Ionis licenses investigational Alzheimer's therapy

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- IONIS-MAPT Rx is designed to selectively reduce production of the protein tau in the central nervous system

- Ionis earns $45 million license fee

CARLSBAD, Calif., Dec. 19, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapeutics, announced today that Biogen, a collaboration partner for neurological diseases, has licensed IONIS-MAPT$_{Rx}$, an antisense therapy designed to selectively reduce production of microtubule-associated protein tau (MAPT), or tau, in the central nervous system. MAPT is believed to contribute to or cause several neurodegenerative diseases, including Alzheimer's disease (AD) and some forms of frontotemporal degeneration (FTD).

"The licensing of IONIS-MAPT$_{Rx}$, currently in a Phase 1 clinical study in mild AD patients, is an important milestone for the program. It brings us another step closer to potentially delivering a therapy to patients who have few or no effective treatment options," said Brett P. Monia, Ph.D., Ionis' chief operating officer.

Tau pathology is a hallmark of AD and other tauopathies and is widely hypothesized to contribute to clinical decline in these neurodegenerative diseases. In preclinical studies, MAPT-targeted antisense treatment demonstrated prevention and reversal of pathology.

Ionis earned a $45 million license fee from Biogen and is eligible to earn up to $155 million in additional milestone payments. Ionis is also eligible to receive royalties on sales of the medicine in the low- to mid-teens. Under terms of the collaboration, Ionis will be responsible for the Phase 1 clinical study of IONIS-MAPT$_{Rx}$ in patients with mild AD that was initiated in 2017 and a one-year long-term extension study that began this year. Biogen will have responsibility for all subsequent studies and any further development, including regulatory filings, and commercialization.

ABOUT THE IONIS AND BIOGEN COLLABORATION

Ionis and Biogen have a broad collaboration combining Biogen's expertise in neurology with Ionis' leadership in antisense technology to develop novel therapies to treat neurological disorders. SPINRAZA, the first commercial drug from this collaboration, is currently a global foundation of care for treatment of patients with spinal muscular atrophy (SMA). Ionis and Biogen are also developing tofersen (BIIB067), which Biogen licensed in 2018, and IONIS-C9Rx (BIIB078), both for amyotrophic lateral sclerosis (ALS), and ION859 (BIIB094) for Parkinson's disease.

ABOUT IONIS PHARMACEUTICALS, INC.

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, drug discovery platform called antisense technology that can treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs. We created the first and only approved treatment for both children and adults with spinal muscular atrophy as well as the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy in adults with hereditary transthyretin amyloidosis. Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 novel medicines designed to treat a broad range of disease, including cardiovascular, neurological, infectious and pulmonary diseases and many more.

To learn more about Ionis visit www.ionispharma.com and follow us on twitter @ionispharma.

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' alliance with Biogen and the development, activity, therapeutic potential, commercial potential and safety of Ionis' antisense medicine for people with Alzheimer's disease, IONIS-MAPT$_{Rx}$. 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, 2018, and its most recent Form 10-Q quarterly filing, 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.

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