Ionis expands partnership with Sobi to include olezarsen commercialization outside the U.S.

March 26, 2025

– Agreement enables olezarsen to reach people living with conditions associated with elevated triglycerides across the world –

– Ionis will continue to independently commercialize TRYNGOLZA™ (olezarsen) in the U.S. –

CARLSBAD, Calif.--(BUSINESS WIRE)--Mar. 26, 2025-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that it has entered into a license agreement under which Sobi® receives exclusive rights in countries outside the U.S., Canada and China to commercialize olezarsen as a potential treatment for familial chylomicronemia syndrome (FCS) and severely elevated triglycerides. Ionis will continue to independently commercialize olezarsen in the U.S. Olezarsen was approved by the FDA in December 2024 under the tradename TRYNGOLZA™ as an adjunct to diet to reduce triglycerides in adults with FCS – the first and only treatment in the U.S. for this rare, debilitating genetic disease.

Olezarsen is currently under review by the European Medicines Agency (EMA) with a potential approval anticipated for the treatment of FCS this year. Sobi will be responsible for future regulatory submissions and commercialization in ex-U.S. geographies except Canada and China. Olezarsen was licensed to Theratechnologies for commercialization in Canada.

“We look forward to deepening our long-standing partnership with Sobi and our shared commitment to improving the lives of people living with conditions marked by severely elevated triglycerides,” said Brett P. Monia, Ph.D., chief executive officer, Ionis. “This agreement enables us to advance our goal of making olezarsen available to patients globally, while focusing our independent commercialization efforts in the U.S. With anticipated data from our robust sHTG Phase 3 program in 2025, we are enthusiastic about the potential for olezarsen to also address the significant medical need in this more prevalent patient population in addition to FCS.”

Sobi® (STO: SOBI) brings deep commercialization expertise for both rare and more prevalent conditions, with operations in more than 30 countries, and is committed to bringing olezarsen to as many global patients as possible. As Ionis’ current European commercialization partner for Waylivra® (volanesorsen), which is the only medicine approved for FCS in Europe, Sobi will leverage existing market expertise and distribution channels to enable an effective olezarsen launch in FCS and future potential expansion to a broader population with severely elevated triglycerides, assuming approval. Under the agreement, Ionis receives an upfront payment, additional payments based on achievement of milestones and a tiered royalty of up to mid-20% range on annual net sales.

"We are thrilled to continue our collaboration with Ionis and bring this potential new medicine to patients if approved," said Guido Oelkers, chief executive officer, Sobi. "This will enable us to further leverage our combined strengths and to continue to deliver our commitment to provide innovative medicines that transform the lives of people with rare and debilitating diseases."

In addition to FCS, Ionis is evaluating olezarsen for the treatment of severe hypertriglyceridemia (sHTG) in three Phase 3 clinical trials – CORE, CORE2 and ESSENCE. Data from ESSENCE are expected mid-2025 and data from CORE and CORE2 are expected in the second half of 2025.

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. FCS is estimated to impact up to approximately 3,000 people in the U.S. People living with FCS are at high risk of acute pancreatitis (AP) 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 Severe Hypertriglyceridemia (sHTG)

sHTG is a disease categorized by triglyceride levels of 500 mg/dL and above. It develops due to primary (genetic) and secondary causes including diet and lifestyle, other medical conditions and certain medications. More than three million people are currently estimated to live with sHTG in the U.S. People living with sHTG are at risk of potentially life-threatening acute pancreatitis (AP) and atherosclerotic cardiovascular disease (ASCVD).

Olezarsen has not been reviewed or approved for the treatment of severe hypertriglyceridemia (sHTG) by any regulatory authority.

About TRYNGOLZA™ (olezarsen)

TRYNGOLZA™ (olezarsen) was approved by theU.S. Food and Drug Administration as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS). 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. It is the only treatment currently indicated in the U.S. for FCS, a potentially life-threatening disease. For more information about TRYNGOLZA, 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 six 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 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™ (olezarsen), 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 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.

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