15 April 2021

Dear members of SMA Europe,

We are pleased to share new two-year data from part 2 of the FIREFISH study, which is assessing Evrysdi™ (risdiplam) in the treatment of infants aged 1-7 months at enrollment with symptomatic Type 1 spinal muscular atrophy (SMA). The data show that risdiplam continued to improve motor function between months 12 and 24, including the ability to sit without support. These longer-term findings – which we are sharing with you in response to your request to receive regular updates - will be presented at the 73rd American Academy of Neurology (AAN) Annual Meeting being held virtually during April 17-22, 2021.

The data build upon one-year findings from FIREFISH Part 2, which measures the ability to sit without support for at least five seconds as its primary endpoint (primary endpoint). At 12 months, 29% (12/41) of infants treated with risdiplam achieved this milestone, and this figure increased to 61% (25/41) in this latest 24-month data. In the natural course of Type 1 SMA, without treatment children are not able to sit without support for five seconds.

The findings also show that safety for risdiplam was consistent with its established safety profile observed in previous studies and there were no medicine-related adverse events leading to participants stopping treatment or withdrawing from the study.

The 24-month FIREFISH data released will also cover additional motor milestones such as the ability to swallow and feed orally, and survival data. For a more in-depth look at the findings, please access the press release at the following location: https://www.roche.com/media/releases/med-cor-2021-04-15.htm

Following last month's European Commission approval of risdiplam, this latest data announcement is yet another reason to express our sincere gratitude to all clinical trial participants, to SMA Europe and members, like you, for playing a key part in the risdiplam development programme. We greatly appreciate your continued support.

If you have any questions about this update, please do not hesitate to contact me.

Sincerely,

Fani Petridis

Fani Petridis, on behalf of the Roche Global SMA Team
Senior Global Patient Partnership Director, Rare Diseases (SMA)