

Who can access NHS England-funded Zolgensma treatment?

Monday 8th March 2021.

These are the questions SMAUK gathered from the patient groups in anticipation of a possible positive decision about access to Zolgensma in England. We are now asking for answers from NHS England, Novartis Gene Therapies and clinicians as soon as is reasonably possible and will publish these on our websites.

- **Clarification as to who is eligible?**
 - Newly diagnosed?
 - Already receiving nusinersen?
 - Already receiving risdiplam?
 - Chosen not to have treatment so far?
 - Pre-symptomatic?
 - Any age limits and variations in criteria according to age range?
 - If so, at what point do they apply e.g. at the age a child was at the time of the announcement, or at the time of the assessment, or at the time of the treatment etc?
 - e.g. if there is a difference between criteria under 6 months and the criteria for 7-12 months (as suggested in the announcement) what criteria apply between 6 months and 7 months?
 - Any weight limit, or other limits?
 - Any cap on numbers?
- **Do they have to live in England?**
 - If someone lives in another part of the UK: Can they move permanently or temporarily to England to access the treatment? If so – are there any time limits on how long they have to have been at the English address or any other criteria?
 - What about people from outside the UK?
- **Where are the Centre(s) that will offer the treatment?**
 - Which one would my child go to?
 - How would my child get referred?
 - When will it actually start happening?
 - Would my child get treated straight away or might they have to wait? If so, how will children be prioritised?
- **If my child is in the eligible group, what first test needs to be done before I know if treatment might be possible for my child?**
 - When and where is this done?
 - How long does it take to get the result?
 - Do we need to isolate during this time? Why is this?
- **If my child is on another treatment at the moment and eligible to move to zolgensma:**
 - Do they need to come off the other treatment?
 - How long do they need to do this before they can have zolgensma treatment?
 - Can my child resume the other treatment after they have had zolgensma?
 - After having had zolgensma, can my child have a further treatment later on?
- **If my child is in one of the eligible groups, are there any reasons why they may not be treated (exclusion criteria)?**
 - Test for the antibodies for the virus that carries the treatment (AVV test) is positive so treatment not possible?

- What other reasons are there?
 - Who decides these reasons apply to my child?
 - Can I appeal?
 - Can I get a second opinion?
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➤ **What is zolgensma and how does it work?**

- Explanation of the term gene therapy (is this a permanent genetic change? would it be passed on to future generations?)
 - Explanation of the mechanism (Which cells and tissues in the body does it reach?)
 - What have clinical trial results and other evidence shown about the possible outcomes with zolgensma treatment?
 - Are there any 'real world' studies of outcomes? If so, what do they show?
 - How long since the first children have been treated, how old are they now? How are they doing? (trials and real-world studies)
 - What are the risks / any safety concerns?
 - How do the results that have been seen so far compare to nusinersen?
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➤ **The treatment process – (once the AVV test results are back and clear)**

- **Where will I need to go/ who will I need to see to discuss my child's treatment?**
- **What tests and 'baseline' assessments take place before the treatment**
 - Why do you do them?
 - How long does it take to do them?
 - Where do we need to be for them?
 - Do we need to isolate during this time?
- **How is the actual treatment delivered?**
 - How long does it take?
 - Does it hurt?
 - What if my child is too unwell for treatment on the day, or there is a problem with staffing or treatment delivery at the Centre?
- **Immediately after treatment what happens?**
 - How long do we need to stay at the Centre, when can we go home?
 - What care do we need to put in place when we get home?
 - How long do we need to do this for?

Do I have to isolate with my child?

- while the tests are being done?
- during treatment?
- immediately afterwards?

Support to access this treatment:

- What help will we be given to get to the treatment Centre and with accommodation and food during all these stages?
- What about the rest of my family – who will take care of them? Time off work / for a partner to do all care?

- Who will arrange all this?

Longer term aftercare

- What special care do I need to give my child?
- What do I need to watch for?
- What follow up tests / assessments are there?
- Where do these take place?
- Do we still need to isolate?

What support is provided throughout and following treatment, including emotional and psychological?

➤ What information will be collected?

- **What data will be collected and how?**
 - Clinical Data Collection
 - Patient Reported Outcome Measures (PROMs)
 - **Where will all this information be kept?**
 - **Who will see the information?**
 - **What will you do with it?**
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➤ Private Access

- **Will England offer private access now?**
 - To people living in England who don't want to wait and have the funding?
 - To people living in other parts of the UK?
 - To people from outside the UK?
 - If yes to any of the above groups, what are the eligibility criteria?