

# Ionis Pharmaceuticals Licenses First Oral Antisense Drug Acting Locally in the GI Tract to Janssen

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CARLSBAD, Calif., July 19, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that it has earned \$10 million from Janssen Biotech, Inc. (Janssen) upon licensing IONIS-JBI1-2.5<sub>Rx</sub>, an orally delivered antisense drug designed to locally inhibit an undisclosed target in the gastrointestinal (GI) tract for the treatment of a GI autoimmune disease. Janssen will assume all global development, regulatory and commercialization responsibilities for IONIS-JBI1-2.5<sub>Rx</sub> once Ionis completes the IND-enabling studies.



"IONIS-JBI1-2.5<sub>Rx</sub> is the first antisense drug to enter development as part of Ionis' collaboration with Janssen to discover and develop orally administered, locally acting RNA-targeted therapeutics for autoimmune diseases in the gastrointestinal tract. We are very pleased to have rapidly advanced this first drug candidate into development within a year and a half of initiating our collaboration with Janssen," said B. Lynne Parshall, chief operating officer at Ionis Pharmaceuticals. "This new drug further expands our technology's reach into a new therapeutic setting and a new route of delivery."

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 for these programs. 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<sub>Rx</sub>, 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).

## **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 Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics™ is a trademark of Ionis Pharmaceuticals, Inc.

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