Highly Specialised Technologies (HST) criteria checklist

Idebenone for treating visual impairment in Leber’s hereditary optic neuropathy [ID547]

**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 – 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.  |

### MA wording: Raxone is indicated for the treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy (LHON).

| **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  | * The disease under consideration is LHON.
* Prevalence rates in England of vision loss caused by LHON have been cited as 1 in 31,000 ([Yu-Wai-Man et al. 2003](https://pubmed.ncbi.nlm.nih.gov/12518276/)) or 1 in 27,400 ([Gorman et al. 2015](https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4737121/)).
* The same studies suggested the prevalence rate of the mutations in mitochondrial DNA that cause LHON to be even higher.
* The company presented the worldwide LHON prevalence estimate in [Esmaeil, Ali, Behbehani 2023](https://www.frontiersin.org/articles/10.3389/fopht.2022.1077395/full) of 1 in 50,000 in support of its routing challenge. However, this source acknowledges variability across different countries and continents. In particular, the same publication refers to the Yu-Wai-Man estimate LHON in the UK of 1 in 31,000 as indicative of geographic variability.
* TSOP considered the new evidence presented by the company but it was not persuaded that the worldwide prevalence estimates were more relevant to the population of England than those from Yu-Wai-Man. Because the UK specific estimates are above 1 in 50,000, this criterion is not met.
 | **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  | * Based on the company’s assumptions given for criterion 1, it proposed it its challenge that the estimated number of people with the LHON mutation and with sight loss is 293.
* It also pointed out that idebenone is reimbursed in Wales under its ultra-orphan processes and that since July 2021, xx people with LHON have been treated with idebenone in Wales out of a population (over 12) of 2.7 million and that in the Republic of Ireland, idebenone has also been reimbursed since 2018, where xx patients have had idebenone out of a total population of around 5.03 million.
* TSOP considered the new evidence presented by the company, but as it had concluded that the prevalence estimates for LHON in England from Yu-Wai-Man were likely to be more relevant, it used 1 in 31,000 to calculate the likely number of people eligible for the treatment in England, which resulted in approximately 471 patients. As a result, TSOP considered that this criterion was not met.
 | **Not Met** |
|  | The very rare condition significantly shortens life or severely impairs its quality  | * Most people with LHON are legally blind, with 97% affected in both eyes.
* Sudden vision loss and lack of treatments can cause severe psychological impacts. LHON has a severe negative impact on quality of life and has been reported to have the [worst VF-14 score compared with other studied ophthalmic disorders](https://pubmed.ncbi.nlm.nih.gov/19255150/) (the VF-14 quality of life questionnaire assess impact of visual disability).
* Having LHON is associated with an almost [2-fold increased risk of mortality](https://iovs.arvojournals.org/article.aspx?articleid=2653922) compared with the general population.
* During the scoping workshop, LHON was described as “catastrophic”, with significantly greater psychological impact than other inherited optic disorders due to the rapid nature of symptoms and the average age of diagnosis (teenagers – early twenties). Clinicians indicated many people with LHON experience depression, anxiety and suicidal thoughts. Psychological impacts can be a barrier to treatment and lead to disengagement with the healthcare service.
* The company also noted the significant burden for carers and family members.
* TSOP agreed that this criterion is met.
 | Met |
|  | There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options. | * Current treatment is supportive only, there are no other approved therapies for LHON in the UK.
* TSOP agreed that this criterion is met.
 | Met |