Engagement Report for Clinical Commissioning Policy Proposition

Unique Reference Number	1742
Policy Title	Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages)
Accountable Commissioner	Rob Coster
Clinical Reference Group	F01. Blood and Marrow Transplant F06. Immunology and Allergy
Which stakeholders were contacted to be involved in policy development?	BMT and specialised Immunology and Aallergy CRG members and registered stakeholders.
Identify the relevant Royal College or Professional Society to the policy and indicate how they have been involved	British Society of Bone and Marrow Transplant (BSBMT)via CRG United Kingdom Primary Immunodeficiency Network (UKPIN) via Policy working group and CRG
Which stakeholders have actually been involved?	• Primary Immunodeficiency UK (PID UK) on PWG • Primary Immunodeficiency UK and Chronic Granulomatous Disorder Society • Newcastle Hospitals NHS Foundation Trust • Teenagers and Young Adults with Cancer's (TYAC) • 4 x individuals • BMT CRG • Immunology and Allergy CRG • Paediatrics CRG

Explain reason if there is any difference from previous question	N/A
Identify any particular stakeholder organisations that may be key to the policy development that you have approached that have yet to be engaged. Indicate why?	Nil
How have stakeholders been involved? What engagement methods have been used?	Policy working group meeting and subsequent contact for policy development. Stakeholder engagement process. 18 day email engagement exercise with registered stakeholders
What has happened or changed as a result of their input?	No change as result of stakeholder feedback
How are stakeholders being kept informed of progress with policy development as a result of their input?	Stakeholders will be kept informed of the policy's progress through NHS England's consultation portal website. Stakeholders who sent in comments have had an email response.
What level of wider public consultation is recommended by the CRG for the NPOC Board to agree as a result of	4 weeks public consultaion

stakeholder involvement?	

Organisation Responding	Feedback Received	PWG response
1. Individual	Respondent recommended up to 12 weeks consultation to include some additional proactive engagement activities during the live consultation period. They didn't provide any further comments	Noted by PWG
2. Teenagers and Young Adults with Cancer's (TYAC)	Respondent supported a consultation period of up to 6 weeks. The respondent provided the below comments: 'Overall, a very good policy proposition. If not already contained within referenced documents, the following should be considered: 1. The need for appropriate psychological support and input for those undergoing HSCT for PID, especially for the teenage and young adult (TYA) population. This should be part of the wider HSCT MDT 2. The need for appropriate social care support and input for those undergoing HSCT for PID, especially for the teenage and young adult (TYA) population with an emphasis on re-engaging with education / employment. This should be part of the wider HSCT MDT. 3. Clarification on the commissioning / funding of on-going treatment for patients in whom there is a graft failure or rejection and a 'rescue' HSCT is required. This may well extend beyond the	Noted Out of scope for policy Out of Scope of Policy Out of Scope of policy

	original '100-days' from the first HSCT procedure outlined in this proposal.' No conflicts of interest were declared.	
4. Individual	Respondent provided the below comment: This appears to reinforce the need for SCID testing at birth, clear benefits shown, let's make no further delay. No conflicts of interest were declared	Noted
5. Newcastle Hospitals NHS Foundation Trust	Respondent supported a public consultation period of up to 6 weeks. They provided the below comments: I am commenting as the Director of Adult Transplantation at Newcastle Hospitals. The excellent review of the available evidence indicates that allogeneic transplantation is the only effective therapy for a small number of adults with late, atypical or complex presentation of primary immunodeficiency. As a supra-regional PID centre for children, Newcastle has a high level of expertise and has been able to deliver transplantation for adults with PID from the Northern half of the UK within our existing capacity, in the last 2 years. Under the urgent interim commissioning policy, we are already experienced in transplantation of adults with CGD, Wiskott-Aldrich,	Comments Noted

	chronic active EBV, CARMIL2 and GATA2 mutation. The interim MDT process for adults has been collaborative and effective. Comments: 1. I have a very favourable impression of the potential benefits for selected patients and strongly support the all ages commissioning policy underpinned by a national MDT structure. 2. I fully support the strategy to have an all ages national review process. Adult practice has much to learn from paediatrics and patient care frequently requires a transition between services, not least when a successful outcome has been achieved in childhood. 3. Although there is clinical overlap, I would suggest that the categories HLH and chronic active EBV are treated separately. They have distinct pathways of care leading up to transplantation and probably very different risk profiles that would be better treated individually for the purposes of governance and audit. The respondent didn't declare any conflicts of interest.	

6. Individual	The respondent supported a consultation period of up to 6 weeks. No further comments were provided. The respondent declared that whey were involved in the policy development as a conflict of interests.	Comments noted
7. Primary Immunodeficiency UK and Chronic Granulomatous Disorder Society	The respondent supported a public consultation period of up to 6 weeks. The only comment given was that PID UK and the CGD Society are highly supportive of the proposed policy which will enable eligible patients to access HSCT regardless of their age. The respondent didn't declare any conflicts of interest.	Comments noted
8. The UK Primary Immunodeficiency Network (UKPIN)	The respondent supported a public consultation period of up to 6 weeks. They provided the below comments: This new policy proposes a change in the commission of haematopoietic stem cell transplant (HSCT) for Primary Immunodeficiency (PID), from a Paediatric only, to an all ages policy. This change appropriately reflects the greater understanding we now have of primary immunodeficiency, as a consequence of advances in,	Comments noted

	and greater availability of next generation genetic sequencing, and advances in HSCT in adult patients with PID. By extending the policy to all ages, this makes the availability of HSCT more equitable, and offers curative and life transforming treatment to adults. Although there is not yet definitive outcome evidence to support HSCT as compared to conservative treatment, as the professional body representing UK immunologists, specialist nurses and healthcare scientists working in PID, we believe HSCT for highly selected adult PID patients is likely to improve life expectance, quality of life and reduce long term healthcare costs. In our opinion, the evidence presented on HSCT outcome in PID (all patients and adult alone), is strong enough to support the benefit of HSCT, as compared to the previous expert clinical experience of managing these highly selected patients with conservative treatment, whatever their age. The potential availability of HSCT for adults offers further benefits beyond equity of treatment. Decisions around transplant for many patients with PID outside infancy are extremely difficult. Many parents struggle with consenting their children for an HSCT with a fixed immediate risk of mortality, where the benefit of HSCT is improved medium term survival and quality of life. By imposing an artificial “age limit” for HSCT, the opportunity for a young adult to consent to an HSCT procedure themselves is removed. Within an “all ages” policy this additional complication of the HSCT decision is removed.	

	Advances in genetic sequencing offers the potential of "Personalised Medicine", where a specific genetic diagnosis guides optimal ongoing treatment. A key therapeutic option in this setting is HSCT. If adults diagnosed with PID are to be offered the most clinically effective and cost effective treatments, as guided by their genetic diagnosis, then HSCT must be available as a treatment option for patients with the most severe genetic diagnoses. Because PID patients who may be considered for HSCT are extremely rare, and the decision around whether to proceed to transplant can be extremely difficult, it is essential that patients are discussed in an expert, multidisciplinary forum as proposed in the policy. We would also agree that HSCTs for PID should continue to be performed in centres with sufficient experience and outcomes that are on par nationally.. We feel that the proposed inclusion and exclusion criteria for PID HSCT are appropriate and would not propose any changes. No conflicts of interests were declared.	