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Welsh Health Specialised
Services Committee (WHSSC)

Specialised Services

Policy Position PP177

Burosumab for treating X-linked hypophosphataemia in children and young people

May 2019

Version 2.0

Document information	
Document purpose	Policy Position
Document name	Burosumab for treating X-linked hypophosphataemia in children aged 1 year and over and in young people with growing bones.
Author	Welsh Health Specialised Services Committee
Publication date	May 2019
Commissioning Team	Women and Children
Target audience	Chief Executives, Medical Directors, Directors of Finance, Chief Pharmacists
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
Document No	PP177
Review date	2023

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Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission Burosumab for children aged 1 year and over and young people with growing bones, with X-linked hypophosphataemia in accordance with the criteria outlined in this document.

In creating this document WHSSC has reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE) and has concluded that Burosumab should be made available¹.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy 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.

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

¹ [Overview | Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance | NICE](#)

1. Introduction

This Policy Position statement has been developed for the planning and delivery of Burosumab for people resident in Wales. This service will only be commissioned by the Welsh Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

X-linked hypophosphataemia (XLH) is a genetic condition that causes significant skeletal deformities in children.

Burosumab is a drug that is administered by injection to reduce abnormal bone growth by allowing the level of phosphate and calcium in the body to return to more normal level, therefore preventing increased change of deformity in bone.

1.2 Aims and Objectives

This Policy Position statement aims to define the commissioning position of WHSSC on the use of Burosumab for people with X-linked hypophosphataemia.

The objectives of this policy are to:

- ensure commissioning for the use of Burosumab is evidence based
- ensure equitable access to Burosumab
- define criteria for people with X-linked hypophosphataemia to access treatment
- improve outcomes for people with X-linked hypophosphataemia

1.3 Epidemiology

X-linked hypophosphataemia (XLH) is the most common form of hereditary hypophosphatemia with a prevalence of approximately 1 in 20,000. The disease affects both sexes equally and is normally diagnosed in early childhood.

As XLH is a genetic disease, it often affects several members of a family. Skeletal abnormalities such as bowed or bent legs, below average height and irregular growth of the skull are early signs of XLH. 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 continuing pain. Other manifestations of XLH include dental problems and hearing loss².

² [Overview | Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance | NICE](#)

1.4 Current Treatment

Current treatment options for X-linked hypophosphataemia are vitamin D supplementation and oral phosphate. Oral phosphate has a complex dosing regimen (often with dosing 4 to 6 times a day), disagreeable taste and unpleasant side effects. People with XLH often need orthopaedic surgery to correct bone deformities.

1.5 New Treatment

Burosumab is administered via subcutaneous injection once every 2 weeks. The recommended starting dose is 0.4 mg/kg, the normal maintenance dose is 0.8 mg/kg and the maximum dose is 2 mg/kg up to 90 mg. Doses should be rounded to the nearest 10 mg. Treatment can begin in children aged 1 year and over and can continue until the bones stop growing. The aim of the purposed treatment is to improve growth, decreased morbidity, prevent skeletal deformities and reduce pain.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by National Institute of Health and Care Excellence (NICE)³ and have concluded that that the use of Burosumab should be made available within the criteria set out in section 2...

³ [Overview | Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance | NICE](#)

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of Burosumab for children aged 1 year and over and young people with growing bones, with X-linked hypophosphataemia, in-line with the criteria identified in the policy.

2.1 Inclusion Criteria

Burosumab is recommended, within its marketing authorisation, for treating X-linked hypophosphataemia (XLH) with radiographic evidence of bone disease in children aged 1 year and over, and in young people with growing bones. It is recommended only if the company provides burosumab according to the [commercial arrangement](#)⁴.

2.2 Exclusion Criteria

Burosumab is only licensed for children aged 1-17 years. Therefore this policy excludes adults aged 18 years and older⁵.

2.3 Stopping Criteria

The adverse reactions listed as very common (that is, occurring in 1 in 10 people or more) in the summary of product characteristics⁶ for Burosumab include:

- injection site reactions
- headache
- pain in the extremities
- decreased vitamin D
- rash
- toothache
- tooth abscesses
- myalgia and dizziness

For full details of adverse reactions and contraindications, see the summary of product characteristics⁵.

NICE acknowledged that, if the symptoms of XLH were to return after stopping Burosumab, there may be consideration of whether continued treatment would be beneficial³. They also noted that people are given the opportunity to stop conventional therapy when bone growth stops.

⁴ <https://www.nice.org.uk/guidance/hst8>

⁵ <https://www.ema.europa.eu/en/medicines/human/EPAR/crysvita>

⁶ [Burosumab for treating X-linked hypophosphataemia in children and young people | Guidance and guidelines | NICE](#)

2.4 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.

2.5 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.

2.6 Patient Pathway (Annex i)

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

Cardiff	Cardiff and Vale University Health Board University Hospital of Wales Cardiff CF14 4XW
Bristol	Bristol Royal Hospital for Children Upper Maudin Street Bristol BS2 8BJ
Birmingham	Birmingham Children's Hospital NHS Foundation Trust Steelhouse Lane Birmingham B4 6NH
Liverpool	Alder Hey Children's NHS Foundation Trust Eaton Road Liverpool L12 2AP
Sheffield	Sheffield Children's Hospital Western Bank Sheffield S10 2TH

2.7 Exceptions

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 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: [Welsh Health Specialised Services Committee \(WHSSC\) | Individual Patient Funding Requests](#)

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.

2.9 Responsibilities

Referrers should:

- inform the patient that this treatment is not routinely funded outside the criteria in this policy, and
- refer via the agreed pathway.

Clinician considering treatment should:

- discuss all the alternative treatment with the patient
- advise the patient of any side effects and risks of the potential treatment
- inform the patient that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with WHSSC for the treatment.

In all other circumstances an IPFR must be submitted.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **National Institute of Health and Care Excellence (NICE) guidance**
 - [Burosumab for treating X-linked hypophosphataemia in children and young people](#). NICE, Highly Specialised Technologies Guidance HST8. 10th October 2018.

This document should be read in conjunction with the following documents:

- **NHS Wales**
 - All Wales Policy: [Making Decisions in Individual Patient Funding requests](#) (IPFR).
- **WHSSC policies and service specifications**
 - CP163, Paediatric Endocrinology, Publication Date TBC

4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

5. Putting Things Right: Raising a Concern

5.1 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 WHSSC.

5.2 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, an IPFR should be submitted.

Further information on making IPFR requests can be found at: [Welsh Health Specialised Services Committee \(WHSSC\) | Individual Patient Funding Requests](#)

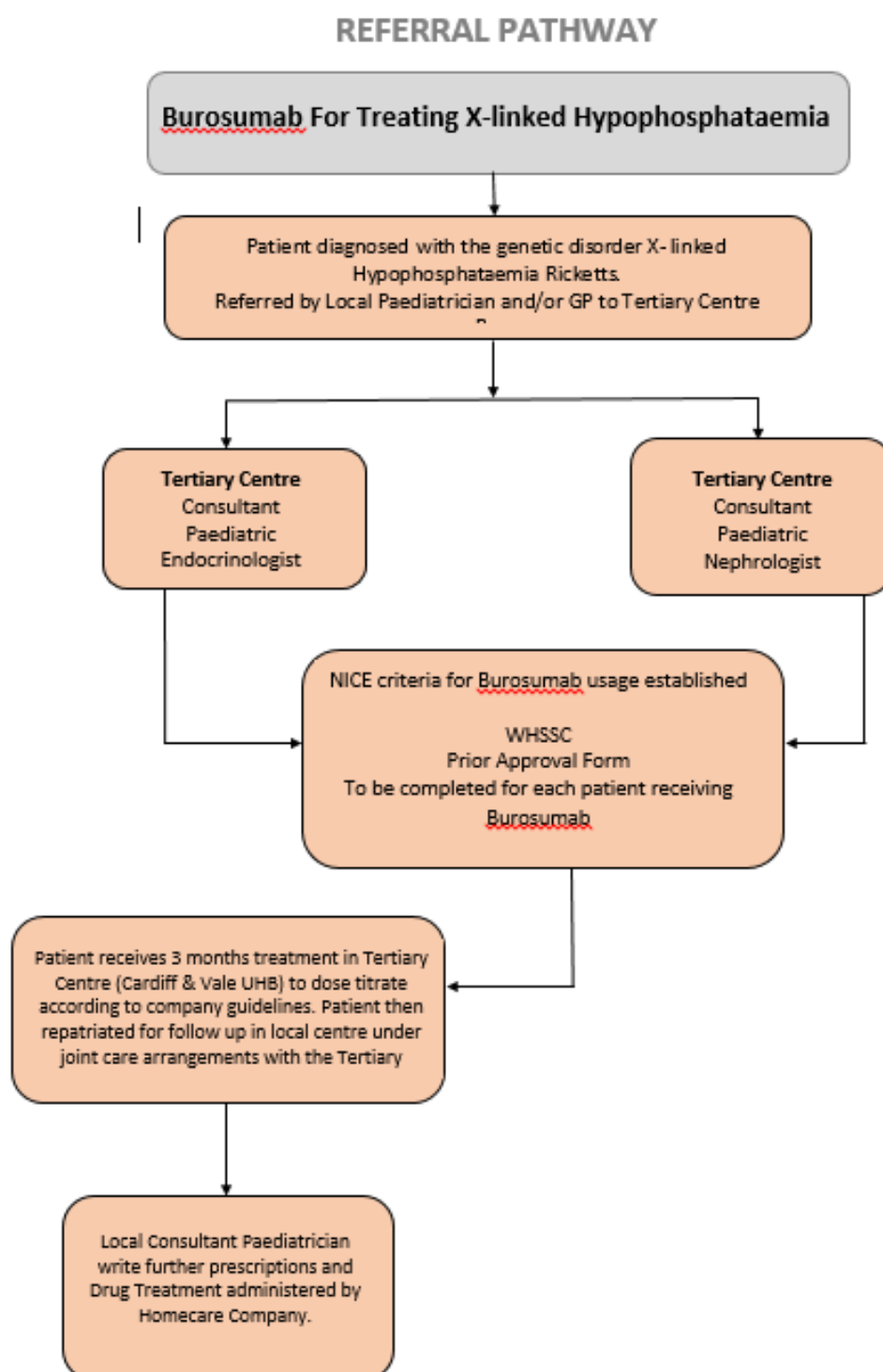
6. Equality Impact and Assessment

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 Welsh Health Specialised Services 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).

This policy has been subjected to an Equality Impact Assessment.

The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

Annex i Patient Pathway



Annex ii Checklist

Burosumab for treating X-linked hypophosphataemia in children and young people

The following checklist should be completed for every patient to whom the policy applies:

- Where the patient meet the criteria **and** the procedure is included in the contract **and** the referral is received by an agreed centre, form should be completed and retained by the receiving centre for audit purposes.
- The patient meets the criteria **and** is received at an agreed centre, but the procedure is not included in the contract. The checklist must be completed and submitted to WHSSC for prior approval to treatment (Appendix 1).
- The patient meets the criteria but wishes to be referred to a non-contracted provider. An Individual Patient Funding Request (IPFR) Form must be completed and submitted to WHSSC for consideration.
- 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.

Annex iii Abbreviations and Glossary

Abbreviations

IPFR	Individual Patient Funding Request
WHSSC	Welsh Health Specialised Services
NICE	National Institute of Health and Care Excellence
XLH	X-linked hypophosphataemia

Glossary

Individual Patient Funding Request (IPFR)

An IPFR is a request to Welsh Health Specialised Services Committee (WHSSC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

Welsh Health Specialised Services Committee (WHSSC)

WHSSC is a joint committee of the seven local health boards in Wales. The purpose of WHSSC is to ensure that the population of Wales has fair and equitable access to the full range of Specialised Services and Tertiary Services. WHSSC ensures that specialised services are commissioned from providers that have the appropriate experience and expertise. They ensure that these providers are able to provide a robust, high quality and sustainable services, which are safe for patients and are cost effective for NHS Wales.

X-linked hypophosphataemia (XLH)

A genetic condition that causes significant skeletal deformities in children.

Appendix 1 Prior Approval Request Form



PRIOR APPROVAL REQUEST FORM

Details of clinician making the referral:	
Name:	
Designation:	
Address:	
Postcode:	
Telephone number:	
Email:	
Secretary name:	
Telephone:	Email:

Patient Details	
First name:	Last name:
Address:	Date of birth:
	NHS number:
Postcode:	Hospital number:

Urgency			
How urgent is the request? (tick as applicable)	Urgent: 24-48 hours	Soon: Within 3 weeks	Non-urgent: 4-6 weeks

Please note: If a decision is required urgently, clinical reasons must be provided. Administrative reasons will not be considered.

Reason for request
<input type="checkbox"/> NICE Approved Drugs <input type="checkbox"/> NICE Technology Appraisals and Highly Specialised Technology Appraisals <input type="checkbox"/> AWMMSG Health Technology Appraisals (including the orphan and ultra-orphan status)

Clinical details
Details of treatment requested (including weight of patient, dosage and duration)
Medical history and current clinical status -: (Please provide a copy of the latest clinical report)
Additional information to support the referral: (e.g. relevant clinical letters/reports)
Cost of treatment:

I confirm that as the patient's Consultant, I have discussed this application and consent has been provided to obtain further clinical information pertinent to this funding request if required.
Clinicians signature:
Date:

Please return this form with a copy of the referral letter to:
Please return completed form to: Patient Care Team Welsh Health Specialised Services , 3a Caerphilly Business Park, CF83 3ED Email: whssc.ipc@wales.nhs.uk or whssc.ipc@nhs.net
If you have any questions, please telephone 01443 443443 ext.78123