

Ionis Licenses New Antisense Drug for Kidney Disease to AstraZeneca

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Ionis earns \$30 million license fee

CARLSBAD, Calif., Feb. 20, 2018 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that it has licensed IONIS-AZ5-2.5_{Rx} (AZD2373) to the global, science-led biopharmaceutical company AstraZeneca (NYSE: AZN). IONIS-AZ5-2.5_{Rx} is a Generation 2.5 antisense drug, discovered by Ionis and designed to inhibit an undisclosed target to treat a genetically associated form of kidney disease. AstraZeneca will be responsible for developing and commercializing IONIS-AZ5-2.5_{Rx}.



"This is the second drug to enter development under our strategic collaboration with AstraZeneca in cardiovascular, metabolic and renal diseases. IONIS-AZ5-2.5_{Rx} is being developed to treat a genetically associated form of kidney disease with a high unmet medical need. We were able to move this program from target validation to a clinical candidate quickly, exemplifying the efficiency of our antisense platform," said Brett P. Monia, Ph.D., chief operating officer and senior vice president of antisense drug discovery and translational medicine at Ionis. "AstraZeneca is a great partner to work with in the cardiometabolic and renal therapeutic area, and we look forward to them moving this program through development."

Ionis will receive a \$30 million upfront payment from AstraZeneca for IONIS-AZ5-2.5_{Rx} and may also receive up to \$300 million in additional development and regulatory milestone payments and tiered, low double-digit royalties from sales of this potential new medicine.

ABOUT IONIS AND ASTRAZENECA

AstraZeneca and Ionis have a strategic alliance focused on leveraging antisense technology to discover and develop antisense therapies for cardiovascular, metabolic and renal diseases. The collaboration builds on a broad existing relationship between the two companies and is supported by Ionis' expertise in cardiovascular disease and antisense technology and AstraZeneca's disease area knowledge as well as drug development expertise and infrastructure. Ionis has generated more than \$220 million to date from the broad strategic collaboration with AstraZeneca in oncology and cardiometabolic and kidney diseases. Beyond AZD8233, and AZD2373, AstraZeneca has the option to license additional antisense drugs upon development candidate nomination and will pay a license fee for each drug licensed plus development and regulatory milestones and sales royalties for each program that AstraZeneca advances. Ionis and AstraZeneca are also collaborating to discover and develop antisense drugs to treat cancer.

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 PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' alliance with AstraZeneca and the therapeutic and commercial potential of AZD2373. 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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Ionis Pharmaceuticals Investor and Media Contact: D. Wade Walke, Ph.D., Vice President, Corporate Communications and Investor Relations, 760-603-2741