IONIS InBrief
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New data reveal residual unmet needs in young SMA patients treated with gene therapy and suggest further potential of using SPINRAZA®

Ionis' partner Biogen recently announced new data from clinical studies aimed at assessing remaining unmet needs for people living with spinal muscular atrophy (SMA) and evaluating the potential impact of SPINRAZA® (nusinersen) in different patient populations.

Growing enrollment in the RESPOND study indicates there are residual unmet clinical needs in infants and toddlers with SMA following treatment with Zolgensma® (onasemnogene abeparvovec). The Phase 4 study is evaluating the clinical benefit and safety of SPINRAZA in infants and toddlers with SMA who have unmet needs following treatment with the gene therapy.

The 16 patients enrolled in RESPOND reported suboptimal clinical status across a variety of measures at baseline, with 13 of 16 showing this in multiple areas, including motor and respiratory functions and swallowing/feeding ability. After beginning SPINRAZA treatment, initial safety findings show three participants experienced a serious adverse event (AE) during the study period; none of these events were considered related to SPINRAZA treatment. The RESPOND study (NCT04488133) is currently enrolling participants at 20 sites worldwide; more information about the eligibility criteria is available at clinicaltrials.gov.

KEY TAKEAWAY

"Ongoing research to address unmet needs within the SMA community suggest further potential of using SPINRAZA®.

Biogen also announced final data from Part A of the ongoing, three-part DEVOTE study evaluating the safety and tolerability of investigational, higher doses of SPINRAZA®. Results from Part A, an open-label safety evaluation period in children and teens with later-onset SMA, suggest that a higher dosing regimen of nusinersen leads to higher levels of the drug in the cerebrospinal fluid and is generally well-tolerated, with most AEs reported considered to be mild in severity. The most common AEs reported were headache and procedural pain. Two serious AEs (fall, femur fracture) were reported in one participant during the study period. No AEs were considered related to nusinersen and some were related to treatment administration. The totality of Part A data supports further development of a higher dose of nusinersen.

Currently, Part B and Part C of DEVOTE evaluating an investigational, higher dose of nusinersen are enrolling at 52 sites worldwide. Information on the DEVOTE trial (NCT04089566) is available at clinicaltrials.gov.

**Nusinersen is currently commercialized under the brand name SPINRAZA® and the U.S. Food and Drug Administration-approved dose is 12 mg. As a foundation of care in SMA, more than 13,000 individuals have been treated with SPINRAZA worldwide.[1]*

Reference:

1. Based on commercial patients, early access patients, and clinical trial participants through March 31, 2022.