



Dear SMA community members

We would like to share with you the status of the Moonfish trial.

Moonfish is studying RG7800, an investigational SMN2 splicing modifier that Roche is developing for the treatment of spinal muscular atrophy (SMA). This programme is in collaboration with the SMA Foundation and PTC Therapeutics.

As we shared earlier this year, an unexpected eye finding was observed in an animal study evaluating the long term safety of RG7800. As a precaution, Roche suspended dosing in the Moonfish trial in April 2015. The U.S. Food and Drug Administration (FDA) agreed with our decision and action plan to further assess this pre-clinical safety finding, and placed the Moonfish trial on clinical hold. Clinical hold status means that we must inform and gain agreement from the U.S. FDA and the national Health Authorities in European countries before resuming the trial.

We have conducted a comprehensive review of safety information, with the assistance of independent scientific and clinical experts. This review identified no safety issues in any of the patients who were dosed with RG7800 in the trial and subsequently followed up for two months after completing treatment.

As patient safety is of the utmost importance, we continue to thoroughly assess the safety finding in ongoing animal studies. At this time, we cannot yet draw a final conclusion regarding the pre-clinical safety finding or a potential restart of the trial.

We remain fully committed to developing treatments for SMA and supporting the SMA community. We will provide you with an update on the Moonfish trial and our SMN2 splicing modifier programme by the end of this year, at the latest.

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

Best regards

A handwritten signature in blue ink, appearing to read "Sangeeta Jethwa".

Sangeeta Jethwa, MD
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