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Welsh Health Specialised Services Committee (WHSSC)

Specialised Services

Policy Position PP154

Use of Plerixafor for Stem Cell Mobilisation in Children, Teenagers and Young Adults with Lymphoma and Paediatric Type Solid Tumours

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Description	NHS Wales will routinely commission this specialised service in accordance with the criteria described in this policy
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Contents

Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission Use of Plerixafor for Stem Cell Mobilisation in Children, Teenagers and Young Adults with Lymphoma and Paediatric Type Solid Tumours accordance with the criteria outlined in this document.

In creating this policy WHSSC has reviewed the relevant guidance issued by NHS England and has concluded that Plerixafor for Stem Cell Mobilisation in Children, Teenagers and Young Adults with Lymphoma and Paediatric Type Solid Tumours should be made available.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy 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.

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

1. Introduction

This Policy Position statement has been developed for the planning and delivery of the use of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours for people resident in Wales. This service is only commissioned by the Welsh Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

1.1 Plain language summary

Currently a small number of patients requiring a stem cell transplant for myeloma, lymphoma or paediatric solid malignant tumours are prevented from proceeding to treatment because it is not possible to collect enough cells. Although a second attempt to collect these cells can be tried, it requires a hospital admission and use of stronger chemotherapy. Plerixafor can be used (without chemotherapy) as an alternative in a second attempt at collecting the stem cells.

Stem Cell Mobilisation – this means the successful increase in the number of stem cells (PBSC) circulating in the blood from where they can be collected.

Stem Cell Harvest – this means the collection of the stem cells (PBSC) from the blood using a cell separator machine.

Solid tumours – this means an abnormal mass of tissue. Solid tumours may be benign (not cancer), or malignant (cancer). Different types of solid tumours are named for the type of cells that form them. Examples of solid tumours are sarcomas, carcinomas, and lymphomas. Leukaemias (cancers of the bone marrow and blood) generally do not form solid tumours.

Apheresis – this is the name given to the flow of the patient's blood through the cell separator during which the stem cells (PBSC) are separated and collected into a separate container in which they can then be frozen for later use.

CD34+ cell – this is the protein expressed on the stem cells (PBSC) that we can detect allowing us to count the number of stem cells in the blood or the harvest.

Target dose – optimal number of PBSC (CD34+ cells) required to safely proceed to a transplant.

Autologous stem cell transplant – this is the process of high dose chemotherapy followed by infusion of the harvested stem cells which will

repopulate the bone marrow and allow the recovery of the patient's blood counts.

1.2 Aims and Objectives

This policy position statement aims to define the commissioning position of WHSSC on the use of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours.

The objectives of this policy are to:

- ensure commissioning for the use of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours is evidence based
- ensure equitable access to the use of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours
- define criteria for children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours to access treatment
- improve outcomes for people with for children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours

1.3 Epidemiology

Approximately 10-20% of patients undergoing stem cell mobilisation fail to collect an adequate number of stem cells to proceed to stem cell transplant at the first attempt.

Data from Nottingham and Glasgow show that approximately 16% of patients will require pre-emptive plerixafor, of which >90% can then successfully be mobilised following intervention with pre-emptive plerixafor, preventing the requirement for second mobilisation attempt (data not published).

1.4 Current Treatment

Patients with cancers such as

- myeloma - a cancer that affects the bone marrow
- lymphoma - a cancer that affects cells of the immune system called 'lymphocytes'
- paediatric-type solid malignant tumours - cancerous tumours; can be successfully treated with chemotherapy followed by transplantation of blood stem cells.

For some children, treatment does not go ahead as it is not possible to collect enough cells. A second attempt to collect these cells can be tried,

however it requires a hospital stay and the use of stronger chemotherapy. This can lead to side effects.

1.5 New Treatment

A medicine called plerixafor can be used without chemotherapy in a second attempt at collecting the stem cells.

- This has been shown to be highly effective.
- The treatment can also be safely given to a patient as an outpatient, i.e. without a hospital stay.

In addition, if patients have 'indicators' that highlight that the collection might fail, they can be given plerixafor before treatment starts. This aims to prevent patients from having a collection failure in the first place.

1.6 What NHS Wales has decided

WHSSC has carefully reviewed the relevant guidance issued by NHS England. We have concluded that there is enough evidence to fund the use of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours, within the criteria set out in section 2.1.

2. Criteria for Commissioning

The Welsh Health Specialised Services Committee approve funding of plerixafor for stem cell collection in children, teenagers and young adults with lymphomas and paediatric-type solid malignant tumours in-line with the criteria identified in the policy.

2.1 Patient selection

Patients eligible for treatment with plerixafor are those with Hodgkin's Disease (HD) Non-Hodgkin's lymphoma (NHL), multiple myeloma (MM) and children, young people and young adults (≤24 years) with lymphomas and paediatric-type solid malignant tumours who meet the standard criteria and are scheduled for an autologous haematopoietic stem cell transplant but:

- Have failed one previous attempt at mobilisation using a standard mobilisation regimen combining chemotherapy + G-CSF or G-CSF alone (rescue treatment). These patients will be offered a second mobilisation attempt with planned use of combination high dose G-CSF and Plerixafor.

or

- While undergoing mobilisation with a standard chemotherapy + G-CSF or G-CSF based regimen, have a low peripheral blood CD34+ cell count on the day of expected harvest and are not considered by the transplant consultant to have a reasonable chance of collecting enough cells (pre-emptive treatment). These patients will be given plerixafor as an unplanned addition to their mobilisation regimen. Enter inclusion criteria/split by disease type if necessary.

2.2 Inclusion Criteria

Patients who have previously failed a mobilisation attempt (rescue) should receive G-CSF (10 µg/kg, or in accordance with protocol) subcutaneously each day for 4 consecutive days (It is usually prescribed to the nearest ampoule size multiple, in accordance with transplant centre policy):

- on the fourth day patients assessed as requiring plerixafor (usually if the peripheral blood CD34+ cell number are < 15 per microlitre) receive a dose of 240 µg/kg in the early evening as a subcutaneous injection into the abdomen (as per EMA license EMA/H/C/1030)
- on the morning of the fifth day, a full blood count and peripheral CD34 count should be performed prior to harvest. It is the responsibility of the Transplant Consultant, to decide whether the harvest should proceed on the basis of the blood CD34+ estimation (usually if above 10 CD34+ cells per microlitre)

- if the count is insufficient to harvest cells that day, or if insufficient stem cells have been harvested, then patients should receive a further dose of GCSF and a repeat dose of plerixafor (240 µg/kg) that evening in an identical fashion to the day before. A second attempt at harvest should be made the following day.

Patients who appear to be failing a mobilisation attempt (pre-emptive) these are patients in whom the CD34+ cell count in the blood is <15 per microlitre on the day of predicted day of stem cell harvest:

- these patients are given a dose of subcutaneous plerixafor with GCSF 10 µg/kg and an attempt at harvesting is made the following day if the repeat CD34+ is sufficient
- if the CD34 level in the blood remains <15 per microlitre then the harvest should be delayed and a further dose of G-CSF and plerixafor may be given that evening.

2.3 Exclusion Criteria

Plerixafor should not be used in pregnant patients, and both male and female patients who are sexually active should be advised to use suitable contraception for three months during and after its use because of the potential harmful effects on the gametes (sperm/ova) and any resulting pregnancy.

Plerixafor is not funded for patients undergoing a first attempt at stem cell mobilisation unless they meet the criteria for pre-emptive therapy.

Plerixafor should not be used for patients who have already received it pre-emptively during a previous failed attempt at mobilisation.

2.4 Stopping Criteria

A maximum of three doses of plerixafor in total may be used.

In general a collection totalling >2 X (106) CD34+ cells per kilogram body weight will be sufficient to adequately support a single high-dose therapy procedure in adults. Paediatric requirements may differ – clinicians should refer to individual treatment protocols.

2.5 Continuation of Treatment

Healthcare professionals are expected to review a patient's health at regular intervals to ensure they are demonstrating an improvement to their health due to the treatment being given.

If no improvement to a patient's health has been recorded then clinical judgement on the continuation of treatment must be made by the treating healthcare professional.

2.6 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.

2.7 Patient Pathway

Patients for stem cell harvesting will normally be referred to the stem cell collection unit by the transplant team with a written prescription detailing the target stem cell dose required as per JACIE and Human Tissue Authority (HTA) recommendations.

Either the transplant team or the collection team (depending on local factors) will be responsible for the authorisation and administration of plerixafor for patients requiring this intervention.

2.8 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, and IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests

2.9 Clinical Outcome and Quality Measures

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

Consent, patient evaluation and investigations prior to the commencement of the mobilisation procedure must be carried out at the stem cell collection centre in accordance with relevant transplant centre policy.

All processes involved in the provision of plerixafor and the subsequent harvesting of peripheral blood stem cells must fulfil Human Tissue Authority (HTA) requirements and must meet JACIE accreditation standards.

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 and for children, teenagers and young adults.

2.10 Responsibilities

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 WHSSC for the treatment.

In all other circumstances an IPFR must be submitted.

3. Documents which have informed this policy

The following documents have been used to inform this policy:

- **NHS England policies**
  - Clinical Commissioning Policy: Use of plerixafor for stem cell mobilisation (updated to include paediatrics), NHS England 16064/P. August 2016

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

- **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: Raising a Concern

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 WHSSC.

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, an IPFR should be submitted.

Further information on making IPFR requests can be found at: Welsh Health Specialised Services Committee (WHSSC) | 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 Welsh Health Specialised Services 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 Codes

Code Category	Code	Description
ICD-10	C81	Hodgkin's Lymphoma
ICD-10	C82	Follicular Lymphoma
ICD-10	C83	Non-follicular Lymphoma
ICD-10	C84	
ICD-10	C85	
ICD-10	C86	
ICD-10	C90	