



TRYNGOLZA® (olezarsen) recommended for approval in the EU by CHMP for familial chylomicronemia syndrome (FCS)

July 25, 2025

– Recommendation based on Phase 3 Balance results, which showed a significant reduction of triglycerides and substantial reduction of acute pancreatitis events with TRYNGOLZA –

– European Commission decision expected by Q4 2025 –

CARLSBAD, Calif.--(BUSINESS WIRE)--Jul. 25, 2025-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) and Sobi® today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion of TRYNGOLZA® (olezarsen) as an adjunct to diet in adult patients for the treatment of genetically confirmed familial chylomicronemia syndrome (FCS). The positive opinion is now referred to the European Commission (EC) for an approval decision, which is expected by Q4 2025.

"Building on the strong early launch of TRYNGOLZA in the U.S., the positive CHMP opinion advances our commitment to expand access to TRYNGOLZA globally," said Brett P. Monia, Ph.D., chief executive officer, Ionis. "TRYNGOLZA has demonstrated significant reductions in triglycerides and substantial reductions in acute pancreatitis events with favorable safety and tolerability. With this robust clinical profile, combined with Sobi's deep commercial market expertise in FCS, TRYNGOLZA has the potential to make a meaningful difference for people living with FCS in the EU."

The CHMP opinion is based on positive data from the Phase 3 Balance study, in which TRYNGOLZA demonstrated a statistically significant reduction in triglyceride levels at six months that was sustained through 12 months. Additionally, TRYNGOLZA demonstrated a substantial and clinically meaningful reduction in acute pancreatitis events over 12 months. TRYNGOLZA showed a favorable safety and tolerability profile. Study results were published in *The New England Journal of Medicine (NEJM)*.

FCS is a rare and genetic form of severe hypertriglyceridemia (sHTG) that prevents the body from breaking down fats and severely impairs the ability to remove triglycerides from the bloodstream. People with FCS often have triglyceride levels of more than 880 mg/dL (10 mmol/L), compared to normal levels of <150 mg/dL (1.7 mmol/L), and are at high risk of developing acute pancreatitis, which can be life-threatening. In the EU, FCS is estimated to impact up to 13 people per million.

Sobi has exclusive rights to commercialize TRYNGOLZA in countries outside the U.S., Canada and China. As Ionis' European commercial partner for Waylivra (volanesorsen), the only medicine currently approved for FCS in the EU, Sobi will leverage existing market expertise and distribution channels to enable an effective TRYNGOLZA launch in FCS, if approved.

"The approval recommendation brings us one step closer toward delivering TRYNGOLZA to people living with FCS in the EU and is a testament to our long-standing support for the FCS community," said Lydia Abad-Franch, M.D., MBA, head of research, development and medical affairs and chief medical officer, Sobi. "Patients with FCS suffer from complications such as acute pancreatitis. These are very severe events, often requiring intensive care and sometimes causing multiorgan failure as well as increasing morbidity and mortality. We believe TRYNGOLZA has the potential to be an important treatment for people living with this rare and serious disease, and we look forward to the final decision from the EC later this year."

TRYNGOLZA was approved in the United States in December 2024 and granted orphan designation in the EU. Olezarsen is also being evaluated for sHTG, a serious condition defined by dangerously high triglycerides (≥ 500 mg/dL), and data from the Phase 3 CORE and CORE2 studies are expected in Q3 2025.

About the Balance Study

Balance is a global, multicenter, randomized, double-blind, placebo-controlled Phase 3 study evaluating the efficacy and safety of olezarsen in patients with FCS at six and 12 months. The primary endpoint was the percent change from baseline in fasting triglyceride levels at six months compared to placebo. Secondary endpoints included percent changes in triglyceride levels at 12 months, percent changes in other lipid parameters and adjudicated acute pancreatitis event rates over the treatment period. Following treatment and the end-of-trial assessments, patients were eligible to enter an open-label extension study to continue receiving olezarsen once every four weeks.

About Familial Chylomicronemia Syndrome (FCS)

FCS is a rare, genetic disease characterized by extremely elevated triglyceride levels. It is caused by impaired function of the

enzyme lipoprotein lipase (LPL). Because of limited LPL production or function, people with FCS cannot effectively break down chylomicrons, lipoprotein particles that are 90% triglycerides. People living with FCS are at high risk of acute pancreatitis in addition to other chronic health issues such as fatigue and severe, recurrent abdominal pain. People living with FCS are sometimes unable to work, adding to the burden of disease.

About TRYNGOLZA® (olezarsen)

TRYNGOLZA® is an RNA-targeted medicine designed to lower the body's production of apoC-III, a protein produced in the liver that is a key regulator of triglyceride metabolism. TRYNGOLZA® (olezarsen) is approved in the United States as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS). For more information about TRYNGOLZA, visit [TRYNGOLZA.com](https://www.ionispharm.com/TRYNGOLZA). TRYNGOLZA is not yet approved for any indication in Europe.

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.

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, cardiology 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 our commercial medicines, olezarsen, additional medicines in development and technologies. 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® and TRYNGOLZA® are trademarks of Ionis Pharmaceuticals, Inc.

View source version on [businesswire.com](https://www.businesswire.com/news/home/20250724782589/en/): <https://www.businesswire.com/news/home/20250724782589/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.