NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Beremagene geperpavec for treating skin wounds associated with dystrophic epidermolysis bullosa

Draft scope

Draft 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) accounts for around 25% of cases and 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 widespread blistering. With increasing age, blistering becomes more localised.

- Recessively inherited DEB (RDEB) – can be mild or severe. Severe RDEBis 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 stomach. Other complications associated with EB can include the development of aggressive skin cancers, dental problems, or malnutrition. 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] Of these, around 1,250 people have DEB.[2]

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.

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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: • closures of unhealed target wounds • time to wound closure • duration of wound closure • percentage of surface area of wound healed • change in total body wound burden • 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.

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	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.

Questions for consultation

Where do you consider beremagene geperpavec will fit into the existing care pathway for dystrophic epidermolysis bullosa? Would beremagene geperpavec be used instead of, before, or after birch bark extract?

Will beremagene geperpavec be used in the same population as birch bark extract for DEB?

Please select from the following, will beremagene geperpavec be:
A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):
For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would beremagene geperpavec be a candidate for managed access?

Do you consider that the use of beremagene geperpavec can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

Please indicate if any of the treatments in the scope are used in NHS practice differently than advised in their Summary of Product Characteristics. For example, if the dose or dosing schedule for a treatment is different in clinical practice. If so, please indicate the reasons for different usage of the treatment(s) in NHS practice. If stakeholders consider this a relevant issue, please provide references for data on the efficacy of any treatments in the pathway used differently than advised in the Summary of Product Characteristics.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit

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and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which the treatment will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

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

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