



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

**CARLSBAD, Calif., Feb. 2, 2017** – Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today it has earned a \$5 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. For this newest target, Ionis will continue to evaluate the target with the goal of advancing this program into development.

“The recent FDA approval of SPINRAZA highlights the potential of our antisense technology to address neurological diseases with high unmet need,” said B. Lynne Parshall, chief operating officer at Ionis. “This latest milestone is one more example of the success of our strategic collaboration with Biogen. Under this collaboration, we have advanced SPINRAZA to commercialization and another four drugs into development. We have also validated four more targets with drug candidates that we are advancing toward development. This work has generated a combined total of approximately \$555 million to date. As we enter 2017, we expect to continue to make significant progress in growing our neurological disease pipeline.”

### **ABOUT IONIS’ STRATEGIC COLLABORATION WITH 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. SPINRAZA™ (nusinersen), approved in the U.S. to treat pediatric and adult patients with spinal muscular atrophy, was the first antisense drug developed with Biogen. Current development-stage programs include antisense drugs to treat patients with amyotrophic lateral sclerosis (ALS), IONIS-SOD1<sub>Rx</sub>, and three additional drugs in development to treat undisclosed neurodegenerative diseases: IONIS-BIIB4<sub>Rx</sub>, IONIS-BIIB5<sub>Rx</sub> and IONIS-BIIB6<sub>Rx</sub>. In addition to these five 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. for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients. Biogen is responsible for commercialization of SPINRAZA. 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 either familial chylomicronemia syndrome or familial partial lipodystrophy; and IONIS-TTR<sub>Rx</sub>, a drug Ionis is developing with GSK to treat patients with TTR amyloidosis. 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, 2015, 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 trademark of Biogen.

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