Isis Pharmaceuticals Earns $2.8M for Advancing ISIS-DMPK-2.5 Rx in Patients with Myotonic Dystrophy Type I

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CARLSBAD, Calif., Nov. 10, 2015 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a $2.8 million milestone payment from Biogen related to the advancement of the ongoing Phase 1/2a study of ISIS-DMPK-2.5$_{Rx}$ in patients with myotonic dystrophy type I (DM1).

"Our collaboration with Biogen has been very productive. ISIS-DMPK-2.5$_{Rx}$ has rapidly advanced into patients with DM1, and we continue to make progress across the board in our drug discovery programs with Biogen. Progress like this advances our severe and rare disease pipeline for neurological disorders, which may, as our programs advance, translate into the potential for significant revenue. Together with Biogen, we have advanced four drugs into the pipeline and have other targets in late-stage research. To date and including the $2.8 million earned today, we have generated nearly $27 million from Biogen related to the advancement of ISIS-DMPK-2.5$_{Rx}$," said B. Lynne Parshall, chief operating officer at Isis Pharmaceuticals.

ISIS-DMPK-2.5$_{Rx}$ is designed to reduce the production of the toxic dystrophia myotonica-protein kinase (DMPK) RNA in cells, including muscle cells, for the potential treatment of DM1. ISIS-DMPK-2.5$_{Rx}$ is being evaluated in a randomized, placebo-controlled, dose-escalation Phase 1/2a study in patients with DM1. For further study information, please visit www.clinicaltrials.gov and search for ISIS-DMPK$_{Rx}$ or by the study number: NCT02312011.

DM1 is a debilitating rare genetic neuromuscular disease characterized by progressive muscle atrophy painful muscle spasms, cataracts, heart conduction defects, endocrine abnormalities and myotonia. DM1, the most common form of muscular dystrophy in adults, affects approximately 150,000 patients in the United States, Europe and Japan. There are currently no disease-modifying therapies. ISIS-DMPK-2.5$_{Rx}$ is the only program in clinical testing that is designed to address the underlying genetic defect that causes DM1. ISIS-DMPK-2.5$_{Rx}$ is a product of Isis' antisense technology, a unique drug discovery and development platform that can directly address the genetic cause of diseases, like DM1, that have been largely untreatable with current therapeutic approaches.

Patients with DM1 have a genetic defect in their DMPK gene in which a sequence of three nucleotides repeats extensively, creating an abnormally long toxic RNA that accumulates in the nucleus of cells and prevents the production of proteins needed for normal cellular function. The number of triplet repeats can increase from one generation to the next, with the devastating consequences of more severe disease in each subsequent generation.

ABOUT ISIS and BIOGEN

Isis and Biogen have a broad strategic alliance focused on leveraging antisense technology to advance the treatment of neurological and neuromuscular disorders. This alliance combines Isis' expertise in antisense technology to evaluate potential neurological targets and discover antisense drugs with Biogen's capability to develop therapies for neurological disorders. Isis is primarily responsible for drug discovery and early development of antisense therapies. Biogen has the option to license each antisense program at a particular stage in development. Current development-stage programs include antisense drugs to treat patients with spinal muscular atrophy (SMA), nusinersen, myotonic dystrophy type 1 (DM1), ISIS-DMPK-2.5$_{Rx}$, and two undisclosed neurodegenerative diseases, ISIS-BIIB3$_{Rx}$, and ISIS-BIIB4$_{Rx}$. In addition to these four drugs, Isis and Biogen have numerous opportunities to evaluate additional targets for the development of drugs to treat neurological disorders.

ABOUT ISIS PHARMACEUTICALS, INC.

Isis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Isis has created a large pipeline of first-in-class or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Isis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; ISIS-TTR$_{Rx}$, a drug Isis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Isis is developing with Biogen to treat infants and children with spinal muscular atrophy. 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, the discovery, development, activity, therapeutic and commercial potential and safety of ISIS-DMPK-2.5$_{Rx}$ for the treatment of myotonic dystrophy type I. 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, 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.

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