NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

HEALTH TECHNOLOGY APPRAISAL PROGRAMME

Equality impact assessment – Scoping

MTA/STA Pegunigalsidase alfa for treating Fabry disease [ID3904]

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

1. Have any potential equality issues been identified during the scoping process (draft scope consultation and scoping workshop discussion), and, if so, what are they?
a) At the Scoping Workshop the clinical expert referred to some evidence showing treatment for Fabry is more cost effective in males with 'classic Fabry' compared to other subgroups (including all females). Concerns were then raised about the potential for inequity of access to treatment based on sex. b) Access to enzyme replacement therapy and migalastat varies by age (agalsidase alpha and beta is licensed for children and adults, migalastat is licensed for people 16yrs and pegunigalsidase alpha license application is for adults 18yrs and over). c) During the consultation phase, one of the consultees said that using the STA process discriminates against patients with a rare condition such as Fabry disease, as treatments should be assessed using an approach that takes into account the rarity of their condition (i.e. HST). d) During the consultation phase, one of the consultees said that to avoid increasing the inequity in care many people with Fabry disease face: • The priorities set out in the Rare Disease Framework published in January 2021 should be included in the appraisal • Best Supportive Care should be removed as a comparator

2.	What is the preliminary view as to what extent these potential equality issues need addressing by the committee?

a) The remit of the NICE committee is to appraise the technology within and across its marketing authorisation. At the scoping workshop it was agreed that males with 'classic Fabry' should not be defined as a separate subgroup in the scope. Where appropriate and where evidence allows, the committee may consider whether its recommendation could have a different impact on a particular subgroup than on the wider population.

b) NICE committee's remit is to appraise the technology within and across its marketing authorisation. The committee may consider whether its recommendations could have a different impact on people protected by the equality legislation than on the wider population.

c) Following the consultation and scoping workshop it was decided that this topic will proceed as a single technology appraisal (STA). This decision was made in consideration of the updated highly specialised technologies routing criteria. As outlined in NICE's Topic Selection manual, "standard technology appraisals methods and processes are designed to be flexible and adaptable for all technologies and conditions. So, they are suitable for most technologies that treat rare conditions and small populations." Although rarity is not a characteristic which is protected by the equality legislation, the NICE process and methods manual specifies that the committee will be mindful that evidence generation is particularly difficult for rare diseases. The methods and processes used by NICE go through rigorous review, assessment and consultation and NICE is required to follow these processes, as described in the Principles that guide NICE's work.

d) Following consultation, the Rare Disease Framework was added to the scope and best supportive care was removed as a comparator, so this issue is no longer relevant.

3.	Has any change to the draft scope been agreed to highlight potential equality issues?

d) No

4.	Have any additional stakeholders related to potential equality issues been identified during the scoping process, and, if so, have changes to the matrix been made?
No additional stakeholders have been identified.	

Approved by Associate Director (name): ……

Date: [xx/xx/year]