Innovate UK

The Innovate UK response to the Innovative Medicines Initiative consultation

Facilitating the translation of advanced therapies to patients in Europe

Innovate UK and ATMP background

1. Innovate UK is the UK's innovation agency, a non-departmental public body sponsored by the UK Government Department of Business, Innovation and Skills. It is the prime channel through which the UK Government incentivises innovation in business. Innovate UK is business-led. Our governing board and executive team is comprised of experienced business innovators and experts. We work with people, companies and partner organisations to find and drive the science and technology innovations that will increase productivity and exports and grow the UK economy.

2. We are working to:
    - accelerate UK economic growth by nurturing small high-growth potential firms in key market sectors, helping them to become high-growth mid-sized companies with strong productivity and export success;
    - build on innovation excellence throughout the UK, investing locally in areas of strength;
    - develop Catapult centres within a national innovation network, to provide access to cutting edge technologies, encourage inward investment and enable technical advances in existing businesses;
    - turn scientific excellence into economic impact and deliver results through innovation, in collaboration with the Research Community and Government; and,
    - evolve our funding models to explore ways to help public funding go further and work harder, while continuing to deliver impact from innovation.

3. In line with our strategy[1] we operate across Government and advise on policies which relate to technology, innovation and knowledge transfer. We also support UK Government departments to become more efficient by supporting them in developing innovative solutions through harnessing the creativity that businesses can offer.

4. Innovate UK was established in July 2007 (as the Technology Strategy Board). We have invested over £1.8 billion in innovation, and have helped more than 7,600 innovative companies in projects estimated to add up to £13.1 billion to the UK economy and created an average of 7 jobs per company we have worked with. Our investment over the last 8 years has meant that every £1 invested has returned up to £7.3 GVA to the economy and created 55,000 jobs. The private sector more than matches that investment, doubling the power of public sector money. We work with nearly every University in the UK to stimulate the commercialisation of leading-edge academic research and innovation.

[1] 'Concept to Commercialisation: A strategy for business innovation, 2011-2015'. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/360620/Concept_to_Commercialisation_-_A_Strategy_for_Business_Innovation_2011-2015.pdf

Innovate UK's Regenerative Medicine Programme

5. We have provided long term support to the area of regenerative medicine and advanced therapies for UK businesses. Since its formation in 2007, Innovate UK has supported work to address translation and commercialisation challenges in the area of advanced therapies and regenerative medicine.

6. In the period 2005 to 2009 Innovate UK (and its predecessor the Technology Programme in the DTI) had committed more than £20 million in cell therapy/regenerative medicine technologies, tissue engineering and cell bioprocessing. This covered a portfolio of some 30 projects involving 20 different companies.

7. In 2008, Innovate UK worked closely with the Bioindustry Association Regenerative Medicine Industry Group (now the Cell and Gene Therapy Advisory Committee) and other industry colleagues to build a Regenerative Medicine Programme, launched in 2009. This programme allowed Innovate UK to undertake a more strategic approach to supporting this nascent industrial sector. The aims of the programme were to ensure that UK businesses could achieve a commercially competitive edge with global impact.

8. Many of the challenges required engagement with the science and clinical communities to address fundamental questions associated with validation of the technology or to access new ideas. Therefore it was important to ensure that companies had the potential to identify and work with key academics and clinicians. Engagement with the Research Councils, particularly the Medical Research Council (MRC), the Biotechnology and Biological Sciences Research Council (BBSRC), the Engineering and Physical Sciences research Council (EPSRC), and the Economic and Social research Council (ESRC), and the Scottish Government, allowed the Regenerative Medicine Programme to develop as a partnership of UK funders working together to address the challenges.

9. Since 2009, through the Regenerative Medicine Programme, the Biomedical Catalyst[2], and a number of other funding streams, Innovate UK has supported a total of 126 advanced therapies and regenerative medicine projects with over £54 million in grants. These project grants have been matched with over £25 million additional funding from industry.

10. These projects have focused on the development and validation of therapeutic products towards clinical application, support for underpinning tools and technologies to evaluate toxicity/efficacy and assist manufacturing, and the evaluation of novel supply chains and business models. These investments will have allowed 11 therapies to advance into clinical trials for the first time, and have allowed companies to raise further investment as a direct consequence of the programme and establish overseas operations.

11. Through the early projects it became apparent that the companies faced many common challenges that would need to be addressed in order for businesses to exploit promising discoveries. Working with the MRC, the BBSRC, the EPSRC, and the ESRC, we explored these challenges further alongside the current research and development position in the joint Research Councils/Innovate UK 'A Strategy for UK Regenerative Medicine'[3]. As a response to these challenges, and to ensure that research in this area connected seamlessly from discovery science through to clinical and commercial application, BBSRC, EPSRC and MRC together established the UK Regenerative Medicine Platform (UKRMP).

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[2] http://www.mrc.ac.uk/funding/science-areas/translation/biomedical-catalyst/
[3] https://www.mrc.ac.uk/documents/pdf/a-strategy-for-uk-regenerative-medicine/

12. Innovate UK established the Cell and Gene Therapy Catapult in 2012 (funding of £70 million for 2012-17) to support the growth of this emerging industry sector and establish the UK as a global centre. The Cell and Gene Therapy Catapult is based at Guy's Hospital in London, a pioneering healthcare centre of excellence. The Catapult provides access to technical, regulatory, clinical and financial expertise and infrastructure, enhancing the UK's key strengths in this area, and enabling the UK to be a global leader in the development and rapid commercial exploitation of cell therapies. There are a number of indicators that suggest the UK is increasing its ability to harness the commercial potential of advanced therapies and regenerative medicine. The number of clinical trials of advanced therapies being carried out in the UK, as captured in the Cell and Gene Therapy. Likewise, the UK manufacturing capacity for gene and cell therapies is also increasing[4]. The number of new UK advanced therapy and regenerative medicine companies has been increasing and it has been noted that within the biopharmaceutical sector in the UK the advanced therapies area shows the highest increase in turnover and growth in employment[5]. In the UK in the last 2 years there has been growth in the commercial sector of regenerative medicine and advanced therapeutics with a number of new UK based pioneering cell and gene therapy companies being formed (e.g. Athena Vision, Autolus, Catapult Therapy TCR, Freeline Therapeutics, Islexa, NightstaRx, Orchard Therapeutics) to develop and commercialise advanced therapeutics. These new companies together with the more established UK companies previously supported by Innovate UK funding; Adaptimmune, Cell Medica, Oxford Biomedica, ReNeuron, and Tissue Regenix have secured publicly declared investments of nearly £400 million since 2014 from venture capital funds, institutional investors and equity placings.

13. The 2012-2013 House of Lords Science and Technology Committee inquiry into regenerative medicine recognised the strength of the UK research base and infrastructure supporting regenerative medicine and highlighted challenges for its commercialisation and clinical application if the UK is to build on its globally competitive position. It recommended that late stage manufacturing capacity should be strengthened within the UK to support the scale-up of treatments in mid to late stage clinical development[6].

14. In the 2014 budget the Chancellor of the Exchequer announced that the Cell and Gene Therapy Catapult, through Innovate UK, had been awarded £55m to create a UK Cell Therapy Manufacturing Centre. This Centre, based at Stevenage, is due to open in 2017 and will be run by the Cell and Gene Therapy Catapult, enabling large-scale manufacture of cell and gene therapies for late-stage clinical trials and commercial supply.

Innovate UK response to the concept paper on advanced therapy medicinal products

15. Innovate UK welcomes the opportunity to respond to the concept paper on advanced therapy medicinal products developed by IMI, in collaboration with key stakeholders. Innovate UK would welcome the opportunity to participate in the second workshop being organised by IMI in 2016. Set out below is our response to the four questions.

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[4] Cell and Gene Therapy GMP Manufacturing in the UK: Capability and Capacity Analysis March 2016. https://ct.catapult.org.uk/wp-content/uploads/2016/05/GMP-report-2016.pdf

[5] Strength and Opportunity 2015. The landscape of the medical technology and biopharmaceutical sectors in the UK. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/521268/BIS-16-237-strength-and-opportunity-2015-UK-medical-and-biopharmaceutical-landscape.pdf

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

16. **Have the key challenges that can be addressed through collaborative, public private initiatives been properly identified?** The IMI concept paper has identified the challenges that exist for the development of advanced therapy medicinal products. ATMPs represent a complex set of therapeutic products that will require specialist manufacture, storage and distribution to deliver the products to patients. Much work still has to be done to translate the potential of ATMP approaches to commercial products that can be used routinely and change the clinical pathway for patients, their disease management and health outcomes.

17. **Which of the proposed potential initiatives should be prioritised?** A number of initiatives have been outlined in the concept paper. However the consultation has asked for missing areas to be identified (see below). As such, full prioritisation is probably best reviewed following receipt of all the responses to the concept paper and all the suggestions for missing areas have been compiled. Of the current initiatives the following are high priority:

    - Industrialisation of manufacturing processes to improve reproducibility and lower the cost of goods
    - Training and development of skills (see below)
    - Development of standards
    - Revision of the use of hospital exemption to ensure it does not undermine the use of commercial products that have already received Marketing Authorisation
    - Increased viral vector production and development of next generation viral vectors

18. **Are any areas missing?** Advanced therapy medicinal products represent novel therapeutic modalities that are far more complex than small molecular weight pharmaceuticals and biopharmaceuticals. To-date only seven ATMPs have so far received European Marketing Authorisation and so it is very early days for ATMPs. While the underpinning science and the potential of these new therapeutic approaches are compelling to those skilled in the art, key areas to further develop would be skills, standards, patient engagement, and education of public, patients, and regulators. In addition the regulatory systems are adapting to the challenges that new therapeutic approaches generate. They will need to continue to evolve as more new therapy types and approaches, such as genome editing, are developed and advance through clinical trials, manufacturing and commercialisation. More fleet of foot adaption of the regulatory systems would help development of future innovative therapies. The ATMPs promise to be highly disruptive to the current clinical treatment setting and systems. Establishing the efficient and successful delivery and administration to patients may best be served through established specialist centres in the UK and may require the design and funding of new clinical centres to accommodate these new approaches.

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19. **What are the key European or national initiatives that IMI shall synergise with?** In the UK there are a number of initiatives and activities that offer potential synergies, these include:

    - The Regenerative Medicines Expert Group (RMEG) (https://www.gov.uk/government/publications/regenerative-medicine-a-uk-pathway)
    - The Advanced Therapies Manufacturing Ministerial Task Force https://connect.innovateuk.org/web/healthktn/article-view/-/blogs/advanced-therapies-and-medicinal-products-ministerial-taskforce-upda-1?_33_redirect=https%3A%2F%2Fconnect.innovateuk.org%2Fweb%2Fhealthktn%2Farticle-view%3Fp_p_id%3D33%26p_p_lifecycle%3D0%26p_p_state%3Dnormal%26p_p

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Key IMI projects with possible overlaps/interest are:

- ADAPT-SMART
- AETIONOMY
- CANCER-ID
- COMPACT
- EBiSC
- GETREAL
- Quic-Concept
- STEMBANCC