30 May 2018

Dear members of the SMA Community

We would like to provide you with an update about olesoxime, an investigational molecule in SMA.

Since we bought olesoxime from Trophos in 2015, we have had many difficulties in developing this molecule for people with SMA. These difficulties have focused on the formulation (the actual liquid preparation of olesoxime), the most appropriate dose to be given and requests from Health Authorities (FDA and EMA) to run a new Phase 3 study.

Over the last few years, treatment options in SMA have also changed dramatically. The emergence of effective treatment has raised the hurdle for how effective a new treatment needs to be, and this has an impact on how we design and run our clinical studies.

We understand the urgency of finding solutions for the SMA community and we have always tried to overcome the difficulties with olesoxime, in the hope of starting a Phase 3 study later this year.

In 2016 we started the OLEOS study, an open label extension study evaluating the long term safety and effectiveness of olesoxime. We had many discussions with Health Authorities (FDA and EMA) and SMA experts, worked to improve the dose and formulation of olesoxime and tried to design the best Phase 3 study possible to be able to show the efficacy of olesoxime.

In addition to this, we have also regularly analysed the data from the ongoing OLEOS study. Whilst the data at 12 months of treatment with olesoxime were initially encouraging, the most recent analysis at 18 months, which was presented at the American Academy of Neurology in April 2018, actually showed a worsening in motor function.

Unfortunately, despite all of our efforts and a strong desire to deliver olesoxime as a medicine to people with SMA, we have concluded that this is not going to be possible. Based on all of the available evidence and the continued difficulties described above, we have decided to stop further development of olesoxime.

Many of you will be very disappointed by this news, as we are too. Our immediate priority is to ensure that those still taking part in the ongoing OLEOS study understand what this decision means for them and that they are provided with appropriate treatment options.

We are working closely with study sites and investigators to help identify options for those still taking part in the OLEOS study and will share more details about this in the coming weeks. We will keep the OLEOS study open until all ongoing participants have an alternative treatment option confirmed.

We remain strongly committed to the SMA community, with the development of RG7916, our oral SMN2 splicing modifier, developed in collaboration with PTC Therapeutics and SMA Foundation. RG7916 is being studied in the ongoing SUNFISH, FIREFISH and JEWELFISH clinical studies. You can find further information about RG7916 on www.clinicaltrials.gov (search for SUNFISH, FIREFISH, JEWELFISH), www.clinicaltrialsregister.eu and on www.roche-sma-clinicaltrials.com

Finally and most importantly, we would like to acknowledge the tremendous contribution of the families who have participated in the olesoxime clinical studies and the entire SMA patient community who inspire us on a daily basis.

Please contact me if you would like to discuss this in more detail at Sangeeta.jethwa@roche.com

Best regards

Sangeeta Jethwa, MD, on behalf of the Roche SMA Team
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