Highly Specialised Technologies (HST) criteria checklist

Beremagene geperpavec for treating wounds associated with dystrophic epidermolysis bullosa

### Introduction

The NICE HST criteria checklist is to highlight where a technology meets/partially meets or does not meet the criteria for routing to the HST programme. Its purpose is to show the details of why a technology may not be appropriate for HST evaluation, but also where it has been identified as suitable. For more information, please see [section 7 of NICE health technology evaluation topic selection: the manual](https://www.nice.org.uk/process/pmg37/chapter/highly-specialised-technologies)

### Key – does the technology meet the criteria? Please use the colour key to advise if the technology meets the criteria

|  |  |
| --- | --- |
| Met | There is clear and strong evidence that this criterion is met |
| Not met | There is no evidence or limited evidence that the criterion is met.  There is some evidence, or the evidence available is unclear |

MA wording: anticipated: \*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*

| **Number** | **Criterion** | **Description of how the technology meets the criteria** | **Does the technology meet the criteria?** |
| --- | --- | --- | --- |
|  | The condition is very rare defined by 1:50,000 in England | Epidermolysis Bullosa is thought to affect 1 in 17,000 births. Around 5,000 people are affected in the UK. (BMJ Best Practice, [Epidermolysis bullosa](https://bestpractice.bmj.com/topics/en-gb/744), March 2019 & Patient UK, [Epidermolysis Bullosa](https://patient.info/doctor/epidermolysis-bullosa-pro#nav-2), February 2016). More than 30 EB subtypes, which vary in the severity of symptoms, ranging from mild to severe have been identified.  Around 25% of EB is dystrophic epidermolysis bullosa (DEB). Prevalence estimates of DEB vary in the literature from around 1 in 90,000 (Petrof et al 2022) or 1 in 68,000 (BMJ Best Practice, 2019) to 1.02 per 50,000 ([Horn et al 1998)](https://pubmed.ncbi.nlm.nih.gov/9155958/). Horn et al estimated the prevalence in Scotland. It's noted that the prevlance is likely to be underestimated, and more complete searching results in higher prevalence estimates with 75% of cases unknown to general practitioners.  The TSOP (Topic Selection Oversight Panel) noted that DEB is caused by genetic mutation, but that symptoms can be milder or severe and that there is clinical overlap with other subtypes of EB. It considers that for criterion 1 the condition is Epidermolysis Bullosa, and therefore this criterion is not met as the prevalence of EB is over 1 in 50,000. | Not met |
|  | Normally no more than 300 people in England are eligible for the technology in its licensed indication and no more than 500 across all its indications | The anticipated license wording covers all people with \*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\* \*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\* \*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*\*. EB is thought to affect around 5,000 people in the UK, of these around 1,250 people have DEB. However, population numbers vary based on sources.  Using EB registry data, ID1505 estimated the prevalence of relevant subgroups of EB in England to be 569 (DEB). This aligns with the information in a recent publication: <https://onlinelibrary.wiley.com/doi/10.1111/bjd.20958>  However, other sources of prevalence rates of EB, and therefore DEB, lead to higher estimates of people potentially eligible for beremagene geperpavec, (such as [Horn et al 1998](https://pubmed.ncbi.nlm.nih.gov/9155958/)) – which estimates a prevalence of 1.02 per 50,000 for DEB – which would mean significantly more than 500 people in England would be eligible for treatment with beremagene geperpavec.  DEB would need to be confirmed by genetic testing, all those with DEB who have a mutation in the COL7A1 gene may be eligible for treatment. Unclear if any additional eligibility criteria will apply.  Severity can vary in DEB, and need for treatment over a lifetime can change – wounds can appear and heal and appear again elsewhere. So, eligibility may be transient. At the scoping workshop that clinical experts suggested that they would offer this treatment to all people with DEB (although they suggested ~60% would take it)  TSOP considers the eligible population for this treatment as those with DEB and therefore is over 500 people. Therefore, this criterion is not met. | Not met. |
|  | The very rare condition significantly shortens life or severely impairs its quality | Epidermolysis bullosa (EB) cause the skin to become very fragile. Any trauma or friction can cause the skin to blister and tear easily. EB can manifest internally affecting areas such as the eye, mouth or stomach. This can lead to vision loss, disfigurement, and other serious complications including the development of aggressive skin cancers, dental problems, or nutritional compromise.  DEB is one of the most severe forms of EB, and can severely impair its quality however severity and symptoms vary across the affected population. | Met |
|  | There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options. | Currently no satisfactory treatment options exist. oleogel-S10 (Birch Bark Extract) is being evaluated by NICE for the same population but TSOP noted that this is not currently routinely commissioned.  TSOP understood there was early evidence from clinical trials suggesting Beremagene geperpavec is likely to provide benefits over current standard clinical management for skin wounds, although it is unclear if these benefits would impact on survival and improve outcomes for internal complications associated with EB which cannot be treated using topical treatment. | Met |