



21<sup>st</sup> December 2016

Dear SMA community,

Roche, together with our collaboration partners, PTC Therapeutics and the SMA Foundation, would like to update you on our investigational clinical development programme for RG7916 (RO7034067), our oral SMN2 splicing modifier in SMA.

We are fully focused on addressing the urgent needs of people living with SMA and our goal is to study RG7916 in as broad a range of patients as possible. There are currently three studies in the RG7916 programme: SUNFISH, FIREFISH and JEWELFISH, which all include different populations.

We are pleased to let you know that SUNFISH (BP39055) and FIREFISH (BP39056) are actively recruiting in Europe and will expand to include additional countries and sites in 2017. JEWELFISH (BP39054) is expected to begin recruiting in Europe and the USA in early 2017.

SUNFISH is evaluating RG7916 in children and young adults (aged 2-25 years) with Type 2 and 3 SMA. It is a randomised, placebo-controlled study of 12 months duration, followed by an open label extension where all study participants will receive RG7916.

FIREFISH is evaluating RG7916 in babies (aged 1-7 months) with Type 1 SMA. All babies will receive RG7916 as there is no placebo in this study.

Both SUNFISH and FIREFISH have two parts:

Part 1: Designed to select the dose of RG7916

Part 2: Assess how safe and effective R7916 is at the selected dose

JEWELFISH (BP39054) is our first step towards exploring RG7916 in a broader population of patients, our goal here is the early understanding of RG7916 in people who have previously received SMN2-targeted therapy. In this study we will evaluate RG7916 safety, tolerability and pharmacokinetics (how the body

handles RG7916) in people (aged 12-60 years) with Type 2 and 3 SMA who have previously taken part in the MOONFISH study (with RG7800), or in a study with another SMN2-targeted therapy, such as nusinersen. Everyone enrolled in JEWELFISH will be given RG7916; there is no placebo in this study.

You can read more about SUNFISH and FIREFISH on [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and <https://www.clinicaltrialsregister.eu/>. JEWELFISH will be posted on these sites in due course.

We will also be sharing more information about these studies at [www.pioneeringhealthcare.com/sma](http://www.pioneeringhealthcare.com/sma)

We look forward to updating you as the RG7916 programme moves forward.

If you have any questions or would like to schedule a telephone call to discuss this further please contact me at [sangeeta.jethwa@roche.com](mailto:sangeeta.jethwa@roche.com)

We wish you a very happy holiday season and all the best for 2017.

Best regards,

A handwritten signature in blue ink, appearing to be "Sangeeta Jethwa". The signature is stylized with loops and a long horizontal stroke extending to the right.

Sangeeta Jethwa, MD  
Patient Partnership Director, Rare Diseases  
Roche Pharma Research & Early Development  
Roche Innovation Centre Basel, Switzerland