



**Clinical Commissioning Policy:
Haematopoietic Stem Cell
Transplantation (HSCT) (All
Ages): Revised**

Reference: NHS England B04/P/a

NHS England

Clinical Commissioning

Policy:

Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised

First published: April 2013

Revised: January 2015

**Prepared by NHS England Specialised Services Clinical Reference Group for
Blood and Marrow Transplantation**

Published by NHS England, in electronic format only

NHS England INFORMATION READER BOX**Directorate**

Medical	Commissioning Operations	Patients and Information
Nursing	Trans. & Corp. Ops.	Commissioning Strategy
Finance		

Publications Gateway Reference: 02663

Document Purpose	Policy
Document Name	B04/P/a Clinical Commissioning Policy: Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised
Author	NHS England
Publication Date	January 2015
Target Audience	CCG Clinical Leaders, CCG Accountable Officers, Foundation Trust CEs , Medical Directors, Directors of PH, Directors of Nursing, NHS England Regional Directors, NHS England Area Directors, Directors of Finance, Communications Leads, NHS Trust CEs
Additional Circulation List	
Description	NHS England will routinely commission Haematopoietic Stem Cell Transplantation (HSCT) for adults and children as set out in the policy. The revision clarifies the criteria and reporting requirements for 'double cord' transplants, the exclusions to the policy and the role of expert advice in assessment of individual funding requests.
Cross Reference	n/a
Superseded Docs (if applicable)	B04/P/a Clinical Commissioning Policy: Haematopoietic Stem Cell Transplantation (HSCT) (All Ages)
Action Required	n/a
Timing / Deadlines (if applicable)	
Contact Details for further information	NHS England Medical Directorate Skipton House London Road SE1 6LH 0300 311 22 33 http://www.england.nhs.uk/commissioning/spec-services/

Document Status

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Policy Statement

NHS England will commission haematopoietic stem cell transplantation for the clinical conditions and their sub-groups indicated in accordance with the criteria outlined in this document.

In creating this policy NHS England has reviewed this clinical condition and the options for its treatment. It has considered the place of this treatment in current clinical practice, whether scientific research has shown the treatment to be of benefit to patients, (including how any benefit is balanced against possible risks) and whether its use represents the best use of NHS resources.

This policy document outlines the arrangements for funding of this treatment for the population in England.

Equality Statement

Throughout the production of this document, due regard has been given to eliminate discrimination, harassment and victimisation, to advance equality of opportunity, and to foster good relations between people who share a relevant protected characteristic (as cited in under the Equality Act 2010) and those who do not share it.

Plain Language Summary

Haematopoietic stem cell transplantation is also known as blood and marrow transplantation (BMT). It is used to treat a wide spectrum of disorders. It is broadly divided into two main groups: autologous and allogeneic transplantation.

Allogeneic haemopoietic stem cell transplantation (HSCT) is used to treat carefully selected patients with a range of malignant and non-malignant blood-related disorders and other specific disorders of the immune system. It involves replacing the bone marrow stem cells of a patient following high-dose therapy, with stem cells from a tissue-type matched or mismatched donor.

Autologous transplantation uses the patient's own stem cells, which are harvested prior to high-dose therapy. It enables the patient to be treated with doses of chemotherapy which are higher than would be possible without subsequent replacement of the harvested cells, because the therapy destroys the patient's remaining stem cell tissue.

This policy will promote equity of access to treatment in England. It confirms the indications for which NHS England has agreed routine funding and the route for obtaining funding for conditions outside this policy.

1. Introduction

Haematopoietic stem cell transplantation (HSCT), also known as blood and marrow transplantation (BMT) is used to treat wide spectrum of haematological, and increasingly, non-haematological disorders. It is broadly divided into two main groups: autologous and allogeneic transplantation. These are explained in more detail in the next section.

Stem cell transplantation, particularly allogeneic transplantation, is a high cost and highly specialised procedure, performed by skilled and experienced transplant teams working in specialist centres. Allogeneic transplantation carries a relatively high mortality and morbidity, and these must be weighed against the potential longer-term survival benefits when considering a patient for transplantation. Rigorous patient selection is of paramount importance.

Because of the large number of possible indications for stem cell transplantation, the degree of variation in clinically important patient and disease parameters, and the diversity of conditioning and transplant regimes, it is extremely difficult to evaluate the clinical and cost-effectiveness of transplantation for every potential clinical condition. Moreover, age is an important factor in determining outcomes; thus the management of children and young people is very different to that in older adults. For all these reasons, current clinical practice in stem cell transplantation is largely based on clinical consensus and published case series.

For the above reasons, the development of a national commissioning policy requires a degree of pragmatism. Previous attempts to develop evidence-based policies have highlighted the paucity of good quality evidence from randomised controlled trials, and the small size and poor quality of the studies upon which current clinical guidelines are based.

This policy document sets out the clinical indications for which autologous and allogeneic transplants will be commissioned routinely by NHS England for adults and children respectively. For a more detailed description of the transplantation services which will be commissioned and the service standards which should be met by transplant centres please refer to the BMT Service Specification.

2. Definitions

Allogeneic haematopoietic stem cell transplantation (HSCT) is used to treat carefully selected patients with a range of malignant and non-malignant haematological disorders and other specific disorders of the immune system. It involves replacing the bone marrow stem cells of a patient following high-dose therapy, with stem cells from a tissue-type matched or mismatched donor.

Patients require detailed pre-transplant assessment and investigations to assess their clinical status and fitness to proceed to transplant. The transplant procedure begins with 'conditioning' therapy (chemotherapy with or without total body irradiation [TBI]) at a range of doses depending on the type and severity of disease being treated. The aim of conditioning is to:

- Kill leukaemia/tumour cells (in malignant diseases)
- Eradicate existing bone marrow tissue (in order to provide space for engraftment of transplanted donor stem cells)

- Suppress the patient's immune system, so as to minimise the risk of graft rejection

Bone marrow, peripheral blood stem cells, or umbilical cord blood stem cells may be used as donor stem cell sources. Use of umbilical cord cells must be in line with the UK Cord Blood Working Group Recommendations for donor selection. Use of double cord units must be notified in advance to the commissioner in view of the likely increased costs and to ensure the selection protocol has been followed.

Autologous transplantation uses the patient's own stem cells, which are harvested prior to high-dose therapy. It is performed as part of dose escalation therapy, mainly in patients with lymphoma and myeloma, although it is also used in certain autoimmune and oncology cases. It enables the patient to be treated with doses of chemotherapy which are higher than would be possible without subsequent replacement of the harvested cells, because the therapy destroys the patient's remaining stem cell tissue.

3. Aim and objectives

This policy aims to:

Specify the clinical indications and their subgroups for which autologous and allogeneic haematopoietic stem cell transplants will be commissioned routinely by NHS England.

The objectives are to:

- Optimise patient outcome after autologous and allogeneic stem cell transplantation
- Reduce variation in access to BMT
- Ensure that BMT is commissioned for those conditions for which there is acceptable evidence of clinical benefit and cost-effectiveness
- Promote the cost-effective use of resources
- Reduce unacceptable variation in clinical practice
- Ensure that experimental treatments are offered only in the context of properly conducted research.

4. Epidemiology and needs assessment

The data below are taken from the BSBMT (British Society of Blood and Marrow Transplantation) registry for stem cell transplant procedures undertaken by BMT centres in the UK and Republic of Ireland. The figures include repeat transplants (including donor lymphocyte infusions) in patients who have previously been transplanted. There are considerable year to year fluctuations in numbers, but an underlying increasing trend. The average annual increase in transplant numbers over this period is 5% per year.

Table 1: Number of transplants by transplant type 2006-2012 inclusive

Year	Allografts	Autografts	Total	% Increase
2006	1144	1563	2706	
2007	1196	1569	2765	2.2
2008	1263	1676	2939	6.3
2009	-	-	-	-
2010	1321	1919	3240	10.2
2011	1440	1917	3357	3.6
2012	1695	2275	3616	7.7

Source: BSBMT Register

Table 2 below shows a breakdown of first transplants by clinical indication for 2012. Myelomas and lymphomas remain the most common indications for autologous transplantation. Most allogeneic transplants are for acute leukaemias, followed by the lymphomas.

Table 2: Number of first transplants by disease category and transplant type 2012

Indication	Allograft	Autograft	Total
Plasma Cell Disease	42	1090	1132
Lymphoma	211	699	910
Acute Leukaemia	611	3	614
MDS/MPS	208	0	208
Solid Tumour	4	149	153
Chronic Leukaemia	107	0	107
Primary Immune Deficiency	38	2	40
Bone Marrow Failure	91	0	91
Haemoglobinopathy	23	0	23
Inherited Disorders of Metabolism	16	0	16
Auto Immune Diseases	2	7	9
Other	8	7	15
Total	1361	1957	3318

Source: BSBMT Register 2012

5. Evidence base

Due to the large number of possible indications for stem cell transplantation, the degree of variation in clinically important patient and disease parameters, and the diversity of conditioning and transplant regimes, it is extremely difficult to evaluate the clinical effectiveness of transplantation for every potential clinical condition.

The BSBMT and UK Paediatric BMT Group HSCT recommendations are well referenced, although the quality of the evidence is generally poor, being based largely on case series and clinical consensus. There is little published evidence as to the cost-effectiveness of BMT.

6. Rationale behind the policy statement

Stem cell transplantation, particularly allogeneic transplantation, is a complex procedure with considerable opportunity costs. This policy will promote equity of access to treatment in England. It confirms the indications for which the NHS in England will fund transplantation routinely, and the process for requesting funding for conditions outside this policy.

7. Criteria for commissioning

Adults

Adult BMT is commissioned according to the British Society of Blood and Bone Marrow Transplantation (BSBMT) table of indications published in **February 2012**. BSBMT (February 2012) recommendations divide indications for adult BMT into four categories:

S = standard of care

CO = clinical option, can be considered after assessment of risks and benefits

D = developmental, further trials are needed

GNR = generally not recommended

- For the purposes of this commissioning policy **first** transplants for indications within categories S and CO (standard of care, and clinical option respectively) are accepted as established clinical practice, and will be commissioned routinely, without need for Individual Funding Request (IFR). Repeat transplants for failure to engraft will also be commissioned routinely. However, **repeat** autologous or allogeneic transplants for relapsed disease will not be commissioned routinely unless explicitly recommended by the BSBMT guidelines (e.g. second autologous transplant for myeloma and POEMS).
- Use of umbilical cord cells must be in line with the UK Cord Working Group Recommendations for donor selection. Use of double cord must be notified in advance to the commissioner to demonstrate the donor selection protocol has been followed.

Exclusions

- Repeat autologous or allogeneic transplants for relapsed disease unless explicitly recommended by the BSBMT guidelines (e.g. second autologous transplant for myeloma and POEMS). IFR approval will otherwise need to be sought where cases meet the criteria for exceptionality.
- Planned tandem transplants unless explicitly recommended by the BSBMT guidelines. IFR approval will otherwise need to be sought where cases meet the criteria for exceptionality.
- Transplants for indications within categories D and GNR will **not** be commissioned routinely, and IFR approval will need to be sought for transplantation of all cases falling within these categories where cases meet the criteria for exceptionality.
- BMT is not commissioned for any indication which is not listed within the

BSBMT (February 2012) table of indications for indications listed in BSBMT indications tables published after February 2012 unless they are specifically confirmed in this policy. IFR approval will therefore need to be sought for transplantation for all indications not specifically listed in the appendix of this policy document.

Children

Paediatric BMT is commissioned according to the BSBMT Paediatric BMT Group HSCT table of indications published in **December 2011**. The Paediatric BMT Group HSCT recommendations (December 2011) divide indications for BMT into four categories:

S = standard of care

CO = clinical option, can be considered after assessment of risks and benefits

D = developmental, further trials are needed

GNR = generally not recommended

- For the purposes of this commissioning policy **first** transplants for indications within categories S and CO (standard of care, and clinical option respectively) are accepted as established clinical practice, and will be commissioned routinely, without need for Individual Funding Request (IFR). Repeat transplants for failure to engraft will also be commissioned routinely. However, **repeat** autologous or allogeneic transplants for relapsed disease will not be commissioned routinely unless explicitly recommended by the UK Paediatric BMT Group HSCT recommendations. IFR approval will otherwise need to be sought.

Exclusions

- Repeat autologous or allogeneic transplants for relapsed disease will not be commissioned routinely unless explicitly recommended by the UK Paediatric BMT Group HSCT recommendations.
- Planned tandem transplants unless explicitly recommended by the BSBMT guidelines. IFR approval will otherwise need to be sought where cases meet the criteria for exceptionality.
- Transplants for indications within categories D and GNR will **not** be commissioned routinely, and IFR approval will need to be sought for transplantation of all cases falling within these categories where cases meet the criteria for exceptionality.
- BMT is not commissioned for any indication which is not listed within the UK Paediatric BMT Group HSCT recommendations (December 2011) or for indications listed in BSBMT indications tables published after December 2011 unless they are specifically confirmed in this policy. IFR approval will therefore need to be sought for transplantation for all indications not specifically listed in the appendix of this policy document.

Policy development

Clinical practice continues to evolve, and the commissioning policy will continue to be reviewed regularly and updated to reflect current evidence.

In the interim, individual funding requests for transplantation in cases which do not meet the policy criteria will be considered on an individual basis by commissioners.

8. Patient pathway

The patient pathway is described in detail in the BMT service specifications for adults and children respectively.

It is the intention of NHS England to achieve convergence on commissioning pathway including currencies and pricing, over time. The specification sets out an approach to defining the BMT pathway as commencing from decision to transplant (-30 days) and ends 100 days following the transplantation procedure. This pathway does not preclude shared-care arrangements for post-transplant follow-up between the transplant centre and local haemato-oncology providers, where this has been agreed between providers. Beyond 100 days, commissioning responsibility will automatically return to the patient's Clinical Commissioning Group.

9. Governance arrangements

The governance arrangements are described in detail in the BMT service specifications for adults and children respectively. All providers of HSCT must have JACIE accreditation.

10. Mechanism for funding

Funding for stem cell transplantation is through the ten Area Teams responsible for specialised commissioning. The funding arrangements are described in detail in the BMT service specifications for adults and children respectively.

Individual funding requests

The IFR process is for cases in which the patient may have exceptional ability to benefit from the proposed treatment, because of their particular clinical circumstances. ([NHSE policy on individual funding requests](#))

IFR requests relating to specialised services commissioned by NHS England are managed by four regional IFR teams. Requests should be submitted electronically using the generic request form ([application form for IFRs](#)), so that the IFR team has all the information it needs in order to consider the request. It is helpful if the requesting clinician indicates the degree of clinical urgency when submitting a request.

HSCT is a highly complex clinical area, and consideration of IFR requests may sometimes require specialist clinical knowledge. If the IFR panel is unable to form a view as to whether a case meets the criteria for funding under the NHSE IFR policy, it may seek expert clinical advice as to the rarity of the clinical circumstances, and the clinical appropriateness of the proposed treatment. If the IFR is for a transplant procedure the BSBMT Adjudication Panel may be consulted.

Research

It is recognised that involvement in clinical trials is an integral part of high quality service provision in stem cell transplantation. Provided that there are no excess treatment costs to commissioners, treatment provided as part of NCRI- approved trials will be commissioned routinely for patients who meet the commissioning policy criteria,. (For example, trials comparing different conditioning regimens will be supported, provided there is no significant cost differential between the treatment arms.)

However, research will not be funded with resources diverted from the provision of routine transplant services. Transplantation undertaken as part of research into the treatment of conditions not covered by the commissioning policy will not be commissioned routinely, irrespective of whether or not the trial has NCRI approval. Similarly, any excess treatment costs relating to trial participation will not be met by the NHS CB unless prior commissioner approval has been obtained.

11. Audit requirements

Complete data must be submitted to the BSBMT registry for all transplants carried out by UK centres. This will enable better evaluation of clinical outcomes broken down by patient and disease-related variables. All centres must undergo regular JACIE inspection. All centres must provide the data required for the BMT Quality Dashboard. Audit requirements are described in more detail in the BMT service specification.

12. Documents which have informed this policy

- British Society for Blood and Marrow Transplants. BSBMT Indications for Adult BMT (February 2012). Available from:
<http://bsbmt.org/wp-content/uploads/2013/12/Indications-Table-Updated-Feb-2012-PDF-Version.pdf>
- British Society for Blood and Marrow Transplants. UK Paediatric BMT Group HSCT recommendations (December 2011). Available from:
http://bsbmt.org/wp-content/uploads/2013/12/UK_Paed_BMT_Gp_HSCT_Indications_23Dec111.pdf
- NHS Blood and Transplant. The Future of Unrelated Donor Stem Cell Transplantation in the UK. Part 1: Findings and Recommendations. A Report from the UK Stem Cell Strategic Forum, July 2010.
http://www.nhsbt.nhs.uk/download/uk_stem_cell_strategic_forum_report.pdf
- Recommendations for a standard UK approach to incorporating umbilical cord blood into clinical transplantation practice: conditioning protocols and donor selection algorithms. Bone Marrow Transplantation (2009) 44, 7–12
<http://www.nature.com/bmt/journal/v44/n1/pdf/bmt2008420a.pdf>

Accessed 14/05/14

13. Links to other policies

B04/P/2 Clinical Commissioning Policy: Use of Plerixafor for Stem Cell Mobilisation
This policy follows the principles set out in the ethical framework that govern the commissioning of NHS healthcare and those policies dealing with the approach to experimental treatments and processes for the management of individual funding requests (IFR).

14. Date of review

This policy will be reviewed in April 2016 unless information is received which indicates that the proposed review date should be brought forward or delayed.

References

1. British Society for Blood and Marrow Transplants. BSBMT Indications for Adult BMT (February 2012). Available from:
<http://bsbmt.org/wp-content/uploads/2013/12/Indications-Table-Updated-Feb-2012-PDF-Version.pdf>
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3. NHS Blood and Transplant. The Future of Unrelated Donor Stem Cell Transplantation in the UK. Part 1: Findings and Recommendations. A Report from the UK Stem Cell Strategic Forum, July 2010.
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<http://www.nature.com/bmt/journal/v44/n1/pdf/bmt2008420a.pdf>