



## Ionis well-positioned for continued momentum and substantial value creation in 2026 with two new independent launches and several pivotal data readouts

January 12, 2026

- TRYNGOLZA® (olezarsen) outperforms expectations, achieved 2025 preliminary U.S. net product sales of \$105M\* as first FDA-approved treatment for FCS; sNDA for sHTG submitted for review –
- Increasing annual TRYNGOLZA peak net sales guidance to >\$2B for sHTG –
- Positive topline results announced for pivotal Phase 3 program of bepirovirsen in chronic hepatitis B; first of five Phase 3 readouts from partnered programs expected this year –

CARLSBAD, Calif.--(BUSINESS WIRE)--Jan. 12, 2026-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) today announced highlights from the Company's 2025 achievements and provided additional updates on key milestones expected in 2026. Ionis will provide a business update at the 44<sup>th</sup> Annual J.P. Morgan Healthcare Conference on Tuesday, January 13 at 8:15am PT; the presentation is available today on the Ionis website [here](#).

"2025 was a defining year for Ionis, as we successfully executed our first two independent launches as a commercial stage biotech company. We expect 2026 to be another transformative year, poised for two additional independent launches of breakthrough therapies – olezarsen for severe hypertriglyceridemia, Ionis' first launch in a large patient population, and zilganersen for Alexander disease, Ionis' first independent launch from our leading neurology pipeline," said Brett P. Monia, Ph.D., chief executive officer, Ionis. "This momentum is further bolstered by the promise of our late-stage Ionis owned and partnered programs, with a total of five Phase 3 readouts and four NDA submissions anticipated this year. With strong execution and multiple key catalysts expected, Ionis is well-positioned to deliver accelerating revenue growth to achieve cash flow breakeven in 2028."

### Preliminary TRYNGOLZA® (olezarsen) 2025 Full Year U.S. Net Sales

- TRYNGOLZA outperformed expectations as the first U.S. Food and Drug Administration (FDA)-approved treatment for familial chylomicronemia syndrome (FCS), generating \$105\* million in preliminary U.S. net product sales in 2025

### 2026 Anticipated Highlights Include:

#### Ionis Owned Programs

- Potential approval and launch of olezarsen as the new standard of care for severe hypertriglyceridemia (sHTG)
  - Submitted U.S. Supplemental New Drug Application (sNDA), following receipt of Breakthrough Therapy Designation
    - Olezarsen achieved a highly statistically significant placebo-adjusted reduction of up to 72% in fasting triglycerides and an 85% reduction in acute pancreatitis events with favorable safety and tolerability in pivotal Phase 3 CORE and CORE2 studies
  - Increasing annual olezarsen peak net sales guidance to >\$2 billion from >\$1 billion based on strong product profile and positive Phase 3 data
- Continued early positive momentum with the launch of DAWNZERA™ (donidalorsen), the first RNA-targeted therapy for hereditary angioedema (HAE)
  - Prescriptions written for all patient segments; growing number of repeat prescribers
  - Expect European Medicines Agency (EMA) approval and launch in Q1 2026
- Potential approval and launch of zilganersen for Alexander disease (AxD), the first and only investigational medicine to demonstrate clinically meaningful and disease-modifying impact
  - U.S. Expanded Access Program (EAP) underway
  - Planned NDA submission in Q1 2026 and launch later this year; received Breakthrough Therapy Designation
  - First anticipated launch from Ionis owned neurology portfolio
- Continued progress of Phase 3 REVEAL study of ION582, a promising investigational medicine for Angelman syndrome (AS)
  - Expect to complete enrollment in 2026 with data expected in 2027

- Multiple Phase 2 data readouts from neurology pipeline

#### Partnered Programs

- Potential approval and launch of bepirovirsen, an Ionis-discovered investigational medicine for chronic hepatitis B (CHB) (GSK)
  - Planned regulatory submissions to health authorities worldwide in 2026 based on positive topline results from pivotal Phase 3 B-Well 1 and B-Well 2 studies, in which bepirovirsen demonstrated a statistically significant and clinically meaningful functional cure rate
  - Full data presentation and publication planned
- Results from Phase 3 Lp(a) HORIZON cardiovascular outcomes study of pelacarsen, an Ionis-discovered investigational medicine for lipoprotein(a) (Lp(a))-driven cardiovascular disease (CVD) (Novartis)
  - Results expected in H1 2026
  - Planned NDA submission
- Results from Phase 3 CARDIO-TTRansform study of eplontersen, Ionis' co-commercialized investigational medicine with AstraZeneca for transthyretin-mediated amyloid cardiomyopathy (ATTR-CM)
  - Results expected in H2 2026
  - Planned sNDA submission
- Additional Phase 3 readouts from partnered programs:
  - Results from IMAGINATION study of sefaxersen for IgA nephropathy (IgAN) (Roche)
  - Results from FUSION study of ulefnersen for Fused in Sarcoma (FUS) Amyotrophic Lateral Sclerosis (ALS) (Otsuka)
- Multiple Phase 2 data readouts, including IONIS-MAPT<sub>Rx</sub> (BIIB080) in Alzheimer's disease (AD) (Biogen) and new Phase 3 clinical trial initiations

\*Indicates preliminary unaudited results.

#### **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) is indicated 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  $\geq$  5%) are injection site reactions, upper respiratory tract infection, urinary tract infection, and abdominal discomfort.

Please see full [Prescribing Information](#) for DAWNZERA.

### 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](https://www.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 and the therapeutic and commercial potential of TRYNGOLZA, DAWNZERA and additional medicines in development and technologies, and our expectations regarding development and regulatory milestones. Any statement describing Ionis' goals, expectations, financial or other projections or guidance, 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 but not limited to those related to our commercial products and the medicines in our pipeline, and particularly 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 Dec. 31, 2024, and most recent Form 10-Q, which are on file with the SEC. Copies of these and other documents are available at [www.ionis.com](https://www.ionis.com).

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