APPG on Stem Cell Transplantation meeting, Tuesday 26th November 2013, 1-2.30pm

Attendees

David Burrowes MP
Baroness Masham of Ilton
Lord Alton of Liverpool
Paul Goggins MP

Dr Lorna Williamson, NHS Blood and Transplant
Guy Parkes, NHS Blood and Transplant
Dr Jon Smythe, NHS Blood and Transplant
Dr Joanna Tilley, Biovault Technical
Heather O'Shea, NHS Blood and Transplant
Vicky Griffin, NHS Blood and Transplant
Dr Pip Patrick, CR UK and UCL Cancer Trials Centre
Rebecca Roberts, Cells4Life
Mil Vukovic, Leukaemia & Lymphoma Research
Orin Lewis, ACLT
Patrick Ojeer, Sickle Cell Society
David Fanthorpe, Future Health Biobank
Professor Chris Bunce, Leukaemia & Lymphoma Research
Professor Charles Craddock, Stem Cell Strategic Oversight Committee
Richard Davidson, Anthony Nolan
Katie Begg, Anthony Nolan
Victoria Moffett, Anthony Nolan
Iana Vidal, Anthony Nolan

David Burrowes opened the meeting, thanking all guests for attending and the speakers for offering to present to the group on the timely topic of clinical trials in bone marrow transplantation.

AGM

Mr Burrowes named the current officers of the group as follows:

David Burrowes MP, Co-Chair
Mark Tami MP, Co-Chair
Fiona Bruce MP, Vice Chair
Baroness Masham of Ilton, Vice Chair
Jim Dobbin, Vice Chair
Chris Ruane, Secretary

Paul Goggins moved that these officers remain the same for the forthcoming year. Baroness Masham and Lord Alton seconded this motion, and this was agreed.

Lord Alton also expressed his thanks to the co-chairs of the group for all their work promoting the interests of the APPG on Stem Cell Transplantation over the last year, and hoped that other members would continue to engage with the important issues raised.

Mr Burrowes also expressed thanks to the various speakers and stakeholders who have informed the meetings over the past year, and Anthony Nolan for administrating the group.

Main discussion – Establishing a clinical trials network in bone marrow transplantation

Professor Charles Craddock opened the discussion. He thanked the APPG for their continued support of patients, and the wider bone marrow transplant community in Parliament, and hoped that they would be able to support the recent progress he has made in building a consensus proposal on establishing a clinical trials network.

He gave a short presentation (attached and available on the website at www.ukstemcell.org) – summary below:

- Although medical knowledge and a larger pool of donors has vastly improved stem cell transplantation, roughly half of patients are still destined to die or experience significant morbidity.
- Clinical trials will improve prospects for patients and also sits well with the Government's current strategy to develop the life sciences industry in the UK.
- Based on the success of the Trials Acceleration Programme (see below), Professor Craddock outlined proposals to establish a clinical trials network for stem cell transplantation. This would be coordinated by a central hub, with 8-10 major centres having additional research nurse resource. The trials would be open to all transplant centres.

Baroness Masham asked if the trials would take place in different parts of the country. Professor Craddock explained that the trials are open to all transplant centres and patients in the UK. The network would be coordinated by a central hub which would ease the regulatory and administrative burden from participating transplant centres; lack of resource to manage these areas is why research and data collection is currently so difficult. In addition, in order to 'kickstart' the programme the network would fund 8-10 research nurses in major centres. They would serve the dual purpose of handling the research so that transplant centres do not use existing resource but also provide additional support for patients and colleagues working in transplantation.

Mr Burrowes asked for clarification on timescales and funding estimates. Professor Craddock stated that it is estimated that the network will need £3.4m funding over four years. A coalition of partners is working on a business case at present. It is important to note that the clinical and research benefits will have implications for improvements in medicine beyond bone marrow transplantation, which is why the coalition is also in discussions with the NIHR Office for Clinical Research Infrastructure (NOCRI) to consider funding from this body.

Mr Burrowes suggested that it might be beneficial to the proposals if the APPG write to the relevant parties or bodies to emphasise the importance of this project and highlight how it might fit in the translational medicine budget. Professor Craddock thanked the APPG for the offer and suggested that a letter to Dame Sally Davies, Chief Medical Officer, and to the NIHR would be beneficial.

Guy Parkes asked if there would be involvement from the British Society of Blood and Marrow Transplantation (BSBMT). Professor Craddock said that the BSBMT Clinical Trials Committee would lead on the governance of the network, with input and support from other partners.

Professor Chris Bunce briefly gave an overview of Leukaemia & Lymphoma Research (LLR) (attached and available on the website at www.ukstemcell.org). He

explained the purpose of the Trials Acceleration Programme (TAP), how it had been established and how it works at present.

Mr Burrowes raised the issue of potential partnerships, perhaps widening the pool of partners to reflect the TAP structure's potential to help improve outcomes for patients in other clinical areas. Professor Bunce replied that there is a strong tradition of collegiate working amongst the haematology community in the UK which is why the TAP has worked so well and a similar programme for stem cell transplantation would also be successful. The TAP structure can be replicated across other disease areas, and can work in the UK, but also within the EU if a wider patient group is needed, particularly for rarer conditions. Thinking ahead, it also has the potential to inform stratified medicine.

Professor Craddock also highlighted the attractive nature of the programme to the development of new drugs. Pharmaceutical companies want to invest in drugs that work well and innovate, and by improving patient access to these drugs through rapid or early assessment, these companies are able to invest in drugs that work within a reasonable timeframe. The aims and objectives behind the TAP and the proposals for a similar network in transplantation resonate across the whole of medicine, as new, innovative therapies are becoming available quicker than ever.

Mr Burrowes suggested a conference or meeting, hosted by the APPG, to identify wider partners for these initiatives such as government bodies and other medical charities. He asked what other potential partners there are, apart from the ones already mentioned.

Professor Bunce said that Cancer Research UK and Myeloma UK are the other obvious partner organisations, however both have their own initiatives to support and fund. Professor Craddock said it is vital that the Medical Research Council (MRC) is involved to bridge the gap between science and clinical application. He also suggested colleagues in the Haematology department at the Weatherall Institute of Molecular Medicine. The NIHR would need to be involved to help develop the strategic proposals.

Professor Craddock also highlighted the need to involve patients, raising awareness about the importance of clinical trials. Katie Begg said that Anthony Nolan has a Patient Experience team who have regular contact with patients so the communication channel is there if needed. Professor Bunce also said that it would be an opportunity to get smaller patient groups on board. LLR also have their own case studies and contact points with patients that might be useful in this context.

Dr Lorna Williamson said that NHS Blood and Transplant (NHSBT) are very supportive of the initiative. They receive NIHR funding for some of the work they do in their labs. This is up for renewal shortly so there is potential to share the resources they currently have with this programme. This will also help to develop a smooth transition from research to clinical practice. Historically NHSBT has provided a similar offering for transfusion trials so would be best placed to provide this resource. Dr Jon Smythe also noted that they meet all the regulatory requirements for these types of trials, having supported a few smaller ones in the past.

Mr Burrowes asked if the speakers had concerns about the amount of attention given to this area by government bodies and other relevant parties. He wanted to know if there is a 'level playing field' in medical research funding or if research into bone marrow transplantation is often overlooked.

Professor Craddock noted that clinical trials as a tool for improving treatment and patient outcomes has only developed considerably over the last 15-20 years so the area is still relatively new, as is the funding. There is now a need to empower patients with the knowledge they need to demand greater investment into clinical trials.

Professor Bunce said that personally he felt that there are assumptions that might impact on the level of funding research into bone marrow transplantation attracts. This is because many don't understand that although the procedure can be curative, patients can still die or end up living with debilitating conditions, which is why clinicians are eager to pursue research into potential improvements to the process.

Orin Lewis said that from the patient/family perspective, having experienced a successful transplant with devastating late effects, it is good to hear that attention is being drawn to what happens beyond the transplant. The importance of this approach needs to be raised with decision-makers so they understand that there is still work to be done in improving survivorship.

Patrick Ojeer also highlighted the use of stem cell transplantation to treat sickle cell patients and said that new research had the potential to be beneficial to these recipients too.

Mr Burrowes agreed that with this sentiment and said he was keen to have some positive outcomes from the discussion. He suggested a future meeting with representatives from the MRC to hear their thoughts on the issue.

Baroness Masham asked Professor Bunce to clarify that there were no major TAP centres north of Leeds. Professor Bunce said that this was correct but that all other transplant centres in the UK were free to recruit to the trials. There are plans to expand, on the condition that partnership funding is secured, and this opens up the potential for new 'major' centres. There is also the option of tapping into existing networks to share resource and infrastructure.

Baroness Masham asked if the programme has implications for travel for patients. Professor Bunce said that the nature of trial means that patients can participate through their local transplant centre, and around their normal treatment patterns so there is no additional travel commitment involved.

Dr Pip Patrick said that her institution, the Cancer Research UK and UCL Cancer Trials Centre, also runs a few trials focused on widening access to bone marrow transplantation. The centre has had difficulties keeping these trials running and out of eight, two have closed due to poor recruitment. She urged clinicians to keep collaborating with her centre and others, and also said that the BSBMT had an important role to play in coordinating the questions that need to be answered by research.

Professor Bunce said that it would also be useful to get clinical studies groups (CSGs) on board and this could help to make sure that centres recruit enough patients.

Baroness Masham asked what age range these trials would be aimed at. Professor Bunce clarified that the participants would usually be adults or older adults. Children and young people are usually treated in a large phase III trial that is currently in operation. Professor Craddock emphasised that stem cell transplantation and treatment is dependent on biological age, not chronological age; having monitored

outcomes over a number of years, clinicians have established that older patients have the same outcomes as young patients.

Mr Burrowes concluded the meeting by summing up the discussion and reiterating that the APPG would be keen to help secure support from key decision-makers as well as promote wider collaboration with potential partners.

Mr Burrowes asked if there was any other business, and confirmed future topics for discussion in the new year as follows:

- Survivorship
- Wider applications for stem cell therapies and research (joint meeting with the APPG for Medical Research)

Mr Burrowes thanked the members, speakers and stakeholders for attending and closed the meeting.