

Ionis to host ATTR symposium highlighting CARDIO-TTRansform Study at 3rd European ATTR Amyloidosis meeting

September 3, 2021

- **Sam Tsimikas, M.D., senior vice president of clinical development, to moderate symposium on ATTR-CM**

- **CARDIO-TTRansform, a Phase 3 study of eplontersen for the treatment of ATTR-CM, will be featured on the Patients Association Channel**

- **Multiple presentations highlight new data and research into transthyretin amyloidosis**

CARLSBAD, Calif., Sept. 3, 2021 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapies, announced today it will present 14 posters and two symposiums highlighting new scientific insights from its ATTR amyloidosis portfolio at the 3rd European ATTR Amyloidosis (EU-ATTR) meeting for Patients and Doctors on September 6-8, 2021. The posters and symposiums will include several presentations about Ionis' ATTR medicines, including its investigational antisense medicine, eplontersen, which is currently being evaluated in two phase 3 trials to potentially treat transthyretin amyloid cardiomyopathy (ATTR-CM) and polyneuropathy (ATTR-PN). The presentations underscore Ionis' commitment to the ATTR amyloidosis community and the company's important work toward advancing treatment options for people impacted by the disease.

The following is a schedule of Ionis' events and activities to be held virtually at EU-ATTR:

- **Tuesday, Sept. 7 at 2:00 p.m. – 2:15 p.m. CEST**

Presentation: Gustavo Buchele, M.D., Ph.D., executive director, clinical development at Ionis, will present study design and trial details for [CARDIO-TTRansform](#), which is currently enrolling, on the Patients Association Channel.

- **Wednesday, Sept. 8 at 2:00 p.m. – 2:45 p.m. CEST**

Satellite Symposium: Sotirios "Sam" Tsimikas, M.D., senior vice president, clinical development and cardiovascular franchise leader at Ionis, is moderating a satellite symposium about ATTR-CM and investigational antisense medicine, eplontersen, for Ionis' CARDIO-TTRansform Study, on the Doctors Channel. He will be joined by renowned cardiologists, Marianna Fontana, M.D., Ph.D., and Ahmad Masri, M.D., M.S. The event will discuss the landscape and future of ATTR-CM treatment and how Ionis aims to potentially change the standards of care with its novel LICA technology.

The following posters will be available to congress attendees at the start of the meeting:

- Evaluation of the Efficacy and Safety of Eplontersen in Patients with Transthyretin-Mediated Amyloid Cardiomyopathy: The CARDIO-TTRansform Study
- Caregiving Burden Among Caregivers of Patients with ATTR Amyloidosis
- Comparison of Symptoms Experienced by Patients with ATTRwt and hATTR: Findings from the Development of an ATTR-Specific Patient Reported Outcomes
- Patient-Reported Outcomes by Time from Symptom Onset to First Pharmacotherapy Among Transthyretin Amyloidosis Patients
- Clinical Characteristics of Patients with Mutations Associated with Hereditary Transthyretin Amyloidosis Versus Those Associated with Other Inherited Cardiovascular Diseases: Insights from a Genetic Testing Programme
- Comparison of Patients with Mutations Associated with Hereditary Transthyretin Amyloidosis Versus Those Associated with Other Neuromuscular Diseases
- Three Newly Recognized Likely Pathogenic Variants of The TTR Gene Causing Hereditary Transthyretin Amyloidosis
- Cardiovascular Symptom Burden Prior to Diagnosis of Transthyretin Amyloid Cardiomyopathy Among Medicare Beneficiaries
- The Patient Journey Prior to Diagnosis of Hereditary Transthyretin Amyloidosis
- Long-Term Efficacy and Safety of Inotersen for Hereditary Transthyretin Amyloidosis: NEURO-TTR Open-Label Extension 3-Year Update
- Inotersen on Neuropathy Symptom and Change Scores in Hereditary Transthyretin Amyloidosis: Subgroups and Responder Analyses
- Efficacy of Inotersen for Neuropathic Impairment Scores in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy
- Responder Analyses Examining the Impact of Inotersen on Neuropathic Impairment Score in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy
- Treatment Satisfaction for Gene Silencing Pharmacotherapies in Hereditary Transthyretin Amyloidosis with Polyneuropathy

Details on presentation times can be found on the program [website](#).

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a premier late-stage pipeline highlighted by industry-leading neurological and cardiometabolic franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision of


becoming one of the most successful biotechnology companies.

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' business, and the therapeutic and commercial potential of Ionis' technologies and products in development. 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, 2020, 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.

 View original content to download multimedia: <https://www.prnewswire.com/news-releases/ionis-to-host-atlr-symposium-highlighting-cardio-tttransform-study-at-3rd-european-atlr-amyloidosis-meeting-301368874.html>

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