Biogen statement to SMA community re: ultra-orphan designation for Spinraza®▼ (nusinersen) in Scotland

The Scottish Medicines Consortium (SMC) has granted nusinersen ultra-orphan designation, allowing the medicine to be reviewed in spinal muscular atrophy (SMA) types 2 and 3 (later onset) through its new ultra-orphan appraisal process. Nusinersen has been routinely available to SMA type 1 (infantile onset) patients in Scotland since May 2018, however later onset patients do not currently have routine access via the NHS.

The SMC's new process, which is more appropriate for nusinersen than the previous review route, launched in October 2018 and allows greater flexibility in evaluating medicines for severe, very rare diseases for which there is a high level of unmet need. The SMC's decision is in line with recommendations submitted to the Scottish Government in 2016 following a review of the system to allow a greater degree of flexibility for the assessment of ultra-orphan medicines. It means ultra-orphan medicines, such as nusinersen, could be made available on the NHS in Scotland for at least three years while further efficacy data are gathered, ahead of a final decision on routine availability.

Biogen is extremely encouraged by the collaboration and flexibility shown throughout the appraisal process in Scotland – the SMC took just four months to review the evidence and make their decision on access for type 1 patients. The review of nusinersen via this new process represents an increased possibility for SMA patients in Scotland beyond type 1, who are in desperate need, to access the medicine.

What happens now?

Biogen welcomes the opportunity to work with the SMC to formalise the data collection and reimbursement arrangements. Subject to successful sign off, nusinersen is expected to be routinely available across types 1, 2, and 3 from April 2019. Between now and April 2019, Biogen will continue to work with all stakeholders in Scotland to finalise the data collection required to aid the SMC's future assessment of the value of nusinersen.

What is the current status across the rest of the UK?

Appraisals of nusinersen remain in progress in England, Wales, and Northern Ireland, with decisions expected in early 2019. Biogen is working closely with the relevant authorities to expedite timelines where possible to make the treatment available to patients as swiftly as possible, and bring equality in access with the 24 countries in Europe (and many more across the world) already making nusinersen available.[1]

[1] Biogen. SMA community update. January 2019.