RESEARCH COUNCIL / TECHNOLOGY STRATEGY BOARD RESPONSE TO THE REPORT FROM THE HOUSE OF LORDS SCIENCE AND TECHNOLOGY COMMITTEE INQUIRY INTO REGENERATIVE MEDICINE

INTRODUCTION

1. This response is submitted by RCUK (on behalf of the Research Councils listed below) together with Technology Strategy Board, and represents their independent views. It does not include or necessarily reflect the views of the Department for Business, Innovation and Skills (BIS). The submission is made on behalf of the following Councils:
   - Biotechnology and Biological Sciences Research Council (BBSRC)
   - Economic and Social Sciences Research Council (ESRC)
   - Engineering and Physical Sciences Research Council (EPSRC)
   - Medical Research Council (MRC)
   and the
   - Technology Strategy Board (TSB)

2. This response focuses on those recommendations from the Report that are directly relevant to the missions of individual Councils identified above and TSB, who have jointly contributed to this response.

SUMMARY

The Research Councils (RC) and Technology Strategy Board (TSB) welcome the Committee’s report, which clearly acknowledges the potential for regenerative medicine to deliver enormous health and economic benefits for the UK. It also rightly highlights the challenges ahead in moving this emerging knowledge towards clinical application and commercialisation, and ensuring the UK remains globally competitive.

The Report recognises the strength of the UK research base and infrastructure that has been assembled through RC and TSB investment in recent years, and the on-going implementation of the 2012 RC/TSB Strategy for UK Regenerative Medicine is adding further cohesion and momentum towards the goal of achieving impact in this area. These actions are already addressing a number of the areas highlighted in the Committee’s Report, through supporting interdisciplinary research that will promote therapeutic development and the future growth of manufacturing capability in line with the emerging needs of the field.

The following section highlights how the RCs/TSB will respond to the recommendations of the Committee that are within our immediate jurisdiction. More broadly the RCs/TSB will seek to coordinate these activities with the responses of the Government and other key stakeholders where possible, and continue to work closely with all relevant sectors to promote the advancement of regenerative medicine in the UK.

RESPONSE

Recommendation 1 - As a matter of urgency, the HRA establish a regulatory advice service. This would build on the expertise of the Office for Life Science toolkit, the newly established MHRA Innovation Office and the experience of regulators. Researchers and companies require more than a web-based service. They should be
assigned a single point of contact to support them in navigating the regulatory system, directing their queries to others where appropriate, but retaining ownership and oversight of the advice process. Such a service would be of short-term value to this (and the broad healthcare) sector until such a time as the regulatory environment is rationalised (paragraph 71).

The RC/TSB agree that action is required to both improve perceptions of the regulatory system and to streamline it, and will continue to work alongside the regulatory agencies in delivering this. The MRC, ESRC and other stakeholders have already begun to address how the research community is supported in this area; for example a workshop was held at MHRA in October 2012 to highlight the key areas of scientific and regulatory uncertainty with a view to informing an update of the MRC/DH Stem Cell Tool Kit and the publication of a series of case studies. It is anticipated that these will be delivered in the coming months, although the revised Tool Kit will need to take account of the response of the HRA to this recommendation. Furthermore a follow-up international workshop is scheduled for September this year in Washington DC (co-sponsored by the MRC, ESRC and a number of North American agencies, and including the participation of MHRA and FDA) to evaluate trans-Atlantic comparators in regulatory oversight with a view to promoting efficiency and best-practice; again, outputs will be published later this year.

Given the combined efforts currently being undertaken to coordinate regulatory advice between the various agencies, which in this area covers a spectrum of approaches and stakeholders, it is considered that a regulatory advisory service providing a single point of contact across all regulators is not warranted at this point.

**Recommendation 5 – The phase II disease teams of the TSB regenerative medicine platform, and other regenerative medicine funding programmes, specifically require researchers to involve manufacturing and scale-up experts in their development process to ensure that translational work is scalable and therefore deliverable to a large number of patients (where the disease area requires this) (paragraph 98).**

This recommendation appears to conflate the TSB regenerative medicine programme, and the RC-led UK Regenerative Medicine Platform – their respective goals are clarified below:

**UK Regenerative Medicine Platform (UKRMP):** This is a £25 million cross Research Council initiative (BBSRC, EPSRC and MRC) whose key goal is to address the technical and scientific challenges associated with translating promising scientific discoveries in this area towards clinical impact i.e. connecting research to application. The role for scale-up and manufacturing in addressing this goal is well-recognised and embedded within the foundation of the UKRMP.

The UKRMP is being delivered in two stages. Stage I constitutes the establishment of up to five interdisciplinary Research Hubs. Cell behaviour, differentiation and manufacturing (scale-up) is a theme for one of these Hubs and an award has recently made to establish this activity (to be publicly announced in September). Stage II of the UKRMP, to be delivered by March 2014, will fund a small number of cutting-edge research consortia (or ‘disease teams’) to undertake multidisciplinary translational programmes in regenerative medicine in target areas amenable to clinical intervention. The call was published in July and highlights the connection between manufacturing and delivery of clinical products. UKRMP II awards will be made in March 2014 and will build upon the Stage I investments in the established Research Hubs that offer capabilities in this area.

The **TSB Collaborative Research & Development competitions** supporting Regenerative Medicine and Cell Therapy business challenges encourage the formation of consortia that
gather relevant expertise to deliver stated project aims and goals. Moreover, each project proposal is appraised, in part, on the quality of the team being formed and their alignment with the aims and goals of the individual project and their ability to deliver these. The TSB will continue to encourage projects to gather relevant expertise in scale-up and manufacturing where appropriate.

Recommendation 6 – The TSB and EPSRC undertake an annual stock-take of regenerative medicine manufacturing capacity and make recommendations to BIS about future needs, with the first survey informing the Government’s review of infrastructure investment. The Cell Therapy Catapult has begun work on such a survey so we recommend that this work is taken as a starting point. BIS must then act to ensure that appropriate infrastructure investment is made to support the field. At the very least, investment should be made in facilities to support the scale-up of treatments in mid to late stage clinical development. Money for this, and other recommendations, should be found by the re-prioritisation of budgets and innovative funding methods (paragraph 100).

The Cell Therapy Catapult has recently completed its survey of regenerative medicine manufacturing capacity in the UK, and the RCs/TSB agree that an appraisal of national capability undertaken on an annual basis would be of value in keeping abreast of the evolving needs of the area in order to ensure that the UK remains globally competitive. The Cell Therapy Catapult is best placed to maintain responsibility for the stock-take in the future, and the TSB and the EPSRC will work closely with the Catapult to progress this activity.

Recommendation 11 – The TSB and Cell Therapy Catapult prioritise its activities to enable the Cell Therapy Catapult to focus on taking high growth potential projects through clinical trial to be phase III trial ready and developing links with the regenerative medicine community (paragraph 124).

The Cell Therapy Catapult receives annual core funding from the TSB, but is independent in its operation. The Cell Therapy Catapult five year business plan was recently approved by the Board of the TSB and lists “Taking high growth potential projects through clinical trial to be phase III trial ready and developing links with the regenerative medicine community” as key activities.

In terms of RC support for therapeutic development and the clinical testing, the MRC provides a number of funding opportunities for translational schemes including the Regenerative Medicine Research Committee and the Biomedical Catalyst (the latter in partnership with TSB). Both of these foster relationships with regenerative medicine community and provide a pipeline into the Cell Therapy Catapult/TSB. Grants will be competitively available to provide seamless support for both academic- and business-led research and development projects, with the aim of developing innovative solutions to healthcare challenges and supporting the maturation of ideas from concept to commercialisation. Finally, as mentioned above, Stage II of the UKRMP focusses on connecting research to application into early stage clinical trials.

Recommendation 12 - Given the large number of potential funders, the TSB, research councils and NIHR should produce an online funding guide, regularly updated, to help researchers and SMEs know where they should apply at each stage of research and development in regenerative medicine (paragraph 125).
This recommendation is welcomed and will be addressed by provision of a single funding portal on the UKRMP website. This will be established by the end of the year and will link to TSB Knowledge Transfer Networks (KTNs) and provide a single information point spanning Research Council, TSB, NIHR and research charity funding opportunities in this area.

**Recommendation 13 - The ESRC and the TSB commission an evaluation of innovative funding models, which spread risk and most likely will contain a degree of government matched funding or be underpinned by government guarantees, and recommend how additional funding could be provided for late stage clinical development in this field. The Government have said that this field has enormous potential and that they will support it. They must “put their money where their mouth is”; BIS and Her Majesty’s Treasury must adopt the policy recommendation of the ESRC and TSB study (paragraph 127).**

This recommendation is welcomed. ESRC and TSB work closely in a number of areas addressing innovation research and entrepreneurship, and are now actively developing a strand of activity to assess innovative support structures in other countries and establish how best to bring the lessons that can be learned into the UK system in a way that most effectively complements the existing UK funding landscape.

**Recommendation 15 - Concern over the cost of patenting, the sufficiency of support available for innovators and questions about the ability of universities to recognise the potential in regenerative medicine patents lead us to conclude that the TSB should set-up a time-limited support fund for regenerative medicine patents. This fund should be open to university researchers who wish to pursue patents beyond the first stage, so that potential income from regenerative medicine products is not lost. Such a fund would help foster this fledgling industry and be a helpful tool until university patent offices are better placed to deal with the potential value of these products (paragraph 137).**

The TSB currently offers funding that can include the costs of patenting. For example, the SMART scheme provides matched funding for small and medium sized businesses, including pre-start-ups and start-ups, which can be used to establish IP position and to protect IP. Likewise, the TSB’s national Innovation Voucher scheme is designed to help businesses work with external experts that can include Intellectual Property advisers. Furthermore, Projects awarded funding through TSB’s Collaborative R&D competitions can claim for costs associated with new patents generated within the project.

Funding to support existing university patents lies outside the TSB remit. However, TSB is currently discussing with the IPO the value of gathering information around the costs of patenting within the regenerative medicine field and the value of doing some research looking at possible regional patenting strategies that if carried out could be shared with university patent offices.

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*Research Councils UK and TSB, 2nd September 2013*