

# Ionis and Biogen Win Prestigious Prix Galien Best Biotechnology Product Award for SPINRAZA

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CARLSBAD, Calif., Oct. 27, 2017 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) announced today that Ionis and Biogen have been awarded the Prix Galien USA Award for the Best Biotechnology Product in 2017 for SPINRAZA® (nusinersen). The Prix Galien USA Award recognizes extraordinary achievement in scientific innovation that improves the state of human health. The award was presented at a ceremony in New York City on October 26, 2017.



"Along with our colleagues at Biogen, we are honored to receive this prestigious award for SPINRAZA, a medical innovation that has already changed the lives of many patients and families suffering from the devastating consequences of spinal muscular atrophy," said Stanley T. Crooke, M.D., Ph.D., chief executive officer and chairman of Ionis. "We believe that SPINRAZA and its potential to help children and adults represents the very best of what our industry can achieve. The recognition of this highly distinguished panel underscores the vision and dedication of the teams behind this drug, and further energizes us to undertake the next challenges we face in our pursuit to help patients with dire medical needs."

The Prix Galien USA Committee is comprised of thirteen renowned leaders from the biomedical industry and academia, including four Nobel Laureates, responsible for evaluating nominees. The Prix Galien was created in France in 1970 by a pharmacist named Roland Mehl and aims to promote significant advances in pharmaceutical research. Each year, the award acknowledges the most impactful drugs introduced into the public market as well as the achievements of the best research team in the pharmaceutical field. It is seen as an influential event by all those interested in pharmaceutical research, including public authorities, scientists, pharmaceutical companies and medical press groups. For this reason, it is also considered the industry's equivalent of the Nobel Prize and the highest accolade for pharmaceutical research and development.

## ABOUT SMA

Spinal Muscular Atrophy (SMA) is characterized by loss of motor neurons in the spinal cord and lower brain stem, resulting in severe and progressive muscular atrophy and weakness. Ultimately, individuals with the most severe type of SMA can become paralyzed and have difficulty performing the basic functions of life, like breathing and swallowing.

Due to a loss of, or defect in the SMN1 gene, people with SMA do not produce enough survival motor neuron (SMN) protein, which is critical for the maintenance of motor neurons. The severity of SMA correlates with the amount of SMN protein. People with Type 1 SMA, the type that requires the most intensive and supportive care, produce very little SMN protein and do not achieve the ability to sit without support or live beyond two years without respiratory support. People with Type 2 and Type 3 produce greater amounts of SMN protein and have less severe, but still life-altering, forms of SMA.

## ABOUT SPINRAZA (nusinersen)

SPINRAZA is being developed globally for the treatment of spinal muscular atrophy (SMA).

SPINRAZA is an antisense oligonucleotide (ASO), using Ionis Pharmaceuticals' proprietary antisense technology, that is designed to treat SMA caused by mutations or deletions in the SMN1 gene located in chromosome 5q that leads to SMN protein deficiency. SPINRAZA alters the splicing of SMN2 pre-mRNA in order to increase production of full-length SMN protein. It was discovered and co-developed by Ionis, a leader in antisense therapeutics, and Biogen. ASOs are short synthetic strings of nucleotides designed to selectively bind to target RNA and regulate gene expression. Through use of this technology, SPINRAZA has the potential to increase the amount of full-length SMN protein in individuals with SMA.

SPINRAZA must be administered via intrathecal injection, which delivers therapies directly to the cerebrospinal fluid (CSF) around the spinal cord, where motor neurons degenerate in patients with SMA due to insufficient levels of SMN protein.

Biogen licensed the global rights to develop, manufacture and commercialize SPINRAZA from Ionis Pharmaceuticals in August 2016 and is now responsible for all development, regulatory and commercialization activities and costs for SPINRAZA. SPINRAZA was first approved by the U.S. Food and Drug Administration (FDA) on December 23, 2016, within three months of regulatory filing. SPINRAZA is also approved in the EU, Japan, Canada, Brazil and Switzerland. Biogen has also submitted regulatory filings in additional countries and plans to initiate additional filings in other countries.

For complete SPINRAZA U.S. prescribing information please visit [www.SPINRAZA.com](http://www.SPINRAZA.com).

## 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 three dozen 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. Drugs that have successfully completed Phase 3 studies include inotersen, an antisense drug Ionis is developing to treat patients with hereditary TTR amyloidosis (hATTR), and volanesorsen, an antisense drug discovered by Ionis and co-developed by Ionis and Akcea Therapeutics to treat patients with either familial chylomicronemia syndrome or familial partial lipodystrophy. Akcea, an affiliate of Ionis, is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious cardiometabolic diseases caused by lipid disorders. If approved, volanesorsen will be commercialized through Ionis' affiliate,

Akcea. Volanesorsen filings for marketing approval have been submitted in the U.S., EU and Canada. Inotersen is progressing toward regulatory filings for marketing authorization. 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 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, 2016, and its most recent quarterly report on Form 10-Q, which are on file with the SEC. 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.

Ionis Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics™ is a trademark of Ionis Pharmaceuticals, Inc. SPINRAZA® is a registered trademark of Biogen.

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