Clinical Commissioning Policy: Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised Reference: NHS England B04/P/a

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

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Policy Statement

NHS England will commission Haematopoietic stem cell transplantation (HSCT) 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.

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 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 preparative conditioning therapy, with stem cells from a tissue-type matched or mismatched donor.

Autologous HSCT 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 a 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 HSCT Service Specification.

2. Definitions

Allogeneic 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 preparative conditioning, 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 current clinical best practice. 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 HSCT 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 solid tumour cases. This would also include in the future transplantation of gene modified autologous stem cells for certain non-malignant cases such as Haemoglobinopathies. 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 HSCT will be commissioned routinely by NHS England.
The objectives are to:

- Optimise patient outcome after autologous and allogeneic HSCT.
- Reduce variation in access to HSCT. Ensure that HSCT 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 BSBMTCT (British Society of Blood and Marrow Transplantation and Cellular Therapy) registry for stem cell transplant procedures undertaken by HSCT 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 2006-2019 inclusive

Year	Allografts	Autografts	Total	% Increase per previous year
2006	1144	1563	2707	
2007	1196	1569	2765	2.10%
2008	1263	1676	2939	5.92%
2009	1301	1771	3072	4.33%
2010	1321	1919	3240	5.19%
2011	1440	1917	3357	3.49%
2012	1453	2163	3616	7.16%
2013	1615	2225	3840	5.83%
2014	1678	2344	4022	4.53%
2015	1610	2503	4113	2.21%
2016	1680	2717	4397	6.46%
2017	1684	2805	4489	2.05%
2018	1675	2811	4486	-0.07%
2019	1726	2854	4580	2.05%
Source: BSBMTCT Registry 2021				

Table 2: Number of patients by indication for Allograft and Autograft procedures (BSBMTCT, 2021)

Indication	Allograft	Autograft	Total
Acute Leukaemia	831	3	834
Chronic Leukaemia	72	2	74
Lymphomas	188	782	970
MDS/MPN	327	2	329
Plasma Cell Disorders inc MM	24	1772	1796
Bone Marrow Failures	106	0	106
Haemoglobinopathy	29	0	29
Solid Tumours	0	202	202
Primary Immune Deficiencies	78	0	78
Inherited Disorders of Metabolism	16	0	16
Other Inherited Disorders	4	0	4
Histiocytic Disorders	17	0	17
Autoimmune Disorders	6	74	80
Other	1	3	4
Total	1699	2840	4539
Source: BSBMTCT Registry 2021			

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 BSBMTCT and UK Paediatric BMT Group HSCT recommendations are well referenced, although the quality of the evidence is generally lacking in large randomised trials, being based largely on case series and clinical consensus. There is little published evidence as to the cost-effectiveness of HSCT.

6. Rationale behind the policy statement

HSCT particularly allogeneic HSCT is a complex procedure with considerable treatment 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.

HSCT indications tables will be reviewed on a bi-annual basis by the HSCT indications subcommittee of the BSBMTCT. The new tables will be submitted to NHS England for review, with a summary of changes and the impact on the overall impact on transplant activity. Any significant material increase in transplant activity would need to be approved in writing by NHS England to BSBMTCT.

7. Criteria for commissioning

Adults and Paediatrics

Adult and Paediatric HSCT are commissioned according to the currently active BSBMTCT table of indications as published on the BSBMTCT website. BSBMTCT recommendations divide indications for adult and Paediatric HSCT into four categories:

1. S = standard of care
2. CO = clinical option, can be considered after assessment of risks and benefits
3. D = developmental, further trials are needed
4. 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 BSBMTCT guidelines (e.g. second autologous transplant for myeloma and POEMS, second allogeneic transplant for relapsed leukaemia if relapse >1yr post first transplant).
- Use of umbilical cord cells must be in line with current clinical best practice. 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 HSCT for relapsed disease unless explicitly recommended by the BSBMTCT guidelines (e.g. second autologous transplant for myeloma and POEMS second allogeneic:

- transplant for relapsed leukaemia if relapse >1yr post first transplant). IFR approval will otherwise need to be sought where cases meet the criteria for exceptionality.
- Planned tandem transplants unless explicitly recommended by the BSBMTCT 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 casesmeet the criteria for exceptionality.
- HSCT is not commissioned for any indication which is not listed within the BSBMTCT currently active table of indications for indications listed in BSBMTCT indications tables published after unless they are specifically confirmed in this policy.

IFR approval will therefore need to be sought for transplantation not included with the current BSBMTCT guidelines.

Children – transplants will be performed with age appropriate indications

Paediatric HSCT services are commissioned adopting the BSBMTCT Paediatric BMT Group HSCT table of indications. The governance of this group sits within the remit of BSBMTCT indications subcommittee. Any changes to the indications must be assessed by the National Paediatric BMT group. This process will not circumvent the NHS England effectiveness policy process, any material changes will be required to follow these processes.

The Paediatric BMT Group HSCT recommendations divide indications for BMT into four categories:

1. S = standard of care
2. CO = clinical option, can be considered after assessment of risks and benefits
3. D = developmental, further trials are needed
4. 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. Second transplants for relapsed disease are commissioned for auto's and allo's under specific circumstances as described in the BSBMTCT indications. IFR approval will otherwise need to be sought for any other additional transplants.

Exclusions

- Repeated transplants above those already routinely commissioned and will require consideration through IFR panel approval.
- Planned tandem transplants unless explicitly recommended by the BSBMTCT 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 casesmeet the criteria for exceptionality.
- HSCT is not commissioned for any indication which is not listed within the current BSBMTCT guidelines 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 BSBMTCT guidelines.

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

8. Patient pathway

The patient pathway is described in detail in the HSCT 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 HSCT 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 lead commissioner.

9. Governance arrangements

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

10. Mechanism for funding

Funding for HSCT is through the lead commissioner for specialised commissioning. The funding arrangements are described in detail in the HSCT 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 NHSEI IFR policy, who may seek expert clinical advice as to the rarity of the clinical circumstances, and the clinical appropriateness of the proposed treatment.

11. 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 England unless prior commissioner approval has been obtained.

12. Audit requirements

Complete data must be submitted to the BSBMTCT 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 HSCT service specification, with specific attention to transplant outcome.

13. Documents which have informed this policy

https://bsbmtct.org/publications/

14. Links to other policies

NHS commissioning » F01. Blood and Marrow Transplantation (england.nhs.uk)

15. Date of review

The policy will be reviewed within 12 months of new HSCT indications guidelines being issued by the subcommittee of the BSBMTCT bi-annually. Any material changes will be subject to NHS England commissioning processes.

Appendix

Description of changes

This policy has been updated to bring it up to date and to reflect changes made in the BSBMTCT Adult and Paediatric clinical indications, which the policy is aligned to. NHSEI and BSBMTCT have worked collaboratively to update and standardise the Policy, the Adult and Paediatric indications. The changes are described below.

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
haematopoietic stem cell transplantation	(HSCT)	Policy statement, Para 1, pg 5	Added abbreviation	August 2021
The policy document outlines the arrangements for funding of this treatment for the population of England	Removed	Policy statement, para 3. Page 5	Not applicable	August 2021
Haematopoietic stem cell transplantation	HSCT	Throughout the document	Abbreviation in	August 2021
high dose	Preparative conditioning	Plain language summary, para 2, pg 5 Definitions, para 1, pg 6	Update of wording	August 2021
BMT	HSCT	Introduction, para 5, pg 6	Update of wording to reflect documentation	August 2021
UK Cord Blood Working Group Recommendations for donor selection	Current clinical best practice	Definitions, para 3, pg 7	Updated wording to reflect practice	August 2021
Oncology	Solid tumours. Also added to the para: This would also include in the future transplantation of gene modified autologous stem cells for certain non-malignant cases such as Haemoglobinopathies	Autologous, pg 7	Updated wording to reflect practice	August 2021
Stem cell transplant	HSCT	Aims and objectives, bullet 2, pg 7	Update of wording	August 2021
BSBMT (British Society of Blood and Marrow Transplant)	BSBMTCT (British Society of Blood and Marrow Transplant and Cellular Therapy)	Throughout the document	Updated to reflect the change / updating of name	August 2021
Table 1 - 2012-table updated	2019 – table updated	Table 1, pg 9	Update of table	August 2021
Table 2 – update of table	Table 2 updated to reflect 2021 data	Table 2, pg 9	Update of table	August 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
Poor	Lacking in large randomised trials	Evidence base, para 2, pg 10	Update of wording	August 2021
Updated wording: 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.	Second transplants for relapsed disease are commissioned for auto's and allo's under specific circumstances as described in the BSBMTCT guidelines. IFR approval will otherwise need to be sought for any other additional transplants	Bullet 1,pg 10	Update of wording – the service is already commissioned, would not impact on existing commissioning – confirmed with Kim Orchard (31 March 2022)	August 2021
Additional paragraph added	HSCT indications tables will be reviewed on a bi-annual basis by the HSCT indications subcommittee of the BSBMTCT. The new tables will be submitted to NHS England for review, with a summary of changes and the impact on the overall impact on transplant activity. Any significant material increase in transplant activity would need to be approved in writing by NHS England to BSBMTCT.	Rationale behind the policy statement, para 2, pg 11	Additional paragraph added in to the section	August 2021
Additional wording	and paediatrics	Commissioning criteria, Added to title, pg 11	Updated title to reflect both adults and paediatrics	August 2021
Amendments and additional wording added to para 1	Adult and Paediatric HSCT are commissioned according to the currently active BSBMTCT table of indications as published on the BSBMTCT website. BSBMTCT recommendations divide indications for adult and Paediatric HSCT into four categories:	Commissioning criteria, para 1, pg 11	Updating of wording to reflect paediatrics, and current BSBMTCT recommendations	August 2021
Additional wording to: (e.g. second autologous transplant for myeloma and POEMS	second allogeneic transplant for relapsed leukaemia if relapse >1yr post first transplant).	Bullet 1, pg 11	Update of wording	August 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
The UK Cord Working Group Recommendations for donor selection	Wording removed – added in current clinical best practice	Bullet 2, pg 11	Removal and amendment to wording	August 2021
Additional wording added to the sentence: e.g. second autologous transplant for myeloma and POEMS	second allogeneic transplant for relapsed leukaemia if relapse >1yr post first transplant	Exclusions, bullet 1, pg 11	Update of wording	August 2021
February 2012 removed	Currently active	Exclusions, bullet 4, pg 11	Wording removed	August 2021
For all indications not specifically listed in the appendix in the policy document	not included with the current BSBMTCT guidelines	Exclusions final para, pg 13	Update of wording	August 2021
Heading: Children	transplants will be performed with age appropriate indications.	Children, pg 13	Update of wording	August 2021
Published in December 2011	The governance of this group sits within the remit of BSBMTCT indications subcommittee. Any changes to the indications must be assessed by the National Paediatric BMT group. This process will not circumvent the NHS England and clinical effectiveness policy process, any material changes will be required to follow these processes.	Children, para 1, pg 13	Update of wording	August 2021
However, **repeat** autologous or allogenic transplants for relapsed disease will not be commissioned routinely unless explicitly recommended by the UK Paediatric BMT Group HSCT recommendations.	Second transplants for relapsed disease are commissioned for auto's and allo's under specific circumstances as described in the BSBMTCT indications	Children para 1, pg 13	Removal and amendment to wording	August 2021
Added into text	IFR approval will otherwise need to be *sought for any other additional transplants.*	Children para 1, pg 13	Update of wording	August 2021
Autologous or allogenic	Repeated transplants above those already routinely	Exclusions Bullet one, pg 13	Update of wording	August 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
transplants for relapsed disease will not be commissioned routinely unless explicitly recommended by UK Paediatric BMT Group HSCT recommendations	commissioned will require consideration through IFR panel approval			
UK Paediatric BMT Group HSCT recommendations (December 2011) or for indications listed in BSBMT indications tables published after December 2011. Appendix of this policy document.	HSCT is not commissioned for any indication which is not listed within the current BSBMTCT guidelines 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 BSBMTCT guidelines.	Exclusions Bullet 4, pg 14	Update of wording	August 2021
Policy development Clinical practice continues to evolve, and the commissioning policy will continue to be reviewed regularly and updated to reflect current evidence.	Section removed	Pg 14	Removal of wording	August 2021
Clinical Commissioning Group	Lead commissioner	Patient Pathway, para 3, pg 15	Update of wording	August 2021
Insertion of wording	active or in the process of renewal of	Governance arrangements, pg 15	Update of wording	August 2021
Ten Area Teams responsible	Lead commissioner	Mechanism for funding, para 1, pg 15	Update of wording	August 2021
It	who	Individual funding requests, para 3, pg 15	Update of wording	August 2021
If the IFR is for a transplant procedure the BMSMT Adjudication Panel may be consulted.	Wording removed	Individual funding requests, para 3, pg 15	Removal of wording	August 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
CB	England	Research Para 2, pg 17	Update of wording	August 2021
Insertion of wording	with specific attention to transplant outcome.	Audit requirements Pg 17	Update of wording	August 2021
Removal of hyperlinks	https://bsbmtct.org/publications/	Documents which have informed this policy. Pg 17	Removal of out of date hyperlinks – new hyperlink inserted	August 2021
Removal of hyperlink	NHS commissioning » F01. Blood and Marrow Transplantation (england.nhs.uk)	Links to other policies Pg 18	Removal of out of date hyperlinks – new hyperlink inserted	August 2021
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.	The policy will be reviewed within 12 months of new HSCT indications guidelines being issued by the subcommittee of the BSBMTCT bi-annually. Any material changes will be subject to NHS England commissioning processes.	Date of review Pg 18	Update of wording	August 2021
Removal of T NHL, Lymphoblastic (non Burkitt) BNHL	2 lines merged and called Lymphoblastic lymphoma, which can be of B or T cell origin and is treated in the same way	Under stem cell source	Update of wording	July 2021
S	CO	Lymphoblastic lymphoma	Amendment	July 2021
CO	GNR	ALCL	Amendment	July 2021
Burkitt NHL and other non-lymphoblastic NHL	High grade Mature B-NHL	Burkitt NHL	Same disease – updated to current name	July 2021
CO	S	High grade Mature B-NHL	Amendment	July 2021
Insertion	Table updated to include current classification system. No change in current activity	Additional wording added in under 'Notes'	Additional wording	July 2021
Gene therapy may be considered in appropriate clinical trials	ADA SCID - S Gene therapy for other PID may be considered in appropriate clinical trials	Primary Immunodeficiencies	Removal and insertion of wording	July 2021
Insertion	NHS England Primary Immunodeficiencies funding has been approved for ADA SCID gene therapy	Primary Immunodeficiencies	Insertion of wording under 'Notes'	July 2021
Insertion	Table 3 updated to reflect current nomenclature and defines those disorders which should no longer be treated with transplant. May slightly reduce number of transplant but as numbers very	Inborn errors of metabolism	Insertion of wording under 'Notes'	July 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
	small with probably not affect overall numbers.			
Benign	Non-Malignant	Haematology	Removal and insertion of wording	July 2021
N/A	Gene therapy may be considered in appropriate clinical trials	Thalassemia	Removal and insertion of wording	July 2021
CO	S	Thalassemia	Amendment	July 2021
N/A	Gene therapy may be considered in appropriate clinical trials	Sickle Cell	Removal and insertion of wording	July 2021
Insertion	No change in clinical activity. Clinical trial availability for red cell disorders highlighted - these are funded separately and therefore included here only for clinical completeness	Haematology	Insertion of wording under 'Notes'	July 2021
CO	S	Acquired aplastic anaemia	Amendment	July 2021
GNR	CO	Diamond-Blackfan anaemia	Amendment	July 2021
GNR	CO	Autoimmune disease (includes refractory immune cytopaenias, Juvenile Inflammatory Arthritis, SLE, Systemic Sclerosis, other)	Amendment	July 2021
Insertion	Change from GNR to CO for haplo for autoimmune disease unlikely to have any impact on current activity. SLE and SS primarily diseases of adulthood. Other indications already transplanted and sibling or unrelated donor identified for the majority of patients.	Autoimmune disease (includes refractory immune cytopaenias, Juvenile Inflammatory Arthritis, SLE, Systemic Sclerosis, other)	Insertion of wording under 'Notes'	July 2021
Insertion -	or if intolerant of hydroxycarbamide	Indications table – 'u'	The changes are for clarification only and will not impact on activity	July 2021
Insertion in to 'u' section	despite transfusions/exchange	Indications table – 'u'	Insertion of wording	July 2021
Insertion in to 'u' section	12 months of	Indications table – 'u'	Insertion of wording	July 2021

Describe what was stated in original document	Describe new text in the document	Section/Paragraph to which changes apply	Describe why document change required	Date change made
Table 3 removed	Table 3 – updated version	Table 3 – indications for HSCT in Inborn errors of metabolism	Removal and updated version inserted	July 2021
S	CO	Acquired aplastic anaemia	This change relates to stem cell source rather than transplant indication.	December 2021
GNR	CO	Diamond Blackfan anaemia	Change to option of haplo for Diamond Blackfan anaemia – exceptionally rare disease and other donors generally available. This change relates to stem cell source rather than transplant indication. It will not make any material difference from a commissioning perspective as it is an extremely rare disease and we usually find a matched donor ie this would equate to 0-1 transplant per year.	December 2021
GNR	CO	Autoimmune disease (includes refractory immune cytopaenias, Juvenile Inflammatory Arthritis, SLE, Systemic Sclerosis, other)	This change relates to stem cell source rather than transplant indication.	December 2021