

ACCESS FOR ADULTS

1. What centres will offer treatment to adults?

NHS England has commissioned eleven centres to treat adult patients as follows:

- Bristol North NHS Trust
- Kings College London NHS Foundation Trust
- Nottingham University Hospitals NHS Trust
- Oxford University Hospitals NHS Foundation Trust
- Salford Royal NHS Foundation Trust
- Sheffield Teaching Hospitals NHS Foundation Trust
- St George's University Hospitals, London NHS Foundation Trust
- Southampton NHS Trust
- The Newcastle upon Tyne Hospitals NHS Foundation Trust
- University College London Hospitals NHS Foundation Trust
- University Hospitals Birmingham NHS Foundation Trust

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:

Adult:

1. Patients with SMA already receiving nusinersen and transitioning to adult services
2. Patients with SMA type 3 who are about to lose ambulation
3. All other ambulant patients with SMA type 3
4. Patients with SMA type 2 who have no spinal fixation and preserved upper limb function
5. Patients with SMA type 2 who have spinal fixation requiring a port or catheter for the administration of nusinersen

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 groups, who will address this with NHS England. This will allow the patient groups to collate intelligence about access issues. This does not prevent you from writing formally to your treating centre or to NHS England.

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

Yes, although this will be dependent on the capacity of the centre (which may be affected by the COVID-19 pandemic) and the acceptance of a referral. 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 I do if my 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 and all the other patients the centre may be considering for access.

5. If my centre doesn't agree to treat me, can I appeal?

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The criteria in the Managed Access Agreement (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, although 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 7) 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 MAA Oversight Committee (See MAA section 8), 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 I have to wait for treatment?

This will depend on what priority you have according to:

- the NHS England guidelines
- your treatment centre's capacity
- how many other adults are on their list for treatment.

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

8. What if I am too unwell to start treatment on an agreed date – will I lose my place on the treatment list?

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

9. How long will each treatment be – will it take long to recover – what time will I need off work?

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

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10. Can I 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/>

11. I will need my partner/a family member with me, and we may need to somewhere to stay. What help can I get?

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

12. What if I'm unwell during treatment and have to miss a dose?

You should have your treatment as soon as possible after the one you have missed and discuss this with the treatment centre. There should always be at least 14 days between treatments.

13. How soon will I know if treatment is working?

The time it takes to see 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. A rule of thumb is that the more unwell the patient or the longer the patient has lived with SMA, the longer it takes 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).

14. 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.

15. Will I get any emotional support throughout? Especially if it's not working as I 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.

16. What if another treatment comes along – will I 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 before they start nusinersen treatment.

17. What if I am on trial for another treatment and I have to come off it, would I be able to start nusinersen treatment?

You wouldn't be prevented from accessing nusinersen treatment just because you have been on another trial. You would need to discuss with your clinical team how this could be managed and what the possible benefits and risks of starting nusinersen treatment would be.

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18. What if I decide 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 you up until that point will remain part of the MAA evaluation.

19. What if clinicians want to stop treatment but 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 (see MAA Section 7) can also offer advice to individual treatment centres on:

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

20. If I'm not eligible for treatment under the terms of the MAA, can I get treatment privately?

Some hospitals offer **private treatment** for neuromuscular conditions, for example, University College London Hospitals:

<https://www.uclh.nhs.uk/PandV/Privatepatients/Pages/Home.aspx>

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

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

Patients should be aware that the clinician will need to demonstrate that the patient's clinical circumstances are exceptional.