



# **Clinical commissioning policy: Haematopoietic Stem Cell Transplantation (HSCT) Teenage and young adults (TYA)**

**Reference: NHS England F01X01/01**

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# **Clinical Commissioning Policy Proposition: Haematopoietic Stem Cell Transplantation (HSCT) Teenage and young adults (TYA)**

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

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## Equality Statement

NHS England has a duty to have regard to the need to reduce health inequalities in access to health services and health outcomes achieved as enshrined in the Health and Social Care Act 2012. NHS England is committed to fulfilling this duty as to equality of access and to avoiding unlawful discrimination on the grounds of age, gender, disability (including learning disability), gender reassignment, marriage and civil partnership, pregnancy and maternity, race, religion or belief, gender or sexual orientation. In carrying out its functions, NHS England will have due regard to the different needs of protected equality groups, in line with the Equality Act 2010. This document is compliant with the NHS Constitution and the Human Rights Act 1998. This applies to all activities for which NHS England is responsible, including policy development, review and implementation.

## Plain Language Summary

Haematopoietic stem cell transplantation (HSCT) 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 haematopoietic stem cell transplantation 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.

Patients aged 16-24 are defined as the teenage and young adult (TYA) patient group. This policy aims to address four childhood diseases that extend into adulthood: immunodeficiencies, inborn errors, haemoglobinopathies and solid tumours, and specify the clinical indications and their subgroups for which the paediatric commissioning criteria will be extended for autologous and allogeneic haematopoietic stem cell transplants for this specific patient group to reflect the underlying disease process and its age-related incidence or prevalence.

NHS England has concluded to support a proposal to routinely commission haematopoietic stem cell transplantation for the teenage and young adult age group for four indications: immunodeficiencies, inborn errors, haemoglobinopathies and solid tumours.

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## 1. Introduction

This document describes the proposal of NHS England to extend existing paediatric commissioning criteria for haematopoietic stem cell transplantation to the teenage and young adult (TYA) (16-24 year olds) patient group for four childhood diseases: immunodeficiencies, inborn errors, haemoglobinopathies and solid tumours.

This is based on agreed criteria to identify suitable indications. These criteria included a set of principles that the indications are childhood diseases that extend into adulthood, and that there is biological plausibility that this is the same disease mechanism that may present later than 16 years of age in some patients.

Four childhood diseases meet these criteria and have different commissioning criteria for adults and children:

- Immunodeficiencies
- Inborn errors
- Haemoglobinopathies
- Solid tumours

Available evidence was considered together with clinical opinion. Based on this, it is proposed that the paediatric commissioning criteria for the four disease areas that have applicability here be extended up to 24 years to include the teenage and young adult age group.

This document also describes the proposed criteria for commissioning, proposed governance arrangements and proposed funding mechanisms.

For the purpose of consultation NHS England invites views on the information that has been taken into account as described in this policy proposition.

A final decision as to whether the proposed additional indications for haematopoietic stem cell transplantation will be routinely commissioned for the teenage and young adult (TYA) patient group is planned to be made by NHS England by June 2016 following a recommendation from the Clinical Priorities Advisory Group.

## 2. The proposed intervention and clinical indication

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 haematopoietic stem cell transplantation (AlloSCT), 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

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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.

This policy document sets out the clinical indications for which autologous and allogeneic transplants will be commissioned routinely by NHS England using paediatric criteria for the teenage and young adult population (16-24 year olds).

The proposed indications have specific features that mean they are paediatric diseases that may extend into early adulthood. Immunodeficiencies, inborn errors and haemoglobinopathies are all genetic diseases that usually present in small children and occasionally in teenagers.

Paediatric solid tumours for which autografts are indicated do not occur regularly in adults.

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.

Second allogeneic transplants for relapsed disease are excluded from this policy. The commissioning of second transplants is defined in the commissioning policy proposition for second haematopoietic stem cell transplant (F01X07).

### 3. 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

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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.

Teenage and young adult (TYA) patients are aged 16-24 years and have a much poorer survival for specific cancers than children, poorer recruitment to clinical trials than children, poorer experience of cancer care than adults, and distinct psychosocial needs compared to adults or younger children (Improving Outcomes Guidance for Children and Young People with Cancer, NICE 2005). Care of these patients often falls between paediatric and adult services; there is a need for change to address this variability.

This can create a challenge for the TYA patients aged 16-24, as either paediatric or adult recommendations may be clinically appropriate in specific cases. This risks creating a situation during transition where a patient turning 18 is no longer eligible for an otherwise appropriate treatment.

### 4. Aim and objectives

This policy aims to:

Address childhood diseases that extend into adulthood and specify the clinical indications and their subgroups for which autologous and allogeneic haematopoietic stem cell transplants will be commissioned routinely by NHS England for this specific patient group to reflect the underlying disease process and its age-related incidence or prevalence. This is to ensure that patients who present with childhood diseases above the age of 16 will receive the appropriate treatment.

Where these diseases occur, most children where it is indicated, will have been treated. This policy ensure that 16-24 are not disadvantaged by being late in being identified as having a childhood disease.

The objectives are to:



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- 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.

### 5. Epidemiology and needs assessment

For immunodeficiencies the prevalence is unknown (PID UK, 2015). A high level estimate for prevalence of primary immunodeficiencies (PID) in the overall population of England is in the region of c. 4,200 (PID UK, 2015; ONS, 2015). Specific estimates for the TYA population could not be obtained as part of the review. If prevalence of PID is distributed homogeneously across age groups, this would imply a prevalence for TYA patients of c. 500 (ONS, 2015). Of these, only a small proportion require transplant. Since 2010, 22 patients aged 16-24 were transplanted for immunodeficiency (BSBMT).

Prevalence of inborn errors is difficult to quantify as there are over 200 different types of inborn errors of metabolism (Grey et al. 2000). For reference, inherited metabolic disorders have a reported birth prevalence of 1 in 784 live births (PHG Foundation). Ignoring mortality, this would translate to a prevalence of c. 8,700 among TYA patients. Since 2010, there have been no transplants for patients aged 16-24 for inborn errors (BSBMT).

The prevalence of haemoglobinopathies in TYAs in England is estimated in the region of 1,200 (National Haemoglobinopathy Registry Report 2014/14; ONS, 2015). Of these, only a small proportion require transplant. Since 2010, 18 patients aged 16-24 were transplanted for haemoglobinopathies (BSBMT).

Finally, the 20 year prevalence of solid tumours in TYA patients is estimated at 13,000 in England in 2014/15 (MacMillan, 2013; ONS, 2015). Of these, only a small proportion require transplant. Since 2010, 87 patients aged 16-24 were transplanted for solid tumours (BSBMT).

This leads to an indicative, high level prevalence for the TYA indications considered in the region of c. 23,000 or a prevalence rate of c. 3 in 1,000 of the TYA population. As more detailed prevalence rates for the specific indications listed above could not be identified in most cases, these figures are illustrative only and likely over-estimate true prevalence. Since 2010, there have been a total of 127 transplants for patients aged 16-24 for these indications.

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## 6. Proposed criteria for commissioning

Haematopoietic stem cell transplantation for the TYA age group (16-24 years old) will be commissioned following the children's criteria in the clinical commissioning policy for HSCT (NHS England B04/P/a) for the indications listed below.

- Immunodeficiency; OR
- Inborn error; OR
- Haemoglobinopathy; OR
- Solid tumour

### Exclusion criteria

All other indications are excluded.

Patients under 16 will be treated according to the criteria for children defined in the clinical commissioning policy for HSCT (NHS England B04/P/a).

Patients aged 16-24 with indications other than those above will be treated according to the criteria in the clinical commissioning policy for HSCT (NHS England B04/P/a).

## 7. Proposed patient pathway

Refer to existing policy for HSCT (NHS England B04/P/a).

## 8. Proposed governance arrangements

Refer to existing policy for HSCT (NHS England B04/P/a).

## 9. Proposed mechanism for funding

Refer to existing policy for HSCT (NHS England B04/P/a).

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### 10. Proposed audit requirements

Refer to existing policy for HSCT (NHS England B04/P/a).

### 11. Documents which have informed this policy proposition

NHS England: Clinical Commissioning Policy: Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised. Reference: NHS England B04/P/a January 2015

Improving Outcomes Guidance for Children and Young People with Cancer, NICE 2005

UK Paediatric BMT Group HSCT Indications, October 2015

UK Paediatric BMT Group HSCT Indications, December 2012

UK Paediatric BMT Group HSCT Indications, December 2011

BSBMT Indications for BMT, October 2013

BSBMT Indications for BMT, February 2012

### 12. Date of review

This document will lapse upon publication by NHS England of a clinical commissioning policy for the proposed intervention that confirms whether it is routinely or non-routinely commissioned (expected by June 2016).

The policy for haematopoietic stem cell transplantation (F01X01) is subject to annual revision.