

NHS England and NHS Improvement: Equality and Health Inequalities Impact Assessment (EHIA) template

A completed copy of this form must be provided to the decision-makers in relation to your proposal. The decision-makers must consider the results of this assessment when they make their decision about your proposal.

- 1. Name of the proposal (policy, proposition, programme, proposal or initiative)¹: Clinical Commissioning Policy: Sapropterin for PKU (All Ages)**
- 2. Brief summary of the proposal in a few sentences**

This clinical commissioning policy proposes the use of sapropterin as a treatment for patients with phenylketonuria (PKU). PKU is an inherited disorder which causes harmful levels of phenylalanine (Phe) (an amino acid found in natural protein) to accumulate in the blood, leading to brain damage, behavioural problems, seizures and cognitive impairment if untreated or poorly controlled in childhood. Adults with raised Phe levels report a range of symptoms including mood change and decreased processing speeds. Current treatment relies on excluding all meat, fish, dairy, nuts and legumes from the diet, and substituting these with artificial amino acids. Patients are required to accurately measure and restrict the amount of phenylalanine they consume. Sapropterin increases the action of a key enzyme which breaks down phenylalanine, and so reduces Phe levels and allows for lessened dietary restrictions and decreased intake of synthetic amino acids. The proposal is developed following an evidence review. Evidence for this treatment shows that there is variation in the benefit derived by patients based on the application of clinical criteria. Therefore the evidence of clinical (and where relevant cost) effectiveness has been used to determine the clinical criteria for individual access to the treatment. It is intended that these criteria are used by treating clinicians in determining the patients who can benefit from the treatment.

- 3. Main potential positive or adverse impact of the proposal for protected characteristic groups summarised**

¹ Proposal: We use the term proposal in the remainder of this template to cover the terms initiative, policy, proposition, proposal or programme.

Please briefly summarise the main potential impact (positive or negative) on people with the nine protected characteristics (as listed below). Please state **N/A** if your proposal will not impact adversely or positively on the protected characteristic groups listed below. Please note that these groups may also experience health inequalities.

Protected characteristic groups	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
<p>Age: older people; middle years; early years; children and young people.</p>	<p>If phenylalanine levels are poorly controlled in childhood patients can have reduced IQ, behavioural problems and developmental delay. Treatment with sapropterin can keep Phe in recommended range, therefore avoiding these adverse outcomes for children.</p> <p>Adolescents and young people are at a higher risk of poor phenylalanine control and poor outcomes as they transition to adulthood.</p> <p>People born prior to the introduction of newborn screening for PKU in 1969 may have late treated or late diagnosed PKU and have intellectual disabilities and high support needs.</p> <p>Adults with PKU are at a higher risk of reduced IQ and cognitive impairments. They may have experienced sustained high phenylalanine levels in their</p>	<p>Sapropterin to be available to infants, children and young people of all ages, as well as adults.</p>

Protected characteristic groups	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	<p>childhood and discontinued treatment at an early age.</p> <p>(Data in relation to impact on IQ: Fannesbeck CJ, McPheeters ML, Krishnaswami S, Lindegren ML, Reimschisel T. Estimating the probability of IQ impairment from blood phenylalanine for phenylketonuria patients: a hierarchical meta-analysis. J Inher Metab Dis. 2013;36(5):757–766. doi:10.1007/s10545-012-9564-0</p> <p>van Wegberg AMJ, MacDonald A, Ahring K, Bélanger-Quintana A, Blau N, Bosch AM, Burlina A, Campistol J, Feillet F, Giżewska M, Huijbregts SC, Kearney S, Leuzzi V, Maillot F, Muntau AC, van Rijn M, Trefz F, Walter JH, van Spronsen FJ. The complete European guidelines on phenylketonuria: diagnosis and treatment. Orphanet J Rare Dis. 2017 Oct 12;12(1):162)</p>	
<p>Disability: physical, sensory and learning impairment; mental health condition; long-term conditions.</p>	<p>There is a higher incidence of learning disabilities and cognitive impairments in the population of people with PKU.</p>	<p>Sapropterin to be made available as a treatment option.</p>

Protected characteristic groups	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	<p>Children and adults with intellectual disability may struggle to understand the importance of restrictions of the low-protein diet and may struggle with weighing foods and monitoring protein intake. Sapropterin can lessen the burden on these people and their carers.</p> <p>The children of parents/carers with learning impairments are at higher risk of poor outcomes, as the parents may struggle with dietary management of PKU.</p>	
Gender Reassignment and/or people who identify as Transgender	N/a	
Marriage & Civil Partnership: people married or in a civil partnership.	N/a	
Pregnancy and Maternity: women before and after childbirth and who are breastfeeding.	<p>Children born to women with high levels of phenylalanine are at risk of maternal PKU syndrome, which results in intellectual disabilities, microcephaly, heart defects and low birthweight. Whilst pregnant women with uncontrolled Phe levels can currently</p>	<p>Sapropterin to made available as a treatment option, which expands access to sapropterin treatment for pregnant women, as well as women planning pregnancy and women post pregnancy.</p>

Protected characteristic groups	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	<p>access treatment with sapropterin, those with unplanned pregnancies may inadvertently expose the foetus to potentially damaging levels of Phe in early pregnancy. Sapropterin treatment for patients of all ages can improve Phe levels in these patients, therefore reducing the risk of maternal PKU syndrome.</p> <p>Women with PKU need to establish low and controlled phenylalanine levels prior to conception to avoid damage to the foetus. This is stressful and difficult and can constrain women’s sexual and reproductive choices and unplanned pregnancies can result.</p> <p>Women with PKU should be considered an at-risk group after child birth. Women with PKU with young children may not be able to cope with dietary treatment. Phe toxicity is linked to low mood and depression.</p>	

Protected characteristic groups	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
Race and ethnicity ²	Certain ethnic groups (non English speakers or Roma/Travelers) may experience greater difficulty controlling phenylalanine levels using dietary management for social and cultural reasons.	Sapropterin to made available as a treatment option.
Religion and belief: people with different religions/faiths or beliefs, or none.	Religious belief may impact upon women with PKU who need to avoid unplanned pregnancy.	
Sex: men; women	N/a	
Sexual orientation: Lesbian; Gay; Bisexual; Heterosexual.	N/a	

4. Main potential positive or adverse impact for people who experience health inequalities summarised

Please briefly summarise the main potential impact (positive or negative) on people at particular risk of health inequalities (as listed below). Please state **N/A** if your proposal will not impact on patients who experience health inequalities.

² Addressing racial inequalities is about identifying any ethnic group that experiences inequalities. Race and ethnicity includes people from any ethnic group incl. BME communities, non-English speakers, Gypsies, Roma and Travelers, migrants etc.. who experience inequalities so includes addressing the needs of BME communities but is not limited to addressing their needs, it is equally important to recognise the needs of White groups that experience inequalities. The Equality Act 2010 also prohibits discrimination on the basis of nationality and ethnic or national origins, issues related to national origin and nationality.

Groups who face health inequalities³	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
Looked after children and young people	<p>Families and patients are considered to spend on average 19 hours per week on dietary compliance, thus affecting every aspect of life and testing patient's self-control. This may be an additional challenge for carers or looked after children. Sapropterin treatment may lessen the requirement for patient carers to monitor the child's protein intake, with a lessening of stress associated with this.</p> <p>Poor phenylalanine control are grounds for social services intervention and therefore child safeguarding measures can apply to children with PKU.</p>	Sapropterin to be made available as a treatment option.
Carers of patients: unpaid, family members.	Families and patients are considered to spend on average 19 hours per week on dietary compliance, thus affecting every aspect of life and testing patient's self-control. Caring responsibilities for patients with PKU can lead to parents to change their job or working hours, so reducing income. Sapropterin treatment may lessen the requirement for patient carers to monitor their child or relatives'	Sapropterin to be made available as a treatment option.

³ Please note many groups who share protected characteristics have also been identified as facing health inequalities.

Groups who face health inequalities ³	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	protein intake, with a lessening of stress associated with this.	
<p>Homeless people. People on the street; staying temporarily with friends /family; in hostels or B&Bs.</p>	<p>Patients with PKU without fixed abode, or staying in temporary housing may lack access to appropriate cooking and food storage facilities to facilitate adherence to dietary requirements. While homeless people do continue to face difficulties accessing prescription medications, the availability of sapropterin may mitigate the negative impacts of poor access to dietary measures</p>	<p>Sapropterin to be made available as a treatment option.</p>
<p>People involved in the criminal justice system: offenders in prison/on probation, ex-offenders.</p>	<p>While people in detained settings may face difficulties in respect of adherence to strict dietary requirements and / or accessing prescription medications, the availability of sapropterin may mitigate the negative impacts of poor access to dietary measures</p>	
<p>People with addictions and/or substance misuse issues</p>	<p>Substance misuse will impact the ability of parents to ensure adherence to dietary treatment of children with PKU. Substance misuse will also impair</p>	

Groups who face health inequalities ³	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	individuals own ability to manage PKU dietary care.	
People or families on a low income	<p>While people in on low incomes may face difficulties in accessing foods necessary to achieve adherence to strict dietary requirements, the availability of sapropterin may mitigate the negative impacts of poor access to dietary measures.</p> <p>Social issues impacting the family will be a risk factor for poor adherence and poor outcomes in children with PKU.</p>	Sapropterin to be made available to patients of all ages
People with poor literacy or health Literacy: (e.g. poor understanding of health services poor language skills).	Improved Phe levels following sapropterin treatment may facilitate relaxed dietary restrictions, therefore reducing the need to read food labels, weigh food and monitor food intake. This is likely to be of more benefit for patients with difficulties with literacy or numeracy.	Sapropterin to be made available to patients of all ages
People living in deprived areas	While people in deprived areas may face difficulties in accessing foods necessary to achieve adherence to strict dietary requirements, the availability of sapropterin may mitigate	

Groups who face health inequalities ³	Summary explanation of the main potential positive or adverse impact of your proposal	Main recommendation from your proposal to reduce any key identified adverse impact or to increase the identified positive impact
	the negative impacts of poor access to dietary measures.	
People living in remote, rural and island locations	While people in remote, rural or island locations may face difficulties in accessing foods necessary to achieve adherence to strict dietary requirements, the availability of sapropterin may mitigate the negative impacts of poor access to dietary measures.	
Refugees, asylum seekers or those experiencing modern slavery	While refugees, asylum seekers and those experiencing modern slavery may face difficulties in respect of adherence to strict dietary requirements and / or accessing prescription medications, the availability of sapropterin may mitigate the negative impacts of poor access to dietary measures.	
Other groups experiencing health inequalities (please describe)	Individuals living in care homes – PKU is a complex and time consuming diet and is not consistently managed in hospital or care home settings.	

5. Engagement and consultation

a. Have any key engagement or consultative activities been undertaken that considered how to address equalities issues or reduce health inequalities? Please place an x in the appropriate box below.

Yes	X	No	Do Not Know
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b. If yes, please briefly list up the top 3 most important engagement or consultation activities undertaken, the main findings and when the engagement and consultative activities were undertaken.

Name of engagement and consultative activities undertaken		Summary note of the engagement or consultative activity undertaken	Month/Year
1	Involvement of Patient-public voice representative	Advice from representative from NSPKU regarding the impact of the dietary restrictions on parent-child relationships and social inclusion	
2	Public consultation	Planned	
3	Patient Impact Report		

6. What key sources of evidence have informed your impact assessment and are there key gaps in the evidence?

Evidence Type	Key sources of available evidence	Key gaps in evidence
Published evidence	Evidence review completed by NICE.	The evidence review has identified clinical benefit for patients including: reduced Phe blood levels, improved Phe tolerance, reduced use of artificial amino acid supplements and improved inattention in children with ADHD symptoms.

Evidence Type	Key sources of available evidence	Key gaps in evidence
		However, the published evidence has not proven an associated improvement in quality of life, and in overall cognitive outcomes. This contrasts with the experience of clinicians on the policy working group who have treated patients with sapropterin and is a key area of further research.
Consultation and involvement findings	Consultation awaited	
Research	N/a	
Participant or expert knowledge For example, expertise with the team or expertise drawn on external to your team	Consultants of metabolic medicine and specialist dieticians have been involved in guiding the evidence review and producing the policy proposition. The Patient Voice representative, able to represent the lived experience of people with PKU, has been involved throughout the development of the proposal.	

7. Is your assessment that your proposal **will** support compliance with the Public Sector Equality Duty? Please add an x to the relevant box below.

	Tackling discrimination	Advancing equality of opportunity	Fostering good relations
The proposal will support?			
The proposal may support?	X	X	
Uncertain whether the proposal will support?			X

8. Is your assessment that your proposal will support reducing health inequalities faced by patients? Please add an x to the relevant box below.

	Reducing inequalities in access to health care	Reducing inequalities in health outcomes
The proposal will support?		
The proposal may support?	X	X
Uncertain whether the proposal will support?		

9. Outstanding key issues/questions that may require further research/additional evidence. Please list your top 3 in order of priority or state N/A

Key issue or question to be answered	Type of research or other evidence that would address the issue and/or answer the question
1 Impact of sapropterin on quality of life and overall cognitive outcomes, with a focus on the patients from groups at risk of experiencing health inequalities, as identified in this assessment.	High quality RCTs with subgroup analysis to identify benefit to patients at risk of experiencing health inequalities.
2	
3	

10. Summary assessment of this EHIA findings

This assessment should summarise whether the findings are that this proposal may or may not make a contribution to advancing equality of opportunity and/or reducing health inequalities, if no impact is identified please summarise why below.

Patients with the following protected characteristics are disproportionately impacted by the adverse effects of PKU: children and young people, adults and children with intellectual disabilities and pregnant women. Patients at risk of health inequalities are also disproportionately impacted both by the dietary restrictions and the adverse outcomes of raised Phe levels including: carers of patients, looked -after children, homeless people, people living in deprived areas, people living in remote areas, people living in deprived areas, people in the criminal justice system, asylum seekers and refugees and patients with poor health literacy or literacy. Subject to approval of the policy proposition which would provide access to individuals meeting certain clinical criteria aimed at ensuring treatment is given to those who will benefit from it, then the proposal may assist in meeting the PSED and reducing health inequalities.

Internal decision-making not for external circulation

11. Contact details re this EHIA

Team/Unit name:	National clinical policy team/ Specialised commissioning		
Contact officer name and post title:	Helen Cooper, clinical policy fellow		
Contact officer e: mail address:	Antonia.cooper@nhs.net		
Contact officer mobile number:	07702408952		
Division name: Specialised commissioning	Directorate name: Medical directorate		

12. Do you or your team need any key assistance to finalise this EHIA? Please delete the incorrect responses. If you require assistance please submit this EHIA and the associated proposal to EHIU (england.eandhi@nhs.net)

Yes X	No		Uncertain	N/A
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13. Assistance sought re the completion of this EHIA:

14

Yes	No
Review of appropriateness of identified impacts, particularly in relation to groups experiencing health inequalities.	

14. Responsibility for EHIA and decision-making

Name of senior manager/ responsible Director: Claire Foreman	Post title: Head of acute programmes	E-mail address: claireforeman@nhs.net
Division name: Specialised commissioning	Directorate name: Medical directorate	

15. Considered by NHS England or NHS Improvement Board or Committee ⁴

Yes	No	Name of the Board or Committee:		
Decision of the Board or Committee	Rejected proposal	Approved proposal unamended	Approved proposal with amendments in relation to equality and health inequalities	
Proposal gave due regard to the requirements of the PSED?		Yes	No	N/A
Summary comments				
Proposal gave regard to reducing health inequalities?		Yes	No	N/A

⁴ This section should only be completed if the proposal is considered by a Board or Committee. If it will not be considered by a Board or Committee please respond N/A.

Summary comments	
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16. Key dates

Date draft EHIA completed:	5 th March
Date draft EHIA circulated to EHIU: ⁵	5 th March
Date draft EHIA cleared by EHIU: ⁶	
Date final EHIA produced:	
Date signed off by Senior Manager/Director: ⁷	
Date considered by Board or Committee:	
Date EHIA published if applicable:	
Review date if applicable ⁸ :	

⁵ If the team producing the proposal has important unresolved issues or questions in relation to equality or health inequalities issues, the advice of the EHIU should be sought. A draft EHIA must also be completed, and attached to the proposal, if the proposal is to be considered through NHS England and NHS Improvement’s Gateway process.

⁶ If the EHIU raises concerns about the proposal, the EHIA should state how these concerns have been addressed in the final proposal.

⁷ The Senior Manager or Director responsible for signing off the proposal is also responsible for signing off the EHIA.

⁸ This will normally be the review date for the proposal unless a decision has been made to have an earlier review date.