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

In the update to the spinal muscular atrophy (SMA) community in August this year, we set out the next steps for the expanded access programme (EAP) for nusinersen.

The EAP was designed as an interim solution between clinical trials and EMA approval, which arrived in May 2017. The UK's EAP opened in Autumn 2016 and was subsequently extended to ensure access for patients with infantile onset SMA (consistent with type 1) until NICE had completed its processes in November 2018.

In the summer we informed the community and clinicians that, in line with the original NICE timelines, the EAP would cease accepting new patients from 1st November. This remains the case.

The EAP is amongst the largest offered in paediatric rare disease. 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. 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 and the EAP should not be seen as a replacement for role of the health service.

Biogen's EAP in the UK has been the last in Europe accepting new patients. Other countries have either stopped enrolment or completed transition to access via the local health service, for example in Scotland.

The programme has provided over 80 eligible children in the UK with nusinersen free of charge and Biogen has committed to ensuring that patients already enrolled on the EAP by 1 November continue to receive access to nusinersen.

However, we recognise that the coming weeks will offer much uncertainty. We are committed to finding an urgent solution and will work with the community, clinicians, NICE, NHS England and the Government to deliver that. We have been clear with all stakeholders that we will consider a 'bridging solution'. This provides an option, once an agreement is imminent, for the medicine to be available before the logistics of NHS reimbursement have been finalised. This could ensure that those patients with an urgent need can still receive the medicine while the details are being finalised.

We urge NICE and NHSE to drive forward a solution without further delay. Providing access to this life-altering medicine is long overdue in England, Wales and NI, when compared to 22 other European countries, including Scotland.

ENDS

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