



8 March 2024

Dear SMA Europe,

In response to your request, we are pleased to share a summary of the SPINRAZA® (nusinersen) data that were presented at the 2024 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference, which took place in Orlando, Florida this week. Please note that these data from the RESPOND study will also be presented at your 4th International Congress on Spinal Muscular Atrophy (SMA) next week.

About the RESPOND Study: *Treatment with nusinersen following gene therapy*

The open-label Phase 4 RESPOND study evaluates clinical outcomes and safety following treatment with nusinersen over a two-year period in infants and toddlers with SMA who have unmet clinical needs after treatment with onasemnogene abeparvovec.

Summary of New Interim RESPOND Data: *Reduced neurofilament light chain levels*

At the MDA conference, Biogen presented interim six-month biomarker data from the initial 29 participants in the RESPOND study. The new data show that plasma neurofilament light chain (NfL) levels, an objective biomarker of axonal injury and neurodegeneration, were reduced in nearly all study participants treated with nusinersen for six months.

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

We thank the SMA community for your efforts to support those living with the condition.

Regards,
Cindy

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