

10 June 2024



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 Cure SMA Research & Clinical Care Meeting 2024, which took place last week in Texas.

Final five-year data from the pivotal FIREFISH ([NCT02913482](#)) study were presented by Roche, marking another milestone on the risdiplam journey. We are grateful to share these findings with you, as critical partners in reaching this milestone.

Summary of data presented

Data from the FIREFISH study confirm risdiplam's long-term efficacy and safety profile in 58 infants with symptomatic Type 1 SMA.¹ After five years of treatment, findings show:

- 91% of children treated with risdiplam were still alive, 81% of whom were alive without permanent ventilation.
- Most children maintained their feeding and swallowing abilities, with 96% able to swallow and 80% able to feed without a feeding tube.
- The majority (59%) of children were able to sit without support for at least 30 seconds.
- The overall rate of adverse events decreased by 66% between year one and the fifth and final year of study. The most common adverse events were upper respiratory tract infection, fever, and pneumonia.

For more details, please see our press release [here](#).

This data read-out marks the end of the FIREFISH study. At Roche, we believe that communicating the results of our completed clinical studies in lay language is the right thing to do for patients. To honour this, in line with EU Clinical Trial Regulation, all study participants will receive a lay person summary of the FIREFISH study via their doctor in the coming months, and we will publish this on the Roche "ForPatients" website.

We would like to take the opportunity to deeply thank all the patients, families, healthcare professionals and patient groups who contributed to this important trial. Throughout the process we have learned so much about treating SMA, and it has been a privilege to be part of the journeys of all the children and their families who made this study possible.

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

Sincerely,

A handwritten signature in black ink that reads "Louisa Danielle Townson".

Louisa Townson, on behalf of the Roche Global SMA Team

References

1. Mazurkiewicz-Beldzińska W, et al. FIREFISH Parts 1 and 2: 5-year efficacy and safety of risdiplam in Type 1 SMA. Presented at Cure SMA Research & Clinical Care Meeting 2024.