Minutes of the UKSCSF Oversight Committee Launch Meeting
Friday 26th Jan

<u>Attendees</u>

In person

- Allameddine Allameddine, Medical Director, Northern Ireland Blood Transfusion Service
- Andy Clark, Consultant Haematologist, NHS Greater Glasgow and Clyde (Virtual)
- Ann O'Leary, Director of Donor and Transplantation Services, Anthony Nolan
- David Burns, Consultant Haematologist, University Hospitals Birmingham (Virtual)
- Ben Doak, National Senior Programme of Care Manager, NHS England
- Charles Craddock (Chair), Academic Director of the Centre for Clinical Haematology, University Hospitals Birmingham NHS Trust, Birmingham and Professor of Haemato-oncology, University of Warwick
- Dammy Abiola, Stem Cell Lead, Department of Health and Social Care
- Daniel Eve, National Programme of Care Senior Manager, Specialised Commissioning, NHS England
- Fiona Dignan, Chair BMT CRG, Consultant Haematologist, Manchester Royal Infirmary
- Gail Miflin, Chief Medical Officer and Director of Clinical Services, NHS Blood and Transplant (NHSBT)
- Guy Parkes, Head of Stem Cell Donation and Transplantation, NHSBT
- Henny Braund, Chief Executive, Anthony Nolan
- Jacqueline Barry, Chief Clinical Officer, Cell and Gene Therapy Catapult
- James Griffin, Consultant Haematologist at NHSBT and University Hospitals Bristol
- Keith Wilson, Director, Director, South Wales BMT Service
- Kath Bainbridge, Head of Rare Diseases and Emerging Therapies, Department of Health and Social Care
- Lilian Hook, Director, Cell, Apheresis and Gene Therapies, NHSBT
- Marc Turner, Professor of Cellular Therapy, Director Scottish National Blood Transfusion Service (Virtual)
- Michael Quinn, Northern Ireland Transplant Programme Lead
- Orin Lewis, Chief Executive, African Caribbean Leukaemia Trust
- Peter McCleave, 100,000 Donors (invited guest for Item 5) (Virtual)
- Robert Wynn, Director of Paediatric Blood and Marrow Transplant Programme, Royal Manchester Children's Hospital
- Victoria Potter, Consultant Haematologist and Transplant Director, King's College Hospital
- Alexandra Ross, UKSCSF Programme Lead, NHSBT (Secretariat)
- Greg Judge, Senior Policy and Public Affairs Manager, Anthony Nolan (Secretariat)
- Yasmin Sheikh, Head of Policy and Public Affairs, Anthony Nolan (Secretariat)

Apologies

- Eduardo Olavarria, President, British Society of Blood and Marrow Transplantation and Cellular Therapy (BSBMTCT)
- Hugh Allen, Chief Strategy Officer, Anthony Nolan
- Robert Danby, Consultant Haematologist, Oxford University Hospitals and Chief Medical and Scientific Officer, Anthony Nolan

Agenda

1. Welcome - Professor Charles Craddock, Forum Chair
2. Update on UK Government/Department of Health and Social Care (DHSC) Priorities – Dr Kath Bainbridge
3. Opportunities for the cell therapy community following the Lord O'Shaughnessy Report on Clinical Trials - Professor Charles Craddock
4. Delivery group progress updates - Delivery Group Chairs
5. Discussion: Proposal from the NBTA - Peter McCleave and Orin Lewis
6. EBMT Congress 2024 - Dr Victoria Potter
7. Meeting Close - Professor Charles Craddock

Minutes

1. Welcome – Professor Charles Craddock, Forum Chair

- Charles welcomed all those attending and provided a summary of the Forum's history, accomplishments, and objectives. He noted that the Forum was set up at the request of the DHSC, and coalesces a unique range of insights and expertise – including clinicians, commissioners, patients, DHSC, industry and the donor registries, all united in the ambition to improve patient outcomes in stem cell transplant and advanced cell therapies and to support a resilient and thriving life sciences sector.

2. Update on UK Government/Department of Health and Social Care (DHSC) Priorities – Dr Kath Bainbridge

- Kath introduced her team as having responsibility within the DHSC for stem cell transplant and emerging therapies. She noted that Health Minister Andrea Leadsom has recently been appointed and has responsibility for transplant within her portfolio, and that Minister Leadsom has welcomed the Forum's work and intends to meet with Professor Craddock as its representative.
- Kath explained that the DHSC continue to support the stem cell transplant ecosystem in the UK, with a strong focus on addressing inequalities, improving resilience and sustainability. This includes work on apheresis capacity, in line with the Forum's recommendations in this area.
- DHSC is also supporting research in the field. The NIHR has awarded £2.1m towards research projects on access, outcomes and delivery of transplants and cellular therapy, including: research aiming to better understand inequalities in the pathway; a pilot project to recruit stem cell donors to the DATRI registry in India; and projects

aiming to optimise cryopreservation techniques to improve CAR-T manufacturing capability and quality.

- A new advanced therapies co-ordination group is being established by the DHSC, which aims to reduce duplication, facilitate joint working between government and public sector partners including MHRA, NICE, MRC, and NIHR; and support a potential Spending Review bid.
- The co-ordination group has an agreed set of “problem statements” to address for the cell and gene therapy ecosystem, developed following stakeholder consultation. The group intends to meet every 6 months, with working groups looking at each problem statement in more detail.
- Keith Wilson noted the importance of including devolved nations in the coordination group, Kath responded that there would indeed be representation from the devolved administrations. Ben Doak further noted the cross-nation collaborations across the national CAR-T panel for lymphoma, ATMP Engage and the UK Rare Diseases and agreed with the importance of sharing best practice across UK.
- Henny Braund highlighted the synergies between the UKSCSF and the DHSC co-ordination group and those in the room agreed to keep both groups updated on areas of mutual concern via the DHSC representatives on the UKSCSF.

3. **Opportunities for the cell therapy community following the Lord O’Shaughnessy Report on Clinical Trials – Professor Charles Craddock**
    - Charles outlined the transformative potential of clinical trials for patient outcomes as well as in terms of the cost savings they bring to the NHS and the wider economy.
    - He noted that Lord O’Shaughnessy’s report sought to address the recent reduction in UK commercial clinical trials activity and summarised the key recommendations of the report, also noting that the report has been accepted in full by the UK government.
    - He also flagged that the report refers to establishment of ACT in 2021, as an innovative trials delivery model which has brought new medicines to the UK that would not otherwise have been available to patients and serves as a template for the future.
    - Jacqueline Barry noted that Catapult have just completed their latest review of clinical trial activity, highlighting year-on-year decreases in UK clinical trial activity as a whole. However for advanced therapies the UK appears to be reasonably attractive with 80% of all trials being commercial. She suggested that there is a strong starting point for the UK whilst recognising the opportunity to streamline and continue building attractiveness for investment.
    - Victoria Potter added that recent developments in the UK clinical trial infrastructure for stem cell transplant, notably the establishment of the IMPACT transplant and cell therapies trials network, have been instrumental in bringing inward investment, supporting protocol harmonisation and improving patient care. She added that the UK has a strong and unique proposition for commercial trial activity and there are opportunities to harness that further.
    - James Griffin echoed the need to address barriers to trial set-up as highlighted in the O’Shaughnessy report. He noted that trial set up procedures at hospital level are currently too lengthy and this impacts competitiveness, whereas trial delivery

following set-up is generally excellent. He welcomed the efforts of the UKSCSF and community through IMPACT.

4. Delivery group progress updates

4.1. Delivery Group 1: Sustainability and resilience of the stem cell supply chain (Ann O'Leary & Guy Parkes)

- Ann O'Leary and Guy Parkes outlined progress and challenges in meeting the UKSCSF recommendations on stem cell supply:
- Donor recruitment has been increasingly challenging and this is evident both in the UK and globally. The proportion of UK donors for UK patients is currently at 27%, this is lower than the 45% target set out in the report. The UK aligned registry members are meeting regularly to discuss how best to jointly address these challenges, and it is clear that multiple strategies are needed to address the changing dynamic in recruitment.
- Recruitment of donors from a minority ethnic background has also been challenging and is at 14.5% across the UK aligned registry for the year to date. While community partnerships have supported recruitment and retention, recruitment numbers are lower than in previous years.
- NHSBT and Anthony Nolan are both actively recruiting to the cord blood bank, with around 28,000 units currently banked and the ambition to reach 30,000 units. The bank is now in the enrichment phase so only those units with the highest cell count and from minority ethnic donors are being stored. Issues such as delayed clamping have made it more difficult to bank high-grade cord units. Efforts to improve awareness and support uptake of cord are continuing at pace through the cord blood education programme jointly delivered by Anthony Nolan and NHSBT.
- There is strong demand for research purposes. Both NHSBT and Anthony Nolan have now established routes to provide cord (and adult) cells for research purposes.
- UK aligned registry members are continuing to develop community partnerships to support targeted recruitment, which is of particular importance for minority ethnic donors. Partnerships take time to develop and can take years before they begin to generate live recruitment onto the registry, so require significant upfront investment.
- Orin Lewis asked for more detail on the recruitment challenges. Ann noted that WMDA data shows donor recruitment has faltered globally since the pandemic, and that the reasons are as yet unclear and will require further research and behaviour analysis. Anecdotally, the pandemic appears to have resulted in changing attitudes to health and altruism, as well as to how people respond to different communication methodologies.
- Charles proposed an action for Ann and Guy to summarise the global context and challenges for recruitment in a short paper for the UKSCSF and asked the secretariat to add this as a topic for further discussion at the next UKSCSF Oversight Committee meeting.
- Charles asked why each of the UK aligned registry members have a different % of minority ethnic donors recruited to date? In response Ann said it depends on what population is being focused on, for example NHSBT and Anthony Nolan focus on younger donors and this results in a higher proportion of minority ethnic donor

registrations. It also has to do with what kind of patient appeals each organisation can support.
- In conclusion, Ann flagged that collection centre capacity is one of our biggest challenges and the single biggest limitation to meeting first choice dates for transplant.

4.2. Delivery Group 2: Securing improvement and equity in access, experience and outcomes for patients – Henny Braund and Dr Fiona Dignan

- Henny Braund and Fiona Dignan provided a reminder of the UKSCSF's recommendations in this area, and summarised progress to date:
- Anthony Nolan has convened a multi-disciplinary pre-habilitation (prehab) specialist interest group which is working to develop a shared definition of prehab in the context of stem cell transplant and CAR-T, as well as a clinical guidelines and patient information.
- Keith Wilson noted that the South Wales BMT centre set up a formal prehab service in 2022 with financial support from Anthony Nolan and Gilead. The group are in the process of writing up the results of their initial intervention, which are promising, and he will keep the UKSCSCF members updated. Charlie noted that there are benefits to patients in terms of outcomes and efficiencies, and cost savings overall for the NHS, from offering services such as prehab.
- Anthony Nolan's PreQoL study to test the feasibility of a digital patient-reported outcomes tool for stem cell transplant is live and recruiting well.
- The NIHR-funded project to explore barriers in the pathway to transplant is in the early stages with a research fellow now recruited, they will work closely with the NHSBT Blood and Transplant Research Unit at the University of Birmingham.
- The NIHR-funded India project (aiming to develop the DATRI registry in India for mutual benefit to patients in India and of a minority background in the UK) has commenced and has recruited to its annual target within the first 3 months, the first transplant in India has taken place with a donor recruited via that project.
- EBMT and BSBMTCT ethnicity categories have been updated to match those used elsewhere in the NHS which should provide more consistency across datasets.
- Fiona noted that the NHS England service specification is being reviewed and there is an opportunity to ensure that the importance of services such as prehab and other wraparound care and support are recognised in the updated specification.

4.3. A new vision for HSCT and ATMP data - Dr Beki James and Greg Judge

- Charles noted that a Co-Convenor has now also been appointed to work alongside Beki on the Data Change Commission.
- Beki James and Greg Judge provided an update on the Commission's plans:
- Approaches to potential members will be shared shortly and the intention is to meet in the next few months to agree priority actions.
- Progress is being made on making patient outcomes data publicly available, this is being led by the BSBMTCT.
- Beki and the secretariat are in discussion with commercial partners to discuss the potential for industry funding to support the Data Change Commission's work.

- Greg requested input from the Oversight Committee on two potential options to explore further; the first would be a Quality of Life tool that would enable patients and HCPs to log patient experience information in real time; the second would be clinical decision-making tool using artificial intelligence to predict outcomes.
- Victoria said both have merit but very difficult and it would be good to see these ideas more worked up and to ask patients for their views. Fiona agreed, whilst Orin noted that patient experience would be his preference. Charles confirmed the importance of patient consultation in prioritising these opportunities
- James flagged that we would need to be mindful of any dependencies as that may affect decisions on what to prioritise, and Guy noted that a lot of work has been going on in the US into AI decision-making tools and it would be important to explore whether those would be appropriate in the UK.
- James further noted that driving improvement in ethnicity data should be a priority for the Commission.
- Beki agreed and added that variation and divergence in coding is a key issue to address, and the Data Commission aims to prioritise this area to enable much more holistic understanding of the patient journey, experiences and outcomes over time.

4.4. Global leadership in HSCT and ATMP research – Professor Rob Wynn

- Rob noted the strength of the UKSCSF lies in bringing together diverse groups with interest in the research capabilities and infrastructure for cell therapies, and provided a summary of activity to date:
- Good progress is being made towards the establishment of a paediatric clinical trials network within the ACT umbrella, with a formal launch intended for within the next year. Initial funding is being provided by the charity Cure Leukaemia. The model may be slightly different to that adopted for adult trials under IMPACT, for example rather than directly funding research nurses the institution will be able to direct funding towards the area with most need e.g. data management, trial staff or other expertise.
- More generally the number of paediatric studies in the UK has improved substantially due to the mandate for paediatric studies to be run for all new products approved for adult indications.
- Rob flagged the importance of investigator initiated studies in addition to industry-led research. Plans for an investigator-led phase 2/3 trial looking at expanded cord blood in children are underway, and a further trial of expanded cord blood in ultra-high risk leukaemia in both adults and children is seeking funding.
- Rob stressed that equity is also a very important priority and the IMPACT leadership are considering how best to embed and test interventions to improve the diversity of trial participants.
- Victoria noted the importance of continuing to build expertise and evidence in cord blood use due to its potential to meet unmet need, and welcomed Rob's work in this regard.
- Keith asked whether there is an established mechanism to monitor which patients are being offered access to trials and whether or not (and why) they decide to proceed, to help tack progress?
- Victoria said we do not have a robust way to measure that at present and agreed that it should be an area to pursue within IMPACT.

4.5. A strong and sustainable world class infrastructure - James Griffin & Victoria Potter

- James provided an update on key priority areas:
- Building apheresis capacity is a significant priority now both for NHS England and the DHSC. There are multiple issues at play, including physical space, nurse capacity, the number of apheresis machines, medical capacity to clear donors, stem cell laboratory capacity and others. Addressing the capacity bottleneck with require a broad set of actions. Daniel Eve reiterated that NHS England recognise the need for more apheresis capacity and recognise that work is required across various strands. NHS England have convened a working group to begin addressing these issues.
- The BSBMTCT workforce survey data has been collected and analysis is expected within the next few months.
- Various educational initiatives are progressing, including an NHBT virtual reality training platform which and collaborative projects with the ATTCs and Catapult on advanced therapies. Charles reminded everyone of the importance of mentorship opportunities for early career researchers and clinicians.

4.6. Community Advisory Group update – Alexandra Ross

- The secretariat have been recruiting to the Community Advisory Group, the opportunity to join was promoted via the Anthony Nolan Patients and Families Panel and expressions of interest have been received by approximately 15 people to date. The secretariat are speaking with everyone individually and will then formally recruit and convene an opportunity for group members to be introduced to the Delivery Group chairs.
- The secretariat are mindful of the need for the group to be as diverse as possible so will undertake further recruitment and promotion activities as required.

5. Discussion: Proposal from the NBTA - Peter McCleave and Orin Lewis

- Peter thanked the Oversight Committee for the invite and reminded the Committee that the NBTA have sent a proposal in writing for the recruitment of minority ethnic donors to the UK registry.
- Peter noted that NBTA are frustrated with the inequalities in access to stem cell transplant and would like the Forum to consider three aspects of their proposal:
    - For the aim for 20% aim of recruitment to be of people from a minority ethnic background to be changed to at least 50%
    - The need for systemic change in the current direction of travel to vastly improve the collection of data
    - Address the perceived siloed working of the individual aligned registry members
- Charles thanked Peter and noted that while the UKSCSF does not as a matter of course provide detailed written responses, by inviting Peter to join the meeting today and facilitating open discussion amongst the Oversight Committee members he hoped there would be assurance that the Forum intends to promote open and constructive discussion.
- Gail Miflin noted there is a unifying desire amongst the NBTA, UK aligned registry and UKSCSF to take action and reduce inequities on the basis of ethnicity. There is also clear alignment with the NBTA on the need for improved data on ethnicity across the system

and progress is being made to address this. All partners also agree on the need for a strong and ambitious target for the recruitment of minority ethnic donors. Gail stated that a multipronged approach is required to achieve our shared aims for equity. This includes ensuring that the UK is ready to adopt technology such as post-transplant cyclophosphamide. She concluded by suggesting that the UK aligned registry should respond to the detailed NBTA proposal, rather than the UKSCSF, to which Charles agreed.

- Orin agreed that there have been positive changes in terms of the consistency of ethnicity data, but the NBTA would like to see a re-prioritisation of recruitment spend to focus on donors from a minority ethnic background. Orin and Peter also noted that in their view the actions being taken by the community are too skewed towards patients in the future rather than trying to address the immediate unmet need.
- James noted that there is increasing diversity within the population in terms of HLA and even if we had 100% of people from a minority ethnic background on the register we wouldn’t be able to find a full match for every patient. However changes in technology are anticipated to remove the need for a very closely matched donor relatively quickly. It will be very important to generate the UK evidence base to support adoption of these types of treatment as soon as possible.
- Victoria agreed that a multipronged approach is important. She added that PTCy is already being used in clinical practice in the US and to some extent in the UK too. She asked Orin and Peter to elaborate on what strategies they suggest should be used to increase the diversity of the registry, noting that the important thing is to ensure that recruitment methodology is as effective as possible to recruit more people from a minority ethnic background.
- In response, Peter said that one area that the NBTA would suggest changing is the age sign-up criteria which differ for each aligned registry member, he suggested there is a need for more alignment to simplify recruitment messaging.
- Keith agreed with Peter and Orin that a higher target for recruitment, whether it is likely to be met or not, may drive ambition. He also agreed with the need for a multi-pronged approach to meet the needs of patients from a minority ethnic background, including developing existing cell sources but also alternative therapies that do not rely on a transplant at all.
- Charles concluded by noting that further discussion of the NBTA’s recommendations will be picked up by the UK aligned registry, who will be asked to report to the UKSCSF on progress. He reiterated the willingness of the UKSCSF to continue collaborating with the NBTA, in particularly on the shared ambitions for data.

6. EBMT Congress 2024 – Dr Victoria Potter

- Victoria noted that EBMT, to be held in April, will provide a forum for further discussion on many of the developments discussed today.

7. Meeting close

- Charles closed the meeting by reiterating the shared ambition of the Forum and others to tackle unacceptable inequality in access and outcomes and to make the UK a global leader in transplant and advanced cell therapies. The next meeting of the UKSCSF Oversight Committee will take place in approximately 6 months.