

September 18th, 2019

Dear Board members of SMA Europe,

In response to your request for information regarding Biogen's ongoing clinical development programs in spinal muscular atrophy (SMA), we are pleased to share with you information regarding the new global clinical trial DEVOTE. The study will evaluate if higher dosing of nusinersen can provide improved efficacy in the treatment of SMA across a broad population of patients.

The DEVOTE study was announced publicly via press release at the 13th Congress of the European Paediatric Neurology Society (EPNS), in Athens.

The study aims to further build on the long-term safety profile and efficacy of nusinersen in a projected enrollment of over 100 patients of all ages, including adults. It will examine the potential for improved efficacy, as well as the safety and tolerability, when administered at a higher dose. DEVOTE will be a Phase 2/3 randomized, controlled dose-escalating study that will be commenced at 50 sites around the world.

The three-part trial will include:

- An open-label safety evaluation.
- A pivotal, double-blind, active control randomized treatment period.
- Followed by an open-label treatment period.
- More information on the trial is available at (<https://clinicaltrials.gov/ct2/show/NCT04089566>)

After the safety evaluation, the trial will have two loading dosing arms of 50mg followed by a maintenance dose of 28mg every four months, this will be in direct comparison with the current approved dosing which uses four loading doses with 12mg maintenance every four months. The third part of the trial will be an open-label evaluation to determine how to safely and efficiently transition patients from the currently approved dose to the higher dose being studied in DEVOTE.

We are very excited for the new study program and would like to thank all the SMA families, caregivers and investigators, who will help us collect this vital data and information.

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. We would also like to thank SMA Europe for their continued partnership.

Best regards,

The Biogen SMA Team