Bench to bedside strategies demonstrate Ionis' foremost expertise in RNA-targeted therapeutics at DIA/FDA Oligonucleotide Conference

October 28, 2019

- Ionis' C. Frank Bennett, Ph.D., head of research, to give keynote address
- 15 presentations highlighting the transformational potential of LICA medicines for a broad range of rare and common diseases

CARLSBAD, Calif., Oct. 28, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in RNA-targeted therapeutics, announced today that Ionis scientists will give presentations highlighting antisense medicines for neurological diseases and two **LI**gand **C**onjugated **A**ntisense (LICA) medicines, IONIS-FXI-L_{Rx} and IONIS-TMPRSS6-L_{Rx} at the DIA/FDA Oligonucleotide-Based Therapeutics Conference in North Bethesda, Maryland, Oct. 28-30, 2019. The presentations by Ionis will showcase the company's expertise in the field of antisense medicines and the potential of these medicines to transform the treatment of patients with rare and common diseases.

C. Frank Bennett, Ph.D., Ionis' senior vice president of research, will present the conference keynote address, 'Antisense Based Therapy for Neurological Diseases' on Tuesday, October 29 at 9:05 a.m. ET. The keynote will highlight the company's neurodegenerative programs including potential first-in-class therapies for diseases such as spinal muscular atrophy (SMA), amyotrophic lateral sclerosis (ALS), and Huntington's disease.

Other data and topics covered in the platform presentations, panels and posters will include:

- The benefit of using antisense medicines to address an unprecedented range of diseases in both rare and broad patient populations
- The therapeutic potential of IONIS-FXI-L_{Rx}, an antisense medicine designed to treat patients with clotting disorders
- The therapeutic potential of IONIS-TMPRSS6-L_{Rx} designed to treat patients with beta-thalassemia
- The Ionis approach to developing antisense medicines to treat patients with ultra-rare diseases
- The Ionis strategy for addressing regulatory agency inquiries regarding RNA-targeted medicines during marketing application reviews

Following is a list of key presentations of Ionis programs and collaborations in Eastern Time:
Oral Presentations:

- Monday, October 28, 9:30 a.m. – 11:00 a.m. 'Development Considerations for a 2′-MOE/DNA-modified ASO for the Treatment of Alexander Disease'
- Monday, October 28, 9:30 a.m. – 11:00 a.m. 'Batten Disease Patient Story Update'
- Monday, October 28, 11:30 a.m. – 1:00 p.m. 'Strategies for Addressing Regulatory Agency Questions During Marketing Application Review for Oligonucleotides Drugs'
- Monday, October 28, 2:00 p.m. – 3:30 p.m. 'IONIS-FXI-L_{Rx}, an FXI GalNac Conjugated Antisense Drug, Produces Potent and Sustained Reduction in FXI Activity in Normal Volunteers'
- Tuesday, October 29, 9:05 a.m. – 10:05 a.m. 'Antisense Based Therapy for Neurological Diseases'
- Wednesday, October 30, 8:00 a.m. – 10:00 a.m. 'New Approaches to Off-Target Assessment for ASOs'

Poster Presentations:

- Monday, October 28, 5:30 p.m. – 6:30 p.m. 'Pharmacodynamics and Toxicology Assessment of IONIS-TMPRSS6-$L_{Rx,}$ a 2'-MOE ASO Conjugate that Targets Human TMPRSS6'
- Monday, October 28, 5:30 p.m. – 6:30 p.m. 'IONIS-FXI-L_{Rx}, an FXI GalNac Conjugated Antisense Drug, Produces Potent and Sustained Reduction in FXI Activity in Normal Volunteers'

The full list of presentations can be found on the DIA website. The above listed dates and times are subject to change. Please check www.diaglobal.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.

IONIS' FORWARD-LOOKING STATEMENT
This press release includes forward-looking statements regarding Ionis' business, the therapeutic and commercial potential of our products, 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, 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, 2018, 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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View original content to download multimedia:http://www.prnewswire.com/news-releases/bench-to-bedside-strategies-demonstrate-ionis-foremost-expertise-in-rna-targeted-therapeutics-at-diafda-oligonucleotide-conference-300945991.html

SOURCE Ionis Pharmaceuticals, Inc.

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