Dr. C. Frank Bennett Receives Leslie Gehry Brenner Prize for Innovation in Science

October 23, 2018

Prestigious award recognizes the potential of Ionis' antisense technology to transform neurodegenerative disease

CARLSBAD, Calif., Oct. 23, 2018 /PRNewswire/ -- Ionis Pharmaceuticals, Inc (NASDAQ: IONS), the leader in antisense therapeutics, today announced that C. Frank Bennett, Ph.D., senior vice president of research and franchise leader for neurological programs at Ionis, has received the Hereditary Disease Foundation's (HDF) 2018 Leslie Gehry Brenner Prize for Innovation in Science. Dr. Bennett was honored for his leadership and continued commitment to developing antisense therapies for Huntington's disease (HD), a rare, progressive, neurodegenerative disorder resulting in deterioration in mental abilities and physical control. IONIS-HTT_{Rx} is designed to treat patients with HD and is the first and only drug to demonstrate reduction of neurotoxic mutant huntingtin protein, the underlying cause of HD, in patients.

The Leslie Gehry Brenner Prize for Innovation in Science award is given to a HD researcher who embodies qualities of inventiveness and imagination in science. Since its creation in 1967, the foundation's mission has been to fund innovative research towards curing HD and impacting other brain disorders. The Foundation created the Leslie Gehry Brenner Prize for Innovation in Science to honor the memory of HDF founder Frank Gehry's daughter.

"I am honored and grateful to be recognized by the Hereditary Disease Foundation and the Gehry family," said Dr. Bennett. "For nearly 30 years, our technology and learnings from developing drugs for diseases like Huntington's have paved the way for new potential therapies for diseases where no therapeutic approaches exist. I am proud of these achievements, but they would not have been possible without the vision and leadership of Dr. Stanley T. Crooke, founder of Ionis Pharmaceuticals, and the commitment of my colleagues to bringing new antisense therapies to patients living with unmet medical needs."

Nancy Wexler, President of the Hereditary Disease Foundation, said "We are thrilled to recognize Dr. Frank Bennett for his innovative work on IONIS-HTT_{Rx} for patients with Huntington's. Frank's accomplishments honor the spirit and memory of Leslie Gehry by embodying originality, spontaneity, precision and rigor – all critical attributes in a scientist."

Dr. Bennett's development of an antisense technology therapy for HD is being leveraged to develop antisense drugs for other neurological diseases, such as Alzheimer's disease and Parkinson's disease. Demonstrating that it is possible to treat HD by using antisense to target the messenger RNA from a specific gene would provide a proof-of-principle for researchers studying other neurodegenerative conditions, encouraging the development of similar treatments for many more patients.

As announced on Oct. 17, Dr. Bennet is also a recipient of the 2019 Breakthrough Prize in Life Sciences. This award acknowledges the innovation behind SPINRAZA® (nusinersen), the first and only approved treatment for patients with spinal muscular atrophy, a devastating neurodegenerative disease that is the leading genetic cause of infant death. The Breakthrough Prize honors the world's top scientists who have made transformative advances toward understanding living systems and extending human life, with one prize dedicated to work that contributes to understanding of neurological diseases. Dr. Bennett and collaborator Adrian Krainer, Ph.D., of Cold Spring Harbor Laboratory were co-recipients of this award.

About Antisense Technology

The instructions for making a protein are transcribed from a gene, or DNA, into a different genetic molecule called messenger RNA (mRNA). This process starts with the partial uncoiling of the two complementary strands of the DNA. One strand acts as a template and information stored in the DNA template strand is copied into a complementary RNA. Messenger RNA, or mRNA, are mature, fully processed RNA that code for proteins. Ribosomes, the cell's factories for manufacturing proteins, translate mRNA into proteins.

The mRNA sequence that carries the information for protein production is called the 'sense' strand. The complementary nucleotide chain that binds specifically to the mRNA sense strand is referred to as the "antisense" strand. Information contained in mRNA can be used to design chemical structures called antisense oligonucleotides (ASOs) or antisense drugs, which resemble DNA and RNA and are the complement of RNA.

Antisense drugs bind with high selectivity to the mRNA they are designed to target and interrupt the cell's protein production process by preventing the mRNA instructions from reaching the ribosome, thus inhibiting the production of the protein. Antisense drugs can also be designed to increase protein production for diseases caused by the lack of a particular protein or can modify the processing, or splicing, of the mRNA, which can alter the composition of the protein.

About Ionis Pharmaceuticals, Inc.

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, proprietary antisense technology platform with the potential to 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 – such as children and adults with spinal muscular atrophy (SMA). We created SPINRAZA® (nusinersen)* and are proud to have brought new hope to the SMA community by developing the first and only approved treatment for this disease.

Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 drugs with the potential to treat patients with cardiovascular disease, rare diseases, neurological diseases, infectious diseases and cancer. We created TEGSEDI™ (inotersen) the world's first RNA-targeted

therapeutic approved for the treatment of polyneuropathy of hereditary transthyretin (TTR) amyloidosis (ATTR) in adult patients that our affiliate Akcea Therapeutics is commercializing. Together with Akcea, we are also bringing new medicines to patients with cardiometabolic lipid disorders.

To learn more about Ionis follow us on twitter @ionispharma or visit http://ir.ionispharma.com/.

*Spinraza is marketed by Biogen.

Ionis' Forward-looking Statement
This press release includes forward-looking statements regarding the therapeutic and commercial potential of Ionis' technologies and products in development, including SPINRAZA® and TEGSEDI™ (inotersen). 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, 2017, and most recent Form 10-Q quarterly filing, which are 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.

View original content to download multimedia:http://www.prnewswire.com/news-releases/dr-c-frank-bennett-receives-leslie-gehry-brenner-prize-for-innovation-in-science-300735821.html

SOURCE Ionis Pharmaceuticals, Inc.

Ionis Pharmaceuticals Investor Contact: D. Wade Walke, Ph.D., Vice President, Investor Relations, 760-603-2741, OR Ionis Pharmaceuticals Media Contact: Roslyn Patterson, Vice President, Corporate Communications, 760-603-2681