

12<sup>th</sup> September 2017

## Start of Part 2 of the SUNFISH study

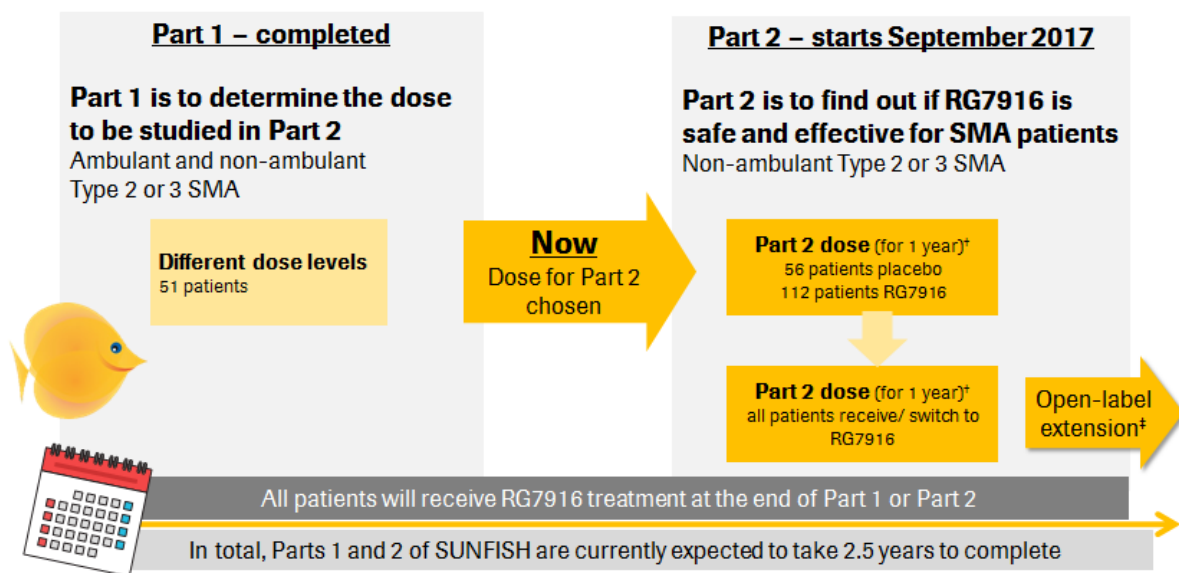
Dear SMA Community

We are focused on progressing our studies with our investigational molecule RG7916 with utmost speed and quality and are pleased to share an update on the SUNFISH study.

RG7916 is an SMN2 splicing modifier, which is given daily by mouth (or g-tube) and distributes widely throughout the body.

Part 1 of the SUNFISH study investigating RG7916 in people with type 2 or 3 SMA (2-25 years of age) has been fully enrolled. Based on the information from Part 1 we have successfully selected a dose and will now begin Part 2, the pivotal part of the study.

Part 1 of SUNFISH assessed how safe and well tolerated RG7916 was at different dose levels. It also assessed the effects of RG7916 in the body (e.g. mRNA and SMN protein levels). An analysis of Part 1 of SUNFISH will be presented at the World Muscle Society conference in October 2017.



† This updated protocol is pending final approval from the Health Authorities

‡ Patients from Parts 1 and 2 of the study will enter the open-label extension following completion of their treatment period

Part 2 of SUNFISH has been designed to assess how safe and effective RG7916 is in non-ambulant children and young adults with Type 2 and Type 3 SMA at the dose selected in Part 1. SUNFISH is a pivotal study, and if positive, these results may be used to support Health Authority approval and access to RG7916. We have updated the SUNFISH design to limit the amount of placebo that participants receive, whilst ensuring the study meets the requirements of Health Authorities.

Enrolment into part 2 of SUNFISH is expected to begin in September 2017 in France and Belgium.

Italy, Switzerland, Germany and Spain will begin later in the year.

Additional countries and sites in Europe, ex-US and in the US will be opened following Health Authority and Ethics Committee approvals.

You can visit [www. Roche-sma-clinicaltrials.com](http://www. Roche-sma-clinicaltrials.com) to read more about our SMA programme and visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and [www.clinicaltrialsregister.eu](http://www.clinicaltrialsregister.eu) for information about countries and sites. Please contact your physician if you are considering taking part in SUNFISH.

We would like to thank those of you who participate in clinical trials. Your very personal contribution is important in advancing our understanding of SMA.

We would also like to acknowledge the collaboration with the SMA community. Your support has been essential in advancing the RG7916 programme.

RG7916 is in clinical development in collaboration with PTC Therapeutics and the SMA Foundation.

Roche is committed to making a difference in SMA and we look forward to updating you on our programme.

Best regards



Sangeeta Jethwa, MD, on behalf of the Roche SMA team

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