GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

Burosumab for X-linked hypophosphataemia (XLH)

Policy Position Statement: PPS177

Document Information	
Document Name	Burosumab for X-linked hypophosphataemia
Document No	PPS177
Document Purpose	Policy Position Statement
Publication date	January 2026
Version No	3.0
Commissioning Team Author	Neurosciences and Long Term Conditions
Target Audience	Chief Executives, Medical Directors, Directors of Finance, Directors of Pharmacy, Rheumatologists, Nephrologists, Endocrinologists, Kyowa Kirin Ltd.
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document Update Information	Moved to new NWJCC template NWJCC commissioning for adults added to policy

Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission burosumab for people with X-linked hypophosphataemia (XLH) 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.

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

1. Introduction

This Policy Position has been developed for the planning and delivery of burosumab for people resident in Wales with XLH. 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)[1,2] and has concluded that burosumab should be made available.

1.1 Background

X-linked hypophosphataemia is a rare genetic condition that causes significant skeletal deformities in young children, continuing into adulthood and resulting in lifelong disability and pain. Because XLH is a genetic condition, it often affects several members of the same family[1].

The genetic mutation which causes XLH occurs in the PHEX gene, which is responsible for maintaining the right balance of phosphate in the body. Due to this genetic anomaly, people with XLH show an increased synthesis of the bone-derived phosphaturic hormone fibroblast growth factor 23 (FGF23), which results in renal phosphate wasting[3]. Phosphate has many physiological functions, one of which being an important contributor to the formation and growth of bones in childhood and bone strength in adults. The kidneys regulate the phosphate levels in the body by excreting excess phosphate in the urine and reabsorbing any additional phosphate required into the bloodstream. In individuals with XLH, the kidneys cannot reabsorb phosphate effectively which causes too much phosphate to be excreted from the body through the urine. Due to this, not enough phosphate is available in the bloodstream for normal bone development[4].

Skeletal abnormalities such as bowed or bent legs, below average height and irregular growth of the skull are early signs of XLH[1]. Children may also present with delayed walking or waddling gait. Bone defects are common in children with XLH, and can cause pain and subsequently limit physical functioning. When bone growth stops, bone deformities become irreversible and can be the source of ongoing pain. Other manifestations of XLH include dental problems and hearing loss. Adults with XLH often have a high disease burden and can have symptoms such as osteomalacia (softening of bones due to low phosphate and consequent impaired mineralization resulting in

[1] Overview | Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance | NICE
[2] Overview | Burosumab for treating X-linked hypophosphataemia in adults | Guidance | NICE
[3] Clinical practice recommendations for the diagnosis and management of X-linked hypophosphataemia
[4] X-linked Hypophosphataemia - Metabolic Support UK

fractures), misalignment of joints, early-onset osteoarthritis and chronic musculoskeletal pain and stiffness, impaired mobility and psychosocial impact. Adult patients can also suffer from enthesopathy (calcification of tendons, ligaments and joint capsules) and, in rare cases, spinal stenosis (a severe late complication which can be painful and debilitating).

Conventional therapy consists of oral phosphate and active vitamin D (such as alfacalcidol) supplements[1]. Oral phosphate has a complex dosing regimen, disagreeable taste and unpleasant side effects.

Burosumab is a human monoclonal antibody that prevents the activity of fibroblast growth factor 23 (FGF23), thereby increasing reabsorption of phosphate through the kidneys and increasing blood concentration of vitamin D[5]. It is administered as a subcutaneous injection every four weeks.

The incidence of XLH is around 1 in 20,000[4]. Extrapolating for Welsh population data, by assuming a live birth rate of 28,000 in Wales per year, it means there is going to be less than 1 new case in Wales per year. The prevalent adult population in England with XLH is estimated around 300 adults, however the figure may be closer to 1000 when including unregistered and undiagnosed XLH[2]. Extrapolating for the Welsh population that equates to between 15 and 50 adults.

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 EIA was carried out by NICE during the evaluation of burosumab. For further details, please refer to the NICE website at: NICE website

[1] Overview | Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance | NICE
[2] Overview | Burosumab for treating X-linked hypophosphataemia in adults | Guidance | NICE
[3] X-linked Hypophosphataemia - Metabolic Support UK
[5] Burosumab | Drugs | BNF | NICE

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[1, 2], alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Burosumab for treating X-linked hypophosphataemia in children and young people (HST8)[1]

Burosumab is recommended, within its marketing authorisation, for treating X-linked hypophosphataemia (XLH) with radiographic evidence of bone disease in babies, children and young people 1 to 17 years. It is recommended only if the company provides burosumab according to the commercial arrangement.

Burosumab for treating X-linked hypophosphataemia in adults (TA993)[2]

Burosumab is recommended, within its marketing authorisation, as an option for treating X-linked hypophosphataemia (XLH) in adults. Burosumab is only recommended if the company provides it according to the commercial arrangement.

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 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

Paediatric Providers

Noah's Ark Children's Hospital for Wales
University Hospital of Wales
Cardiff and Vale University Health Board
Heath Parc
Cardiff
CF14 4XW

Birmingham Children's Hospital NHS Foundation Trust
Steelhouse Lane
Birmingham
B4 6NH

Alder Hey Children's NHS Foundation Trust
Eaton Road
Liverpool
L12 2AP

Adults Providers

University Hospital of Wales
Heath Parc
Cardiff
CF14 4XW

University Hospital Llandough
Penlan Road

Penarth
CF64 2XX

The Robert Jones and Agnes Hunt Orthopaedic Hospital
Oswestry
Shropshire
SY10 7AG

2.6 Patient Pathway (Annex i)

Please see pathway in Annex i.

Designated provider sites offer both face to face and telephone consultations.

2.7 Mechanism for funding

Burosumab 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 burosumab is prescribed and administered in accordance with its marketing authorisation. Burosumab is available as 10mg solution for injection, 20mg solution for injection and 30mg solution for injection[6] with the cost being £2,992.00, £5,984.00 and £8,976.00 respectively (excluding VAT; company's evidence submission)[7]. The company has a commercial arrangement. This makes burosumab 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 is discontinued, it is the responsibility of the prescribing team to discontinue the Blueteq® form.

[6] EMC - burosumab
[7] Medicinal forms | Burosumab | Drugs | BNF | NICE

2.8 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 burosumab at the agreed discounted price.
- Providers are to ensure the need to approve burosumab 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.

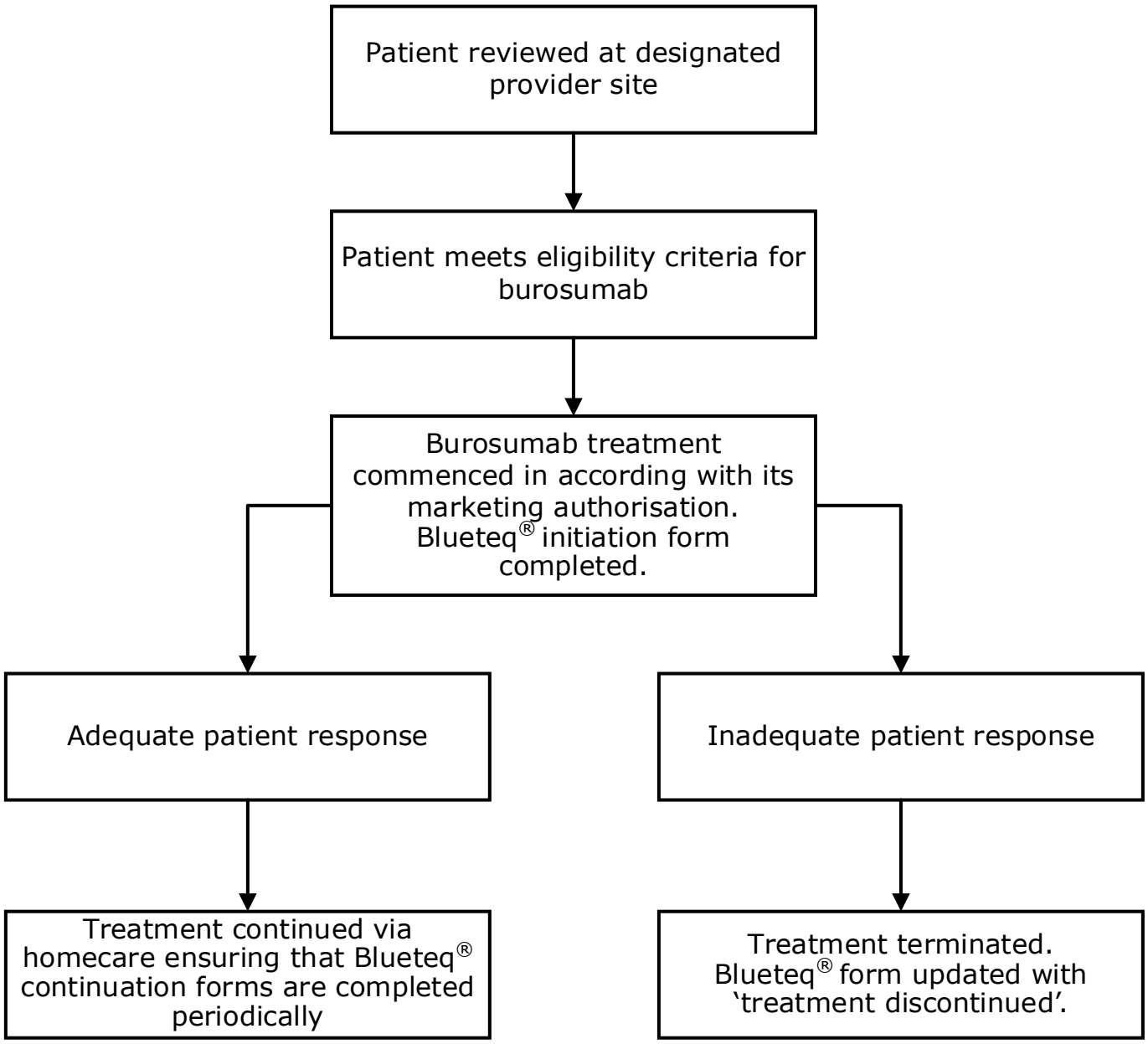
If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales 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.

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

Annex i Patient Pathway



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-10	E83.3	Disorders of phosphorus metabolism and phosphatases
		• Acid phosphatase deficiency
		• Familial hypophosphataemia
		• Hypophosphatasia
		• Vitamin-D-resistant:
		  ○ osteomalacia
		  ○ rickets

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