NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

HEALTH TECHNOLOGY APPRAISAL PROGRAMME

Equality impact assessment – Guidance development

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

The impact on equality has been assessed during this appraisal according to the principles of the NICE equality scheme.

Final draft guidance

(when no draft guidance was issued)

1.	Have the potential equality issues identified during the scoping process been addressed by the committee, and, if so, how?
Yes. Stakeholders noted the following: • A significant proportion of people with Duchenne muscular dystrophy (DMD) have poor outcomes because of learning or behavioural difficulties, ADHD, autism, and pre-existing psychiatric difficulties, making up around 30% of the adult population. A significant proportion of people with DMD cannot have corticosteroids because of these issues and have worse outcomes. The randomised controlled trial uses givinostat as an add on treatment to standard corticosteroid treatment. Therefore, this subgroup is excluded may not have access to givinostat if it is approved, increasing inequality of access to care. • DMD is a rare condition and there is limited access to comprehensive studies and inclusion in novel therapeutic randomised controlled trials and the impact on equity of care. • People with DMD have varying levels of disability and so should not have to travel excessive distances to have treatment. The preliminary view was that the committee can only evaluate a technology within its marketing authorisation and with the evidence available. The committee understood the evidence relating to corticosteroid use, and did not	

make any specific recommendations or specify requirements relating to corticosteroid use. The committee considered the difficulties in evidence generation associated with DMD as a rare condition. It understood that many people with DMD have learning or behavioural difficulties and the impact of this on the evidence base. Difficulties with access to care services are beyond the scope of this technology appraisal.

2.	Have any other potential equality issues been raised in the submissions, expert statements or academic report, and, if so, how has the committee addressed these?

Yes, in addition to points raised during scoping the stakeholders highlighted that rare diseases disproportionately affect disabled and paediatric populations. For example, they raised concerns about:

- issues with measures of carer's health-related quality of life
- limitations with paediatric health-related quality-of-life measures
- inappropriate standards for paediatric disability
- the requirement for restoring to full health for the non-reference 1.5% rate for cost and health effects and
- the exclusion of social care costs.

Stakeholders also:

- noted that NICE processes do not account for living with a severe disability or for those who will become wheelchair users (disabled people have worse health outcomes, raising questions about health inequalities)
- felt that NICE's methods guide fails to operationalise flexibility, which could breach NICE's duty to consider equity and long-term societal impact
- thought that limiting any recommendation to an ambulant starting population would constitute discrimination
- suggested that the Medicines for Children Policy could apply

- noted potential health inequalities and associative discrimination in employment of parent carers, further highlighting that parent carers suffer poor health outcomes and are predominantly female, and

- explained the intersectionality of protected characteristics, including age, disability, sex, and pregnancy or maternity characteristics under the Equality Act 2010, and the compounded disadvantage these characteristics can have on carers.

The committee acknowledged these points and discussed all potential issues raised in detail. In particular, please see sections 3.8, 3.18, 3.39 and 3.40 of the final draft guidance for more information.

The committee recognised that generating evidence in rare, progressive diseases starting in childhood, such as Duchenne muscular dystrophy, is very difficult. However, it noted that NICE's manual on technology appraisal and highly specialised technologies guidance includes flexibility to appropriately consider evidence in such rare diseases, and considered that flexibility in its decision making.

The committee also understood the nature of Duchenne muscular dystrophy and its profound impact on people with the condition, as well as their carers and families. It recognised the importance of effective treatments that could delay disease progression and considered the potential role of givinostat in addressing this unmet clinical need.

The committee considered the restriction of the population to people who are ambulant at the start of treatment. It noted that evidence was only available for this population, and it would expect the nature and scale of clinical benefits and the cost effectiveness to be meaningfully different in people who were non-ambulant at the start of treatment. It considered that excluding a non-ambulant starting population because of a lack of evidence would be a proportionate means of making evidence-based recommendations and ensuring a cost-effective use of NHS resources. The committee concluded that it was only able to make recommendations for people who start givinostat while ambulant, in line with the evidence that was presented by the company.

The committee concluded that it had explored the potential equality issues during its deliberations and determined that there were no further equality issues that could be addressed in a technology appraisal, nor any additional steps or adjustments required in light of its public sector equality duties.

3.	Have any other potential equality issues been identified by the committee, and, if so, how has the committee addressed these?
No.	

4.	Do the preliminary recommendations make it more difficult in practice for a specific group to access the technology compared with other groups? If so, what are the barriers to, or difficulties with, access for the specific group?
The population is restricted to people who are ambulant at the start of treatment, and so excludes people who are non-ambulant. This issue is explored in question 2 in this document, and in section 3.8 of the FDG.	

5.	Is there potential for the preliminary recommendations to have an adverse impact on people with disabilities because of something that is a consequence of the disability?
The population is restricted to people who are ambulant at the start of treatment, and so excludes people who are non-ambulant. This issue is explored in question 2 in this document, and in section 3.8 of the FDG.	

6.	Are there any recommendations or explanations that the committee could make to remove or alleviate barriers to, or difficulties with, access identified in questions 4 or 5, or otherwise fulfil NICE's obligations to promote equality?
No.	

7.	Have the committee's considerations of equality issues been described in the final draft guidance, and, if so, where?
Yes, in section 3.8, 3.18, 3.39 and 3.40.	

Approved by Associate Director (name): Ian Watson

Date: 29 April 2026