Akcea Therapeutics Supports World Lipodystrophy Day

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CAMBRIDGE, Mass., March 31, 2016 /PRNewswire/ -- Akcea Therapeutics, a subsidiary of Ionis Pharmaceuticals (NASDAQ: IONS) focused on developing and commercializing new treatments for serious cardiometabolic diseases, recognizes World Lipodystrophy Day and has partnered with Lipodystrophy United (lipodystrophyunited.org) to raise awareness of those living with lipodystrophy.

"We are proud to join forces with the patient community in celebration of World Lipodystrophy Day. We stand in support of all those affected by familial partial lipodystrophy (FPL). We recognize the urgent need to bring new treatments to these patients. The progress we have made with our Phase 3 drug, volanesorsen, reflects our commitment to developing and commercializing transformative therapies that address the root causes of serious, underserved cardiometabolic disorders, like FPL," said Paula Soteropoulos, President and Chief Executive Officer of Akcea.

FPL is a rare, genetic disorder characterized by metabolic abnormalities, including hypertriglyceridemia and extreme insulin resistance, and abnormalities in the distribution of body fat. Patients with FPL have a high risk at an early age of diabetes, liver disease, and cardiovascular disease. In addition, extreme hypertriglyceridemia puts them at risk for potentially life-threatening pancreatitis. Since it is most often an inherited, autosomal dominant disorder, FPL can be passed down from one generation to the next in affected families.

World Lipodystrophy Day is an international campaign designed to focus attention on the impact of living with lipodystrophy. March 31, 2016, marks the third World Lipodystrophy Day. On this day, patient organizations from countries and regions all over the world will hold awareness-raising activities.

ABOUT VOLANESORSEN
Akcea Therapeutics and Ionis Pharmaceuticals are conducting two Phase 3 studies evaluating volanesorsen in patients with FPL and in patients with familial chylomicronemia syndrome. The Phase 3 study, BROADEN, is a randomized, double-blind, placebo-controlled, multi-center, international study in approximately 50 patients with FPL. The study's primary objective is to evaluate the efficacy and safety of a 300 mg once weekly dose of volanesorsen given over 12 months. The primary endpoint of the study is percent change in fasting triglycerides from baseline after three months of dosing. For more information about The BROADEN Study, please go to clinicaltrials.gov (identifier # NCT02527343) or go to www.apociii.com.

Volanesorsen is a Gen. 2.0+ antisense drug designed to reduce the production of apoC-III, a protein that acts as a key regulator of triglyceride levels in the blood. Patients with elevated triglyceride levels are at significant risk for coronary artery disease and diabetes. Extremely high triglyceride levels put patients at risk of pancreatitis, a serious and potentially life-threating illness. In addition, elevated levels of apoC-III are recognized as an independent contributor to cardiovascular disease. Volanesorsen is currently in Phase 3 trials for two rare, genetic diseases.

ABOUT LIPODYSTROPHY UNITED
Lipodystrophy United (LU) is an organization of committed individuals living strong with lipodystrophy. LU's mission is

to provide an interactive community, facilitating support and education for anyone affected by this rare disease.

ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics is a development and commercialization company focused on transforming the lives of patients with serious cardiometabolic lipid disorders. Established as a wholly owned subsidiary of Ionis Pharmaceuticals, Inc., Akcea has a robust portfolio of development-stage drugs covering multiple targets and disease states using advanced RNA-targeted antisense therapeutics. Akcea's drug pipeline includes novel antisense drugs designed to address a number of lipid risk factors, including LDL-Cholesterol, apoC-III, triglycerides and Lp(a). Akcea's most advanced program, volanesorsen, is in Phase 3 development to treat patients with ultra-orphan lipid disorders that are characterized by extremely high triglycerides and apoC-III, including familial chylomicronemia syndrome (FCS) and familial partial lipodystrophy (FPL). Akcea is located in Cambridge, Massachusetts. Additional information about Akcea is available at www.akceatx.com.

ABOUT IONIS PHARMACEUTICALS, INC.

Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTR_{Rx}, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

FORWARD-LOOKING LANGUAGE STATEMENT

This press release includes forward-looking statements regarding Ionis Pharmaceuticals Inc., its wholly owned subsidiary, Akcea Therapeutics, Inc, and the therapeutic and commercial potential of Akcea's products in development, including volanesorsen. 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, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. 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 December 31, 2015, which is on file with the SEC. Copies of this 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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