



Ionis reports second quarter 2020 financial results and recent business achievements

August 5, 2020

On track to achieve 2020 objectives and financial guidance

Webcast today, August 5, 2020, at 11:30 a.m. Eastern Time

CARLSBAD, Calif., Aug. 5, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported its financial results for the second quarter of 2020 and recent business highlights.



"The first half of this year was marked by numerous important achievements. Our Phase 3 programs progressed, we advanced our cardio-renal, metabolic and neurological disease franchises and added new medicines to our Ionis-owned pipeline. Together, these catalysts moved us closer to delivering 10 or more marketing applications through 2025, which we expect to result in a number of new commercial medicines. In the second half of this year, we are expanding the reach of our technology in neurological and pulmonary diseases, as studies in sporadic ALS and COPD get underway. We also look forward to proof-of-concept data from additional mid-stage programs," said Brett P. Monia, Ph.D., chief executive officer at Ionis. "Our achievements, together with our significant financial resources, position us to realize my vision for Ionis – to lead in the delivery of transformational medicines for patients around the world."

Second Quarter 2020 Financial Results and Highlights

- On track to achieve financial guidance of being meaningfully profitable this year
 - Net income of \$8 million on a non-GAAP basis and a net loss of \$32 million on a GAAP basis
- Achieved quarter over quarter growth in commercial and R&D revenues
 - Commercial revenue from SPINRAZA[®] (nusinersen) royalties increased to \$72 million
 - Product sales from TEGSEDI[®] (inotersen) and WAYLIVRA[®] (volanesorsen) increased to \$16 million
 - R&D revenue increased to more than \$55 million, including \$26 million from Ionis' neurological disease franchise, \$13 million from the oncology franchise and more than \$10 million from the cardio-renal franchise
- Cash position of more than \$2.3 billion provides substantial financial resources to continue executing on strategic goals

"We ended the second quarter with net income on a non-GAAP basis, an increase compared to our first quarter results. Looking ahead, we are maintaining our 2020 financial guidance and expect revenue and earnings growth in the second half of this year. With our substantial resources, we are well positioned to continue executing on our ambitious agenda and to deliver increasing value near-term and into the future," said Elizabeth L. Hougen, chief financial officer of Ionis.

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.

Commercial Medicine Highlights

- SPINRAZA: a global foundation-of-care for the treatment of spinal muscular atrophy (SMA) patients of all ages
 - \$495 million in worldwide sales in the second quarter of this year
 - More than 11,000 patients were on SPINRAZA treatment worldwide at the end of the second quarter, including patients across commercial, expanded access and clinical trial settings
 - The Phase 4 RESPOND study to evaluate SPINRAZA benefit in patients with a suboptimal clinical response to Zolgensma[®] (onasemnogene abeparvovec) is expected to begin early next year

- o The DEVOTE study evaluating a higher dose of SPINRAZA with the potential to deliver even greater efficacy in SMA patients of all ages is progressing
- o New clinical data from the NURTURE and SHINE studies, as well as new real-world data, further support SPINRAZA's durable efficacy and established safety profile across SMA patients of all ages
- TEGSEDI: the only approved at-home subcutaneous therapy for the treatment of hereditary transthyretin amyloidosis (hATTR) with polyneuropathy in adult patients
 - o Commercially available in 15 countries
 - o Reimbursement approved in Portugal, Spain, Italy and Austria
 - o Expanding commercial availability in additional EU countries and in Latin America this year
- WAYLIVRA: the only approved treatment in the EU for adults with genetically confirmed familial chylomicronemia syndrome (FCS) at high risk for pancreatitis
 - o Launch progressing in Germany, Austria, Greece and through the ATU in France; launching in additional EU countries this year
 - o Filed for marketing approval in Brazil; refiling new drug application for U.S. marketing authorization

Second Quarter 2020 and Recent Pipeline Highlights

- Completed enrollment in the global GENERATION HD1 Phase 3 study of tominersen in patients with Huntington's disease
- Progressed multiple neurological disease medicines under Ionis' broad Biogen collaboration
 - o Published data from the Phase 1/2 study of tofersen in the *New England Journal of Medicine*
 - o Progressed the IONIS-MAPT_{Rx} long-term extension study in patients with Alzheimer's disease and achieved a \$12 million milestone payment
 - o Advanced ION464 into a Phase 1/2 study in patients with multiple system atrophy and achieved an \$18 million milestone payment
- Advanced medicines for the treatment of cancer and immune-mediated GI disease
 - o Licensed ION736 to AstraZeneca for the treatment of cancer and achieved a \$13 million license fee
 - o Initiated a Phase 1 study of ION253 for the treatment of immune-mediated GI disease and achieved a \$5 million milestone payment from Janssen
- Expanded the Ionis-owned pipeline with the addition of ION363 for the treatment of FUS-ALS

Upcoming Catalysts

- Report clinical proof-of-concept results for four or more programs
- Present positive Phase 2 results from vupanorsen and AKCEA-APOCIII-L_{Rx}
- Initiate a Phase 3 study of AKCEA-APOCIII-L_{Rx} in patients with FCS
- Initiate a registration study of ION363 in patients with FUS-ALS
- Initiate a Phase 1/2 study of ION541 in patients with sporadic ALS
- Initiate a Phase 2 study of IONIS-ENaC-2.5_{Rx} in patients with chronic obstructive pulmonary disease (COPD)
- Initiate a Phase 2 study of IONIS-FXI-L_{Rx} in patients with clotting disorders
- Initiate a Phase 2 study of IONIS-HBV_{Rx} in patients with hepatitis B virus infection
- Advance additional new medicines into development

Revenue

Ionis' revenue was comprised of the following (amounts in millions):

	Three months ended, Six months ended			
	June 30,		June 30,	
	2020	2019	2020	2019
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$72	\$71	\$138	\$130
Product sales, net	16	10	32	17
Licensing and royalty revenue	2	7	4	9
Total commercial revenue	90	88	174	156
R&D Revenue:				
Amortization from upfront payments	28	41	49	75
Milestone payments	7	12	30	52
License fees	15	22	15	173
Other services	6	1	11	5
Total R&D revenue	56	76	105	305
Total revenue	\$146	\$164	\$279	\$461

Operating Expenses

Ionis' operating expenses for the second quarter of 2020 increased compared to the same period in 2019 driven by its investments in advancing its strategic priorities, including advancing the Phase 3 program for AKCEA-TTR-L_{Rx} and other

medicines in its Ionis-owned pipeline.

Net Loss Attributable to Noncontrolling Interest in Akcea

At June 30, 2020, Ionis owned approximately 76 percent of Akcea. The Company's net loss attributable to noncontrolling interest in Akcea for the second quarter of 2020 was consistent with the same period last year. The line titled "Net loss attributable to noncontrolling interest in Akcea" on Ionis' statement of operations reflects the portion of Akcea's net income or loss attributable to the other owners of Akcea's common stock.

Net Income (Loss) Attributable to Ionis Common Stockholders

Ionis' net loss attributable to Ionis' common stockholders for the second quarter of 2020 was larger compared to the same period in 2019 primarily due to higher revenue and lower operating expenses in the same period last year.

Balance Sheet

Ionis ended June 2020 with cash, cash equivalents and short-term investments of more than \$2.3 billion, compared to \$2.5 billion at December 31, 2019.

Webcast

Today, at 11:30 a.m. Eastern Time, Ionis will conduct a live webcast to discuss this earnings release and related activities. Interested parties may access the webcast [here](#). A webcast replay will be available for a limited time at the same address.

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 potentially treat a broad range of diseases, including neurological, cardio-renal, metabolic, infectious, and pulmonary diseases.

To learn more about Ionis visit www.ionispharma.com or follow us on twitter @ionispharma.

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, including those related to the impact COVID-19 could have on our business, and including but not limited to those related to our commercial products and the medicines in our pipeline, and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. 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, 2019, 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 Investor Contact:

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

Ionis Pharmaceuticals Media Contact:

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

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

	Three months ended		Six months ended	
	June 30,		June 30,	
	2020	2019	2020	2019
	(unaudited)			
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$72	\$71	\$138	\$130
Product sales, net	16	10	32	17
Licensing and royalty revenue	2	7	4	9
Total commercial revenue	90	88	174	156
Research and development revenue under collaborative agreements	56	76	105	305
Total revenue	146	164	279	461
Expenses:				
Cost of products sold	3	1	6	2
Research, development and patent	122	106	239	213
Selling, general and administrative	73	76	147	143
Total operating expenses	198	183	392	358
Income (loss) from operations	(52)	(19)	(113)	103
Other income, net	8	2	7	2
Income (loss) before income tax benefit (expense)	(44)	(17)	(106)	105
Income tax benefit (expense)	-	7	4	(24)
Net income (loss)	\$(44)	\$(10)	\$(102)	\$81
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	12	9	22	3
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	\$(32)	\$(1)	\$(80)	\$84
Basic net income (loss) per share	\$(0.23)	\$(0.01)	\$(0.58)	\$0.62
Diluted net income (loss) per share	\$(0.23)	\$(0.01)	\$(0.58)	\$0.61
Shares used in computing basic net income (loss) per share	139	140	139	139
Shares used in computing diluted net income (loss) per share	139	140	139	142

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

	Six months ended			
	June 30, 2020			
	(unaudited)			
	Ionis			
	Ionis	Akcea	Eliminations	Consolidated
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$138	\$-	\$-	\$138
Product sales, net	-	32	-	32
Licensing and royalty revenue	4	-	-	4
Total commercial revenue	142	32	-	174
Research and development revenue under collaborative agreements	98	7	-	105
Intercompany revenue	7	-	(7)	-
Total revenue	247	39	(7)	279
Expenses:				
Cost of products sold	-	10	(4)	6

Research, development and patent expenses	197	47	(5)	239
Selling, general and administrative	59	88	-	147
Profit/ loss share for TEGSEDI commercialization activities	10	(10)	-	-
Total operating expenses	266	135	(9)	392
Loss from operations	(19)	(96)	2	(113)
Other income, net	4	3	-	7
Loss before income tax benefit	(15)	(93)	2	(106)
Income tax benefit	3	1	-	4
Net loss	<u>\$(12)</u>	<u>\$(92)</u>	<u>\$2</u>	<u>\$(102)</u>
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	<u>\$-</u>	<u>\$-</u>	<u>\$22</u>	<u>\$22</u>
Net loss attributable to Ionis Pharmaceuticals, Inc. common stockholders	<u>\$(12)</u>	<u>\$(92)</u>	<u>\$24</u>	<u>\$(80)</u>

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, 2020	Six months ended June 30, 2019	2020	2019
	(unaudited)			
As reported research, development and patent expenses according to GAAP	\$122	\$106	\$239	\$213
Excluding compensation expense related to equity awards	(26)	(24)	(52)	(49)
Non-GAAP research, development and patent expenses	<u>\$96</u>	<u>\$82</u>	<u>\$187</u>	<u>\$164</u>
As reported selling, general and administrative expenses according to GAAP	\$73	\$76	\$147	\$143
Excluding compensation expense related to equity awards	(22)	(18)	(37)	(39)
Non-GAAP selling, general and administrative expenses	<u>\$51</u>	<u>\$58</u>	<u>\$110</u>	<u>\$104</u>
As reported operating expenses according to GAAP	\$198	\$183	\$392	\$358
Excluding compensation expense related to equity awards	(48)	(42)	(89)	(87)
Non-GAAP operating expenses	<u>\$150</u>	<u>\$141</u>	<u>\$303</u>	<u>\$271</u>
As reported income (loss) from operations according to GAAP	\$(52)	\$(19)	\$(113)	\$103
Excluding compensation expense related to equity awards	(48)	(42)	(89)	(87)
Non-GAAP income (loss) from operations	<u>\$(4)</u>	<u>\$23</u>	<u>\$(24)</u>	<u>\$190</u>
As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	\$(32)	\$(1)	\$(80)	\$84
Excluding compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	(45)	(39)	(84)	(78)
Income tax effect related to compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	5	14	11	13
Non-GAAP net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	<u>\$8</u>	<u>\$24</u>	<u>\$(7)</u>	<u>\$149</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) attributable to Ionis Pharmaceuticals, Inc. common shareholders were adjusted from GAAP to exclude compensation expense related to equity awards and the related tax effect. Compensation expense related to equity awards 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, 2020	December 31, 2019
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,349	\$2,500
Contracts receivable	28	63
Other current assets	155	158
Property, plant and equipment, net	173	154
Other assets	374	358
Total assets	<u>\$3,079</u>	<u>\$3,233</u>
Liabilities and stockholders' equity:		
Other current liabilities	\$132	\$155
Current portion of deferred contract revenue	100	118
0.125% convertible senior notes	445	435
1% convertible senior notes	284	275
Long-term obligations, less current portion	75	75
Long-term deferred contract revenue	448	490
Total Ionis stockholders' equity	1,377	1,471
Noncontrolling interest in Akcea Therapeutics, Inc.	218	214
Total stockholders' equity	<u>\$1,595</u>	<u>\$1,685</u>
Total liabilities and stockholders' equity	<u>\$3,079</u>	<u>\$3,233</u>

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

	June 30, 2020 (unaudited)			
	Ionis			
	Ionis	Akcea	Eliminations	Consolidated
Assets:				
Cash, cash equivalents and short-term investments	\$1,959	\$390	\$-	\$2,349
Contracts receivable	14	14	-	28
Other current assets	136	24	(5)	155
Property, plant and equipment, net	167	6	-	173
Other assets	1,122	94	(842)	374
Total assets	<u>\$3,398</u>	<u>\$528</u>	<u>\$(847)</u>	<u>\$3,079</u>
Liabilities and stockholders' equity:				
Other current liabilities	\$102	\$35	\$(5)	\$132
Current portion of deferred contract revenue	100	-	-	100
0.125% convertible senior notes	445	-	-	445
1% convertible senior notes	284	-	-	284
Long-term obligations, less current portion	61	14	-	75
Long-term deferred contract revenue	451	-	(3)	448
Total stockholders' equity before noncontrolling interest	1,955	479	(1,057)	1,377
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	218	218
Total stockholders' equity	<u>\$1,955</u>	<u>\$479</u>	<u>\$(839)</u>	<u>\$1,595</u>
Total liabilities and stockholders' equity	<u>\$3,398</u>	<u>\$528</u>	<u>\$(847)</u>	<u>\$3,079</u>

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