SMA Highlights: Updates from



Dear SMA Community,

It was so wonderful to meet with many of you at Cure SMA's Annual Conference in June – the first in-person event since 2019. We wish to congratulate Cure SMA on organizing a successful event which brought together more than 2,300 community members from more than 30 different countries, both records for the event.

We were also excited to present updates from our SPINRAZA® (nusinersen) clinical development program during the research and clinical care meetings; more information below.

We look forward to continuing to engage with the SMA community throughout 2022 at community education and fundraising events both online and around the United States.

- The Biogen Team

GREAT TO SEE YOU!



Thank you to everyone who stopped by our booth to share your SMA stories or attend our symposia, 'Perspectives on SMA: Information and Personal Stories on Presymptomatic and Later Onset SMA.' Or perhaps you chatted with us at one of our two posters during the Family-Friendly Poster Sessions.

It was great to see and hear from you during Cure SMA's Annual Conference!

PUBLICATIONS AND DATA UPDATES

During the Cure SMA 2022 Research & Clinical Care Meeting, Biogen was pleased to make available:

- Updated preliminary safety data from Phase 4
 RESPOND study on the impact of SPINRAZA in infants
 and toddlers who previously received gene therapy¹
- Final Part A DEVOTE data which continue to support the development of new therapeutic approaches with nusinersen in children and teens with later-onset SMA²
- Additional data on the long-term NURTURE study regarding the percentage of participants who attained motor milestones as assessed by World Health Organization (WHO) criteria³

SMA CLINICAL TRIAL PROGRAM STATUS*

- Clinical Trials Ongoing Around the World
- 7 U.S. Enrollment Sites Open for <u>DEVOTE</u>
- 12 U.S. Enrollment Sites Open for RESPOND
 - 7 U.S. Enrollment Sites Open for ASCEND

*As of July 15, 2022

INDICATION

SPINRAZA® (nusinersen) is a prescription medicine used to treat spinal muscular atrophy (SMA) in pediatric and adult patients.

SELECTED IMPORTANT SAFETY INFORMATION

Increased risk of bleeding complications has been observed after administration of similar medicines. Your healthcare provider should perform blood tests before you start treatment with SPINRAZA and before each dose to monitor for signs of these risks. Seek medical attention if unexpected bleeding occurs.

Please see Important Safety Information continued on page 2 and click here to access full <u>Prescribing Information</u>.

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Increased risk of kidney damage, including potentially fatal acute inflammation of the kidney, has been observed after administration of similar medicines. Your healthcare provider should perform urine testing before you start treatment with SPINRAZA and before each dose to monitor for signs of this risk.

The most common side effects of SPINRAZA include lower respiratory infection, fever, constipation, headache, vomiting, back pain, and post-lumbar puncture syndrome.

These are not all of the possible side effects of SPINRAZA. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Before taking SPINRAZA, tell your healthcare provider if you are pregnant or plan to become pregnant.

Click here to access full Prescribing Information.

This information is not intended to replace discussions with your healthcare provider.

REFERENCES

- 1. Proud C, et al. Baseline characteristics and initial safety results in RESPOND: A phase 4 study of nusinersen in children with SMA who received onasemnogene abeparvovec. 2022 Cure SMA Conference, Anaheim, CA, United States. June 16-19, 2022, oral presentation.
- 2. Finkel RS, et al. Results from the end of Part A of the ongoing 3 part DEVOTE study to explore higher doses of nusinersen in SMA. 2022 Cure SMA Conference, Anaheim, CA, United States. June 16-19, 2022, oral presentation.
- 3. Kuntz N, et al. Nusinersen Effect in Infants in the Presymptomatic Stage of SMA: 4.9-Year Interim of the NURTURE Study. 2022 Cure SMA Conference, Anaheim, CA, United States. June 16-19, 2022, oral presentation.

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