

18 July 2023

Dear SMA Community,

In response to your request for information on data presented at the 2023 Cure SMA annual conference, we are pleased to share updates on the following:

- Interim results from the RESPOND study* showed that most participants with suboptimal motor function at baseline improved at six months following treatment with nusinersen. Patients in the study had previously received gene therapy with onasemnogene abeparvovec.
 - No serious side effects were considered related to nusinersen or led to study withdrawal. No new emerging safety concerns have been identified in enrolled participants who received nusinersen after onasemnogene abeparvovec.
 - RESPOND is an ongoing, phase 4, multicenter, single-arm, open-label study to evaluate clinical outcomes and safety following treatment with nusinersen in infants and toddlers with SMA who have unmet clinical needs after treatment with onasemnogene abeparvovec.
 - Changes in motor function were measured using the Hammersmith Infant Neurological Examination Section 2 (HINE-2). The HINE is a simple, scoreable, standardized clinical neurological examination for infants between two and 24 months and section 2 is dedicated to motor function skills.

- A literature review and meta-analysis** of clinical trials and real-world studies undertaken by Biogen evaluating the effectiveness of nusinersen on infantile-onset SMA patients found that the improvements in motor function and motor milestones observed in those receiving nusinersen in real-world studies were greater than or comparable to those observed in clinical trials, and that patients continued to improve with longer duration of nusinersen treatment.

Biogen recognizes the SMA community's interest in the evolving SMA treatment landscape and will continue to share updates upon request.

Please contact us if you have further questions.

Best regards,

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Director Patient Advocacy SMA, Europe/Canada/Partner Markets, Biogen

References :

*Interim Results From the Ongoing RESPOND Study Evaluating Nusinersen in Children With Spinal Muscular Atrophy Previously Treated With Onasemnogene Apeparvovec-
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**Effectiveness of Nusinersen in Individuals With Infantile-onset Spinal Muscular Atrophy: A Systematic Literature Review and Meta-Analysis of Clinical Trials and Real-world Studies
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