

Isis Pharmaceuticals Earns \$5 Million in Milestone Payment From GSK for Advancement of ISIS-GSK5 Rx

November 3, 2014

CARLSBAD, Calif., Nov. 3, 2014 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a \$5 million milestone payment from GSK related to the designation of ISIS-GSK5_{Rx} as a development candidate. ISIS-GSK5_{Rx} is an antisense drug designed to treat an undisclosed ocular disease.



"ISIS-GSK5_{Rx} is the second drug in our collaboration with GSK to enter development to treat an ocular disease. This drug utilizes the unique properties of antisense to selectively reduce the production of a target known to be involved in various ocular diseases. We look forward to advancing this program and sharing more information on the target and disease," said B. Lynne Parshall, chief operating officer at Isis. "Our collaboration with GSK has been very productive and has resulted in a number of novel antisense drugs in our pipeline to treat a diverse range of diseases."

ABOUT ISIS PHARMACEUTICALS, INC.

Isis is exploiting its leadership position in antisense technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 33 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 and rare and cardiovascular disease. These include a novel triglyceride lowering drug, ISIS-APOCIII_{Rx}, for patients with familial chylomicronemia syndrome; ISIS-TTR_{Rx}, which Isis is developing with GSK to treat patients with the polyneuropathy form of TTR amyloidosis; and, ISIS-SMN_{Rx}, which 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 discovery, development, activity, therapeutic and commercial potential and safety of antisense drugs to treat ocular diseases. 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, 2013, 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 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. Regulus Therapeutics[™] is a trademark of Regulus Therapeutics Inc. KYNAMRO[®] is a registered trademark of Genzyme Corporation.

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