

Ionis Earns \$10 Million Milestone Payment from Biogen for Advancing a New Program in its Neurology Collaboration

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CARLSBAD, Calif., July 17, 2017 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that it has earned a \$10 million milestone payment from Biogen associated with the validation of an undisclosed neurological disease target. Ionis and Biogen have a broad strategic collaboration to develop drugs to treat patients with neurological disorders. Ionis will continue to evaluate this newest target with the goal of advancing this program into development.



"We continue to make substantial progress in validating new targets and developing new drugs for neurological diseases using our antisense technology. SPINRAZA, the first drug to come out of our neurology collaboration with Biogen, exemplifies the potential of our antisense technology in the neurological disease space. To date, our collaboration with Biogen has generated a combined total of nearly \$650 million," said B. Lynne Parshall, chief operating officer at Ionis Pharmaceuticals. "As we continue to advance our neurological disease pipeline, we look forward to bringing additional important medicines to patients suffering from neurodegenerative diseases."

ABOUT IONIS' STRATEGIC COLLABORATION WITH BIOGEN

Ionis and Biogen have a broad strategic collaboration combining Biogen's expertise in neurology with Ionis' leadership in antisense technology to develop novel therapies to treat neurological disorders. Ionis 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. Under this collaboration, Ionis developed and licensed SPINRAZA to Biogen. SPINRAZA is approved in the U.S., EU, Japan and Canada for the treatment of patients with spinal muscular atrophy (SMA). Also under this collaboration, Ionis and Biogen are developing IONIS-SOD₁R_X to treat amyotrophic lateral sclerosis, IONIS-MAPT_{RX} to treat Alzheimer's Disease, and IONIS-BIIB₅R_X, IONIS-BIIB₆R_X and IONIS-BIIB₇R_X to treat undisclosed neurodegenerative diseases. In addition to these six drugs, Ionis and Biogen have numerous opportunities to evaluate additional targets for the development of drugs to treat neurological disorders.

ABOUT IONIS PHARMACEUTICALS, INC.

Ionis 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, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over three dozen drugs in development. SPINRAZA[®] (nusinersen) is a drug that has been approved in the U.S., Europe, Japan and Canada for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. Drugs that have successfully completed Phase 3 studies include volanesorsen, a drug Ionis is developing and plans to commercialize through its subsidiary, Akcea Therapeutics, to treat patients with either familial chylomicronemia syndrome or familial partial lipodystrophy; and inotersen (IONIS-TTR_{RX}), a drug Ionis is developing with GSK to treat patients with TTR amyloidosis. Both drugs are progressing toward regulatory filings in the second half of 2017. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' relationship with Biogen and the discovery, development, activity, therapeutic potential, safety and commercialization of drugs under Ionis' relationship with Biogen. Any statement describing Ionis' 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. Ionis' 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 Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended December 31, 2016, 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, "Ionis," "Company," "we," "our," and "us" refers to Ionis Pharmaceuticals and its subsidiaries.

Ionis Pharmaceuticals[™] is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics[™] is a trademark of Ionis Pharmaceuticals, Inc. SPINRAZA[®] is a registered trademark of Biogen.

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