



October 30th, 2025
Phase 3 Pivotal Trial Initiation Notice
to US PAGs & scientific societies in response to unsolicited questions

Subject: Biogen to Initiate Phase 3 Pivotal Study Evaluating Investigational Drug Salanersen

Dear Cure SMA & the SMA Community,

Per your request, we are pleased to share that Biogen plans to initiate Phase 3 development to evaluate the effectiveness and safety of salanersen, an investigational drug for the treatment of spinal muscular atrophy (SMA). Phase 3 initiation follows the announcement of the positive interim results of the Phase 1 study of salanersen, which informed the design of the studies.

Biogen is preparing for enrollment of the pivotal study (STELLAR-1) which will assess the efficacy and safety of salanersen in treatment-naïve presymptomatic infants with SMA. We plan to begin recruiting in the US by December 2025. STELLAR-1 is an open-label, single-arm study evaluating salanersen in a total of 30 infants who are ≤6 weeks of age, have a genetic diagnosis of SMA with 2 or 3 copies of the *SMN2* gene, have not developed symptoms of SMA (i.e., are presymptomatic), and have not received another treatment for SMA. Participants will receive salanersen 80 mg (through intrathecal injection) every 12 months during the five-year study. Through the study, researchers look to understand the number of participants with 2 copies of the *SMN2* gene that can sit without support at 12 months and the number of participants with 3 copies of the *SMN2* gene that can walk alone at 18 months. Other endpoints will evaluate long-term safety and tolerability, pharmacokinetics, and the effect of salanersen on biomarkers. More details about the STELLAR-1 study ([NCT07221669](https://clinicaltrials.gov/ct2/show/study/NCT07221669)) are available at ClinicalTrials.gov.

Biogen is also planning additional Phase 3 studies to evaluate salanersen in the broader SMA population, including treatment-naïve and previously-treated patients. More details on the additional studies will be shared in the months ahead.

We kindly invite your support in increasing awareness of STELLAR-1 and in helping families who may be interested in participating to then talk to their doctor about the study. Families interested in learning more about this clinical trial can also contact Biogen at clinicaltrials@biogen.com or call the Biogen Clinical Trials Center at 866-633-4636 to be connected with a Patient Navigator.

At Biogen, we are deeply committed to advancing research for people living with SMA. We are very grateful to the SMA community and patient advocacy leaders for your tireless efforts in supporting individuals and families impacted by this condition.

Sincerely,
Biogen