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# **Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy**

## **Policy Position Statement: PPS191**

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PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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# Contents

Policy Statement.....	4
Welsh Language .....	4
Decarbonisation.....	4
Disclaimer.....	4
1. Introduction.....	6
1.1 Background.....	6
1.2 Equality Impact Assessment.....	7
2. Recommendations .....	8
2.1 Inclusion Criteria .....	8
2.2 Exclusion Criteria .....	8
2.3 Continuation of Treatment .....	8
2.4 Acceptance Criteria .....	8
2.5 Transition arrangements.....	8
2.6 Designated Providers.....	9
2.7 Patient Pathway (Annex i).....	9
2.8 Mechanism for funding .....	9
2.9 Clinical Outcome and Quality Measures.....	10
2.10 Action to be taken.....	10
3. Putting things right.....	11
3.1 Raising a Concern .....	11
3.2 Individual Patient Funding Request (IPFR).....	11
Annex i Patient Pathway .....	12
Annex ii Codes .....	13
Contact Us .....	14

# Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission nusinersen for children up to 17 years old with spinal muscular atrophy in accordance with the criteria outlined in this document.

## Welsh Language

NWJCC is committed to treating the English and Welsh languages on the basis of equality, and endeavour to ensure commissioned services meet the requirements of the legislative framework for Welsh Language, including the [Welsh Language Act \(1993\)](#), the [Welsh Language \(Wales\) Measure 2011](#) and the [Welsh Language Standards \(No.7\) Regulations 2018](#).

Where a service is provided in a private facility or in a hospital outside of Wales, the provisions of the Welsh language standards do not directly apply but in recognition of its importance to the patient experience, the referring health board should ensure that wherever possible patients have access to their preferred language.

In order to facilitate this, NWJCC is committed to working closely with providers to ensure that in the absence of a Welsh speaker, written information will be offered and people have access to either a translator or 'Language-line' if requested. Where possible, links to local teams should be maintained during the period of care.

## Decarbonisation

NWJCC is committed to taking assertive action to reducing the carbon footprint through mindful commissioning activities. Where possible and taking into account each individual patient's needs, services are provided closer to home, including via digital and virtual access, with a delivery chain for service provision and associated capital that reflects the NWJCC commitment.

## Disclaimer

NWJCC assumes that healthcare professionals will use their clinical judgement, knowledge and expertise when deciding whether it is appropriate to apply this document.

This document may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian, or Local Authority.

Policy Position Statement:

PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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NWJCC disclaims any responsibility for damages arising out of the use or non-use of this policy.

# 1. Introduction

This Policy Position Statement has been developed for the planning and delivery of nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy resident in Wales. This service will only be commissioned by the NHS Wales Joint Commissioning Committee (NWJCC) and applies to residents of all seven Health Boards in Wales.

In creating this document NWJCC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)<sup>1</sup> and has concluded that nusinersen should be made available.

## 1.1 Background

Spinal muscular atrophy (SMA) is a rare, progressive neuromuscular disease caused by a genetic mutation in the SMN1 gene on chromosome 5q. People with the condition have a range of symptoms, including muscle weakness, and have worsening physical disability, mobility loss and respiratory dysfunction.

SMA can be grouped into 5 main types (types 0 to 4), based on the age of onset and the maximum motor function reached.

Type 0 SMA is the most severe and affects babies before birth. The babies do not develop any motor skills and often survive for only a few weeks after birth.

Type 1 SMA affects children in the first 6 months of life. These children are unable to sit or roll because of severe muscle weakness, which gets worse over time. The muscle weakness also affects swallowing and breathing, and typically results in death within 2 years.

Type 2 SMA, the onset of symptoms for this type is between 7 months and 18 months. Children with this condition can sit independently at diagnosis. However, progressive loss of motor function means they have a reduced life expectancy compared with the general population.

Type 3 SMA, there are varying degrees of muscle weakness, which appear between 18 months and 18 years. People with this condition can have normal lifespan, and walk or sit unaided at some point, but many lose mobility over time.

Type 4 SMA, the least severe, affects adults, who may have only mild motor impairment and live a normal lifespan.

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<sup>1</sup> [Overview | Nusinersen for treating spinal muscular atrophy | Guidance | NICE](#)

## Policy Position Statement:

### PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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It is estimated there are there are between 600 and 1200 children and adults in the UK living with SMA. Clinical experts suggested that, of all diagnosed cases of SMA, around 60% are type 1 and around 40% are types 2 and 3. Types 0 and 4 are rarely diagnosed.

Current treatments for spinal muscular atrophy are based on symptom control and aim to maintain movement and function for as long as possible and to improve quality of life. This involves a multidisciplinary approach including respiratory, gastroenterology and orthopaedic care, as well as nutritional support, physiotherapy, assistive technologies, occupational therapy and social care.

Nusinersen is an antisense oligonucleotide<sup>2</sup>. It is designed to modify the product of the SMN2 “backup” gene to produce more functional SMN protein<sup>3,4</sup>. It is the first available disease-modifying treatment for SMA, which includes all types 1-4<sup>2</sup>.

## 1.2 Equality Impact Assessment

The Equality Impact Assessment (EIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable NHS Wales Joint Commissioning Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

An EIA was carried out by NICE during the evaluation of nusinersen. For further details, please refer to the NICE website at: [Nusinersen for treating spinal muscular atrophy | NICE EQIA](#)

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<sup>2</sup> [Spinraza 12 mg solution for injection - Summary of Product Characteristics \(SmPC\) - \(emc\) | 2715](#)

<sup>3</sup> [Committee discussion | Nusinersen for treating spinal muscular atrophy | Guidance | NICE](#)

<sup>4</sup> <https://smauk.org.uk/more-detail-on-how-nusinersen-works-in-sma>

## 2. Recommendations

The recommendations below represent the views of NICE, arrived at after careful consideration of the evidence available. Health professionals are expected to take into account the relevant NICE guidance<sup>1</sup>, alongside the individual needs, preferences and values of the patient.

### 2.1 Inclusion Criteria

Nusinersen is recommended as an option for treating spinal muscular atrophy (SMA) only if:

- they are a child or young person is aged between 0-17 years old
- have pre-symptomatic SMA, or SMA types 1, 2 or 3, **and**
- the conditions in the managed access agreement are followed

### 2.2 Exclusion Criteria

People aged 18 years and older<sup>5</sup>.

### 2.3 Continuation of Treatment

Healthcare professionals are expected to review a patient's health at regular intervals to ensure they are demonstrating an improvement to their health due to the treatment being given.

If no improvement to a patient's health has been recorded then clinical judgement on the continuation of treatment must be made by the treating healthcare professional.

### 2.4 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

### 2.5 Transition arrangements

Transition arrangements should be in line with [Transition from children's to adults' services for young people using health or social care services NICE guidance NG43 and the Welsh Government Transition and Handover Guidance.](#)

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<sup>5</sup> Commissioning responsibility of nusinersen for people aged 18 years and older lies with Health Boards.

## Policy Position Statement:

### PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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Transition involves a process of preparation for young people and their families for their transition to adulthood and their transition to adult services. This preparation should start from early adolescence 12-13 year olds. The exact timing of this will ideally be dependent on the wishes of the young person but will need to comply with local resources and arrangements.

The transition process should be a flexible and collaborative process involving the young person and their family as appropriate and the service.

The manner in which this process is managed will vary on an individual case basis with multidisciplinary input often required and patient and family choice taken into account together with individual health board and environmental circumstances factored in.

## 2.6 Designated Providers

Patients should be referred for assessment of eligibility for treatment to a Paediatric Neurologist or a Paediatric Neurosurgeon at the following centres:

Children's Hospital for Wales  
University Hospital of Wales  
Health Park Way  
Cardiff  
CF14 4XW

Alder Hey Children's Hospital  
East Prescott Road  
Liverpool  
L14 5AB

## 2.7 Patient Pathway (Annex i)

See annex i for patient pathway.

## 2.8 Mechanism for funding

Nusinersen will only be funded for patients registered via the Blueteq<sup>®</sup> system and where an appropriately constructed MDT has approved its use within highly specialised centres.

Where the patient meets the criteria in this policy and the referral is received by an agreed centre, a Blueteq<sup>®</sup> form should be completed for approval.

For further information on accessing and completing the Blueteq<sup>®</sup> form please contact NWJCC using the following email address: [NWJCCblueteq@wales.nhs.uk](mailto:NWJCCblueteq@wales.nhs.uk).

## Policy Position Statement:

### PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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If a non-contracted provider wishes to treat a patient that meets the criteria they should contact NWJCC at [NWJCCipc@wales.nhs.uk](mailto:NWJCCipc@wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that nusinersen is prescribed and administered in accordance with its marketing authorisation. Nusinersen is available as 12mg/5ml solution for injection vials<sup>6</sup>. The list price is £75,000.00 per 5ml vial (excluding VAT; company's evidence submission). The company has a commercial arrangement. This makes nusinersen available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTTC Commercial Medicines Access References Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

If treatment is discontinued, it is the responsibility of the prescribing team to discontinue the Blueteq<sup>®</sup> form.

## 2.9 Clinical Outcome and Quality Measures

The centre must enable the patient's, carer's and advocate's informed participation and to be able to demonstrate this. Provision should be made for patients with communication difficulties and for children, teenagers and young adults.

## 2.10 Action to be taken

- Providers and NWJCC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.
- Providers are to ensure that all providers are purchasing nusinersen at the agreed discounted price.
- Providers are to ensure the need to approve nusinersen at the appropriate MDT and are registering use on the Blueteq<sup>®</sup> system, and the treatment will only be funded where the Blueteq<sup>®</sup> minimum dataset is fully and accurately populated.
- The provider should work to written quality standards and provide monitoring information to NWJCC on request.

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<sup>6</sup> [Medicinal forms | Nusinersen | Drugs | BNF | NICE](#)

## 3. Putting things right

### 3.1 Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided.

The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern.

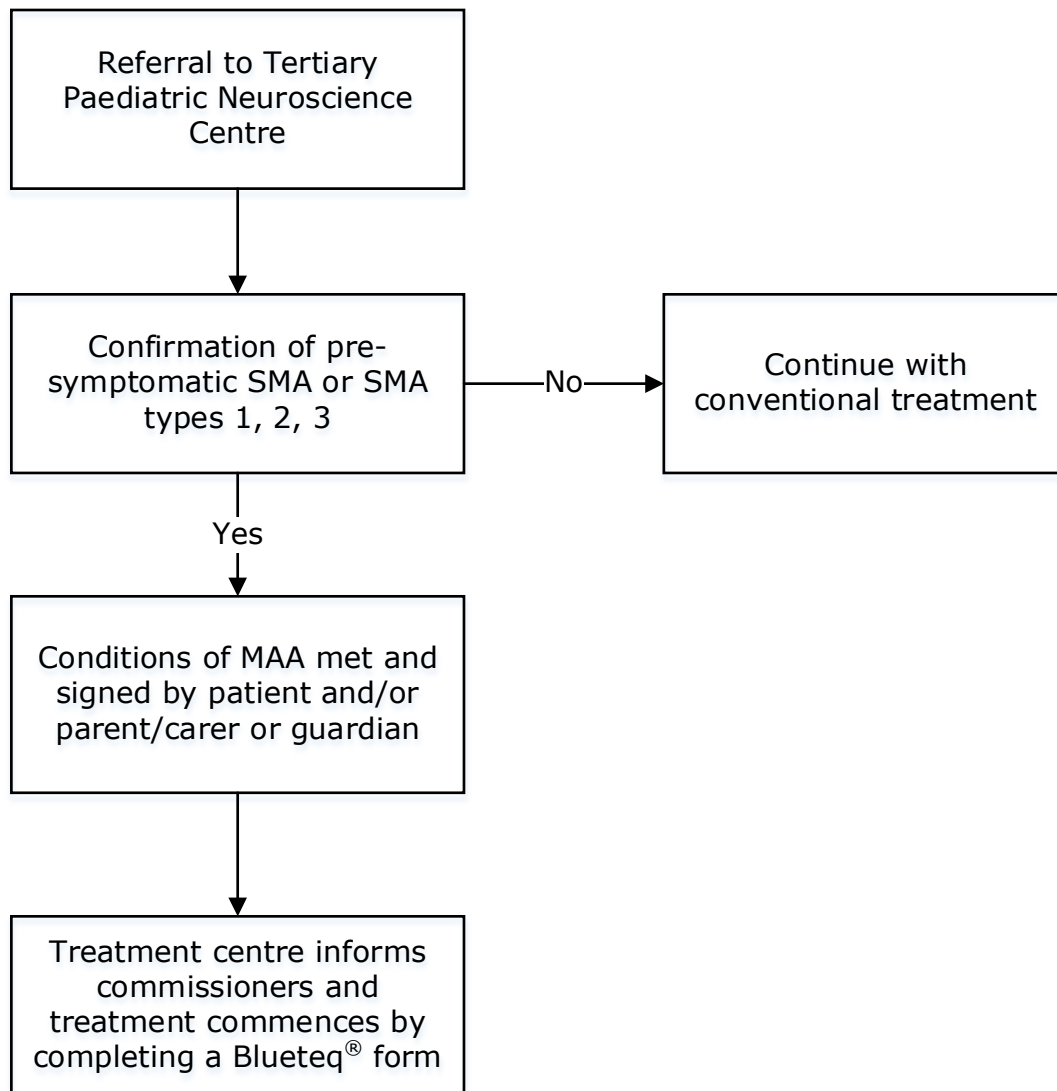
If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for [NHS Putting Things Right](#). For services provided outside NHS Wales the patient or their representative should be guided to the [NHS Trust Concerns Procedure](#), with a copy of the concern being sent to NWJCC.

### 3.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

Further information on making IPFR requests can be found at: [Individual Patient Funding Requests](#)

## Annex i Patient Pathway



Policy Position Statement:

PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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## Annex ii Codes

The list of ICD codes is indicative and is not exhaustive. Additional codes may be used for contract monitoring purposes, furthermore some codes may cover indications not included within this policy.

Code Category	Code	Description
ICD	G12	Spinal muscular atrophy and related syndromes

Policy Position Statement:

PPS191, Nusinersen for treating children and young people up to 17 years old with spinal muscular atrophy

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## Contact Us

If you have a question related to this document you can contact us using one of the methods outlined below.

If you would like this document in an alternative format and/or language, please contact us for assistance.

### Email:

NWJCC consultation mailbox – [nwjccconsultation@wales.nhs.uk](mailto:nwjccconsultation@wales.nhs.uk)

### Telephone:

General Enquiries – 01443 433112

### Website:

[Contact us - NHS Wales Joint Commissioning Committee](#)

### Writing:

If you wish to contact the NHS Wales Joint Commissioning Committee, you can write to us at one of our locations below, we welcome correspondence in Welsh or English:

#### South Wales Offices

Unit 1, Charnwood Court, Heol Billingsley, Nantgarw, CF15 7QZ

Unit G1 The Willowford, Main Avenue, Treforest Industrial Estate, Pontypridd, CF37 5YL

#### North Wales Offices

Unit 3, Media Point - Unit 3, Mold Business Park, Mold, CH7 1XY

Preswylfa, Hendy Road, Mold, CH7 1PZ