



## Ionis reports first quarter 2023 financial results

May 3, 2023

*QALSODY approved for SOD1-ALS; MAA under review in EU*

*Reported positive Phase 3 eplontersen ATTRv-PN data; December 22, 2023 PDUFA date*

*On track to achieve 2023 financial guidance*

CARLSBAD, Calif., May 3, 2023 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) (the "Company"), today reported financial results for the first quarter of 2023. Financial results are summarized below:



	Three months ended March 31,	
	2023	2022
	(amounts in millions)	
Total revenue	\$131	\$142
Operating expenses	\$245	\$199
Operating expenses on a non-GAAP basis	\$218	\$173
Loss from operations	(\$114)	(\$57)
Loss from operations on a non-GAAP basis	(\$87)	(\$31)

### Financial Highlights

- Revenue for the first quarter of 2023 was in line with expectations and included revenue from numerous diverse sources
- Operating expenses increased in the first quarter of 2023 compared to the prior year as planned, reflecting investments in advancing Ionis' pipeline, technology and go-to-market activities for eplontersen, olezarsen and donidalorsen
- Cash and short-term investments of \$2.3 billion at March 31, 2023 enables continued investment in creating future growth opportunities
- Reaffirmed 2023 financial guidance

"2023 is off to a strong start. With QALSODY's approval, it joins SPINRAZA as a new groundbreaking medicine to treat a devastating neurological disease, further validating our RNA-targeting therapeutic platform. We also achieved another important milestone with our recent positive eplontersen Phase 3 data. We believe the positive efficacy and safety data, and the attractive self-administered dosing profile, position eplontersen to be an important treatment for ATTRv-PN patients, who today are underserved. We look forward to the first potential approval of eplontersen in the U.S. in December," said Brett P. Monia, Ph.D.,

chief executive officer of Ionis. "In addition, we further expanded our industry-leading late-stage pipeline to seven programs across nine indications following the start of GSK's bepirovirsen hepatitis B program. In the second half, we plan to report results from our olezarsen Phase 3 FCS study, which if positive, positions us for our first independent commercial launch. These recent achievements, together with our upcoming milestones, continue to build value for Ionis stakeholders."

### Recent Late-Stage Pipeline Highlights

- FDA granted Biogen accelerated approval of QALSODY (tofersen) for patients with SOD1-ALS
- FDA accepted eplontersen NDA for patients with polyneuropathy caused by hereditary TTR amyloidosis (ATTRv-PN) with a PDUFA date of December 22, 2023
- Presented positive week-35 and week-66 data from the Phase 3 NEURO-TTRansform study of eplontersen in patients with ATTRv-PN
- GSK advanced bepirovirsen into Phase 3 development in patients with chronic hepatitis B

### Recent Additional Pipeline Updates

- Biogen presented data demonstrating IONIS-MAPT<sub>Rx</sub> (BIIB080) substantially reduced tau protein in patients with early-stage Alzheimer's disease
- Continued to focus R&D efforts by discontinuing two programs that did not meet Ionis' target product profile, cimdelsen for acromegaly and sapablursen for beta-thalassemia. Ionis continues to advance the Phase 2 sapablursen study for polycythemia vera

### First Quarter 2023 Financial Results

"Our first quarter results were in line with our expectations. We generated meaningful revenue while continuing to invest in key growth opportunities across our business. These results keep us on track to achieve our 2023 financial guidance," said Elizabeth L. Hougen, chief financial officer of Ionis. "We plan to continue investing in areas with the greatest potential to drive growth. As such, we expect our investments to grow modestly as we advance and expand our late-stage pipeline and move our near-term commercial opportunities toward the market. Additionally, as we keep more programs for ourselves, we expect a greater proportion of commercial revenues compared to R&D revenues, and our commercial revenues to be the primary driver of future revenue growth."

### Revenue

Ionis' revenue was comprised of the following:

	Three months ended	
	March 31,	
	2023	2022
	(amounts in millions)	
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$50	\$54
TEGSEDI and WAYLIVRA revenue, net	7	6
Licensing and royalty revenue	11	12
Total commercial revenue	68	72
Research and development revenue:		
Amortization from upfront payments	16	17
Milestone payments	23	27
License fees	-	2
Other services	-	4
Collaborative agreement revenue	39	50
Eplontersen joint development revenue	24	20
Total research and development revenue	63	70
Total revenue	\$131	\$142

Ionis continued to derive its revenue for the first quarter of 2023 from diverse sources, with approximately half coming from commercial products and half from numerous partnered programs. Commercial revenue for the first quarter of 2023 included \$50 million from SPINRAZA royalties. Global SPINRAZA product sales of \$443 million decreased six percent in the first quarter of 2023, compared to the same period last year primarily due to the impact from foreign currency, fewer new patient starts in the U.S. and channel dynamics.

R&D revenue for the first quarter of 2023 included \$24 million from AstraZeneca for its share of the global Phase 3 development costs for eplontersen, \$20 million from Biogen for advancing several neurology disease programs and \$15 million from GSK for advancing bepirovirsen into Phase 3 development. Already in the second quarter, the Company earned \$16 million in a milestone payment from Biogen when QALSODY was approved in the U.S.

## Operating Expenses

Ionis' operating expenses increased in the first quarter of 2023 compared to the same period in 2022, consistent with expectations. As Ionis advanced its robust pipeline, study costs increased as most of the Company's Phase 3 studies were either fully enrolled or approaching full enrollment resulting in higher R&D expenses year over year. Additionally, as Ionis prepares to launch eplontersen, olezarsen and donidalorsen, the Company's SG&A expenses also increased year over year.

## Balance Sheet

As of March 31, 2023, Ionis' cash, cash equivalents and short-term investments increased to \$2.3 billion compared to \$2.0 billion at December 31, 2022 primarily due to the \$500 million Ionis received from Royalty Pharma in January 2023. Ionis' working capital also increased over the same period primarily due to the Company's higher cash and short-term investments balance. Additionally, the Company recorded a long-term liability for future royalties due to Royalty Pharma in the first quarter of 2023.

## Webcast

Management will host a conference call and webcast to discuss Ionis' first quarter 2023 results at 11:30 a.m. Eastern time on Wednesday, May 3, 2023. 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 2023 earnings slides click [here](#).

## About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been a leader in RNA-targeted therapy, pioneering new markets and changing standards of care. Ionis currently has four marketed medicines and a promising late-stage pipeline highlighted by cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop and deliver life-transforming therapies.

To learn more about Ionis visit [www.ionispharma.com](http://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 QALSODY (tofersen), SPINRAZA (nusinersen), TEGSEDI (inotersen), WAYLIVRA (volanesorsen), eplontersen, olezarsen, donidalorsen, ION363, pelacarsen, bepirovirsen, Ionis' technologies and Ionis' other products in development. 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. 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, 2022, which is on file with the Securities and Exchange Commission. 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" all refer to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals® is a registered 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. QALSODY™ is a trademark of Biogen. 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,	
	2023	2022
Revenue:		
Commercial revenue:		
SPINRAZA royalties	\$50	\$54

TEGSEDI and WAYLIVRA revenue, net	7	6
Licensing and royalty revenue	11	12
Total commercial revenue	68	72
Research and development revenue:		
Collaborative agreement revenue	39	50
Eplontersen joint development revenue	24	20
Total research and development revenue	63	70
Total revenue	131	142
Expenses:		
Cost of sales	1	4
Research, development and patent	198	161
Selling, general and administrative	46	34
Total operating expenses	245	199
Loss from operations	(114)	(57)
Other income (expense):		
Interest expense related to sale of future royalties	(16)	-
Other income (expense), net	17	(7)
Loss before income tax expense	(113)	(64)
Income tax expense	(11)	(1)
Net loss	(\$124)	(\$65)
Basic and diluted net loss per share	(\$0.87)	(\$0.46)
Shares used in computing basic and diluted net loss per share	143	142

**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, 2023 2022	
	(unaudited)	
<b>As reported research, development and patent expenses according to GAAP</b>	\$198	\$161
Excluding compensation expense related to equity awards	(20)	(19)
<b>Non-GAAP research, development and patent expenses</b>	<u>\$178</u>	<u>\$142</u>
<b>As reported selling, general and administrative expenses according to GAAP</b>	\$46	\$34
Excluding compensation expense related to equity awards	(7)	(7)
<b>Non-GAAP selling, general and administrative expenses</b>	<u>\$39</u>	<u>\$27</u>
<b>As reported operating expenses according to GAAP</b>	\$245	\$199
Excluding compensation expense related to equity awards	(27)	(26)
<b>Non-GAAP operating expenses</b>	<u>\$218</u>	<u>\$173</u>
<b>As reported loss from operations according to GAAP</b>	(\$114)	(\$57)
Excluding compensation expense related to equity awards	(27)	(26)
<b>Non-GAAP loss from operations</b>	<u>(\$87)</u>	<u>(\$31)</u>
<b>As reported net loss according to GAAP</b>	(\$124)	(\$65)
Excluding compensation expense related to equity awards and related tax effects	(27)	(26)
<b>Non-GAAP net loss</b>	<u>(\$97)</u>	<u>(\$39)</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, 2023	December 31, 2022
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,347	\$1,987
Contracts receivable	14	26
Other current assets	182	190
Property, plant and equipment, net	85	74
Right-of-use assets	179	182
Other assets	78	75
Total assets	<u>\$2,885</u>	<u>\$2,534</u>
Liabilities and stockholders' equity:		
Other current liabilities	\$167	\$221
Current portion of deferred contract revenue	92	91
0% convertible senior notes, net	623	622
0.125% convertible senior notes, net	545	545
Liability related to sale of future royalties, net	505	-
Long-term lease liabilities	177	178
Long-term obligations, less current portion	16	16
Long-term deferred contract revenue	273	288
Total stockholders' equity	<u>487</u>	<u>573</u>
Total liabilities and stockholders' equity	<u>\$2,885</u>	<u>\$2,534</u>

## 2023 Key Value Driving Events<sup>(1)</sup>

Regulatory Actions		
Program	Indication	Regulatory Action
QALSODY	SOD1-ALS	NDA approval (achieved)
		EU approval <sup>2</sup>
Eplontersen (TTR)	ATTRv polyneuropathy	NDA approval
		OUS filings

Key Clinical Achievements		
Program	Indication	Event
Eplontersen (TTR)	ATTRv polyneuropathy	Phase 3 data (week 35 & 66) (achieved)
Olezarsen	FCS	Phase 3 data
Eplontersen (TTR)	ATTR cardiomyopathy	Phase 3 full enrollment
Donidalorsen (PKK)	HAE	Phase 3 full enrollment

Phase 3 Initiations		
Program	Indication	Timing

Bepirovirsen (HBV)	Hepatitis B virus infection	H1:23 (achieved)
IONIS-FB-L <sub>Rx</sub>	Immunoglobulin A nephropathy	H1:23

- (1) Timing expectations based on current assumptions and subject to change.
- (2) CHMP opinion anticipated in Q4:2023.

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