

SPINRAZA (NUSINERSEN) NUSINERSEN ACCESS FOR CHILDREN

1. What Centres will offer treatment to children?

NHS England currently commissions treatment from the following 13 paediatric neurology centres in England that have the expertise to deliver nusinersen treatment:

- Alder Hey Children's NHS Foundation Trust
- Cambridge University Hospitals NHS Foundation Trust
- Great Ormond Street Hospital for Children NHS Foundation Trust
- Guy's & St Thomas' NHS Foundation Trust
- Leeds Teaching Hospitals NHS Trust
- Manchester University NHS Foundation Trust
- Nottingham University Hospitals NHS Trust
- Oxford University Hospitals NHS Foundation Trust
- Sheffield Children's NHS Foundation Trust
- The Newcastle upon Tyne Hospitals NHS Foundation Trust
- University Hospital Southampton NHS Foundation Trust
- University Hospitals Bristol NHS Foundation Trust
- University Hospitals of North Midlands NHS Trust

GP referrals and enquiries should be directed to the Paediatric Neurology Department at the treatment centre.

If there are other centres that wish to deliver the service, they will need to make a formal application to NHSE to demonstrate that they have the expertise to do this. Discussions will take place with individual providers to ensure that they have the capability and capacity to deliver the service.

2. Will the service be the same across the country?

NHS England has produced referral guidance to assist centres in delivering the service. This includes a framework to advise treatment centres on which patients should be prioritised for treatment. This should help to ensure a consistent approach to treatment that is based on clinical need.

The following groups of patients should be prioritised for treatment:

- Children who have SMA type 1 who have been receiving nusinersen through the Expanded Access Programme (and who will continue to receive treatment)
- Children who have SMA type 1 who have been diagnosed since the Expanded Access Programme closed
- Any further children diagnosed with SMA type 1
- Pre-symptomatic children who: have a confirmed genetic diagnosis of SMA; have two SMN2 copies or fewer; and in whom SMA type 0 is not yet apparent
- Children who have SMA type 2 who have not been ambulant and who have been diagnosed in the previous six months or so.

Access for other children who meet the entry criteria will be managed by the treating centre.

If there are concerns about equity of access, NHS England will review the situation. Any concern you have should be discussed with one of the patient advisory groups, who will address this with NHS England. This will allow the patient advisory groups to collate information about access issues. This does not prevent you from writing formally to your treating centre or to NHS England.

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3. Can we choose which centre to go to for treatment?

Yes, though it is recommended that you choose the centre nearest to you to avoid unnecessary travel and to ensure that you receive care for all aspects of your SMA from a team who is familiar with both you and managing SMA.

4. What do we do if our nearest centre is not supporting access to nusinersen?

You can ask your consultant to refer you to a centre that is providing access. That centre should consider the NHS England priority guidelines to decide when to discuss the possibility of treatment with you as well as all the other patients the centre may be considering for access.

5. If our centre doesn't agree to treat my child, can we appeal?

The criteria in the MAA are to be used consistently across the country, therefore it is unlikely that one centre will consider that someone is not eligible, and another will think otherwise. However, though there is no formal appeal process, you can seek a second opinion from another treatment centre.

You will need to ask your GP to refer you to another centre on the list of agreed providers and you will need to ask your treating centre to pass on relevant clinical information to the treating centre where you are seeking the second opinion. If the second centre agrees that you should have access to treatment, there will need to be a discussion between the two clinical teams and with you to decide on the best location for treatment.

The MAA Clinical Panel (see MAA Section) can also offer advice to individual treatment centres on:

- diagnosis
- the MAA Starting and Stopping Criteria
- administration of the drug.

6. Who will monitor centre practice?

NHS England will use its usual contract management routes to ensure that each centre is delivering a high-quality service.

The Managed Access Oversight Committee (See MAA section), chaired by NICE, will collate information from each centre including, for example, whether they are collecting all the data required. If a centre is not collecting the MAA information that it should, this will be followed up with that individual centre.

7. How long will my child have to wait for treatment?

This will depend on what priority your child has according to:

- the NHS England guidelines (see the MAA section)
- your treatment centre's capacity
- how many other children are on their waiting list for treatment.

You can ask your treatment centre to give you an idea of how long you will need to wait.

8. My child is due for spinal surgery soon – I'm worried they will miss out on treatment – what should we do?

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You should discuss the options with your treatment centre. If your local treating centre needs advice, the MAA Clinical Panel can offer advice on the feasibility of administration of the drug following surgery.

9. My child needs ventilation for 16 hours a day but I really want them to get treatment. Can I reduce their ventilation so that they will be eligible or am I putting them at risk?

You should discuss your child's ventilation requirements and the reasons for this and your wish for your child to have access to treatment with your treatment centre. Do not change your child's treatment or ventilation plan without prior discussion and agreement with your treating clinician.

10. What if my child is too unwell to start treatment on an agreed date – will they lose their place on the treatment list?

Treatment centres will need to carefully schedule treatments, especially the loading doses. If your child is unwell, it may mean that they will not be able to start treatment on the expected date, but they should still be prioritised in line with the NHS England priority guidelines.

11. How long will each treatment be – will it take long to recover – what time will we need off nursery or school?

Recovery time is different for each patient. If it is the first time your child has been given the treatment, or the procedure is complicated, or your child needs sedation, you can expect they may need longer in hospital. If the procedure is very straight forward and they have received it multiple times previously then a minimal time in hospital may be all that your child needs. You can talk this through with your medical team

12. I am worried my child will be frightened by the treatment, who will explain to them what the treatment involves and make sure my child is comfortable to go ahead

Individual treatment centres will have staff trained in administering intrathecal injections. They will be able to explain what the treatment involves and whether, for example, sedation or anaesthesia are appropriate.

13. Can we get help with my travel costs?

If you are in receipt of benefits and/or on a low income, you can get help with travel costs through the Healthcare Costs Travel Scheme:

<https://www.nhs.uk/using-the-nhs/help-with-health-costs/healthcare-travel-costs-scheme-htcs/>

14. Will I be able to stay with my child during the actual treatment? Will there be anywhere to stay and any help with accommodation costs in general?

You should ask your treatment centre about any possible accommodation options.

15. What if my child is unwell during treatment and has to miss a dose?

Your child should have their treatment as soon as possible after the one they have missed. You should discuss this with the treatment centre. There should always be at least 14 days between treatments.

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16. How soon will we know if treatment is working?

The time it takes to see a response from therapy is variable between patients and depends on many different factors. Some patients respond very quickly, within months, others take longer, up to a year. Families with children have sometimes reported responses before they appear on motor assessment scores. A rule of thumb is that the more unwell the patient or the longer the patient has lived with SMA, the longer it may take to show benefit from therapy.

You will be seen by your medical team at least twice each year (4 - 6 monthly) to review how treatment is going and collect information (see Data Collection Section).

17. What tests will the medical team do to find out?

The medical team will measure a range of assessments. These may include muscle function, breathing function, swallowing ability, measures of scoliosis.

18. Will we get any emotional support throughout? Especially if it's not working as we hoped.

Many hospitals can provide emotional support services. If this service does not exist at your hospital, you should consider contacting your GP for local emotional support services. The SMA patient charities are also a good source of support.

19. What if another treatment comes along – will my child be able to swap or have two treatments at the same time?

You should discuss this with your treatment centre. NHS England has asked all treatment centres to confirm that they have discussed all treatment options with patients and families before they start nusinersen treatment.

20. What if my child is on trial for another treatment and has to come off it, would they be able to start nusinersen treatment?

Your child would not be prevented from accessing nusinersen treatment just because they have been on another trial. You would need to discuss how this could be managed and what the possible benefits and risk of starting nusinersen treatment would be with your child's clinical team.

21. What if, during the MAA, my child transitions from paediatric to adult care? Will they have to go to a different centre and have a different team delivering their treatment? Will there be a way of making sure they still want treatment?

It is expected that individuals will transition from paediatric to adult care between the ages of 16 and 18 years. In some cases, patients may be treated at the same centre and, in other cases, they may need to transfer to another centre. There will be specific transition arrangements in each case which will include making sure you and your child / young adult are fully informed about what is happening and why and changes in consent to treatment. Once a child turns 18 the decision to consent to treatment rests solely with them as an adult (unless they lack capacity to make a decision).

22. What if my child or I want to stop treatment?

You can choose to stop treatment at any time during the lifetime of the MAA but this should be a decision that you take jointly with your treatment centre. Any information gathered about your child up until that point will remain part of the MAA evaluation.

23. What if clinicians want to stop treatment but my child or I want to carry on?

The criteria in the MAA are to be used consistently across the country, therefore it is unlikely that one centre will consider that someone is not eligible, and another will think otherwise. However, though there is no formal appeal process, you can seek a second opinion from another treatment centre.

You will need to ask your GP to refer you to another centre on the list of agreed providers and you will need to ask your treating centre to pass on relevant clinical information to the treating centre where you are seeking the second opinion. If the second centre agrees that you should continue to have access to treatment, there will need to be a discussion between the two clinical teams and with you to decide on the best location for continuing treatment.

The MAA Clinical Panel can also offer advice to individual treatment centres on:

- the MAA Starting and Stopping Criteria
- administration of the drug.

Patients and their families should discuss this with their treatment centre. NHS England has asked all treatment centres to confirm that they have discussed all treatment options and clearly explained how the MAA works with patients and families before initiating them on nusinersen.

24. If my child is not eligible for treatment under the terms of the MAA, can they get treatment privately?

Some hospitals offer **private treatment** for neuromuscular conditions, for example, Great Ormond Street:

<https://www.gosh.com.kw/conditions/neuromuscular>

A patient's clinician can submit an **Individual Funding Request (IFR)** in line with NHS England policy:

<https://www.england.nhs.uk/publication/individual-funding-requests-for-specialised-services-a-guide-for-patients/>

Patients and families should be aware that if an IFR application is made the clinician will need to demonstrate that the patient's clinical circumstances are exceptional.