

## **Biogen Community Q&A**

### **What does this approval mean?**

The FDA has approved SPINRAZA™ (nusinersen) in the U.S. as a treatment for SMA in pediatric and adult patients. The FDA approved SPINRAZA based on data from multiple clinical studies which included more than 170 individuals with SMA. The data package included the interim analysis of ENDEAR, a Phase 3 controlled study evaluating SPINRAZA in infantile-onset, as well as open-label data from uncontrolled studies in pre-symptomatic and symptomatic patients with, or likely to develop, Types 1, 2 and 3 SMA.

In ENDEAR, a pivotal controlled clinical study, infantile-onset SMA patients treated with SPINRAZA achieved and sustained clinically meaningful improvement in motor function compared to untreated study participants. In addition, a greater percentage of patients on SPINRAZA survived compared to untreated patients. In open-label uncontrolled studies, some patients achieved milestones such as ability to sit unassisted, stand or walk when they would otherwise be unexpected to do so and maintained milestones at ages when they would be expected to be lost. The overall findings of these studies support the effectiveness of SPINRAZA across the range of SMA patients, and appear to support the early initiation of treatment.

Although the indication in the US Prescribing Information (USPI) is not limited to any specific type of SMA, the USPI highlights efficacy and safety data from the sham-controlled ENDEAR study, and states that results of ENDEAR were supported by open-label uncontrolled trials conducted in symptomatic patients with SMA and in pre-symptomatic patients (with or likely to develop Type I, II, or III SMA). There is no data on the efficacy and safety of nusinersen in individuals with Type 0 or Type IV SMA included in the USPI.

### **When will SPINRAZA™ (nusinersen) be available?**

SPINRAZA will be made available for shipment in the U.S. to healthcare providers or institutions in approximately one week. Biogen has developed an extensive plan with the goal of providing a continuous and sufficient supply of SPINRAZA to support the potential demand. However, as with any new treatment, we expect there will be variation across institutions and treatment centers as to when clinicians can begin scheduling and dosing individuals.

### **Will insurance cover SPINRAZA? How will I navigate insurance reimbursement?**

Insurance coverage varies from plan to plan and is dependent on the individual-specific insurance policy. However, Biogen recognizes that insurance coverage of SPINRAZA is a key consideration for families. As part of our ongoing commitment to the SMA community, Biogen has created a support services program in the U.S. called SMA360°™\*. SMA360° support provides certain services that address non-medical barriers to access. These include: logistical assistance, product education, insurance benefits investigations, and financial assistance. A complete list of the SMA360° offerings can be found at [www.spinraza.com](http://www.spinraza.com) in the coming days.

### **Who will be eligible for financial assistance programs in the U.S.?**

Each individual's eligibility for Biogen's financial assistance will be dependent on a number of factors. For this reason, we suggest you contact an SMA Support Coordinator at 1-844-4SPINRAZA (1-844-477-4672) 8:30am-8:00pm EST to get more information about these services.

**What is the price of SPINRAZA?**

The price of SPINRAZA is not yet available. Biogen is committed to helping individuals who may benefit from our therapies obtain access to them; our goal is for no one to forgo treatment based on financial limitations or insurance status.

**Is the cost of SPINRAZA dependent upon individual weight?**

No, SPINRAZA is not a weight-based medication.

**What will happen to individuals participating in SHINE or other ongoing studies?**

Biogen is in the process of transitioning all participants in the clinical trials into open-label studies where they will have an opportunity to receive SPINRAZA. Individuals in any study **other than** EMBRACE or NURTURE will enter SHINE to receive SPINRAZA and continued follow-up. Individuals who are participating in EMBRACE and NURTURE will continue to receive SPINRAZA and assessments within the extended follow-up phase of these studies.

Biogen intends to continue the SHINE, EMBRACE and NURTURE studies in order to follow individuals in a clinical study setting and continue to collect important data to better understand the long-term safety and efficacy of SPINRAZA. While we have not yet determined the exact timing of how long these studies will run, we believe these are very important studies and we hope that all of the individuals involved will continue their participation.

While individuals are participating in SHINE, EMBRACE or NURTURE, Biogen will continue to cover the costs of SPINRAZA and associated study costs.

The health and well-being of individuals in the clinical trials remains our top priority. We are extremely grateful to the families who have been involved in the SPINRAZA development program and thank them for their continued participation.

**What will happen to individuals currently enrolled in the Expanded Access Program (EAP) in the U.S.?**

The Expanded Access Program (EAP) for nusinersen will close in the U.S. to new individual enrollment now that the FDA has approved the New Drug Application (NDA) for nusinersen.

Individual clinicians and the team at Biogen will assist individuals currently participating in the EAP in the U.S. to transition to the approved therapy. For more information, families currently enrolled in the EAP should contact an SMA Support Coordinator at 1-844-4SPINRAZA ([1-844-477-4672](tel:1-844-477-4672)).

\*SMA360° services from Biogen are available only to those who have been prescribed SPINRAZA in the U.S.

**Indication**

SPINRAZA 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 at baseline and before each dose of SPINRAZA to monitor for early signs of these risks. Seek medical attention if unexpected bleeding occurs.

**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 at baseline and before each dose of SPINRAZA to monitor for early signs of this risk.

**The most common side effects of SPINRAZA include** upper and lower respiratory tract infections, complete or partial collapse of a lung or lobe of a lung, constipation, headache, 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.

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

Please see full [Prescribing Information](#) for additional important safety information.