

4 September 2024

Dear SMA Europe,

In response to your request, we are pleased to provide an update on Biogen's Phase 2/3 DEVOTE study evaluating the safety and efficacy of a higher dose regimen of nusinersen for the treatment of spinal muscular atrophy (SMA).

DEVOTE is a three-part study that enrolled 145 patients across all ages and SMA types. In Part B, the pivotal cohort of DEVOTE, treatment-naïve children with infantile-onset SMA (n=75) were randomized 2:1 to receive the investigational higher dose regimen of nusinersen - *which comprises a more rapid loading regimen, two 50 mg doses 14 days apart, and a higher maintenance regimen of 28 mg every four months* - or the currently available nusinersen 12 mg regimen.

Today Biogen announced topline results from Part B of DEVOTE:

- The study met its primary endpoint, achieving a statistically significant improvement in motor function, assessed using the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), from baseline to six months for the higher dose cohort as compared to a matched untreated control group from the Phase 3 ENDEAR study;
- Results favored the higher dose regimen relative to the untreated control group across secondary endpoints and trended in favor of the higher dose regimen over the currently available 12 mg regimen among key biomarker and efficacy measures; and
- The higher dose regimen was generally well tolerated with reported adverse events generally consistent with SMA and the known safety profile of nusinersen.

Detailed results from DEVOTE will be presented at upcoming medical conferences, and Biogen plans to submit for regulatory approval of this investigational dose regimen.

Biogen is committed to supporting individuals with SMA and their families by advancing research that aims to answer critical questions for the community. We are deeply appreciative of the patients, caregivers, and investigators who have participated in the DEVOTE study and recognize the vital role they play in advancing SMA research. In addition, we thank the SMA community for your efforts to support those living with the condition.

We are happy to answer any questions you may have regarding the DEVOTE study and are available to provide updates in the future upon request.

Kind Regards,  
Cindy

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