Ionis Chief Scientific Officer C. Frank Bennett, Ph.D. to receive Rainwater Prize for Outstanding Innovation in Neurodegenerative Disease Research

January 26, 2023

CARLSBAD, Calif., Jan. 26, 2023 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) today announced that Chief Scientific Officer C. Frank Bennett, Ph.D., has been named a co-recipient of the Rainwater Prize for Outstanding Innovation in Neurodegenerative Disease Research. Dr. Bennett shares this year's prize with Don W. Cleveland, Ph.D., chair and distinguished professor of cellular and molecular medicine at University of California, San Diego and Timothy M. Miller, M.D., Ph.D., vice chair of research and David Clayson professor of neurology at Washington University School of Medicine. The prizes will be presented during the Eurotau 2023 conference on April 27, 2023, in Lille, France.

Drs. Bennett, Cleveland and Miller are being recognized for their work advancing antisense technology for the treatment of neurodegenerative diseases including amyotrophic lateral sclerosis (ALS), Huntington's disease, Alzheimer's disease and tauopathies. Their long-term collaboration has resulted in four investigational medicines entering clinical studies, including tofersen, currently under regulatory review for market authorization for the treatment of SOD1-ALS, with a Prescription Drug User Fee Act (PDUFA) action date of April 25, 2023; tominersen, currently in a Phase 2 clinical study for the treatment of Huntington's Disease; and IONIS-MAPT$_{Rx}$/BIIB080, for the treatment of Alzheimer's disease, frontotemporal degeneration and progressive supranuclear palsy. The foundational science the team established was pivotal to the advancement of SPINRAZA® (nusinersen), for the treatment of spinal muscular atrophy, into clinical studies and ultimately approval in 2016.

"I'm humbled to be recognized by the Rainwater Charitable Foundation with this prestigious award. It represents an affirmation for the work that everyone at Ionis is doing to deliver transformational genetic medicines to patients battling grave diseases," said Dr. Bennett. "I am equally honored to share the prize with my esteemed colleagues, Drs. Cleveland and Miller, whose expertise, dedication and tireless efforts have contributed significantly to bringing hope to patients and their families."

A founding member of Ionis, Dr. Bennett is responsible for continuing to advance Ionis' technology and expand its discovery platform. He is also the franchise leader for the gene editing program at Ionis. In 2020, Dr. Bennett received the Lifetime Achievement Award from the Oligonucleotide Therapeutics Society. In 2019, he was a co-recipient of the Breakthrough Prize in Life Sciences for his contributions to the discovery and development of SPINRAZA (with Adrian Krainer, Ph.D.) and a co-recipient of the inaugural Healy Center International Prize for Innovation in amyotrophic lateral sclerosis (ALS). Dr. Bennett also received the 2018 Hereditary Disease Foundation's Leslie Gehry Brenner Prize for Innovation in Science for his leadership and continued commitment to developing antisense therapies for Huntington's disease. He has been involved in the development of antisense oligonucleotides as therapeutic agents, including research on the application of oligonucleotides for inflammatory, neurodegenerative diseases, oligonucleotide delivery, pharmacokinetics and medicinal chemistry. Dr. Bennett, who is on the global list of Highly Cited Researchers™ – recognizing scientists whose peer-reviewed papers rank in the top 1% by citations for field and publication year – has published more than 230 papers in the field of antisense research and development and he is an inventor on more than 175 issued patents.

The Rainwater Prize Program recognizes scientific progress toward new treatments for neurodegenerative diseases associated with the accumulation of tau protein in the brain and fosters scientific discovery by elevating awareness of the gaps in neurodegenerative research, bringing new researchers into the tauopathy field, and awarding scientific achievements that could lead to innovative, effective treatments.

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 cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop and deliver life-transforming therapies.

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

SPINRAZA®, tofersen and IONIS-MAPT$_{Rx}$/BIIB080 are licensed to Biogen. Tominersen is licensed to Roche.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of Ionis' technologies, SPINRAZA, tofersen, tominersen, IONIS-MAPT$_{Rx}$ /BIIB080 and other 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 Dec. 31, 2021, and the most recent Form 10-Q quarterly filing, which are on file with the Securities and Exchange Commission. Copies of these 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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Ionis Investor Contact: 760-603-2331; Ionis Media Contact: 760-603-4679