NICE announces more people eligible for nusinersen following review of Managed Access Agreement

PRESS RELEASE

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NICE has today (DATE 2020) announced that more people with the rare genetic disorder spinal muscular atrophy (SMA) are to benefit from nusinersen (also called Spinraza and made by Biogen) following a review of data collected as part of the Managed Access Agreement (MAA).

The review, involving Biogen, patient groups, clinicians, SMA REACH UK and NHS England and NHS Improvement, assessed whether new evidence had become available to support a change in the MAA treatment eligibility criteria. Specifically, it looked at whether people with type III SMA who are unable to walk can benefit from nusinersen and therefore should be included in the MAA.

The MAA is a special arrangement between NICE, NHS England and NHS Improvement and Biogen. It allows patients to access treatment with nusinersen while at the same time collecting data on its impact in groups where additional evidence is required to address the uncertainties identified by the independent NICE appraisal committee in its original assessment of nusinersen. The MAA is also designed to address the financial risk and challenges for implementation in the NHS. It is not routine commissioning of nusinersen in line with its marketing authorisation.

Meindert Boysen, deputy chief executive and director of the Centre for Health Technology Assessment at NICE, said: "There are people with SMA who are not able to access treatment with nusinersen under the terms of the MAA which began in July 2019.

"At the time we made a commitment that we would review new evidence on the potential benefits of nusinersen for type III SMA patients who are not currently receiving it. We are therefore pleased that the review has concluded that it is

appropriate to extend the clinical eligibility criteria to allow access to nusinersen for type III SMA patients who aren't able to walk. It will also allow the removal of the rule which meant that patients who lose the ability to walk after 12 months of treatment wouldn't be eligible for further treatment."

Service capacity to deliver nusinersen to this wider NHS patient cohort is being developed and will expand further as COVID restrictions on social distancing ease. Where services are already established, some patients will be able to access nusinersen immediately.

Further details about the outcome of the review will be available on the NICE website at: https://www.nice.org.uk/guidance/TA588.

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