Dear members of the SMA community,

In response to your request for information, we are delighted to inform you that today the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending the granting of a marketing authorization for SPINRAZA® (nusinersen) to treat patients with spinal muscular atrophy (SMA). The positive CHMP opinion, which was reviewed under the accelerated assessment program, and completed ahead of anticipated timelines, recognizes the compelling unmet need for an effective SMA treatment in Europe. Nusinersen is the first SMA treatment recommended by CHMP for approval in the European Union (EU).

The CHMP recommended an indication for the treatment of 5q SMA, which refers to the most common form of the disease and represents approximately 95% of all SMA cases. This recommendation was primarily based on data from two pivotal controlled studies, ENDEAR (infantile-onset SMA) and CHERISH (later-onset SMA), which both demonstrated the clinically meaningful efficacy and favorable safety 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 recommended indication. The overall findings of these studies support the efficacy and safety of nusinersen in individuals with SMA.

The CHMP positive opinion is now referred to the European Commission (EC), which grants marketing authorizations for centrally authorized medicines in the EU. A decision from the EC is expected within the next few months. The final decision will be applicable to all 28 EU member countries plus Iceland, Norway and Liechtenstein.

Subject to approval by the EC, access to the treatment will vary between countries based on the access and reimbursement pathway established in each country. Biogen recognizes that cost and access to treatments are key considerations for patients, providers, payers, and policy makers and we are already working with national reimbursement authorities across the EU to support the reimbursement and availability of nusinersen.

We are incredibly grateful to the entire SMA community for your continued support. We would not have reached this historic moment without you and your families, the physicians and researchers who have supported the nusinersen program.

We will continue to work closely with patient organizations and partner with healthcare systems to ensure that patients who may benefit from nusinersen will have access to therapy, once approved in the EU. We remain committed to transparent and timely communications and will continue to be available to provide any requested updates.

Sincerely,

Biogen