All-Party Parliamentary Group on Stem Cell Transplantation
All-Party Parliamentary Group on Medical Research

NOTE OF MEETING

Harnessing stem cells for patients and economic growth

Dining Room A, House of Commons

On Tuesday 15th July 2014, the APPG on Stem Cell Transplantation and the APPG on Medical Research hosted a joint roundtable lunch meeting titled '*Harnessing stem cells for patients and economic growth*.' The meeting brought together scientists, clinicians, expert stakeholders, government officials, MPs and Peers to discuss the potential of, and barriers to, the future of innovative new stem cell therapies and the growth of the UK regenerative medicine industry.

The meeting was hosted by David Burrowes MP, Co-Chair of the APPG on Stem Cell Transplantation, and Chaired by Lord Leslie Turnberg, Chair of the APPG on Medical Research.

This note is a record of the meeting and does not necessarily represent the views of either the APPG Stem Cell Transplantation or the APPG on Medical Research, or any of the funding organisations.

Speakers:

Dr Roger Barker, Department of Clinical Neurosciences, Cambridge Centre for Brain Repair

Dr Barker spoke about his research into replacing damaged dopamine cells in the brains of people with Parkinson's disease with dopamine cells derived from embryonic stem cells. He said that a clinical trial for the therapy was possible in the next five years.

However, he has found that varying interpretations of EU directives and regulation in different European countries has meant that he is restricted in efficiently running out such trials in a number of countries. Also, due to the pioneering nature of the research, he has been delayed as regulators scope out new regulations for the novel production and delivery techniques of treatments. He also noted that there has previously been far too much haste in progressing through the clinical trial phases, without proper assessment of pre-clinical data. This has led to premature and negative results in previous trials that have held back the field.

Dr Aurore Saudemont, Senior Immunotherapy Researcher, Anthony Nolan Research Institute

Dr Saudemont spoke about her research, which explores the potential of umbilical cord blood stem cells to counteract the potential immunological complications a patient may face following a stem cell transplant. She was unable to develop UK-only clinical trials due to the small size of the UK patient population so explored the potential at a European level, only to be faced with similar barriers interpreted in varying ways across a number of European countries. She also highlighted the innovative nature of immunotherapy research, which in itself was a barrier when trying to gain approval for clinical trials, and the difficulties faced when trying to translate research from bench to bedside.

Dr Rob Buckle, Director, UK Regenerative Medicine Platform

Dr Buckle gave a strategic overview of the current stem cell research environment in the UK, highlighting the fact that there are few licensed regenerative medicine therapies ready for the clinic. He pointed out that cells also offer exciting possibilities as delivery vehicles for other therapies and in the production of biological drugs, as well as being therapies in themselves.

The UK is very strong in the field of stem cell research and regenerative medicine and progress in this fast-moving field is very positive, he said. However, while public and charity funders have invested heavily in early-stage research, investment by industry is moderate and largely SME-based, reflecting uncertainty over the best approach to develop, validate and market regenerative therapeutics. The major challenge remains in translating the basic science into the clinic. We are still at least 10-15 years off routine clinical use of stem cells, he believed. He highlighted the work of the Technology Strategy Board, the Medical Research Council and the UK Regenerative Medicine Platform (UKRMP) in addressing key funding, scientific and regulatory barriers. Key challenges that the UKRMP is addressing include:

- How to control cell production and differentiation, and manufacture at scale
- Modelling realistic human in-vivo environments for testing cell therapies
- Tracking cell therapies once they have entered the body for safety evaluation
- Cell therapy delivery and targeting
- Modulating immune responses post-therapy delivery.

Emyr Harries, Scientific Policy Manager – Cell Therapy and Regenerative Medicine, Department of Health

Emyr spoke about current Department of Health (DH) initiatives in place to foster innovation in stem cell transplantation and research. He explained the work of the Regenerative Medicine Expert Group, which was established following the Government response to the House of Lords Science and Technology Committee Inquiry into Regenerative Medicine.[1] The aim of the expert group is to bring together all stakeholders, including academia, industry, regulators, clinicians, NHS England, patient representatives, the UK Blood Services and NICE, to develop a Strategy so that the NHS is ready and able to deliver regenerative medicine treatments. He confirmed that they are due to provide their Strategy to the Secretary of State for Health by the end of 2014. He also drew attention to DH's stem cell transplantation improvement programme, in collaboration with Anthony Nolan and NHS Blood and Transplant, which has received £16 million additional funding over the last four years to improve stem cell services in the UK. The Stem Cell Oversight Committee has been tasked with planning for the next phase of the programme, which will begin in 2015.

Keith Thompson, Chief Executive, Cell Therapy Catapult

Keith explained the role of the Cell Therapy Catapult (CTC) in translational science as part of a wider national programme to bridge the gap between UK science and commercialisation. The CTC works to address business, clinical, regulatory, and manufacturing and supply barriers to promote investment in products and boost confidence in the UK as a secure ecosystem for the development of cell therapy products. He stressed the need to present clinical data and case studies to potential investors in the UK and internationally to exemplify the strength of the UK industry and to encourage investment from private industry to grow the industrial base and capture long term economic benefits.

[1] http://www.publications.parliament.uk/pa/ld201314/ldselect/ldsctech/23/23.pdf

He noted that a number of large global pharmaceutical companies, including Novartis, have recently made investments in the field following promising clinical trials in the US.

Discussion:

A number of key issues were addressed during the discussion between the speakers and the roundtable:

Collaboration between scientists and regulators

- Frank and open discussion between scientists and regulators is needed at the early stages of research to clarify potential regulatory problems and barriers.
- Organisations such as the CTC help steer and advise scientists through the regulatory process. An exemplar framework is being developed in collaboration with DH to demonstrate the aspects needed from a regulatory standpoint when conducting research.
- Initiatives such as the MHRA Innovation Office are a crucial way for researchers to engage with regulators at all stages of the research and trial process, and shows a willingness from key regulators to engage in open and honest discussion.
- It was recognised that the HTA, HRA, HFEA and the MHRA Innovation Office are working together to respond to the House of Lords Science and Technology Report to develop a 'one-stop shop' for scientists to engage with regulators about plans and research processes.

Collaboration between scientists and clinicians

- It is crucial that early conversations take place between all relevant groups from scientific and clinical backgrounds to discuss the science, the aims and the clinical applications of the research, as well as how practically to translate that research into clinical practice.
- Clinical trials need to inform the basic research in a feedback loop so that information gained will benefit the research process.

Stimulating investment

- Investment follows good data and, whilst the UK is benefitting from some early stage investment, there is a need for researchers and companies to demonstrate their long-term sustainability. Securing patent protection for cell therapies can be difficult and act as a barrier to attracting investment.
- Organisations such as the CTC are working with companies and products to help them become attractive for investment.
- We need to showcase the successful translation of stem cell therapies to clinic in order to build confidence and investment in the UK industry. Reducing the costs associated with this process is a key way of achieving success, and can be achieved by building evaluative processes into phase two and three clinical trials prior to the NICE evaluation process.
- It was recognised that investment often comes once a cell based product is in the market and therefore focus and investment is needed in the commercialisation of innovative new therapies. It is also crucial that requirements for market access and aspects of health economics are built into commercialisation plans at an early stage.

Animal studies

- Animal models pose particular challenges for stem cell research. In some cases there will not be a suitable animal model for the transplantation of human cells, although it is possible to design specific animal models with humanised tissue using sophisticated techniques. Researchers, including those working in industry, are increasingly looking for alternatives to animals.
- Different European regulators have differing views on the use and benefits of animal studies.UK regulators judge cases on a case-by-case basis, assessing the necessity for evidence derived from animal studies based on scientific value. This pragmatic approach is more attractive to foreign investors.

Clinical Trials

- It was noted that developments in recent years have meant that many regulators are developing far more rapid approval processes for innovative techniques and processes. This was welcomed.
- Concern was raised however that as processes become more streamlined there is an increased risk of rushing to clinical trial and, similarly, a risk of premature conclusions being drawn from short clinical trials, or early findings. It is important to bear in mind that some cell therapies can act very quickly inside the body whereas others may take years to prove effective. Issues arising from premature trials have affected investment in the past and therefore it is crucial that these mistakes are not repeated.

The meeting was closed by Mark Tami MP, Co-Chair of the APPG on Stem Cell Transplantation.

Attendees:

Dr Roger Barker	Department of Clinical Neurosciences	Cambridge Centre for Brain Repair
Katie Begg	Head of Policy and Public Affairs	Anthony Nolan
Dr Helen Bodmer	Head, MRC, BBSRC and Health Research Team	Research Councils Unit, Department for Business, Innovation and Skills
Dr Catherine Booth	Founder & Managing Director	Epistem Ltd
Dr Laura Boothman	Policy Manager	Arthritis Research UK
Sophie Broster-James	Public Affairs and Stakeholder Engagement Manager	Medical Research Council
Dr Rob Buckle	Director	UK Regenerative Medicine Platform
Jane Bunce	Senior Public Affairs and Policy Officer	Medical Research Council
David Burrowes MP	Co-Chair	APPG on Stem Cell Transplantation
Dr Alison Cave	Head of Cellular, Developmental & Physiological Sciences	Wellcome Trust
Simon Butler	Policy and Public Affairs Manager	Anthony Nolan
Dr Hollie Chandler	Policy Advisor	Cancer Research UK
Dr Emily Colme-Seymour	Director	London Regenerative Medicine Network
Lord Davis of Coity		House of Lords
Matthew Durdy	Chief Business Officer	Cell Therapy Catapult
Timothy Fox	Commercial Director	Anthony Nolan
Dr Zoë Freeman	Public Affairs and Communications Executive	BioIndustry Association
Dr Michaela Frye	Epithelial Stem Cell Homeostasis and Cancer Laboratory	Cambridge Stem Cell Institute
Baroness Gardner of Parkes		House of Lords
Rebecca Gladstone	Policy and Public Affairs Officer	Anthony Nolan
Professor Sian Harding	Director	British Heart Foundation Cardiovascular Regenerative Medicine Centre, Imperial College
Emyr Harries	Scientific Policy Manager – Cell Therapy and Regenerative Medicine	Health Science and Bioethics Division, Department of Health
Baroness Howe of Idlicote CBE		House of Lords
Lord Kakkar		House of Lords

Professor Cay Kielty	Director	EPSRC & MRC Centre for Doctoral Training in Regenerative Medicine
Professor Sue Kimber	Co-Director Professor of Stem Cells and Development	North West Embryonic Stem Cell Centre, University of Manchester
Dr Robin Lovell-Badge	Head of Stem Cell Biology and Developmental Genetics	MRC National Institute for Medical Research
Rachael Mann	Policy and Public Affairs Officer	Association of Medical Research Charities
Baroness Masham of Ilton		House of Lords
Baroness Manzoor		House of Lords
Professor Andrew McCaskie	Director	Arthritis Research UK Tissue Engineering centre
Dr Erik Miljan	Director	Simply Cells Ltd
Caterina Minelli	Secondee , Science and Innovation Strategy Team	Department for Business, Innovation and Skills
Dr Natalie Mount	Chief Clinical Officer	Cell Therapy Catapult
Baroness O'Neill of Bengarve		House of Lords
Lord Patel		House of Lords
Ben Ridley-Johnson	Policy Intern	Wellcome Trust
Professor Sally Roberts	Director of Spinal Research	Arthritis Research UK Tissue Engineering centre
Dr Aurore Saudemont	Senior Immunotherapy Researcher	Anthony Nolan
Dr Jill Shepherd	Regulation Manager	Human Tissue Authority
Michael Sullivan	Lead Technologist - Regenerative Medicine and Cell Therapy	Technology Strategy Board
Mark Tami MP	Co-Chair	APPG on Stem Cell Transplantation
Keith Thompson	Chief Executive	Cell Therapy Catapult
Lord Turnberg	Chair	APPG on Medical Research
Dr Martin Turner	Senior Policy Advisor	Association of Medical Research Charities
Dr Naho Yamazaki	Head of Policy	The Academy of Medical Sciences