



8 September 2016

Dear SMA community,

Roche, together with our collaboration partners PTC Therapeutics and the SMA Foundation, would like to provide you with an update on our SMN2 splicing modifier programme. Please note that this programme is separate to the Roche-led olesoxime programme.

In April 2015, the Moonfish clinical study investigating a compound known as RG7800 in people with SMA was placed on clinical hold. This was a precautionary measure after an unexpected eye finding was identified in an animal study. Concentrations of RG7800 tested in this animal study were higher than those received by any of the study participants in the Moonfish study. We can confirm that no safety issues have been identified in anyone who received RG7800 during the Moonfish study. At this time, further development of RG7800 remains on hold.

In January 2016, we began a clinical study in healthy individuals with RG7916, a second compound and investigational medicine for SMA. We are pleased to share with you that based on information from this study, we are moving RG7916 forwards in development. We are planning to begin clinical studies of RG7916 in people with SMA Type 1 and Type 2/3 by the end of 2016 and are awaiting Health Authority feedback on our studies. This feedback will determine the final countries, sites and timelines for the studies and we will provide an update in due course.

Patients who participated in the Moonfish study will have the opportunity to enrol in an open label study with RG7916, if they fulfil study criteria and wish to participate. These patients will be contacted by their clinical trial site to update them on this development.

If you would like to read more about these studies and stay updated on their progress, please visit <https://www.clinicaltrialsregister.eu> or <https://clinicaltrials.gov>. Our studies with RG7916 are scheduled to be posted on these websites very soon.

Our commitment to helping the SMA community remains strong as we continue to pursue new medicines for the treatment of SMA. We will update you on RG7916 and our SMA programme as appropriate.

Your participation in clinical studies is critical to advance the scientific understanding of SMA. In collaboration with our partners, PTC Therapeutics and the SMA Foundation, we would like to thank those of you who participate in our clinical studies.

If you have any questions, or would like to discuss further, please contact sangeeta.jethwa@roche.com.

Best regards,

A handwritten signature in blue ink, consisting of several 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