3 October 2018

Dear SMA community,

We are happy to share an update on our FIREFISH and SUNFISH studies of risdiplam. We would also like to acknowledge the tremendous support and partnership of SMA Patient Groups around the world and to thank those people with SMA who are participating in these studies in advancing research in SMA.

Risdiplam is an investigational, SMN2 splicing modifier that is given by mouth (or g-tube) and distributes widely throughout the body. Risdiplam is designed to help the SMN2 gene produce more SMN protein. We are developing risdiplam in collaboration with PTC Therapeutics and the SMA Foundation.

At the 23rd International Annual Congress of the World Muscle Society in Mendoza, Argentina, we presented an update of the risdiplam programme, including interim clinical data from Part 1 of the FIREFISH and SUNFISH studies. Further information about the data can be found here: www.roche.com/media/releases.

FIREFISH

News:
Updated results from approximately 6 months of treatment with risdiplam in FIREFISH Part 1 have been presented at the World Muscle Society Congress and you can read more about this in the link above.
Enrolment in FIREFISH Part 2 is ongoing and is almost complete.

FIREFISH background:
FIREFISH is a two-part study in babies between 1 to 7 months of age with Type 1 SMA. It is an open label study, which means that all babies receive risdiplam and there is no placebo. The objective of Part 1 was to assess the safety and determine the dose of risdiplam for Part 2 of the study. Part 1 has been completed and enrolled 21 patients. The participants of Part 1 are continuing to receive risdiplam and are now enrolled in the open-label extension phase of the study.

Part 2 will assess the safety and efficacy of risdiplam in approximately 40 babies. The main analysis will be based on how many babies can sit without assistance at one year of treatment.

SUNFISH

News:
Updated results from 12 months of treatment in SUNFISH Part 1 have been presented at the World Muscle Society Congress and you can read more about this in the link above.
We are also happy to share that enrolment in SUNFISH Part 2 is now complete and includes 180 participants.

SUNFISH background
SUNFISH is a two-part study evaluating risdiplam in people with Type 2 and 3 SMA between 2 and 25 years of age. It is placebo controlled, with two out of every three participants receiving risdiplam and one receiving placebo. The objective of Part 1 was to assess the safety profile and concentration of risdiplam at different dose levels as well as the level of SMN protein in blood. Part 1 has been completed and enrolled 51 participants. The participants from Part 1 of SUNFISH are continuing to receive risdiplam and are now enrolled in the open-label extension phase of the study.

Part 2 is the pivotal part of the study and will assess efficacy and safety of risdiplam after 12 months of treatment.

You can read more about the risdiplam studies (including FIREFISH, SUNFISH and JEWELFISH) on www.clinicaltrials.gov, www.clinicaltrials.register.eu and www.roche-sma-clinicaltrials.com.

We will be sharing updates on risdiplam as the programme advances, including more details about RAINBOWFISH, our planned study in pre-symptomatic babies.

Thank you to those of you who are participating in clinical studies in SMA and please contact me at Sangeeta.jethwa@roche.com if you would like additional information.

Kind regards

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