Ionis reports fourth quarter and full year 2025 financial results and highlights progress on key programs

February 25, 2026

- TRYNGOLZA® generated $108 million in net product sales in 2025, the first year of launch –

- Olezarsen sHTG launch preparations on track, sNDA submitted -

- Exceeded 2025 financial guidance, 2026 guidance reflects commitment to independently deliver a steady cadence of innovative medicines to patients -

CARLSBAD, Calif.--(BUSINESS WIRE)--Feb. 25, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) (the "Company") today reported financial results and provided key updates for the fourth quarter and year ended December 31, 2025.

"2025 was a defining year for Ionis, marked by the successful execution of our first two independent launches and multiple positive data readouts across our pipeline, positioning Ionis for continued success in 2026," said Brett P. Monia, Ph.D., chief executive officer of Ionis. "This year, we are poised for two additional independent launches of groundbreaking therapies — olezarsen for severe hypertriglyceridemia, our first launch in a broad patient population, and zilganersen for Alexander disease, our first launch from our leading neurology pipeline. Our partnered pipeline is also on track for multiple groundbreaking Phase 3 readouts, beginning with the recent positive data for bepirovirsen in chronic hepatitis B to be followed by two cardiovascular outcome trials — the pelacarsen Lp(a) HORIZON trial mid-year 2026 and the eplontersen CARDIO-TTRansform trial in the second half of 2026. Together, this progress positions Ionis to continue delivering a steady cadence of transformative medicines to people living with serious diseases, fueling substantial growth and long-term value creation."

Fourth Quarter and Full Year 2025 Summary Financial Results[(1)]:

	Three months ended December 31,		Year ended December 31,	
	2025	2024	2025	2024
	(amounts in millions)			
Total revenue	$203	$227	$944	$705
Operating expenses	$418	$337	$1,326	$1,180
Operating expenses on a non-GAAP basis	$375	$301	$1,192	$1,050
Loss from operations	($215)	($110)	($382)	($475)
Loss from operations on a non-GAAP basis	($172)	($74)	($248)	($345)

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

Recent Financial Highlights

- Revenue for the year ended December 31, 2025 substantially exceeded expectations due to continued commercial success. In addition, Ionis earned substantial R&D revenue, including a $280 million upfront payment for the global license of sapablursen to Ono Pharmaceutical Co., Ltd. in the second quarter of 2025
- Operating expenses for the year ended December 31, 2025 were in line with expectations and increased year over year from investments related to commercialization efforts for TRYNGOLZA, DAWNZERA and WAINUA
- Cash and short-term investments of $2.7 billion as of December 31, 2025, included refinancing proceeds Ionis plans to use to repay its 2026 Convertible Notes

Fourth Quarter and Full Year 2025 Financial Results

"In 2025 we exceeded our revenue guidance, driven by growing commercial revenue from our independent launches and substantial R&D revenue from continued pipeline success," said Elizabeth L. Hougen, chief financial officer of Ionis. "In 2026, we will continue to invest in go-to-market activities to support our ongoing and upcoming independent launches, including the recent expansion of our top-tier sales force ahead of our expected olezarsen sHTG launch. We anticipate growth in product revenues,

together with additional royalties, to position Ionis to achieve cash flow breakeven in 2028 and generate substantial and sustainable positive cash flow for years to come."

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 net product sales of $50 million in the fourth quarter of 2025, a 56% increase over the prior quarter, and $108 million for the year ended December 31, 2025
  - Approved and launched in the European Union (EU) as an adjunct to diet in adult patients for the treatment of genetically confirmed FCS
- Olezarsen on track to launch this year as a transformational medicine for severely elevated triglycerides (sHTG), assuming approval
  - Positive groundbreaking results in the pivotal Phase 3 CORE and CORE2 studies in sHTG presented at the American Heart Association Conference, in a late-breaking session, and published in the *New England Journal of Medicine*
  - sNDA submitted for marketing approval in U.S.
- DAWNZERA™ (donidalorsen), the first and only RNA-targeted prophylactic therapy for hereditary angioedema (HAE) in patients 12 years of age and older
  - Generated net product sales of $7 million in the fourth quarter of 2025, in the first full quarter on the market
    - Encouraging early launch momentum with prescriptions written for all patient segments and growing number of repeat prescribers
  - Approved in the European Union (EU) in January and recently launched for the routine prevention of recurrent attacks of HAE in patients 12 years of age and older
  - Positive one-year results from OASISplus open-label extension cohort published in the *Journal of Asthma and Allergy*
- Zilganersen on track for launch this year as the first and only medicine to demonstrate clinically meaningful and disease-modifying impact in children and adults with Alexander disease (AxD), assuming approval
  - NDA submitted with approval decision anticipated in H2:2026
  - Expanded access program (EAP) in U.S. underway

Recent Highlights – Partnered Medicines

- SPINRAZA® (nusinersen) for the treatment of spinal muscular atrophy (SMA) generated global sales of $356 million and $1.5 billion resulting in royalty revenue of $54 million and $212 million in the fourth quarter and the year ended December 31, 2025, respectively
  - High dose approved and launched in the EU; under review for marketing approval in U.S. (PDUFA date of April 3, 2026)
  - Positive high dose results from pivotal DEVOTE study published in *Nature Medicine*
- WAINUA® (eplontersen) (WAINZUA in EU) for the treatment of adults with polyneuropathy of hereditary transthyretin-mediated amyloidosis (ATTRv-PN) generated sales of $69 million and $212 million resulting in royalty revenue of $16 million and $49 million in the fourth quarter and the year ended December 31, 2025, respectively
  - Launches underway in numerous regions, including the EU; recently approved in China; additional submissions in progress to expand WAINUA access globally
- Bepirovirsen, a potential first-in-class medicine for chronic hepatitis B (CHB), achieved primary endpoint and demonstrated a statistically significant and clinically meaningful functional cure rate in B-Well 1 and B-Well 2 Phase 3 studies
  - Presentation planned for European Association for the Study of the Liver (EASL) Congress 2026, assuming acceptance
  - Global regulatory filings planned beginning in Q1:2026 with 2026 anticipated launch, assuming approval
- Ulefnersen for the treatment of FUS-ALS granted U.S. Fast Track designation
- Sapablursen for the treatment of polycythemia vera (PV) demonstrated positive Phase 2 results, which were presented at American Society of Hematology (ASH) conference; Ono advancing sapablursen into Phase 3 development
- Opemalirsen for the treatment of APOL1-mediated chronic kidney disease (AMKD) granted U.S. Fast Track designation

Revenue

Ionis' revenue was comprised of the following:

	Three months ended December 31,		Year ended December 31,	
	2025	2024	2025	2024
	(amounts in millions)			
Revenue:				
Commercial revenue:				
Product sales, net:				

TRYNGOLZA sales, net	$50	$-	$108	$-
DAWNZERA sales, net	7	-	8	-
Total product sales, net	57	-	116	-
Royalty revenue:				
SPINRAZA royalties	54	64	212	216
WAINUA royalties	16	10	49	20
Other royalties	6	3	24	21
Total royalty revenue	76	77	285	257
Other commercial revenue	8	9	35	36
Total commercial revenue	**141**	**86**	**436**	**293**
Research and development revenue:				
Collaborative agreement revenue	52	97	466	333
WAINUA joint development revenue	10	44	42	79
Total research and development revenue	**62**	**141**	**508**	**412**
Total revenue	**$203**	**$227**	**$944**	**$705**

Commercial revenue for the fourth quarter and the year ended December 31, 2025, increased 64% and 49%, respectively, compared to the same periods in 2024. This increase was primarily driven by TRYNGOLZA product sales. Higher royalty revenue also contributed to the year over year increase.

The remainder of the Company's revenue came from programs under its R&D collaborations, including a $280 million upfront payment for the global license of sapablursen to Ono Pharmaceutical Co., Ltd. in the second quarter of 2025, reflecting the value that Ionis' pipeline and technology continue to generate.

Operating Expenses

Operating expenses increased modestly for the fourth quarter and the year ended December 31, 2025, which was in line with expectations. The increase was driven by investments to support the launches of TRYNGOLZA, DAWNZERA and WAINUA.

Balance Sheet

As of December 31, 2025, Ionis' cash, cash equivalents and short-term investments increased to $2.7 billion, compared to $2.3 billion on December 31, 2024, primarily due to the refinancing proceeds Ionis received from its convertible debt issuance in the fourth quarter, which Ionis plans to use to repay its 2026 Convertible Notes.

2026 Financial Guidance

The Company's 2026 financial guidance reflects its evolution to a fully integrated commercial-stage biotechnology company independently launching multiple medicines and advancing commercialization efforts for additional upcoming planned launches. As a result, the Company expects to earn substantial revenue from numerous diverse sources, including increasing commercial revenue. The Company is currently awaiting acceptance of its olezarsen sNDA submission, as such the Company's 2026 financial guidance assumes a standard review timeline. With acceptance anticipated shortly, the Company expects to provide TRYNGOLZA and DAWNZERA product level guidance at its first quarter 2026 earnings. The Company expects a modest increase in its non-GAAP operating expenses in line with its plan to invest in independent launches and advance its wholly owned pipeline of innovative medicines. The Company expects that these investments will enable Ionis to deliver accelerating value. Overall, the Company anticipates total revenue to grow approximately 20 percent year over year and its non-GAAP operating loss to be similar to 2025, excluding the one-time sapablursen upfront payment recognized in 2025.

Full Year 2026 Guidance	
Revenue	$800- $825 million
Operating loss on a non-GAAP basis	$500-550 million
Cash, cash equivalents and short-term investments	~$1.6 billion

Webcast and Other Updates

Management will host a conference call and webcast to discuss Ionis' fourth quarter and full year 2025 results at 8:30 a.m. Eastern time on Wednesday, February 25, 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 fourth quarter and full year 2025 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, 2024, 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 December 31,		Year ended December 31,	
	2025	2024	2025	2024
	(unaudited)			
Revenue:				
Commercial revenue:				
Product sales, net	$57	$-	$116	$-
Royalty revenue	76	77	285	257
Other commercial revenue	8	9	35	36
Total commercial revenue	141	86	436	293
Research and development revenue:				
Collaborative agreement revenue	52	97	466	333
WAINUA joint development revenue	10	44	42	79
Total research and development revenue	62	141	508	412
Total revenue	203	227	944	705
Expenses:				

Cost of sales	8	4	16	11
Research, development and patent	280	245	916	902
Selling, general and administrative	130	88	394	267
Total operating expenses	418	337	1,326	1,180
Loss from operations	(215)	(110)	(382)	(475)
Other income (expense):				
Interest expense related to the sale of future royalties	(18)	(19)	(73)	(73)
Other income, net	5	22	75	88
Loss before income tax benefit (expense)	(228)	(107)	(380)	(460)
Income tax benefit (expense)	(1)	3	(1)	6
Net loss	($229)	($104)	($381)	($454)
Basic and diluted net loss per share	($1.41)	($0.66)	($2.38)	($3.04)
Shares used in computing basic and diluted net loss per share	162	158	160	150

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 December 31,		Year ended December 31,	
	2025	2024	2025	2024
	(unaudited)			
As reported research, development and patent expenses according to GAAP	$280	$245	$916	$902
Excluding compensation expense related to equity awards	(29)	(25)	(90)	(92)
Non-GAAP research, development and patent expenses	$251	$220	$826	$810
As reported selling, general and administrative expenses according to GAAP	$130	$88	$394	$267
Excluding compensation expense related to equity awards	(13)	(11)	(42)	(37)
Non-GAAP selling, general and administrative expenses	$117	$77	$352	$230
As reported operating expenses according to GAAP	$418	$337	$1,326	$1,180
Excluding compensation expense related to equity awards	(43)	(36)	(134)	(130)
Non-GAAP operating expenses	$375	$301	$1,192	$1,050
As reported loss from operations according to GAAP	($215)	($110)	($382)	($475)
Excluding compensation expense related to equity awards	(43)	(36)	(134)	(130)
Non-GAAP loss from operations	($172)	($74)	($248)	($345)
As reported net loss according to GAAP	($229)	($104)	($381)	($454)
Excluding compensation expense related to equity awards and related tax effects	(43)	(36)	(134)	(130)
Non-GAAP net loss	($186)	($68)	($247)	($324)

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)

	December 31, 2025	December 31, 2024
	(unaudited)	
Assets:		
Cash, cash equivalents and short-term investments	$2,677	$2,298
Contracts receivable	66	92
Other current assets	247	230
Property, plant and equipment, net	123	94
Right-of-use assets	239	162
Other assets	172	127
Total assets	$3,524	$3,003
Liabilities and stockholders' equity:		
Current portion of deferred contract revenue	$74	$79
0% convertible senior notes due 2026, net – current	432	-
Other current liabilities	277	229
0% convertible senior notes due 2030, net	751	-
1.75% convertible senior notes due 2028, net	568	565
0% convertible senior notes due 2026, net	-	629
Liability related to sale of future royalties, net	551	542
Long-term lease liabilities	262	162
Long-term obligations, less current portion	28	52
Long-term deferred contract revenue	92	157
Total stockholders' equity	489	588
Total liabilities and stockholders' equity	$3,524	$3,003

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	●
Zilganersen	Alexander disease	U.S. submission	●
		U.S. approval decision	●
Nusinersen (high dose)	SMA	EU approval decision	Achieved
		U.S. approval decision	●

Eplontersen	ATTR-CM	Regulatory submission(s)	●
Bepirovirsen	HBV	Regulatory submission(s)	●
		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	●

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

● Indicates that the milestone is anticipated in 2026.

View source version on businesswire.com: https://www.businesswire.com/news/home/20260225250070/en/

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.