Blood and Marrow Transplantation Services and QIPP

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1. Executive Summary

This report is one in a series produced by the Specialised Healthcare Alliance looking at various services, including Blood and Marrow Transplantation (BMT), which have been prioritised by the National Specialised Commissioning Group in relation to QIPP (a policy which aims to deliver quality and productivity at a time of spending constraint). This report was particularly informed by a stakeholder workshop on BMT services organised by the Alliance and the South Central Specialised Commissioning Group on 4 November 2010. A wide range of stakeholders including commissioners, clinicians and representatives from patient organisations attended the workshop.

The report sets out some background information on QIPP and BMT services before seeking to distil the major themes explored during the workshop in relation to 1) clinical indications, 2) treatment and care, 3) outcomes and 4) commissioning. For each theme, some context and background with regard to the key issues is given (including some salient issues which it was not possible to discuss at the workshop), as well as an overview of the discussion at the event.

Among the most important points to emerge, attention is drawn to:

- The need to develop a consensus as to what constitutes ‘robust evidence’;
- The possibility of developing a national board/expanding the use of the BSBMT Adjudication Panel to reduce inconsistencies in commissioning and clinical practice;
- The potential for using the patient survey developed by Lymphoma Association, Myeloma UK and Leukaemia care to develop relevant patient outcome measures and ensure that the service is patient centred;
- The need to develop designation for Hematopoietic stem cell transplantation (HSCT) to improve quality and efficiency;
- The potential for using CQUINs to improve data collection where necessary.

2. Background

2.1 What is ‘QIPP’?

Quality, Innovation, Productivity and Prevention (QIPP) is the policy being used by the NHS to find £15-20 billion of savings identified by Sir David Nicholson as necessary in 2011/14 as a result of rapidly rising demand for services and a challenging fiscal climate.

The overall aim of the scheme is to combine improvements in quality of care with efficiency savings. Ideally, quality and productivity will go hand-in-hand, providing a better service for the patient, as well as cost savings for the NHS as a whole.

The National Specialised Commissioning Group (NSCG) has prioritised ten services for taking forward the QIPP agenda. Each Specialised Commissioning Group (SCG) is responsible for one of these services with the South Central SCG leading for BMT services. The Specialised Healthcare Alliance is looking at nine services in relation to QIPP with the aim to ensure a balanced discussion between the four strands of QIPP.
2.2 BMT services – the current picture

Collectively, haematological cancers are the fifth most common type of cancer in the UK, accounting for seven per cent of all cancers. Incidence of the individual types of haematological cancer is low but overall incidence appears to be rising (particularly non-Hodgkins lymphoma). The three main groups of haematological cancers are:

1) **Leukaemias** - malignant proliferations of blood–forming stem cells within the bone marrow;
2) **Lymphomas** - malignant proliferations of lymphocytes, in which the abnormal cells are found mainly in lymph nodes or extranodal lymphoid tissues;
3) **Myelomas** - malignant proliferation of plasma cells, which are highly specialised lymphoid cells normally responsible for production of antibodies.

An estimated 45,000-50,000 hematopoietic stem cell transplants (bone marrow, peripheral blood stem cell, or cord blood transplants) are performed annually worldwide to treat patients with life-threatening malignant and non-malignant diseases. In the 53 centres in England reporting transplant activity to the BSBMT, there were 2765 transplants reported in 2007. Due to advances in transplantation, long-term experience and ongoing clinical trials, the list of indications for which transplant is a standard treatment option continues to grow¹ and this number is expected to have risen to 3000 in 2010/11.²

Specialised Services for Blood and Marrow Transplantation (all ages) is included in the revised Specialised Services National Definitions Set (SSNDS) and is the responsibility of the SCGs. However, the proposals for England set out in the White Paper *Equity and Excellence: Liberating the NHS*³ give responsibility for national and regional commissioning, as defined by the National Definitions Set, to the NHS Commissioning Board.

HSCT is a treatment used in the management of a wide range of haematological disorders.⁴ It is used most frequently in the treatment of haematological cancers, bone marrow failure syndromes and for some solid tumours. Transplants are carried out after ‘conditioning’ (total body irradiation (TBI) and/or high dose chemotherapy) which is used to eliminate cancer cells by the destruction of rapidly dividing tissue in the bone marrow.

There are two types of HSCT transplant:

1. **Autologous transplantation** involves the re-infusion of bone marrow or peripheral blood stem cells taken from the patient after treatment. The patient’s blood stem cells are collected in advance and then returned to them after they receive high doses of chemotherapy. In general autologous transplant is used in patients unfit for allogeneic transplant, either due to age, co-morbidity or lack of a suitable donor or where it is possible to render the bone marrow temporarily relatively free of

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¹ National Marrow Donor Programme available [here](#).
² National overview of commissioning BMT.
³ Link to document: [here](#).
⁴ SSNDS Definition No.2, Specialised Services for Blood and Marrow Transplantation (all ages) (3rd edition) available [here](#).
malignant cells so that a stem cell product free of disease can be collected from the patient.

2. Allogeneic transplantation uses stem cells donated by another person for transplantation. Increasingly cord blood cells as well as haploidentical cells (usually from a parent) are used as the source of stem cells in both adults and children. However, the genetic disparity between donor and recipient can result in a number of life-threatening complications including graft versus host disease (GvHD), graft rejection and delayed immune reconstitution. Because of the higher associated risks, both long term and short term, allogeneic transplant is primarily used in patients with diseases that are hard to eradicate from bone marrow using chemotherapy, fitter patients and those with suitable donors.

In the case of autologous transplantation the conditioning therapy is responsible for tumour reduction and is the main function of the procedure. If the stem cells are obtained from a donor (allogeneic transplantation) the conditioning is designed to reduce tumour load but also to eliminate residual recipient host haemopoiesis and to provide suppression of the recipient’s immune system so as to permit engraftment of donor cells. The transplanted cells replace the patient’s own blood-forming marrow cells with healthy cells.

2.3 QIPP and BMT services

Years of experience, trials and improving outcomes in patients have lead to a steady increase in the number of patients and conditions which are suitable for transplants, also incurring a rise in costs. QIPP provides BMT services with the opportunity to identify areas for improvement in the service as well as to look for efficiency savings.

3. Main Themes

3.1 Clinical indications

The purpose of this section is to review the current indications for HSCT in children and adults and to explore how a stronger evidence base could be developed to support commissioning decisions.

The main conclusions from this section are:

- The need to develop a consensus as to what constitutes ‘robust evidence’;
- The need to systematically collect and analyse observational data over time to demonstrate results, where randomised controlled trials are not feasible;
- The possibility of developing a national board/expanding the use of the BSBMT Adjudication Panel to reduce inconsistencies in the commissioning process.

Indications

The British Society of Blood and Marrow Transplantation (BSBMT) aims to provide an up-to-date indications table for transplanter, referring haematologists and purchasers. The
indications table is updated annually and is used widely by clinicians and commissioners in the decision-making process. The Children’s Cancer and Leukaemia Group developed an equivalent indications table for children which is currently being updated.

While there is extensive work taking place in this area, there are still conversations to be had regarding the evidence base that supports the indications tables. The small size of the HSCT patient population means that randomised controlled trials (RCTs) are rarely possible. This is especially true of children’s indications where the umbrella term given to a condition can refer to a disease with huge genetic diversity or diversity in its manifestation. For indications tables to carry the appropriate weight in commissioning decisions there needs to be some level of consensus as to what constitutes ‘robust evidence’. Widening the evidence base to include registries of observational data is a possible way for the treatments outcomes to be recorded and analysed and to extend the evidence base beyond the limited scope of RCTs.

A further point concerned the way in which the criteria for access indications are currently being used, with clinicians seemingly treating the CO (clinical opinion) indication as S (standard of care)\(^5\). It was suggested that this use of clinical indicators made it difficult for commissioners to gauge what impact the variation in CO cases had on treatment outcomes. While there is a further discussion to be had regarding the appropriate governance of clinical decision making, there was a level of agreement that the annually revised indicators should be shared with commissioners before general release.

A further suggestion tabled at the workshop was that a national forum or board could improve consistency throughout England when indications table do not provide adequate guidance. The BSBMT Adjudication Panel currently offers independent adjudication on cases where indications or funding are disputed. The expansion of this body and the inclusion of non-clinical perspectives may be an option for improving consistency in commissioning where the limitation of indications is recognised.

**National standards and guidelines**

A central role of the BSBMT is to oversee JACIE (Joint Accreditation Committee of the International Society for Cell Therapy and the European Group of Blood and Marrow Transplantation). JACIE is a Europe-wide initiative aimed at promoting high-quality medical and laboratory practice via a system of voluntary standards, inspection and accreditation.\(^6\)

In order to apply for accreditation, allogeneic transplant centres must carry out a minimum of 10 procedures of each specific type of transplant offered per year and for autologous transplant centres a minimum of five per year (JACIE Standards 4th edition 2009). In addition to providing accreditation, JACIE ensures that centres have quality

\(^5\) S = standard of care, CO = clinical option, can be considered after assessment of risks and benefits, D = developmental, further trials are needed, GNR = generally not recommended. BSBMT indications for BMT available [here](#).

\(^6\) SSNDS DefinitionNo.2, Specialised Services for Blood and Marrow Transplantation (all ages) (3rd edition) available [here](#).
management systems to ensure high standards of practice.\textsuperscript{7} NICE recommends that transplants should only be performed in centres meeting JACIE standards.\textsuperscript{8}

Improving consistency in clinical management and outcomes has been highlighted as an area for attention within the QIPP agenda. In addressing perceived inconsistencies in commissioning, clinical practice and outcomes, JACIE accreditation could be used to greater effect, as while most UK-based centres performing allografts are JACIE accredited, many autograft centres are not. It has also been suggested that if the JACIE minimum is felt to be too low, commissioners could work together to agree a “number needed to treat” per annum, which each centre must reach to achieve accredited status.

Designation, on the other hand, provides an opportunity to improve efficiency and drive up standards in part by limiting the number of transplant centres and consequently increasing the number of transplants per centre. Even as the numbers of transplants performed increases, the annual case load in any given centre is small and designation will ensure that centres, especially those performing cord blood or allogeneic transplants, have large enough patient populations.

The creation of a national board such as the one suggested above may provide another opportunity to limit inconsistencies in commissioning and clinical practice and provide best practice guidelines.

\section*{3.2 Treatment and care}

The purpose of this section is to consider the key issues and messages covering all elements of the care pathway and potential future clinical developments.

The main conclusions from this section are:

- The need to develop a protocol by which commissioners can officially fund trials where there is only observational data available;
- The possibility of national procurement for blood and drugs.

\section*{Patient Pathway}

HSCT is delivered by a multidisciplinary team including specialised medical staff and nurses. Treatment can also be supported by clinical services including cardiology, dermatology, dietetics, gastroenterology, laboratory medicine including histopathology, imaging, infectious diseases, nephrology, palliative care, pharmacy, respiratory medicine and access to intensive care.\textsuperscript{9}

The patient pathway comprises four stages shown below:

\begin{itemize}
\item \textsuperscript{7} About JACIE available from \url{here}.
\item \textsuperscript{8} NICE (2002) Improving outcomes in haematological cancers.
\item \textsuperscript{9} SSNDS Definition No.2, Specialised Services for Blood and Marrow Transplantation
\end{itemize}
1. **Pre-transplant assessment:** One or more out-patient attendances for adults/children of pre-transplant assessment for autograft/allograft. This includes all tests and assessment and tissue typing where appropriate.

2. **Harvest episode:** The harvest of the bone marrow or stem cells from the patient or donor including volunteer unrelated donors (VUD) and donors from overseas.

3. **Transplant:** The relevant operating procedure codes (OPCS) procedure codes for the transplant episode are listed in the SSNDS Appendix.

4. **Post transplant follow-up:** this will vary depending on the level of complexity.
   - i) Simple – out-patient or day care attendance
   - ii) Complex I – in-patient admissions post allograft (any type) without GvHD
   - iii) Complex II – in-patient admissions with GvHD, and/or anti-fungal therapy, reinfusion of stem cells without additional conditioning, cell therapy for infection, and treatment of GvHD and/or disease relapse.

Although the HSCT patient pathway is comparatively straightforward it can span months or years in the pre- and post-transplantation stage. The importance of follow-up and long-term monitoring was emphasised during the workshop, particularly the collection of data.

**Horizon scanning**

As the numbers of people who have undergone donor stem cell transplants increase, doctors gain knowledge of how the process works and safety and technological developments decrease toxicity of transplants. However, more research is needed for donor transplantation to improve with regards to safety and long term outcomes. In broad terms the current research taking place looks at the best forms of preparatory treatment, ways of preventing GvHD and techniques for speeding bone marrow recovery time.\(^\text{10}\)

SCGs do not provide funding for transplants that are viewed to be in ‘trial’ phase, although they may do unwittingly. The limitation of such funding restrictions is that for cases where there is no precedent of treatment, transplantation can be overlooked, especially when a particular manifestation of a haematological cancer is rare. It has been suggested that, where transplants are considered necessary (by an adjudication panel) and there is no previous protocol for treatment, SCGs should fund the treatment costs and data should be collected to contribute to an observational registry database. The development of a nation board could play an important role in adjudicating which transplants should be granted trial status. Further suggestions for clinical innovation that arose from discussions at the workshop emphasised the importance of international surveillance, especially in Europe, to keep abreast of successful developments and treatment options.

**Donation**

Clinical outcomes have traditionally been best with Human leukocyte antigen HLA-matched\(^\text{11}\) siblings. However recent evidence has shown that unrelated donor transplant, identified by highly specific molecular tests, can produce

\(^{10}\) Macmillian cancer [Link](#).

\(^{11}\) The human Leukocyte antigen test is used to assess compatibility of donors tissue
outcomes equivalent to sibling transplants. Stem cell registries such as Anthony Nolan are able to provide HLA-compatible donors through such processes.

Cord blood, collected from the umbilical cord, is increasingly used for allogeneic transplant. The NHS Cord Blood Bank was established in 1996 and is an important resource for allogeneic transplant where no HLA-matched donor is available or where transplantation is urgently required. A significant concern looking forwards is the growing cost of procuring voluntary unrelated stem cells (VUS), with the UK seeing a 20 per cent rise in the cost of acquisition.

Another area singled out for discussion at the workshop was inconsistencies in procurement and prescribing patterns for antifungal drugs. A possible reason for such inconsistencies is the lack of available diagnostic facilities to determine whether an infection is present. Addressing these limitations will be essential to keep costs down and to provide high quality and consistent care across centres.

3.3 Outcomes

The purpose of this section is to consider what is important to patients and how patient reported outcome measures (PROMs) and patient reported experience measures (PREMs) can be developed for use within contracts.

The main conclusion from this section is:

- The potential for using the patient survey developed by Lymphoma Association, Myeloma UK and Leukaemia care to develop relevant patient outcome measures and ensure that the service is patient centred.

Liberating the NHS places emphasis on the importance of a service which is patient and public centred and underlines the government’s intention to expand the use of PROMs ‘across the NHS wherever practicable.’

PROMs are a way of measuring the health gain to patients after a particular surgical procedure. At present, PROMs are only used for patients having hip or knee replacements, varicose vein surgery or hernia surgery. The patient’s health gain is typically measured using short, pre- and post- operative surveys which are filled out by the patient and which measure patients’ health status or health-related quality of life at a single point in time.

Participants at the workshop expressed concern that the application of PROMs and PREMs to BMT services would be complicated in part because the patient is likely to have a worse quality of life in the immediate time following the transplant than prior to it. Another significant question that has arisen around PROMs more generally is the difficulty in identifying which patient experiences and outcomes are important and how exactly non clinical outcomes can be measured effectively and consistently.

The survey developed by Lymphoma Association, Myeloma UK and Leukaemia Care and completed by over 100 patients identifies some issues which should

12 SSNDS Definition No.2, Specialised Services for Blood and Marrow Transplantation
inform future patient surveys. A significant finding was the number of patients who felt “cast adrift” after sustained contact with the hospital and medical staff, signalling a need for better post treatment support networks. Some other common themes highlighted by the survey included the importance of psychological support, the environment of the centre such as natural light and access to a private bathroom and travel times to the treatment centre.

3.4 Commissioning

The purpose of this section is to explore the variances in current commissioning arrangements, to consider the development of a more consistent approach, and how to manage expenditure on HSCT and improve quality.

The main conclusion from this section is:

- The potential for using CQUINs to improve data collection
- The need to develop designation for HSCT to improve quality and efficiency.

As with other rarer cancers, new treatments for haematological cancers have vastly improved in recent years. However, where new drugs are not available nationally, pressures on local budgets lead to a so-called “postcode lottery”. Other inconsistencies in commissioning, costs and management have resulted in inconsistent service provision.

Most SCGs are developing commissioning policies and service specifications. For example, London SCG introduced a pathways and tariff structure for 2009-11.

Yorkshire and the Humber Specialised Commissioning Team (SCT), on behalf of all the SCGs, led on the development of the designation documentation for BMT. The four essential elements of designation of a BMT service are:

- JACIE Accreditation;
- Demonstration of a clear service model;
- Compliance with all essential elements of the local SCG service;
- Compliance with the local SCG commissioning policy.

Stakeholders discussed the importance of data collection for benchmarking purposes and to improve centres’ performance. It was also thought that for data to have a greater effect on quality, clinical practice needed to be standardised, for example through compulsory JACIE accreditation.

On reflecting upon cost and efficiency improvements, the potential to develop different care pathways for autografts and allografts emerged. It was suggested that while allografts would always need to be performed in specialist centres, autografts did not require air filtration, amongst other things, and could be performed at District General Hospitals. The PbR costing model developed by Yorkshire and Humberside SCG further supports the possible development of separate care pathways with the autograft care package acting as the basic bundle onto which other aspects of the pathway could be added.
A further development in the commissioning process is the need for commissioners to have a better understanding of which parts of the pathway they are procuring from providers as compared with PCTs. The national aim is thus to develop and implement a common procurement tariff structure which informs all stakeholders. This will also be used to support PbR development and shared care arrangements between tertiary centres and District General Hospitals.

Commissioning for Quality and Innovation (CQUIN)

The Commissioning for Quality and Innovation (CQUIN) payment framework makes a proportion of providers’ income conditional on outcomes which demonstrate improvements in quality and innovation in specified areas of care. The goals set by CQUINs may be described as ‘stretch’ goals since they aim to encourage improvements in the quality of care provided, over and above core requirements. Five of the SCGs have included CQUIN schemes in their 2009/10 contracts.

Participants understood CQUINs in a variety of ways, some viewing them as a means to address underperformance rather than to inspire innovation. Although there was no consensus about how CQUINs should be used in the future, some stakeholders felt that CQUINs could be a powerful tool for improving data collection, especially in areas where data is below par, such as that relating to follow-up and long-term monitoring.