Biogen's Salanersen Receives FDA Breakthrough Therapy Designation for Spinal Muscular Atrophy

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- Designation is supported by an exploratory analysis from the Phase 1b study showing that some children with SMA previously treated with gene therapy who had suboptimal clinical status experienced slowing of neurodegeneration and clinically meaningful improvements in motor function following initiation of salanersen
- Salanersen is an investigational antisense oligonucleotide dosed once-yearly with the potential to be a meaningful therapy in the future SMA treatment landscape

CAMBRIDGE, Mass., June 04, 2026 (GLOBE NEWSWIRE) -- Biogen Inc. (Nasdaq: BIIB) announced today that the U.S. Food and Drug Administration (FDA) has granted salanersen Breakthrough Therapy Designation for the treatment of spinal muscular atrophy (SMA). Breakthrough Therapy designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). Salanersen is an investigational novel antisense oligonucleotide (ASO) and has the potential to offer high efficacy in SMA with once-yearly dosing.

"The FDA's designation of salanersen as a breakthrough therapy recognizes that there is continued unmet need in spinal muscular atrophy, and there is more that can be done for people impacted by the disease," said Diana Castro, M.D., of the Neurology Rare Disease Center in Flower Mound, Texas. "In the Phase 1b study of salanersen, we saw unexpected improvements on exploratory endpoints in children previously dosed with gene therapy who gained critical functions, such as sitting and walking, after receiving salanersen. We are excited about the potential of salanersen and eager to help advance the Phase 3 program."

The FDA's decision is based on data from the Phase 1b study of salanersen, which were recently presented at the 2026 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference and the 5th International Scientific Congress on SMA (SMA Europe 2026). After initiation of once-yearly salanersen in children with SMA who had a suboptimal response to prior gene therapy, clinically meaningful improvements in motor function were observed as well as slowing of neurodegeneration, as measured by reduced neurofilament levels. Salanersen was generally well-tolerated in the study.

"This designation reflects the FDA's continued commitment to SMA and its recognition of the potential meaningful impact salanersen may offer," said Kenneth Hobby, President of Cure SMA. "It affirms what our SMA community has recently communicated to the agency: urgent, unmet needs remain, and promising therapies deserve a rapid path forward."

"This designation reflects the FDA's determination that salanersen has the potential to demonstrate substantial improvement over available therapies," said Stephanie Fradette, Pharm.D., Head of the Rare Neurology Development Unit at Biogen. "This is a significant milestone for our SMA portfolio as we advance the Phase 3 studies designed to establish the role of salanersen in the future SMA treatment landscape."

The salanersen Phase 3 program consists of three global studies:

- STELLAR-1 (recruiting), an open-label study, will evaluate the effects of salanersen in young (under 6 weeks old), treatment-naïve and clinically presymptomatic infants with a genetic diagnosis of SMA
- SOLAR (recruiting), an open-label study, will evaluate the effects of salanersen in teens and adults (aged 15–60 years) with SMA who are either treatment-naïve or previously treated with risdiplam
- STELLAR-2 (recruitment expected to begin in June 2026), a randomized, double-blind, sham-controlled study, will evaluate the effects of salanersen when initiated ~6 months after onasemnogene abeparvovec-xioi in infants with SMA who received presymptomatic treatment with gene therapy at 6 weeks of age or younger

More information on the STELLAR-1 study (NCT07221669), STELLAR-2 (NCT07444450) and SOLAR (NCT07444476) are available at clinicaltrials.gov.

About Salanersen
Salanersen (BIIB115) is a novel, intrathecally administered antisense oligonucleotide (ASO) in development for SMA. Salanersen is designed to correct splicing of *SMN2* pre-mRNA to increase production of SMN protein. It has a new chemistry that leads to high potency, enabling the potential for high efficacy with once-yearly dosing.

Salanersen is being evaluated in three global Phase 3 studies designed to evaluate safety and efficacy of 80 mg administered once-yearly in a broad spectrum of individuals living with SMA. Biogen licensed the global development, manufacturing and commercialization rights for salanersen from Ionis Pharmaceuticals, Inc. Salanersen was discovered by Ionis.

Salanersen Phase 1b Study Results
The Phase 1b study included participants (n=24, aged 0.5-12 years), who received at least 2 doses of salanersen (40 mg or 80 mg). The 80 mg dose will be further evaluated in the Phase 3 studies.

In participants who received salanersen 40 mg and 80 mg and had elevated baseline concentrations of neurofilament light chain (NfL), a potential marker of ongoing neurodegeneration, meaningful reductions (75%) in NfL levels were observed at six months; these reductions were sustained throughout the follow-up period. All 24 participants treated with salanersen experienced increases from baseline on one or more endpoints. Notably, 12 of the 24 achieved at least one new WHO motor milestone, and all participants maintained the motor milestones documented at their baseline. Salanersen has been generally well-tolerated at both 40 and 80 mg doses in the ongoing Phase 1 study, and most adverse events (AEs) have been mild to moderate in severity. As of the analysis, the most common AEs in the 40 mg group were upper respiratory tract infection and vomiting, and the

most common AEs in the 80 mg group were pyrexia and upper respiratory tract infection.

About Spinal Muscular Atrophy (SMA)
SMA is a rare, genetic, neuromuscular disease that affects individuals of all ages. It is characterized by a loss of motor neurons in the spinal cord and lower brain stem, resulting in progressive muscle atrophy and weakness.[1] SMA is caused by a deficiency in the production of survival motor neuron (SMN) protein due to a damaged or missing *SMN1* gene, with a spectrum of disease severity.[1] Some individuals with SMA may never sit; some sit but never walk; and some walk but may lose that ability over time.[2] In the absence of treatment, children with the most severe form of SMA would usually not be expected to reach their second birthday.[1]

SMA impacts approximately 1 in 10,000 live births,[3-6] is a leading cause of genetic death among infants[7] and causes a range of disability in teenagers and adults.[2]

About Biogen
Founded in 1978, Biogen is a leading biotechnology company that pioneers innovative science to deliver new medicines to transform patients' lives and to create value for shareholders and our communities. We apply deep understanding of human biology and leverage different modalities to advance first-in-class treatments or therapies that deliver superior outcomes. Our approach is to take bold risks, balanced with return on investment to deliver long-term growth. We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - Facebook, Instagram, LinkedIn, X, YouTube.

Biogen Safe Harbor
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, about the potential clinical effects of salanersen; the potential benefits, safety and efficacy of salanersen, including the potential to slow neurodegeneration and improve motor function; the clinical development program for salanersen; the identification and treatment of SMA; our research and development program for the treatment of SMA; the potential of our commercial business and pipeline programs, including salanersen; 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," "assume," "believe," "contemplate," "continue," "could," "estimate," "expect," "forecast," "goal," "guidance," "hope," "intend," "may," "objective," "outlook," "plan," "possible," "potential," "predict," "project," "prospect," "should," "target," "will," "would" or the negative of these words or 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. Given their forward-looking nature, these statements involve substantial risks and uncertainties that may be based on inaccurate assumptions and could cause actual results to differ materially from those reflected in such statements.

These forward-looking statements are based on management's current beliefs and assumptions and on information currently available to management. Given their nature, we cannot assure that any outcome expressed in these forward-looking statements will be realized in whole or in part. We caution that these statements are subject to risks and uncertainties, many of which are outside of our control and could cause future events or results to differ materially from those stated or implied in this document, including, among others, uncertainty of our long-term success in developing, licensing, or acquiring other product candidates or additional indications for existing products; expectations, plans, prospects and timing of actions relating to product approvals, approvals of additional indications for our existing products, sales, pricing, growth, reimbursement and launch of our marketed and pipeline products; the potential impact of increased product competition in the biopharmaceutical and healthcare industry, as well as any other markets in which we compete, including increased competition from new originator therapies, generics, prodrugs and biosimilars of existing products and products approved under abbreviated regulatory pathways; our ability to effectively implement our corporate strategy; difficulties in obtaining and maintaining adequate coverage, pricing, and reimbursement for our products; the drivers for growing our business, including our dependence on collaborators and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; risks related to commercialization of biosimilars, which is subject to such risks related to our reliance on third-parties, intellectual property, competitive and market challenges and regulatory compliance; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; and the occurrence of adverse safety events, restrictions on use with our products, or product liability claims; and any other risks and uncertainties that are described in other reports we have filed with the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov.

These statements speak only as of the date of this press release and are based on information and estimates available to us at this time. Should known or unknown risks or uncertainties materialize or should underlying assumptions prove inaccurate, actual results could vary materially from past results and those anticipated, estimated or projected. Investors are cautioned not to put undue reliance on forward-looking statements. A further list and description of risks, uncertainties and other matters can be found in our Annual Report on Form 10-K for the fiscal year ended December 31, 2025 and in our subsequent reports on Form 10-Q. Except as required by law, we do not undertake any obligation to publicly update any forward-looking statements whether as a result of any new information, future events, changed circumstances or otherwise.

Digital Media Disclosure
From time to time we have used, or expect in the future to use, our investor relations website (investors.biogen.com), the Biogen LinkedIn account (linkedin.com/company/biogen-) and the Biogen X account (https://x.com/biogen) as a means of disclosing information to the public in a broad, non-exclusionary manner, including for purposes of the SEC's Regulation Fair Disclosure (Reg FD). Accordingly, investors should monitor our investor relations website and this social media channel in addition to our press releases, SEC filings, public conference calls and webcasts, as the information posted on them could be material to investors.

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MEDIA CONTACT:
Biogen
Madeleine Shin
+1-781-464-3260
public.affairs@biogen.com

INVESTOR CONTACT:
Biogen
Tim Power
+1 781 464 2442
IR@biogen.com