

## Akcea and Ionis to Present Phase 2 Results of AKCEA-APOCIII-L(Rx) in Late-Breaking Presentation at ESC Congress 2020

August 18, 2020

**Data presentation will highlight efficacy, safety and tolerability of investigational medicine in patients with hypertriglyceridemia and cardiovascular disease**

BOSTON and CARLSBAD, Calif., Aug. 18, 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 study of AKCEA-APOCIII-L<sub>Rx</sub> 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, taking place August 29-September 1, 2020.



AKCEA-APOCIII-L<sub>Rx</sub> is an antisense drug developed using Ionis' proprietary **Ligand Conjugated Antisense (LICA)** technology platform and is designed to reduce the production of apolipoprotein C-III (apoC-III), a protein produced in the liver that plays a central role in the regulation of serum triglycerides. AKCEA-APOCIII-L<sub>Rx</sub> was discovered by Ionis and has been co-developed by Akcea and Ionis. A prior Phase 1/2 study of AKCEA-APOCIII-L<sub>Rx</sub> in healthy volunteers with elevated triglyceride levels showed dose-dependent reductions in apoC-III, triglycerides and atherogenic lipoproteins, and increase in high-density lipoprotein cholesterol (HDL-C) following treatment.

Following are details about the Phase 2 study late-breaking virtual presentation at ESC Congress 2020, which will be made available on the Akcea website:

- Late-Breaking Science in Lipids: Apolipoprotein C-III Reduction in Cardiovascular Disease by Professor Jean-Claude Tardif, Montreal Heart Institute, University of Montreal  
*Oral Presentation: Saturday, August 29, 2020, 2:50 a.m. ET/8:50 a.m. CEST*

### **ABOUT AKCEA-APOCIII-L<sub>Rx</sub>**

AKCEA-APOCIII-L<sub>Rx</sub> is a ligand conjugated antisense (LICA) drug designed to reduce the production of apolipoprotein C-III, or apoC-III. ApoC-III is a protein produced in the liver that plays a central role in the regulation of serum triglycerides. Genetically reduced levels of apoC-III are correlated to lower levels of triglycerides and lower risk of cardiovascular disease whereas elevated levels of apoC-III correlate with high triglyceride levels that have been associated with multiple metabolic abnormalities, such as insulin resistance and/or metabolic syndrome as well as elevated cardiovascular event risk. AKCEA-APOCIII-L<sub>Rx</sub> is in Phase 2 development for hypertriglyceridemia and established cardiovascular disease. Akcea and Ionis intend to pursue development in familial chylomicronemia syndrome, or FCS, and are considering pursuing development in additional indications.

### **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<sub>Rx</sub>, vupanorsen (AKCEA-ANGPTL3-L<sub>Rx</sub>), AKCEA-APOCIII-L<sub>Rx</sub>, and AKCEA-TTR-L<sub>Rx</sub>, 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](http://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](http://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-APOCIII-L<sub>Rx</sub>. Any statement describing Akcea's or Ionis' goals, expectations, financial or other projections, intentions or beliefs, including the commercial potential of AKCEA-APOCIII-L<sub>Rx</sub> 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.

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

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