

Isis Pharmaceuticals Earns \$10 Million Milestone Payment From Biogen

April 30, 2015

CARLSBAD, Calif., April 30, 2015 /PRNewswire/ -- Isis Pharmaceuticals, Inc. (NASDAQ: ISIS) announced today it has earned a \$10 million milestone payment from Biogen associated with the validation of an undisclosed target to treat a neurological disorder. This is the ninth program to advance, which includes four drugs in development, under Isis' and Biogen's broad strategic alliance to discover and develop novel therapies to treat neurological and neuromuscular disorders.



"Together with Biogen, we have made significant progress advancing multiple programs. In our pipeline, we have four drugs in development with Biogen, including ISIS-SMN_{RX}, ISIS-DMPK-2.5_{RX} and two drugs to undisclosed targets. In addition, we have a number of earlier stage programs, like this one, that we will be advancing this year," said B. Lynne Parshall, chief operating officer at Isis Pharmaceuticals. "To date, we have generated \$329 million in payments from our broad collaboration with Biogen."

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), 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 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 38 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 and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and partial lipodystrophy; ISIS-TTR_{RX}, a drug Isis is developing with GSK to treat patients with the polyneuropathy and cardiomyopathy forms of TTR amyloidosis; and ISIS-SMN_{RX}, a drug Isis is developing with Biogen 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 www.isispharm.com.

ISIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Isis' strategic relationship with Biogen and the discovery, development, activity, therapeutic potential, safety and commercialization of drugs under Isis' relationship with Biogen. 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, which is on file with the SEC. Copies of this 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[™] is a trademark of Isis Pharmaceuticals, Inc. KYNAMRO[®] is a registered trademark of Genzyme Corporation.

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