



TRYNGOLZA® (olezarsen) recommended for approval in the EU by CHMP for severe hypertriglyceridemia (sHTG)

October 16, 2026

CARLSBAD, Calif.--(BUSINESS WIRE)--Oct. 16, 2026-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending the indication extension for TRYNGOLZA® (olezarsen) as an adjunct to diet, in combination with other lipid-lowering therapies, for the treatment of adult patients with severe hypertriglyceridemia (sHTG, fasting triglyceride levels of ≥ 880 mg/dL [≥ 10 mmol/L]). If confirmed by the European Commission (EC), TRYNGOLZA would be the first and only RNA-targeted therapy authorized in the EU for adults with sHTG and triglyceride levels ≥ 880 mg/dL (≥ 10 mmol/L). The positive CHMP opinion comes ahead of the previously anticipated timeline, with a final EC decision expected in the fourth quarter of 2026.

"Following TRYNGOLZA's approval for sHTG in the U.S. in June, today's positive CHMP opinion marks an important step towards making this treatment available to patients with sHTG in the EU," said Brett P. Monia, Ph.D., chief executive officer, Ionis. "The recommendation builds on the robust clinical evidence from the CORE and CORE2 studies and reflects the continued collaboration between Ionis and Sobi as we work towards a potential European approval."

The positive CHMP opinion is based on results from the pivotal Phase 3 CORE and CORE2 studies. These studies met their primary endpoint, with TRYNGOLZA demonstrating a statistically significant placebo-adjusted mean reduction in fasting triglyceride levels of up to 72% at six months, sustained through 12 months. In a pooled analysis across the two studies, TRYNGOLZA demonstrated a statistically significant 85% reduction in adjudicated acute pancreatitis events over 12 months compared with placebo. TRYNGOLZA demonstrated a favorable safety and tolerability profile. The [data](#) were published in *The New England Journal of Medicine*.

sHTG is commonly defined by very high triglycerides ≥ 500 mg/dL (≥ 5.65 mmol/L) and characterized by an increased risk of acute pancreatitis and other serious health complications. Approximately two million people are living with sHTG in the EU5, including approximately 700,000 with triglyceride levels ≥ 880 mg/dL (≥ 10 mmol/L).

"The CHMP's positive opinion reflects the strength of the clinical evidence and brings us one step closer to offering TRYNGOLZA as a new treatment option to help reduce the risk of acute pancreatitis in patients with sHTG in the EU," said Lydia Abad-Franch, M.D., head of research and development and medical affairs and chief medical officer, Sobi. "Acute pancreatitis is a potentially life-threatening complication of sHTG that can place a significant burden on patients through frequent hospitalizations, intensive care treatment, irreversible complications and reduced quality of life. We look forward to the final decision from the EC."

TRYNGOLZA is [approved](#) in the U.S. as an adjunct to diet to reduce triglycerides and the risk of acute pancreatitis in adults with sHTG (triglycerides ≥ 500 mg/dL) and in the U.S., EU and other countries for adults with familial chylomicronemia syndrome (FCS), a rare form of sHTG.

TRYNGOLZA was discovered and developed by Ionis, which also commercializes the medicine in the U.S. Sobi and Ionis entered into a license agreement under which Sobi has exclusive rights to commercialize TRYNGOLZA outside the U.S., excluding Canada and China.

About the CORE and CORE2 Studies

CORE ([NCT05079919](#); n=617) and CORE2 ([NCT05552326](#); n=446), conducted with The TIMI Study Group, are Phase 3 global, multicenter, randomized, double-blind, placebo-controlled trials investigating the safety and efficacy of olezarsen for severe hypertriglyceridemia (sHTG). Participants aged 18 and older with triglyceride levels ≥ 500 mg/dL (≥ 5.65 mmol/L) were enrolled. Participants were required to be on standard of care therapies for elevated triglycerides. At baseline, 47% and 37% of participants had fasting triglycerides ≥ 880 mg/dL (≥ 10 mmol/L) in CORE and CORE2, respectively. Participants were randomized to receive 50 mg or 80 mg of olezarsen or placebo every four weeks via subcutaneous injection for 12 months.

About Severe Hypertriglyceridemia

Severe hypertriglyceridemia (sHTG) is commonly defined by very high triglycerides ≥ 500 mg/dL (≥ 5.65 mmol/L) and characterized by an increased risk of acute pancreatitis and other serious health complications. Considered a medical emergency, acute pancreatitis causes debilitating abdominal pain that often requires prolonged hospitalization, can lead to permanent organ damage and can become life-threatening. Preventing the first attack is key. In people with a history of acute pancreatitis episodes, the risk of future attacks is even greater. Current standard of care therapies for sHTG and lifestyle modifications (such as diet and exercise) do not sufficiently or consistently lower triglyceride levels or reduce the risks of sHTG in all patients. More than 3 million

people are living with sHTG in the U.S., including approximately 1 million who are considered high risk. High-risk sHTG includes those with triglycerides ≥ 880 mg/dL (≥ 10 mmol/L) or triglycerides ≥ 500 mg/dL and a history of acute pancreatitis or other comorbidities. Approximately 2 million people are living with sHTG in the EU5, including approximately 700,000 with triglyceride levels ≥ 880 mg/dL.

About TRYNGOLZA® (olezarsen)

TRYNGOLZA® (olezarsen) is an RNA-targeted therapy designed to lower the body's production of apoC-III, a protein produced in the liver that regulates triglyceride metabolism in the blood. TRYNGOLZA is approved in the United States as an adjunct to diet to reduce triglycerides (TG) and the risk of acute pancreatitis in adults with severe hypertriglyceridemia (sHTG: TG greater than or equal to 500 mg/dL). TRYNGOLZA is also approved in the United States, European Union and other countries for adults with familial chylomicronemia syndrome (FCS), a rare form of sHTG. Visit [TRYNGOLZA.com](https://www.ionis.com/TRYNGOLZA) for more information.

INDICATIONS

Familial Chylomicronemia Syndrome (FCS)

TRYNGOLZA (olezarsen) is indicated as an adjunct to diet to reduce triglycerides (TG) in adults with familial chylomicronemia syndrome (FCS).

Severe Hypertriglyceridemia (sHTG)

TRYNGOLZA is indicated as an adjunct to diet to reduce TG and the risk of acute pancreatitis in adults with severe hypertriglyceridemia (sHTG: TG ≥ 500 mg/dL).

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.

Liver Enzyme Abnormalities

TRYNGOLZA can cause increases in liver enzymes and hepatic fat in adults. Increases in liver enzymes were more frequently reported with the 80 mg dose as compared with the 50 mg dose. Consider liver enzyme testing before TRYNGOLZA initiation or an increase in dosage and when clinically indicated thereafter. If persistent elevations in liver enzymes occur, consider dose interruption and/or dose reduction. If serious hepatic injury with clinical symptoms and/or hyperbilirubinemia or jaundice occurs, promptly discontinue TRYNGOLZA.

ADVERSE REACTIONS

Adverse Reactions for FCS

Most common adverse reactions in patients with FCS (incidence $>5\%$ of TRYNGOLZA-treated patients and $>3\%$ higher frequency than placebo) were injection site reactions, decreased platelet count, and arthralgia.

Adverse Reactions for sHTG

Most common adverse reactions in patients with sHTG (incidence $\geq 2\%$ higher than placebo) were injection site reactions and liver enzyme increases.

Please see full [Prescribing Information](#) for TRYNGOLZA. Please see Important Safety Information throughout and 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, 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, Ionis' technologies and 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. 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® and TRYNGOLZA® are trademarks of Ionis Pharmaceuticals, Inc.

View source version on businesswire.com: <https://www.businesswire.com/news/home/20261015217326/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.