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

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): Delayed-release mercaptamine bitartrate for patients with nephropathic cystinosis (age >1 year) [URN 2338]

2. Brief summary of the proposal in a few sentences

Nephropathic cystinosis is a rare autosomal recessive genetic disease. It is an incurable condition that results in the accumulation of cystine crystals within almost any cell in the body. This reduces life expectancy and is associated with significant morbidity throughout childhood and adult life. The physical manifestations of nephropathic cystinosis include renal involvement, with children presenting with Fanconi syndrome, developing into chronic kidney disease often requiring dialysis or transplant in adulthood. The only current licensed treatment is immediate release (IR) mercaptamine bitartrate. This acts to reduce cystine accumulation in cells, and when started early, it delays the development of renal failure and other complications. IR mercaptamine bitartrate has a strict 6-hour dosing schedule, taken preferably with or just after food. Strict adherence to the dosing schedule is required to achieve optimal white blood cell cysteine levels. Poor short-term control of cystine levels due to delayed or skipped doses leads to abnormal accumulation of cystine and irreversible cellular damage. Routine poor adherence with the dosing schedule leads to poor patient outcomes, such as faster time to renal failure requiring transplant or dialysis. Therefore, patients and carers of young children must wake during the night to be fully compliant with the dosing schedule and children and adolescents in education will also need medication during the school day. In addition to difficulties keeping to the strict dosing regimen, tolerability issues such as GI side effects, halitosis and body odour may also deter patients from taking treatment as recommended. As a result, up to 75% of patients do not fully adhere to the regimen, which can result in poor disease control.

The policy is for the use of delayed-release (DR) mercaptamine bitartrate taken every 12 hours in patients who have difficulty adhering to the current IR treatment or are unable to tolerate IR mercaptamine bitartrate with a view to improving adherence and therefore improving disease outcomes.

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

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
Age: older people; middle years; early years; children and young people.	Nephropathic cystinosis if a lifelong condition, although it is generally diagnosed in children before the age of 2 years. The policy is for patients who are aged 1 year and older, in keeping with the evidence returned in the independent evidence review. Young children have higher sleep needs than adults. While the current regimen is disruptive for both children and adults, it is more disruptive for the youngest patients for whom sleep directly relates to their development. Therefore, this policy may have an increased potential positive in this younger age group, who could benefit more from this less disruptive medication regimen. Young people in school may find taking medication during the school day very disruptive. Side effects such as halitosis and body odour may have an impact in social situations and personal relationships at school. Therefore, they are likely to see a benefit in a reduced twice daily dosing schedule, resulting in a more normal school life	This policy aims to make DR mercaptamine bitartrate available for patients of who meet the eligibility criteria. Patients under the age of 1 year with continue to be able to access current standard treatment, IR mercaptamine bitartrate.

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
Disability: physical, sensory and learning impairment; mental health condition; long-term conditions.	Nephropathic cystinosis is a long-term condition that results in chronic kidney disease possibly requiring dialysis or transplant by adulthood. The aim of the policy is to improve patients' outcomes whilst on DR mercaptamine bitartrate, potentially delaying the progression of chronic kidney disease and other complications including thyroid disease, diabetes and neurological function by improving adherence, providing a potential positive impact. However, as nephropathic cystinosis is managed in specialised centres, it is acknowledged that people living with disabilities may face increased travel costs to attend hospitals for appointments. Cystinosis centres are required to establish a networked model of care to enable services to be delivered as part of a co-ordinated system of care including local hospitals and primary and community care services providing expert services and delivering care close to patients' homes where possible. In addition, this impact may be offset by a reduction in emergency or unscheduled care.	Commissioned providers should work with the patient and other relevant agencies (e.g. GP, Local Authority, charities) to ensure adequate referral access and attendance support for people living with disabilities. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access. Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/
Gender Reassignment and/or people who identify as Transgender	There should be no direct negative or positive impact on this group as people who have undergone gender reassignment and/or people who identify as transgender have not been identified as a high-risk group.	The service is inclusive of all individuals if they meet the inclusion criteria.

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
Marriage & Civil Partnership: people married or in a civil partnership.	There should be no direct negative or positive impact on this group as marriage/civil partnership has not been identified as a high-risk group.	The service is inclusive of all individuals if they meet the inclusion criteria.
Pregnancy and Maternity: women before and after childbirth and who are breastfeeding.	Nephropathic cystinosis is a life-long condition, and therefore DR mercaptamine bitartrate has the potential to impact on this protected group. Pregnancy: There is no adequate data from the use of delayed release mercaptamine bitartrate in pregnant women. Studies in animals have shown reproductive toxicity, including teratogenesis (see section 5.3 of SmPC). The potential risk for humans is unknown. The effect on pregnancy is unknown. Therefore, it should not be used in pregnancy, particularly during the first trimester, unless clearly necessary (Summary of Product Characteristics - SmPC). If a pregnancy is diagnosed or planned, the treatment should be carefully reconsidered." Breast-feeding: Cysteamine excretion in human milk is unknown. However, due to the results of animal studies in breast feeding female and neonates, breast-feeding is contra-indicated in women taking DR mercaptamine bitartrate (SmPC).	Nephropathic cystinosis is managed at specialist centres, allowing for MDT discussion and careful consideration in how to treat those that are planning on becoming pregnant or who are pregnant.

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[1]	Cystinosis affects approximately 1 in 100,000 to 200,000 newborns worldwide. It is not known to be more common in people of different race or ethnicity. However, the difficulty adhering the current regimen can lead to renal failure more quickly. People from ethnic minority groups often wait a year longer for a transplant than Caucasian patients. People from minority groups are also more likely to have type B blood and with current low organ donation rates from these populations, adding to the difficulty in suitability for transplant. This policy would benefit this protected characteristic, as improved adherence will postpone and reduce the need for transplant.	All patients who meet the inclusion criteria would be considered for DR mercaptamine bitartrate treatment. There should not be any adverse impact on this protected characteristic group through creating this policy.
Religion and belief: people with different religions/faiths or beliefs, or none.	Religion is not known to be a risk factor for nephropathic cystinosis. This policy will promote access to DR mercaptamine bitartrate regardless of religion.	All patients who meet the inclusion criteria would be considered for DR mercaptamine bitartrate treatment. There should not be any adverse impact on this protected characteristic group through creating this policy.

[1] 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.

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
Sex: men; women	Nephropathic cystinosis affects both males and females. Males with cystinosis may be infertile with negligible sperm counts due to the condition. Adherence to treatment can improve fertility and therefore this policy will have a particular benefit for men. There is no expected adverse impact on this protected characteristic group. This policy offers patients with nephropathic cystinosis DR mercaptamine bitartrate regardless of sex.	All patients who meet the inclusion criteria would be considered for DR mercaptamine bitartrate treatment. There should not be any adverse impact on this protected characteristic group through creating this policy.
Sexual orientation: Lesbian; Gay; Bisexual; Heterosexual.	There should be no direct negative or positive impact on people based on their sexual orientation compared to all patients with nephropathic cystinosis.	All patients who meet the inclusion criteria would be considered for DR mercaptamine bitartrate treatment. There should not be any adverse impact on this protected characteristic group through creating this policy.

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.**

Groups who face health inequalities[2]	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	Looked-after children and young people may face additional barriers to accessing therapeutics. Therefore, they may have less chance of meeting the eligibility criteria to receive DR mercaptamine bitartrate. The reduced medication burden may be of benefit to looked after children and young people as it will be less disruptive to their schooling and sleep. If eligible for treatment, looked after young people will require someone to bring them to appointments, provide consent for treatment, and guide administering of mercaptamine bitartrate, which may be more difficult than for children with a permanent guardian or carer.	The specialised cystinosis service has a responsibility to promote equity of access allowing everyone with cystinosis to follow a comprehensive disease care pathway.
Carers of patients: unpaid, family members.	Carers are expected to be directly positively affected by this policy. DR mercaptamine bitartrate is expected to significantly reduce carer burden, particularly in carers of young children. This is because it will reduce the dosing schedule to twice a day, rather than the current four times a day (including an	The availability of DR mercaptamine bitartrate will provide a treatment option for patients, potentially reducing carer burden. Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/

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

Groups who face health inequalities[2]	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
	overnight dose). This is expected to positively affect carers. However, starting the DR formulation may require an increase in hospital clinic appointments requiring carer support to facilitate this. This might be offset by a reduction in emergency and unscheduled care to address progressive renal disease.	Cystinosis centres support a service model to enable care to be delivered as close to patients' homes where possible. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access. Equity of access for all patients including those on low incomes who may find access particularly challenging should be considered.
Homeless people. People on the street; staying temporarily with friends /family; in hostels or B&Bs.	There are no identified potential positive or adverse impacts of this policy on this group. The lack of a permanent base for which follow-up appointments could be co-ordinated may be challenging in this cohort of patients. Those who are homeless could be at risk of adverse outcomes due to lack of access to services and/or incomplete follow up.	Consideration needs to be given to groups that face inequalities, including homeless people, to ensure that access to the service is enabled/supported.
People involved in the criminal justice system: offenders in prison/on probation, ex-offenders.	There are no identified potential positive or adverse impacts of this policy on this group.	Consideration needs to be given to groups that face inequalities, including people involved in the criminal justice system, to ensure that access to the service is enabled/supported.

Groups who face health inequalities[2]	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
People with addictions and/or substance misuse issues	There are no identified potential positive or adverse impacts of this policy on this group.	Consideration needs to be given to groups that face inequalities, including people with addiction and/or substance misuse issues, to ensure that access to the service is enabled/supported.
People or families on a low income	As nephropathic cystinosis care is managed at specialist centres, this may prove costly to families who are having to travel significant distances on a low income. However, a benefit may be seen in the improved quality of life for patients and their families with a reduced administration schedule. It is also noted that those from a lower socio-economic group are more likely to develop chronic kidney disease, progress faster towards kidney failure and die earlier. This policy will allow for a more tolerable regimen for patients in this group, providing a possible benefit.	Consideration needs to be given to groups that face inequalities, including people or families on low incomes, to ensure that access to the service is enabled/supported. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access. Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/
People with poor literacy or health Literacy: (e.g. poor understanding of health services poor language skills).	Individuals with poor literacy/health literacy may not have access to the same information and resources as those more literate. This may limit their agency and ability to access DR mercaptamine bitartrate.	Consideration needs to be given to groups that face inequalities, including people with poor literacy or health literacy, to ensure access to the service is enabled/supported. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access.

Groups who face health inequalities[2]	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
		Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/.
People living in deprived areas	As nephropathic cystinosis care is managed at specialist centres, this may prove difficult for patients living in deprived areas who are having to travel to centres from a deprived area.	Consideration needs to be given to groups that face inequalities, including people living in deprived areas, to ensure that access to the service is enabled/supported. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access. Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/.
People living in remote, rural and island locations	As nephropathic cystinosis care is managed at specialist centres, this may prove difficult for patients living in remote, rural and island locations who have to travel further.	Consideration needs to be given to groups that face inequalities, including people living in remote/ rural/ island locations, to ensure that access to the service is enabled/supported. Commissioned providers should establish shared care arrangements with the patient's local renal centre to support access. Prescribing centres need to ensure eligible patients and carers are aware of the NHS Healthcare Travel

Groups who face health inequalities[2]	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
		Costs Scheme https://www.nhs.uk/nhs-services/help-with-health-costs/healthcare-travel-costs-scheme-htcs/.
Refugees, asylum seekers or those experiencing modern slavery	Refugees and asylum seekers with an active application or appeal are fully entitled to free NHS care (British Medical Association, 2020). Refused asylum seekers are not necessarily entitled to secondary NHS care free of charge. Their ability to access care depends on whether the care is immediately necessary/urgent or non-urgent and whether specific exemptions apply. Refused asylum seekers must always receive immediately necessary and urgent treatment regardless of their chargeable status or ability to pay (BMA, 2020).	Commissioned providers should work with the patient and other relevant agencies (e.g., GP, Local Authority, charities) to ensure adequate referral, access and attendance support for refugees, asylum seeker or experiencing modern slavery.
Other groups experiencing health inequalities (please describe)	Not applicable	Not applicable

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

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	Stakeholder testing	There was a 2-week stakeholder engagement period with key stakeholders as per NHS England's standard methods.	7th-21st October 2024
2	Public Consultation	Not formally required	
3			

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	Independent evidence review commissioned.	No studies were identified that reported activities of daily living or progression of non-renal cystinosis associated disease. No cost-effectiveness studies were identified for inclusion.
Consultation and involvement findings (Planned)		
Research		
Participant or expert knowledge For example, expertise within the team or expertise drawn on external to your team		

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?		x	x
The proposal may support?	x		
Uncertain whether the proposal will support?			

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?	x	x
The proposal may support?		
Uncertain if the proposal will support?		

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

Key issue or question to be answered		Type of consultation, research or other evidence that would address the issue and/or answer the question
1		
2		
3		

10. Summary assessment of this EHIA findings

This policy will advance equality of opportunity and/or reducing health inequalities. As demonstrated in this document, carers who are having to administer a medication every 6 hours are hugely impacted by the current available treatment regimen. A 12-hour

dosing regimen will allow carers and patients to be able to sleep through the night, and reduce disruption during the day, reducing a burden on the entire family. The lack of sleep disruption will also improve the lives of adult patients. The difficulty adhering the current regimen can lead to renal failure more quickly. People from ethnic minority groups often wait a year longer for a transplant than Caucasian patients. People from minority groups are also more likely to have type B blood and with current low donation rates from these populations, adding to the difficulty in suitability for transplant. This policy would benefit this protected characteristic, as improved adherence will postpone and reduce the need for transplant.

11. Contact details re this EHIA

Team/Unit name:	Internal Medicine Programme of Care
Division name:	Specialised Commissioning
Directorate name:	System Development Group
Date EHIA agreed:	
Date EHIA published if appropriate:	