

Dear members of the SMA community,

In response to your request for information, we are delighted to inform you that today the European Commission (EC) has granted a marketing authorization for SPINRAZA<sup>®</sup> (nusinersen) for the treatment of 5q spinal muscular atrophy (SMA) which refers to the most common form of the disease and represents approximately 95% of all SMA cases. Nusinersen, the first approved treatment in the European Union (EU) for SMA, was reviewed under the European Medicine Agency's accelerated assessment program, intended to expedite access to patients with unmet medical needs.

This approval was primarily based on data from two pivotal controlled studies, ENDEAR (infantile-onset SMA; individuals most likely to develop Type 1 SMA) and CHERISH (later-onset SMA; individuals most likely to develop Type 2 or 3 SMA), which both demonstrated the clinically meaningful efficacy and favorable benefit-risk profile of nusinersen. In addition, data from open-label studies in pre-symptomatic and symptomatic individuals who have or are likely to develop Types 1, 2 or 3 SMA were consistent with the results of the pivotal studies and were considered supportive of the approved indication. The overall findings of these studies support the efficacy and safety of nusinersen across a range of individuals with SMA and appear to support early initiation of treatment.

In September 2016, in response to the urgent need for the treatment of the most severely affected SMA patients, Biogen launched one of the largest pre-approval, Expanded Access Programmes (EAP) in rare disease, free of charge. The Biogen sponsored EAP includes eligible individuals with infantile-onset SMA (most likely to develop Type 1) and has led to the treatment of more than 350 children in 17 European countries.

Biogen recognizes that cost and access to treatments are key considerations for patients, health care professionals, payers, and policy makers. Despite marketing authorization, the timing to the treatment availability will vary based on the access and reimbursement pathway established in each country. Biogen is working with health systems and government agencies across the EU to secure broad access to nusinersen as soon as possible. To minimize the gap between EC authorization and access to nusinersen, the EAP in Europe will remain open for new patients for a period of time following marketing authorization, if allowed per local regulations. The actual date of closure for new patients will be defined by Biogen on a country by country basis. All patients enrolled in the EAP will continue to receive the medicine free of charge, until local access agreements are completed. For further information, please reach out to your treating physician.

Thank you for the support we consistently receive from the entire SMA community. We would not have reached this historic moment without you and your families, the physicians and

researchers who have supported the nusinersen program. This important milestone brings new hope to patients, families and the SMA community and reflects our commitment to improving the lives of patients with serious neurological diseases. Our commitment to the SMA community remains steadfast. We still have much work to do and we will continue to work tirelessly to ensure that patients who may benefit from nusinersen will receive access as quickly as possible, now that it has been approved in the EU.

Sincerely,

Biogen