

Engagement Report

Topic details

Title of policy or policy statement: Allogenic Haematopoietic Stem Cell Transplantation (Allo-HSCT) for adult transfusion dependent thalassaemia (TDT)

Programme of Care: Blood and Infection

Clinical Reference Group: Blood and Marrow Transplant

URN: 2120

1. Summary

This report summarises the feedback NHS England received from engagement during the development of this policy, and how this feedback has been considered.

The policy proposition went out to stakeholder engagement between 5th July to 19th July 2022. There were 3 responses.

2. Background

This policy proposition proposes that allo-HSCT will be made available as a routine commissioning treatment option for adults with TDT.

TDT is a complex multi-system disease and cardiac, liver and bone disease are significant problems. Iron overload can cause tissue damage and impaired function of affected organs. Current management of adult patients with TDT is complex and generally involves supportive care. This policy proposition is restricted to adults as allo-HSCT is already commissioned for a number of disorders including children aged up to 18 with TDT. Allo-HSCT treats the underlying cause of the genetic defect and is intended to be offered to adult patients with TDT when a HLA-matched donor is found and they are deemed fit for transplant.

The clinical policy proposition was developed through conducting an externally conducted evidence review and by a Policy Working Group (PWG) consisting of transplant experts, haematology experts, patient representatives, a public health specialist and a specialist commissioner for NHS England.

3. Engagement

The Programme of Care (PoC) has decided that the proposition offers a clear and positive impact on patient treatment, by potentially making a new treatment available

which widens the range of treatment options without disrupting current care or limiting patient choice, and therefore further public consultation was not required. This decision has been assured by the Patient Public Voice Advisory Group.

Respondents were asked the following consultation questions:

- Do you support the proposition for Allogenic Haematopoietic Stem Cell Transplantation (Allo-HSCT) for adult transfusion dependent thalassaemia to be available through routine commissioning based on the evidence review and within the criteria set out in this document?
- Do you believe that there is any additional information that we should have considered in the evidence review?
- Do you believe that there are any positive and/or negative impacts on patient care as a result of making this treatment option available?
- Do you have any further comments on the proposal?
- Please provide any comments that you may have about the potential positive and negative impacts on equality and health inequalities which might arise as a result of the proposed policy that have been described?
- Does the Patient Impact Summary present a true reflection of the patient and carers lived experience of this condition?
- Do you declare any conflict of interests relating to this document or service area?

4. Engagement Results

Three responses were received: one pharmaceutical company, one clinician and one individual.

All respondents supported the policy proposition and deemed that it would have a positive impact on patients.

In line with the 13Q assessment, further public consultation was deemed to not be required.

5. How has feedback been considered?

Responses to engagement have been reviewed by the Policy Working Group and the Blood and Infection PoC. The following themes were raised during engagement:

Keys themes in feedback	NHS England Response
Positive/negative impacts on patient care	
This is a potential curative intervention. A successful transplant can be transformational both physically and psychologically for patients.	Noted
There are risks to fertility, but appropriate interventions to support fertility have been considered.	Noted
Proposed Patient Pathway	
The patient numbers impacted seems low. It hasn't been possible to review any Service Impact report but whether the	This will be addressed in the financial modelling of the policy proposition.

number is as low as suggested or higher, this organisation is keen to confirm the additional costs such as prophylaxis used amongst many other supportive agents and services with in the care of each transplant patient are also fully funded.	
Changes/addition to policy	
No changes	Noted

6. Has anything been changed in the policy proposition as a result of the stakeholder testing and consultation?

No changes have been made to the policy proposition based on the engagement response.

7. Are there any remaining concerns outstanding following the consultation that have not been resolved in the final policy proposition?

No.