September 2nd 2019

Dear Board members of SMA Europe,

In response to your request for information regarding Biogen’s ongoing trials in spinal muscular atrophy (SMA). We are pleased to share with you information regarding the recently presented results of the NURTURE study. The NURTURE study is an ongoing Phase 2, open-label study of 25 pre-symptomatic patients with SMA who received their first dosing before six weeks old.

In the study, nusinersen was administered to infants six weeks old or younger (n=25), who were in the pre-symptomatic stage, genetically-diagnosed with SMA and likely to clinically develop Type 1 or 2.

The updated results, were formally presented at the Cure SMA Annual SMA Conference in Anaheim, CA (June 28-July 1, 2019) and the 5th Congress of the European Academy of Neurology (EAN) in Oslo, Norway (June 29-July 2, 2019).

All study participants were alive, without a need for permanent ventilation, and were sitting independently, in comparison to the natural history of this disease where no patients with SMA Type 1 would be able to do so and patients with SMA Type 2 would need assistance. Overall, the study showed that nusinersen was well-tolerated with no safety concerns identified after up to nearly four years of treatment.

As we continue the NURTURE study, it should be noted that these interim results add to the longest study of SMA pre-symptomatic infants, after up to 45.1 months of analysis, nusinersen continues to demonstrate efficacy and safety in comparison to the natural history of SMA. The study median age was nearly three years old, with 88 percent of the study participants walking independently, with many of them doing so in the normal timeframe for an infant. As the community will be aware, in the natural history of SMA, patients with SMA Type 1 or Type 2 are never able to walk independently. This new data shows that patients treated with nusinersen had continuous improvement in sitting and walking independently, with the overwhelming majority of patients achieving motor milestones in a normal timeframe.

We would like to thank all the SMA families, caregivers and investigators, who continue to help us collect this vital data and information. We would also like to thank SMA Europe for their continued partnership.

As a team we remain a dedicated committed partner to this community and will continue to be available to provide updates in the future, when requested.

Best regards,

The Biogen SMA Team