

## Isis Pharmaceuticals Earns \$2 Million From GSK for the Advancement of ISIS-TTR Rx

July 30, 2013

CARLSBAD, Calif., July 30, 2013 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a \$2 million milestone payment from GlaxoSmithKline (NYSE: GSK) related to the advancement of the ongoing Phase 2/3 study of ISIS-TTR<sub>Rx</sub> in patients with familial amyloid polyneuropathy (FAP).

ISIS-TTR<sub>Rx</sub> is an antisense drug in development 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. Isis has earned \$20 million in upfront and milestone payments prior to first patient dosed in the ongoing Phase 2/3 study. The \$2 million milestone payment announced today is the first milestone of the \$50 million in milestone payments Isis is eligible to earn as the Phase 2/3 study progresses. In addition, if GSK elects to exercise its option to exclusively license the ISIS-TTR<sub>Rx</sub> program, Isis is eligible to receive a license fee, regulatory and sales milestone payments and double-digit royalties on sales of ISIS-TTR<sub>Rx</sub>.

The Phase 2/3 study of ISIS-TTR<sub>Rx</sub> is a randomized, double-blind, placebo-controlled, international study designed to support an application for marketing approval of ISIS-TTR<sub>Rx</sub> in patients with FAP. The fifteen month study will measure the effects of ISIS-TTR<sub>Rx</sub> on neurological dysfunction and on quality-of-life. For further study information, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and search for the identifier number NCT01737398.

### 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 28 drugs to treat a wide variety of diseases with an emphasis on cardiovascular, metabolic, severe and rare diseases, and cancer. Isis' partner, Genzyme, is commercializing Isis' lead product, KYNAMRO™, in the United States for the treatment of patients with HoFH. Genzyme is also pursuing marketing approval of KYNAMRO in other markets. Isis' patents provide strong and extensive protection for its drugs and technology. Additional information about Isis is available at [www.isispharm.com](http://www.isispharm.com).

### ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Isis' strategic alliance with GlaxoSmithKline, and the discovery, development, activity, therapeutic and commercial potential and safety of ISIS-TTR<sub>Rx</sub>. Any statement describing Isis' goals, expectations, financial or other projections, intentions or beliefs, including the commercial potential of KYNAMRO, 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, 2012 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. KYNAMRO™ is a trademark of Genzyme Corporation.

SOURCE Isis Pharmaceuticals, Inc.

D. Wade Walke, Ph.D. Executive Director, Corporate Communications and Investor Relations, 760-603-2741; Amy Blackley, Ph.D., Associate Director, Corporate Communications, 760-603-2772