Update on the Salanersen Development Program

September 10, 2026

Dear Spinal Muscular Atrophy (SMA) Community,

Biogen remains committed to addressing unmet needs in SMA through continued innovation and development of new treatment options. Central to this commitment is our research pipeline, including investigational therapies such as salanersen, which is currently under development. Beyond our pipeline, we continue to build on progress in SMA through the recently approved high dose regimen of SPINRAZA and investigational delivery innovations such as the ThecaFlex implantable device. Together, these advancements reflect our focus on expanding options for people living with SMA.

In response to requests we received from the SMA community for updates on the salanersen program, we are pleased to share that the Phase 3 salanersen studies are now open for enrollment. The pivotal STELLAR-1 study has also enrolled and dosed its first two participants in the United States and first participant in Japan.

What is Salanersen?
Salanersen is an investigational medicine being studied as a potential treatment for SMA. It is a type of medicine called an antisense oligonucleotide (ASO) that is designed to help the body produce more SMN protein. Salanersen uses a novel chemical structure with high potency, offering the potential for high efficacy with once-yearly intrathecal dosing.

Salanersen Clinical Development Program:
Salanersen is being evaluated in three global Phase 3 studies[1] designed to assess the safety and efficacy of once-yearly dosing in a broad range of individuals living with SMA. These studies are actively recruiting and will follow participants for up to 5 years to evaluate long-term outcomes. Data will be evaluated at multiple timepoints throughout the studies, with key analyses occurring before the completion of the full follow-up period. Salanersen remains investigational and has not been approved for use outside of clinical trials.

The program is comprised of the following studies:

- **STELLAR-1**, an open-label study, will evaluate the effects of salanersen in very young (under 6 weeks of age), treatment-naïve and clinically presymptomatic infants with a genetic diagnosis of SMA (NCT07221669). Approximately 40 sites are planned globally, with sites opening on a rolling basis. Families interested in learning more about eligibility and enrollment can find additional information and connect with participating centers through the STELLAR-1 myTomorrows website.

[1] Phase 3 studies provide the evidence regulators need to consider approving a new medicine.

- **STELLAR-2**, a randomized, double-blind, sham-controlled study, will evaluate the effects of salanersen when initiated in infants with SMA approximately 6 months after receiving onasemnogene abeparvovec-xioi as presymptomatic treatment at 6 weeks of age or younger (NCT07444450). Approximately 35 sites are planned globally, with sites opening on a rolling basis. Families interested in learning more about eligibility and enrollment can find additional information and connect with participating centers through the STELLAR-2 myTomorrows website.

- **SOLAR**, an open-label study, will evaluate the effects of salanersen in teens and older adults with SMA (aged 15–60 years) who are either treatment-naïve or previously treated with risdiplam (NCT07444476). Approximately 50 sites are planned globally, with sites opening on a rolling basis. Families interested in learning more about eligibility and enrollment can find additional information and connect with participating centers through the SOLAR myTomorrows website.

Additional information about the designs of the Phase 3 salanersen studies will be shared during an upcoming community webinar hosted by Cure SMA on September 17, 2026.

Individuals and families interested in participating are encouraged to speak with their healthcare provider about whether they may be eligible for the study. Individuals who are not receiving care at a participating study site may still have opportunities to participate through referral to a participating center.

We deeply appreciate the SMA community's ongoing partnership and contributions to scientific research.

Sincerely,
Stephanie Fradette, on behalf of the Biogen Development Team