

Ionis reports first quarter 2020 financial results and recent business achievements

May 6, 2020

On track to achieve 2020 financial guidance Webcast today, May 6, 2020, at 11:30 a.m. Eastern Time

CARLSBAD, Calif., May 6, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today reported its financial results for the first quarter of 2020 and recent business highlights.

"We achieved numerous value-driving catalysts in the first quarter, setting us up to achieve our strategic objectives for the year. Our commercial medicines continued to perform well, led by SPINRAZA. Our late-stage pipeline continues to advance, and we are particularly pleased that the tominersen Phase 3 study is fully enrolled, bringing us closer to potentially delivering this medicine to patients with Huntington's disease. Additionally, AKCEA-APO(a)-L_{Rx} was granted fast track designation in the U.S., underscoring the significant value this medicine could deliver to the millions of patients with Lp(a)-driven cardiovascular disease," said Brett P. Monia, chief executive officer at Ionis. "Thanks to the commitment and resilience of our employees, we delivered strong first quarter results while effectively managing the challenges inherent with the global COVID-19 pandemic, keeping us on track to achieve our 2020 goals. We plan to initiate the pivotal study for AKCEA-APOCIII-L_{Rx} in patients with FCS, bringing us to six pivotal studies underway this year. We also plan to refile the WAYLIVRA U.S. NDA and report additional proof-of-concept data from several of our programs this year. Our significant financial strength enables us to invest in our highest priorities, including advancing our Ionis-owned pipeline and our technology, and strengthening our commercial capabilities. Together, these achievements keep us positioned to deliver NDAs for ten or more of our medicines through 2025."

Financial Results and Highlights

"We are reaffirming our 2020 financial guidance, including ending 2020 meaningfully profitable. We expect our results to be driven by continued significant commercial revenue and R&D revenue from numerous programs," said Elizabeth L. Hougen, chief financial officer of Ionis. "We remain well-capitalized, with a strong balance sheet and \$2.4 billion in cash and investments. Enabled by our financial strength, we have the resources to execute on our near- and longer-term strategic priorities, even in the challenging COVID-19 pandemic environment."

- Growing commercial revenues combined with a substantial base of R&D revenues
 - Commercial revenue from SPINRAZA[®] (nusinersen) royalties increased by more than 10 percent to \$66 million compared to Q1 2019
 - Product sales from TEGSEDI[®] (inotersen) and WAYLIVRA[®] (volanesorsen) more than doubled to \$15 million compared to Q1 2019
 - R&D revenue of \$49 million included over \$25 million from Ionis' neurology disease franchise and \$15 million from its cardiometabolic franchise
- First quarter results in line with projections to be meaningfully profitable this year
 - Operating loss and net loss of \$61 million and \$48 million, respectively, on a GAAP basis
 - Non-GAAP operating loss and net loss of \$20 million and \$15 million, respectively
- Cash position of \$2.4 billion provides substantial financial strength to continue executing on strategic goals

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
 - Worldwide sales increased to \$565 million in the first quarter, a 9 percent increase compared to the first quarter of 2019
 - Worldwide patients on treatment increased to approximately 10,800 at the end of the first quarter, including patients across commercial, expanded access and clinical trial settings
 - Patient treatment is underway in the Phase 2/3 DEVOTE study evaluating the safety, tolerability and potential to achieve even greater efficacy with a higher dose of SPINRAZA
 - Data from an independent study published in *Lancet Neurology* demonstrated statistically significant improvement in motor function with SPINRAZA treatment in teens and adults
- TEGSEDI: launched in multiple markets for the treatment of hereditary transthyretin amyloidosis (hATTR) with polyneuropathy in adult patients
 - Commercially available in 12 countries
 - Launching in additional EU countries this year and expanding in Latin America through PTC Therapeutics
 - Results from the NEURO-TTR Phase 3 open-label extension study were published in the *European Journal of Neurology*
- WAYLIVRA: launched in the EU as the only approved treatment for adults with genetically confirmed familial chylomicronemia syndrome (FCS) at high risk for pancreatitis
 - Launch progressing in Germany, Austria and through the ATU in France
 - Launching in additional EU countries this year

Pipeline Highlights

- Roche completed enrollment in the global, GENERATION HD1 Phase 3 study in patients with Huntington's disease
- Initiated the CARDIO-TTRansform Phase 3 clinical trial for AKCEA-TTR-L_{Rx} in patients with TTR-mediated amyloid cardiomyopathy
- Two medicines granted Fast Track Designation by the U.S. FDA
 - AKCEA-APO(a)-L_{Rx} for the treatment of cardiovascular disease due to elevated Lp(a) levels
 - IONIS-C9_{Rx} for the treatment of C9orf72-ALS
- Ionis generated more than \$20 million as numerous partnered medicines advanced
 - \$10 million from AstraZeneca for ION532, targeting APOL1 for the treatment of kidney disease
 - \$7.5 million from Biogen for IONIS-MAPT_{Rx} for the treatment of Alzheimer's disease
 - \$5 million from Dynacure for IONIS-DNM2-2.5_{Rx} for the treatment of centronuclear myopathies
- Ionis and Akcea reported positive topline results for AKCEA-APOCIII-L_{Rx} and vupanorsen (AKCEA-ANGPTL3-L_{Rx})
- Results from the Phase 2 study of AKCEA-APO(a)-L_{Rx} in patients with Lp(a)-driven cardiovascular disease, highlighting the favorable safety and tolerability profile and the potential to address a major area of unmet need, were published in the *New England Journal of Medicine*
- Initiated a Phase 1 study of ION224, an Ionis-owned medicine in development for the treatment of NASH

Upcoming Catalysts

- Initiate the Phase 3 study of AKCEA-APOCIII-L_{Rx} in patients with FCS
- Refile WAYLIVRA new drug application for U.S. marketing authorization
- File for WAYLIVRA marketing approval in Brazil with PTC Therapeutics
- Report clinical proof-of-concept results for four or more programs
- Initiate a first-in-human study of ION541 in patients with sporadic ALS, conducted by Biogen
- Advance five or more new medicines into development, including several Ionis-owned medicines

Revenue

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

	Three months ended, March 31,	
	2020	2019
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$66	\$60
Product sales, net	15	7
Licensing and royalty revenue	3	1
Total commercial revenue	84	68
R&D Revenue:		
Amortization from upfront payments	21	36
Milestone payments	23	40
License fees	-	150
Other services	5	3
Total R&D revenue	49	229
Total revenue	\$133	\$297

R&D revenue in the first quarter of 2019 included \$185 million from two large items, including \$150 million for the license of AKCEA-APO(a)-L_{Rx}.

Operating Expenses

Operating expenses increased for the first quarter of 2020, compared to the same period in 2019, principally due to Ionis' investments in the global launches of TEGSEDI and WAYLIVRA, the Phase 3 program for AKCEA-TTR-L_{Rx} and the Company's Ionis-owned pipeline.

Income Tax Expense (Benefit)

Ionis recorded an income tax benefit in the first quarter of 2020, compared to income tax expense in the same period in 2019. Ionis recorded an income tax benefit in the first quarter of 2020 because it generated a pre-tax loss.

Net (Income) Loss Attributable to Noncontrolling Interest in Akcea

At March 31, 2020, 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 (income) loss attributable to noncontrolling interest in Akcea" on Ionis' statement of operations. Ionis recognized a net loss attributable to noncontrolling interest in Akcea in the first quarter of 2020 compared to net income in the first quarter of 2019. Net income attributable to noncontrolling interest in Akcea in the first quarter of 2019 was due to the significant license fee revenue

Akcea earned when Novartis licensed AKCEA-APO(a)-LR_x.

Net Income (Loss) Attributable to Ionis Common Stockholders

Ionis' net loss attributable to Ionis' common stockholders for the first quarter of 2020 was primarily due to Ionis' investments in advancing its strategic priorities. Ionis' net income attributable to Ionis' common stockholders for the first quarter of 2019 was primarily due to the \$150 million in revenue the Company earned when Novartis licensed AKCEA-APO(a)-LR_x combined with lower operating expenses compared to the first quarter of 2020.

Balance Sheet

Ionis ended the first quarter of 2020 with cash, cash equivalents and short-term investments of \$2.4 billion, nearly flat compared to \$2.5 billion at December 31, 2019. During the first quarter of 2020, Ionis repurchased 1.5 million shares of its common stock under its share repurchase program for a total purchase price of \$91 million.

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, cardiovascular, infectious, and pulmonary diseases.

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, 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, 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 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	
	March 31,	
	2020	2019
	(unaudited)	
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$66	\$60
Product sales, net	15	7
Licensing and royalty revenue	3	1
Total commercial revenue	84	68
Research and development revenue under collaborative agreements	49	229
Total revenue	133	297
Expenses:		
Cost of products sold	3	1

Research, development and patent	116	107
Selling, general and administrative	75	68
Total operating expenses	194	176
Income (loss) from operations	(61)	121
Other income (expense):		
Investment income	10	12
Interest expense	(11)	(12)
Income (loss) before income tax benefit (expense)	(62)	121
Income tax benefit (expense)	3	(31)
Net income (loss)	<u>\$(59)</u>	<u>\$90</u>
Net (income) loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	11	(6)
Net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders	<u>\$(48)</u>	<u>\$84</u>
Basic net income (loss) per share	<u>\$(0.35)</u>	<u>\$0.63</u>
Diluted net income (loss) per share	<u>\$(0.35)</u>	<u>\$0.62</u>
Shares used in computing basic net income (loss) per share	139,429	138,582
Shares used in computing diluted net income (loss) per share	139,429	141,537

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

	Three months ended, March 31, 2020 (unaudited)			
	Ionis			
	<u>Ionis</u>	<u>Akcea</u>	<u>Eliminations</u>	<u>Consolidated</u>
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$ \$66	\$-	\$ -	\$66
Product sales, net	-	15	-	15
Licensing and royalty revenue	3	-	-	3
Total commercial revenue	69	15	-	84
Research and development revenue under collaborative agreements	48	1	-	49
Intercompany revenue	1	-	(1)	-
Total revenue	118	16	(1)	133
Expenses:				
Cost of products sold	-	5	(2)	3
Research, development and patent expenses	99	17	-	116
Selling, general and administrative	29	46	-	75
Profit/ loss share for TEGSEDI commercialization activities	7	(7)	-	-
Total operating expenses	135	61	(2)	194
Loss from operations	(17)	(45)	1	(61)
Other income (expense):				
Investment income	9	1	-	10
Interest expense	(11)	-	-	(11)
Loss on early retirement of debt	-	-	-	-
Loss before income tax benefit	(19)	(44)	1	(62)
Income tax benefit	2	1	-	3
Net loss	<u>\$(17)</u>	<u>\$(43)</u>	<u>\$1</u>	<u>\$(59)</u>
Net loss attributable to noncontrolling interest in Akcea Therapeutics, Inc.	<u>\$-</u>	<u>\$-</u>	<u>\$11</u>	<u>\$11</u>
Net loss attributable to Ionis Pharmaceuticals, Inc. common stockholders	<u>\$(17)</u>	<u>\$(43)</u>	<u>\$12</u>	<u>\$(48)</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, March 31,	
	2020	2019
	(unaudited)	
As reported research, development and patent expenses according to GAAP	\$116	\$107
Excluding compensation expense related to equity awards	(26)	(25)
Non-GAAP research, development and patent expenses	<u>\$90</u>	<u>\$82</u>
As reported selling, general and administrative expenses according to GAAP	\$75	\$68
Excluding compensation expense related to equity awards	(15)	(21)
Non-GAAP selling, general and administrative expenses	<u>\$60</u>	<u>\$47</u>
As reported operating expenses according to GAAP	\$194	\$176
Excluding compensation expense related to equity awards	(41)	(46)
Non-GAAP operating expenses	<u>\$153</u>	<u>\$130</u>
As reported income (loss) from operations according to GAAP	\$(61)	\$121
Excluding compensation expense related to equity awards	(41)	(46)
Non-GAAP income (loss) from operations	<u>\$(20)</u>	<u>\$167</u>
As reported net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	\$(48)	\$84
Excluding compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	(39)	(42)
Income tax effect related to compensation expense related to equity awards attributable to Ionis Pharmaceuticals, Inc. common stockholders	6	(1)
Non-GAAP net income (loss) attributable to Ionis Pharmaceuticals, Inc. common stockholders according to GAAP	<u>\$(15)</u>	<u>\$127</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)

	March 31, December 31,	
	2020	2019
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,384	\$2,500
Contracts receivable	29	63
Other current assets	145	158
Property, plant and equipment, net	164	154
Other assets	369	358
Total assets	<u>\$3,091</u>	<u>\$3,233</u>
Liabilities and stockholders' equity:		
Other current liabilities	\$137	\$155
Current portion of deferred contract revenue	121	118
0.125% convertible senior notes	440	435
1% convertible senior notes	280	275
Long-term obligations, less current portion	75	75
Long-term deferred contract revenue	468	490
Total Ionis stockholders' equity	1,360	1,471
Noncontrolling interest in Akcea Therapeutics, Inc.	210	214
Total stockholders' equity	<u>1,570</u>	<u>1,685</u>
Total liabilities and stockholders' equity	<u>\$3,091</u>	<u>\$3,233</u>

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

	March 31, 2020 (unaudited)			
	Ionis AkceaEliminations			Ionis Consolidated
Assets:				
Cash, cash equivalents and short-term investments	\$1,963	\$421	\$-	\$2,384
Contracts receivable	17	12	-	29
Other current assets	121	26	(2)	145
Property, plant and equipment, net	159	5	-	164
Other assets	1,116	96	(843)	369
Total assets	\$3,376	\$560	\$(845)	\$3,091
Liabilities and stockholders' equity:				
Other current liabilities	104	35	(2)	137
Current portion of deferred contract revenue	120	1	-	121
0.125% convertible senior notes	440	-	-	440
1% convertible senior notes	280	-	-	280
Long-term obligations, less current portion	61	14	-	75
Long-term deferred contract revenue	470	-	(2)	468
Total stockholders' equity before noncontrolling interest	1,901	510	(1,051)	1,360
Noncontrolling interest in Akcea Therapeutics, Inc.	-	-	210	210
Total stockholders' equity	\$1,901	\$510	\$(841)	\$1,570
Total liabilities and stockholders' equity	\$3,376	\$560	\$(845)	\$3,091

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SOURCE Ionis Pharmaceuticals, Inc.

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