

Ionis Pharmaceuticals and MD Anderson Form Strategic Alliance to Advance Novel Cancer Therapeutics

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CARLSBAD, Calif. and HOUSTON, May 23, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) and The University of Texas MD Anderson Cancer Center today announced the formation of a strategic alliance to discover and develop novel cancer therapeutics. Combining advances made with Ionis' antisense technology, which provides enhanced potency for effectively engaging targets in solid and liquid tumors, with MD Anderson's expertise will allow Ionis and MD Anderson to develop novel therapeutic strategies to treat patients with a variety of cancers.



"MD Anderson is dedicated to accelerating the end of cancer. As part of our [Moon Shots Program](#), we identified many new, traditionally undruggable cancer targets, which could offer a potentially fresh approach to treating cancer," said [Gordon Mills, M.D., Ph.D.](#), chair, [systems biology](#) at MD Anderson. "This collaboration allows our experts to take advantage of a novel technology capable of targeting undruggable targets that we believe will add to our ability to develop therapeutic strategies that will transform patient outcomes."

Mills is co-leader of the [Breast and Ovarian Cancer Moon Shot](#), one of 12 in MD Anderson's effort to reduce cancer deaths by accelerating conversion of scientific discoveries into new therapies, prevention approaches and early detection.

"MD Anderson has undertaken groundbreaking work in identifying novel cancer targets and building pre-clinical cancer models that will streamline our collective drug discovery efforts. Their genetically defined patient populations should greatly shorten the overall development timelines and enhance our drug discovery efforts as we move our programs forward," said Brett Monia, senior vice president, antisense drug discovery at Ionis Pharmaceuticals. "Our Generation 2.5 chemistry coupled with the expertise of a world leading cancer center, like MD Anderson, will drive our oncology drug development program and pipeline."

In the collaboration, Ionis and MD Anderson will work together to validate novel undruggable cancer targets selected based on human genomic data. Ionis will lead the drug discovery efforts against mutually agreed upon novel targets and MD Anderson will lead development activities through clinical proof of concept. Following clinical proof of concept, Ionis and MD Anderson plan to identify a partner to complete development and to commercialize each drug with Ionis leading business development efforts.

"We are excited to have MD Anderson as a strategic partner for our cancer franchise. This alliance allows Ionis and MD Anderson to combine their collective expertise and resources to identify and ultimately develop novel anti-cancer drugs," said Sarah Boyce, chief business officer at Ionis Pharmaceuticals. "This alliance enables Ionis and MD Anderson to focus on their respective areas of expertise, thereby facilitating rapid progress in moving each drug through the clinic. We believe this collaboration has the opportunity to create novel therapeutic agents that can provide great benefit for cancer patients."

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 and/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, if approved, plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTR_{Rx}, 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.

ABOUT MD ANDERSON

The University of Texas MD Anderson Cancer Center in Houston ranks as one of the world's most respected centers focused on cancer patient care, research, education and prevention. The institution's sole mission is to end cancer for patients and their families around the world. MD Anderson is one of only 44 comprehensive cancer centers designated by the National Cancer Institute (NCI). MD Anderson is ranked No. 1 for cancer care in the U.S. News & World Report's "Best Hospital's" survey. It has ranked as one of the nation's top two hospitals since the survey began in 1990, and has ranked first 11 of the past 14 years. MD Anderson receives a cancer center support grant from the NCI of the National Institutes of Health (P30 CA016672).

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis Pharmaceuticals' business, and its agreement with MD Anderson. 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 at www.ionispharma.com.

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