

SMA Community Update from Biogen UK/Ireland, as requested by SMA Trust and SMA Support on 16th June 2017 (and for reactive communication to EAP sites in UK)

Current status of EAP in the UK

As the SMA community is aware, due to the high unmet need, Biogen opened a pre-approval global expanded access programme (EAP) for eligible patients in infantile-onset SMA (consistent with Type 1) in Autumn 2016.

Following the recent marketing authorisation of nusinersen, Biogen understands there has been uncertainty within the SMA community on the impact marketing authorisation will have on the current status of the EAP and more specifically, any changes to the programme in the UK. As communicated recently, Biogen remains committed to keeping the EAP open in Europe for new patients for a period of time and dates of closure will be discussed and agreed on a country by country basis following reviews of local reimbursement processes and timelines. It is our intention to keep the EAP open for new patients for the remainder of the year in the UK as we continue to engage with NHS England on the EAP in England and with NICE on the reimbursement pathway for nusinersen.

To ensure appropriate and timely access to nusinersen, it is imperative that continued and meaningful progress is made on the reimbursement pathway. Biogen is working with all relevant stakeholders to enable a viable funding pathway and discussions with NICE, other HTA bodies and the NHS are ongoing. We will re-evaluate the progress of the reimbursement progress and the EAP towards the end of 2017. Biogen reserves the right to close the EAP for new patients at any point if challenges to access for patients persist or remain unresolved as the process progresses and evolves over time.

Importantly, children enrolled in the Biogen UK EAP will remain in the free of charge programme until local reimbursement and access is established. In the event of a negative reimbursement decision or where a patient falls outside of the reimbursement criteria, Biogen is committed to ensuring the patients still have access to nusinersen.

Biogen remains supportive of the ongoing efforts of members of the clinical and advocacy community who are actively addressing the EAP challenges already identified within individual hospitals.

To this end, Biogen continues to receive and assess EAP applications from individual hospitals within the process already established. As of July 11th 2017, the EAP is active in 7 sites in England, 2 in Scotland and 1 in Northern Ireland. Additional sites have been approved but are not yet active.