

# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## HEALTH TECHNOLOGY APPRAISAL PROGRAMME

### Equality impact assessment – Guidance development

#### STA Cipaglucosidase alfa with miglustat for treating Pompe disease

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

#### Final appraisal determination

1. Have the potential equality issues identified during the scoping process been addressed by the committee, and, if so, how?
<ul style="list-style-type: none"><li>• Ease of access to treatment:<ul style="list-style-type: none"><li>○ The committee heard that people will have the option for the treatment to be administered at home via homecare. This is the same as existing therapies.</li></ul></li><li>• Differences in prevalence or incidence of a disease in different ethnic groups:<ul style="list-style-type: none"><li>○ Issues related to differences in prevalence or incidence of a disease cannot be addressed in a technology appraisal</li></ul></li><li>• Some people may find it difficult to ingest the miglustat pill (bulbar weakness is common in Pompe disease).<ul style="list-style-type: none"><li>○ This was discussed during committee and patient expert input confirmed that this issue can be resolved by dissolving the tablet in water.</li></ul></li></ul>
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?
<ul style="list-style-type: none"><li>• The appraisal process should not prejudice access to suitable treatments based on the rarity of the condition:</li></ul>

- The committee considered the rarity of the condition and the challenges that this presents to evidence generation when making their recommendation (see section 3.10)
- Disabled people should have access to as many treatments as practical, even those that might be fractionally better or better tolerated.
  - The committee heard from the patient expert that small clinical changes can have a large impact on quality of life. For example, they can help people maintain their independence. This was considered by the committee (see section 3.2).

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 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?

No

5. Is there potential for the recommendations to have an adverse impact on people with disabilities because of something that is a consequence of the disability?

No

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 appraisal determination, and, if so, where?

Yes. See section 3.17.

The challenges of generating evidence due to the rarity of Pompe disease was discussed in section 3.10.

The importance of small clinical benefits for people with Pompe was discussed in section 3.2.

**Approved by Associate Director (name):** .....Janet Robertson.....

**Date:** 26 May 2023