

Dear Spinal Muscular Atrophy Community Members,

We would like to update you on the clinical development of RG7800, an investigational SMN2 splicing modifier that is being studied as a potential treatment for spinal muscular atrophy (SMA). This compound is the result of a collaboration between Roche, PTC Therapeutics, and the SMA Foundation.

In November 2014, Roche initiated the Moonfish trial (Phase 1b/2a) based on supportive results from a trial in healthy volunteers and animal toxicology studies over 13 weeks. The primary objective of this trial is to investigate the safety and tolerability of RG7800, while also measuring the concentration of the compound in the body over time (pharmacokinetics) and the effect on the splicing of SMN2 mRNA and SMN protein production.

In parallel to the Moonfish trial, we have been investigating the effects of the long term use of RG7800 in animals. These animal studies are a standard requirement in the development of new medicines. In this study, we observed an unexpected safety finding in the eye of animals. As patient safety is of the utmost importance to us, we immediately suspended dosing in the Moonfish trial as a precautionary measure.

It is important to note that an internal review of safety data from all patients involved in the Moonfish trial has been conducted and no safety issues were identified. The eye finding in the animal study was observed at RG7800 concentrations above those explored in patients. We are now thoroughly assessing this animal study finding and will confirm the next steps for the Moonfish trial in the near future.

All patients enrolled in the Moonfish trial are being informed of this finding and will be followed up according to the study protocol. At the time of dosing suspension, the first cohort of the Moonfish trial had completed treatment and three patients had started receiving treatment in a second dose cohort. No emerging safety issues with RG7800 have been identified in the trial. We would like to stress that the decision to suspend dosing has been taken as a precautionary measure given how important patient safety is to us.

We would like to thank you for your ongoing support of our SMA programme. Roche, PTC Therapeutics, and the SMA Foundation are fully committed to developing treatments for SMA and we will keep you apprised of the next steps for Moonfish.

Best regards,

On behalf of the RG7800 development team,



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