22 December 2015

Dear SMA community

The collaboration between Roche, PTC Therapeutics and the SMA Foundation would like to provide you with an update on the advance of our SMN2 splicing modifier programme. This programme is separate to the Roche-led olesoxime programme.

In April 2015, we shared with you that the Moonfish clinical study, investigating SMN2 splicing modifier RG7800 in people with SMA, was placed on clinical hold. This was a precautionary measure after an unexpected safety finding was identified in an animal study exploring long term treatment with RG7800 at exposures above those observed in the Moonfish study. The Moonfish study remains on hold as we continue investigations to understand these findings.

We are pleased to share with you that we have moved forward with development of a second SMN2 splicing modifier. This new investigational medicine, RG7916, will now advance to its very first clinical study in healthy individuals in January 2016, which will provide important information about the safety profile, pharmacokinetics (what the body does to the medicine) and effects of RG7916 in healthy individuals. RG7916 has different characteristics relative to RG7800, which may influence how the medicine interacts with the body and this will be evaluated in the study.

We have received questions on the inclusion of Itraconazole in this study. Itraconazole is an approved medicine which will be used as a tool to help understand the metabolism, or break down, of RG7916 in humans. This is common practice for this type of study.

Please visit https://www.clinicaltrialsregister.eu/about.html or https://clinicaltrials.gov/ct2/show/NCT02633709?term=SMA+RG7916&rank=1 if you would like to read more about this clinical study.

Information from this study will help us to compare the two SMN2 splicing modifiers RG7800 and RG7916 and decide how the programme advances further. We look forward to providing you with an update about this in the first half of 2016.

Our commitment to helping the SMA community remains strong as we continue to pursue new medicines for the treatment of SMA.

In collaboration with our partners, PTC Therapeutics and the SMA Foundation, we would like to thank you for sharing your experience of living with SMA; you inspire us every day.

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

With kind regards,

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