NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Beremagene geperpavec for treating skin wounds associated with dystrophic epidermolysis bullosa

Final scope

Remit/evaluation objective

To appraise the clinical and cost effectiveness of beremagene geperpavec within its marketing authorisation for treating skin wounds associated with dystrophic 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] Dystrophic epidermolysis bullosa (DEB) can be either dominantly or recessively inherited.[2] DEB is a group of diseases in which blisters heal with dystrophic scarring. Milia (tiny white spots), result from damage to hair follicles. Symptoms can vary significantly by subgroup:

- Dominantly inherited DEB (DDEB) – appears at birth or infancy with localised or widespread blistering.

- Recessively inherited DEB (RDEB) – can be mild or severe. Severe RDEB is characterised by widespread blistering at birth followed by extensive dystrophic scarring, especially on the extremities. This can cause deformity of the hands and feet. The degree of severity depends on the specific mutation that causes DEB along with environmental factors.

As well as external blisters, EB can manifest internally affecting areas such as the eyes, mouth or oesophagus. Other complications associated with EB can include the development of aggressive skin cancers, dental problems, or malnutrition. More severe forms of EB are associated with the presence of many chronic wounds which need specialised atraumatic dressings. EB is usually diagnosed in babies and children and is thought to affect 1 in 17,000 births.[3] In March 2021, 2,361 people in England and Wales were registered as having all forms of EB.[4] DEB prevalence in England is estimated to be approximately 569 people.[4]

There is currently no cure for EB. Aims of treatments are to control symptoms, avoid skin damage, improve quality of life and reduce the risk of developing complications such as infection and malnutrition.[1] NICE highly specialised technology guidance 28 recommends birch bark extract as an option for treating partial thickness wounds associated with dystrophic and junctional epidermolysis bullosa in people aged 6 months and over. Given the complex needs of children with EB, treatment is usually carried out by a multidisciplinary team. For some, treatment can be carried out at home with clinical oversight given by specialised EB services.

The technology

Beremagene geperpavec (B-VEC) does not currently have a marketing authorisation in the UK for treating wounds in people with dystrophic epidermolysis bullosa. It has been studied in a clinical trial in people aged 6 months and over with dystrophic epidermolysis bullosa compared with placebo.

Intervention(s)	Beremagene geperpavec
Population(s)	People with dystrophic epidermolysis bullosa
Subgroups	If the evidence allows the following subgroups will be considered: • dominant dystrophic epidermolysis bullosa • recessive dystrophic epidermolysis bullosa
Comparators	Established clinical management without beremagene geperpavec including, but not limited to: • treatments which can help ease and control infections, pain and other aspects of DEB • birch bark extract
Outcomes	The outcome measures to be considered include: • proportion of complete wound healing • time to wound closure • duration of wound closure • percentage of body surface area with wounds • pain • change in itching • incidence of squamous cell carcinoma • mortality • adverse effects of treatment • health-related quality of life.
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	**Related highly specialised technology appraisals:** Birch bark extract for treating epidermolysis bullosa (2023) NICE highly specialised technology guidance 28.

References

1. NHS (2021). Epidermolysis bullosa. Available at https://www.nhs.uk/conditions/epidermolysis-bullosa/

2. DEBRA (2024). Epidermolysis bullosa. Available at What is EB? | DEBRA UK

3. Mellerio JE; Epidermolysis bullosa care in the United Kingdom. Dermatol Clin. 2010 Apr28(2):395-6

4. Petrof G, et al. The epidemiology of epidermolysis bullosa in England and Wales: data from the national epidermolysis bullosa database. Br J Dermatol. 2022 May;186(5):843-848.