

Title: Preferences and Utilities for Treatment Attributes in Type 2 and Non-Ambulatory Type 3 Spinal Muscular Atrophy in the United Kingdom

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Appendix A: Attributes & levels

1 Treatment effectiveness

1.1 Motor function

Treatments can vary in terms of their average effectiveness in improving the motor function of patients with SMA. This means that actual treatment effect for your child could vary from the average treatment effectiveness. A simplified scale of motor function is shown below:

1	Cannot sit
2	Can sit with some support (e.g. with back support or arm support)
3	Can sit independently for a few seconds
4	Can sit independently for a longer period of time but cannot stand
5	Can sit independently and stand with assistance , but cannot walk
6	Can sit independently and stand and walk with assistance
7	Can sit, stand and walk independently for a few steps (less than 10 meters)
8	Can sit, stand and walk independently over longer distances (more than 10 meters)

We will describe the effectiveness of the treatment – on average – in terms of worse, stable or better motor function.

- **Worse**
After 12 months, motor function will have deteriorated by one level on the motor function scale.
- **Stable**
After 12 months, motor function will remain on the current level of function.
- **Better**
After 12 months, motor function will have improved by one level on the motor function scale.

1.2 Breathing

Treatments can vary in terms of their average effectiveness in improving breathing ability among patients with SMA. This means that the actual treatment effect for your child could vary from the average treatment effectiveness. We will describe the effectiveness of the treatment – on average – in terms of worse, stable or better breathing function.

- ***Worse***

After 12 months, breathing function will get worse.

- ***Stable***

After 12 months, breathing function will stay the same.

- ***Better***

After 12 months, breathing function will get better.

2 Administering the treatment

Treatments can vary in terms of whether they are administered by injection or orally. The different treatments you will see will be administered using the following methods:

- ***Injection into the spine at the lower back***

Administered by injection in the lower back using a needle inserted into the space around the spinal cord, also known as a lumbar puncture. When your child first starts the treatment, they will receive it every 2 weeks, then 1 month apart. After your child would need to return to hospital every 4 months for an injection.

- ***Oral***

An oral liquid to be taken once daily at home. It can also be administered via a gastric tube at home after you have received some training.

3 Side effects and reactions to the treatment

People can experience a reaction to their treatment immediately after taking it or develop side effects at a later stage. These reactions may vary in severity (mild, moderate or severe).

3.1 Eyesight monitoring

Your child's eyes may need to be monitored. This involves a scan, which can image the eye, and possible further examinations as appropriate:

- No eye monitoring required.
- Eye monitoring before start of treatment required and additional monitoring during treatment if your child has symptoms.
- Eye monitoring before start of treatment required and twice a year for the first 2 years.

3.2 Reactions caused by treatment

Treatments could also cause reactions, lasting between less than 24 hours and 3–4 days.

- No fever, headache, vomiting and/or body pain due to treatment.
- Fever, headache, vomiting and/or body pain for 1–2 days every 4 months.
- Fever, headache, vomiting and/or body pain for 3–4 days every 4 months.

3.3 [ADULT PATIENTS ONLY] Need for contraception

For some treatments it is essential that you use an effective contraception while you are taking the treatment because the treatment poses a significant risk to any pregnancy. Depending on the treatment you use you can expect:

- There is no requirement to use contraception.
You must agree to use an effective contraception.