

## **Akcea Therapeutics Establishes Canadian Subsidiary Appoints Canadian General Manager**

CAMBRIDGE, Mass., Sept. 06, 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 announced the establishment of its Ottawa-based Canadian subsidiary and the appointment of Jared Rhines as General Manager for Akcea Therapeutics Canada Inc. This important step reflects Akcea's commitment to Canadian patients, their caregivers and the Canadian medical community. Mr. Rhines plans to build and lead Akcea Therapeutics Canada, setting the stage for the potential commercial introduction in 2018 of the organization's most advanced drug, volanesorsen, for the treatment of familial chylomicronemia syndrome (FCS). In addition, Akcea Therapeutics Canada has joined Innovative Medicines Canada, the association that represents the innovative pharmaceutical industry in Canada.

Mr. Rhines has led the successful launch of three orphan drug products in Canada. In addition, his prior roles have included extensive international experience in drug and device development, regulatory submissions and approvals, and commercialization in Canada, Asia, Latin America, the United States and Europe. Based in Ottawa, he most recently served as Group Vice President and General Manager, Canada for Horizon Pharma. He is a past member of the Board of Directors of Innovative Medicines Canada and its Health Research Foundation. He holds a Bachelor of Arts from the University of Pennsylvania and a Master of Business Administration from Duke University's Fuqua School of Business.

"We are delighted to formally establish our presence in Canada as part of the expansion of Akcea's global operations. I am also pleased to have Jared join us in Canada to lead our new subsidiary," said Paula Soteropoulos, president and chief executive officer of Akcea. "Today is a milestone for Akcea and our connections to this country, which run very deep. Canada can be justifiably proud of its world-renowned scientists in lipidology who have played a critical role in the clinical trials for volanesorsen. The opportunity for volanesorsen in FCS began with a Canadian study, and the clinical program has included six trial sites in Canada. In fact, many of the patients enrolled in the landmark, multi-national, Phase 3 APPROACH study in FCS were from Canada. Additionally, Jared brings vital knowledge of Canada's unique healthcare system and regulatory environment and enjoys strong professional relationships that will be invaluable in collaborating with our many stakeholders."

"Being able to help patients with a rare disease is extremely humbling and personally motivating," said Jared Rhines, general manager of Akcea Therapeutics Canada. "Building awareness of FCS and volanesorsen in Canada, listening and collaborating with our stakeholders across the country and providing local market intelligence and responsiveness is how we will bring value to patients, caregivers, and the medical community. I am confident and excited about helping shape Akcea Canada and the contributions we will be able to make in Canada and globally. As a member of Innovative Medicines Canada, Akcea will now be present and vocal in representing the particular challenges of rare diseases within the association that speaks for Canada's research-based pharmaceutical companies."

### **ABOUT VOLANESORSEN, FCS AND FPL**

Volanesorsen, a product of Ionis' proprietary antisense technology, is in development for two rare metabolic disorders: FCS and FPL. Volanesorsen is designed to reduce the production of ApoC-III, a protein produced in the liver that plays a central role in the regulation of plasma triglycerides and may also affect other metabolic parameters.

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](http://www.fcsfocus.com) and through the FCS Foundation at <http://www.livingwithfcs.org> and the LPLD Alliance at [www.lpldalliance.org](http://www.lpldalliance.org).

FPL is a severe, rare genetic metabolic disorder characterized by an inability of the body to store fat in normal locations. This results in high levels of triglycerides in the bloodstream, abnormal fat distribution around and within organs, such as the liver and heart, and a range of metabolic abnormalities, including severe insulin resistance. People with FPL are at increased risk of acute pancreatitis in addition to other long-term, progressive manifestations, such as premature cardiomyopathy, atherosclerosis, and liver disease. Additional information on FPL is available through Lipodystrophy United at [www.lipodystrophyunited.org](http://www.lipodystrophyunited.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 with the potential to treat multiple diseases, including volanesorsen, AKCEA-APO(a)-LRx, AKCEA-ANGPTL3-LRx and AKCEA-APOCIII-LRx. All four drugs were discovered and are being co-developed by Ionis, a leader in antisense therapeutics, based on Ionis' proprietary antisense technology. The most advanced drug in its pipeline, volanesorsen, is under regulatory review in the U.S. and EU for the treatment of familial chylomicronemia syndrome, or FCS, and is currently in Phase 3 clinical development for the treatment of familial partial lipodystrophy, or FPL. 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](http://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.

Akcea Therapeutics™ is a trademark of Ionis Pharmaceuticals, Inc. Ionis Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc.

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<http://www.ionispharma.com>

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