Highly Specialised Technologies (HST) criteria checklist

Omaveloxolone for treating Friedreich's ataxia in people 16 years and over

Introduction

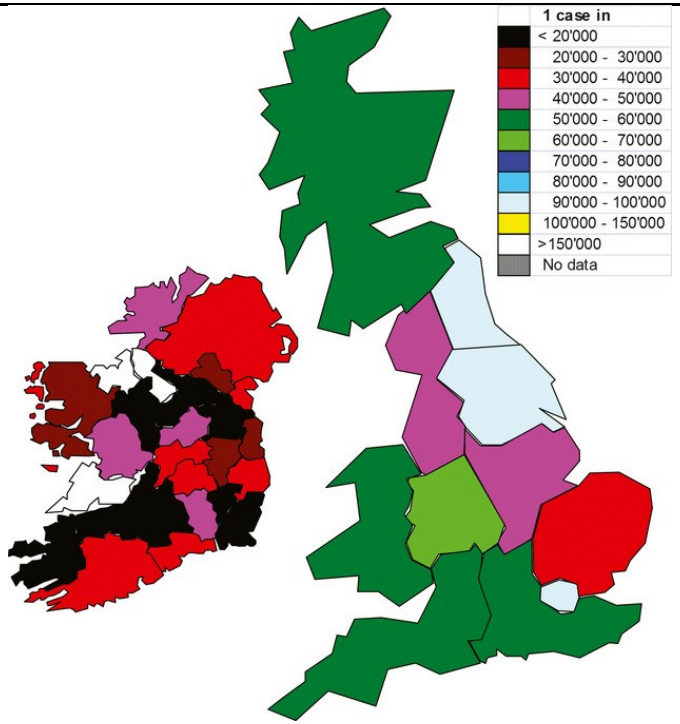
The NICE HST criteria checklist is to highlight where a technology meets/partially meets or does not meet the criteria for routing to the HST programme. Its purpose is to show the details of why a technology may not be appropriate for HST evaluation, but also where it has been identified as suitable. For more information, please see section 7 of NICE health technology evaluation topic selection: the manual

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

Met	There is clear and strong evidence that the criterion is met
Not met	There is some, but not enough clear evidence that the criterion is met or There is no evidence or limited evidence that the criterion is met.

MA wording: [redacted]

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
1.	The disease is very rare defined by 1:50,000 in England	Limited data on prevalence estimates in the UK. Those available very widely. • The estimate for England calculated using regional data from Vankan (2013) is 1 in 56,695 people. The same paper reports a UK prevalence of 1 in 54,000 for the UK but this is affected by high rates in Northern Ireland.	Met



Source: Vankan P, 2013 using data provided by Ataxia UK

- NIHRIO report states incidence of 1:50,000 for UK
- EMA states 0.5 in 10,000 (or 1 in 20,000) for Europe, but this includes countries with a higher prevalence such as Spain (1 in 20–25,000) and France (1 in 43,000) (Source: Vankan P, 2013)

Given that the data sources specific to the UK and England suggest the prevalence of the condition is lower than 1:50,000 people, the prioritisation board considered that this criterion was met.

2.	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	• Using the prevalence estimate from Vankan (2013) and an English population of 57,106,398 (ONS population estimates 2022) gives an estimate of the population in England eligible for the technology of around 1,000. • UK prevalence estimates are 3,400, using the EMA prevalence of 1 in 20,000 or 1,100 from Vankan 2013. It's likely that these are overestimates for the eligible population in England given that: • Any estimates based on the UK must take into account the higher prevalence of the condition in Northern Ireland (see criterion #1). • A proportion would be under 16 years old and therefore ineligible for the technology. Currently unclear how many people this would be in the NHS. Ataxia UK has stated that its disease register has 706 people with Friedreich's ataxia in England, of whom 634 are 17 or over. Calculations it submitted separately gave a likely population of 622 patients over 16. Considering this, it is very likely that more than 300 people in England would be eligible for the treatment as part of its anticipated license authorisation. The prioritisation board considered if flexibility could be applied for this criterion but decided that as the eligible population within the anticipated marketing authorisation was very likely to be substantially above 300 people, it was not persuaded that flexibility could be applied. Therefore, the criterion is not met.	Not met
3.	The very rare disease for which the technology is indicated significantly shortens life or severely impairs quality of life	• Symptoms begin between 5-15 years, and many lose ability to ambulate by their mid-20s. Symptoms include difficulty walking, inability to coordinate movements, muscle weakness, speech issues, damage to the heart muscle and diabetes. • The brain, spinal cord and nerves, and heart and pancreas can be damaged due to production of toxic forms of oxygen due to iron accumulation. • Shortens life span, most often through consequences of cardiomyopathy; mean age at death is 37.5 years. The prioritisation board considered that this criterion was met.	Met

4.	There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.	• Currently no NICE recommended treatments. • Current treatment focuses on symptom management, including speech and language therapy and physiotherapy, occupational therapy. Neurologists, cardiologists, orthopaedic surgeons and orthotists are also involved in the care of people with FA. • The MOXIe trial showed that omaveloxolone showed a significant neurological function improvement compared with placebo, and was generally safe and well tolerated. People receiving omaveloxolone also experienced improvement in muscular symptoms. The prioritisation board considered that this criterion was met.	Met