About SMA

SMA is a genetic disease characterized by the 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 most severe, life-threatening form, produce very little SMN protein and do not achieve the ability to sit without support or live beyond 2 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 symptoms. 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.

To support awareness and education in SMA, Biogen has launched Together in SMA in the United States. Together in SMA is a program created to provide informational materials and resources to the SMA community. Learn more at www.TogetherinSMA.com.

About Nusinersen

Nusinersen is investigational, potentially disease-modifying therapy for the treatment of SMA that was discovered and developed by Ionis Pharmaceuticals (NASDAQ: IONS), a leader in antisense therapeutics. Nusinersen is an antisense oligonucleotide (ASO) that is designed to alter the splicing of SMN2, a gene that is nearly identical to SMN1, in order to increase production of fully functional SMN protein.7
ASOs are short synthetic strings of nucleotides designed to selectively bind to target RNA and regulate gene expression. Through use of this technology, nusinersen has the potential to increase the amount of functional SMN protein in infants and children with SMA.

Both the U.S. and EU have granted nusinersen Orphan Drug status. Additionally, both the U.S. and EU regulatory agencies have granted special status to nusinersen, including Fast Track Designation and Priority Review in the U.S. and Accelerated Assessment in the EU.

Biogen exercised its option to worldwide rights to nusinersen in August 2016.

Biogen and Ionis Pharmaceuticals acknowledge support from the following organizations for nusinersen: Cure SMA, Muscular Dystrophy Association, and SMA Foundation, intellectual property licensed from Cold Spring Harbor Laboratory and the University of Massachusetts Medical School.

About Biogen

Through cutting-edge science and medicine, Biogen discovers, develops and delivers worldwide innovative therapies for people living with serious neurological, autoimmune and rare diseases. Founded in 1978, Biogen is one of the world's oldest independent biotechnology companies and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies. For more information, please visit www.biogen.com. Follow us on Twitter.

Biogen Safe Harbor

This press release contains forward-looking statements, including statements relating to the safety and efficacy of nusinersen, as well as clinical trial results and plans, potential regulatory filings and expected timelines and the submission of applications to regulatory authorities and the timing thereof. These statements may be identified by words such as "believe," "except," "may," "plan," "potential," "will" and similar expressions, and are based on our current beliefs and expectations. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Factors which could cause actual results to differ materially from our current expectations include the actual timing and content of submissions to and decisions made by the regulatory authorities regarding marketing authorization applications for nusinersen and the actual timing and final results of the nusinersen clinical trials. For more detailed information on the risks and uncertainties associated with our drug development and commercialization activities, please review the Risk Factors section of our most recent annual report or quarterly report filed with the Securities and Exchange Commission. Any forward-looking statements speak only as of the date of this press release and we assume no obligation to update any forward-looking statement.


Source: Biogen

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