

APPENDIX A
USING OTHER DISEASE-MODIFYING MEDICINES FOR SPINAL MUSCULAR ATROPHY (SMA) AFTER ONASEMNOGENE ABEPARVOVEC TREATMENT

Group 1: children who have had onasemnogene abeparvovec only and have not previously been treated with other disease-modifying medicines for SMA

Group 2: children who have had onasemnogene abeparvovec and who have had previous treatment with other disease-modifying medicines for SMA

Children in these two groups can only access other medicines for SMA within the NHS if treatment with onasemnogene abeparvovec is not successful, as defined below AND following advice from the NHS England Clinical Panel.

The role of the NHS England Clinical Panel is to provide expert advice to treatment centres about individual patients in respect of the Starting and Stopping Criteria. It is for individual treatment centres to take decisions about individual patients.

The eligibility criteria of nusinersen and risdiplam will include, or be amended to, the following:

Starting criteria:

- Must not have had successful treatment with onasemnogene abeparvovec.

Stopping criteria:

- Has successful treatment with onasemnogene abeparvovec.

All other eligibility criteria for the corresponding treatment will apply.

Non-successful treatment with onasemnogene abeparvovec is defined as per the criteria below.

Definition of non-success of onasemnogene abeparvovec

(a) **A reduction in motor ability**, defined as:

Total worsening in scale score corroborated by two consecutive measurements from any two of the following three scales:

- >2 points on horizontal kick or 1 point on other HINE scores excluding voluntary grasp
- >4 points on the CHOP INTEND scale
- >3 points on the RHS scale

A scaled equivalent of these losses would apply if a domain was unmeasurable / not suitable. These scores are derived from the minimal clinical indicators of difference. For example, if a patient deteriorates on one scale (e.g. loses >3 points on the RHS scale) but maintains stability or demonstrates improvement on another scale that has been measured since baseline (e.g. RULM), the patient's treatment with onasemnogene abeparvovec would be considered to be successful. A treating clinician must refer any case to the NHS England Clinical Panel for advice on non-success in respect of deterioration in scale scores.

AND/OR

- (b) **A deterioration in respiratory function**, defined as an increasing requirement for respiratory support overnight and/or, for that patient, an uncharacteristic increase in respiratory infections requiring hospital treatment that cannot be accounted for by aspiration or intrinsic lung disease.