TRYNGOLZA® (olezarsen) approved in the European Union for familial chylomicronemia syndrome (FCS)

September 19, 2025

CARLSBAD, Calif.--(BUSINESS WIRE)--Sep. 19, 2025-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) and Sobi® today announced that TRYNGOLZA® (olezarsen) has been approved in the European Union (EU) as an adjunct to diet in adult patients for the treatment of genetically confirmed familial chylomicronemia syndrome (FCS). The approval follows the positive opinion of the Committee for Medicinal Products for Human Use.

The approval is based on positive data from the Phase 3 Balance study, in which TRYNGOLZA 80 mg demonstrated a statistically significant reduction in fasting 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)*.

"The EU approval of TRYNGOLZA is a significant advance for the treatment of FCS," said Brett P. Monia, Ph.D., chief executive officer, Ionis. "TRYNGOLZA has the potential to be a transformative treatment option for FCS patients in the EU who are at risk of debilitating and life-threatening acute pancreatitis attacks. We are proud to work with Sobi, a long-standing partner of Ionis and the FCS community, to make TRYNGOLZA available to people with FCS in the EU."

FCS is a rare and genetic form of severe hypertriglyceridemia (sHTG). People with FCS often have triglyceride levels of more than 880 mg/dL (10 mmol/L), compared to normal levels of less than 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.

"TRYNGOLZA is the next step in our support for the FCS community in Europe. It has a strong safety and efficacy profile, with significant reductions in triglyceride levels and a notable decrease in acute pancreatitis events, which impact morbidity, mortality and quality of life," said Lydia Abad-Franch, M.D., MBA, head of research, development, and medical affairs (RDMA) and chief medical officer at Sobi. "This builds on our commitment to FCS that began with Waylivra (volanesorsen), the only approved treatment for FCS in Europe until now. With the European Commission's approval of TRYNGOLZA for FCS, we are looking forward to providing this therapy to eligible patients with this rare and debilitating condition across the EU."

Sobi has exclusive rights to commercialize TRYNGOLZA in countries outside the U.S., Canada and China. TRYNGOLZA is also being evaluated for sHTG, defined by severely high triglycerides ≥500 mg/dL (5.65 mmol/L), and positive topline results from the Phase 3 studies were announced in September 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 at six and 12 months 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 and the European Union for adults with familial chylomicronemia syndrome (FCS). Olezarsen is also being evaluated for severe hypertriglyceridemia (sHTG), defined by high fasting triglyceride levels ≥500 mg/dL. For more information, visit TRYNGOLZA.com.

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, 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 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.

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