Biogen

Biogen UK SMA Community statement, 07 August 2019

Dear Liz, Gennadiy, Doug, Kacper, Robert, Clare and Jonathan,

As requested, please find below response to your enquiries regarding access to SPINRAZA®▼ (nusinersen) in the UK following the recently finalised Managed Access Agreement (MAA).

Biogen welcomes the finalisation of the MAA, which marks an important milestone for the spinal muscular atrophy (SMA) community. Securing access to nusinersen for infants, children, and adults with 5q SMA, including pre-symptomatic, SMA types I, II and III has been achieved through intensive and collaborative working between the SMA community, clinicians, NHS England (NHSE), NICE and Biogen.

We recognise the concerns of patients and families about some aspects of the MAA and have responded to your specific questions below:

Update on SMA Type III non-ambulant and generation of further data

Q: The loss of upper body strength, arm and hand movement has a huge impact on people's quality of life, the equipment care and support they need. Why to date hasn't this been a marker for the success of nusinersen in terms of both clinical and patient outcome measurements?

We agree with the SMA community that ambulation status is not the sole indicator of impact of SMA on individuals and we presented this argument during our negotiations. However, as the holders of NHS budget expenditure, NICE and NHSE have the final say on who is included. Whilst upper body data was included in the submission, at present, in the excluded groups of the MAA, they deemed the uncertainty too high for the evidence provided to recommend access for these groups.

The generation of data across all parts of the patient population in complex, rare diseases like SMA that cover a broad spectrum of paths and outcomes is challenging and an ongoing process. This is particularly true for a first-of-its-kind medicine such as nusinersen.

Biogen is focused on furthering our understanding of SMA and generating data in support of the clinical value of nusinersen, and this includes exploring additional outcome measures and markers of success. Biogen is currently funding more than 25 SMA research projects worldwide, 7% of which are focused on the adult / teenage population.[1]

We are working with major registries around the world, many of which contain treated type III patients. However, the data in these registries cannot yet demonstrate the evidence required by NHSE and NICE in these groups. Biogen will submit this data for review as soon as we believe it is sufficiently robust to be considered by NICE and NHSE.

Q: What steps is Biogen taking over what timescale to record and capture these additional Clinical and Patient Reported Outcome Measures for the children who will be included in the MAA?

The route to the best outcomes for patients living with SMA and on our therapy is through collaboration between all parties. We believe that a central registry in the UK, owned and managed by the clinical community, is the best way to ensure timely generation of high-quality data.

Biogen is committed to supporting SMA REACH for this purpose. We are grateful to patients, their families and clinical teams for their collaboration with the network and data generation efforts, and the government organisations supporting this approach.

The need for additional tools to precisely measure the impact of therapies on patients' lives has been recognised during the NICE appraisal process. We are committed to furthering this, and we are working with Strathclyde University to create a tool to measure the real impact of treatment on SMA patients – patient reported outcome measures (PROMs). We will be engaging the clinical and patient community to ensure the tool's suitability to generate the data required.

The PROMs will form a key aspect of data collected for the MAA and, once we have sufficient evidence, we will present our findings back to NICE / NHSE to continue to demonstrate the impact of nusinersen.

Q: What steps is Biogen taking over what timescale to record and capture these additional Clinical and Patient Reported Outcome Measures globally for children and adults?

Biogen will be generating additional data for all patient groups and furthering our understanding of how nusinersen helps people living with SMA. We are collaborating with multiple clinician-owned registries from round the world, as well as continuing our own clinical trial programs and supporting independent nusinersen research.

We are currently funding more than 25 SMA research projects worldwide to advance our understanding of the condition, including the following areas: registry, newborn screening, adults with SMA, the natural history of SMA (how the disease progresses when not treated), quality of life (measuring change in the real lives of patients), biomarkers (chemical / laboratory test measures of SMA disease severity) and treatment administration in scoliotic (twisted) spines.[1]

Qu: What steps is Biogen taking over what timescale to collate, publish and present these results and any other clinical evidence re: the efficacy of the drug for those who are non-ambulant & diagnosed with SMA type III?

The NICE process and the subsequent MAA eligibility criteria are evidence-based and so dependent on the available data. Although nusinersen has been used to treat more than 8,400 patients worldwide and maintains the largest body of interventional clinical trial data to date in SMA,[2] it was deemed that additional data would be required in order to be able to include type III paediatric patients who have lost the ability to walk independently for 12 months or longer, and adults with symptomatic type III SMA who have lost the ability to walk independently.

As outlined above, we are focused on evaluating the effectiveness of nusinersen, including among non-ambulant SMA type III patients, through our ongoing clinical programmes, collaborations with registries and the funding of independent SMA research. Biogen remains committed to publishing data which advances our understanding of treatment in this population, as soon as it is deemed sufficiently robust.

Q: What steps is Biogen taking over what timescale to present the case to the MAA oversight committee that the efficacy outcome measures should be expanded so that potentially other groups can be included in the MAA.

We will work closely with the MAA oversight committee to understand how best to submit further evidence to refine and improve the Agreement.

We will continue to strive for access for all those who may benefit from nusinersen. Biogen is committed to continue collaborating with the patient and medical community, the government, NHSE and NICE, as we believe this is the best route to achieve this.

Both NICE and NHSE have agreed to a reasonable middle ground where they have committed to review the Agreement when new data is available on the impact of the medicine on those people with SMA who have lost ambulation and currently fall outside the MAA criteria.

Update on access in Wales and Northern Ireland

NICE published the final guidance for nusinersen on 24th July 2019, containing the evidence considered, final recommendation and MAA arrangements. This guidance is applicable across England, Wales and Northern Ireland. As per section 7.1 of the MAA, funding for type I SMA patients provided under this MAA is already available. As per section 7.2 of the MAA, treatment for type II and III SMA patients will be funded as soon as individual trusts are able to make services available within 90 days of publication of the NICE guidance ID1069 (i.e. by 22nd October 2019).

The Health Authorities in both Northern Ireland and Wales are in the process of reviewing their services now that there is a decision on funding and patients will be contacted by the treating hospital.

Update on access in Scotland

In Scotland, nusinersen is now available for clinicians to prescribe through the new ultra-orphan pathway for the treatment of types II and III 5q SMA. It has already been accepted by the Scottish Medicines consortium (SMC) for routine use for type I SMA since May 2018. Clinicians will be adopting a phased approach to its introduction, starting with children and then moving on to the adult population. The phased approach means that services can be safely configured to support the care associated with both the administration of the medicine and ongoing care of the patient. There are no formal exclusion criteria for type I, II and III SMA patients in Scotland, however patients will be assessed on an individual basis and the decision to initiate treatment and when to stop will be taken by their multidisciplinary team.

It has taken a long time to get to this point, and we want, once again, to pay tribute to the courage of the SMA community. We believe that the nusinersen process has demonstrated the need for reform of the NICE process, particularly for rare, spectrum conditions with a major paediatric impact. We have been encouraged by NICE's recent initiation of a review of its appraisal methods, which will include a public consultation of its proposals next year.[3]

References

1. Biogen. Spinraza LCM: Medical IIT/SRA Portfolio. January 2019.
2. Biogen. Data on file.
3. NICE. Changes we're making to health technology evaluation. Accessed via: https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-

technology-appraisal-guidance/changes-to-health-technology-evaluation. Accessed: August 2019.