

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

HEALTH TECHNOLOGY APPRAISAL PROGRAMME

Equality impact assessment – Guidance development

STA Sapropterin for treating phenylketonuria [ID1475]

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

Consultation

1. Have the potential equality issues identified during the scoping process been addressed by the committee, and, if so, how?

During the scoping process, it was highlighted that some people have particular difficulties adhering to conventional dietary management of phenylketonuria (PKU), including people with physical and mental health difficulties and disabilities, people with particular housing or caring arrangement, and people from particular family origins (including people with Irish Traveller family origins and people whose first language is not English).

During the scoping process it was also highlighted that the carer burden of PKU falls particularly upon women, as primary carers for children with PKU. The burden of managing the restrictive dietary requirements was emphasised.

It was noted that sapropterin is currently funded by NHS England for some people who are pregnant, and that it may have particular benefits in this group.

Consultees noted that people who are treated late or not treated for their PKU, and people who are lost to follow up, may have particular challenges.

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?

Issues were raised regarding children, young people, women of child-bearing age, pregnant women and disabled adults.

3. Have any other potential equality issues been identified by the committee, and, if so, how has the committee addressed these?

The committee discussed all these groups.

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 preliminary recommendations were age-based and recommended sapropterin for children under the age of 18 only.

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 ACD acknowledges that some people may have greater difficulty adhering to conventional dietary management of PKU and are at higher risk of being unable to control their phenylalanine. See section 3.25

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?

Section 3.25 of the ACD set out explanation why recommending sapropterin for certain groups of adults cannot be justified given the cost-effectiveness estimates.

7. Have the committee's considerations of equality issues been described in the appraisal consultation document, and, if so, where?

Section 3.25 of the ACD

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

Date: 18 February 2021

Final appraisal determination

(when an ACD issued)

1. Have any additional potential equality issues been raised during the consultation, and, if so, how has the committee addressed these?

The previous equality issues were raised during consultation on the ACD.

2. If the recommendations have changed after consultation, are there any recommendations that 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 recommendation has increased the age range for which sapropterin is considered cost effective. The committee was aware that age-based recommendations must be objectively justified, and they should be avoided when possible. It considered the justification in this case is the need to secure acceptable cost efficacy in the interests of the NHS as a whole. Age itself is both an indicator of potentially greater benefit, coupled with lower cost of treatment. The committee explored alternative approaches but could not find any better alternative.

The final guidance also recommends sapropterin in pregnancy. NICE is aware that a recommendation for use during pregnancy is necessarily only of

benefit to people who become pregnant. This is permitted by s.17(6)(a) of the Equality Act 2010.

3. If the recommendations have changed after consultation, 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?

The committee understood that some people may have greater difficulty sticking to conventional dietary management of PKU and are at higher risk of being unable to control their phenylalanine levels. See section 3.32

4. If the recommendations have changed after consultation, are there any recommendations or explanations that the committee could make to remove or alleviate barriers to, or difficulties with, access identified in questions 2 and 3, or otherwise fulfil NICE's obligations to promote equality?

The committee accepted that some groups might plausibly benefit from treatment to a somewhat greater (but unquantified) extent than the adult population at large. But, while the committee was mindful of the need to seek to reduce inequalities and advance equality of opportunity, given the high ICERs for treatment in the adult population it did not consider that any wider recommendation would be an appropriate use of NHS resources. See section 3.32

5. Have the committee's considerations of equality issues been described in the final appraisal determination, and, if so, where?

Sections 3.32, 3.36 and 3.37 of the FAD

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

Date: 11 August 2021