Biogen to Present New Interim Data from Its Phase 1/2 Clinical Study of Tofersen (BIIB067) for the Potential Treatment of a Subtype of Familial Amyotrophic Lateral Sclerosis (ALS)

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- *Tofersen, an antisense oligonucleotide (ASO) that selectively targets the genetic driver of disease, is being studied for the potential treatment of ALS in adults with a confirmed superoxide dismutase 1 (SOD1) mutation*
- *Late breaking interim data to be presented at the American Academy of Neurology Annual Meeting (AAN) show a statistically significant reduction in SOD1 protein levels and a numerical trend towards slowing of clinical decline in SOD1-ALS patients treated with tofersen*
- *Biogen recently initiated VALOR, a phase 3 clinical trial to confirm the efficacy and safety of tofersen in SOD1-ALS patients*

CAMBRIDGE, Mass., May 01, 2019 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced today interim results of a phase 1/2 study of tofersen, an antisense oligonucleotide (ASO) being studied for the potential treatment of amyotrophic lateral sclerosis (ALS) in adults with a confirmed superoxide dismutase 1 (SOD1) mutation. The data will be presented at the American Academy of Neurology Annual Meeting (AAN) in Philadelphia, PA (May 4-10, 2019).

“The interim results of this study, which achieved proof-of-biology and proof-of-concept, support the initiation of a phase 3 clinical trial to confirm the efficacy and safety of tofersen in SOD1-ALS patients and further demonstrate the potential of ASOs to target the genetic driver of disease,” said Michael Ehlers, M.D., Ph.D., executive vice president, research and development at Biogen. “We are committed to bringing a potential breakthrough therapy to patients with ALS and we are expediting our efforts with the aim of addressing this urgent unmet need.”

The Phase 1/2 study of tofersen in SOD1-ALS is a randomized, placebo-controlled, single- and multiple-ascending dose study evaluating the safety, tolerability, pharmacokinetics, pharmacodynamics and exploratory efficacy endpoints of tofersen in 70 patients with ALS. In the multiple ascending dose portion of the study, 50 participants with SOD1 mutations were randomized to receive tofersen (20 mg, 40 mg, 60 mg, or 100 mg) or placebo for 12 weeks.

In the interim analysis, treatment with tofersen 100 mg (n=10) over a three-month period resulted in a statistically significant lowering of SOD1 protein levels in the cerebrospinal fluid (p=0.002) and a numerical trend towards slowing of clinical decline as measured by the ALS Functional Rating Scale-Revised (ALSFRS-R), slow vital capacity and muscle strength compared to placebo (n=12). Specifically, the mean change in ALSFRS-R score from baseline to Day 85 was -1.1 in the tofersen 100 mg group compared to -5.3 in the placebo group, on a 48-point scale. Across clinical measures, separation from the placebo group was most apparent in patients with fast progressing disease. The safety and tolerability profile in this analysis supports continued development of tofersen in ALS. The data will be presented by study co-principal investigator Timothy M. Miller, M.D., Ph.D., of the Washington University School of Medicine in St. Louis, MO on May 7, 2019 at 11:45 a.m. ET as a late breaking oral presentation.

“ALS is a devastating disease and there are currently no therapeutic options that reliably slow or halt its rapid progression,” said Merit Cudkowicz, M.D., co-principal investigator and the director of the Healey Center at Massachusetts General Hospital. “We are encouraged by the positive results of this phase 1/2 study of tofersen in patients with SOD1-ALS and are excited to advance this clinical program to a phase 3 trial to further investigate its therapeutic potential.”

In March 2019 the first patient was dosed in the Phase 3 VALOR study of tofersen in adults with ALS with a confirmed SOD1 mutation. The VALOR study aims to assess the efficacy and safety of tofersen versus placebo. The primary endpoint of this study is an analysis based on the ALSFRS-R score, which is a validated rating instrument that monitors the progression of disability in patients with ALS. Biogen is collaborating with regulators to further define the scope of the clinical data package required to support the registration of tofersen.

ALS is a rare and fatal neurodegenerative disease characterized by motor neuron loss in the brain and spinal cord that is responsible for controlling voluntary muscle movement. Symptoms may vary depending on the location of the motor neuron failure and may include limb weakness, difficulty breathing and trouble speaking and swallowing. There is a growing body of evidence that mutations within multiple genes are believed to cause ALS. ALS with SOD1 mutations is a rare subtype of familial ALS and accounts for approximately two percent of all ALS cases.

About tofersen
Tofersen is an antisense oligonucleotide (ASO) being developed for the treatment of ALS with SOD1 mutations. Tofersen binds to SOD1 mRNA, allowing for its degradation by RNase-H and reduction of SOD1 protein production. This is thought to decrease the toxicity of mutant SOD1 and potentially provide therapeutic benefit through improved survival and function to people with ALS with SOD1 mutations. Tofersen demonstrated proof-of-biology and proof-of-concept in a Phase 1/2 study. Biogen licensed tofersen from Ionis Pharmaceuticals, Inc. under a collaborative development and license agreement.

About Biogen
At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world’s first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp, and today has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first and only approved treatment for spinal muscular atrophy, and is focused on advancing neuroscience research programs in MS and neuroimmunology, Alzheimer’s disease and dementia, movement disorders, neuromuscular disorders, acute neurology, neurocognitive disorders, pain, and ophthalmology. Biogen also commercializes biosimilars of advanced biologics.

We routinely post information that may be important to investors on our website at www.biogen.com. To learn more, please visit www.biogen.com and follow us on social media – Twitter, LinkedIn, Facebook, YouTube.

Biogen Safe Harbor Statement

This news release contains forward-looking statements, including statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, including statements about results from the Phase 1/2 study of tofersen; the potential clinical effects of tofersen; the potential benefits, safety and efficacy of tofersen; the clinical development program for tofersen; the potential of our commercial business and pipeline programs, including tofersen; the anticipated benefits and potential of our collaboration arrangements with Ionis; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as “aim,” “anticipate,” “believe,” “could,” “estimate,” “expect,” “forecast,” “intend,” “may,” “plan,” “potential,” “possible,” “will,” “would” and other words and terms of similar meaning. 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. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including without limitation, uncertainty of success in the development and potential commercialization of tofersen; the risk that we may not fully enroll our clinical trials or enrollment will take longer than expected; unexpected concerns may arise from additional data, analysis or results obtained during our clinical trials; regulatory authorities may require additional information or further studies, or may fail or refuse to approve or may delay approval of our drug candidates, including tofersen; the occurrence of adverse safety events; the risks of other unexpected hurdles, costs or delays; failure to protect and enforce our data, intellectual property and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; and third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from our expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risk factors identified in our most recent annual or quarterly report and in other reports we have filed with the Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this press release.

We do not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments or otherwise.

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