

Consultation Report

Topic details

Title of policy or policy statement:	Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages)
Programme of Care:	Blood and Infection
Clinical Reference Group:	F01-BMT
URN:	1742

1. Summary

This report summarises the outcome of a public consultation that was undertaken to test the policy proposition.

2. Background

Primary Immunodeficiencies (PID) are a group of rare inherited diseases affecting the immune system. The roles of the immune system are to recognise and attack infection, respond to tissue damage, perform tumour surveillance and prevent autoimmunity. Patients with severe PID may be unable to produce normal levels of immunoglobulins (antibodies), have dysfunctional immune cells, and are at risk of frequent and life-threatening infections, irreversible organ damage, severe inflammation, autoimmunity and cancer. Without treatment, many patients would die before reaching adulthood.

Allogeneic Haematopoietic Stem Cell Transplant (Allo-HSCT) replaces the patient's own bone marrow stem cells with healthy stem cells isolated from an appropriately tissue-type matched or mis-matched donor. As the inherited genetic mutation in PID affects immune cells derived from bone marrow stem cells, replacing the mutation carrying stem cells with healthy stem cells and is potentially the only cure for the immune deficiency. Allo-HSCT is currently available for children to the age of eighteen with PID and for adults under the Urgent Clinical Commissioning Policy Statement: Allogeneic stem cell transplantation for adults with Primary Immune Deficiency disorders this policy will provide an all ages policy based on clinical criteria for access rather than age.

3. Publication of consultation

The policy was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 22nd February to 25th March 2019. Consultation comments have then been shared with the Policy Working Group to enable full consideration of feedback and to support a decision on whether any changes to the policy might be recommended.

Respondents were asked the following consultation questions:

- Has all the relevant evidence been taken into account?
- Does the impact assessment fairly reflect the likely activity, budget and service impact? If not, what is inaccurate?
- Does the policy proposition accurately describe the current patient pathway that patients experience? If not, what is different?

- Please provide any comments that you may have about the potential impact on equality and health inequalities which might arise as a result of the proposed changes that have been described?
- Are there any changes or additions you think need to be made to this document, and why?

4. Results of consultation

A total of 11 responses were received, including 3 from patients and carers, 1 from clinician, 4 from patient groups, 3 service providers and 1 from professional bodies. In total, 9 responses indicated support for the proposition, Five of the eleven responses highlighted the policy will have a positive impact on equality, as it removes existing age limits, shifting to a policy based on clinical criteria for the intervention, rather than age-based criteria for access.

One response was concerned that the policy will impact on current providers of paediatric services. There is no plan to change current paediatric services and this will be communicated with publication of policy. One service provider noted that patients may see other professions not listed in pathway. The policy working group recognises that patients may see a variety of health professionals in pathway but it is not key to process.

Although one respondent did not agree that all the evidence had been considered and activity, budget and service impact of the policy had been accurately identified, the response did not include any further detail. Therefore, the PWG were unable to make any amendments based on this feedback.

5. How have consultation responses been considered?

Responses have been carefully considered and noted in line with the following categories:

- Level 1: Incorporated into draft document immediately to improve accuracy or clarity
- Level 2: Issue has already been considered by the CRG in its development and therefore draft document requires no further change
- Level 3: Could result in a more substantial change, requiring further consideration by the CRG in its work programme and as part of the next iteration of the document
- Level 4: Falls outside of the scope of the specification and NHS England's direct commissioning responsibility

6. Has anything been changed in the policy as a result of the consultation?

No changes were made to the policy proposition as a result of consultation because comments fell predominantly into Level 2 responses or were in support of the policy proposition as written

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

In order to address concerns regarding the implications of the proposition, we intend to write to commissioned providers of paediatric HSCT for primary immunodeficiencies to confirm there will be no changes to the currently commissioned services as a result of implementing the policy proposition. This action will be taken following approval of the policy.