Ionis Pharmaceuticals Provides Update on IONIS-TTRRX Program

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CARLSBAD, Calif., April 7, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today provided an update on the IONIS-TTR_{Rx} program. IONIS-TTR_{Rx} is currently being evaluated by IONIS in an ongoing Phase 3 study, NEURO-TTR, in patients with transthyretin (TTR) familial amyloid polyneuropathy. A Phase 3 study, CARDIO-TTR, is also being planned by GSK to evaluate IONIS-TTR_{Rx} in patients with TTR amyloid cardiomyopathy.

GSK is in the process of finalizing the protocol for the CARDIO-TTR study with the U.S. Food and Drug Administration (FDA). In that process, the FDA has placed a clinical hold on the CARDIO-TTR study while GSK provides answers to questions about the protocol stemming from Ionis' ongoing NEURO-TTR study. The NEURO-TTR study, which is regulated by a separate division at the FDA, continues as planned and is on track to complete in the first half of 2017.

"A clinical hold is a tool the FDA often uses while a sponsor answers questions the FDA may have about a clinical study," commented Lynne B. Parshall, chief operating officer at Ionis. "We are working closely with GSK, who have an option to exclusively license the IONIS-TTR_{Rx} program, to support their efforts to quickly answer the FDA questions and initiate the study. We hope GSK will be in a position to advance the CARDIO-TTR study as quickly as possible to potentially bring IONIS-TTR_{Rx} to patients with TTR amyloid cardiomyopathy who are in serious need of treatment options that can address the root cause of their disease."

ABOUT TTR AMYLOIDOSIS

TTR amyloidosis is a severe, progressive and fatal disease with multiple overlapping clinical manifestations. There are three forms of TTR amyloidosis, FAP, FAC and wt-TTR amyloidosis, and all are caused by the inappropriate formation and aggregation of TTR amyloid deposits in various tissues and organs, including peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to organ failure and eventually death. Although TTR amyloidosis is fatal, therapeutic options for the treatment of patients with TTR amyloidosis are very limited and there are currently no disease-modifying drugs available.

FAP is characterized by the accumulation of misfolded mutated TTR protein primarily in the peripheral nerves. Patients with FAP experience ongoing debilitating nerve damage throughout their body resulting in the progressive loss of motor functions, such as walking. These patients also accumulate TTR in other major organs, which progressively compromises their function and eventually leads to death within five to fifteen years of disease onset. There are an estimated 10,000 FAP patients worldwide.

TTR-related cardiomyopathy is characterized by the accumulation of misfolded TTR protein primarily in the cardiac muscle. Patients experience ongoing debilitating heart damage resulting in progressive heart failure, which results in death within 5 to 7 years from disease onset. TTR-related cardiomyopathy includes both the genetic form of the disease, FAC, and the wild-type form of the disease, wt-TTR amyloidosis. There are an estimated 40,000 FAC patients worldwide. Patients with FAC begin to experience symptom onset between 50 and 60 years of age, whereas patients with wt-TTR amyloidosis usually begin to experience symptom onset ten or more years later, generally over 70 years of age. There are an estimated 200,000 wt-TTR amyloidosis patients worldwide.

Often patients with the polyneuropathy form of TTR amyloidosis will also have TTR build up in the heart and also experience cardiomyopathy symptoms. Similarly, patients with the cardiomyopathy form of TTR amyloidosis may often have TTR build up in their peripheral nerves and can experience nerve damage and progressive difficulty with motor functions.

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 a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and 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.

IONIS PHARMACEUTICALS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding the therapeutic and commercial potential of IONIS-TTR_{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, 2015,which is on file with the SEC. Copies of this 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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