12 October 2017

SUNFISH Study Update
Results of Part 1 and start of Part 2

Dear SMA Community

We are pleased to share an update on the SUNFISH study evaluating RG7916 in people aged 2-25 years old with Type 2/3 SMA. RG7916 is an SMN2 splicing modifier that is taken by mouth (or by g-tube) and is being developed in collaboration with PTC Therapeutics and the SMA Foundation.

SUNFISH is a two-part study in which Part 1 will allow selection of the dose of RG7916.
Part 2 is designed to assess the effectiveness and is the pivotal registration part of the study.
If positive, results may be used to support Health Authority submissions and potential approval and access to RG7916.

The first group of participants enrolled into SUNFISH Part 1 has received RG7916 for more than 10 months and an interim analysis was recently presented at the International Conference of the World Muscle Society in France. In this analysis, all participants (51 people) had received RG7916 for 28 days or longer. SMN protein increased by up to two and a half times. This increase has been sustained throughout the duration of treatment (up to 250 days). To date, RG7916 remains well-tolerated at all doses and no-one has withdrawn from any RG7916 study due to drug related side effects.

The information from Part 1 has allowed us to confirm the dose of RG7916 for Part 2 of SUNFISH and the first patient has been enrolled into Part 2.

We would like to thank the SMA community and all of the families who participate in clinical trials. Your participation has helped to advance the development of RG7916 as well as the research in SMA.

If you would like additional information about the SMA program, please visit www.roche-sma-clinicaltrials.com, www.clinicaltrials.gov (search for SUNFISH) and www.clinicaltrialsregister.eu.

Please contact your physician if you are interested in taking part in SUNFISH.

Roche is committed to making a difference in SMA and we look forward to providing future updates on RG7916.

Best regards

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