Ionis Pharmaceuticals Provides Update on IONIS-TTR Rx Program

May 26, 2016

Conference call scheduled for 9 am Eastern Time today

CARLSBAD, Calif., May 26, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today provided an update on the IONIS-TTR_{Rx} program. Ionis is currently evaluating IONIS-TTR_{Rx} in an ongoing Phase 3 study, NEURO-TTR, in patients with transthyretin (TTR) familial amyloid polyneuropathy. Dr. Merrill Benson is also evaluating IONIS-TTR_{Rx} in an investigator-initiated Phase 2 open-label study in patients with TTR-related amyloid cardiomyopathy.

GSK, which has an option to exclusively license IONIS-TTR_{Rx}, has decided not to initiate a Phase 3 outcome study, CARDIO-TTR, which was planned to evaluate IONIS-TTR_{Rx} in patients with TTR amyloid cardiomyopathy. As announced in April 2016, the U.S. Food and Drug Administration (FDA) had placed this study on clinical hold as a result of safety findings in the ongoing NEURO-TTR study. GSK will consider options for TTR amyloid cardiomyopathy once additional clinical data are available from the ongoing studies.

Both the NEURO-TTR and Dr. Benson's study should provide important data on patients with TTR amyloid cardiomyopathy, which could contribute to the design of an optimal Phase 3 study. In addition to Dr. Benson's investigator-initiated study in patients with TTR-related amyloid cardiomyopathy, the NEURO-TTR study is also evaluating patients with TTR-related cardiomyopathy using a cardiac substudy to evaluate patients who have cardiac involvement in addition to their polyneuropathy. Approximately half of the patients in the NEURO-TTR study also have TTR-related amyloid cardiomyopathy. The NEURO-TTR study and Dr. Benson's study are both proceeding on track, with the data from the NEURO-TTR study planned in the first half of 2017. Updated data from Dr. Benson's study will be presented at the International Symposium on Amyloidosis in July 2016. Together, Ionis and GSK, are actively preparing for an NDA filing.

"We are committed to advancing IONIS-TTR_{Rx} as a potentially first-in-class and best-in-class therapy for all patients suffering from TTR amyloidosis, a severe, progressive and fatal disease. We are encouraged by the strong retention we have observed in the NEURO-TTR study and the robust participation and the substantial reductions in TTR protein we are observing in the open-label extension study. We are also encouraged by the results to date from Dr. Benson's study in a small number of patients with the cardiac form of the disease as presented at the European Congress on Hereditary ATTR Amyloidosis meeting in Paris last year," said Dr. Brett Monia, senior vice president of Ionis antisense drug discovery.

Dr. Helen Merianos, Medicine Development Leader for GSK's TTR amyloidosis program, said: "GSK remains committed to developing innovative medicines for the treatment of amyloidosis and to our ongoing collaboration with Ionis to develop IONIS-TTR_{Rx} for TTR amyloidosis. We will assess the results from the NEURO-TTR study to inform our next steps for TTR amyloid cardiomyopathy."

Conference Call

At 9:00 a.m. Eastern Time today, May 26, 2016, Ionis will conduct a live webcast conference call to provide an update on the IONIS-TTR_{Rx} program. Interested parties may listen to the call by dialing 877-443-5662 or access the webcast at www.ionispharma.com. A webcast replay will be available for a limited time at the same address.

ABOUT TTR AMYLOIDOSIS

TTR amyloidosis is a severe, progressive and fatal disease with multiple overlapping clinical manifestations. There are three forms of TTR amyloidosis: familial amyloid polyneuropathy, FAP, familial amyloid cardiomyopathy, FAC, and wild type (wt)-TTR amyloidosis. The disease is caused by the accumulation of misfolded TTR protein in a broad range of 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. Therapeutic options for the treatment of patients with TTR amyloidosis are very limited.

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 compromise 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 amyloid 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 amyloid 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, 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.

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