Isis Pharmaceuticals Earns $3.5 Million From Biogen Idec

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CARLSBAD, Calif., May 9, 2013 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that is has earned a $3.5 million milestone payment from Biogen Idec associated with the dosing of the first patient in a Phase 2 study of ISIS-SMN$_{Rx}$ in infants with spinal muscular atrophy (SMA).

The Phase 2 study of ISIS-SMN$_{Rx}$ is an open-label, multiple-dose, dose-escalation pilot study, which will include eight infants who have been diagnosed with SMA. To meet enrollment criteria, infants must be between the ages of three weeks and seven months, live in close proximity to a study site and pass screening evaluations conducted at study sites. The study will be conducted at centers in the United States and Canada. For further study information, please visit www.clinicaltrials.gov and search for the identifier number NCT01839656.

ABOUT ISIS-SMN$_{Rx}$

ISIS-SMN$_{Rx}$ is designed to alter the splicing of a closely related gene (SMN2) to increase production of fully functional SMN protein. The United States Food and Drug Administration granted orphan drug status and fast track designation to ISIS-SMN$_{Rx}$ for the treatment of patients with SMA. Isis is currently in collaboration with Biogen Idec to develop and potentially commercialize the investigational compound, ISIS-SMN$_{Rx}$, to treat all types of SMA. Under the terms of the January 2012 agreement, Isis is responsible for global development and Biogen Idec has the option to license the compound until completion of the first successful Phase 2/3 study. ISIS-SMN$_{Rx}$ is currently being evaluated in a Phase 1b/2a multiple-dose, dose-escalation study in children with SMA. In this study, children will either receive two or three doses of ISIS-SMN$_{Rx}$ over the course of the study.

Isis acknowledges support from the following organizations for ISIS-SMN$_{Rx}$: Muscular Dystrophy Association, SMA Foundation, Families of SMA and intellectual property licensed from Cold Spring Harbor Laboratory and the University of Massachusetts Medical School.

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™, inthe 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.

ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Isis' strategic alliance with Biogen Idec, and the discovery, development, activity, therapeutic and commercial potential and safety of ISIS-SMN$_{Rx}$. 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.

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