

Isis Pharmaceuticals Earns \$15 Million From GSK for Advancing ISIS-TTR Rx

March 2, 2015

CARLSBAD, Calif., March 2, 2015 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a \$15 million milestone payment from GSK related to advancing the Phase 2/3 study of ISIS-TTR_{Rx} in patients with familial amyloid polyneuropathy (FAP).



"We are very pleased with the progress we are making with ISIS-TTR_{Rx}, and plan to report data from the Phase 2/3 study in 2017. We have many patients who have completed the fifteen months of dosing and are now receiving ISIS-TTR_{Rx} in an open-label extension study," said B. Lynne Parshall, chief operating officer at Isis Pharmaceuticals.

ISIS-TTR_{Rx} is an antisense drug Isis is developing with GSK for the treatment of transthyretin amyloidosis, a severe and rare genetic disease characterized by progressive dysfunction of peripheral nerve and/or heart tissues. Including this milestone payment, Isis has generated \$60 million in upfront and milestone payments for advancing ISIS-TTR_{Rx}. In addition, if GSK elects to exercise its option to exclusively license ISIS-TTR_{Rx}, Isis is eligible to receive a license fee, regulatory and sales milestone payments and double-digit royalties on sales of ISIS-TTR_{Rx}.

The Phase 2/3 study of ISIS-TTR_{Rx} is a randomized, double-blind, placebo-controlled, international study designed to support an application for marketing approval of ISIS-TTR_{Rx} in patients with FAP. The fifteen month study will measure the effects of ISIS-TTR_{Rx} on neurological dysfunction and on quality-of-life. ISIS-TTR_{Rx} is a drug that is designed to inhibit the production of all forms of TTR, and to treat all types of transthyretin-related amyloidosis. For further study information, please visit www.clinicaltrials.gov and search for the identifier number NCT01737398.

ABOUT ISIS PHARMACEUTICALS, INC.

Isis is exploiting its leadership position in RNA-targeted technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 38 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic, severe and rare diseases, including neurological disorders, and cancer. Isis' partner, Genzyme, is commercializing Isis' lead product, KYNAMRO[®], in the United States and other countries for the treatment of patients with homozygous FH. Isis has numerous drugs in Phase 3 development in severe/rare diseases and cardiovascular diseases. These include ISIS-APOCIII_{Rx}, a drug Isis is developing through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with severely high triglycerides, such as patients with familial chylomicronemia syndrome; ISIS-TTR_{Rx}, a drug Isis is developing with GSK to treat patients with the polyneuropathy form of TTR amyloidosis; and, ISIS-SMN_{Rx}, a drug Isis is developing with Biogen Idec to treat infants and children with spinal muscular atrophy, a severe and rare neuromuscular disease. Isis' patents provide strong and extensive protection for its drugs and technology. Additional information about Isis is available at www.isispharm.com.

ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Isis' strategic alliance with GSK, and the development, activity, therapeutic and commercial potential and safety of ISIS-TTR_{Rx}. Any statement describing Isis' 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. Isis' 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 Isis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Isis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Isis' programs are described in additional detail in Isis' annual report on Form 10-K for the year ended December 31, 2014, which is on file with the SEC. Copies of this and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Isis," "Company," "we," "our," and "us" refers to Isis Pharmaceuticals and its subsidiaries.

Isis Pharmaceuticals[®] is a registered trademark of Isis Pharmaceuticals, Inc. Akcea Therapeutics[™] is a trademark of Isis Pharmaceuticals, Inc. KYNAMRO[®] is a registered trademark of Genzyme Corporation.

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