Consultation Report

Topic details

Title of policy or policy statement:	Allogeneic Haematopoietic Stem Cell Transplantation for adults with sickle cell disease
Programme of Care:	Blood and Infection
Clinical Reference Group:	F01 BMT and F05 Haemoglobinopathies
URN:	1830

1. Summary

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

2. Background

Sickle cell disease (SCD) is an inherited disease affected around 12-15,000 individuals in the UK mainly of black and minority ethnic groups, especially certain subgroups of this including Black African, Black Caribbean and Arab-Indian backgrounds. It causes a lifelong anaemia, episodes of severe pain and other problems including an increased risk of stroke, renal failure, heart and lung problems and leg ulcers. It is associated with a reduced life expectancy, severe chronic health problems and reduction in quality of life. Allogenic haematopoietic stem cell transplantation (allo-HSCT) is the only currently available therapy that can cure sickle cell disease. It is currently offered to children, but not adults. The clinical effects of SCD are variable but those with severe sickle cell disease will require ongoing treatments and frequent hospital admissions. Allogeneic stem cell transplantation is a potential cure for sickle cell disease.

This policy for routine commissioning in patients with severe sickle cell disease with a related donor that is fully matched to the recipient (sibling). However, there is not enough evidence to make the allo-HSCT available for patients with severe sickle cell disease using a matched donor who is not related to the recipient (matched unrelated donor) or a related donor (e.g. sibling, parent, child) that is half matched to the recipient (haploidentical).

3. Publication of consultation

The policy proposition was published and sign-posted on NHS England's website and was open to consultation feedback for a period of 30 days from 13/09/2019 to 11/10/2019. Consultation comments have then been shared with the Policy Working Group (PWG) 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 accurate?
- 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 10 responses were received, including 2 from patients and carers, 4 from clinicians, 2 from patient groups, 1 service provider and 1 from a professional body. In total, 9 responses indicated support for the proposition (one did not provide any comments), 5 of the 10 responses highlighted the policy will have a positive impact on equality, as it removes existing age limits and extends the existing access to this treatment based on clinical criteria for the intervention to a particular sub group of adult patients following clinical review of the currently available evidence. It was highlighted that certain subgroups of people from black and minority ethnic backgrounds experienced a higher prevalence of SCD, including Black African, Black Caribbean and Arab-Indian backgrounds, but it was noted that this policy would apply across all patients with SCD. There was concern raised by 3 of the 10 respondents about the exclusion of patients who have a haploidentical match for a stem cell transplant but acknowledged the need for further evidence to support this indication.

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

Of the responses, most fell into level 2 as already having been considered by the PWG and therefore not resulting in any changes to the draft documents. Further clarification was made to the Integrated Impact Assessment following feedback on the number of patients eligible for this indication (from 1 respondent) and clarification on the populations most affected by this condition (from 2 respondents).

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

A minor amendment was made to the indications (section 8) for use in the policy proposition to clarify this following feedback from 1 respondent.

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

None.