GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome in people 6-17 years of age

Policy Position Statement: PPS298

Document Information	
Document Name	Setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome in people 6-17 years of age
Document No	PPS298
Document Purpose	Policy Position Statement
Publication Date	April 2025
Version No	1.0
Commissioning Team Author	Women and Children
Target Audience	Chief Executives, Medical Directors, Directors of Finance, Directors of Pharmacy, Consultant Paediatricians, Genetics, Nephrology, Ophthalmology, Urology, Dietetics, Endocrinology, Gynaecology, Rhythm Pharmaceutical UK Ltd
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy

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Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) propose to commission setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome in people 6-17 years of age 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 judgment, 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.

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 setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome in people aged 6-17 years 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)[1] and has concluded setmelanotide should be made available.

1.1 Background

Bardet Biedl Syndrome (BBS) is a rare genetic disorder with a wide variety of symptoms[2]. These symptoms include, but are not limited to, retinal degeneration (which can lead to blindness), obesity (due to hyperphagia – a feeling of always needing to eat)[1], reduced kidney function, polydactyly (extra digits of the hands or feet). These symptoms are used to diagnose BBS in an individual before genetic testing has been completed or if the genetic testing does not reveal a mutation in a gene known to cause BBS[2].

More than 20 genes are associated with BBS but the underlying cause, regardless of gene involvement, is malfunction of primary cilia[2]. Cilia plays a key role in communication between different cells. BBS is thus categorized as a ciliopathy, or a disease of the cilia.

The variability in presentation and severity of the syndrome together with the rarity of the condition can lead to delayed diagnosis and a lack of adequate local health care[3]. As genetic testing for BBS has improved it is becoming clearer that the symptoms and severity of symptoms are even more diverse than originally thought.

BBS is associated with large quality of life impact due to the aforementioned multiple co-morbidities[4]. Currently, there is no published evidence on life expectancy. However, renal failure and obesity related co-morbidities are thought to be major causes of death.

As there is no cure for BBS and treatment focuses on treating and managing the associated symptoms[3]. As such, multi-disciplinary care is needed. Patients with BBS have access to specialist NHS centres in the United Kingdom (based in London and Birmingham), offering multidisciplinary clinics.

[1] Overview | Setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome | Guidance | NICE
[2] What is BBS? — Bardet Biedl Syndrome Foundation
[3] What is Bardet-Biedl Syndrome? - BBS UK
[4] 1st Evaluation meeting Lead team presentation (nice.org.uk)

Setmelanotide works by binding (attaching) to melanocortin-4 receptors[5]. This receptor works by regulating hunger, satiety (feeling of being full after eating), and energy expenditure. By binding to this receptor setmelanotide decreases hunger and increases satiety and energy expenditure. Together these actions promote weight loss[5].

The incidence of BBS is approximately 1:100,000 babies born[3,4]. By extrapolating for live births in Wales this equates to less than one new case per year. The company (Rhythm Pharmaceuticals) estimates 472 people in England have genetically confirmed BBS. Based on the current Welsh population[6] that equates to a prevalence of around 23 people in Wales.

1.2 Equality Impact Assessment

The Equality Impact Assessment (EQIA) 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 EQIA was carried out by NICE during the evaluation of setmelanotide. For further details, please refer to the NICE website at: NICE EQIA

[3] What is Bardet-Biedl Syndrome? - BBS UK
[4] 1st Evaluation meeting Lead team presentation (nice.org.uk)
[5] Setmelanotide | Drugs | BNF | NICE
[6] Population and household estimates, Wales - Office for National Statistics

2. Recommendations

The NHS Wales Joint Commissioning Committee will approve funding of setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome in people aged 6-17 years of age in line with the criteria outlined in this policy.

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[7], alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

- Setmelanotide is recommended as an option for treating obesity and hyperphagia in genetically confirmed Bardet-Biedl syndrome (BBS) in people aged 6 years and over, only if they are aged between 6 and 17 years when treatment starts. These people can carry on having setmelanotide as adults until they need to stop.
- Setmelanotide is only recommended if the company provides it according to the commercial arrangement.
- This recommendation is not intended to affect treatment with setmelanotide that was started in the NHS before this guidance was published. People having treatment outside this recommendation may continue without change to the funding arrangements in place for them before this guidance was published, until they and their NHS healthcare professional consider it appropriate to stop. For children or young people, this decision should be made jointly by them, their healthcare professional, and their parents or carers.

2.2 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.

2.3 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

[7] Overview | Setmelanotide for treating obesity and hyperphagia in Bardet-Biedl syndrome | Guidance | NICE

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.

2.4 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.

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.

2.5 Designated Providers

Patients will be referred to one of the designated centres below for treatment:

Great Ormond Street Children's Hospital (GOSH)
Great Ormond Street
London
WC1N 3JH

Birmingham Children's Hospital (BCH)
Steelhouse Lane
Birmingham
B4 6NH

2.6 Patient Pathway

Patients should be referred to one of the designated providers listed in section 2.5, for assessment of eligibility for treatment included in this policy.

2.7 Mechanism for funding

Setmelanotide will only be funded for patients registered via the Blueteq® 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® form should be completed for approval.

For further information on accessing and completing the Blueteq® form please contact NWJCC using the following email address: NWJCCblueteq@wales.nhs.uk.

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

Funding is approved on the basis that setmelanotide is prescribed and administered in accordance with its marketing authorisation PLGB 55587/0001, is available as Imcivree® 10mg/ml solution for injection[8]. The cost is £2,376.00 (excluding VAT) for one 10mg/ml solution for injection vial[5]. The company has a commercial arrangement. This makes setmelanotide available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales should refer to the AWTTC Commercial Medicines Access References Tool (CMART) for further information on the Patient Access Scheme (PAS) price.

If treatment with setmelanotide is considered homecare should be utilised for ongoing supply if appropriate.

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

2.8 Clinical Outcome and Quality Measures

The Provider must work to written quality standards and provide monitoring information to the lead commissioner.

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.

[8] IMCIVREE 10 mg/ml solution for injection - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)

2.9 Action to be taken

- Health Boards 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 they are purchasing setmelanotide at the agreed discounted price.
- Providers are to ensure the need to approve setmelanotide at the appropriate MDT and are registering use on the Blueteq® system, and the treatment will only be funded where the Blueteq® minimum dataset is fully and accurately populated.
- The Provider should work to written quality standards and provide monitoring information to NWJCC on request.

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.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated

If the patient wishes to be referred to a provider outside of the agreed pathway, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

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

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