Ionis Pharmaceuticals Licenses Second Orally Delivered Antisense Drug to Janssen

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- IONIS-JBI2-2.5 Rx designed to treat GI autoimmune disease
- Ionis Earns $5 Million License Fee from Janssen

CARLSBAD, Calif., Nov. 16, 2017 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that it has licensed a second orally delivered Generation 2.5 antisense drug to Janssen Biotech, Inc. (Janssen) for which Ionis earned $5 million. IONIS-JBI2-2.5$_{Rx}$ is designed to locally reduce the production of an undisclosed target in the gastrointestinal (GI) tract for the treatment of a GI autoimmune disease. Janssen will now assume all global development, regulatory, and commercialization responsibilities for IONIS-JBI2-2.5$_{Rx}$ for GI diseases.

"We are pleased that IONIS-JBI2-2.5$_{Rx}$ is advancing into development because we believe this drug has the potential to treat autoimmune disorders in the GI tract that are underserved with current therapies," said Frank Bennett, Ph.D., senior vice president of research at Ionis Pharmaceuticals. "Our collaboration with Janssen has been very productive. This is the second drug Janssen has licensed in little over a year, which is the direct result of the speed and efficiency of our antisense technology."

Under its global collaboration agreement with Janssen, Ionis is eligible to receive nearly $800 million in development, regulatory and sales milestone payments and license fees. In addition, Ionis will receive tiered royalties that on average are double-digits on sales from any product that is successfully commercialized. IONIS-JBI1-2.5$_{Rx}$, the first collaboration target, was licensed to Janssen last year and Janssen has assumed all global development, regulatory and commercialization responsibilities related to this drug.

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) has been approved in global markets for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. Drugs that have successfully completed Phase 3 studies include inotersen, an antisense drug Ionis is developing to treat patients with hereditary TTR amyloidosis (hATTR), and volanesorsen, an antisense drug discovered by Ionis and co-developed by Ionis and Akcea Therapeutics to treat patients with either familial chylomicronemia syndrome or familial partial lipodystrophy. Akcea, an affiliate of Ionis, is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders. If approved, volanesorsen will be commercialized through Ionis' affiliate, Akcea. Inotersen filings for marketing approval have been submitted in the U.S. and EU. Volanesorsen filings for marketing approval have been submitted in the U.S., EU, and Canada. 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 the therapeutic and commercial potential regarding Ionis' alliance with Janssen, Ionis research, development and commercial opportunities in developing antisense drugs to treat autoimmune disease in the gastrointestinal tract. 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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