

## **Ionis Pharmaceuticals Earns \$5 Million Milestone Payment from Biogen for Advancing a New Program Under Its Broad Neurology Strategic Collaboration**

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CARLSBAD, Calif., Dec. 22, 2015 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today it has earned a \$5 million milestone payment from Biogen associated with the validation of an undisclosed target to treat patients with an undisclosed neurological disorder. Ionis and Biogen have a broad strategic collaboration to develop drugs to treat patients with neurological disorders. For this newest target, Ionis will continue to evaluate the target with the goal of advancing this program into development.

"Our strategic collaboration with Biogen is very successful. Our most advanced program, nusinersen, is currently in Phase 3 clinical studies, and we have a number of other drugs in development. We benefit tremendously from the expertise Biogen brings to our partnered programs. We also benefit financially as these programs advance. Across our four collaborations, we have generated more than \$350 million from Biogen as our programs have advanced," said B. Lynne Parshall, chief operating officer at Ionis. "As we enter 2016, we expect to continue to expand our collaboration with Biogen as we identify new targets and move new antisense drugs into development."

### **ABOUT IONIS and BIOGEN**

Ionis and Biogen have a broad strategic alliance focused on leveraging antisense technology to advance the treatment of neurological and neuromuscular disorders. This alliance combines Ionis' expertise in antisense technology to evaluate potential neurological targets and discover antisense drugs with Biogen's capability to develop therapies for 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. Current development-stage programs include antisense drugs to treat patients with spinal muscular atrophy (SMA), nusinersen; myotonic dystrophy type 1 (DM1), IONIS-DMPK-2.5Rx; amyotrophic lateral sclerosis (ALS), IONIS-SOD1Rx; and an undisclosed neurodegenerative disease, IONIS-BIIB4Rx. In addition to these four 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 Pharmaceuticals 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 a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTRRx, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at [www.ionispharma.com](http://www.ionispharma.com).

### **IONIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT**

This press release includes forward-looking statements regarding Ionis' strategic 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, 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 December, 2015, the Company changed its name from Isis Pharmaceuticals, Inc. to Ionis Pharmaceuticals, Inc.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our," and "us" refers to Ionis Pharmaceuticals and its subsidiaries.

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