

9 July 2025

Dear SMA Europe,

We are pleased to respond to your request by sharing that Biogen announced topline results from the Phase 1 study of salanersen (BIIB115), an antisense oligonucleotide (ASO) being developed for the treatment of spinal muscular atrophy (SMA). These data were presented at the SMA Research & Clinical Care Meeting hosted by Cure SMA, 25-27 June 2025.

Salanersen has a similar mechanism of action to nusinersen but has a novel modification to the chemical backbone.

The Phase 1 study to evaluate the safety, tolerability and pharmacokinetics of salanersen has two parts. In Part A, four ascending doses were evaluated in healthy adult male volunteers. In Part B, two ascending doses are being evaluated in children living with SMA who previously received gene therapy. The Phase 1 study is ongoing, with participants scheduled to receive two doses of salanersen and followed for two years.

Interim results are from Part B (n=24) in individuals that received a single dose of either 40 mg or 80 mg salanersen.

- Salanersen was generally well tolerated at doses of 40 mg and 80 mg.
- In participants with elevated baseline concentrations of NfL (indicating ongoing neurodegeneration), initiation of salanersen led to mean reductions in NfL of 70% at six months. These reductions were sustained through the one-year dosing interval.
- In participants who had completed one-year of follow-up (n=8, all receiving 40 mg salanersen), 50% achieved new WHO motor milestones, whilst mean HFMSE and RULM scores increased by 3.3 points (SD 4.46) and 5.3 (SD 4.75) points respectively.

Based on the Phase 1 data, Biogen is currently engaging with global health authorities regarding the design of the Phase 3 studies.

More details about the Phase 1 study of salanersen are available at clinicaltrials.gov.

Biogen is deeply appreciative for those in the SMA community – from patients and caregivers to investigators – for their significant contributions to SMA research. Biogen remains committed to making a meaningful impact for those affected by SMA.

Sincerely,



Daniela Cohen
Biogen International