NICE National Institute for Health and Care Excellence

Highly Specialised Technologies (HST) criteria checklist

Givinostat for treating Duchenne muscular dystrophy in people 6 years and over: ID6323

Introduction

The NICE HST criteria checklist highlights 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 or may not be appropriate for HST evaluation. 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.

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
1.	The condition is very rare as defined by less than 1:50,000 in England	• Approximately 2,500 people in the UK have Duchenne muscular dystrophy (DMD)[1] This equates to 2112 people with DMD in England (based on the proportion of UK's population [0.84*2500] in England)[2]	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	• Givinostat does not hold a marketing authorisation in any other indications. • The company's anticipated MA is for: [redacted] • The population in the company's pivotal trials is for ambulatory males aged 6 and over and non-ambulant males aged 9 to 17 years. • There is a lack of evidence for the prevalence of people with DMD by age group in England, however it is not expected to be below 300 for people 6 years of age and older. • The company estimated in their response to stakeholder consultation that approximately 1,000 people may be eligible for treatment with Givinostat in England.	Not met

Number	Criterion	Description of how the technology meets the criteria	Does the technology meet the criteria?
3.	The very rare disease for which the technology is indicated significantly shortens life or severely impairs quality of life	• Symptoms typically present at a young age (1-3 years old) and are severely debilitating, these include but are not limited to motor, cardiac and respiratory function. • DMD often results in children requiring the use of a wheelchair by age 12 and developing cardiomyopathy by age 18. • The average lifespan of people with DMD is less than 30 years.	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 condition and focus on alleviating symptoms and maintaining muscle strength. • Interventions may include the use of steroids (associated with several side effects) and physical aids (such as wheelchairs, leg braces or crutches), exercise, physiotherapy, and occasionally orthopaedic surgery. • In addition, other supportive treatments such as dietetic advice, prevention and treatment of bone fragility and the management of complications of long-term steroid therapy are required. • Ataluren is recommended for treating Duchenne muscular dystrophy in a subset of people with a nonsense mutation in the dystrophin gene in people 2 years and over who can walk (HST 22). Ataluren is not available for all the people who may be eligible under the full anticipated marketing authorisation for Givinostat. • Givinostat is proposed to address progression of the condition. • There is an unmet need in this population and this treatment appears to address some of the underlying pathology of DMD.	Met

References

1. Muscular dystrophy UK. Duchenne muscular dystrophy (DMD) – Overview. Available from: https://www.musculardystrophyuk.org/conditions/duchenne-muscular-dystrophy-dmd. Accessed: June 2024

2. Office for National Statistics. Population estimates. Available from: Population estimates - Office for National Statistics (ons.gov.uk). Accessed: June 2024