GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu Joint Commissioning Committee

Birch bark extract for treating epidermolysis bullosa in people aged 6 months and older

Policy Position Statement: PPS284

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Document information	
Document purpose	Policy Position Statement
Document name	Birch bark extract for treating epidermolysis bullosa in people aged 6 months and older
Author	NHS Wales Joint Commissioning Committee
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Target audience	Chief Executives, Medical Directors, Directors of Finance, Directors of Planning, Chief Pharmacists, Clinical Leads, Paediatric Dermatology Consultants, Dermatologists, Wound Care Management Consultants
Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
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Contents

Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission birch bark extract for people aged 6 months and older with epidermolysis bullosa 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 birch bark extract for people aged 6 months and older with epidermolysis bullosa 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 that birch bark extract should be made available.

1.1 Background

Epidermolysis bullosa (EB) is a group of rare connective tissue diseases that cause the skin to be fragile and to blister easily. Most people who have epidermolysis bullosa inherit a mutated gene from their parents. The gene mutation means certain skin anchoring proteins are not expressed correctly or are disrupted. This results in very fragile skin that blisters and breaks frequently, particularly in response to minor trauma or friction. Many of the wounds that happen in EB are partial thickness wounds, meaning they extend through multiple different layers of the skin surface[1].

The body surface area percentage (BSAP) score is one way of measuring EB disease severity. This, however, only describes the amount of the skin surface covered in wounds; it does not capture the impact or location of wounds, and does not describe other aspects of the condition, such as damage to the gastrointestinal tract. In addition to the direct symptoms of EB, there is an increased risk of infections and squamous cell carcinoma, and of nutritional issues linked to effects on the mucosal surfaces of the gastrointestinal tract. EB can therefore have substantial effects on quality of life[1, 2].

There are currently no licensed disease-modifying treatments for EB, and treatment options aim only to manage symptoms. Symptom management has three broad categories[1].

1. Wound management: including bathing to wash wounds, lancing and draining blisters and using non-adhesive dressings and bandages to manage open wounds. Topically applied steroid creams and antimicrobial creams (in the event of infections) are also used off label.
2. Surgical procedures: used to manage complications of EB such as fusion of fingers and oesophagael structures.

[1] Overview | Birch bark extract for treating epidermolysis bullosa | Guidance | NICE
[2] committee-papers (nice.org.uk)

3. Pain management: includes pharmacological and non-pharmacological interventions to manage the pain from EB and from surgical and wound management procedures.

Birch Bark Extract (Filsuvez®) is a non-aqueous gel, which contains a dry extract from two species of birch bark consisting of naturally occurring substances. These are known as triterpenes, including betulin, betulinic acid, erythrodiol, lupeol and oleanolic acid. The precise mechanism of action of the active substance in wound healing is not known. Cell culture assays with human primary keratinocytes and fibroblasts, and *ex vivo* studies with porcine skin, show that the extract, including the main component betulin, modulate inflammatory mediators and are associated with activation of intracellular pathways known to be involved in keratinocyte differentiation and migration, wound healing, and closure[3,4].

Information on the epidemiology of EB is limited. Throughout the European Union (EU), it is estimated that EB (all subtypes) affects less than 50 per 1 million[2]. DEB and JEB (the focus of this position policy) are life-long, chronic, inherited disorders. For the EB subtypes corresponding to the licensed indication of birch bark extract, an incidence of 14.4 and 3.7 per million is suggested for patients living in the UK with DEB and JEB, respectively. Assuming constant prevalence across the constituent countries of the UK, estimates suggest patient numbers in Wales of 32 DEB patients and 3 JEB patients[5,6]

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 birch bark extract. For further details, please refer to the NICE website at: Committee discussion | Birch bark extract for treating epidermolysis bullosa | Guidance | NICE

[3] Filsuvez, common name-birch bark extract (europa.eu)
[4] Stevenson M, Carroll C, Ren S, Rawdin A, Ren S, Clowes M. Birch bark extract for treating skin wounds associated with dystrophic and junctional epidermolysis bullosa. A Single Technology Appraisal: School of Health and Related Research (ScHARR); 2023.
[5] Petrof G, Papanikolaou M, Martinez AE, Mellerio JE, McGrath JA, Bardhan A, et al. The epidemiology of epidermolysis bullosa in England and Wales: data from the national epidermolysis bullosa database. Br J Dermatol 2022; 186:843-8.
[6] committee-papers (nice.org.uk)

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

2.1 Inclusion Criteria

Birch bark extract is recommended, within its marketing authorisation, as an option for treating partial thickness wounds associated with dystrophic and junctional epidermolysis bullosa in people aged 6 months and over. It is only recommended if the company provides it according to the commercial arrangement[8].

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.

[7] Overview | Birch bark extract for treating epidermolysis bullosa | Guidance | NICE

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

Treatment with birch bark extract will be initiated and overseen by four specialist centres:

- Birmingham Women's and Children's NHS Foundation Trust
- University Hospitals Birmingham NHS Foundation Trust
- Great Ormond Street Hospital for Children NHS Foundation Trust
- Guy's and St Thomas' NHS Foundation Trust

2.6 Patient Pathway (Annex i)

Once initiated, birch bark extract gel should be applied at each wound dressing change by the patient or their carer.

2.7 Mechanism for funding

Birch bark extract 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.

Birch bark extract (Filsuvez®) gel is for cutaneous application only, and should be applied to the wound surface at a thickness of approximately 1 mm, then covered by a sterile non-adhesive wound dressing. The gel should be in direct contact with the wound and applied at each wound dressing change.

Each tube contains 23.4g of cutaneous gel (for single use only) at a cost of £275.33 excluding VAT per tube. The company has a commercial access agreements. This makes Birch Bark extract 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 CMART for further information on the Patient Access Scheme (PAS) price.

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.
- NWJCC are to ensure that all providers are purchasing birch bark extract at the agreed discounted price.
- Providers are to ensure the need to approve birch bark extract 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.
- Providers are to determine estimated patients numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme EAMS.
- 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)

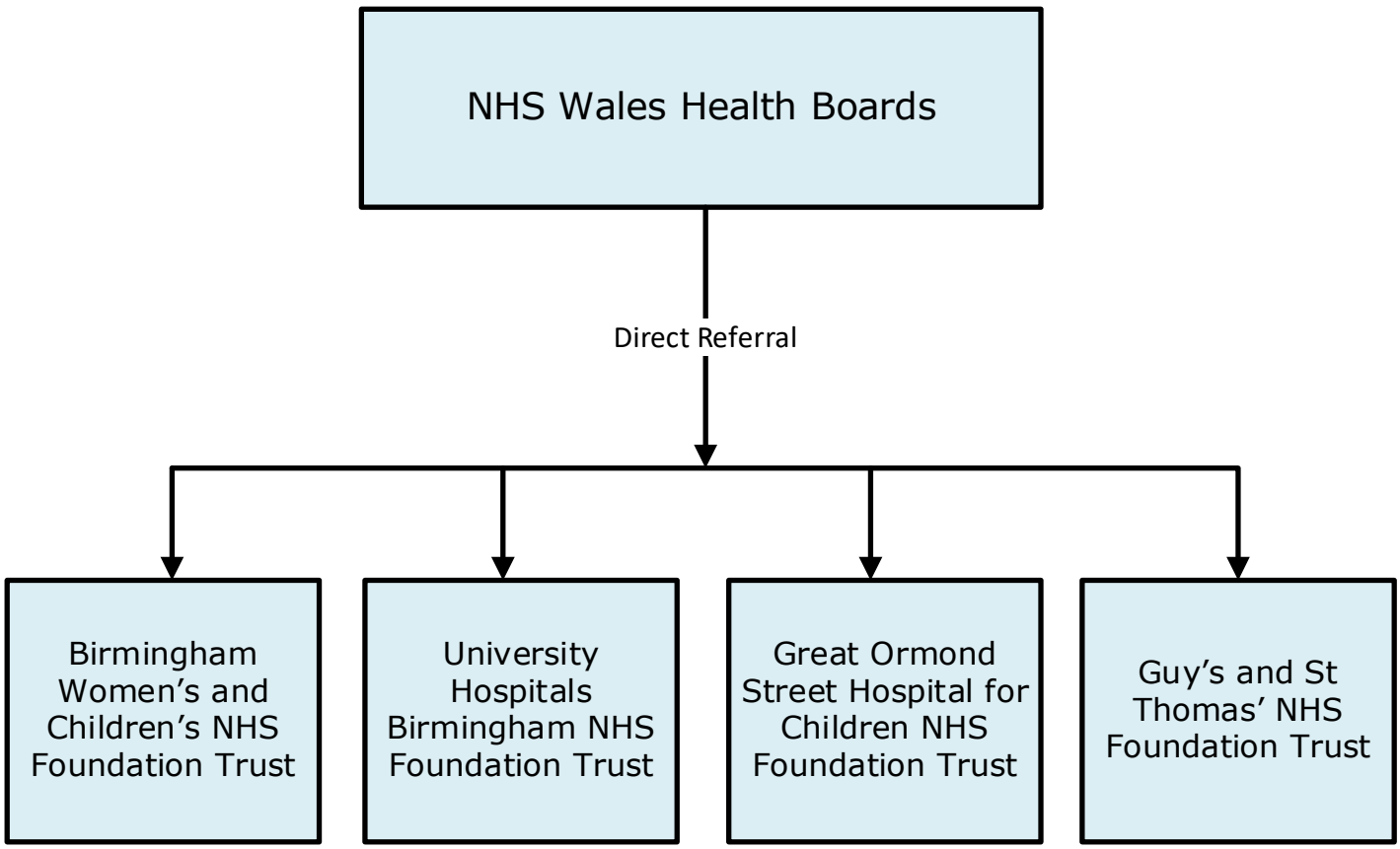
If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All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

Annex i Patient Pathway



Annex ii Codes

Code Category	Code	Description
Q81	Q81.9	Epidermolysis bullosa, unspecified
Q81	Q81.2	Epidermolysis dystrophica