



APPG
Stem Cell
Transplantation

ANTHONY
NOLAN

saving the lives
of people with
blood cancer

CORD BLOOD TRANSPLANTATION: MEETING THE UNMET DEMAND

Progress review

Supported by Anthony Nolan
March 2015

This is not an official publication of the House of Commons or the House of Lords. It has not been approved by either House or its committees. All-Party Groups are informal groups of Members of both Houses with a common interest in particular issues. The views expressed in this Report are those of the Group.



CONTENTS

FOREWORD	4
BACKGROUND	5
1. MONITORING PROGRESS	6
2. INCREASING SUPPLY	7
3. IMPROVING STANDARDS	8
4. FACILITATING RESEARCH	9
CONCLUSION	10
APPENDIX: PROGRESS AGAINST 2012 RECOMMENDATIONS	11

FOREWORD

In January 2012, the APPG on Stem Cell Transplantation published 'Meeting the Unmet Demand', which highlighted the potential of cord blood donations to transform our ability to meet the needs of every patient who requires a stem cell transplant.

We made a number of recommendations, building on those of the UK Stem Cell Strategic Forum, designed to support four key outcomes: monitoring progress, increasing supply, improving standards and facilitating research. Our proposals set out a way forward for all stakeholders – the APPG included – to work together to maximise the lifesaving potential of cord blood transplantation.

In the three years since its publication we have seen progress in a number of areas. Cord banking rates have tripled, a quarter of all cord transplants in the UK are now sourced domestically, and the cost of transplants to the NHS has decreased dramatically.

These improvements in the effectiveness and sustainability of cord collection have a lifesaving impact. Over 60% of black, Asian and minority ethnic patients are now able to find a well matched stem cell donor; this figure stood at 40% in 2010. But while we have taken steps forward, a long journey remains if we are to eliminate unmet patient demand for stem cell transplantation. We also share the Stem Cell Strategic Oversight Committee's desire to see more progress in the area of stem cell research.

As with our 2012 report, this document endorses and builds upon the latest findings and recommendations of the UK Stem Cell Strategic Oversight Committee. In this update we acknowledge the progress that has been made and underline where there is a need for greater focus, and we renew our challenge to the government to do all it can to ensure that the UK meets its potential to become the world leader in this exciting area of regenerative medicine.



Handwritten signatures of David Burrowes and Mark Tami.

**David Burrowes MP and Mark Tami MP,
Co-Chairs, APPG on Stem Cell Transplantation**

BACKGROUND

Cord blood remains in the placenta and umbilical cord after a baby is born. Cord blood is rich in stem cells, which can be used to cure a broad range of life-threatening diseases, including blood cancers and other haematological disorders. Since the first unrelated cord blood transplant took place in 1993, public cord blood banks have been established around the world, including the Anthony Nolan and NHS Cord Blood Bank in the UK.

In 2010 the UK Stem Cell Strategic Forum was established by the government to produce a report advising on future options for the provision and use of stem cells from both unrelated adult donors and cord blood. Following the publication of its report that year¹, the Stem Cell Strategic Oversight Committee ('the Oversight Committee') was created to ensure the progress of key recommendations within the Forum's report.

In January 2012 the APPG on Stem Cell Transplantation endorsed and built upon the Forum's recommendations relating to cord blood in 'Meeting the Unmet Demand'. In November 2014 the Oversight Committee completed an updated report which assessed progress and set revised targets for securing sustainable provision of stem cells for transplantation in the UK. This report made a series of recommendations for increasing the supply of adult donor cells and growing the UK's cord blood inventory.

BOX 1

KEY FINDINGS OF THE UK STEM CELL STRATEGIC OVERSIGHT COMMITTEE RELATING TO CORD BLOOD TRANSPLANTATION (2014/15)

UK-sourced cord blood is increasingly meeting the needs of UK patients.

- Cord banking rates in the UK have tripled since 2011/12.
- In 2014 over 25% of cord blood transplants used donations from UK donors; compared with just 10% in 2010.
- Over 60% of black, Asian and minority ethnic (BAME) patients are now able to find a well matched donor. This represents a significant improvement on the 40% figure cited in 2010.

The use of UK-sourced cord blood saves the NHS money.

- UK-sourced cord blood donations are priced at £14,500, compared to around £30,000 for an imported donation.
- The estimated cost per QALY² for an inventory of 50,000 cord blood donations has fallen from £27,000 in 2010 to £9,400.
- The growth in UK-to-UK provision of unrelated donor stem cells contains costs at a time of increased transplant activity.

1 'The Future of Unrelated Donor Stem Cell Transplantation in the UK' (July 2010)

2 A Quality-adjusted life year (QALY) is the measure of the state of health of a person or group in which the benefits, in terms of length of life, are adjusted to reflect the quality of life. One QALY is equal to one year of life in perfect health

1. MONITORING PROGRESS

In 'Meeting the Unmet Demand', the APPG commended the government for supporting the UK Stem Cell Strategic Forum's recommendations, and underlined the importance of the Forum's Oversight Committee in providing focus and drive for the community seeking to deliver them. The report recommended that the progress against each recommendation be kept under review by the Oversight Committee, alongside relevant scientific developments, levels of unmet demand and trends in the use of stem cell transplantation (recommendations 1-4, 2012).

The Oversight Committee has concluded a review of progress achieved since its 2010 report, and published its findings in 'Unrelated Stem Cell Transplantation in the UK: Effective, Affordable, Sustainable' in November 2014. The APPG welcomes the progress that has been recorded since the establishment of the Forum, which are summarised in box 1.

A reappraisal of inventory utilisation rate by the Oversight Committee in 2012 led to a recommendation to bank only those donations containing over 14×10^8 pre-processing total nucleated cells (TNC)³. This is significant because donations containing larger volumes of cells are clinically preferable for transplant, and utilisation rates increase accordingly. As such the APPG welcomes this development as a step towards ensuring a high quality and financially sustainable cord blood inventory. Monitoring the inventory utilisation rate will be particularly important in light of the Oversight Committee's recommendations for phased growth in donations, summarised in box 2. A utilisation rate of 1% per annum is required to ensure long-term funding to achieve an inventory of 50,000 donations.

BOX 2

KEY RECOMMENDATIONS OF THE UK STEM CELL STRATEGIC OVERSIGHT COMMITTEE RELATING TO CORD BLOOD TRANSPLANTATION (2014/15)

A cord blood inventory of 50,000 donations best meets the needs of patients.

- The Anthony Nolan and NHS Cord Blood Bank should establish an inventory of 30,000 cord blood donations by 2018.
- Beyond 2018, inventory growth to 50,000 donations should be funded via income generated through donation provision.
- Inventory utilisation should be maximised by banking only those donations likely to contain a clinically useful dose of stem cells, equivalent to 14×10^8 total nucleated cells before processing; 30-50% of donations should include BAME parentage.

A national stem cell transplantation trials network should be established.

- This network should facilitate and promote high quality prospective, randomised and controlled early phase clinical trials.
- The UK is failing to exploit the economic opportunity presented by its unique access to world-class science and a large and coherent transplant population.

Recommendation 1

The UK Stem Cell Strategy Oversight Committee should continue to review relevant scientific developments, levels of unmet demand and trends in the use of stem cell transplantation on a regular basis.

Recommendation 2

The Oversight Committee should monitor the inventory utilisation rate on an annual basis to ensure that sufficient income will be generated to fund the growth from 30,000 to 50,000 cord blood donations after 2018.

³ This threshold refers to the total nucleated cells contained in a cord blood sample after collection and before processing the unit

2. INCREASING SUPPLY

The APPG welcomes the improvements to the supply of UK cord blood donations, in particular the threefold rise in banking rates since 2011/12. More patients are now able to find an acceptably matched donor, thanks largely to the expansion of the cord collection programme to 14 NHS hospitals.

Continuity of government funding for cord blood collection has been vital in delivering these improvements. In April 2013 the Department of Health announced an extra investment of £4 million, taking the government's total investment in stem cell services to £12 million since 2011. The APPG welcomes this ongoing commitment and calls on the government to maintain funding to ensure an inventory of 30,000 donations can be achieved by 2018, in line with the Oversight Committee's recommendation.

The APPG recognises that the cord blood collection programmes run by NHS Blood and Transplant and Anthony Nolan are designed to use resources as efficiently as possible and to maximise the quality of the cord blood inventory (recommendation 5, 2012). Resources are being targeted at collection centres in ethnically diverse areas with high birth rates and high collection rates, with 24-hour collection operations in some centres.

In 'Meeting the Unmet Demand' the APPG highlighted the importance of reducing reliance on imported cells, and outlined an intention to ensure further research and assessment is carried out into the savings that can be achieved through extending the domestic cord blood inventory (recommendation 6, 2012). The recent analysis outlined in the Oversight Committee's report has underlined the potential for savings to the NHS as a result of increasing the number of UK-sourced cord blood donations; in 2014 this represented an avoided cost of around £270,000 per year.

The APPG also considered the sustainability of funding for cord blood transplants as a curative therapy for NHS patients (recommendations 7-8, 2012). The APPG welcomes the decision taken in 2014 by NHS England to approve the commissioning of double cord transplants, on the advice of the Blood and Marrow Transplant Clinical Reference Group. The APPG understands that a tariff for stem cell transplant is currently in development by NHS England, which will standardise commissioning across the country. It is important that such standardisation maintains the stability of commissioning in cord blood transplantation to secure patient access.

Recommendation 3

The Department of Health should continue to fund public cord blood banking to ensure an inventory of 30,000 donations will be achieved by 2018.

Recommendation 4

Anthony Nolan and NHS Blood and Transplant should continue to focus their activity towards banking the highest quality donations, as efficiently as possible, in the most ethnically diverse locations across the UK.

Recommendation 5

NHS England should ensure stability in commissioning of cord blood transplant to secure patient access.

3. IMPROVING STANDARDS

In 'Meeting the Unmet Demand' the APPG put forward two recommendations in relation to the regulatory framework for stem cell transplantation, and acknowledges progress in each of these areas:

1. In January 2013 the government announced that the Human Tissue Authority and Human Fertilisation and Embryology Authority would retain their existing functions, in line with the consultation response submitted by the APPG in September 2012 (recommendation 9, 2012).

2. In September 2012 the European Parliament published a resolution calling on the European Commission to improve transparency, standards and levels of cooperation between member states with regard to cord blood collection (recommendation 10, 2012).

To ensure the continued raising of standards, the APPG also recommended that the Oversight Committee investigate the training and education that should be required for cord collectors (recommendation 11). Recent amendments to NICE guidance⁴ on cord clamping have prompted the Oversight Committee to recommend the monitoring of stem cell yields, and the provision of further information to midwives about public cord blood banking and stem cell transplantation. Further improvements in standards can be achieved through partnership working and sharing of best practice between collection centres.

In April 2013 the APPG brought together stakeholders in the field of cord blood banking to consider the implications of private banking, standards of information and the potential for further collaboration in the industry (recommendation 12, 2012). The APPG welcomes the Human Tissue Authority's forthcoming public information campaign, which aims to ensure that mothers are fully equipped to make an informed decision.

Recommendation 6
Anthony Nolan and NHS Blood and Transplant should work in close partnership to align standards of training across the UK's cord collection facilities, and regularly share best practice to ensure the continued improvement of operational standards.

Recommendation 7
Anthony Nolan, NHS Blood and Transplant and private cord blood banks should work with the Human Tissue Authority to ensure mothers are fully equipped to make an informed decision about cord blood banking.

⁴ National Institute of Clinical Excellence, Intrapartum care: care of healthy women and their babies during childbirth. Published December 2014. nice.org.uk/guidance/cg190

4. FACILITATING RESEARCH

Research continues to be critical in unlocking the potential of cord blood to save lives, and yet is the area in which progress has been most lacking.

In 2012 the APPG recommended that that the Departments for Health and Business, Innovation and Skills should review the adequacy of funding for research into haematopoietic stem cell transplantation in the UK, and endorsed the Oversight Committee's recommendation for a clinical trials network for stem cell transplantation (recommendations 13+15, 2012).

Since the publication of 'Meeting the Unmet Demand' the APPG has made representations to government in support of measures to facilitate funding and establish the required structures for world class stem cell research to take place. The APPG regrets that very little progress has been made in this respect, which is underlined by the Oversight Committee's recent reinforcement of its call for a national stem cell transplantation trials network.

In July 2014 the all-party groups on Stem Cell Transplantation and Medical Research jointly hosted a roundtable meeting on the growth of regenerative medicine in the UK. The meeting brought together scientists, clinicians, expert

stakeholders, government officials, MPs and Peers to discuss the potential of, and barriers to, the future development of innovative new stem cell therapies. The discussion highlighted the need for close collaboration between scientists and clinicians, and for a greater focus on developing a robust framework for clinical trials.

Recommendation 8
A national stem cell transplantation trials network should be established to facilitate and promote high-quality research into cord blood as a curative therapy for patients with blood cancer and blood disorders.

CONCLUSION

In 'Meeting the Unmet Demand', the APPG on Stem Cell Transplantation called on the NHS to declare an aspiration for the UK to be a world leader in the research, collection and transplantation of cord blood (recommendation 14, 2012). While no firm declaration has been made, progress has been made in a number of areas in the last three years.

To maintain this positive trajectory, the APPG calls on government, the NHS and the third sector to continue to work in partnership with renewed focus and commitment to achieve the next two key milestones; an inventory of 30,000 high quality cord blood units by 2018, and the establishment of a stem cell transplantation trials network.

In 2014 the first ever Life Sciences Minister was appointed with a remit to make the UK the best place in the world to discover and develop 21st century healthcare innovations. This is a welcome reflection of the UK's bright future as a global leader in regenerative medicine and, with continued support, cord blood can make a very significant contribution to achieving the government's vision.

APPENDIX: PROGRESS AGAINST THE APPG'S 2012 RECOMMENDATIONS

Recommendation	Progress since 2012
1 The Strategic Forum Oversight Committee should undertake a progress review of implementation of the Stem Cell Strategic Forum recommendations by the end of 2012, and the findings should be used to inform the next spending review.	The Oversight Committee concluded a review of progress achieved since its 2010 report, and published its findings in 'Unrelated Stem Cell Transplantation in the UK: Effective, Affordable, Sustainable' in November 2014.
2 The Strategic Forum Oversight Committee should review on an ongoing basis the scientific developments that have impact on the aims of the Stem Cell Strategic Forum recommendations, including the accepted cord unit total nucleated cell counts and CD34 counts.	A reappraisal of inventory utilisation rate in 2012 led to a recommendation to bank only those donations containing over 14×10^8 total nucleated cells.
3 The Strategic Forum Oversight Committee should review the unmet demand for stem cell transplants on an annual basis to ensure that the figure is current and to monitor progress.	The Oversight Committee's report completed in November 2014 included a revised estimate of unmet demand.
4 The Strategic Forum Oversight Committee should work with the British Society of Blood and Marrow Transplantation to maintain an observatory of indications, publishing trends in the use of stem cell transplantation for different diseases and by cell source.	The British Society of Blood and Marrow Transplantation maintains an observatory of indications, including stem cell source and disease type, for all patients.
5 The Strategic Forum Oversight Committee should determine the optimum locations of the 13 cord blood collection hospitals outlined in the Stem Cell Strategic Forum report.	To maximise efficiency and inventory growth, Anthony Nolan and NHS Blood and Transplant target their resources towards collection centres in ethnically diverse areas with high birth rates and collection rates, and run 24 hour collection operations in some centres.
6 The APPG on Stem Cell Transplantation should ensure further research and assessment is carried out into the savings that can be achieved through extending the cord blood bank inventory.	The Oversight Committee's 2014 report underlined the potential for savings to the NHS as a result of increasing the number of UK-sourced cord blood donations; in 2014 this represented an avoided cost of around £270,000 per year.
7 The Stem Cell Strategic Forum agreed that cord blood is a proven, potentially curative therapy for both paediatric and adult patients and, in recognition of this, commissioners should ensure that all patients have access to this therapy.	In 2014 NHS England approved the commissioning of double cord transplants, on the advice of the Blood Marrow Transplant Clinical Reference Group.
8 Commissioners should investigate the possible impact of a best practice tariff to reimburse designated centres for securing high quality cord blood donations.	A tariff for stem cell transplant is currently in development by NHS England, which aims to standardise commissioning across the country.
9 The APPG on Stem Cell Transplantation should invite submissions from interested parties on the future regulation of stem cell transplantation and respond to the forthcoming government consultation on regulation.	In January 2013 the government announced that the Human Tissue Authority and Human Fertilisation and Embryology Authority would retain their existing functions, in line with the consultation response submitted by the APPG in September 2012.
10 The APPG on Stem Cell Transplantation should invite submissions on how the EU Tissue and Cells Directive could better service patients through international joint working and make relevant recommendations.	In September 2012 the European Parliament published a resolution calling on the European Commission to improve transparency, standards and levels of cooperation between member states in regards to cord blood collection.
11 The Strategic Forum Oversight Committee should investigate the standard training and education that should be required for cord collectors and others present at childbirths where cord collection is taking place.	Amendments to NICE guidance on cord clamping have prompted the Oversight Committee to recommend the monitoring of stem cell yields, and the provision of further information to midwives about public cord blood banking and stem cell transplantation in blood cancers and blood disorders.
12 The APPG on Stem Cell Transplantation should invite submissions on the advantages and disadvantages of private banking, the potential for collaboration between the two sectors and the standards of information that should be available to mothers when deciding whether to bank cord blood and whether to do so privately or publicly.	In April 2013 the APPG brought together stakeholders in the field of cord blood banking to consider the implications of private banking, standards of information and the potential for further collaboration in the industry. In 2015 the Human Tissue Authority will launch a public information campaign aiming to ensure that mothers are fully equipped to make an informed decision.
13 The Departments for Health and Business, Innovation and Skills should jointly review the available funding for research into haematopoietic stem cell transplantation in the UK and report on the adequacy of funding of this field to support their priorities within regenerative medicine overall.	The APPG on Stem Cell Transplantation has made representations to government in support of measures to facilitate funding and establish the required structures for stem cell research to take place.
14 As part of its core purpose in promoting research, the NHS should declare an aspiration for Britain to be a world leader in research into the understanding of cord blood, its collection, its use in treatments; and for all stem cell transplant patients to have the opportunity to take part in a clinical trial.	In 2014 the position of Life Sciences Minister was created to support the government's vision of making the UK the world leader in healthcare innovation, including regenerative medicine. In July 2014 the APPGs on Stem Cell Transplantation and on Medical Research jointly hosted a roundtable meeting on the future of regenerative medicine in the UK. The meeting brought together scientists, clinicians, expert stakeholders, government officials, MPs and Peers to discuss the potential of, and barriers to, the growth of innovative new stem cell therapies.
15 The Strategic Forum Oversight Committee should engage relevant stakeholders to create a clinical trials network for stem cell transplantation.	The Oversight Committee reinforced its call for a national stem cell transplantation clinical trials network in November 2014.

'THE APPG CALLS ON
GOVERNMENT, THE NHS
AND THE THIRD SECTOR
TO CONTINUE TO WORK
IN PARTNERSHIP WITH
RENEWED FOCUS AND
COMMITMENT.'

For further information about the
All Party Parliamentary Group on Stem
Cell Transplantation please contact:
Rebecca Gladstone
Policy and Public Affairs Officer
Anthony Nolan
rebecca.gladstone@anthonymolan.org

Anthony Nolan
2 Heathgate Place
London NW3 2NU
0303 303 03 03
anthonymolan.org
appg-stemcell.org.uk
@APPGStemCell