SMA Highlights: Updates from Biogen

Dear SMA Community,

We are pleased to share a new format for Biogen's ongoing updates for the community. We hope this layout allows you to more easily find updates and information related to Biogen's clinical research, community outreach and SPINRAZA® (nusinersen) programs. We look forward to hearing your feedback as well.

The upcoming 2021 Virtual SMA Conference organized by Cure SMA is always a highlight for our team. We are excited to connect with the SMA community at our virtual booth and during our live Family Symposium. Also, we look forward to sharing updates and outcomes from exciting research with healthcare providers and researchers through several posters and talks at the 2021 Virtual SMA Research & Clinical Care Meeting.

– The Biogen Team

SPINRAZA PROGRAM UPDATES

95 **Support and Treatment Education Program (STEP) events in 2021**

3700 **Adults treated with SPINRAZA out of 11,000+ people treated worldwide***

58 **Countries where SPINRAZA is approved**

*Based on commercial patients, early access patients and clinical trial participants through December 2020

PUBLICATIONS AND DATA UPDATES

Earlier this year, Biogen presented new data from the SPINRAZA clinical development program at the **American Academy of Neurology (AAN) annual meeting**.

- Key highlights included results reinforcing the potential of **neurofilament levels as a biomarker** for treatment response in SMA. Researchers analyzed blood samples collected from 75 people with SMA enrolled in the CHERISH/SHINE studies.[1]
- The use of biomarkers could improve the understanding of disease mechanisms and interventions for SMA and other diseases. Biogen has included the measurement as an exploratory endpoint in its **DEVOTE** and **RESPOND** studies.

Earlier this year, researchers from Stanford University published a paper in *Neurology Clinical Practice* titled, **Nusinersen Treatment in Adults with Spinal Muscular Atrophy**.[2] This paper adds important new information to the already substantial body of independent, observational studies (real-world evidence) describing clinical outcomes of SPINRAZA use in adults with SMA as reported in at least 10 other publications.[3-12]

SMA CLINICAL TRIAL PROGRAM STATUS†

7 **Global Clinical Trials Ongoing**

5 **U.S. Enrollment Sites Open for DEVOTE**

7 **U.S. Enrollment Sites Open for RESPOND**

† As of 6/1/21

INDICATION

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

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 Prescribing Information.

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IMPORTANT SAFETY INFORMATION (cont'd)

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. Farrar MA et al. Baseline Plasma Phosphorylated Neurofilament Heavy Chain (pNF-H) Level Is Associated With Future Motor Function in Nusinersen-treated Individuals With Later-onset Spinal Muscular Atrophy (SMA). Presented at the AAN 2021 Virtual Annual Meeting, Presentation S13.005.
2. Duong T et al. Nusinersen Treatment in Adults with Spinal Muscular Atrophy. Neurol Clin Prac 2021; Online Ahead of Print. doi: 10.1212/CPJ.0000000000001033.
3. Hagenacker T et al. Nusinersen in adults with 5q spinal muscular atrophy: a non-interventional, multicentre, observational cohort study. Lancet Neurol. 2020 Apr;19(4):317-325. doi: 10.1016/S1474-4422(20)30037-5. Epub 2020 Mar 18.
4. Maggi L et al. Nusinersen safety and effects on motor function in adult spinal muscular atrophy type 2 and 3.Neurol Neurosurg Psychiatry. 2020 Sep 11:jnnp-2020-323822. doi: 10.1136/jnnp-2020-323822. Epub ahead of print. PMID: 32917822.
5. Veerapandiyan A et al. Nusinersen for older patients with spinal muscular atrophy: A real-world clinical setting experience. Muscle Nerve. 2020;61(2):222-226. doi:10.1002/mus.26769.
6. Yeo CJJ et al. Prospective Cohort Study of Nusinersen Treatment in Adults with Spinal Muscular Atrophy. J Neuromuscul Dis. 2020;7(3):257-268. doi:10.3233/JND-190453.
7. Lam K, Wu A. Clinical Outcome of Adult Spinal Muscular Atrophy Patients Treated with Nusinersen: A Case Series Review. Perm J. 2020;25:1. doi:10.7812/TPP/20.163
8. Konersman CG et al. Nusinersen treatment of older children and adults with spinal muscular atrophy. Neuromuscul Disord. 2021 Mar;31(3):183-193. doi: 10.1016/j.nmd.2020.12.006. Epub 2020 Dec 20.
9. Osmanovic A et al. Treatment expectations and patient-reported outcomes of nusinersen therapy in adult spinal muscular atrophy. J Neurol. 2020 Aug;267(8):2398-2407. doi: 10.1007/s00415-020-09847-8. Epub 2020 May 2.
10. Walter MC et al. Safety and Treatment Effects of Nusinersen in Longstanding Adult 5q-SMA Type 3 - A Prospective Observational Study. J Neuromuscul Dis. 2019;6(4):453-465. doi: 10.3233/JND-190416.
11. Elsheikh B et al. Safety, Tolerability, and Effect of Nusinersen in Ambulatory Adults With 5q-SMA. Front Neurol. 2021 May 20;12:650535. doi: 10.3389/fneur.2021.650535.
12. Elsheikh B et al. Safety, Tolerability, and Effect of Nusinersen in Non-ambulatory Adults With Spinal Muscular Atrophy. Front Neurol. 2021 Apr 16;12:650532. doi: 10.3389/fneur.2021.650532.

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