

Ionis Pharmaceuticals Earns Milestone Payment from Janssen for Advancing a New Program Under GI Collaboration

December 22, 2016

- Ionis Earns \$5 Million Milestone

CARLSBAD, Calif., Dec. 22, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that it has earned \$5 million milestone payment from Janssen Biotech, Inc. (Janssen) associated with the validation of an undisclosed target to treat patients with a gastrointestinal (GI) autoimmune disease. Under the collaboration, Ionis and Janssen will continue to evaluate the target with the goal of advancing an antisense drug into development.



"In just two short years, our collaboration with Janssen has proven to be very successful. Earlier this year, we earned \$10 million when Janssen licensed IONIS-JB11-2.5_{Rx}, an orally delivered antisense drug for the treatment of a GI autoimmune disease. The program announced today marks the second to advance under our collaboration with Janssen," said B. Lynne Parshall, chief operating officer at Ionis. "We are excited by the rapid advancement of both programs and look forward to the continued progress of this collaboration."

Under the terms of the agreement, which covers three programs, 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.

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 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 either familial chylomicronemia syndrome or familial partial lipodystrophy; IONIS-TTR_{Rx}, a drug Ionis is developing with GSK to treat patients with TTR amyloidosis; and SPINRAZATM (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.

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' alliance with Janssen, and Ionis' research, development and commercial opportunities in developing antisense drugs to treat autoimmune diseases 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, 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 PharmaceuticalsTM is a trademark of Ionis Pharmaceuticals, Inc. Akcea TherapeuticsTM is a trademark of Ionis Pharmaceuticals, Inc. SPINRAZATM is a trademark of Biogen.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/ionis-pharmaceuticals-earns-milestone-payment-from-janssen-for-advancing-a-new-program-under-gi-collaboration-300382789.html>

SOURCE Ionis Pharmaceuticals, Inc.

Ionis Pharmaceuticals Investor and Media Contact: D. Wade Walke, Ph.D., Vice President, Corporate Communications and Investor Relations, 760-603-2741