Ionis Licenses Novel Antisense Drug for the Treatment of Centronuclear Myopathy to Dynacure

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Ionis earns $5 million license fee from Dynacure

CARLSBAD, Calif. and STRASBOURG, France, Nov. 9, 2017 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) and Dynacure announced today that Dynacure has licensed IONIS-DNM2-2.5$_{Rx}$ (Dyn101), a Generation 2.5 antisense drug targeting dynamin 2 for the treatment of centronuclear myopathy (CNM), for which Ionis earned a $5 million license fee in the form of Dynacure equity. Dynacure will now assume all development and commercialization responsibilities for the program.

"CNM is a rare, debilitating disease affecting children and young adults and results in progressive muscular weakness. We are pleased that Dynacure has opted to license IONIS-DNM2-2.5$_{Rx}$, which we believe has the potential to bring benefit to patients with CNM," said Brett P. Monia, senior vice president of drug discovery and franchise leader for oncology and rare diseases at Ionis Pharmaceuticals.

"We are delighted with the productivity and efficiency of our collaboration with Ionis. The identification of a development candidate is an important step for our alliance with Ionis and the Dynacure team. Importantly, this development puts us one step closer to potentially finding a new treatment for patients suffering with CNM, a devastating disease with no adequate treatments," said Stephane van Rooijen, chief executive officer of Dynacure.

Under its collaboration agreement with Dynacure, Ionis is eligible to receive additional cash or equity of up to more than $205 million in milestone payments. In addition, Ionis is eligible to receive royalties on future product sales of the drug under this collaboration.

ABOUT CENTRONUCLEAR MYOPATHY (CNM)
Centronuclear myopathies (CNMs) is an umbrella term for a group of rare genetic muscle disorders affecting children and young adults. These disorders are characterized by muscle weakness that can range from mild to profound. CNM, caused by mutations in the *DNM2* gene, is highly variable in presentation and severity, presenting at birth, during childhood or in adulthood. When *DNM2*-related CNM occurs during infancy or early childhood, common symptoms include hypotonia, generalized weakness, facial muscle weakness, ptosis, and ophthalmoplegia. Affected children may exhibit delays in attaining motor milestones, such as holding their head up. Facial weakness can cause infants to have a weak sucking ability and/or experience difficulties swallowing, potentially resulting in feeding difficulties. Eventually, affected individuals can develop breathing (respiratory) complications.

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.

ABOUT DYNACURE
Dynacure develops new treatments for patients suffering from serious orphan disorders. The first drug discovery program of Dynacure focuses on Centronuclear Myopathies (CNM), a rare debilitating disease affecting children and young adults. The Dyn101 development program is based on the modulation of the Dynamin 2 protein expression through the use of an antisense oligonucleotide developed in collaboration with Ionis Pharmaceuticals, the leading biopharmaceutical company in RNA-targeted drug discovery.

Dynacure was founded in 2016 as a spin-off from the IGBMC (Institute of Genetic and Molecular and Cellular Biology – Unistra/INSERM/CNRS) of Strasbourg, by SATT Conectus, Kurma Partners, IdInvest and Ionis Pharmaceuticals. Additional information about Dynacure is available at www.dynacure.fr.

IONIS' FORWARD-LOOKING STATEMENT
This press release includes forward-looking statements regarding the therapeutic and commercial potential regarding Ionis' alliance with Dynacure, Ionis research, development and commercial opportunities in developing antisense drugs to treat neuromuscular diseases. 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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