Isis Pharmaceuticals Earns \$10 Million Milestone Payment From Biogen Idec for Advancing New Drug Into Development to Treat a Neurodegenerative Disease

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--Fourth Drug to Enter Development Under Broad Strategic Collaboration

CARLSBAD, Calif., Feb. 5, 2015 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today that it has earned a \$10 million milestone payment from Biogen Idec for initiating the investigational new drug (IND)-supporting studies of ISIS-BIIB4_{Rx}. ISIS-BIIB4_{Rx} is a recently identified development candidate designed to inhibit an undisclosed target to treat a neurodegenerative disease. ISIS-BIIB4_{Rx} is the fourth drug to enter development as part of Isis' broad collaboration with Biogen Idec and is a distinct target and disease indication from ISIS-BIIB3_{Rx}, which entered development in late 2014.



"Our collaboration with Biogen Idec is one of the most productive collaborations we have. We have significantly expanded the application of our technology in neurology, and we now have four drugs in development as part of our broad Biogen Idec collaboration. As each of these programs advances, not only will we continue to earn additional milestone payments, but we will also continue to benefit from Biogen Idec's active participation in these programs, lending their resources and expertise," said C. Frank Bennett, Ph.D., senior vice president of research at Isis Pharmaceuticals.

As ISIS-BIIB4_{Rx} advances in development, Isis is eligible to receive up to \$249 million in additional milestone payments and a license fee, including amounts related to the costs of clinical trials. In addition, Isis is eligible to receive double-digit royalties from sales of ISIS-BIIB4_{Rx}.

ABOUT ISIS and BIOGEN IDEC

Biogen Idec and Isis 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 Idec's capability to develop therapies for neurological disorders. Isis is primarily responsible for drug discovery and early development of antisense therapies. Biogen Idec 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), ISIS-SMN_{Rx}, myotonic dystrophy type 1 (DM1), ISIS-DMPK_{Rx}, and two undisclosed neurodegenerative diseases, ISIS-BIIB3_{Rx}, and ISIS-BIIB4_{Rx}. In addition to these four drugs, Isis and Biogen Idec have numerous opportunities to evaluate additional targets for the development of drugs to treat neurological disorders.

ABOUT ISIS PHARMACEUTICALS, INC.

Isis is exploiting its leadership position in RNA-targeted technology to discover and develop novel drugs for its product pipeline and for its partners. Isis' broad pipeline consists of 34 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/rare diseases and cardiovascular diseases. These include ISIS-APOCIII_{Rx}, a drug Isis is developing through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with severely high triglycerides, such as patients with familial chylomicronemia syndrome; ISIS-TTR_{Rx}, a drug Isis is developing with GSK to treat patients with the polyneuropathy form of TTR amyloidosis; and, ISIS-SMN_{Rx}, a drug 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 <u>www.isispharm.com</u>.

ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Isis' strategic relationship with Biogen Idec and the discovery, development, activity, therapeutic potential, safety and commercialization of ISIS-BIIB4_{Rx} for the treatment of an undisclosed neurodegenerative disease. 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. Akcea Therapeutics^m is a trademark of sis Pharmaceuticals, Inc. KYNAMRO[®] is a registered trademark of Genzyme Corporation.

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