NICE National Institute for Health and Care Excellence

Highly Specialised Technologies (HST) criteria checklist

Vamorolone for treating Duchenne muscular dystrophy [ID4024]

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: Not available

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
1.	The condition is very rare defined by 1:50,000 in England	• The prevalent population of people in the UK with Duchenne muscular dystrophy (DMD) is estimated to be around 2,500.[1]	Not 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	• Vamorolone does not hold a marketing authorisation in any other indications. • The population suitable for treatment is expected to include all those diagnosed with DMD. No exclusion is expected based on specific characteristics or manifestations associated with the condition. Therefore the number of people eligible for treatment with vamorolone in England is expected to be in excess of 300.	Not met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
3.	The very rare condition significantly shortens life or severely impairs its quality	• Symptoms typically present at a young age (1-3 years) and are severely debilitating, these include but are not limited to motor, cardiac and respiratory function. • DMD often results in children being wheelchair bound by age 12 and developing cardiomyopathy by age 18. • The average lifespan of patients with DMD is less than 30 years.[2]	Met

4.	There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.	• Most treatment options do not treat the underlying cause of the disease and focus on alleviating symptoms and maintaining muscle strength.
		• Corticosteroids are standard of care, with prednisone and deflazacort the two most prescribed steroids for DMD.
		  ○ At the scoping workshop clinicians and patient experts emphasised corticosteroids are not satisfactory treatments for DMD.
		  ○ Experts explained that management of side effects of corticosteroids was extensive and resulted in significant impact on quality of life.
		  ○ Ataluren is recommended by NICE in a subset of DMD patients in HST22. Ataluren aims to treat the underlying cause of disease rather than alleviating symptoms. It reduces the need for steroids and is therefore unlikely to be a relevant comparator.
		• Vamorolone is a new oral anti-inflammatory technology. It has a different mechanism of action resulting in the same treatment efficacy as steroids but with fewer side effects.
		  ○ At the scoping workshop clinicians agreed vamorolone would not be more clinically effective than corticosteroids in treating
			Met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
		DMD and that the benefits of vamorolone were in the reduction of severe side-effects and compliance with therapy. TSOP acknowledged stakeholder feedback at the workshop that corticosteroids were not satisfactory treatment and extensive management of side effects is required for patients and clinicians. TSOP agreed this criteria was met.	

References

1. Muscular dystrophy. Duchenne muscular dystrophy (DMD) – Overview. Available from: https://www.musculardystrophyuk.org/conditions/duchenne-muscular-dystrophy-dmd . Accessed: 24th May 2023.

2. Broomfield, J., et al. 2021. Life Expectancy in Duchenne Muscular Dystrophy. Neurology. Available from: https://n.neurology.org/content/97/23/e2304 Accessed: 24th May 2023.

Highly specialised technologies vision and routing criteria

The Highly Specialised Technologies Programme is designed to be used in exceptional circumstances. Its purpose is to evaluate technologies for very rare diseases that have:

- small numbers of patients
- limited or no treatment options
- challenges for research and difficulties with collecting evidence, because of the uniqueness of the disease.

The Highly Specialised Technologies Programme aims to:

- encourage research on, and innovation for, very rare conditions when there are challenges in generating an evidence base that is robust enough to bring the product to market

- secure fairer and more equitable treatment access for very small populations with very rare diseases
- recognise that an approach that maximises health gain for the NHS may not always be acceptable: it could deliver results that are not equitable.

The Highly Specialised Technologies Programme acknowledges that:

- It is important for NICE to apply appropriate limits on the very rare populations that can potentially be routed to the programme. This is because the Highly Specialised Technologies Programme is a deliberate departure from the standard technology appraisal process (valuing the benefits from these technologies more highly by having a much higher incremental cost-effectiveness ratio [ICER] threshold) for the reasons outlined above.
- Each time NICE routes a topic to the Highly Specialised Technologies Programme it is deciding that, if the technology is recommended, the NHS must commit to allocate resources that would have otherwise been used on activities that would be expected to generate greater health benefits.
- NICE has sought to strike a balance between the desirability of supporting access to treatments for very rare diseases against the inevitable reduction in overall health gain across the NHS that this will cause. Both considerations are valid and important, and neither can be given absolute priority over the other. Therefore, the Highly Specialised Technologies Programme criteria and their anticipated application intentionally do not seek to capture every case when there are challenges in generating an evidence base or when there is a small population with a rare disease.
- This approach ensures that technologies routed to the Highly Specialised Technologies Programme fulfil the vision of the programme and manages the displacement in the wider NHS.

However, it can be difficult to identify the exceptional circumstances when the highly specialised technologies methods and processes should be used because of the difficulty in getting the information needed. Proxy information is often relied on and used to make subjective judgements. The routing criteria identify which technologies should be routed for highly specialised technologies guidance. These criteria help make subjective judgements as informed, justifiable, consistent and predictable as possible. NICE's capacity to develop highly specialised technologies guidance can react to need and there is no limit on the number of technologies that can be routed.

The final routing criteria for the Highly Specialised Technologies Programme are:

- The disease is very rare – defined as 1:50,000 population in England.
- 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 very rare disease for which the technology is indicated significantly shortens life or severely impairs quality of life.
- There are no other satisfactory treatment options, or the technology is likely to offer significant additional benefit over existing treatment options.