

## Presentations at the 2018 American Academy of Neurology Highlight the Broad Potential of Antisense Drugs for Neurological Diseases

April 17, 2018

- Special conference session focused on therapeutic potential of antisense drugs to treat neurological diseases --
- Fourteen presentations on Ionis' antisense drugs to treat neurological diseases, including SMA, amyloidosis, Huntington's disease, Alzheimer's disease, and ALS --

CARLSBAD, Calif., April 17, 2018 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), the leader in antisense therapeutics, today announced that Ionis and its collaborators will present data from its neurological disease programs at the 70<sup>th</sup> American Academy of Neurology (AAN) meeting in Los Angeles, California from April 21-27, 2018.



Ionis' neurological disease programs will be highlighted in 9 presentations and 5 posters. Topics will include:

- The benefits SPINRAZA<sup>®</sup> (nusinersen) provides for individuals with spinal muscular atrophy (SMA) across a broad range of disease.
- The long-term efficacy and safety of inotersen in patients with hereditary ATTR.
- The potential benefits of IONIS-HTT<sub>Rx</sub> (RG6042) based on Phase 1/2 data in patients with Huntington's Disease.
- Demonstration of robust reductions in MAPT (tau) in the CNS of nonhuman primates with a good safety and tolerability profile for IONIS-MAPT<sub>Rx</sub>, tau-lowering antisense drug, to treat patients with Alzheimer's disease being evaluated in a Phase 2 study.
- The therapeutic potential of antisense therapies against multiple targets for amyotrophic lateral sclerosis (ALS) that demonstrate rescue of disease symptoms in animal models.
- Reductions and reversal of disease endpoints in animal models of Lafora disease.

In addition to the topics listed above, the conference will feature a special session entitled, 'Neuroscience in the Clinic: Antisense Oligonucleotide (ASO) Therapy', Monday, April 23, 3:30 p.m.-5:30 p.m. During this session, Ionis scientists, as well as scientists and external clinical investigators collaborating with Ionis, will discuss the broad potential of antisense drugs to treat neurological diseases. Dr. Frank Bennett, Ionis' senior vice president of research and franchise leader for the neurological programs, will provide a scientific overview on antisense therapy and Dr. Morie Gertz will present safety and efficacy data on inotersen.

Below are additional presentations and posters to be presented by Ionis scientists and their collaborators:

- Sidney Carter Award in Child Neurology: 'Spinal Muscular Atrophy is a Treatable Neurodegenerative Disease'; Presidential plenary session, oral presentation on Sunday, April 22, 10:45 a.m.-11:15 a.m.
- 'Open Label Extension of the Phase 3 Study NEURO-TTR to Assess the Long-term Efficacy and Safety of Inotersen in Patients with Hereditary Transthyretin Amyloidosis'; Poster session on Sunday, April 22, 11:30 a.m.-5:30 p.m.
- 'Burden of Hereditary Transthyretin Amyloidosis with Polyneuropathy in Patients Enrolled in the Phase 3 Study NEURO-TTR'; Poster session on Sunday, April 22, 11:30 a.m.-5:30 p.m.
- 'Inotersen Improves Norfolk Quality of Life-Diabetic Neuropathy Measures in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy in the Phase 3 Study NEURO-TTR'; Platform presentation on Sunday, April 22, 2:00 p.m.
- 'Design of the First-in-Human Study of IONIS-MAPT<sub>Rx</sub>, a Tau-lowering Antisense Oligonucleotide, in Patients with Alzheimer Disease'; Platform presentation on Sunday, April 22, 2:00 p.m.
- 'Ambulatory Function and Fatigue in Nusinersen-treated Children with Spinal Muscular Atrophy'; Poster Session on Monday, April 23, 11:30 a.m.-7:00 p.m.
- 'Safety and Efficacy of Nusinersen in Infants/Children with Spinal Muscular Atrophy (SMA): Part 1 of the Phase 2 EMBRACE Study'; Poster session on Monday, April 23, 11:30 a.m.-7:00 p.m.
- 'Spinocerebellar Ataxia Type 2 (SCA2) Spinal Cord Transcriptome Sequencing Informs Understanding on ALS'; Platform presentation on Monday, April 23, 4:18 p.m.
- 'Safety and Efficacy of Inotersen in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy (NEURO-TTR)'; Poster presentation on Monday, April 23 at 4:30 p.m.
- 'Effects of IONIS-HTT<sub>Rx</sub> in Patients with Early Huntington's Disease, Results of the First HTT-Lowering Drug Trial'. Plenary presentation on Tuesday, April 24 at 9:15 a.m.

- 'Strand-specific antisense oligonucleotides for C9ORF72 ALS/FTD'; Platform presentation on Tuesday, April 24 at 4:06 p.m.
- 'Longer-term Assessment of the Safety and Efficacy of Nusinersen for the Treatment of Infantile-onset Spinal Muscular Atrophy (SMA): An Interim Analysis of the SHINE Study'; Emerging Science presentation on Tuesday, April 24 at 5:45 p.m.
- 'Characterization of Later Childhood/Adult Spinal Muscle Atrophy Patients and Their Transitions of Care within U.S. Hospitals'; Poster session on Wednesday, April 25, 11:30 a.m.-7:00 p.m.
- 'Antisense Oligonucleotide Therapy for Lafora Disease'; Platform presentation on Friday, April 27 at 4:18 p.m.

Complete abstracts for the presentations can be accessed on the AAN website. The above listed dates are subject to change. Details on presentation times or changes to presentation dates can be found on the AAN website. Please check [www.aan.com](http://www.aan.com) for the latest information.

#### **ABOUT IONIS PHARMACEUTICALS, INC.**


Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class or best-in-class drugs, with over 40 drugs in development. SPINRAZA® (nusinersen) has been approved in global markets for the treatment of spinal muscular atrophy (SMA). Biogen is responsible for commercializing SPINRAZA. Inotersen and volanesorsen are two antisense drugs that Ionis discovered and successfully advanced through Phase 3 studies. Inotersen is under regulatory review for marketing approval in the U.S. and EU for the treatment of patients with hereditary ATTR amyloidosis. Volanesorsen is under regulatory review for marketing approval in the U.S., EU and Canada for the treatment of patients with familial chylomicronemia syndrome, or FCS. Volanesorsen is also in a Phase 3 study in patients with familial partial lipodystrophy, or FPL. Akcea, an affiliate of Ionis focused on developing and commercializing drugs to treat patients with serious and rare diseases, will commercialize inotersen and volanesorsen, if approved. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at [www.ionispharma.com](http://www.ionispharma.com).

#### **IONIS' FORWARD-LOOKING STATEMENT**

This press release includes forward-looking statements regarding Ionis Pharmaceuticals' business and the therapeutic and commercial potential of Ionis' technologies and products in development, including SPINRAZA, inotersen and volanesorsen. 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, 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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