16 March 2021

Dear members of SMA Europe,

As per your request to receive regular updates about the risdiplam clinical development program, we are pleased to share with you new 2-year longer-term data from part 2 of the SUNFISH clinical trial. These data will be presented at the 2021 Muscular Dystrophy Association (MDA) Virtual Clinical & Scientific Conference taking place from March 15-18.

SUNFISH part 2 is an ongoing, global study evaluating the efficacy and safety of risdiplam in people aged 2-25 years with Type 2 or non-ambulant Type 3 spinal muscular atrophy (SMA). The study is placebo-controlled which means that patients were treated with risdiplam (n=120) or with placebo and risdiplam (n=60; patients in the placebo arm received placebo for 12 months followed by risdiplam treatment for 12 months).

As you will see in the enclosed press release, these new results of nearly a year of additional follow-up, show that people treated with risdiplam continue to demonstrate sustained benefit from the treatment. In more detail, gains in motor function seen after 12 months of treatment with risdiplam either continued to improve or were maintained at month 24 across a number of primary and secondary endpoint measures. In addition, the number of serious adverse events, high-grade adverse events and treatment-related adverse events observed decreased in the second year versus the first year, further demonstrating the favorable safety profile of risdiplam.

Please access the press release at the following location:
https://www.roche.com/media/releases/med-cor-2021-03-16.htm

We would like to thank SMA Europe and the wider community for your continued partnership and support, especially the many people and families living with SMA who have participated in the risdiplam clinical development programme. We will be in touch very soon with further important updates!

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)