Isis Earns $10 Million Milestone Payment from Biogen Idec for Advancement of ISIS-DMPK Rx to Treat Myotonic Dystrophy

October 16, 2013

CARLSBAD, Calif., Oct. 16, 2013 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a $10 million milestone payment from Biogen Idec related to the selection and advancement of ISIS-DMPK$_{Rx}$ to treat myotonic dystrophy type I (DM1).

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"We are very pleased with the successes we are having in our alliance with Biogen Idec. The progress we have made is evidenced in our spinal muscular atrophy and myotonic dystrophy programs. In less than two years, we discovered and advanced ISIS-DMPK$_{Rx}$ into development, and we plan to begin human clinical studies next year," said B. Lynne Parshall, chief operating officer at Isis. "This summer we and Biogen Idec built upon our successful relationship to create a broad strategic alliance that combines our antisense technology with Biogen Idec's knowledge of neurodegenerative diseases and global reach, with the goal of bringing new drugs to the market to treat patients with neurological disorders."

DM1 is a rare genetic neuromuscular disease characterized by progressive muscle atrophy, weakness and disabling muscle spasms. DM1, the most common form of muscular dystrophy in adults, is estimated to affect approximately 150,000 patients in the United States, Europe and Japan. DM1 is caused by a genetic defect in the dystrophia myotonica-protein kinase (DMPK) gene in which a sequence of three nucleotides repeats extensively, creating an abnormally long toxic RNA, which accumulates in the cell and prevents the production of proteins needed for normal cellular function. The severity and age of onset of DM1 correlates with the number of triplet repeats, which increases from one generation to the next. There are no disease-modifying therapies for patients with DM1 and current treatments are intended to manage symptoms and minimize disability. ISIS-DMPK$_{Rx}$ is designed to correct the underlying genetic defect that causes DM1.

"Myotonic dystrophy represents an ideal opportunity for antisense as the disease-causing gene produces a toxic RNA that is not easily targeted with traditional therapeutic approaches," said C. Frank Bennett, Ph.D., senior vice president of research at Isis. "In our preclinical studies, we and our collaborators, Drs. Charles Thornton and Thurman Wheeler at the University of Rochester, have been able to target the toxic RNA with antisense, remove the toxic RNA and restore normal cell function. We look forward to working with Biogen Idec to move this program into human clinical trials."

In June 2012, Isis entered into an alliance with Biogen Idec to discover and develop an antisense drug targeting DMPK for the treatment of DM1. Under the terms of the agreement, Isis received an upfront payment of $12 million and is eligible to receive up to $59 million in milestone payments associated with the clinical development of ISIS-DMPK$_{Rx}$, including this $10 million milestone payment. Biogen Idec has the option to license ISIS-DMPK$_{Rx}$ from Isis up through completion of the Phase 2 study. Isis could receive up to another $200 million in a license fee and regulatory milestone payments plus double-digit royalties on sales of ISIS-DMPK$_{Rx}$. Isis is responsible for global development of ISIS-DMPK$_{Rx}$ through completion of Phase 2 clinical studies, with Biogen Idec providing advice on the clinical study design and regulatory strategy. If Biogen Idec exercises its option, it will assume global development, regulatory and commercialization responsibilities.

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 30 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 for the treatment of patients with HoFH. 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' alliance with Biogen Idec and the discovery, development, activity, therapeutic potential, safety and commercialization of ISIS-DMPK$_{Rx}$ for the treatment of DM1. 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, 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.

SOURCE Isis Pharmaceuticals, Inc.

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