Akcea and Ionis Complete Licensing Transaction to Commercialize Inotersen for hATTR

April 17, 2018

Akcea shareholders approved the transaction on April 16 at a Special Meeting of Stockholders

Ionis licenses Akcea worldwide rights to inotersen and AKCEA-TTR-L_{Rx}

CAMBRIDGE, Mass. and CARLSBAD, Calif., April 17, 2018 (GLOBE NEWSWIRE) -- Akcea Therapeutics, Inc. (NASDAQ:AKCA), an affiliate of Ionis Pharmaceuticals, Inc., and Ionis Pharmaceuticals, Inc. (NASDAQ:IONS) today announced the two companies have completed a previously announced transaction licensing the exclusive, worldwide rights from Ionis to Akcea for inotersen and AKCEA-TTR-L_{Rx}, formerly IONIS-TTR-L_{Rx}.

Inotersen is under regulatory review in the U.S. and EU with approvals planned for mid-2018 for the treatment of hereditary transthyretin amyloidosis, or hATTR. hATTR is a systemic, progressive and fatal disease. Akcea and Ionis are also developing AKCEA-TTR-L_{Rx} for hereditary and wild-type forms of ATTR and plan to commence clinical studies for AKCEA-TTR-L_R in 2018.

In addition, following the close of the transaction, today, Sarah Boyce joined Akcea as president and a member of the Akcea board of directors reporting to Paula Soteropoulos, Akcea's chief executive officer. Ms. Boyce was formerly the chief business officer at Ionis.

The transaction was subject to certain closing conditions, including a non-waivable condition that the stock purchase agreement, the license agreement and related agreements and the transaction be approved by the affirmative vote of holders representing a majority of the issued and outstanding shares of common stock other than Ionis and its affiliates, which excluded a vote of Akcea's directors and officers. This affirmative vote was obtained at a special meeting of Akcea stockholders on April 16, 2018.

TRANSACTION TERMS

Under the agreement, Akcea paid Ionis an upfront licensing fee of $150 million through the issuance of 8,000,000 shares of common stock priced at $18.75 per share. Akcea obtained rights to commercialize inotersen and AKCEA-TTR-L_{Rx} globally. To support the incremental resources required for the launch of inotersen and to progress the clinical development program for ACKEA-TTR-L_{Rx}, Ionis purchased $200 million of Akcea common stock, or 10,666,666 shares, priced at $18.75 per share. Upon closing this transaction, Ionis' ownership in Akcea increased by 7%, from 68% to 75%, totaling 64,114,545 shares. Regulatory approval of inotersen and AKCEA-TTR-L_{Rx} in the U.S. and EU will trigger milestone payments to Ionis of $50 million and $40 million, respectively, for each drug, with additional milestone payments due upon approval of both programs in various other geographies. The initial milestone payments may be payable in Akcea common stock at fair market value. Commercial profits and losses from inotersen will be split 60% to Ionis and 40% to Akcea until the first commercial sale of AKCEA-TTR-L_{Rx}, after which the profits and losses will be shared 50/50. The costs of the development of AKCEA-TTR-L_{Rx} and the profits from its commercialization will be shared 50/50. The license for the two drugs also includes various sales milestone payments of up to nearly $1.3 billion. For this transaction, Ionis was advised by Stifel, Nicolaus & Company, Incorporated and Akcea was advised by Cowen and Company, LLC.

ABOUT INOTERSEN

Inotersen is an antisense drug designed to reduce the production of transthyretin, or TTR protein, to treat TTR amyloidosis (ATTR), a systemic, progressive and fatal disease.

Inotersen is currently under regulatory review for marketing authorization in the U.S. and EU. The U.S. Food and Drug Administration has granted Orphan Drug Designation and Fast Track Status to inotersen and the European Medicines Agency has granted Orphan Drug Designation to inotersen.

ABOUT HEREDITARY TRANSTHYRETIN AMYLOIDOSIS (hATTR)

hATTR is a progressive, systemic, and fatal genetic disease caused by the inappropriate formation and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy.

Ultimately, hATTR results in death within three to fifteen years of symptom onset. Therapeutic options for the treatment of patients with hATTR are limited and there are currently no disease-modifying drugs approved for hATTR. There are an estimated 50,000 patients with hATTR worldwide.

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 or best-in-class drugs, with over 40 drugs in development. SPINRAZA® (nusinersen) has been approved in global markets for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. Inotersen and volanesorsen are two antisense drugs that Ionis discovered and successfully advanced through Phase 3 studies. Inotersen is under regulatory review for marketing approval in the U.S. and EU for the treatment of patients with hereditary ATTR amyloidosis. Volanesorsen is under regulatory review for marketing approval in the U.S., EU and Canada for the treatment of patients with familial chylomicronemia syndrome, or FCS. Volanesorsen is also in a Phase 3 study in patients with familial partial lipodystrophy, or FPL. Akcea, an affiliate of Ionis focused on developing and commercializing drugs to treat patients with serious and rare diseases, will commercialize inotersen and volanesorsen, if approved. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

ABOUTAKCEATHERAPEUTICS

Akcea Therapeutics, an affiliate of Ionis Pharmaceuticals, Inc. (NASDAQ:IONS), is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea is advancing a mature pipeline of six novel drugs, including inotersen,

volanesorsen, AKCEA-APO(a)-L_{Rx}, AKCEA-ANGPTL3-L_{Rx}, AKCEA-APOCIII-L_{Rx}, and AKCEA-TTR-L_{Rx}, all with the potential to treat multiple diseases. All six drugs were discovered by and are being co-developed with Ionis, a leader in antisense therapeutics, and are based on Ionis' proprietary antisense technology. Inotersen is under regulatory review in the U.S. and EU for the treatment of hereditary transthyretin amyloidosis (hATTR). Volanesorsen is under regulatory review in the U.S., EU and Canada 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. Akcea is a global company headquartered in Cambridge, Massachusetts. Additional information about Akcea is available at www.akceatx.com.

IONIS' AND AKCEA'S FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the recently announced transaction between Ionis and Akcea, Ionis' and Akcea's business and the therapeutic and commercial potential of inotersen, AKCEA-TTR-L_{Rx} and other products in development. Any statement describing Ionis' or Akcea's goals, expectations, financial or other projections, intentions or beliefs, including the commercial potential of inotersen, volanesorsen or other of Ionis' or Akcea's 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. Ionis' and 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 Ionis' and 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 Ionis and Akcea. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' and Akcea's programs are described in additional detail in Ionis' and Akcea's annual reports on Form 10-K for the year ended December 31, 2017, 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.

Source: Ionis Pharmaceuticals, Inc.