Government Response to the House of Commons Science and Technology Committee Inquiry into Regenerative Medicine

Presented to Parliament
by the Secretary of State for Health
by Command of Her Majesty

August 2017

Cm 9491

Government Response to the House of Commons Science and Technology Committee Inquiry into Regenerative Medicine

Presented to Parliament
by the Secretary of State for Health
by Command of Her Majesty

August 2017

Cm 9491

This publication is available at www.gov.uk/government/publications

Any enquiries regarding this publication should be sent to us at Regenerative Medicines Policy Team, Science Research and Evidence Directorate Room 132, Richmond House, 79 Whitehall, London SW1A 2NS

ISBN 978-1-5286-0006-4

CCS0817761364 08/17

Printed on paper containing 75% recycled fibre content minimum

Printed in the UK by the APS Group on behalf of the Controller of Her Majesty’s Stationery Office

Government Response Regenerative Medicine Inquiry

Introduction

This document sets out the Government's response to the House of Commons Science and Technology Committee report on Regenerative Medicine, chaired by Rt Hon Stephen Metcalfe MP. Detailed responses to each of the recommendations in the Committee's report can be found from page [3] onwards.

The Government continues to actively support the regenerative medicine sector. This includes therapies regulated as Advanced Therapy Medicinal Products (ATMPs) and also tissue- and cell-based therapies which lie outside the scope of ATMP regulation. The Government is committed to supporting the development of safe and effective new therapies, an environment that supports the provision of these therapies within the healthcare system and an Industrial Strategy that promotes the growth of the Life Sciences Sector. It is clear that the benefits that these therapies have long promised are now much closer to being realised with potentially transformative therapies in late-stage clinical trials.

The Government is pleased to see that the report notes the significant progress that has been made since the House of Lords Science and Technology Committee previously examined this topic in 2013. Research funders have worked to improve the coordination between the different funding agencies (see *A Strategy for UK Regenerative Medicine 2012*[1]). This strategy has demonstrated the benefits that have come from the development of a regenerative medicine network that has been able to support a critical mass of activity and therefore meets the needs of this dynamic and multidisciplinary scientific field[2].

An innovative feature of the support for the regenerative medicine sector has been the creation of the Cell and Gene Therapy Catapult (CGTC). The CGTC, like the network of other Catapults, represents a new element of Innovate UK's strategy to support the translation of innovative ideas into commercial products. Innovate UK has been testing the Catapult concept as a way to deliver a step change in the UK's ability to commercialise its research. The impact of the CGTG will be strengthened with the development of a dedicated Good Manufacturing Practice (GMP) manufacturing centre due to be completed in 2017. The centre will enable companies to manufacture products for clinical trials and early commercial launch. The centre will also foster collaborative activity leading to new systems, skills and productivity which will dramatically increase the UK manufacturing capacity and remove a bottle neck in development. This will help encourage developers to anchor production in the UK as they build their long term manufacturing capability. Advanced therapies continue to be a priority area for Innovate UK and the sector has received significant support via funding competitions and other supporting activities. In addition to the CGTC, the Medicines and Healthcare products Regulatory Agency (MHRA) is aware of and provides support to a range of other groups and associations working in this sector each of which strengthens the wider ATMP science and manufacturing network and hospital supply chain.

The potential economic impact of developing a strong UK regenerative medicine sector is an important factor in the Government's approach to regenerative medicine. Although still a niche sector, the rate at which firms are currently being established in the UK means this has been one of the fastest growing biopharmaceutical sectors[3]. The pace of development of the UK advanced therapies sector was one of the drivers behind the establishment of an industry-

[1] http://www.mrc.ac.uk/publications/browse/regenerative-medicine-strategypdf/
[2] http://www.ukrmp.org.uk/wp-content/uploads/2015/04/UKRMP-Annual-report-2016.pdf
[3] https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/525102/bis-16-237-strength-and-opportunity-2015-UK-medical-and-biopharmaceutical-landscape.pdf

led Advanced Therapies Manufacturing Taskforce which published an action plan for attracting and growing manufacturing of this new class of medicines in the UK[4].

The majority of regenerative medicines will be based on therapies involving the donation and transplantation of organs, tissues and cells. The national blood services continue to be leaders in these elements of regenerative medicine. NHS Blood and Transplant (NHSBT) and the Scottish National Blood Transfusion Service (SNBTS) have developed specialist GMP-compliant manufacturing facilities that may play an important role in supporting the manufacture of early phase clinical trials particularly where the commercial potential of the therapy remains a challenge to define. The blood services also represent a significant pool of skilled staff familiar with the challenges of delivering cell-based therapies. Overall, it is likely that as new regenerative therapies enter the clinical arena, they will be delivered on the foundation of existing transplant centres and other centres of excellence including in the clinical academic centres and by working in collaboration with their pharmacy departments.

Regulators have an important role to play in a sector where cutting edge therapies pose new and unexpected regulatory challenges. Through early engagement with product developers, the UK regulators have actively supported the translation, adoption and diffusion of innovative products. The Regulatory Advice Service for Regenerative Medicine (RASRM) provides a single point of access to free, clear, expert advice about the regulation of these therapies. The development of this service has resolved many of the concerns regarding the apparent complexity of the regulatory environment in the UK where regenerative therapies frequently involve several regulators. RASRM has recently included NICE, which provides an early opportunity for ATMP developers to engage with a regulator they would normally not see until after the regulatory stage. This has two main benefits for the developers – an early opportunity to consider the value proposition of such products and to help speed access to these products in line with the Accelerated Access Review (AAR) by working with regulators in parallel rather than in series. The Committee is correct to note that the process of Brexit offers an opportunity to review ATMP regulation in the UK and how it might be adapted to reflect national perspectives.

Government support for regenerative medicines should not be seen in isolation but as part of a broader strategy of related activities. In particular, the Government has invested significantly in genomics most notably through the 100,000 Genomes Project with the ambition for the UK to continue as a world-leader in the field. There are 13 Genomic Medicines Centres (GMCs) in England. Scotland and Northern Ireland have established their own GMCs and Wales is also joining the project via the establishment of a GMC. The Welsh Government launched its Genomics for Precision medicine Strategy on 6 July 2017. The Strategy outlines how £6.8m will be invested to build genetics and genomics capabilities in Wales, to allow patients to have faster, more accurate diagnosis and support treatment decisions (or precision medicine). The Strategy will also develop research in genetics and genomics, grow knowledge and skills amongst the NHS and non-NHS workforce and build strategic partnerships to underpin these areas. The Government also continues to support the implementation of the UK Rare Disease Strategy by 2020. Since the majority of rare diseases have an underlying genetic basis, many effective treatments for rare diseases may involve a form of gene therapy. This means that there is a clear synergy between advances in the understanding of the genetic basis of rare diseases and the development of advanced therapies including gene and cell therapies for patients with rare diseases.

The Government supports regenerative medicine medical and health research, particularly through the Medical Research Council and the National Institute for Health Research. The NIHR funds and supports health research funding, from early translational through clinical and on to applied health research. This includes a broad range of research in regenerative

[4] http://www.abpi.org.uk/our-work/mmip/Documents/Advanced-Therapies-Manufacturing-Taskforce-report.pdf

medicine, including cell therapies, tissue engineering, gene therapy and biomedical engineering. NIHR funds significant research infrastructure in the NHS in England and regenerative medicine is an important element of the research portfolio.

The Regenerative Medicine Expert Group (RMEG) was specifically tasked with preparing the NHS for the delivery of advanced therapies. The group made a series of recommendations in their report[5] and progress on delivering these was published last year[6]. In addition, there has been an increased emphasis on accelerating the adoption of new and innovative treatments within the healthcare system. The advanced therapies manufacturing action plan highlighted the need for a swift, predictable and viable route to market. The AAR, published in October 2016, set out recommendations for ways to make it simpler for NHS patients to access innovative technologies. The response to the AAR will streamline the process for development and adoption of new technologies, including regenerative medicines. NHS England has been working to develop mechanisms that allow patients to receive new innovative therapies as part of the specialised commissioning process. This includes the use of managed access agreements that help to balance access to treatments against value for money and affordability.

In conclusion, regenerative medicine has an important role to play in delivering therapies that offer treatments or potentially cures in areas of unmet medical need[7]. The Government continues to work to create an integrated environment that allows the delivery of these therapies and supports the development of a world-leading regenerative medicine industry in the UK. Whilst the Committee has called for a strategy for regenerative therapies, the Government favours a broader approach. Ensuring innovative technologies that the NHS needs can be developed and adopted quickly is central to both the AAR and also more generally the Life Sciences Industrial Strategy being developed by the sector. Getting the overall strategy for innovation in healthcare in place will mean that the potential of regenerative therapies can be fully realised, improving patient treatments and driving economic benefits for the UK.

1. The next Government should work with UK Research & Innovation to achieve a balance of investment in both basic scientific research and the translational research that it underpins, and to identify any research gaps in the light of the significant changes in the regenerative medicine sector over recent years. (Paragraph 18)

The Government agrees that all research funders involved with the development of regenerative medicine need to support a balanced and coherent approach to funding regenerative medicine. UK funders continue with this approach to use a coordinated set of delivery mechanisms through the continued implementation of the aforementioned 2012 Research Council/Innovate UK Strategy for UK Regenerative Medicine. Coordination of health research support is further supported by the Office for Strategic Coordination of Health Research (OSCHR), which brings together the principal public funders of health research including the Medical Research Council (MRC), National Institute of Health Research (NIHR) and Innovate UK along with the health departments in the devolved administrations. OSCHR provides an environment in which collaboration amongst research funders can thrive and has helped to facilitate the establishment of research programmes in key areas. Through these mechanisms, regenerative medicine has been supported by a coordinated set of delivery mechanisms across the sector.

[5] https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/415919/build-on-potential.pdf
[6] https://ct.catapult.org.uk/sites/default/files/publication/RMEG-report-2016.pdf
[7] https://ct.catapult.org.uk/sites/default/files/25901_CellTherapy_AnnualReview16_INTERACTIVE.pdf

The funding approach adopted by the Research Councils has been to balance support between basic and translational research. The aim has been to create an environment where frontier bioscience can thrive where researchers are encouraged to continue to ask fundamental biological questions and address problems[8]. There is also an important role for 'reverse translation', where the successes and failures of applied research feedback to inform further basic science. This will enable the development of regenerative medicines to be delivered in an iterative and holistic fashion.

The Research Councils have made a substantial investment in translational research of £80m including the £25m UK Regenerative Medicine Platform[9] since 2012. Strong financial support for basic research remains in place. In the most recent Research Council regenerative medicine portfolio analysis, 65% of the 255 awards active at 1 January 2016 were underpinning research i.e. 'basic' (Technology Readiness Level 1 & 2), with 27% at the early pre-clinical stage (Technology Readiness Level 3 & 4), and smaller numbers of project further down the developmental path. Funding support for regenerative medicine is continually evaluated, for example, in February 2016 the MRC's Regenerative Medicine Research Committee funding scheme was revised to focus on the earliest translational space in order to offer improved support for emerging opportunities.

Moreover, in recognition of the broad training needs for the emerging regenerative medicine industry, strategic effort has been placed in promoting interdisciplinary training. For example, both the MRC and Engineering and Physical Sciences Research Council (EPSRC) provide strong postgraduate training programmes which link to clinical and physical science disciplines. These complement other targeted training such as the £11m investment in 2014 in three Regenerative Medicine Centres for Doctoral Training in Leeds[10], Loughborough[11] and Manchester[12].

In March 2017 funding to support the UK Regenerative Medicines Platform (UKRMP) beyond 2018 was announced. The MRC, EPSRC and Biotechnology and Biological Sciences Research Council (BBSRC) have committed to spend a further £17m over five years to build on the Platform's successes and continue to support the development of therapies for clinical application. Although the timescales for the development of new therapies may take many years, investment in UKRMP is starting to bear fruit with new tools, reagents and protocols being made available for use by the wider research community[13]. Further to the success of Research Council coordination via UKRMP, the establishment of UK Research and Innovation (UKRI) will further strengthen the level of coordination between research funders and is an important element of the Industrial Strategy.

In terms of commercialisation, Innovate UK continues to play an important role in ensuring that translational research meets the changing needs of the sector, both directly through the support for enabling technologies and through the work of the CGTC. Since 2012 Innovate UK has awarded £54m in grants to business-led regenerative medicine and advanced therapy projects. These project grants have been matched with over £25m additional funding from industry. The close relationship between the Catapult innovators and industry means that it is uniquely placed to help guide investment towards areas of research with the strongest needs and demand from industry and therefore the greatest potential to support the development and commercialisation of new therapies.

[8] http://www.bbsrc.ac.uk/research/frontier-bioscience/
[9] UK Regenerative Medicine Platform (UKRMP): http://www.ukrmp.org.uk/
[10] www.imbe.leeds.ac.uk/doctoral-training-regenerative-medicine
[11] www.dtcregen-med.com
[12] www.regenmedcdt.manchester.ac.uk
[13] 2016 Annual report: http://www.ukrmp.org.uk/wp-content/uploads/2015/04/UKRMP-Annual-report-2016.pdf

The Committee noted that the health system in the UK can support the clinical development of regenerative therapies utilising the high quality research infrastructure provided by the NIHR and the established capacity in the procurement, processing and distribution of cells and tissues for transplant. The largest funder of health research with an annual budget of just over £1 billion, the overall aim of the NIHR is to improve the health and wealth of the nation through research working with the life sciences industries and charities to benefit all, actively involving patients and the public at every step.

NIHR research funding spans from early translational, through clinical and to applied health research. Between 2012 and 2017 the NIHR provided over £44m funding on regenerative medicine focussed research themes in NIHR Biomedical Research Centres and Units. In 2014/15 the Biomedical Research Centres and Units had over 150 active regenerative medicine research projects and leveraged research projects worth a total of over £90m from external funders for regenerative medicine research.

In 2016 as a result of a new competition for NIHR Biomedical Research Centres (NIHR BRCs) designation and funding, twenty new NIHR BRCs have been funded, each involving a partnership between the NHS in England and universities. They are supported for the next five years (from April 2017) with total investment of £816m. Many of these Centres are investigating diseases and conditions where regenerative medicines may offer important new therapies. For example, the Cambridge BRC is applying stem cell science to cell and organ transplantation and the Guy's & St Thomas's BRC is developing advanced therapies across a range of diseases.

A particular challenge for clinical trials for regenerative medicines is that they often require significant supporting infrastructure to enable clinical trials to be undertaken safely. The NIHR supports a network of Clinical Research Facilities (CRF) for Experimental Medicine that supports high-intensity clinical research, including for the development of regenerative medicines. Funding of £112.3m will support 23 NIHR CRFs (over five years from April 2017) to allow patient-orientated commercial and non-commercial experimental medicine studies. Clinical trials are further supported by the NIHR Clinical Research Network (NIHR CRN), providing world-class health service infrastructure to initiate and deliver clinical trials in the NHS in England, and research funded by the public, charity and industry sectors. The NIHR CRN has worked with the CGTC and the Department for International Trade to ensure companies coming into the UK are signposted to specific information regarding the UK environment for research, including ATMPs, thereby bringing clinical research business into the UK.

In autumn 2017, the NIHR CRN will, in a project co-sponsored by the British Society of Cell and Gene Therapy, establish best practice around issues such as pharmacy, site capabilities, workforce development and governance. This will ensure that the NIHR CRN is able to effectively support research involving gene and cell therapies.

The Welsh Government, through Health and Care Research Wales funds the Brain Repair and Intracranial Neurotherapeutics (BRAIN) Unit. The BRAIN Unit is based upon an All Wales, multidisciplinary collaboration to develop innovative therapies for neurodegenerative diseases. The Unit is developing improved systems for delivering treatments directly to the brain, including cellular therapies. The Unit has a Neuroscience Research Unit to provide safe, high quality facilities for commercial and non-commercial clinical trials and a GMP facility for the manufacture of foetal cells for clinical trials and treatment.

The MRC Centre for Regenerative Medicine (CRM) is housed within the Scottish Centre for Regenerative Medicine (SCRM) building, on the Edinburgh Bioquarter site shared by the Royal Infirmary Hospital and the University's Clinical Research facilities. With new state-of-the-art facilities and a 230+ team of scientists and clinicians, CRM is positioned uniquely to translate scientific knowledge to industry and the clinic. Research at CRM is aimed at

developing new treatments for major diseases including cancer, heart disease, liver failure, diabetes, and degenerative diseases such as multiple sclerosis and Parkinson's. The team also trains the next generation of basic and clinical scientists and acts as a centre for excellence in public engagement and source of advice for policy makers.

2. The next Government should review how regulatory 'hospital exemptions' are used for Advanced Therapy Medicinal Products across the UK, to assess how EU ATMP regulations might be adapted for the UK post-Brexit in order to reflect our own perspectives on the optimal balance between safety and accelerated access to cutting-edge technologies. (Paragraph 26)

The Hospital Exemption (HE) was established under the ATMP Regulation (2007/1394) to provide patients with flexible access to ATMPs provided they have been "prepared on a non-routine basis and used within the same Member State in a hospital in accordance with a medical prescription for an individual patient". It should be noted that while UK regulation is aligned to the European Union, this regulation can only be changed by the European Commission. In some Member States the exemption acts as a disincentive to developers where it is used to supply products even if there is a licensed alternative since there are no clear 'stop' criteria to cease supply when an equivalent authorised product is available. The Government has made representations to the Commission on a number of occasions on the way the exemption is drafted and applied across the EU and is amenable to reviews both at European level and post-EU Exit. Any review would aim to ensure that the exemption is not used to supply unlicensed ATMPs in competition with authorised products.

In addition to the Hospital Exemption scheme, it is also possible to supply ATMPs as an unlicensed medicine under the well-established UK "specials" scheme where the product is supplied under the derogation that applies under Article 5(1) of Directive 2001/83/EC (the main European Medicines Directive) [14]. Under this derogation, an unlicensed medicine may be supplied to meet the special clinical needs of an individual patient under the direct responsibility of the clinician where an equivalent licensed product is unavailable – this prevents competition with and undermining of authorised products. Many of the sites engaged in the development of ATMPs in the UK hold a manufacturing specials licence and this scheme has the advantage of a clear 'stop' signal where authorised products meet the special clinical needs of patients.

There is the potential for the UK working outside the EU regulatory system to adapt regulations for ATMPs on a national basis. The MHRA has set up a cross-Agency Brexit task force to manage the full implications of the UK's vote to leave the European Union (EU). The Office for Life Sciences has also created a joint industry and government Steering Group to inform the UK's negotiating priorities. The Group will consider a broad range of issues which impact on this sector including people, regulation and research funding, and examine some of the opportunities presented by EU Exit. As part of this work, the MHRA and Human Tissue Authority (HTA) are developing a range of different scenarios for ATMP regulation after exit from the EU in 2019. Any proposed changes will need to be introduced in a way that accelerates the development of new therapies without reducing safeguards to patient safety and public confidence.

In the meantime, the MHRA is taking forward a range of activities to support the sector. For example, it has a cross-Agency Horizon Scanning Working Group which monitors and evaluates scientific, technological and commercial developments. It is also taking forward

[14] Regulation 167 of the Human Medicines Regulations 2012 sets out the exemption from the requirement for a medicinal product, placed on the market in the UK to hold a marketing authorisation. This exemption flows from Article 5(1) of Directive 2001/83/EC.

recommendation 6 of the industry-led Advanced Therapy Manufacturing Action Plan[15]. This includes *developing a long-term regulatory strategy and plan for the MHRA to lead in global standards, supporting the scientific activities and international outreach of NIBSC.* There are few physical or written standards in the ATMP area given that the majority of products are at pre-clinical or early stage clinical trial stages and the science and technology of manufacture and testing are changing and improving continually, which makes the development of standards challenging. In addition, in an early stage industry sector with very rapid scientific and technological development, there is a need for defining the appropriate use of any reference standard, i.e. enabling developers to produce consistent medicines, including enabling them to update their manufacturing process more easily, rather than binding them too much into existing technologies in case there is significant development into newer formats. The development of standards will continue in 2017 as part of the overall activity on the Advanced Therapies Manufacturing Action Plan. The Government recognises the importance of regulations to remain aligned with EU requirements and standards.

Finally, the MHRA, HTA and the Human Fertilisation and Embryology Authority (HFEA) have established strong connections with scientific, regulatory and industry groups in the advanced therapies field. Their representation on the Advanced Therapies Manufacturing Taskforce and UKRMP means that they have been able to highlight research needed to support the regulatory assessment of these therapies. Regulators also remain active in policy development. As an example, the MHRA and HTA have worked together to develop policy related to the collection of blood for advanced therapy manufacture to make the best use of existing facilities, approvals and infrastructure without introducing additional regulatory requirements.

3. The Catapult should nevertheless extend its support more widely, to make it available to both experienced and new innovators in the regenerative medicine sector. (Paragraph 33)

The Government established the CGTC with a specific mandate to grow the industrial sector to bring health and wealth to the UK. The CGTC aims to support the development in the UK of a long term manufacturing industry with its attendant supply chain.

Since its establishment, the CGTC has worked to catalogue the capability and activity for regenerative medicine in academia and industry, creating databases covering preclinical, clinical and manufacturing activity across the UK. There are currently 22 manufacturing facilities, licensed for the production of ATMPs including both cell and gene therapies. In total, these facilities comprise of 5,500m^2 clean room production space and employ over 400 staff.

The completion of the new CGTC manufacturing centre later this year will add 7,000m^2 of additional clean room production facilities. The facility is supported by innovative supply, Quality Control, Quality Assurance and business systems. The flexible nature of the facilities means that cell and gene therapies can be produced using a wide range of production methods. The facility is designed to operate at large scale with commercial grade quality systems to complement the excellent existing, largely academic/public sector, manufacturing network.

The manufacturing centre is designed to attract inward investors to the UK and to support collaborators that have spun out of academia into SMEs and who need facilities and support to grow their pipeline. It will supply large scale trials including in the NHS and other health systems.

[15] Follow link provided on this website: https://ktn-uk.co.uk/perspectives/maintaining-the-uk-lead-in-advanced-therapy-medical-research

With its primary focus in supporting the commercialisation of new therapies, there also has been extensive engagement by the CGTC with the academic community. In the first four years of operation,19 of 70 projects were collaborations with academics such as on health economics, the development of target product profiles, process development, clinical trial design and regulatory strategy. To encourage engagement, the Catapult has run a number of "dragons den" for academics, an annual manufacturing course with University College London, and well attended information days on a range of topics on commercialisation. The Catapult is also frequently consulted on an *ad hoc* basis for support and guidance by academics and engages actively public research funders and a range of fora.

We know that many new innovators in the regenerative medicine can find the apparent complexity of navigating the regulatory and health economic appraisal landscape in the UK challenging. This theme has been recognised by Government agencies who have worked to improve the way in which advice and support is made available to developers of regenerative medicines. For example:

- The RASRM service was launched on 13 October 2014 and is run by the UK's independent and expert regulators – the Health Research Authority (HRA), HFEA, HTA, MHRA, and NICE (since 2017) with links to other specialist bodies such as the Health and Safety Executive (HSE) and the Department for Environment, Food and Rural Affairs (DEFRA). The service offers research and development professionals across academia, industry and the NHS (including clinicians) a single point of access to free, clear, expert advice about the regulation of regenerative medicine.
- The MHRA's Innovation Office continues to provide free regulatory advice across all areas, including on manufacturing. By September 2016, 346 queries had been received by the Innovation Office. Of these, 62% of applicants are SMEs, academic or NHS institutions (112 queries from SMEs, 85 from academia and 19 from NHS). The MHRA has now published nine case studies highlighting the work of their Innovation Office, showing how they help organisations that are developing innovative medicines and medical devices, or using novel manufacturing processes, to effectively understand and navigate the regulatory process.

4. The next Government should nevertheless work with NHS England and Clinical Commissioning Groups to create the appropriate financial incentives to stimulate regenerative medicine research and innovation within the NHS, which will encourage more clinicians to become more involved in research. The next Government should also support work by NHS England and NICE to deliver a 'fast track' appraisal system for emerging regenerative medicine therapies. (Paragraph 51)

NHS England is establishing a wider commercial function following taking on the commercial medicines unit and is already working to deliver innovative new managed access arrangements for highly specialised technologies for rare diseases, some cancer drugs and drugs with high budget impact going forward. The AAR set out the potential for a win:win, whereby earlier patient access to promising new treatments could generate a valuable NHS real world evidence base that better informs commercial negotiations. Regenerative medicines might be one such category of treatments where this approach could offer greater value for patients, industry and tax payers.

NICE and NHS England consulted on changes to the NICE Technology Appraisals and Highly Specialised Technologies (HST) programmes from October 2016 to January 2017. The proposals included the introduction of a new 'fast track' technology appraisal process for those new technologies which fall below an incremental cost-effectiveness ratio (ICER) of £10,000 per QALY (quality adjusted life year), to get these treatments to patients more

quickly. Following consultation, this proposal was approved and the new 'fast track' process is being implemented.

Some regenerative medicines and cell therapies are used to treat cancers and here, NICE and NHS England arrangements for cancer drugs would apply, including the potential use of the Cancer Drugs Fund. For cancer drugs, the majority of NICE's technology appraisal process is undertaken in parallel with the European Medicines Agency (EMA) regulatory processes such that NICE issues draft guidance ahead of Marketing Authorisation (shortly after a positive opinion is issued by the EMA Committee for Medicinal Products for Human Use). As a result, for cancer drugs recommended by NICE, funding is available from the point of Marketing Authorisation.

Regenerative medicines outside of cancer indications that did not meet the requirements for the 'fast track' process would be evaluated through NICE's standard processes if selected for evaluation by NICE. There is an expectation of draft guidance being issued by NICE within 6 months of Marketing Authorisation. For interventions for Specialised Services (where it is expected most regenerative medicines will be commissioned) not being appraised by NICE, NHS England will form National Clinical Policy as set out in its methods[16]. This will apply an assessment of the clinical evidence base proportionate to the type of intervention.

The NIHR has a number of mechanisms for encouraging more clinicians to become more involved in research including providing research project funding and funding for research sessions through the NIHR CRN. NIHR training for the next generation of clinical researchers is essential to supporting clinicians in their research careers. The NIHR supports research training and career development programmes that support the development of future leaders across all clinical professions and disciplines. Cross-disciplinary research is particularly important to regenerative medicines and the NIHR Integrated Academic Training Programme provides protected time to undertake pre-doctoral research training for clinicians undertaking specialty training - themes include bioinformatics, genomics and clinical pharmacology. NIHR Research Professorships support outstanding clinical academics to undertake research, including regenerative medicine such as cell and gene therapies for children with leukaemia and immunodeficiency; prevention of blindness from retinal diseases; and improving outcomes after stem cell and organ transplant.

5. The next Government should also work with the biotech sector and with NHS England and NICE to agree new reimbursement payment models which take greater account of the value of regenerative medicine therapies that offer cures, reduce healthcare costs and make treatments available earlier to patients. (Paragraph 52)

The Government will ensure that NICE, NHS, CGTC the healthcare industry associations continue to work collaboratively to develop strategies that support the adoption of innovative therapies with substantial benefit to the patient and the healthcare system.

The Pharmaceutical Price Regulation Scheme already provides for patient access schemes through which companies can propose a range of different commercial arrangements that improve the value proposition of drugs undergoing a NICE appraisal. In response to a recommendation from RMEG, NICE has worked with the University of York to explore how assessment and appraisal could be applied to regenerative medicines. The study included a broad exploration of the applicability of NICE technology appraisals methods as well as detailed consideration of hypothetical example products within a number of possible scenarios. A key finding was that in circumstances where there is a combination of great uncertainty but potentially very substantial patient benefits, innovative payment methods need

[16] https://www.england.nhs.uk/wp-content/uploads/2012/11/op-model.pdf

to be developed to manage and share risk to facilitate timely patient access while the evidence is immature[17].

6. NHS England needs to take a lead on regenerative medicine by including it explicitly in its Personalised Medicine strategy. This would send a strong signal to the sector of the NHS's commitment and willingness to adopt new and emerging therapies. (Paragraph 57)

NHS England has established a personalised medicines team with oversight from the Chief Scientific Officer. The initial focus has been on embedding genomic medicine in the NHS, building on the 100,000 Genomes Project but NHS England will look to include Regenerative Medicine in this portfolio. As with all emerging technologies, NHS England offers 'Stakeholder Surgeries' to organisations ready to introduce interventions to guide them how commissioning can support spread once evidence of clinical benefit has been established.

7. The next Government should work with UK Research & Innovation, industry, academic researchers and the health sector to develop a strategy for Advanced Therapies, which should include regenerative medicine and cell therapies. The strategy should be aligned to the Government's response to the Accelerated Access Review and the strategic objectives outlined in the Government's Industrial Strategy Green Paper. (Paragraph 58)

The Government continues to actively work to create an environment that will support the development of a world-leading regenerative medicine industry. Support for the development and adoption of innovations in the NHS is central to both the AAR and also more generally the Life Sciences Industrial Strategy. The response to the AAR is expected in due course. The Government looks forward to ongoing collaboration with the life sciences sector, looking ahead to a sector deal that will build on the Life Sciences Industrial Strategy. The Strategy looks to capitalise on opportunities in the UK across the sector, including in Advanced Therapies.

[17] https://www.nice.org.uk/Media/Default/About/what-we-do/Science%20policy%20and%20research/regenerative-medicine-study-march2016-2.pdf