NICE National Institute for Health and Care Excellence

# Nusinersen for treating spinal muscular atrophy (TA588) managed access agreement

## Clinical eligibility criteria evidence review process

The nusinersen managed access agreement (MAA) includes a commitment to review new evidence that becomes available for patients with non-ambulant type III spinal muscular atrophy (SMA) after 1 year (from the start of the agreement) that may affect the treatment eligibility criteria within the MAA. The evidence review will follow the process steps outlined below.

If the evidence review concludes that nusinersen provides comparable benefits for patients with non-ambulant type III SMA resulting in a recommendation to revise the MAA eligibility, starting and stopping criteria, then the newly eligible group(s) of patients may have access to nusinersen treatment.

If the evidence review concludes there is not enough new information to revise the MAA eligibility, starting and stopping criteria, then groups of patients who are currently ineligible for nusinersen treatment will have access to best supportive care only.

## Step 1: Outline of evidence review objectives

NICE's Managed Access (MA) team prepares an outline of objectives for the External Assessment Centre (EAC), company and Managed Access Oversight Committee (MAOC) and sets out the scope of the evidence review.

## Step 2: Review initiation: notification of deadline for new evidence submission

The company and MAOC members are given formal notice of the need to submit data within 28 days to initiate the evidence review process. All new evidence is shared with the company in the first instance.

## Step 3: External evidence review

The EAC assesses the new evidence and delivers recommendations in line with the Outline of Objectives document.

## Step 4: Clarification questions and responses

During the review, the EAC sends any clarification questions to the company.

The company has 7 days to respond to clarification questions.

## Step 5: Managed Access Oversight Committee (MAOC) review

The MAOC reviews the recommendations from the external evidence review and indicates whether it supports the recommendations of the EAC.

## Step 6: Stakeholder engagement

The MA Team prepares a brief concerning the outcome of the evidence review for circulation to the MAOC.

The MAOC is invited to submit any comments or requests for clarifications during a 7-day consultation period.

Points of clarification are reviewed by the MA Team and updated details are incorporated into the final briefing stage that follows.

A further meeting with stakeholders will be held if required.

## Step 7: Final briefing

NICE’s MA team produces a brief summarising the evidence submitted and a short statement concerning the outcome for the MAOC for information only, before publication.

## Step 8: Final recommendation publication

The evidence submitted and a short statement concerning the outcome (and an amended, executed MAA, if applicable) are published on the NICE website.

## Membership of the Managed Access Oversight Committee

The Managed Access Oversight Committee (MAOC) is a group of key stakeholders (including the agreement signatories) convened by the NICE Managed Access (MA) team to monitor the progress of the MAA throughout the agreement term. The nusinersen MAOC membership is as follows:

### Voting members

- A representative from NHS England and NHS Improvement
- Two paediatric clinical experts in the treatment of spinal muscular atrophy in children
- One clinical expert in the treatment of spinal muscular atrophy in adults
- One physiotherapist involved in the treatment of spinal muscular atrophy
- A representative from Spinal Muscular Atrophy UK (patient organisation)
- A representative from Treat SMA (patient organisation)
- A representative from MDUK (patient organisation).

### Non-voting members

- NICE Managed Access Associate Director
- NICE Technical Advisor or Analyst
- NICE Senior Manager Evidence Generation and Oversight
- SMA-REACH Clinical/Academic representative
- SMA-REACH (Global) Trial Manager
- A representative from the adult SMA data network
- Two standing representatives from Biogen (company) and 1 substitute representative. **Note:** Biogen representatives will be present for the first

part of the MAOC review meeting only (during presentation of the evidence). The MAOC will deliberate and make its decision in private.

## Observers/advisors

- NICE Technology Appraisals Committee C Chair (MAOC evidence review meeting chair)
- NICE Technology Appraisals Committee C clinical member
- Representatives from the NICE External Assessment Centre.

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