Biogen statement to SMA community re: EAP in England, Wales and Northern Ireland

As many within the spinal muscular atrophy (SMA) community are aware, Biogen opened the nusinersen global expanded access programme (EAP) in Autumn 2016. Biogen designed the EAP to provide an interim solution for patients with infantile onset SMA (consistent with type 1) between the end of the clinical trials and EMA approval, which was granted in May 2017. In the UK, it was extended further to support continued access for these patients until NICE had completed its appraisal. Two years on, we are extremely proud to have provided over 80 eligible children in the UK with nusinersen free of charge.

It has come to our attention that there has been some discussion within the SMA community in regard to the future of the EAP in the UK, and Biogen would like to take this opportunity to provide some background and clarity. As per the original planned timeframes provided by NICE - completion of the appraisal process and therefore final appraisal document (FAD) was scheduled for November 2018. Biogen agreed to extend the EAP in England, Wales and Northern Ireland in line with this timeframe, and the programme will be closed to new patients as of 1 November 2018.

While the EAP goes beyond what many companies offer, it was designed as a short-term solution, and we are aware that it has not met the needs of all patients with SMA, particularly type 2 and 3 patients where the clinical trial evidence indicates that there is potential benefit. It is therefore critical for the SMA community that a long-term and sustainable plan for access to nusinersen is established in the UK for all who may benefit. This can only be provided by the NHS (particularly NHS England), and the EAP should not be seen as a replacement for role of the health service. Biogen continues to remain focussed on working with NICE and NHS England to agree the terms of a managed access agreement ahead of 1 November. Biogen has committed to ensuring that patients already enrolled on the EAP by 1 November continue to receive access to nusinersen.

During the past weeks, we have informed relevant members of the clinical community (as well as patient advocacy groups) of this decision to allow clinicians to ensure that all eligible patients are enrolled onto the EAP ahead of 1 November. The programme in England, Wales and Northern Ireland will be the final EAP in Europe still accepting new patients ahead of November 2018. Other countries have either stopped enrolment or completed transition to access via the local health service, for example in Scotland.

Biogen is committed to working towards a sustainable solution, and we continue to collaborate with stakeholders from NICE and NHS England, as well as SMA Support UK, The SMA Trust, Muscular Dystrophy UK and TreatSMA, with the aim of finalising a managed access agreement ahead of November 2018. We urge that the terms be agreed by NICE and NHS England without further delay to ensure access for patients who may benefit from nusinersen, recognising that 20 countries across Europe have already provided access to nusinersen via their healthcare systems, and bringing an end to the uncertainty faced by patients in England, Wales and Northern Ireland.

Date of Preparation August 2018/ SPZ-PAN-0050

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