

## THE TREATMENT PROCESS

### 1. How is the treatment delivered?

Nusinersen is delivered directly into the cerebrospinal fluid (CSF).

Doctors access the CSF using a lumbar puncture – a needle is inserted through the skin into the space between the vertebrae (back bones) of the spine. Doctors may use ultrasound or another imaging method to locate the best place for the insertion. They usually use a local anaesthetic such as 'numbing cream'. Some patients need a sedative and occasionally a general anaesthetic may be considered necessary. The treatment centre will have an individual discussion with you about what the best option would be for you / your child.

A small amount of CSF is collected and then nusinersen is injected over one to three minutes.

Only specially trained healthcare professionals are allowed to give intrathecal injections

The treatment can only be delivered by lumbar puncture. This procedure needs to take place within one of the Neuro-muscular treatment centres.

### 2. What are the risks of lumbar puncture in general?

You can read about this in the leaflets here:

<https://smauk.org.uk/treatment-information-leaflets>

And in here:

<https://www.hey.nhs.uk/patient-leaflet/lumbar-puncture/>

### 3. How long does each treatment take to be delivered?

The total length of time the treatment takes will depend on many factors. The procedure can be very quick or can take a long time. The treatment centre is best placed to advise how long it may take to treat you / your child.

### 4. How frequently does treatment need to be delivered?

Injections are given as follows:

A series of first doses called 'loading doses' are administered to get the level of the drug to an effective concentration within the CSF. Loading doses take place:

- On the first day of treatment, day 0
- Then around day 14, day 28 and day 63

There should always be at least 14 days between doses.

Once the loading doses have all been delivered, there are then maintenance doses every 4 months to maintain the drug concentration level in the CSF.

### 5. What if the next injection is delayed – due to my / my child's illness or a problem with staffing at the treatment centre?

If a dose is missed, the next dose should be administered as soon as possible. The timing for this will depend where you / your child are within the treatment schedule. If you have any

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questions or concerns about ensuring a safe time between doses or a dose being given later than planned, you should discuss these with your / your child's treating clinician.

### **6. How long does treatment need to continue?**

Treatment needs to continue unless the treating clinician and you / you and your child agree that it should stop or a patient meets the stopping criteria of the Managed Access Agreement (see MAA Section).

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

Though they are not formal guidelines, the International Standards of Care for SMA describe which assessments and interventions families and adults should expect to find in any neuromuscular centre anywhere. They were agreed in November 2017 by a committee of international healthcare and patient experts.

You can access the family guide to them [here](#) or at:

<https://smauk.org.uk/international-standards-of-care-for-sma>

### **7. How long will each treatment be – will it take long to recover – what time will we need off school and work?**

Recovery time is different for each patient. If the procedure is very straight forward and the patient has received it multiple times previously, then a minimal time in hospital may be all that is required. If it is the first time that treatment is given to a patient, or the procedure to deliver treatment is complicated, or if sedation is used, then a longer stay in hospital after the procedure may be necessary.

### **8. 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 or not, for example, sedation or anaesthesia are appropriate.