

# DATA COLLECTION

## 1. What data will be collected?

The Managed Access Agreement (MAA) for nusinersen includes a Data Collection Agreement that outlines the types of data that will be collected from people on nusinersen treatment during the managed access period. This patient data is intended to answer uncertainties and knowledge gaps that were identified by the NICE appraisal committee during the original review of nusinersen.

At the end of the MAA, NICE will perform an appraisal (review) of the clinical and cost effectiveness of nusinersen for treating Spinal Muscular Atrophy (SMA). This review will take into consideration the data that has been collecting during the managed access period, in addition to other information on the drug that may have been collected from other sources (e.g. clinical trials and studies taking place outside of the UK).

There are three key areas of data collection which aim to help answer the uncertainties and knowledge gaps that were identified by the NICE appraisal committee during the original review of nusinersen:

- Clinical data collection (test results from your regular appointments)
- Patient Reported Outcome Measures (PROM) data collection (questionnaires that collect information about your and your caregiver's wellbeing)
- Resource utilisation data collection (information about your use of health and public services)

The MAA data collection period lasts for a minimum of three years with a decision whether to routinely fund and provide the treatment on the NHS expected within five years from the start of the agreement (July 2019).

You will have an opportunity to fully discuss what information will be collected and how it will be kept before you start treatment. You will be asked to sign two consent forms:

1. one to receive treatment as part of the MAA, and consent to the agreement terms
2. one to provide your permission to share your data with [SMA REACH UK](#), the registry that is collecting and storing data as part of the MAA.

### 1.1 Clinical Data Collection

This information will be gathered in clinic at your treatment centre and shared with the SMA REACH UK registry. It will include:

- Basic patient details including age and sex
- Molecular genetics diagnosis
- Details of nusinersen treatment and any other therapies a patient is receiving
- Motor function
- Mobility
- Scoliosis, including details of any spinal surgery
- Fractures
- Ventilation / respiratory events (e.g. For infections)
- Survival information

This information will be collected when the treatment is started ('baseline') and then twice a year afterwards with each visit being at least four months apart.

Clinical data collection from your routine clinical appointments will continue if you stop treatment.

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## 1.2 Patient Reported Outcome Measures (PROMs)

Often clinical trials do not capture all the outcomes relevant to patients and their carers that may impact their everyday life. PROMs or Quality of Life (QoL) data can help demonstrate the effect that a treatment has on a patient's and caregiver's well-being and their ability to perform everyday tasks. This data collection goes beyond what can be seen with clinical measurements.

Quality of Life (QoL) questionnaires are one way of capturing this information. If used for this MAA, questions may cover information about:

- Mobility/walking
- Ability to undertake daily activities: eating, washing and dressing, self-transfer etc.
- Limb/joint weakness
- Pain
- Fatigue
- Breathing difficulties, choking or swallowing
- Speech and other forms of communication
- Difficulties sleeping at night or daytime sleepiness
- Weight changes
- Problems with digestion
- Psychological and emotional well-being
- Ability to attend school / college
- Loss of earnings and productivity (employment)
- Ability to participate in society
- Impact on family

Biogen are working with other organisations (including patient advocacy groups) to find the best way to collect PROM data as part of the MAA, and are aware of the need to capture the views of both treated children, their parents and treated adults (and potentially anyone else who is key in an adult's life). These Q&As will be updated as soon as further information about the PROMs programme is available

## 1.3 Resource utilisation

Measuring resource utilisation is important to understand the impact treatment has on the cost of care. The way that this information will be captured is still to be agreed between NHS England, NICE and Biogen. It will include:

- Number of patient admissions
- Number of medical investigations and therapies
- Use of medical equipment
- Costs associated with personnel involved in the management of SMA patients (e.g. the costs of employing personal assistants to provide care and support)

## 2. Where will all this information be kept?

Clinical patient information will be kept centrally by the SMA REACH UK network, managed by the Dubowitz Neuromuscular Centre, Great Ormond Street Hospital and the MRC Neuromuscular Centres in London and Newcastle. This is an independent group of NHS healthcare professionals who work together to establish national agreement on medical and physiotherapy assessments, and standards of care for patients with Spinal Muscular Atrophy.

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Data will be kept secure and protected on the SMA REACH database in accordance with national regulations.

Biogen will provide funds to support SMA REACH UK.

More information on the SMA REACH UK registry can be found here: <http://www.smareachuk.org/>

This information sheet will be updated when it is decided where the PROMs and resource utilisation information will be stored.

### **3. Who will see the information?**

Information will be shared pseudo-anonymously with members of the MAA Clinical Panel (See MAA Section), NICE, NHS England and Biogen. Pseudonymised means replacing characteristics of personal data with a pseudonym, a unique value that does not allow the person to be directly identified without the use of additional information.

### **4. If further treatments are recommended by NICE, would the same data collection system apply?**

No, not necessarily, arrangements for data collection on a treatment are decided on a case by case basis. They would depend on a number of factors including the NICE appraisal committee's decision.