**NHS ENGLAND SPECIALISED SERVICES**

# CLINICAL PANEL REPORT

Date: May 2022

Intervention: Allogeneic Haematopoietic Stem Cell Transplantation (Allo HSCT)

Indication: Transfusion dependent thalassaemia (adult)

URN: 2120

Gateway: 2, Round 2

Programme: Blood and Infection

CRG: Haemoglobinopathies

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## Information provided to the Panel

Evidence Review undertaken by NICE

Evidence to Decision Making Report

Clinical Priorities Advisory Group (CPAG) Summary Report

Policy Proposition – tracked and clean versions

Blueteq™ Form

Equalities and Health Inequalities Assessment (EHIA)

Patient Impact Assessment (PIA)

Policy Working Group (PWG) Appendix

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This policy proposition recommends allogenic haematopoietic stem cell transplantation (allo-HSCT) as a curative treatment option for adults with transfusion dependent thalassaemia (TDT). TDT is a complex multi-system disease and cardiac, liver and bone disease are significant problems. This was considered at Clinical Panel in April 2022 and further work was requested, mainly for clarification.

Clinical Panel were presented with the actions requested at the last meeting and the Policy Working Group's consideration and response to those. Panel members considered these had been addressed adequately.

EHIA – no further comments received.

PIA – no further comments received.

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## Recommendation

Clinical Panel recommends that the proposition progresses as a routine commissioning policy proposition.

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## Why the panel made these recommendations

Clinical Panel members considered that the amendments made were satisfactory and the proposition reflects the evidence base.

**Documentation amendments required**

Policy Proposition:

- Include a sentence stating that this treatment is intended to be curative and therefore no further transfusions or iron overload chelation therapy would be required, unless the transplant failed.

  The following statements in the policy proposition were amended to include the above additions:
  *Allo-HSCT is also known as bone marrow transplantation (BMT). It is a curative intervention already used to treat a wide spectrum of disorders, including children with TDT up to the age of 18 years.*

  *Allo-HSCT treats the underlying cause of the genetic defect. It is a curative intervention for patients with TDT and patients who undergo HSCT will have no ongoing transfusion or iron chelation requirement. Indeed, failure of the transplant results in a recurrence of transfusion dependence.*

Declarations of Interest of Panel Members: None

Panel Chair: Anthony Kessel, Clinical Director, National Clinical Policy Team

**POC assurance**: The amendments to the PP have been made by the clinical fellow and the PWG through June 2022.

**Post panel amendment**

4th July 2023

During the assurance process for prioritisation, it was noted that the EHIA refferred to current pregnancy and breastfeeding being an exclusion criterion. However, the policy proposition did not reflect this exclusion.

This exclusion criterion was based on the conditioning chemotherapy being harmful to the foetus and was agreed with the Policy Working Group. This change has also been approved by the Medicines Lead, Clinical Policy Director and Medical Director and has been reflected in the policy proposition document.