Ionis reports first quarter 2026 financial results and highlights progress on key programs

April 29, 2026

- TRYNGOLZA® (olezarsen) showed increasing demand in FCS driven by strong launch execution –

- Olezarsen sNDA accepted by the FDA for Priority Review; sHTG launch preparations on track –

– Increasing annual TRYNGOLZA peak net sales guidance to >$3B for sHTG –

- Zilganersen NDA for Alexander disease accepted for Priority Review, paving way for Ionis' first independent launch from leading neurology pipeline –

- Strong first-quarter performance and outlook for the year supports improved 2026 financial guidance -

CARLSBAD, Calif.--(BUSINESS WIRE)--Apr. 29, 2026--

Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) (the "Company") today reported financial results and provided key updates for the first quarter ended March 31, 2026.

"Ionis' strong performance in the first quarter of 2026 underscores the strength of our commercial and R&D engines. Our independent launches are increasingly contributing to revenue, driven by strong commercial execution, and we are on track for two additional groundbreaking independent launches in 2026 — olezarsen for severe hypertriglyceridemia, our first medicine for a broad patient population, and zilganersen for Alexander disease, the first launch from our leading neurology pipeline," said Brett P. Monia, Ph.D., chief executive officer of Ionis. "In addition, we look forward to multiple key value-driving events this year, including results from pivotal Phase 3 partnered programs. These include presentation of positive bepirovirsen data in chronic hepatitis B next month at EASL, as well as results from the landmark pelacarsen Lp(a) HORIZON and eplontersen CARDIO-TTRansform cardiovascular outcomes trials later this year."

First Quarter 2026 Summary Financial Results[(1)]:

	Three months ended March 31,	
	2026	2025
	(amounts in millions)	
Total revenue	$246	$132
Operating expenses	$364	$278
Operating expenses on a non-GAAP basis	$321	$249
Loss from operations	($118)	($146)
Loss from operations on a non-GAAP basis	($75)	($117)

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

First Quarter 2026 Financial Highlights

- Revenue increased 87% in the first quarter of 2026 compared to the same period last year, driven by continued commercial success. In addition, Ionis earned substantial R&D revenue, including $95 million in payments from both clinical and regulatory milestones from multiple partnerships
- Operating expenses for the quarter ended March 31, 2026 were in line with expectations and increased year over year primarily from investments related to the commercialization efforts for TRYNGOLZA and DAWNZERA as well as launch preparations for olezarsen in sHTG and zilganersen in Alexander disease
- Cash and short-term investments were $1.9 billion as of March 31, 2026. The change in cash and short-term investments from year end 2025 was primarily related to the $633 million the Company used for the maturity of the 0% convertible notes due on April 1, 2026
- Increasing annual olezarsen peak net sales guidance to >$3 billion from >$2 billion to reflect increasing confidence in the sHTG market opportunity for olezarsen

First Quarter 2026 Financial Results

"Ionis entered 2026 with strong momentum. We continued this momentum with the first quarter financial results reflecting increased commercial revenue from our independent launches and robust R&D revenue when compared to the same period last year," said Elizabeth L. Hougen, chief financial officer of Ionis. "Based on our strong year-to-date revenue performance, accelerating momentum and positive outlook for the rest of the year, we are improving our 2026 financial guidance. The strong performance we expect in 2026 will support substantial growth and long-term value creation and our goal of reaching cash-flow breakeven in 2028."

Recent Highlights - Wholly Owned Medicines

- TRYNGOLZA® (olezarsen), the first FDA-approved treatment for adults living with familial chylomicronemia syndrome (FCS) as an adjunct to diet
  - Generated U.S. net product sales of $27 million in the first quarter of 2026, reflecting continued strong demand, offset by a decrease in net price
  - Launch initiated in the European Union (EU) by Sobi
- Olezarsen on track to launch this year as a transformational medicine for severe hypertriglyceridemia (sHTG), assuming approval
  - sNDA accepted by the FDA for Priority Review for the treatment of sHTG with a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2026
  - The European Medicines Agency (EMA) accepted an indication extension application in March for the treatment of adult patients with sHTG
- DAWNZERA™ (donidalorsen), the first and only RNA-targeted prophylactic therapy for hereditary angioedema (HAE) in patients 12 years of age and older
  - Generated U.S. net product sales of $16 million in the first quarter of 2026, an increase of 125% versus the fourth quarter of 2025
  - Launch initiated in the EU by Otsuka
  - Positive one-year results from OASISplus open-label extension cohort published in the *Journal of Asthma and Allergy*
- Zilganersen on track to launch this year as the first and only medicine to demonstrate clinically meaningful and disease-modifying benefit in children and adults with Alexander disease (AxD), assuming approval
  - New Drug Application (NDA) for AxD accepted by FDA for Priority Review with PDUFA target action date of September 22, 2026
  - Expanded access program (EAP) underway
  - Positive additional results from the pivotal study presented at the American Academy of Neurology 2026 annual meeting

Recent Highlights – Partnered Medicines

- SPINRAZA® (nusinersen) for the treatment of spinal muscular atrophy (SMA) generated global sales of $374 million in the first quarter of 2026, resulting in royalty revenue of $44 million
  - SPINRAZA high dose regimen approved and launched in the U.S. and EU
- WAINUA® (eplontersen) (WAINZUA in EU) for the treatment of adults with polyneuropathy of hereditary transthyretin-mediated amyloidosis (ATTRv-PN) generated global sales of $51 million in the first quarter of 2026, resulting in royalty revenue of $11 million
  - Launches underway in numerous regions, including the EU and China; submissions in progress to expand WAINUA access globally
  - Phase 3 CARDIO-TTRansform study design and baseline characteristics to be presented at the Annual Congress of the Heart Failure Association of the ESC 2026
- Bepirovirsen, a potential first-in-class medicine for chronic hepatitis B (CHB), achieved the primary endpoint demonstrating a statistically significant and clinically meaningful functional cure rate in the B-Well 1 and B-Well 2 Phase 3 studies
  - GSK to present the positive Phase 3 data at the European Association for the Study of the Liver (EASL) Congress 2026
  - On track for a 2026 launch with global regulatory filings underway, assuming approval
    - NDA filing accepted by FDA for Priority Review with PDUFA date of October 26, 2026; granted Breakthrough Therapy designation
    - Accepted for regulatory review in EU, Japan, and China

Revenue

Ionis' revenue was comprised of the following:

	Three months ended March 31,	
	2026	2025
Revenue	(amounts in millions)	

Commercial revenue:		
Product sales, net:		
TRYNGOLZA sales, net	$ 27	$ 6
DAWNZERA sales, net	16	-
Total product sales, net	43	6
Royalty revenue:		
SPINRAZA royalties	44	48
WAINUA royalties	11	9
Other royalties	3	7
Total royalty revenue	58	64
Other commercial revenue	7	6
Total commercial revenue	**108**	**76**
Research and development revenue:		
Collaborative agreement revenue	120	46
WAINUA joint development revenue	18	10
Total research and development revenue	**138**	**56**
Total revenue	**$ 246**	**$ 132**

Commercial revenue for the first quarter ended March 31, 2026, increased 42%, compared to the same period in 2025. This increase was primarily driven by TRYNGOLZA and DAWNZERA product sales. Higher research and development revenue also contributed to the year-over-year revenue increase including approximately $95 million in milestone payments from multiple partnerships.

Operating Expenses

Operating expenses for the first quarter ended March 31, 2026, were driven from investments primarily related to commercialization efforts for TRYNGOLZA and DAWNZERA as well as launch preparations for olezarsen in sHTG and zilganersen in Alexander disease.

Balance Sheet

As of March 31, 2026, Ionis' cash, cash equivalents and short-term investments decreased to $1.9 billion, compared to $2.7 billion on December 31, 2025. At March 31, 2026, Ionis had an escrow deposit of $633 million, which the Company used for the maturity of its 0% convertible notes due on April 1, 2026.

2026 Financial Guidance

Ionis improved its 2026 financial guidance to reflect the strong revenue performance experienced year-to-date and the Company's outlook for the balance of 2026. Overall, the Company increased total revenue and decreased operating loss both by $75 million. The improvements were driven by Priority Review for TRYNGOLZA, strong first quarter R&D revenue and the anticipated continued success of the Company's ongoing commercial launches.

Full Year 2026 Guidance	Previous Guidance	New Guidance
Total Revenue	$800-825 million	$875-900 million
TRYNGOLZA product sales, net	NA	$100-110 million
DAWNZERA product sales, net	NA	$110-120 million
Operating loss on a non-GAAP basis	$500-550 million	$425-475 million
Cash, cash equivalents and short-term investments	>$1.6 billion	>$1.6 billion

Webcast and Other Updates

Management will host a conference call and webcast to discuss Ionis' first quarter 2026 results at 8:30 a.m. Eastern time on Wednesday, April 29, 2026. 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 2026 earnings slides click here.

Ionis' Marketed Medicines

INDICATION for TRYNGOLZA® (olezarsen)

TRYNGOLZA® (olezarsen) was approved by the U.S. Food and Drug Administration as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS).

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS
TRYNGOLZA is contraindicated in patients with a history of serious hypersensitivity to TRYNGOLZA or any of the excipients in TRYNGOLZA. Hypersensitivity reactions requiring medical treatment have occurred.

WARNINGS AND PRECAUTIONS
Hypersensitivity Reactions
Hypersensitivity reactions (including symptoms of bronchospasm, diffuse erythema, facial swelling, urticaria, chills and myalgias) have been reported in patients treated with TRYNGOLZA. Advise patients on the signs and symptoms of hypersensitivity reactions and instruct patients to promptly seek medical attention and discontinue use of TRYNGOLZA if hypersensitivity reactions occur.

ADVERSE REACTIONS
The most common adverse reactions (incidence >5% of TRYNGOLZA-treated patients and >3% higher frequency than placebo) were injection site reactions, decreased platelet count and arthralgia.

Please see full Prescribing Information for TRYNGOLZA.

INDICATION for DAWNZERA™ (donidalorsen)

DAWNZERA™ (donidalorsen) was approved by theU.S. Food and Drug Administration for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients 12 years of age and older.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

DAWNZERA is contraindicated in patients with a history of serious hypersensitivity reactions, including anaphylaxis, to donidalorsen or any of the excipients in DAWNZERA.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions
Hypersensitivity reactions, including anaphylaxis, have been reported in patients treated with DAWNZERA. If signs and symptoms of serious hypersensitivity reactions occur, discontinue DAWNZERA and institute appropriate therapy.

ADVERSE REACTIONS
Most common adverse reactions (incidence ≥ 5%) are injection site reactions, upper respiratory tract infection, urinary tract infection, and abdominal discomfort.

Please see full Prescribing Information for DAWNZERA.

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.

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).

About Ionis Pharmaceuticals, Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has marketed medicines and a leading pipeline in neurology, cardiometabolic disease and select 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 Ionis.com and follow us on X

(Twitter), LinkedIn and Instagram.

Ionis Forward-looking Statements

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, technologies and our expectations regarding development and regulatory milestones. 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, 2025, 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, INC.
SELECTED FINANCIAL INFORMATION
Condensed Consolidated Statements of Operations
(In Millions, Except Per Share Data)

	Three months ended March 31,	
	2026	2025
	(unaudited)	
Revenue:		
Commercial revenue:		
Product sales, net	$43	$6
Royalty revenue	58	64
Other commercial revenue	7	6
Total commercial revenue	108	76
Research and development revenue:		
Collaborative agreement revenue	120	46
WAINUA joint development revenue	18	10
Total research and development revenue	138	56
Total revenue	246	132
Expenses:		
Cost of sales	3	1
Research, development and patent	210	201
Selling, general and administrative	151	76
Total operating expenses	364	278
Loss from operations	(118)	(146)
Other income (expense):		
Interest expense related to the sale of future royalties	(17)	(19)
Other income, net	42	18
Loss before income tax expense	(93)	(147)

Income tax expense	-	-
Net loss	($93)	($147)
Basic and diluted net loss per share	($0.56)	($0.93)
Shares used in computing basic and diluted net loss per share	165	159

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,	
	2026	2025
	(unaudited)	
As reported cost of sales according to GAAP	$3	$1
Excluding compensation expense related to equity awards (1)	-	-
Non-GAAP cost of sales	$3	$1
As reported research, development and patent expenses according to GAAP	$210	$201
Excluding compensation expense related to equity awards	(25)	(20)
Non-GAAP research, development and patent expenses	$185	$181
As reported selling, general and administrative expenses according to GAAP	$151	$76
Excluding compensation expense related to equity awards	(18)	(9)
Non-GAAP selling, general and administrative expenses	$133	$67
As reported operating expenses according to GAAP	$364	$278
Excluding compensation expense related to equity awards	(43)	(29)
Non-GAAP operating expenses	$321	$249
As reported loss from operations according to GAAP	($118)	($146)
Excluding compensation expense related to equity awards	(43)	(29)
Non-GAAP loss from operations	($75)	($117)
As reported net loss according to GAAP	($93)	($147)
Excluding compensation expense related to equity awards and related tax effects	(43)	(29)
Non-GAAP net loss	($50)	($118)

(1) Amounts appear as zero due to rounding in millions.

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, 2026	December 31, 2025
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	$1,919	$2,677
Escrow deposits	633	-
Contracts receivable	74	66
Other current assets	312	247
Property, plant and equipment, net	142	123
Right-of-use assets	235	239
Other assets	135	172
Total assets	$3,450	$3,524
Liabilities and stockholders' equity:		
Current portion of deferred contract revenue	$69	$74
0% convertible senior notes due April 2026 – current	433	432
Other current liabilities	215	277
0% convertible senior notes due 2030, net	752	751
1.75% convertible senior notes due 2028, net	569	568
Liability related to sale of future royalties, net	558	551
Long-term lease liabilities	259	262
Long-term obligations, less current portion	28	28
Long-term deferred contract revenue	76	92
Total stockholders' equity	491	489
Total liabilities and stockholders' equity	$3,450	$3,524

Key 2026 Value Driving Events[1]

New Product Launches

Program	Indication	Location	
DAWNZERA	HAE	EU	Achieved
Olezarsen	sHTG	U.S.	•
Zilganersen	Alexander disease	U.S.	•
Bepirovirsen	CHB	U.S. & Japan	•

Regulatory Actions

Program	Indication	Regulatory Action	
Donidalorsen	HAE	EU approval decision	Achieved
Olezarsen	sHTG	U.S. approval decision	•
		EU submission	Achieved
Zilganersen	Alexander disease	U.S. submission	Achieved
		U.S. approval decision	•
Nusinersen (high dose)	SMA	EU approval decision	Achieved
		U.S. approval decision	Achieved
Eplontersen	ATTR-CM	Regulatory submission(s)	•
Bepirovirsen	HBV	Regulatory submission(s)	Achieved

		Regulatory decision(s)	•
Pelacarsen	Lp(a)- CVD	U.S. submission	•

Key Phase 3 Clinical Events

Program	Indication	Event	
Obudanersen	Angelman syndrome	Phase 3 enrollment completion	•
Bepirovirsen	HBV	B-Well data	Achieved
Pelacarsen	Lp(a)-CVD	Lp(a) HORIZON data	•
Eplontersen	ATTR-CM	CARDIO-TTRansform data	•
Sefaxersen	IgAN	IMAGINATION data	•
Ulefnersen	FUS-ALS	FUSION data	•
Salanersen	SMA	Phase 3 initiation	•
Sapablursen	Polycythemia Vera	Phase 3 initiation	•

Key Phase 2 Clinical Events

Program	Indication	Event	
IONIS-MAPT$_{Rx}$/ BIIB080	Alzheimer's disease	Phase 2 CELIA data	•
Tominersen	Huntington's disease	Phase 2 GENERATION HD2 data	•
Tonlamarsen	Uncontrolled hypertension	Phase 2 data	Achieved

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

- Indicates that the milestone is anticipated in 2026.

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Ionis Investor Contact:
D. Wade Walke, Ph.D.
IR@ionis.com
760-603-2331

Ionis Media Contact:
Hayley Soffer
media@ionis.com
760-603-4679

Source: Ionis Pharmaceuticals, Inc.