

**SPECIALISED COMMISSIONING - CLINICAL EVIDENCE EVALUATION
CRITERIA FOR A PROPOSITION FOR A CLINICAL COMMISSIONING POLICY
FOR ROUTINE COMMISSIONING**

URN: 1742

TITLE: Allogeneic haematopoietic stem cell transplant for primary immunodeficiencies (all ages)

CRG: Blood & Marrow Transplant

NPOC: Blood & Infection

Lead: Claire Foreman

Date: 09/05/18

This policy is being considered for:	For routine commissioning	X	Not for routine commissioning	
Is the population described in the policy the same as that in the evidence review including subgroups?	No. The evidence review identified a large number of paper and applying the evidence review methodology, restricted studies to those with over 100 participants. These studies included very few adults and participants were largely children. The PWG advise that there are trials in adults that would usefully inform the policy, but have fewer than 100 participants.			
Is the intervention described in the policy the same or similar as the intervention for which evidence is presented in the evidence review?	Yes. Panel noted that studies suggest that more recently treated patients may have better outcomes than patients treated in more historic studies.			
Is the comparator in the policy the same as that in the evidence review? Are the comparators in the evidence review the most plausible comparators for patients in the English NHS and are they suitable for informing policy development?	The studies did not include a control group, with conclusions based on expected outcomes for patients with these conditions.			
Are the clinical benefits demonstrated in the evidence review consistent with the eligible population and/or subgroups presented in the policy? Are the clinical harms	Yes. Clinical Panel noted that although there are large studies, these are not controlled and may tend to overestimate the benefits compared with those reported in randomised controlled trails.			

<p>demonstrated in the evidence review reflected in the eligible and /or ineligible population and/or subgroups presented in the policy?</p>	<p>Yes. The harms are well recognised. Stem cell transplant is associated with a significant risk of death and other complications. A number of clinical features increase the risk of poor outcome, such as active infection and significant organ damage.</p>
<p>Rationale Is the rationale clearly linked to the evidence?</p>	<p>The Panel understand that the fundamental basis for the inclusion criteria is that the benefits of transplant are likely to exceed the risk. The expected course of the underlying condition is key in making this judgement. Stem cell transplant is established for children however, a further evidence review is required to include adults to ensure that extrapolation of outcomes from children to adults is justified.</p> <p>The Panel has recommended some adaptations to the inclusion criteria, including the removal of bullet point 8 and further clarification on when the benefits are likely to significantly outweigh the risks. The Panel were clear that a decision on treatment should be determined by a national MDT. This is because the national MDT is best placed to understand the natural history of the condition and reach a judgement on whether the benefits of stem cell transplant are likely to significantly outweigh the risks.</p> <p>The amended policy should be discussed with Clinical Panel Chair when the additional evidence review covering the adult population is available. The revised policy should then return to Panel.</p>
<p><u>Advice</u> The Panel should provide advice on matters relating to the evidence base and policy development and prioritisation. Advice may cover:</p> <ul style="list-style-type: none"> • Uncertainty in the evidence base • Challenges in the clinical interpretation and applicability of policy in clinical practice • Challenges in ensuring policy is applied appropriately • Likely changes in the pathway of care and therapeutic advances that may result in the 	<p>The Panel noted that an existing urgent policy statement will be replaced by this policy.</p> <p>The Panel requested that amendments were made as outlined above. In addition:</p> <ul style="list-style-type: none"> • Section 7 – identify which criteria are built upon evidence base and which are based upon clinical consensus. This should be explicit. • Bullet point 8 will be removed. This will be replaced by a statement to clarify that patients should have an assessment by a national MDT in England as likely to derive a significant net benefit of transplant, taking into account the risk of transplant. • Panel are aware of the significant cost of stem cell transplant and advise that the policy should identify patients with a <u>significant net</u> benefit taking account of the risk and the underlying disease. Marginal net benefit would be unlikely to represent value to the NHS.

need for policy review.			
Overall conclusion	This is a proposition for routine commissioning and	Should proceed for routine commissioning	X
		Should reversed and proceed as not for routine commissioning	
	This is a proposition for not routine commissioning and	Should proceed for not routine commissioning	
		Should be reconsidered by the PWG	

Overall conclusions of the panel

Report approved by:

David Black

Clinical Panel Co-Chair

22nd May 2018

Post meeting note:

In response to panel advice, it has been made clear in the commissioning criteria, which criteria are built upon evidence base and which are based upon clinical consensus. However the PWG and POCB questioned the validity of this request in the policy proposition as it is not consistent with other policies. The PWG expressed concern that this will be used to remove criteria currently in use for the commissioning of paediatric HSCT for PID

Bullet point 8 has been removed as requested and replaced with a statement to clarify that patients should have an assessment by a regional/national MDT in England as likely to derive a significant net benefit of transplant, taking into account the risk of transplant.

The final point was considered by the PWG and they felt the proposed criteria for commissioning reflects the significant benefit of transplant as the only available cure for these patients where the PID is potentially life threatening and cannot be managed effectively with treatment of symptoms alone. The overall impact on NHS will be taken into account in the impact assessment.