

Akcea Presents Data Demonstrating Significant Burden of Illness in Familial Chylomicronemia Syndrome

Full results from global IN-FOCUS study presented at NORD Rare Diseases and Orphan Products Breakthrough Summit

CAMBRIDGE, Mass., Oct. 16, 2017 (GLOBE NEWSWIRE) -- Akcea Therapeutics, Inc. (NASDAQ:AKCA), an affiliate of Ionis Pharmaceuticals, Inc., focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders, today reported that new data from the global IN-FOCUS study were presented at the National Organization for Rare Diseases (NORD) Rare Diseases and Orphan Products Breakthrough Summit in Washington, D.C.

The full data set is derived from 166 respondents with familial chylomicronemia syndrome (FCS) in 10 countries in North America, Europe, South America and South Africa. The data demonstrate the disease burden patients with FCS experience, including cognitive, emotional, and physical impairments that affect daily life. In addition to the day-to-day complications of the disease, patients with FCS are at risk of recurrent, potentially fatal, acute pancreatitis. Findings from IN-FOCUS show these manifestations diminish quality of life, can impact career choices and affect the ability to secure and maintain employment.

The full data set from the IN-FOCUS study were presented at the NORD summit in a poster titled: “The Investigation of Findings and Observations Captured in Burden of Illness Survey in Patients with Familial Chylomicronemia Syndrome – Final Results from the IN-FOCUS Study.” The data confirmed an earlier interim analysis of the IN-FOCUS study published in the [Expert Review of Cardiovascular Therapy](#). There was no discernible regional difference in patient-reported disease characteristics.

Dr. Michael Davidson, director, Preventive Cardiology, Department of Medicine, University of Chicago, said: “These data illuminate the range and depth of impact that patients with FCS experience on a daily basis. Despite considerable efforts in trying to manage their symptoms through strict diet, patients live in ongoing fear of hospitalization from acute pancreatitis. Due to chronicity and severity of symptoms, these patients are under-employed, and isolated from family and friends. These data underscore a significant unmet need in FCS.”

“The IN-FOCUS study is the first comprehensive assessment of the patient experience of living with FCS and increases our understanding of the disease. This study gives us and the medical community important insights into aspects of the disease from a patient perspective that have been under-recognized and not previously characterized in literature,” said Dr. Louis O’Dea, chief medical officer of Akcea.

“The results of the IN-FOCUS study represent a true achievement in giving patients a voice to articulate the symptoms, burdens and outcomes of living with FCS. We are encouraged further by the recent increase in disease awareness. We are hopeful that these collective efforts will lead to faster diagnosis, better understanding, and eventually, treatment options for patients with FCS,” said Lindsey Sutton, co-president of the FCS Foundation.

To view data from the interim analysis of the IN-FOCUS study, please click [here](#).

ABOUT FCS

FCS is a severe, rare disorder characterized by extremely high levels of triglycerides, daily symptoms such as abdominal pain, and the risk of recurrent, potentially fatal, acute pancreatitis. People with FCS are unable to effectively metabolize large, triglyceride-rich lipid particles called chylomicrons due to a deficiency in

lipoprotein lipase, an enzyme that helps to break down triglycerides. There is no effective therapy available. Additional information on FCS is available at www.fcsfocus.com and through the FCS Foundation at <http://www.livingwithfcs.org> and the LPLD Alliance at www.lpldalliance.org.

ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics, an affiliate of Ionis Pharmaceuticals, Inc., is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders. Akcea is advancing a mature pipeline of four novel drugs, including volanesorsen, AKCEA-APO(a)-LRx, AKCEA-ANGPTL3-LRx and AKCEA-APOCIII-LRx, all with the potential to treat multiple diseases. All four drugs were discovered by and are being co-developed with Ionis, a leader in antisense therapeutics, and are based on Ionis' proprietary antisense technology. Akcea is building the infrastructure to commercialize its drugs globally with a focus on lipid specialists as the primary call point. Akcea is located in Cambridge, Massachusetts. Additional information about Akcea is available at www.akceatx.com.

FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the business of Akcea Therapeutics, Inc. and the therapeutic and commercial potential of 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 its final prospectus for its initial public offering and its most recent quarterly report on Form 10-Q, which are on file with the SEC.

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

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