14 October 2024

Dear members of SMA Europe,

As part of our ongoing partnership and in response to your request for risdiplam and SMA clinical development programme updates, we are pleased to share news from the 29th Annual Congress of the World Muscle Society, which took place last week in Prague.

Roche was proud to present two-year data from the RAINBOWFISH study, alongside four poster presentations to help advance broader understanding of SMA, including a retrospective analysis of feeding abilities at year 2 in the FIREFISH study and three real world data initiatives.

Summary of data presented from the RAINBOWFISH trial

The RAINBOWFISH trial (NCT03779334) is an open-label study investigating risdiplam in infants who were up to six weeks old and not yet displaying symptoms of SMA when they entered the study. Two-year data demonstrate ongoing efficacy and safety of risdiplam in infants treated before symptoms appeared.[1] For more details, please see our press release here.

Notably, after two years of treatment:

- All 18 children with three or more copies of the *SMN2* gene achieved standing and walking milestones as assessed by BSID-III* and HINE-2* tests. Most children achieved these milestones within World Health Organization (WHO) windows of typical child development.
- Of the five children with two copies of the *SMN2* gene, all could sit and three could stand and walk independently.
- All children were able to swallow and feed orally and none required permanent ventilation. Furthermore, children showed cognitive skills typical of those without SMA, as assessed by the BSID-III Cognitive Scale.
- There were no deaths or Adverse Events (AEs) leading to withdrawal or treatment discontinuation. The most common AEs were teething, gastroenteritis, diarrhoea, eczema and fever.

We would like to extend our sincere appreciation to all the families, healthcare professionals, and patient groups who contributed to this study. These results add to a growing body of evidence that demonstrate the importance of newborn screening and early intervention in babies born with SMA. As proud members of initiatives like the European Alliance for Newborn Screening for SMA, we hope to see the inclusion of SMA in newborn screening programs around the world.

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

Sincerely,

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

References

1. Servais L, et al. RAINBOWFISH: 2-year efficacy and safety data of risdiplam in infants with presymptomatic SMA. Presented at World Muscle Society Annual Congress, 2024.

* BSID-III, Bayley Scales of Infant and Toddler Development, third edition (BSID-III); HINE-2, Hammersmith Infant Neurological Examination, Module 2

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