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The SOLAR Study: Phase 3 Study to Evaluate the Efficacy and Safety of Salanersen in Participants Aged 15 to 60 Years with Spinal Muscular Atrophy

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Objective

- To investigate the efficacy, safety, and PK of once-yearly intrathecal salanersen, an antisense oligonucleotide, in individuals aged 15 to 60 years with SMA, across both treatment-naive and risdiplam-treated populations.

Introduction

Background and Unmet Need

- DMTs have improved survival and motor development in SMA; however, unmet needs remain and are understudied in older teens and adults.
- Frequently identified unmet needs in this population include the stabilization or improvement of muscle strength, motor function, fatigue, respiratory function, swallowing, daily activities, and communication.[1]

Overview of Salanersen

- Salanersen (BIIB115) is a novel, intrathecally administered antisense oligonucleotide in development for SMA.
- Salanersen corrects splicing of *SMN2* pre-mRNA by binding to a region also targeted by nusinersen and is designed to increase SMN protein.
- Salanersen has a new backbone chemistry that leads to high potency, enabling the potential for high efficacy with once-yearly dosing.

Rationale for the SOLAR Study

- Phase 1 salanersen study (NCT05575011/EudraCT 2022-000956-12) is ongoing.
- Interim results show that in children with SMA who were previously treated with intravenous onasemnogene abeparvovec (OA), salanersen is well tolerated, drives sustained reductions of plasma NfL, and demonstrates consistent functional improvements (see associated oral presentation at MDA 2026 for further details*).
- These findings support the SOLAR Phase 3 study (NCT07444476) evaluating once-yearly intrathecal salanersen in older individuals with SMA.

Study Design

- SOLAR is an open-label, Phase 3 study evaluating salanersen 80 mg in participants with SMA aged 15 to 60 years.
- Endpoints assess multidomain clinical function (motor, respiratory, bulbar), PK, and safety/tolerability (**Figure 1**).
  - The primary efficacy evaluation in the treatment-naive cohort will be the change from baseline in HFMSE total score at 12 months of follow-up, based on the lack of expected improvement in this population without treatment.
  - The risdiplam-treated cohort will enable evaluation of the potential benefit of salanersen following risdiplam.
- The study will enroll a total of 90 participants into 2 cohorts (treatment-naive [n = 60] and risdiplam-treated [n = 30]).
  - Both ambulatory and non-ambulatory participants will be enrolled into each cohort.
- Key eligibility criteria are shown in **Figure 2**.
- The total study duration is 5 years, during which participants will receive 1 intrathecal dose of salanersen 80 mg annually, beginning on Day 1. A 1-year follow-up period will occur after the final dose (**Figure 3**).

Recruitment

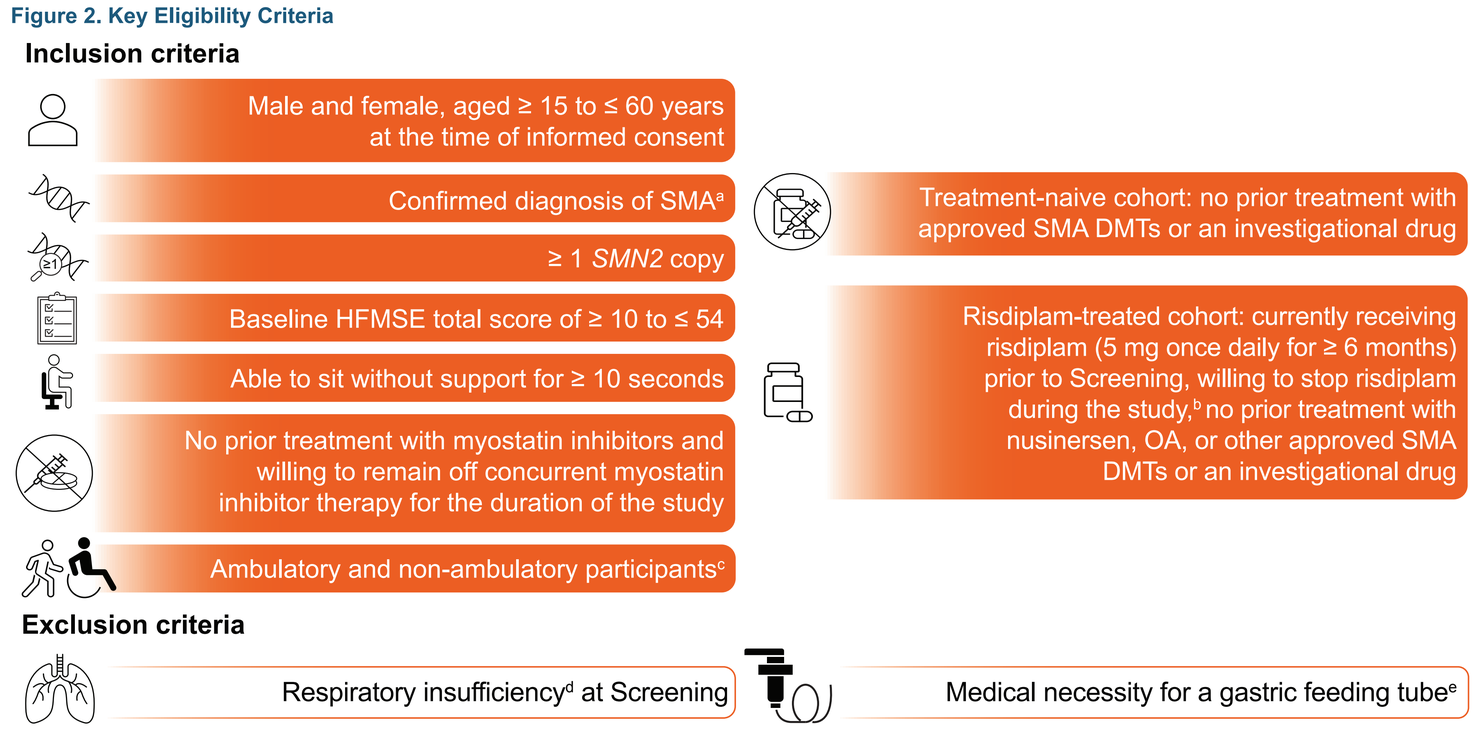
- The SOLAR study will be conducted at approximately 50 sites globally (see ClinicalTrials.gov for an up-to-date list of study sites) (**Figure 4**).
- The study is planned to start in Q2 2026.

Figure 1. Key Study Endpoints

Primary objective: To evaluate the clinical **efficacy** of salanersen	**Primary efficacy endpoint**	Change from baseline in HFMSE total score at 12 months in the treatment-naive cohort
	Secondary efficacy endpoints	HFMSE[a] ● RULM[b] ● 6MWT[c] (ambulatory participants only) ● CMAP amplitudes ● PGI-C ● SMAIS-ULM
	Key exploratory efficacy domains	Respiratory function ● Bulbar function ● Quality of life
Secondary objective: To evaluate the **safety, tolerability**, and **PK** of salanersen	**Secondary safety endpoints**	AEs ● SAEs
	Secondary PK endpoints	CSF salanersen concentrations ● Serum salanersen concentrations

All secondary and exploratory endpoints will be studied over the entire study.
[a]Endpoints include change from baseline in total score and the proportion of participants with a ≥ 3-point change from baseline. [b]Endpoints include change from baseline in total score and the proportion of participants with a ≥ 2-point change from baseline. [c]Endpoints include change from baseline in total distance walked and the proportion of ambulatory participants with a ≥ 30-meter change from baseline.

Figure 2. Key Eligibility Criteria

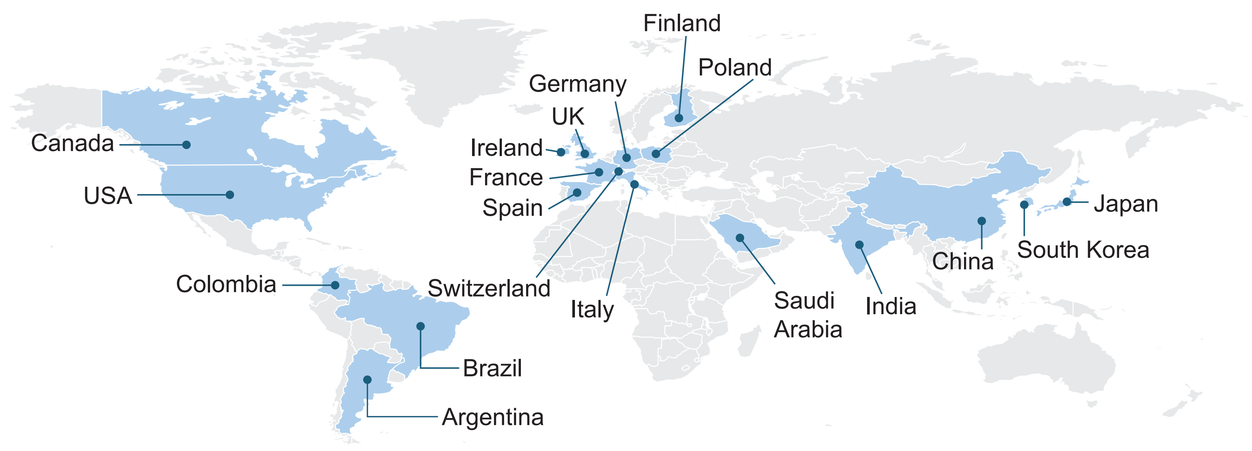


Additional eligibility criteria apply.
[a]Genetic documentation of 5q SMA homozygous gene deletion or mutation or compound heterozygous mutation and have clinical signs and symptoms consistent with SMA. [b]Last dose of risdiplam to be taken before the first dose of salanersen. [c]Ambulatory participants must be able to walk at least 10 meters independently without assistance and must be willing and able to complete the 6MWT at Screening. [d]Defined by the medical necessity for invasive or non-invasive ventilation for > 6 hours during a 24-hour period (except for nocturnal bilevel positive airway pressure). [e]Where the majority of nutrition is provided by this route as assessed by the Investigator.

Figure 3. SOLAR Study Design

[a]Follow-up visits become less frequent over the study duration.

Figure 4. SOLAR Target Countries



Acronyms

6MWT = Six-Minute Walk Test; AE = adverse event; CMAP = compound muscle action potential; CSF = cerebrospinal fluid; DMT = disease-modifying therapy; HFMSE = Hammersmith Functional Motor Scale Expanded; mRNA = messenger ribonucleic acid; NfL = neurofilament light chain; OA = onasemnogene abeparvovec; PGI-C = Patient Global Impression of Change; PK = pharmacokinetics; Q = quarter; RULM = Revised Upper Limb Module; SAE = serious adverse event; SMA = spinal muscular atrophy; SMAIS-ULM = Spinal Muscular Atrophy Independence Scale – Upper Limb Module; *SMN2* = *survival motor neuron 2*; WHO = World Health Organization.

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Reference

1. Peterson IS *et al. Neurol Ther* 2025;14:1083–92.

Disclosures

PS: Abcuro, Alexion, AMO Pharma, Arcellx, Argenx, Arthex, Astellas, Avidity, Biogen, Catalyst, CSL Behring, Dyne, Edgewise, EpiCrispr Bio, Genentech/Roche, Grifols, NMD Pharma, Novartis, Novartis Gene Therapies, Pfizer, PTC Therapeutics, Sarepta, Solid, UCB; **TH:** Biogen, Novartis, Roche; **AK-P:** AveXis, Biogen, Novartis, PTC, Roche; **CO'C:** Biogen, Roche, Tanabe; **OH:** Allergen, Biogen Idec, Cytokinetics, Janssen Cilag, Merck-Serono, Novartis, Ono Pharmaceuticals, Sanofi Aventis; Editor-in-Chief of *Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration*; **LE:** Biogen, BridgeBio, Catalyst, Edgewise, Genentech, Novartis, Scholar Rock; **SF**, **PS**, **SA**, **JI**, **JC**, **NS**, **SW**, **AV-B**, **GG**, **YL**, **RF**, **LB**, **JH:** employees of Biogen and may hold stock.

Clinical Trial Registration

SOLAR, NCT07444476.

*Oral presentation title: Phase 1 Interim Results Evaluating the Safety, Tolerability, Pharmacokinetics, and Exploratory Efficacy of Salanersen for Spinal Muscular Atrophy

SOLAR STUDY

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