



Ionis reports first quarter 2024 financial results

May 7, 2024

WAINUA™ launch on track; EU and Canada approval decisions expected this year

Olezarsen NDA submitted to FDA for FCS; preparing EU regulatory submission

Positive Phase 3 donidalorsen data for HAE; preparing regulatory submissions

On track to achieve 2024 financial guidance

CARLSBAD, Calif., May 7, 2024 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) (the "Company"), today reported financial results for the first quarter ended March 31, 2024.

"Ionis is off to a great start in 2024, as we continue to execute on our vision to bring better futures to people with serious diseases. The WAINUA launch for hereditary ATTR polyneuropathy is progressing well with AstraZeneca. And we are one step closer to our first independent launch with our NDA submission for olezarsen, which is supported by robust data positioning olezarsen to make a profound difference for people with FCS," said Brett P. Monia, Ph.D., chief executive officer of Ionis. "We look forward to presenting positive Phase 3 donidalorsen data, along with data from our open-label extension and 'switch' studies in patients with HAE at EAACI later this month, setting the stage for Ionis' second independent launch. Additionally, we have multiple upcoming data readouts from our mid-stage programs that, if positive, could advance into Phase 3 development, further strengthening our ability to deliver a steady cadence of potentially transformational medicines for years to come."

First Quarter 2024 Summary Financial Results⁽¹⁾:

	Three months ended March 31,	
	2024	2023
	(amounts in millions)	
Total revenue	\$119	\$131
Operating expenses	\$269	\$245
Operating expenses on a non-GAAP basis	\$238	\$218
Loss from operations	(\$150)	(\$114)
Loss from operations on a non-GAAP basis	(\$119)	(\$87)

(1) Reconciliation of GAAP to non-GAAP basis contained later in this release.

Financial Highlights

- Revenue for the first quarter of 2024 earned from numerous diverse sources, including a new source of royalty revenue with the launch of WAINUA in the U.S.
- Continued strategic investments to bring WAINUA, olezarsen and donidalorsen to patients drove increased operating expenses in the first quarter of 2024 compared to the same period last year
- Cash and short-term investments of \$2.2 billion as of March 31, 2024 enable continued investments to drive increasing value, including supporting our planned upcoming launches
- Reaffirmed 2024 financial guidance

Recent Marketed Medicines Highlights

- WAINUA for the treatment of adults with polyneuropathy of hereditary transthyretin-mediated amyloidosis (ATTRv-PN) generated sales of \$5 million in the first partial quarter of launch resulting in royalty revenue of \$1 million for Ionis in the first quarter of 2024
- SPINRAZA for the treatment of spinal muscular atrophy (SMA) generated global sales of \$341 million resulting in royalty revenue of \$38 million in the first quarter of 2024
 - Biogen presented new positive neurofilament light chain (NfL) biomarker data from the Phase 4 RESPOND study of SMA patients adding further evidence supporting the potential benefit of SPINRAZA in infants and toddlers who had unmet medical needs after treatment with gene therapy

Recent Late-Stage Pipeline Highlights

- Eplontersen granted Fast Track designation by the FDA for the treatment of patients with ATTR cardiomyopathy
- Olezarsen achieved multiple milestones advancing it closer to potentially addressing two distinct populations of patients with urgent unmet need, familial chylomicronemia syndrome (FCS) and severe hypertriglyceridemia (sHTG):
 - Submitted NDA to the FDA for FCS
 - Presented positive Phase 3 Balance study data in patients with FCS with a simultaneous publication in the *New England Journal of Medicine*
 - Presented positive Phase 2b Bridge study data in patients with HTG and sHTG with a simultaneous publication in the *New England Journal of Medicine*
 - Opened Expanded Access Program (EAP) for FCS in the U.S.
 - Granted Breakthrough Therapy and Orphan Drug designations by the FDA for the treatment of patients with FCS
 - Completed enrollment of the Phase 3 CORE pivotal study and ESSENCE supportive exposure study for sHTG; CORE2 confirmatory pivotal study on track to fully enroll mid-year
- Donidalorsen achieved multiple milestones advancing it closer to potentially becoming a first-in-class RNA-targeted prophylactic treatment for people with hereditary angioedema (HAE):
 - Reported positive topline data from the Phase 3 OASIS-HAE study in patients treated every four weeks or every eight weeks; preparing to submit NDA
 - Opened EAP for HAE in the U.S.
 - Granted Orphan Drug designation by EMA
- Bepirovirsen granted Fast Track designation by the FDA for the treatment of patients with chronic hepatitis B (CHB)

Recent Other Pipeline Highlights

- Reported positive Phase 2 data for ION224 (DGAT2) in patients with metabolic dysfunction-associated steatohepatitis (MASH)
- Initiated the Phase 1/2 Orbit study of ION356 (PLP1) in patients with Pelizaeus-Merzbacher disease (PMD)

First Quarter 2024 Financial Results

"Our first quarter results keep us on track to achieve our 2024 financial guidance. With the launch of WAINUA in the U.S. underway, we are excited to add WAINUA royalties to our meaningful revenues in the first quarter. We believe WAINUA is uniquely positioned in this growing market to address the needs of ATTRv-PN patients who remain significantly underserved, especially as it is the only approved medicine with monthly dosing that can be self-administered via an auto injector," said Elizabeth L. Hougen, chief financial officer of Ionis. "We continued to invest our capital resources in our near-term commercial opportunities, wholly owned pipeline and technology. We expect our modest expense growth this year to be driven by our activities to support the WAINUA launch and planned launches for olezarsen and donidalorsen with R&D expenses approaching steady state as several late-stage studies have recently ended. We believe the investments we are making today and plan to make over the next few years position Ionis to drive increasing value for patients and stakeholders."

Revenue

Ionis' revenue was comprised of the following:

	Three months ended	
	March 31,	
	2024	2023
Revenue:	(amounts in millions)	
Commercial revenue:		
SPINRAZA royalties	\$38	\$50
WAINUA royalties	1	-
Other commercial revenue:		
TEGSEDI and WAYLIVRA revenue, net	9	7
Licensing and other royalty revenue	11	11
Total commercial revenue	59	68
Research and development revenue:		
Amortization from upfront payments	42	16
Milestone payments	7	23
Collaborative agreement revenue	49	39
WAINUA joint development revenue	11	24
Total research and development revenue	60	63
Total revenue	\$119	\$131

Commercial revenue in the first quarter of 2024 included a new source of royalty revenue with the launch of WAINUA in the U.S. during the first quarter of 2024. While the number of patients on SPINRAZA treatment remained consistent globally, royalties decreased year over year primarily due to the timing of shipments in several markets outside the U.S. Ionis' commercial revenue in the first quarter of 2024 also included royalties from the net sales of QALSODY, which Biogen launched in the second quarter of 2023.

R&D revenue in the first quarter of 2024 included increased revenue from the amortization of upfront payments compared to the same period last year due to the new collaborations Ionis entered into last year with Roche and Novartis. This increase was offset by decreases in milestone payments due to timing and WAINUA joint development revenue, which decreased as development activities relating to ATTRv-PN wound down with the launch of WAINUA underway.

Operating Expenses

Ionis' operating expenses increased in the first quarter of 2024 compared to the same period in 2023, consistent with expectations. SG&A expenses increased year over year primarily due to the launch of WAINUA in the U.S. and launch preparation activities for olezarsen and donidalorsen. R&D expenses increased compared to the same period last year due to the timing of development activities and are expected to stabilize in 2024 as several late-stage studies have ended.

Balance Sheet

As of March 31, 2024, Ionis' cash, cash equivalents and short-term investments decreased to \$2.2 billion compared to \$2.3 billion at December 31, 2023. As the year progresses, the Company plans to continue deploying its capital resources toward growth opportunities. Ionis' working capital also decreased over the same period primarily due to the Company's lower cash and short-term investments balance.

Webcast

Management will host a conference call and webcast to discuss Ionis' first quarter 2024 results at 11:30 a.m. Eastern time on Tuesday, May 7, 2024. Interested parties may access the webcast [here](#). A webcast replay will be available for a limited time at the same address. To access the Company's first quarter 2024 earnings slides click [here](#).

For more information about SPINRAZA and QALSODY, visit <https://www.spinraza.com/> and <https://www.qalsody.com/>, respectively. QALSODY is approved under accelerated approval based on reduction in plasma neurofilament light chain (NfL) observed in patients treated with QALSODY. Continued approval may be contingent upon verification of clinical benefit in confirmatory trial(s).

INDICATION for WAINUA™ (eplontersen)

WAINUA injection, for subcutaneous use, 45 mg is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults.

IMPORTANT SAFETY INFORMATION for WAINUA™(eplontersen)

WARNINGS AND PRECAUTIONS

Reduced Serum Vitamin A Levels and Recommended Supplementation WAINUA leads to a decrease in serum vitamin A levels. Supplement with recommended daily allowance of vitamin A. Refer patient to an ophthalmologist if ocular symptoms suggestive of vitamin A deficiency occur.

ADVERSE REACTIONS

Most common adverse reactions (≥9% in WAINUA-treated patients) were vitamin A decreased (15%) and vomiting (9%).

Please see link to [U.S. Full Prescribing Information](#) for WAINUA.

About Ionis Pharmaceuticals, Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has five marketed medicines and a leading pipeline in neurology, cardiology, and other areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients. To learn more about Ionis, visit ionispharma.com and follow us on [X \(Twitter\)](#) and [LinkedIn](#).

Ionis' Forward-looking Statement

This press release includes forward-looking statements regarding Ionis' business, financial guidance and the therapeutic and commercial potential of our commercial medicines, additional medicines in development and technologies. 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 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. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. 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, 2023, and most recent Form 10-Q, which are on file with the Securities and Exchange Commission. 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" all refer to Ionis Pharmaceuticals and its subsidiaries.

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

	Three months ended, March 31,	
	2024	2023
	(unaudited)	
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$38	\$50
WAINUA royalties	1	-
Other commercial revenue	20	18
Total commercial revenue	59	68
Research and development revenue:		
Collaborative agreement revenue	49	39
WAINUA joint development revenue	11	24
Total research and development revenue	60	63
Total revenue	119	131
Expenses:		
Cost of sales	2	1
Research, development and patent	214	198
Selling, general and administrative	53	46
Total operating expenses	269	245
Loss from operations	(150)	(114)
Other income (expense):		
Interest expense related to the sale of future royalties	(18)	(16)
Other income, net	25	17
Loss before income tax expense	(143)	(113)
Income tax expense	-	(11)
Net loss	(\$143)	(\$124)
Basic and diluted net loss per share	(\$0.98)	(\$0.87)
Shares used in computing basic and diluted net loss per share	146	143

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

	Three months ended March 31,	
	2024	2023
	(unaudited)	
As reported research, development and patent expenses according to GAAP	\$214	\$198
Excluding compensation expense related to equity awards	(22)	(20)
Non-GAAP research, development and patent expenses	\$192	\$178
As reported selling, general and administrative expenses according to GAAP	\$53	\$46
Excluding compensation expense related to equity awards	(9)	(7)

Non-GAAP selling, general and administrative expenses	<u>\$44</u>	<u>\$39</u>
As reported operating expenses according to GAAP	\$269	\$245
Excluding compensation expense related to equity awards	<u>(31)</u>	<u>(27)</u>
Non-GAAP operating expenses	<u>\$238</u>	<u>\$218</u>
As reported loss from operations according to GAAP	(\$150)	(\$114)
Excluding compensation expense related to equity awards	<u>(31)</u>	<u>(27)</u>
Non-GAAP loss from operations	<u>(\$119)</u>	<u>(\$87)</u>
As reported net loss according to GAAP	(\$143)	(\$124)
Excluding compensation expense related to equity awards and related tax effects	<u>(31)</u>	<u>(27)</u>
Non-GAAP net loss	<u>(\$112)</u>	<u>(\$97)</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 loss from operations, and non-GAAP net loss were adjusted from GAAP to exclude compensation expense related to equity awards and the related tax effects. Compensation expense related to equity awards are non-cash. 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, 2024	December 31, 2023
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,206	\$2,331
Contracts receivable	5	98
Other current assets	204	213
Property, plant and equipment, net	73	71
Right-of-use assets	169	172
Other assets	107	105
Total assets	<u>\$2,764</u>	<u>\$2,990</u>
Liabilities and stockholders' equity:		
Current portion of deferred contract revenue	\$130	\$151
0.125% convertible senior notes, net – short-term	44	44
Other current liabilities	153	253
1.75% convertible senior notes, net	563	562
0% convertible senior notes, net	626	625
Liability related to sale of future royalties, net	525	514
Long-term lease liabilities	169	171
Long-term obligations, less current portion	42	42
Long-term deferred contract revenue	215	241
Total stockholders' equity	297	387
Total liabilities and stockholders' equity	<u>\$2,764</u>	<u>\$2,990</u>

Key 2024 Value Driving Events⁽¹⁾

New Product Launches		
Program	Indication	Achieved
WAINUA	ATTRv-PN	•
Olezarsen	FCS	
QALSODY (EU)	SOD1-ALS	

Regulatory Actions			
Program	Indication	Regulatory Action	Achieved
Eplontersen	ATTRv-PN	Additional OUS filings	•

		EMA approval decision	
		Additional OUS approval decision(s)	
Olezarsen	FCS	NDA filing	
		FDA approval decision	
		EU filing	
		Canada filing	
Donidalorsen	HAE	NDA filing	
QALSODY	SOD1-ALS	EMA approval decision	

Key Phase 3 Clinical Data Events			
Program	Indication	Event	Achieved
Olezarsen	FCS	Balance study full data	•
Donidalorsen	HAE	OASIS-HAE topline data	•
Donidalorsen	HAE	OASIS-HAE full data	
Donidalorsen	HAE	OASIS-Plus: OLE + Switch data	

Key Phase 2 Clinical Data Events			
Program	Indication	Event	Achieved
Donidalorsen	HAE	3-year Phase 2 OLE data	
IONIS-FB-L _{Rx}	IgAN	Phase 2 data	
IONIS-FB-L _{Rx}	GA	GOLDEN study data	
ION224 (DGAT2)	NASH	Phase 2 data	•
ION582 (UBE3A)	Angelman syndrome	HALOS study data	
ION541 (ATXN2)	ALS	ALSpire study data	

(1) Timing expectations based on current assumptions and subject to change.

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