Akcea Therapeutics Announces Launch of IN-FOCUS, a Research Study to Assess Impact of Familial Chylomicronemia Syndrome

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People affected by FCS invited to share their experience

CAMBRIDGE, Mass., June 29, 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 the launch of the first-ever research study to understand the multi-dimensional impact of familial chylomicronemia syndrome (FCS) from the perspective of patients. Through this study, called the INvestigation of Findings and Observations Captured in bUrden of Illness Survey in FCS Patients (IN-FOCUS), people affected by FCS can share information about their experience living with the disease anonymously, using a simple web-based survey design.

FCS, also known as lipoprotein lipase deficiency (LPLD) or type 1 hyperlipoproteinemia, is a rare, genetic disease characterized by extremely high levels of triglycerides, the risk of pancreatitis and multiple other complications, which impact patients' daily lives. To date, a holistic assessment of this impact has never been measured from the patient's perspective.

"By participating in the IN-FOCUS study, people living with FCS have a historic opportunity to help other patients, as well as clinicians and care providers, better understand this rare but very serious condition," said Dr. Louis O'Dea, chief medical officer of Akcea. "This study will document the experience of patients suffering from FCS and build a new level of awareness for this under-diagnosed condition that can help guide treatment and support in the years ahead."

Participants will be asked a series of questions to confirm that only patients who are living with FCS are captured in the study. Adults over 18 years of age living with FCS who qualify will then be invited to complete an anonymous survey by navigating directly to www.fcsinfocus.com. Participants will be asked to:

1. share information about how FCS affects different aspects of their life;
2. document experiences related to their symptoms, co-morbidities, health complications, hospital visits, etc.; and,
3. assess whether and how well FCS can be managed by modifications in diet and lifestyle alone.

The survey is anonymous; at no time will participants be asked to reveal any self-identifying information. They will also have the option to respond "Don't Know" to queries if they are uncomfortable sharing any of the requested information.

All people 18 years and older living with FCS are encouraged to participate. Clinicians and care providers working with the FCS community are invited to share information about the survey with patients and encourage them to consider participating.

"This research will help advance medical understanding of diseases of lipid metabolism, especially those that have been under-recognized or for which there are critical gaps in the medical literature," according to Dr. Michael Davidson from the University of Chicago. "With the support of lipid-focused clinicians, we can develop a new cross-disciplinary understanding of FCS and support both medical professionals and FCS patients. Together we can add many essential new insights to the scientific body of knowledge that can help us better identify, understand and manage FCS."

"Many patients and clinicians have confirmed details about the life-altering experience of having FCS, including periods of persistent severe pain, fear of developing pancreatitis, extreme fatigue and many psychosocial challenges, but precise and insightful assessments of disease burden from the patients' perspective have not been captured," said Dr. O'Dea. "As a company focused on transforming the lives of patients with serious cardiometabolic diseases caused by lipid disorders, we at Akcea are committed to advancing understanding of these diseases, both through our clinical trials, as well as through important medical initiatives such as the IN-FOCUS study."

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. Additional information on FCS is available at www.fcsfocus.com.

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 Phase 3

development 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-TTRRx, 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 subsidiary of Ionis Pharmaceuticals and the therapeutic and commercial potential of Akcea's drugs in development, including volanesorsen, and other drugs 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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To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/akcea-therapeutics-announces-launch-of-in-focus-a-research-study-to-assess-impact-of-familial-chylomicronemia-syndrome-300291669.html

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