Biogen statement to SMA community re: NICE recommendation of Spinraza®▼ (nusinersen)

An agreement has been reached to recommend the funding of nusinersen on the NHS for the treatment of infants, children and adults with 5q spinal muscular atrophy (SMA), including pre-symptomatic and SMA types I, II and III. This decision is a momentous occasion for SMA patients, their families, and the patient organisations who support them, who have fought tirelessly for access to this treatment. It has been achieved through intensive and collaborative working between the SMA community, the National Institute for Health and Care Excellence (NICE), NHS England, and Biogen. Everyone involved should be applauded for their commitment to achieving a resolution.

The decision marks equality in access with 24 other countries in Europe, including Scotland, (and many more around the world) already funding nusinersen.[1]

Biogen will continue to work with health authorities to ensure this welcome decision translates into access as soon as possible for those awaiting treatment, which includes providing NHS England with access to nusinersen for type I patients immediately.

SMA patients currently receiving nusinersen on the expanded access programme (EAP)

Biogen opened the nusinersen global expanded access programme (EAP) in Autumn 2016, one of the largest in rare disease. Biogen designed the EAP to provide a temporary solution for patients with type I SMA between the end of the clinical trials and EMA approval in May 2017. In the UK, it was extended further to support continued access for these patients whilst the NICE process continued. By the time the programme closed to new patients in November 2018, we had provided over 80 eligible children in the UK with nusinersen free-of-charge. We will continue to meet our obligations to these patients until the new agreement with NICE and NHS England is in place. Once the agreement is in place and the NHS takes over the funding of type I SMA patients, there will be no interruption of treatment experienced by patients. Biogen will work with all stakeholders to ensure a smooth transition.

Access to nusinersen in Scotland

The Scottish Medicines Consortium (SMC) recommended the routine funding of nusinersen for the treatment of symptomatic type I SMA (infantile onset) in Scotland in May 2018. In February 2019, the SMC granted nusinersen ultra-orphan designation, allowing the medicine to be reviewed in SMA types II and III via the SMC's new, more flexible appraisal route for ultra-orphan disease medicines. This also enables type II and type III patients to access the medicine via the NHS whilst the review is underway. Prior to being assessed by the SMC's ultra-orphan pathway, types II and III patients had access to nusinersen via the SMC's Peer Approved Clinical System Tier 2 (PACS Tier 2) from May 2018.

References

1. Biogen. SMA Community Update. April 2019.