NHS England and NHS Improvement: 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)[1]:

Clinical Commissioning Policy Proposition: Alglucosidase alfa for patients with infantile-onset Pompe disease (all ages)

2. Brief summary of the proposal in a few sentences

This is a clinical commissioning policy for the use of alglucosidase alfa for patients with infantile-onset Pompe disease.

Pompe disease is a very rare disease which causes damage to muscles and can lead to mobility difficulty, breathing problems and death before two years in untreated patients. Infantile-onset Pompe disease is when the symptoms start in children under one year old. Patients with infantile-onset Pompe disease can be treated with a medication called alglucosidase alfa, which replaces the enzyme that may be missing or not working properly. Alglucosidase alfa is given as an injection into a vein.

This policy recommends the use of alglucosidase alfa at an increased frequency (once every week, rather than once every two weeks) and/or at an increase dose (40mg/kg rather than 20mg/kg).

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.	The policy is for infantile-onset Pompe disease. This includes patients that developed symptoms before 12 months of age. If patients are started on treatment before the age of 12 months and respond well, treatment can continue throughout childhood and into adulthood if required.	Treatment options for patients with late-onset Pompe disease will not be affected by this policy. The policy recommends an increased frequency and/or dose of alglucosidase alfa for patients with infantile-onset Pompe disease, which should improve outcomes for patients in this group. The policy is based upon an evidence review, which shows the clinical effectiveness and safety of this treatment regime in the infantile-onset Pompe disease population.
Disability: physical, sensory and learning impairment; mental health condition; long-term conditions.	Patients with infantile-onset Pompe disease who are not treated develop skeletal muscle weakness causing reduced exercise tolerance and may lead to a physical disability. The policy is expected to have a positive impact on this group.	Treatment of patients with infantile-onset Pompe disease with increased frequency and/or dose of alglucosidase alfa reduces their symptoms.
Gender Reassignment and/or people who identify as Transgender	No impact on this group has been identified.	N/A
Marriage & Civil Partnership: people married or in a civil partnership.	No impact on this group has been identified.	N/A
Pregnancy and Maternity: women before and after childbirth and who are breastfeeding.	There is no evidence available for the use of alglucosidase alfa in women who are pregnant or breastfeeding. The Summary of Product Characteristics recommend that alglucosidase alfa should not be used unless clearly	The treatment for infantile-onset Pompe disease is initiated when symptoms developed before 12 months of age. Therefore, this only applies to patients who are treated successfully during childhood and become pregnant later in life. Treatment with alglucosidase alfa may need to be

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
	necessary in this group of patients. This may cause a negative impact on this group.	temporarily withheld during pregnancy or breastfeeding and should be discussed with the specialist MDT.
Race and ethnicity[2]	The incidence of Pompe disease (infantile-onset and late-onset) varies across different ethnic groups and geographic location from as high as 1 in 14,000 in African American populations to 1 in 600,000 in Portuguese populations. Estimates of infantile-onset Pompe disease in Europe are around 1 in 138,000 births. However, the impact on these groups of this treatment is not known, as it is not known if the prognosis of infantile-onset Pompe disease is significantly influenced by ethnicity or if treatment outcomes are different in these groups.	The policy recommends the use of alglucosidase alfa at an increased frequency and/or dose for patients with infantile-onset Pompe disease. As this disease is more prevalent in African American populations, it may have a more positive impact on this group.
Religion and belief: people with different religions/faiths or beliefs, or none.	No impact on this group has been identified.	N/A

[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.

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	Most studies show no difference in the sex ratio between males and females with infantile-onset Pompe disease.	N/A
Sexual orientation: Lesbian; Gay; Bisexual; Heterosexual.	No impact on this group has been identified.	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.**

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
Looked after children and young people	The policy recommends alglucosidase alfa for patients with infantile-onset Pompe disease with symptom onset before 12 months of age. Therefore, treatment and follow-up are required at a young age and throughout childhood and into adulthood. Treatment is delivered intravenously, with regular follow-up with a specialist metabolic multidisciplinary team, which may require frequent travel to a tertiary specialist centre.	Access to a specialist centre will ensure accurate diagnosis and monitoring and appropriate recommendation for treatment due to clinical expertise and experience. Longer term impact on the quality of life could be improved through treatment of the patient, which in turn could have a beneficial impact on overall family life, work opportunities and prosperity.
Carers of patients: unpaid, family members.	The policy recommends alglucosidase alfa for patients with infantile-onset	Access to a specialist centre will ensure accurate diagnosis and monitoring and appropriate

[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
	Pompe disease with symptom onset before 12 months of age. Therefore, treatment and follow-up are required at a young age and throughout childhood and into adulthood. Treatment is delivered intravenously, with regular follow-up with a specialist metabolic multidisciplinary team, which may require frequent travel to a tertiary specialist centre.	recommendation for treatment due to clinical expertise and experience. Longer term impact on the quality of life could be improved through treatment of the patient, which in turn could have a beneficial impact on overall family life, work opportunities and prosperity.
Homeless people. People on the street; staying temporarily with friends /family; in hostels or B&Bs.	No impact on this group has been identified.	N/A
People involved in the criminal justice system: offenders in prison/on probation, ex-offenders.	No impact on this group has been identified.	N/A
People with addictions and/or substance misuse issues	No impact on this group has been identified.	N/A
People or families on a low income	Treatment and follow-up are required at a young age and throughout childhood and into adulthood. Treatment is delivered intravenously, with regular follow-up with a specialist metabolic multidisciplinary team, which may require frequent travel to a tertiary specialist centre.	Access to a specialist centre will ensure accurate diagnosis and monitoring and appropriate recommendation for treatment due to clinical expertise and experience. However, this may come at an increased travel cost to patients and carers.
People with poor literacy or health Literacy: (e.g. poor understanding of health services poor language skills).	No impact on this group has been identified.	N/A

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
People living in deprived areas	Treatment and follow-up are required at a young age and throughout childhood and into adulthood. Treatment is delivered intravenously, with regular follow-up with a specialist metabolic multidisciplinary team, which may require frequent travel to a tertiary specialist centre.	Access to a specialist centre will ensure accurate diagnosis and monitoring and appropriate recommendation for treatment due to clinical expertise and experience. However, this may come at an increased travel cost to patients and carers.
People living in remote, rural and island locations	Treatment and follow-up are required at a young age and throughout childhood and into adulthood. Treatment is delivered intravenously, with regular follow-up with a specialist metabolic multidisciplinary team, which may require frequent travel to a tertiary specialist centre.	Access to a specialist centre will ensure accurate diagnosis and monitoring and appropriate recommendation for treatment due to clinical expertise and experience. However, this may come at an increased travel cost to patients and carers.
Refugees, asylum seekers or those experiencing modern slavery	No impact on this group has been identified.	N/A
Other groups experiencing health inequalities (please describe)	No further impacts have been identified.	N/A

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 engagement (planned)	There will be a two-week stakeholder engagement period with key stakeholders as per NHS England's standard methods for policy development.	TBC
2	Policy working group	The policy working group that is developing the policy is made up of specialist clinicians, a public health consultant, pharmacist, a patient public voice representative, a commissioner and a clinical policy fellow to offer a wide range of opinions and backgrounds.	Throughout the policy development process
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	Ausems MG, Verbiest J, Hermans MP, Kroos MA, Beemer FA, Wokke JH, Sandkuijl LA, Reuser AJ, van der Ploeg AT. Frequency of glycogen storage disease type II in the Netherlands: implications for diagnosis and genetic counselling. European Journal of Human Genetics. 1999. 6(6):713-716. Elenga N, Verloes A, Mrsic Y, Basurko C, Schaub R, Cuadro-Alvarez E, Kom-Tchameni R, Carles G, Lambert V, Boukhari R, Fahrasmane A, Jolivet A, Nacher M, Benoist J-F. Incidence of infantile Pompe disease in the Maroon	N/A

Evidence Type	Key sources of available evidence	Key gaps in evidence
	population of French Guiana. BMJ Paediatrics Open. 2018, 2(1):e000182. Ficicioglu C, Ahrens-Nicklas RC, Barch J, Cuddapah SR, Diboscio BS, DiPerna JC, Gordon PL, Henderson N, Menello C, Luongo N, Ortiz D, Xiao R. Newborn screening for Pompe disease: Pennsylvania experience. International Journal of Neonatal Screening. 2020. 6(4): 89. Martiniuk F, Chen A, Mack A, Arvanitopoulos, E, Chen Y, Rom WN, Codd WJ, Hanna B, Alcabes P, Raben N, Plotz P. Carrier frequency for glycogen storage disease type II in New York and estimates of affected individuals born with the disease. American Journal of Medical Genetics. 1998. 79(1): 69-72.	
Consultation and involvement findings	TBC	N/A
Research	N/A	N/A
Participant or expert knowledge For example, expertise within the team or expertise drawn on external to your team	N/A	N/A

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?			
Uncertain whether the proposal will support?	X	X	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?		
Uncertain if the proposal will support?	X	X

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	N/A	
2		
3		

10. Summary assessment of this EHIA findings

The policy recommends an increased frequency and/or dose of alglucosidase alfa for patients with infantile-onset Pompe disease, which improves outcomes. To be eligible for treatment, symptoms must have onset before the age of 12 months. No change to treatment options are being made for patients with late-onset Pompe disease (where symptoms onset after the age of 12 months). Incidence is highest in the African American population and improved outcomes may have a bigger positive impact in this group.

11. Contact details re this EHIA

Team/Unit name:	
Division name:	
Directorate name:	
Date EHIA agreed:	
Date EHIA published if appropriate:	

Commented [DH1]: Needs the generic details in?