

1.	<p>Treatment & Condition</p> <p>Nusinersen for treating spinal muscular atrophy.</p>
2.	<p>Associated appraisal body & Summary of ruling</p> <p>NICE Technology Appraisal guidance TA588 (July 2019).</p> <p>Nusinersen is recommended as an option for treating 5q spinal muscular atrophy (SMA) only if:</p> <ul style="list-style-type: none"> • people have pre-symptomatic SMA, or SMA types 1, 2 or 3 and • the conditions in the managed access agreement are followed.
3.	<p>Number of people in Northern Ireland expected to take up service/therapy</p> <p>According to estimates provided by the Service, there are currently 4 patients who will be eligible immediately for treatment with nusinersen in line with this NICE guidance. There are another 3 patients who may be eligible in the future.</p>
4.	<p>Patient Access Scheme Availability</p> <p>(<u>Yes</u>/No)</p> <p>The company (Biogen Idec) has a commercial arrangement. This makes nusinersen available to the NHS with a discount. The size of the discount is commercial in confidence.</p>
5.	<p>Infrastructure Requirements</p> <p>Any additional infrastructure costs associated with the introduction of new therapies will be dealt with as part of the routine commissioning process.</p>
6.	<p>Expected implementation period</p> <p>There is no impediment to immediate implementation for new patients.</p>
7.	<p>Commissioning arrangements</p> <p>This regimen will be formally commissioned by the HSCB/PHA via the Specialist Services Commissioning Team initially on a cost-per-case (CPC) basis. Thereafter, numbers of patients who received or are receiving treatment will be reviewed and consideration will be given to moving to recurrent funding to support this regimen</p>
8.	<p>Monitoring arrangements</p> <p>The HSCB cost per case process will generate quarterly reports on the number of applications.</p>

9. DoH (NI) Legislative/Policy Caveats

This advice does not override or replace the individual responsibility of health professionals to make appropriate decisions in the circumstances of their individual patients, in consultation with the patient and/or guardian or carer. This would, for example, include situations where individual patients have other conditions or complications that need to be taken into account in determining whether the NICE guidance is fully appropriate in their case.

The Rural Needs Act NI 2016 has been considered and this guidance, which is purely of a technical nature, is not regarded as galling within the scope of the act.