17 March 2025

Dear members of SMA Europe,

In response to your request for important risdiplam and SMA clinical development programme updates, we are pleased to share news from the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference 2025, which is taking place this week in Dallas, Texas.

Final five-year data from the pivotal SUNFISH (NCT02908685) study will be presented by Roche, marking another milestone on the risdiplam journey. The study has concluded and we share this final readout with you, with our sincere thanks as critical partners in reaching this milestone.

Summary of data presented

As a reminder, the SUNFISH study assessed the efficacy and safety of risdiplam in people aged 2-25 years with Type 2 or non-ambulant Type 3 SMA. In 2020, the study met its primary endpoint, showing improvement in motor function from baseline in people who received risdiplam versus placebo. After the first 12 months, all participants had the opportunity to enter an 'open label' extension, in which everyone received risdiplam for another four years, including those who had previously received placebo. In total, the study lasted five years.

Exploratory results from the SUNFISH study showed continued stabilisation of motor function in people with Type 2 and 3 SMA, aged 2-25 years at enrollment, who received risdiplam for up to five years.[1] Findings, as reported in our press release, included:

- The increase in motor function after the first year of risdiplam treatment remained generally stable at five years as measured by changes in Motor Function Measure 32 (MFM-32).
- Patients (>12 years of age) and caregivers reported continuous improvement or stabilisation in levels of independence for performing daily activities such as dressing, picking up objects and washing, as measured by the SMA Independence Scale (SMAIS-ULM).
- The overall rates of adverse events (AEs) and serious adverse events (SAEs) were reflective of the underlying disease and were consistent with previous data. No treatment-related AEs led to withdrawal from the study.

We would like to take this opportunity to express our gratitude to all the patients, families and caregivers, healthcare professionals and patient groups who have been a critical part of this study. We are continuing to learn more and more about managing SMA and the role of risdiplam in this, and it has been a privilege to have so many people participate, contribute and make this study possible.

If you have any questions about the information provided, please do not hesitate to reach out.

Sincerely,

Louisa Danielle Townson

Louisa Townson, on behalf of the Roche Global SMA Team
Global Patient Partnership

References

1. Servais L, et al. SUNFISH Parts 1 and 2: 5-year efficacy and safety data of risdiplam in Types 2 and 3 spinal muscular atrophy (SMA). Presented at Muscular Dystrophy Association (MDA) Clinical and Scientific Conference, Dallas, TX, USA; March 16–19, 2025

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