GIG CYMRU NHS WALES
Cyd-bwyllgor Comisiynu
Joint Commissioning Committee

# Haematopoietic Stem Cell Transplantation (HSCT) For Adults

## Policy Position Statement: PPS142

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# Contents

# Policy Statement

NHS Wales Joint Commissioning Committee (NWJCC) will commission Haematopoietic Stem Cell Transplantation (HSCT) for adults resident in Wales in accordance with the criteria outlined in this document.

In creating this document NWJCC has reviewed the relevant guidance issued by NHS England and has concluded that Haematopoietic Stem Cell Transplantation (HSCT) for adults should be made available.

## Welsh Language

NWJCC is committed to treating the English and Welsh languages on the basis of equality, and endeavour to ensure commissioned services meet the requirements of the legislative framework for Welsh Language, including the Welsh Language Act (1993), the Welsh Language (Wales) Measure 2011 and the Welsh Language Standards (No.7) Regulations 2018.

Where a service is provided in a private facility or in a hospital outside of Wales, the provisions of the Welsh language standards do not directly apply but in recognition of its importance to the patient experience, the referring health board should ensure that wherever possible patients have access to their preferred language.

In order to facilitate this, NWJCC is committed to working closely with providers to ensure that in the absence of a Welsh speaker, written information will be offered and people have access to either a translator or 'Language-line' if requested. Where possible, links to local teams should be maintained during the period of care.

## Decarbonisation

NWJCC is committed to taking assertive action to reducing the carbon footprint through mindful commissioning activities. Where possible and taking into account each individual patient's needs, services are provided closer to home, including via digital and virtual access, with a delivery chain for service provision and associated capital that reflects the NWJCC commitment.

## Disclaimer

NWJCC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this document.

This document may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to

the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian, or Local Authority.

NWJCC disclaims any responsibility for damages arising out of the use or non-use of this policy.

# 1. Introduction

This Policy Position Statement has been developed for the planning and delivery of Haematopoietic Stem Cell Transplantation (HSCT) for adults resident in Wales. This service will only be commissioned by the NHS Wales Joint Commissioning Committee (NWJCC) and applies to residents of all seven Health Boards in Wales.

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 relatively high mortality and morbidity risks, 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 regimens, 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 Wales for 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 NWJCC Service Specification for Haematopoietic Stem Cell Transplant (CP79).

## 1.1 Plain Language summary

Haematopoietic Stem Cell Transplantation (HSCT) is also known as blood and marrow transplantation (BMT). HSCT is a term that encompasses a variety of procedures. Its essence is the ablation and replacement of the bone marrow. It is used to treat a wide spectrum of disorders and is broadly divided into two main groups: autologous and allogeneic transplantation.

Allogeneic haematopoietic 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.

The source of the progenitor cells (stem cells) also varies. Currently stem cells obtained from peripheral blood are the most common source of cells for transplantation. Bone marrow itself or umbilical cord blood can also be used as sources for stem cells.

There are several methods by which the marrow is prepared including large doses of chemo-radiotherapy (traditional transplants) or by using primarily immunosuppressive strategies (reduced intensity or non-myeloablative transplants).

The procedure for transplantation can be divided into several phases:

- **Pre-transplant work-up**: Pre-transplant work-up includes assessment of eligibility for transplantation, tissue typing of donors if applicable and basic investigations for fitness of both donor and recipient.
- **Mobilisation**: Mobilisation involves collection of stem cells from either the donor or the patient and processing the cells in the laboratory.
- **Conditioning**: Conditioning prepares the marrow for transplantation by either myeloablation or immunosuppression.
- **Transplantation and engraftment**: The cells are transplanted in the form of an intravenous infusion and the patients are then monitored until they have engrafted (recovered enough neutrophil numbers to reduce the risk of infection).
- **Post-transplant follow-up**: Post-transplant follow-up varies according to the nature of the transplant itself, complications and late effects. Complications such as infection and graft versus host disease need to be managed by the transplant team.

## 1.2 Aims and Objectives

This Policy Position Statement aims to define the commissioning position of NWJCC on the use of Haematopoietic Stem Cell Transplantation (HSCT) for adults.

The objectives of this policy are to:

- ensure commissioning for the use of HSCT is evidence based
- ensure equitable access to HSCT
- define criteria for adults with HSCT to access treatment
- improve outcomes for people undergoing HSCT

## 1.3 Epidemiology

The data below are taken from the British Society of Blood and Marrow Transplantation and Cellular Therapy (BSBMTCT) registry for adult stem cell transplants undertaken by BMT centres in the UK and Republic of Ireland. There are considerable year to year fluctuations in numbers, but an underlying increasing trend.

**Table 1: Number of first transplants by transplant type 2006-2022**

| Year | Allografts | Autografts | Total | % Increase |
|---|---|---|---|---|
| 2006 | 1075 | 1488 | 2563 | - |
| 2007 | 1124 | 1478 | 2602 | 1.5 |
| 2008 | 1170 | 1560 | 2730 | 4.9 |
| 2009 | 1200 | 1623 | 2823 | 3.4 |
| 2010 | 1231 | 1801 | 3032 | 7.4 |
| 2011 | 1317 | 1788 | 3105 | 2.4 |
| 2012 | 1361 | 1957 | 3318 | 6.9 |
| 2013 | 1525 | 1981 | 3506 | 5.7 |
| 2014 | 1582 | 2079 | 3661 | 4.4 |
| 2015 | 1530 | 2190 | 3720 | 1.6 |
| 2016 | 1603 | 2424 | 4027 | 8.3 |
| 2017 | 1594 | 2483 | 4077 | 1.2 |
| 2018 | 1567 | 2465 | 4032 | -1.1 |
| 2019 | 1627 | 2521 | 4148 | 2.9 |
| 2020 | 1387 | 1867 | 3254 | -21.6 |
| 2021 | 1504 | 2436 | 3940 | 21.1 |
| 2022 | 1463 | 2749 | 4212 | 6.9 |

Table 2 shows a breakdown of first transplants by clinical indication for 2022. Myeloma and lymphoma remain the most common indications for autologous transplantation. Most allogeneic transplants are for acute leukaemias.

**Table 2: Number of first transplants by selected disease category and transplant type 2022**[1]

| Indication | Allograft | Autograft | Total |
|---|---|---|---|
| Plasma Cell Disease/myeloma | 16 | 1779 | 1795 |
| Lymphoma (HL and NHL) | 111 | 769 | 880 |
| Acute Leukaemia and other myeloid malignancies | 1025 | 6 | 1031 |
| Solid Tumour | 0 | 118 | 118 |
| Chronic Leukaemia | 51 | 1 | 52 |
| Primary Immune Deficiency | 70 | 0 | 70 |
| Bone Marrow Failure | 116 | 0 | 116 |
| Haemoglobinopathy | 36 | 0 | 36 |
| Auto Immune Diseases | 3 | 76 | 79 |

## 1.4 About the treatment

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)
- 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 for donor selection. Use of double cord units must be notified in

[1] https://bsbmtct.org/activity/2022

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.

## 1.5 What NHS Wales has decided

NWJCC has carefully reviewed the relevant guidance issued by NHS England. We have concluded that there is enough evidence to fund HSCT within the criteria set out in section 2.1.

# 2. Criteria for Commissioning

The NHS Wales Joint Commissioning Committee approve funding of Haematopoietic Stem Cell Transplantation (HSCT) for adults in line with the criteria identified in the policy.

## 2.1 Inclusion Criteria

Adult HSCT is commissioned according to the currently active BSBMTCT table of indications for haematological malignancy.

BSBMTCT recommendations divide indications for adult HSCT into four categories:

| Category | Abbreviation |
|---|---|
| Standard of care | S |
| Clinical option, can be considered after assessment of risks and benefits | CO |
| Developmental, further trials are needed | D |
| Generally not recommended | GNR |

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 the need for Individual Patient Funding Request (IPFR).

For non-malignant conditions, NWJCC commissions according to specific criteria under the following policies:

- CP224 Allogeneic Haematopoietic Stem Cell Transplant (HSCT) for people of all ages with Sickle Cell Disease (SCD)
- CP247 Autologous Haematopoietic Stem Cell Transplant for people aged 18 years and over with previously treated relapsing multiple sclerosis (draft)
- PP225 Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages) (draft)

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.

Patients can be entered into clinical trials by the provider where the patient's treatment will comply with this Policy Position. Where the treatment falls outside the Policy, prior approval must be sought from NWJCC before entering the patient into the trial.

NWJCC will not fund treatment for indications which are outside this Policy Position and for which prior approval has not been formally given.

## 2.2 Exclusion Criteria

Areas excluded from this commissioning policy are:

- HSCT is not commissioned for any indication which is not listed within the BSBMTCT currently active table of indications unless they are specifically confirmed in this policy.
- Children under the age of 16[2]

## 2.3 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

## 2.4 Patient Pathway (Annex i)

Referral is from secondary care consultant clinicians including haematologists, oncologists and, rarely, from other non-cancer specialists such as neurologists, immunologists and rheumatologists.

All patients with haematological malignancies should be discussed at, and referred by, their local Haematology Cancer MDT.

Referrals should be made to the HSCT centre.

Discharge information shall be sent to the relevant healthcare professionals in a timely manner following the patient's discharge. A clearly defined after care programme should be communicated to the patient and communication should be timely and continuous.

[2] Younger patients aged 16 and 17 may be referred to the adult service, who are physiologically more "young adult" than paediatric. Such patients are recommended to be treated in a Teenage and Young Adult (TYA) facility.

The transplant team must report back to the referring consultant team on the progress of the patient in a timely and continuous manner. The follow-up process may run for up to ten years depending on the type of transplant and any complications. Complications such as infection and graft versus host disease will be managed by the transplant team.

Good quality written information must be made available to patients in line with JACIE standards. This will be reviewed at a minimum every two years in line with JACIE standards.

Unless the urgent need for treatment precludes the possibility, all patients of reproductive age shall be offered a review by reproductive medicine prior to starting infertility-inducing treatment.

## 2.5 Exceptions

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If the patient wishes to be referred to a provider outside of the agreed pathway, an IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

## 2.6 Clinical Outcome and Quality Measures

The Provider must work to written quality standards and provide monitoring information to the lead commissioner.

The centre must enable the patient's, carer's and advocate's informed participation and to be able to demonstrate this. Provision should be made for patients with communication difficulties.

Detailed requirements are described in in the NWJCC Service Specification for Haematopoetic Stem Cell Transplantation (CP79).

## 2.7 Responsibilities

Health Boards and NWJCC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the treatment will be commissioned.

Referrers should:

- inform the patient that this treatment is not routinely funded outside the criteria in this policy, and
- refer via the agreed pathway.

Clinician considering treatment should:

- discuss all the alternative treatment with the patient
- advise the patient of any side effects and risks of the potential treatment
- inform the patient that treatment is not routinely funded outside of the criteria in the policy, and
- confirm that there is contractual agreement with NWJCC for the treatment.

In all other circumstances an IPFR must be submitted.

# 3. Documents which have informed this policy

This NWJCC Policy Position Statement adopts the commissioning criteria of the NHS England commissioning policy: Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised. NHS England B04/P/a July 2021.

This document should be read in conjunction with the following documents:

- **NHS Wales Joint Commissioning Committee policies and service specifications**
  - Haematopoietic Stem Cell Transplantation for Adults (CP79), Service Specification
  - CP224 Allogeneic Haematopoietic Stem Cell Transplant (HSCT) for people of all ages with Sickle Cell Disease (SCD)
  - CP247 Autologous Haematopoietic Stem Cell Transplant for people aged 18 years and over with previously treated relapsing multiple sclerosis (draft)
  - PP225 Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages) (draft)
  - CP91 Extracorporeal Photopheresis (ECP) for the Treatment of Chronic Graft versus Host Disease in Adults

- **National Institute of Health and Care Excellence (NICE) guidance**
  - Haematological Cancers: Improving Outcomes. NICE Guideline NG47, May 2016.

- **NHS England policies**
  - Haematopoietic Stem Cell Transplantation (HSCT) (All Ages): Revised. NHS England B04/P/a July 2021.
  - 2013/2014 NHS Standard Contract for Haematopoietic Stem Cell Transplantation (Adult). NHS England Service Specification, B04/S/a. October 2013.
  - Allogeneic Haematopoietic Stem Cell Transplant for Primary Immunodeficiencies (all ages) Clinical Commissioning Policy

- **British Society for Blood and Marrow Transplants and Cellular Therapy (BSBMTCT)**
  - BSBMTCT Indications for Adult BMT

- **NHS Wales**
  - All Wales Policy: Making Decisions in Individual Patient Funding requests (IPFR).

# 4. Date of Review

This document will be reviewed when information is received which indicates that the policy requires revision.

# 5. Putting Things Right

## 5.1 Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided.

The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern.

If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales the patient or their representative should be guided to the NHS Trust Concerns Procedure, with a copy of the concern being sent to NWJCC.

## 5.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The request will then be considered by the All Wales IPFR Panel.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated.

If the patient wishes to be referred to a provider outside of the agreed pathway, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Individual Patient Funding Requests

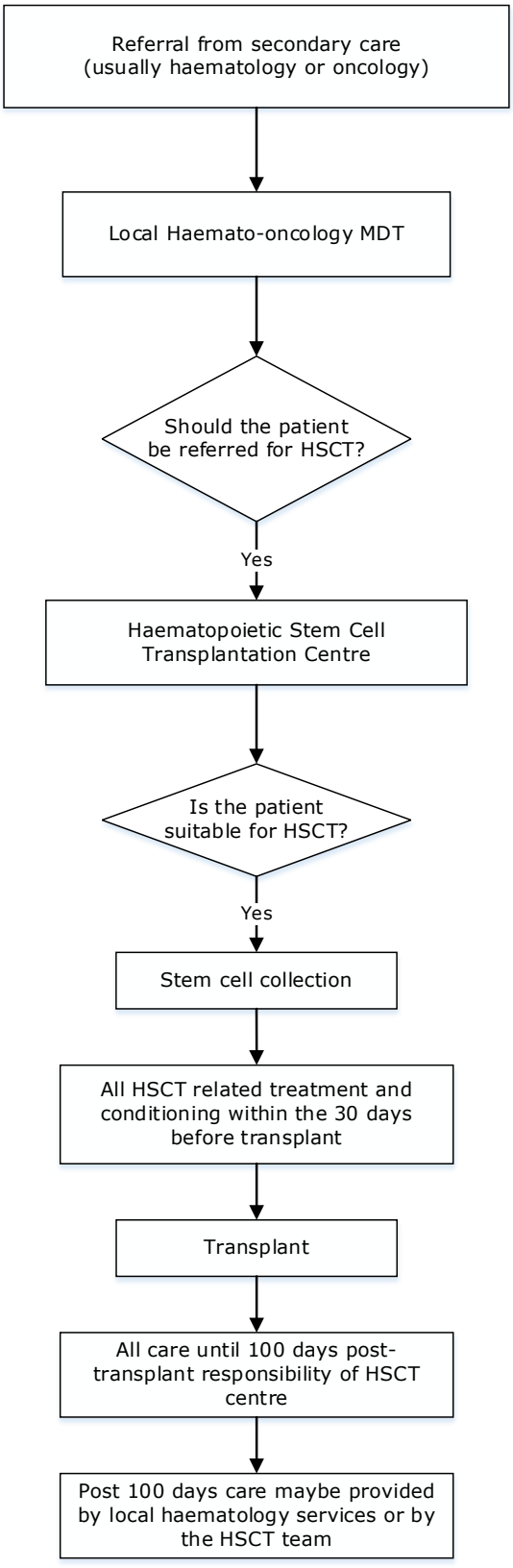
# 6. Equality Impact and Assessment

The Equality Impact Assessment (EQIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable NHS Wales Joint Commissioning Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

This policy has been subjected to an Equality Impact Assessment.

The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

# Annex i Patient Pathway



# Annex ii Codes

| Code Category | Code | Description |
|---|---|---|
| OPCS | W341 | Autograft of bone marrow |
| | W342 | Allograft of bone marrow NEC |
| | W343 | Allograft of bone marrow from sibling donor |
| | W344 | Allograft of bone marrow from matched unrelated donor |
| | W345 | Allograft of bone marrow from haploidentical donor |
| | W346 | Allograft of bone marrow from unmatched unrelated donor |
| | W348 | Other specified graft of bone marrow |
| | W349 | Unspecified graft of bone marrow |
| | X334 | Autologous peripheral blood stem cell transplant |
| | X335 | Syngeneic peripheral blood stem cell transplant |
| | X336 | Allogeneic peripheral blood stem cell transplant |
| | W991 | Allograft of cord blood stem cells to bone marrow |
| | W998 | Other specified graft of cord blood stem cells to bone marrow |
| | W999 | Unspecified graft of cord blood stem cells to bone marrow |

# Annex iii Abbreviations and Glossary

## Abbreviations

| | |
|---|---|
| **BMT** | Blood and Marrow Transplant |
| **BSBMTCT** | British Society of Blood and Marrow Transplantation and Cellular Therapy |
| **HSCT** | Haematopoietic Stem Cell Transplant |
| **IPFR** | Individual Patient Funding Request |
| **JACIE** | Joint Accreditation Committee ISCT Europe |
| **NWJCC** | NHS Wales Joint Commissioning Committee |

## Glossary

### Individual Patient Funding Request (IPFR)

An IPFR is a request to NHS Wales Joint Commissioning Committee (NWJCC) to fund an intervention, device or treatment for patients that fall outside the range of services and treatments routinely provided across Wales.

### NHS Wales Joint Commissioning Committee (NWJCC)

NWJCC is a joint committee of the seven local health boards in Wales. The purpose of NWJCC is to ensure that the population of Wales has fair and equitable access to the full range of Tertiary Services. NWJCC ensures that services within our portfolio are commissioned from providers that have the appropriate experience and expertise. They ensure that these providers are able to provide a robust, high quality and sustainable services, which are safe for patients and are cost effective for NHS Wales.