

Akcea Therapeutics Announces Presentations of New Data Underscoring Burden of Disease in Familial Chylomicronemia Syndrome

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**--Largest study conducted to date involving people living with FCS--
--Findings to be presented during American Society for Preventative Cardiology Congress--**

CAMBRIDGE, Mass. , Sept. 15, 2016 /PRNewswire/ -- Akcea Therapeutics, a wholly-owned subsidiary of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders, today announced that the company will present new data on the clinical characteristics, impact on quality of life and psychosocial consequences of living with familial chylomicronemia syndrome (FCS). The findings will be presented in two poster presentations at the American Society for Preventative Cardiology (ASPC) 2016 Congress being held September 16-18 in Boca Raton, Florida . These new data reflect findings from the largest study in patients with FCS conducted to date and provide new insights on the disease burden from the perspective of patients and caregivers.



A subsidiary of Ionis Pharmaceuticals, Inc.

"As is often the case, studies such as these in rare patient populations provide insights into the disease and the patients' experience of their disease that are missing from the literature. While each patient's experience of his or her disease is personal and unique, there are common symptoms uncovered, sometimes under-recognized, that build and broaden the medical community's understanding and approach to treating the patient and his or her condition," said Dr. Louis O'Dea, chief medical officer of Akcea. "We are committed to building a comprehensive understanding of FCS by using multiple methodologies to collect additional data relevant to patients' experience living with FCS."

As part of its efforts to better capture the burden of illness experienced by FCS patients, Akcea recently launched the IN-FOCUS study at www.fcsinfo.com. Information gathered from IN-FOCUS can help raise awareness about this rare, severe disease, lead to a better understanding of the burden of this disease and inform treatment approaches for patients with FCS.

Akcea and its collaborators will present the following two posters at the ASPC meeting.

One poster titled "Characterizing Familial Chylomicronemia Syndrome: Baseline Data of the APPROACH Study" presents data from patients participating in APPROACH, the pivotal Phase 3 study evaluating the efficacy and safety of volanesorsen for the treatment of patients with FCS. The findings from this poster provide insights on the diversity of the overall patient population and the clinical characteristics of the disease.

A second poster titled "The Clinical and Psychosocial Burden of Patients with Familial Chylomicronemia Syndrome" presents findings from a facilitator-moderated session, which details the impact of FCS on multiple quality of life measures for patients and caregivers.

ABOUT FAMILIAL CHYLOMICRONEMIA SYNDROME (FCS)

FCS is a rare, genetic disorder and may also be called familial chylomicronemia or Fredrickson Type 1 hyperlipoproteinemia, or familial lipoprotein lipase deficiency. People with FCS are unable to effectively clear lipid particles called chylomicrons. As a result, they have extremely high levels of triglycerides and are at risk of significant morbidity and mortality, including potentially life-threatening pancreatitis. To learn more about FCS visit fcsfocus.com and to learn about the FCS Foundation visit <http://www.livingwithfcs.org/>.

ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics is focused on developing and commercializing drugs for patients with serious cardiometabolic diseases caused by 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. The drugs in Akcea's pipeline are designed using Ionis' advanced RNA-targeted antisense technology to address a number of lipid risk factors, including, ApoC-III, triglycerides, Lp(a) and LDL-cholesterol. Akcea's most advanced program, volanesorsen, is in two Phase 3 programs to treat patients with either familial chylomicronemia syndrome (FCS) or familial partial lipodystrophy (FPL), two orphan lipid disorders that are characterized by extremely high triglycerides and ApoC-III. 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 either familial chylomicronemia syndrome or 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 STATEMENT

This press release includes forward-looking statements regarding the business of Akcea Therapeutics, Inc. , a wholly owned subsidiary of Ionis Pharmaceuticals and the therapeutic and commercial potential of Akcea's technologies and products in development, including volanesorsen, and other products in development. Any statement describing Akcea's 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. Akcea's 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 Akcea's forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Akcea. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Akcea's programs are described in additional detail in Akcea's parent company, Ionis Pharmaceuticals, Inc.'s annual report on Form 10-K for the year ended December 31, 2015 , and its most recent quarterly report on Form 10-Q, which are on file with the SEC . Copies of these and other documents are available at www.ionispharma.com.

In this press release, unless the context requires otherwise, "Akcea," "Company," "we," "our," and "us" refers to Akcea Therapeutics.

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SOURCE Ionis Pharmaceuticals, Inc.

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