Ionis' antisense technology to be featured during virtual RNA at the Bench and Bedside II Conference

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CARLSBAD, Calif., Nov. 10, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that accomplished Ionis scientists will highlight the company's pioneering advancements in research and drug development in antisense technology during the RNA at the Bench and Bedside II Conference, to be held virtually Nov. 11-13, 2020. Plenary session presentations will include a review of the cardiovascular Phase 3 clinical trials Lp(a) HORIZON evaluating pelacarsen and CARDIO-TTRansform evaluating AKCEA-TTR-L_{Rx}; updates on how medicines developed using Ionis' advanced **LI**gand-**C**onjugated **A**ntisense (LICA) technology are showing promise as potential treatments for heart failure and metabolic diseases; and a discussion on technology advances in the delivery of antisense medicines, including oral and aerosol administration.

More than 30 speakers from across industry and academia will present at the conference, which is sponsored by Ionis, University of California San Diego School of Medicine, Nature Biotechnology and Nature Structural & Molecular Biology. The conference was inspired by Ionis founder and executive chairman of the board Stanley T. Crooke, M.D., Ph.D., who pioneered antisense technology and RNA-targeted therapeutics more than 30 years ago.

"As the leader in RNA-targeted therapeutics, Ionis is excited to participate in the RNA at the Bench and Bedside II Conference, which this year emphasizes clinical advances in cardiovascular and metabolic diseases. Ionis has established vast experience in cardio-metabolic diseases and this, along with many breakthrough advances in our technology, have created a rich pipeline of potential first-in-class medicines to treat a wide range of cardio-metabolic diseases with significant unmet medical need," said Brett P. Monia, Ph.D., Ionis' chief executive officer.

Following are Ionis presentations scheduled for the conference:

- Using ligand conjugated oligonucleotides for the treatment of metabolic disease, Richard Lee, Ph.D., executive director, antisense drug discovery
- How antisense drugs work: molecular mechanisms of cellular pharmacokinetics, pharmacodynamics and toxicity, KEYNOTE IV, Stanley T. Crooke, M.D., Ph.D., founder and executive chairman of the board
- Targeted delivery of ASO therapeutics, Punit Seth, Ph.D., vice president, medicinal chemistry
- RNA-targeted therapies for heart failure, Adam Mullick, Ph.D., vice president, drug discovery
- RNA-targeted therapeutics for cardiovascular disease, Sotirios "Sam" Tsimikas, M.D., Ph.D., senior vice president, clinical development and leader, cardiovascular franchise
- Characterization of phase separation in different phosphorothioate oligonucleotide-containing subcellular structures, Xue-Hai Liang, executive director, core antisense research

In addition to plenary sessions, numerous posters by Ionis scientists will be presented during the conference:

- Integrated assessment of Phase 2 studies on GalNAc3-conjugated 2'-o-methoxyethyl modified chimeric antisense oligonucleotides, Brenda Baker, Ph.D., executive director, development communications
- Population PKPD modeling of ION-682884, an antisense oligonucleotide in development for treatment of transthyretin amyloidosis, John Diep, M.S., PharmD, assistant director, preclinical development
- Enhanced antisense oligonucleotide delivery via AGTR1 targeting, Carol Kuo, Ph.D., senior research associate, antisense drug discovery
- Lipid based permeation enhancers potentiated sodium caprate-dependent oral bioavailability of GalNAc3-conjugated antisense oligonucleotides following intra-jejunal dosing in rats, Kaustubh Kulkarni, M.S., Ph.D., assistant director, preclinical development
- Subcellular fractionation and evaluation of hepatic cells after antisense oligonucleotide treatment in mice, Sue Murray, director, antisense drug discovery
- Mechanism-based population pharmacokinetic/pharmacodynamic (PKPD) Modeling of IONIS-TMPRSS6-L_{Rx} to guide early clinical development, Charvi Nanavati, Ph.D., principal scientist, preclinical development
- Pre-mRNA levels can be increased due to enhanced transcription by certain RNase H1-dependent antisense oligonucleotides, Joshua Nichols, senior research associate, core antisense research
- Towards a mechanistic understanding of ASO-mediated innate immune responses, Adam Pollack, Ph.D., senior scientist, core antisense research
- DYRK1a antisense oligonucleotides conjugated to GLP-1R agonist promote pancreatic beta cell proliferation, Andy Powers, Ph.D., assistant director, metabolic drug discovery
- Inhibition of the nonsense mediated decay pathway and translation termination machinery by antisense oligonucleotides upregulate nonsense-mutated CFTR expression and function, Eddie Sanderlin, Ph.D., postdoctoral fellow, antisense drug discovery
- Binding of phosphorothioate oligonucleotides with RNase H1 protein can cause conformational changes and alter the interactions of RNase H1 with other proteins, Lingdi Zhang, Ph.D., senior scientist, core antisense research

Ionis collaborators and partners will also present on Ionis research and clinical development stage programs during the conference:

- Targeted delivery of RNA therapeutics to pancreatic β-cells, Shalini Andersson, AstraZeneca, USA
- RNA targeting therapies for thromboembolic disorders, Harry Buller, Academic Medical Center, Amsterdam
- Antisense therapy has contributed to understanding and therapy of hyperlipoproteinemia KEYNOTE VI: Joe Witztum, University of California San Diego, USA
- Lp(a) an under recognized CV disease risk, Tom Thuren, Novartis, USA

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 all patients, 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.

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