Ionis and Akcea win prestigious Prix Galien Award for TEGSEDI® (inotersen) as Best Biotechnology Product

October 30, 2020

BOSTON and CARLSBAD, Calif., Oct. 30, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) and its wholly owned subsidiary, Akcea Therapeutics, received the Prix Galien USA Award for the Best Biotechnology Product in 2020 in recognition of TEGSEDI, the first and only self-administered, subcutaneous treatment for the polyneuropathy of hereditary ATTR amyloidosis in adults. This Prix Galien award recognizes outstanding achievements in biomedicine that improve the human condition and was presented at a virtual ceremony on Thursday, Oct. 29.

"We are thrilled to accept this award for TEGSEDI – a treatment that has already provided substantial benefit for those who have been devastated with the polyneuropathy associated with hereditary transthyretin amyloidosis," said Brett P. Monia, Ph.D., chief executive officer at Ionis, and a founding scientist at Ionis who led the discovery and development of TEGSEDI. "Our vision in the discovery and development of TEGSEDI was to improve the lives of patients living with the devastating effects of this rare genetic disease that impacts the lives of generations of families. I would like to thank our teams at Akcea and Ionis for their tireless work and dedication in developing TEGSEDI and giving hope to patients worldwide."

The international Prix Galien Award is presented annually by the Galien Foundation. The Prix Galien USA Committee comprises renowned leaders from the biomedical industry and academia, who together are responsible for evaluating nominees. To qualify, each candidate must be U.S. Food and Drug Administration-approved for marketing within the last five years and demonstrate significant potential to advance human health worldwide. Since its inception in 1970, the Prix Galien is considered the biopharmaceutical and medical industries' equivalent of the Nobel prize.

ABOUT HEREDITARY TRANSTHYRETIN (hATTR) AMYLOIDOSIS
Hereditary ATTR amyloidosis is a severe, progressive, and life-threatening disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, the heart and intestinal tract. The progressive accumulation of TTR amyloid deposits in these organs often leads to intractable peripheral sensorimotor neuropathy, autonomic neuropathy, and/or cardiomyopathy, as well as other disease manifestations. Hereditary ATTR amyloidosis causes significant morbidity and progressive decline in quality of life, severely impacting activities of daily living. The disease often progresses rapidly and can lead to premature death. The median survival is 4.7 years following diagnosis. Additional information on hereditary ATTR amyloidosis, including a full list of organizations supporting the hATTR amyloidosis community worldwide, is available at www.hattrchangethecourse.com or by visiting www.hATTRGuide.com.

ABOUT *TEGSEDI® (INOTERSEN)*
TEGSEDI was approved by the U.S. Food and Drug Administration (FDA) for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. TEGSEDI, discovered and developed by Ionis Pharmaceuticals, is the world's first and only subcutaneous RNA-targeting drug designed to reduce the production of human transthyretin (TTR) protein. TEGSEDI also received marketing authorization in the European Union and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis.

The approval is based on data from the NEURO-TTR study that was a Phase 3 randomized (2:1), double-blind, placebo-controlled, 15-month, international study in 172 patients with hATTR amyloidosis with symptoms of polyneuropathy. In NEURO-TTR, TEGSEDI demonstrated significant improvement compared to placebo in measures of neuropathy and quality of life as measured by the modified Neuropathy Impairment Score +7 (mNIS+7) and in the Norfolk Quality of Life Questionnaire-Diabetic Neuropathy (Norfolk QOL-DN) total score. Patients treated with TEGSEDI experienced similar benefit regardless of subgroups such as age, sex, race, region, Neuropathy Impairment Score (NIS), Val30Met mutation status, and disease stage.

The approval is also based on data from the NEURO-TTR Open Label Extension (OLE) that is an ongoing study for patients who completed the NEURO-TTR study, designed to evaluate the long-term efficacy and safety of TEGSEDI.

For TEGSEDI's full prescribing information, please visit www.TEGSEDI.com.

About Akcea Therapeutics, Inc.
Akcea Therapeutics, Inc., is a wholly owned subsidiary of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA therapeutics. Akcea commercializes TEGSEDI® (inotersen) and WAYLIVRA® (volanesorsen), and with Ionis is advancing a mature pipeline of novel medicines discovered by Ionis and based on Ionis' proprietary antisense technology. TEGSEDI is approved in the U.S., E.U., Canada and Brazil, and WAYLIVRA is approved in the E.U. For more information about Akcea please visit www.akceatx.com.

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 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 potentially treat a broad range of disease, including neurological, cardio-renal, metabolic, infectious, and pulmonary diseases. To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the therapeutic and commercial potential of TEGSEDI (inotersen) and Ionis' technologies and products in development, including the business of Akcea Therapeutics, Inc., Ionis' wholly owned subsidiary. 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, including those related to the impact COVID-19 could have on our business, and including but not limited to those related to our commercial products and the medicines in our pipeline, and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. 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, 2019, and the 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.

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