

Ionis' leadership in RNA-targeted therapeutics recognized at the Oligonucleotide Therapeutics Society annual meeting

October 10, 2019

- Ionis scientists, including CEO Stanley Crooke, M.D., Ph.D., recognized with prestigious Paper of the Year Award for their work on advancing core antisense research

- Ionis' Holly Kordasiewicz, Ph.D., executive director of neurological disease drug discovery, to receive distinguished Young Investigator Award

CARLSBAD, Calif., Oct. 10, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapeutics, announced today that its scientists will present data highlighting advances in the company's antisense technology that have the potential to address an unprecedented range of diseases at the 15th Annual Meeting of the Oligonucleotide Therapeutics Society (OTS) in Munich, Germany, Oct. 13-16, 2019.

["Chemical modification of PS-ASO therapeutics reduces cellular protein-binding and improves the therapeutic index,"](#) published in *Nature Biotechnology*, is the 2019 recipient of the OTS *Paper of the Year Award*, recognizing the most impactful paper in the field of oligonucleotide therapeutics. The publication highlights the value of basic research in advancing RNA-targeted drug discovery and opens new horizons for antisense technology to bring even better medicines to patients. Xue-Hai Liang, Ph.D., executive director of core antisense research at Ionis, will accept the award on behalf of the publication's contributing authors, including Ionis CEO and founder Stanley Crooke, M.D., Ph.D.

Holly Kordasiewicz, Ph.D., Ionis' executive director of neurological disease drug discovery, is the recipient of the 2019 OTS *Mary Ann Liebert, Inc. publishes Young Investigator Award*. The Young Investigator Award recognizes the outstanding achievements and contributions by a professional scientist in the field of oligonucleotide therapeutics who has recently received his or her doctoral degree. Dr. Kordasiewicz is acknowledged for her dedication to discovering transformative antisense medicines for devastating neurodegenerative diseases, including spinal muscular atrophy, Huntington's disease and amyotrophic lateral sclerosis. Dr. Kordasiewicz will receive her award and give a brief talk on her research at the upcoming meeting.

Following is a schedule of all Ionis presentations in Central European Time:

Oral Presentations:

- Monday, October 14, 2:45 p.m. – 3:00 p.m. 'Cleavage of pre-mRNA in the Nucleus by RNase H1-dependent Antisense Oligonucleotides Causes RNA Polymerase II Transcription Termination'
- Tuesday, October 15, 10:20 a.m. – 10:35 a.m. 'Development and Characterization of AZD8701, a High Affinity Antisense Oligonucleotide Targeting FOXP3 to Relieve Immunosuppression in Cancer'
- Tuesday, October 15, 11:25 a.m. – 11:45 a.m. *Mary Ann Liebert, Inc. publishes Young Investigator Award*, 'Antisense Oligonucleotides for the Treatment of Neurodegenerative Diseases'
- Tuesday, October 15, 11:45 a.m. – 12:05 p.m. *Paper of the Year Award*, 'Chemical modification of PS-ASO therapeutics reduces cellular protein-binding and improves the therapeutic index'

Poster Presentations:

- Sunday, October 13, 6:00 p.m. – 7:30 p.m. 'Invalidation of GSK3B in the Pancreatic Beta Cell as a Therapeutic Target for T2D'

- Monday, October 14, 5:35 p.m. – 7:30 p.m. 'Improved Safety & Tolerability Profile with Ligand Directed Delivery of Antisense Oligonucleotides in Humans: An Integrated Comparison of Parent 2'-OMethoxyethyl Chimeric ASOs to the GalNAc3-Conjugates'
- Monday, October 14, 5:35 p.m. – 7:30 p.m. 'Controlling Chirality of Phosphorothioates in Antisense Oligonucleotides Does Not Enhance Potency or Duration of Effect in the CNS'
- Monday, October 14, 5:35 p.m. – 7:30 p.m. 'A novel and translational role for autophagy in antisense oligonucleotide trafficking and activity'

Complete abstracts, details on presentation times and changes to presentation dates can be found on the OTS website. The above listed dates are subject to change. Please check www.oligotherapeutics.org for the latest information.

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 both 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 treat a broad range of diseases including cardiovascular diseases, neurological diseases, infectious diseases, pulmonary diseases and cancer.

To learn more about Ionis visit www.ionispharma.com and follow us on twitter @ionispharma.

 View original content to download multimedia: <http://www.prnewswire.com/news-releases/ionis-leadership-in-rna-targeted-therapeutics-recognized-at-the-oligonucleotide-therapeutics-society-annual-meeting-300936161.html>

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