	-	(24)
Net income (loss)	\$101	\$(10)	\$(10)	\$81
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	-	-	3	3
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$101	\$(10)	\$(7)	\$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, June 30,		Six months ended, June 30,	
	2019	2018	2019	2018
	(unaudited)			
As reported research, development and patent expenses according to GAAP	\$106	\$102	\$213	\$206
Excluding compensation expense related to equity awards	(24)	(19)	(49)	(39)
Non-GAAP research, development and patent expenses	<u>\$82</u>	<u>\$83</u>	<u>\$164</u>	<u>\$167</u>
As reported selling, general and administrative expenses according to GAAP	76	66	143	110
Excluding compensation expense related to equity awards	(18)	(14)	(39)	(24)
Non-GAAP selling, general and administrative expenses	<u>\$58</u>	<u>\$52</u>	<u>\$104</u>	<u>\$86</u>
As reported operating expenses according to GAAP	\$183	\$168	\$358	\$316
Excluding compensation expense related to equity awards	(42)	(34)	(87)	(63)
Non-GAAP operating expenses	<u>\$141</u>	<u>\$134</u>	<u>\$271</u>	<u>\$253</u>
As reported income (loss) from operations according to GAAP	\$(19)	\$(50)	\$103	\$(54)
Excluding compensation expense related to equity awards	(42)	(34)	(87)	(63)
Non-GAAP income (loss) from operations	<u>\$23</u>	<u>\$(16)</u>	<u>\$190</u>	<u>\$9</u>
As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	\$(1)	\$(40)	\$84	\$(42)
Excluding compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	(39)	(30)	(78)	(57)
Non-GAAP net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	<u>\$38</u>	<u>\$(10)</u>	<u>\$162</u>	<u>\$15</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)

June 30, December 31,

	2019	2018
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,262	\$2,084
Contracts receivable	32	13
Other current assets	121	111
Property, plant and equipment, net	135	132
Other assets	328	328
Total assets	<u>\$2,878</u>	<u>\$2,668</u>
Liabilities and stockholders' equity:		
Other current liabilities	\$ 104	\$ 120
Current portion of deferred contract revenue	137	160
1% convertible senior notes	587	568
Long-term obligations, less current portion	76	65
Long-term deferred contract revenue	517	567
Total Ionis stockholders' equity	1,269	1,049
Noncontrolling interest in Akcea Therapeutics, Inc.	188	139
Total stockholders' equity	<u>1,457</u>	<u>1,188</u>
Total liabilities and stockholders' equity	<u>\$2,878</u>	<u>\$2,668</u>

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

	June 30, 2019 (unaudited)			
			Ionis	
	Ionis	Akcea	Eliminations	Consolidated
Assets:				
Cash, cash equivalents and short-term investments	\$1,966	\$296	\$ -	\$2,262
Contracts receivable	23	9	-	32
Other current assets	106	23	(8)	121
Property, plant and equipment, net	130	5	-	135
Other assets	949	101	(722)	328
Total assets	<u>\$3,174</u>	<u>\$434</u>	<u>\$(730)</u>	<u>\$2,878</u>
Liabilities and stockholders' equity:				
Other current liabilities	\$78	\$35	\$ (9)	\$104
Current portion of deferred contract revenue	121	16	-	137
1% convertible senior notes	587	-	-	587
Long-term obligations, less current portion	61	15	-	76
Long-term deferred contract revenue	518	-	(1)	517
Total stockholders' equity before noncontrolling interest	1,809	368	(908)	1,269
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	188	188
Total stockholders' equity	<u>1,809</u>	<u>368</u>	<u>(720)</u>	<u>1,457</u>
Total liabilities and stockholders' equity	<u>\$3,174</u>	<u>\$434</u>	<u>\$(730)</u>	<u>\$2,878</u>

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