Akcea Therapeutics Announces Completion of Enrollment in Phase 3 COMPASS Trial of Volanesorsen

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Study designed to support regulatory filings in U.S., E.U. and Canada for volanesorsen

CAMBRIDGE, Mass., May 16, 2016 /PRNewswire/ -- Akcea Therapeutics, a subsidiary of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) focused on developing and commercializing innovative treatments for serious cardiometabolic diseases, today announced the completion of enrollment in the COMPASS study, a randomized, double-blind, placebo-controlled, Phase 3 study of volanesorsen in patients with severe hypertriglyceridemia.

The COMPASS study (ClinicalTrials.gov identifier: NCT02300233) is one of three ongoing global trials that form the Phase 3 program for volanesorsen. It is designed to provide important safety data in support of future regulatory filings in the U.S., E.U. and Canada for volanesorsen in two indications – familial chylomicronemia syndrome (FCS) and familial partial lipodystrophy (FPL). Akcea's two other ongoing international, multi-center Phase 3 studies of volanesorsen are the APPROACH study in patients with FCS and the BROADEN study in patients with FPL.

"The COMPASS study was designed to yield important data to strengthen our robust safety database for volanesorsen beyond what we will collect in our ongoing APPROACH and BROADEN trials," said Dr. Louis O'Dea, Akcea's chief medical officer. "Our success in reaching this important milestone ahead of schedule demonstrates our ability to successfully conduct a complex, multi-center global clinical trial. Most importantly, we expect the safety data available from the 115 patients enrolled in this trial will bolster what we anticipate will be a compelling case in support of timely access to volanesorsen for people with FCS and FPL."

The APPROACH Phase 3 study in FCS patients completed enrollment at the end of 2015 and data from this study are expected in the first half of 2017. Data from the BROADEN Phase 3 study in FPL are planned for the first half of 2018.

ABOUT VOLANESORSEN, FCS and FPL
Volanesorsen is an antisense drug in development intended to treat patients with severely high triglycerides either as a single agent or in combination with other triglyceride-lowering agents. 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.

Volanesorsen is currently being evaluated in a Phase 3 study in patients with FCS (APPROACH). 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

A second Phase 3 study of volanesorsen (BROADEN) has begun in patients with familial partial lipodystrophy (FPL). FPL is a rare lipid disorder characterized by abnormal fat distribution across the body and a range of metabolic abnormalities, including severe insulin resistance, dyslipidemia and hypertriglyceridemia, hepatic steatosis and, in affected women, features of hyperandrogenism. People with FPL often present with polycystic ovarian syndrome (PCOS) or unusually insulin-resistant diabetes, and are at increased risk of acute pancreatitis in addition to long-term, progressive consequences including premature cardiovascular disease and liver disease, resulting in cirrhosis. They are unable to store fat or triglycerides in normal fat stores, so excess triglycerides are stored in the liver and muscle and accumulate at high levels in the bloodstream. Additional information on FPL is available through Lipodystrophy United at www.lipodystrophyunited.org.

For more information about this clinical trial program for volanesorsen, please visit www.apociii.com.

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 either familial chylomicronemia syndrome (FCS) or familial partial lipodystrophy (FPL), two ultra-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 and/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, if approved, 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 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 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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To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/akcea-therapeutics-announces-completion-of-enrollment-in-phase-3-compass-trial-of-volanesorsen-300268661.html

SOURCE Ionis Pharmaceuticals, Inc.

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