27 February 2020

Dear members of SMA Europe,

As part of our ongoing partnership and following your request to be kept updated on the risdiplam clinical development program, we are pleased to let you know that recruitment for the clinical trial JEWELFISH is now complete.

This milestone marks the enrolment of 174 participants from across the globe. The open-label study investigates the safety, tolerability and efficacy of risdiplam in people with SMA aged 6 months to 60 years, previously enrolled in study BP29420 (Moonfish) with the splicing modifier RO6885247 or previously treated with olesoxime, nusinersen or onasemnogene abeparvovec-xioi.[1]

This means that RAINBOWFISH is now the only global clinical trial with risdiplam currently recruiting. RAINBOWFISH is designed to explore the effect and safety of risdiplam in new-born babies up to 6 weeks old with a genetic diagnosis of SMA, who have not yet shown symptoms[2]. More information about RAINBOWFISH and its trial sites can be found on the ClinicalTrials.gov website (ClinicalTrials.gov Identifier: NCT03779334).

As always, we are ever thankful to the SMA patient organizations for their support as well as all the patients and families who have participated in the clinical trial program, with the ultimate aim to bring an effective, new treatment option to those who need it most.

We look forward to providing further updates on the trial program milestones, as they become available.

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

Sincerely,

Fani Petridis

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

[1] https://clinicaltrials.gov/ct2/show/NCT03032172?term=Jewelfish&draw=2&rank=1

[2] https://clinicaltrials.gov/ct2/show/NCT03779334?term=Rainbowfish&draw=2&rank=1