

Dear members of the SMA community,

In response to your request for information, we would like to provide you with an update on Biogen's global Expanded Access Program (EAP).

Based on the high unmet need in SMA, and to bridge the gap between the closure of the sham-controlled phase 3 ENDEAR study and regulatory approval across the EU+, Biogen is sponsoring an EAP for eligible individuals with infantile-onset SMA (most likely to develop Type 1). This program enables wide access to nusinersen, subject to applicable inclusion criteria and as permitted according to local laws and regulations, during the time before a potential regulatory approval.

Opening an EAP in infantile-onset SMA has taken longer than expected in many countries due to the operational complexities in opening and running an EAP in a previously untreated disease. Despite this, as the demand from individuals with infantile-onset SMA increased, and in response to requests from clinicians, we are currently further expanding the nusinersen infantile-onset EAP to multiple countries. This expansion is subject to local laws and regulations, where it can be operationalized and where there is a path to long-term availability and reimbursement of nusinersen. Even with this expansion, there are still many more infants to be treated.

Given these complexities and the anticipated accelerated marketing authorization in the EU, Biogen's global EAP will not extend beyond the infantile-onset (most likely to develop Type 1) SMA population. We will continue to focus on the urgent treatment of individuals with infantile-onset SMA, based on the severity and high risk of mortality and the ability for centers to meet the current demand from these patients. At the time of marketing authorization in Europe, governments will begin their reimbursement processes so that local citizens can gain access through reimbursement to nusinersen within their healthcare system.

In addition, clinical sites are working diligently to increase capacity and ensure infrastructure is in place to accommodate the current and future needs of individuals with SMA seeking treatment.

Patients currently enrolled in this EAP will remain in the program until local approval and access in their country is established. At that time, their treating physician will begin to transition patients enrolled in the EAP to the reimbursed medicine. Upon EU marketing authorization, changes to the EAP, including the ongoing enrollment of new patients or termination of the program, will be determined.

We understand the Biogen EAP will not meet the needs of the entire SMA community. Obtaining regulatory approval across the globe remains our number one priority, as we believe it is the best route to long-term sustainable access to nusinersen for the greater SMA community. We will continue to work with local regulatory agencies including the European Medicine Agency (EMA) to expedite approval. Subject to a positive Committee for Medicinal Products for Human Use (CHMP) opinion and approval by the European Commission (EC), access to the treatment will vary between countries based on the local reimbursement pathway. We are also deeply focused on collaborating with government agencies to accelerate reimbursement and broad access across Europe.

We are thankful to the entire SMA community for your continued support, and in particular, the individuals and families who have participated in our studies and supported the nusinersen program.

We remain committed to transparent and timely communications and will continue to be available to provide requested updates.

Sincerely,

Biogen