NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technology Evaluation

Birch bark extract for treating skin wounds associated with dystrophic and junctional epidermolysis bullosa [ID1505]

Final Scope

Remit/evaluation objective

To appraise the clinical and cost effectiveness of birch bark extract within its marketing authorisation for treating skin wounds associated with dystrophic and junctional epidermolysis bullosa.

Background

Epidermolysis bullosa (EB) is a general term used to describe a group of rare inherited skin disorders that cause the skin to become very fragile. Any trauma or friction can cause the skin to blister and tear easily. There are different types of EB, and the condition is classified according to where on the body the blistering takes place and which layer of skin is affected[1]. Symptoms can vary significantly by subgroup:

- dystrophic epidermolysis bullosa (DEB) – accounts for around 25%[2] of cases and can be either dominantly or recessively inherited. Dominant DEB is the mildest form of DEB, with recessive DEB associated with more severe symptoms. Blistering occurs below the basement membrane zone in the upper part of the dermis (lower layer of the skin). In mild forms blistering is limited to the hands, feet, knees, and elbows, but may be widespread in more severe cases.

- junctional epidermolysis bullosa (JEB) – the rarest and most severe type is classified as either 'generalised severe' (Herlitz) or 'generalised intermediate' (non-Herlitz). In the severe form blistering can cover large regions of the body including the lining of the mouth and digestive tract. Around 40% of children born with generalized severe (Herlitz) JEB, the more severe form of JEB, will not live past the first year and most won't survive five years[1].

- epidermolysis bullosa simplex (EBS) – accounts for 70%[2] of cases and tends to be milder, although blisters can occur anywhere on the body they are often confined to the palms and soles. EBS has a low risk of serious complications.

- Kindler Syndrome is also a rare type of EB which is characterised by blisters which are formed in different layers of the skin and symptoms such as pigmentation and photosensitivity.

As well as external blisters, EB can manifest internally affecting areas such as the eye, mouth or stomach. Other complications associated with EB can include the development of aggressive skin cancers, dental problems, or nutritional compromise.

EB is usually diagnosed in babies and children and is thought to affect 1 in 17,000 births with around 5,000 people affected in the UK[3].

There is currently no cure for EB. Treatments help ease and control symptoms. It aims to avoid skin damage, improve quality of life and reduce the risk of developing complications such as infection and malnutrition[1]. Given the complex needs of children with EB, treatment is usually carried out by a multidisciplinary team.

The technology

Birch bark extract (Filsuvez, Amryt Pharma) consists of an active ingredient of dry, refined extract from birch bark. Oleogel-S10 contains 10% of birch bark extract in 90% of sunflower oil. It is applied topically.

Birch bark extract has a marketing authorisation in Great Britain for treating partial thickness wounds associated with dystrophic and junctional epidermolysis bullosa (EB) in patients 6 months and older.

Intervention(s)	Birch bark extract
Population(s)	People aged 6 months and older with: • Dystrophic epidermolysis bullosa (DEB) or • Junctional epidermolysis bullosa (JEB)

Subgroups	If the evidence allows the following subgroups will be considered. These include:
	• Dystrophic epidermolysis bullosa (DEB)
	  ○ dominant DEB
	  ○ recessive/severe generalised DEB
	• Junctional epidermolysis bullosa (JEB)
	  ○ generalised severe (Herlitz)
	  ○ generalised intermediate (non-Herlitz)

Comparators	Current clinical management without birch bark extract (including, but not limited to, treatments which can help ease and control infections, pain and other aspects of EB)
Outcomes	The outcome measures to be considered include: • closures of unhealed target wounds • time to wound closure • percentage of surface area of wound healed • change in total body wound burden • incidence and severity of wound infection • pain • change in itching

	• mortality • adverse effects of treatment • health-related quality of life (for patients and carers)
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	None
Related National Policy	The NHS Long Term Plan, 2019. NHS Long Term Plan NHS England (2018/2019) NHS manual for prescribed specialist services (2018/2019) Chapter 50. Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 1,2 and 4. https://www.gov.uk/government/publications/nhs-outcomes-framework-2016-to-2017

References

1. NHS choices. Epidermolysis bullosa. Available at https://www.nhs.uk/conditions/epidermolysis-bullosa/ (assessed 12th March 2020)

2. DEBRA. Epidermolysis bullosa/ Available at https://www.debra.org.uk/types-of-eb/intro (accessed 12th March)

3. Mellerio JE; Epidermolysis bullosa care in the United Kingdom. Dermatol Clin. 2010 Apr28(2):395-6, xiv. doi: 10.1016/j.det.2010.02.015.