Ionis receives FDA Fast Track designation for olezarsen in patients with familial chylomicronemia syndrome

January 31, 2023

- *FCS is a rare and debilitating genetic disease often leading to significant risk of acute, potentially fatal pancreatitis*
- *If approved, olezarsen would be the first approved treatment for FCS in the U.S.*

CARLSBAD, Calif., Jan. 31, 2023 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that the U.S. Food and Drug Administration (FDA) has granted olezarsen Fast Track designation for the treatment of familial chylomicronemia syndrome (FCS).

FCS is a debilitating genetic disease characterized by severely high levels of plasma triglycerides and a risk of unpredictable and potentially fatal acute pancreatitis. In addition to acute pancreatitis, FCS patients are at risk of chronic complications due to permanent organ damage, including chronic pancreatitis and type 3c diabetes. There are currently no approved therapies for the treatment of FCS in the U.S.

Fast Track designation is designed to expedite the FDA's review of innovative, new drugs that demonstrate the potential to address unmet medical need.

"The FDA Fast Track designation for olezarsen recognizes the urgent need for an effective treatment for FCS, a debilitating rare disease affecting people with very limited treatment options and an elevated risk of painful and potentially fatal bouts of pancreatitis," said Richard S. Geary, Ph.D., executive vice president and chief development officer at Ionis. "We look forward to working collaboratively with the FDA to bring forward a safe and effective treatment for FCS patients as quickly as possible."

Ionis fully enrolled its global Phase 3 BALANCE study of olezarsen in adult patients with FCS last year. The company plans to share data from the BALANCE study in the second half of 2023. In addition to FCS, Ionis is evaluating olezarsen in severe hypertriglyceridemia (SHTG). More information on the BALANCE study (NCT04568434) is available at www.clinicaltrials.gov.

About familial chylomicronemia syndrome (FCS)

FCS is a rare, genetic disease estimated to affect 3,000 to 5,000 people worldwide and is characterized by extremely elevated triglyceride levels. FCS can lead to many chronic health issues including severe, recurrent abdominal pain, fatigue, high risk of life-threatening pancreatitis and abnormal enlargement of the liver or spleen. In addition, people with FCS are often unable to work, adding to their disease burden. In severe cases, patients can have bleeding into the pancreas, serious tissue damage, infection, and cyst formation, as well as damage to other vital organs such as the heart, lungs, and kidneys. Additional information on FCS is available at www.fcsfocus.com and through the FCS Foundation at www.livingwithfcs.org.

About Olezarsen

Olezarsen (formerly IONIS-APOCIII-L_{Rx}) is an investigational LICA medicine designed to inhibit the production of apoC-III for patients who are at risk of disease due to elevated triglyceride levels. ApoC-III is a protein produced in the liver that regulates triglyceride metabolism in the blood. People with severely elevated triglycerides, such as people with FCS, are at high risk for acute pancreatitis and an increased risk of cardiovascular disease.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a premier late-stage pipeline highlighted by industry-leading cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop and deliver life-transforming therapies.

To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of Ionis' technologies, olezarsen 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 related to the impact COVID-19 could have on our business, and 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. 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, 2021, and the most recent Form 10-Q quarterly filing, 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" refers to Ionis Pharmaceuticals and its subsidiaries.

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Ionis Pharmaceuticals Investor Contact: 760-603-2331; Ionis Pharmaceuticals Media Contact: 760-603-4679