April 28, 2020

Dear Members of SMA Europe,

As part of our ongoing partnership and following your request to receive important and timely information about the risdiplam clinical development program, we are pleased to share with you an update on part 2 of the FIREFISH clinical trial. FIREFISH Part 2 is a pivotal global study evaluating risdiplam in infants aged 1 – 7 months old with symptomatic Type 1 spinal muscular atrophy (SMA).

As you will see in the enclosed press release, part 2 of the FIREFISH clinical trial has met its primary endpoint with 29% of infants sitting without support for five seconds by month 12, as assessed by the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development Third Edition (BSID-III). In addition, 18 (43.9%) infants were able to hold their head upright, 13 (31.7%) were able to roll to the side and 2 (4.9%) infants were able to stand with support, as measured by the Hammersmith Infant Neurological Examination 2 (HINE-2). Safety for risdiplam in the FIREFISH study was consistent with its previously reported safety profile and no new safety signals were identified.

Please access the press release at the following location:
https://www.roche.com/media/releases/med-cor-2020-04-28.htm

The data were selected for the 72nd American Academy of Neurology (AAN) Annual Meeting and will be made available online via virtual presentation in the coming weeks.

We want to thank you for your continued partnership and everyone in the community, especially the patients and families who have participated in the risdiplam clinical development programme. We would not be where we are today without you! We look forward to providing further updates as they become available.

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 Director, Global Patient Partnership - Rare Diseases (SMA)