Oleogel-S10 for treating skin wounds associated with epidermolysis bullosa ID1505

### 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.

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

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

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

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| **Criterion** |  | |
| **The target patient group for the technology in its licensed indication is so small that treatment will usually be concentrated in very few centres in the NHS** | The entire population of people with epidermolysis bullosa (EB) is estimated to be around 5000 (see scope for references). There are currently 4 HST centres providing treatment for EB (2 for adults and 2 for children).  The clinical trial only included rarer disease subgroups: dystrophic epidermolysis bullosa (DEB), junctional epidermolysis bullosa (JEB) and Kindler syndrome (KS). Together these subgroups make up around 30% of EB cases.  Workshop, consultation comments and follow up clinical queries:  Using EB registry data, the company estimate the prevalence of relevant subgroups of EB in England to be 569 (DEB), 61 (JEB) and 18 (KS) respectively, leading to a total population of 648.  From the prevalent population (648) the company estimate 100-150 would be “treatable patients” with JEB, DEB and Kindler in England.  This number assumes people would only be eligible for treatment in line with the inclusion criteria in the pivotal trial. Which required a target wound over 3 weeks old and of reasonable size, between 10cm2 and 50cm2.  Clinical experts stated that there is huge variability between patients. For some subgroups up to 75% of the population may be candidates for treatment at some point during their lifetime. They noted that symptoms and manifestations of EB can come in peaks and troughs.  While there are currently 4 HST centres providing treatment for EB, treatment with Oleogel-S10 will primarily be administered at home by patients, carers or parents.  Re-branding  Oleogel-S10 was approved in the European Union in January 2016 for the treatment of partial thickness wounds in adults, this could include burns, skin grafts, meaning the size of the eligible population would far exceed the size recommended for HST. The 2016 license was issued under the trade/brand name [epivalsan](https://www.ema.europa.eu/en/medicines/human/EPAR/episalvan).   The panel heard that the product Episalvan was not available in England and Amryt had confirmed the broad license for Episalvan would be withdrawn if a MA was granted for the re-branded product with a smaller eligible population.  **Conclusion:  Target population size is uncertain but fewer than 500 people expected to need treatment over a lifetime.** | **Met** |
| **The target patient group is distinct for clinical reasons** | Yes, epidermolysis bullosa is clinically distinct with established subgroups.  Workshop, consultation comments and follow up clinical queries:  People would only be eligible for treatment in line with the inclusion criteria in the pivotal trial. People would have a target wound over 3 weeks old and of reasonable size, between 10cm2 and 50cm2.  **Conclusion:  The severe EB populations is clinically distinct, but eligibility may be transient as the symptoms and manifestations of EB change for individuals over a lifetime.** | **Met** |
| **The condition is chronic and severely disabling** | Yes, the subgroups included in the trial are the more severe forms of EB.  **Conclusion:  DEB, JEB and KS subgroups are chronic and severely disabling. The most severe form of EB can severely limit life expectancy.** | **Met** |
| **The technology is expected to be used exclusively in the context of a highly specialised service** | Workshop, consultation comments and follow up clinical queries:  EB is commissioned through a highly specialised service.  Clinical experts stated that administration would be done by patients, carers, or parents at home but treatment is started or recommended by highly specialised services. Continuous use would be overseen and prescribed by HSS not local dermatologists or GPs.  **Conclusion:**  **Treatment is started or recommended by highly specialised services and would not subsequently it may be prescribed locally via secondary or primary care settings.** | **Met** |
| **The technology is likely to have a very high acquisition cost** | Cost unknown but likely to be high. | **Met** |
| **The technology has the potential for lifelong use** | Symptoms associated with JEB, DEB and KS continue from childhood into adulthood. There is no cure for EB or treatment of the underlying condition.  Clinical experts noted that people with more severe EB, can have multiple, recurrent and chronic wounds which are difficult to heal. These people may require treatment multiple times, perhaps at different sites. The experts also noted that symptoms and manifestations of EB can come in peaks and troughs.  Oleogel-S10 is an acute treatment for EB wounds and does not treat the underlying condition. Treatment does not stop further wounds occurring.  **Conclusion:** **People may not require treatment continuously but the need for treatment may be lifelong. It is unclear what proportion of people with EB would use this treatment over a lifetime, but it has the potential for lifelong use.**  **It is also noted that Oleogel-S10 does not treat the underlying condition of EB.** | **Met** |
| **The need for national commissioning of the technology is significant** | Yes. There is an unmet need for treatments for EB.EB is commissioned as a highly specialised service.  **Conclusion:** **Limited treatment options and no licensed treatments currently exist for EB** | **Met** |