Akcea and Ionis to Present Phase 2 Clinical Data of Vupanorsen (AKCEA-ANGPTL3-L (Rx)) at ESC Congress 2020

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Late-breaking presentation to focus on efficacy and safety data of vupanorsen in patients with hypertriglyceridemia, type 2 diabetes and non-alcoholic fatty liver disease

BOSTON and CARLSBAD, Calif., Aug. 24, 2020 /PRNewswire/ -- Akcea Therapeutics, Inc. (NASDAQ: AKCA), a majority-owned affiliate of Ionis Pharmaceuticals, Inc., and Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), today announced that data from the Phase 2 clinical trial of vupanorsen (AKCEA-ANGPTL3-L_{Rx}), an investigational antisense therapy being developed to treat patients with certain cardiovascular diseases, will be presented in a Late Breaking Clinical Trial Session at the upcoming ESC Congress 2020, the annual meeting of the European Society of Cardiology, which is expected to be the world's largest online gathering of cardiovascular professionals and is taking place August 29-September 1, 2020.

Vupanorsen was developed using Ionis' proprietary LIgand Conjugated Antisense (LICA) technology platform to reduce the production of angiopoietin-like 3 (ANGPTL3) protein, a key regulator of triglyceride and cholesterol metabolism, in the liver. The Phase 2 dose-ranging clinical trial evaluated the safety and efficacy of vupanorsen in patients with hypertriglyceridemia, type 2 diabetes, and non-alcoholic fatty liver disease (NAFLD).

In November 2019, Akcea and Ionis announced the closing of the worldwide exclusive licensing agreement with Pfizer Inc. for vupanorsen (AKCEA-ANGPTL3-L_{Rx}). Under terms of the agreement, Akcea and Ionis received a $250 million upfront license fee, which was split equally between the two companies. Pfizer is responsible for all development and regulatory activities and costs beyond those associated with this Phase 2 study.

Following are details about the late-breaking virtual presentation at ESC Congress 2020, which will be made available on the Akcea website:

- Late-Breaking Science in Lipids: ANGPTL3 Antisense Oligonucleotide to Lower Triglycerides by Daniel Gaudet, M.D., professor of medicine, Department of Medicine, University of Montreal
  Oral Presentation: Saturday, August 29, 2020, 3:00 a.m. ET/9:00 a.m. CEST

ABOUT VUPANORSEN (AKCEA-ANGPTL3-L_{Rx})

AKCEA-ANGPTL3-L_{Rx} (vupanorsen) is an investigational antisense therapy being developed to treat patients with certain cardiovascular diseases. This antisense medicine is designed to reduce the production of angiopoietin-like 3 (ANGPTL3) protein, a key regulator of triglyceride and cholesterol metabolism, in the liver. AKCEA-ANGPTL3-L_{Rx} was developed using Ionis' advanced LIgand Conjugated Antisense (LICA) technology platform. The potential therapeutic benefits of ANGPTL3 reduction are supported by the discovery that people with a genetic deficiency in ANGPTL3 have reduced levels of low-density lipoprotein cholesterol (LDL-C) and triglycerides, and a decreased risk of cardiovascular disease.[1] In a previous Phase 1 study, patients treated with AKCEA-ANGPTL3-L_{Rx} achieved robust, dose-dependent reductions in ANGPTL3, triglycerides, LDL-C, non-HDL-C and total cholesterol with a positive safety and tolerability profile.[2] AKCEA-ANGPTL3-L_{Rx} was discovered by Ionis and has been co-developed by Akcea and Ionis.

ABOUT AKCEA THERAPEUTICS

Akcea Therapeutics, Inc., a majority-owned affiliate of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), is a biopharmaceutical company focused on developing and commercializing medicines to treat patients with serious and rare diseases. Akcea is commercializing TEGSEDI® (inotersen) and WAYLIVRA® (volanesorsen), as well as advancing a mature pipeline of novel medicines, including AKCEA-APO(a)-L_{Rx}, vupanorsen (AKCEA-ANGPTL3-L_{Rx}), AKCEA-APOCIII-L_{Rx}, and AKCEA-TTR-L_{Rx}, with the potential to treat multiple diseases. All six drugs were discovered by Ionis, a leader in antisense therapeutics, and are based on Ionis' proprietary antisense technology. TEGSEDI is approved in the U.S., E.U., Canada and Brazil, and WAYLIVRA is approved in the E.U. Akcea is headquartered in Boston and is building the infrastructure to commercialize its medicines globally. Additional information about Akcea is available at www.akceatx.com and you can follow us on Twitter at @akceatx.

ABOUT IONIS PHARMACEUTICALS, INC.

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, drug discovery platform called antisense technology that can treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs. We created the first and only approved treatment for children and adults with spinal muscular atrophy as well as the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy in adults with hereditary transthyretin amyloidosis. Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 novel medicines designed to potentially treat a broad range of disease, including neurological, cardio-renal, metabolic, infectious, and pulmonary diseases. To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

AKCEA AND IONIS FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the business of Akcea Therapeutics, Inc. and Ionis Pharmaceuticals, Inc. and the therapeutic and commercial potential of AKCEA-ANGPTL3-L_{Rx}. Any statement describing Akcea's or Ionis' goals, expectations, financial or other projections, intentions or beliefs, including the commercial potential of AKCEA-ANGPTL3-L_{Rx} or other of Akcea's or Ionis' drugs in development 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 and Ionis' 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 and Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Akcea and Ionis. In particular, we caution you that our forward-looking statements are subject to the ongoing and developing circumstances related to the COVID-19 pandemic, which may have a material adverse effect on our business, operations and future financial results. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Akcea's and Ionis' programs are described in additional detail in Akcea's and Ionis' quarterly reports on Form 10-Q and annual reports on Form 10-K, which are on file with the SEC. Copies of these and other documents are available from each company.

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

References

1. JAMA Cardiol. 2018 Oct 1;3(10):957-966.
2. N Engl J Med. 2017 Jul 20;377(3):222-232.

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SOURCE Akcea Therapeutics, Inc.; Ionis Pharmaceuticals, Inc.

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