

Ionis reports second quarter 2023 financial results

August 9, 2023

Reported Phase 3 data showing eplontersen continued to show improvement in ATTRv-PN through 85 weeks; December 22, 2023 PDUFA date Completed enrollment in donidalorsen OASIS-HAE study and eplontersen CARDIO-TTRansform ATTR-CM study keeping Phase 3 data readouts on track

On track to achieve 2023 financial guidance

CARLSBAD, Calif., Aug. 9, 2023 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) (the "Company"), today reported financial results for the second quarter of 2023. Financial results are summarized below:

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	Three months ended		Six months ended	
	June 30,	June 30,	June 30,	June 30,
	2023	2022	2023	2022
	(amounts in millions)			
Total revenue	\$188	\$134	\$319	\$276
Operating expenses	\$279	\$220	\$523	\$419
Operating expenses on a non-GAAP basis	\$252	\$195	\$469	\$368
Loss from operations	(\$91)	(\$86)	(\$204)	(\$143)
Loss from operations on a non-GAAP basis	(\$64)	(\$61)	(\$150)	(\$92)

Financial Highlights

- Revenue increased for the second quarter and first half of 2023 by 40% and 16% compared to the same periods last year, respectively, driven by significant partner payments
- Operating expenses increased in the second quarter and first half of 2023 compared to the prior year as planned, reflecting investments in advancing Ionis' pipeline and go-to-market activities for eplontersen, olezarsen and donidalorsen
- Cash and short-term investments of \$2.4 billion as of June 30, 2023 enables continued investments to drive increasing value
- Well-capitalized balance sheet reflects 2024 convertible note refinancing that extended maturity to 2028 while maintaining a low coupon and retaining the flexibility to mitigate potential equity dilution
- Reaffirmed 2023 financial guidance

"Ionis is creating significant value in 2023 as we successfully execute on our strategy to bring a steady cadence of transformational medicines to the market. Our growing late-stage pipeline now includes eight medicines for 10 indications, highlighted by eplontersen. Based on the strong and consistent data generated to date and an attractive self-administration profile, we expect eplontersen to be an important new medicine for people with ATTRv-polyneuropathy. We also added QALSODY to our commercial portfolio, a breakthrough treatment for people with SOD1-ALS further strengthening Ionis' leadership in RNA-based therapies for neurological diseases," said Brett P. Monia, Ph.D., chief executive officer of Ionis. "In the second half, we expect continued positive momentum including the Phase 3 readout for olezarsen in familial chylomicronemia syndrome and the potential approval of eplontersen for ATTRv-PN in December."

Recent Highlights From Commercial Medicines

- Biogen presented interim data from the Phase 4 RESPOND study of SMA patients demonstrating improved motor function in most participants treated with SPINRAZA who had unmet medical needs after treatment with gene therapy
- FDA granted Biogen accelerated approval of QALSODY (tofersen), a first-in-class medicine for patients with SOD1-ALS

Recent Highlights From Near-Term Commercial Opportunities

- Reported positive results from the Phase 3 NEURO-TTRansform study in patients with ATTRv-PN showing eplontersen continued to halt neuropathy disease progression and improve quality of life through 85 weeks
- Completed enrollment of the Phase 3 CARDIO-TTRansform study of eplontersen in patients with ATTR cardiomyopathy, the largest study ever conducted in ATTR-CM; on track for data readout as early as H1:2025
- Licensed eplontersen Latin America rights to AstraZeneca
- Completed enrollment of the Phase 3 OASIS-HAE study of donidalorsen in patients with hereditary angioedema; on track for data readout in H1:2024
- Reported positive topline Phase 2 open label extension data of donidalorsen in patients with hereditary angioedema treated for two years

Recent Highlights From Partnered Programs

- Roche advanced IONIS-FB-L_{Rx} into Phase 3 development in patients with immunoglobulin A nephropathy
- GSK presented durable response data from the Phase 2 B-Sure long-term follow-up study of bepirovirsen in complete responder patients from the Phase 2b B-Clear study of patients with HBV
- Completed enrollment in the Phase 2 GOLDEN study of IONIS-FB-L_{Rx} in patients with geographic atrophy
- AstraZeneca initiated a Phase 2b study of ION839 (AZD2693) targeting PNPLA3 to treat patients with NASH
- Entered collaboration with Novartis to advance a next generation program targeting Lp(a) for cardiovascular disease

Second Quarter 2023 Financial Results

"Our results for the first half of the year keep us on track to achieve our 2023 guidance. We continued to generate substantial and sustained revenue, that together with our well-capitalized balance sheet, allows us to continue investing in key opportunities across our business," said Elizabeth L. Hougen, chief financial officer of Ionis. "With three near-term commercial opportunities that have a combined multi-billion-dollar peak sales potential and a steady cadence of medicines poised to follow closely behind, we are positioned to drive substantial revenue growth and long-term value for shareholders."

Revenue

Ionis' revenue was comprised of the following:

	Three months ended		Six months ended	
	June 30,		June 30,	
	2023	2022	2023	2022
Revenue:	(amounts in millions)			
Commercial revenue:				
SPINRAZA royalties	\$61	\$60	\$111	\$113
Other commercial revenue:				
TEGSEDI and WAYLIVRA revenue, net	11	10	17	17
Licensing and royalty revenue	6	8	18	20
Total commercial revenue	78	78	146	150
Research and development revenue:				
Amortization from upfront payments	15	18	29	36
Milestone payments	51	18	74	45
License fees	20	-	20	2
Other services	4	3	6	6
Collaborative agreement revenue	90	39	129	89
Eplontersen joint development revenue	20	17	44	37
Total research and development revenue	110	56	173	126
Total revenue	\$188	\$134	\$319	\$276

Ionis' revenue increased in the second quarter and first half of 2023 compared to the same periods in 2022 because of increased payments from partnered programs. Ionis believes its substantial and sustainable revenue is an important source of funding that supports the Company's investments to bring potentially transformational medicines to the market.

Commercial revenue for the second quarter and first half of 2023 included \$61 million and \$111 million from SPINRAZA royalties, respectively. Global SPINRAZA product sales of \$437 million and \$880 million were essentially flat for the second quarter and first half of 2023, respectively, compared to the same periods last year reflecting SPINRAZA's resilience against emerging competition. Ionis' commercial revenue in the second quarter and first half of 2023 also included royalties from the U.S. launch of QALSODY.

R&D revenue essentially doubled for the second quarter of 2023 and increased more than 35% for the first half of 2023 compared to the same periods in 2022 reflecting the value Ionis' technology is creating as numerous partnered programs advanced.

Operating Expenses

Ionis' operating expenses increased in the second quarter and first half of 2023 compared to the same periods 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 June 30, 2023, Ionis' cash, cash equivalents and short-term investments increased to \$2.4 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. In the first quarter of 2023 the Company recorded a long-term liability for future royalties due to Royalty Pharma. In June 2023 Ionis issued \$575 million of senior convertible notes due in June 2028 with an interest rate of 1.75%. Ionis used the majority of the proceeds to repurchase \$434 million of its 2024 convertible notes. The Company plans to utilize the residual proceeds to settle the 2024 notes that remain outstanding.

Webcast

Management will host a conference call and webcast to discuss Ionis' second quarter 2023 results at 11:30 a.m. Eastern time on Wednesday, August 9, 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 second 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 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, ulefnersen, pelacarsen, bepirovirsen, IONIS-FB-L_{RX}, 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, 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.

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, Six months ended			
	June 30,		June 30,	
	2023	2022	2023	2022
	(unaudited)			
Revenue:				
Commercial revenue:				
SPINRAZA royalties	\$61	\$60	\$111	\$113
Other commercial revenue	17	18	35	37
Total commercial revenue	78	78	146	150
Research and development revenue:				
Collaborative agreement revenue	90	39	129	89
Eplontersen joint development revenue	20	17	44	37
Total research and development revenue	110	56	173	126
Total revenue	188	134	319	276
Expenses:				
Cost of sales	3	5	4	9
Research, development and patent	230	181	428	342
Selling, general and administrative	46	34	91	68
Total operating expenses	279	220	523	419
Loss from operations	(91)	(86)	(204)	(143)
Other income (expense):				
Interest expense related to the sale of future royalties:	(18)	-	(33)	-
Other income (expense), net	32	(17)	47	(24)
Loss before income tax expense	(77)	(103)	(190)	(167)

Income tax expense	(8)	(2)	(20)	(3)
Net loss	(\$85)	(\$105)	(\$210)	(\$170)
Basic and diluted net loss per share	(\$0.60)	(\$0.74)	(\$1.47)	(\$1.20)
Shares used in computing basic and diluted net loss per share	143	142	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		Six months ended	
	June 30, 2023	2022	June 30, 2023	2022
	(unaudited)			
As reported research, development and patent expenses according to GAAP	\$230	\$181	\$428	\$342
Excluding compensation expense related to equity awards	(19)	(19)	(39)	(38)
Non-GAAP research, development and patent expenses	<u>\$211</u>	<u>\$162</u>	<u>\$389</u>	<u>\$304</u>
As reported selling, general and administrative expenses according to GAAP	\$46	\$34	\$91	\$68
Excluding compensation expense related to equity awards	(7)	(6)	(14)	(13)
Non-GAAP selling, general and administrative expenses	<u>\$39</u>	<u>\$28</u>	<u>\$77</u>	<u>\$55</u>
As reported operating expenses according to GAAP	\$279	\$220	\$523	\$419
Excluding compensation expense related to equity awards	(27)	(25)	(54)	(51)
Non-GAAP operating expenses	<u>\$252</u>	<u>\$195</u>	<u>\$469</u>	<u>\$368</u>
As reported loss from operations according to GAAP	(\$91)	(\$86)	(\$204)	(\$143)
Excluding compensation expense related to equity awards	(27)	(25)	(54)	(51)
Non-GAAP loss from operations	<u>(\$64)</u>	<u>(\$61)</u>	<u>(\$150)</u>	<u>(\$92)</u>
As reported net loss according to GAAP	(\$85)	(\$105)	(\$210)	(\$170)
Excluding compensation expense related to equity awards and related tax effects	(27)	(25)	(54)	(51)
Non-GAAP net loss	<u>(\$58)</u>	<u>(\$80)</u>	<u>(\$156)</u>	<u>(\$119)</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)

	June 30,	December 31,
	2023	2022
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	\$2,385	\$1,987
Contracts receivable	28	26
Other current assets	203	190
Property, plant and equipment, net	92	74
Right-of-use assets	177	182

Other assets	86	75
Total assets	\$2,971	\$2,534

Liabilities and stockholders' equity:

Other current liabilities	\$192	\$221
Current portion of deferred contract revenue	96	91
1.75% convertible senior notes, net	561	-
0% convertible senior notes, net	624	622
0.125% convertible senior notes, net	114	545
Liability related to sale of future royalties, net	510	-
Long-term lease liabilities	175	178
Long-term obligations, less current portion	17	16
Long-term deferred contract revenue	254	288
Total stockholders' equity	428	573
Total liabilities and stockholders' equity	\$2,971	\$2,534

2023 Key Value Driving Events⁽¹⁾

Regulatory Actions			
Program	Indication	Regulatory Action	Achieved
QALSODY	SOD1-ALS	NDA approval	•
		EU approval ²	
Eplontersen (TTR)	ATTRv polyneuropathy	NDA approval	
		OUS filings	

Key Clinical Data Events			
Program	Indication	Event	Achieved
Eplontersen (TTR)	ATTRv polyneuropathy	Phase 3 data (week 35, 66 & 85)	•
Olezarsen (APOCIII)	FCS	Phase 3 data	
Donidalorsen (PKK)	HAE	Phase 2, OLE 1-year data	•
Donidalorsen (PKK)	HAE	Phase 2, OLE 2-year data	•

Enrollment Achievements			
Program	Indication	Event	Achieved
Eplontersen (TTR)	ATTR cardiomyopathy	Phase 3 full enrollment	•
Donidalorsen (PKK)	HAE	Phase 3 full enrollment	•
IONIS-FB-LRx	Geographic Atrophy	Phase 2 full enrollment	•

Phase 3 Initiations		
Program	Indication	Achieved
Bepirovirsen (HBV)	Hepatitis B virus infection	•
IONIS-FB-LRx	Immunoglobulin A nephropathy	•

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

(2) CHMP opinion anticipated in Q4:2023.

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