



House of Commons  
Science and Technology  
Committee

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**Regenerative medicine**

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**Fifteenth Report of Session 2016–17**

*Report, together with formal minutes relating  
to the report*

*Ordered by the House of Commons  
to be printed 26 April 2017*

## Science and Technology Committee

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### Contacts

All correspondence should be addressed to the Clerk of the Science and Technology Committee, House of Commons, London SW1A 0AA. The telephone number for general inquiries is: 020 7219 2793; the Committee's e-mail address is: [scitechcom@parliament.uk](mailto:scitechcom@parliament.uk).

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## Summary

Regenerative medicine has enormous potential, offering treatments for diseases and disorders by providing the body itself with the means to repair, replace, restore and regenerate damaged or diseased cells, tissues and organs. The UK has considerable strengths in this field, underpinned by world-class research. Its continual development depends crucially, however, on an appropriate balance between basic scientific research and the translational research that it underpins.

It is important that the regulatory environment for regenerative medicine remains flexible to accommodate new and diverse approaches while also maintaining robust review processes to ensure that the most promising therapies are made available to patients. The next Government should review how regulatory ‘hospital exemptions’ are used for Advanced Therapy Medicinal Products (ATMPs) and how EU ATMP regulations might be adapted for the UK post-Brexit to reflect our own perspectives on the optimal balance between safety and accelerated access to cutting-edge technologies.

Having a universal NHS provides a receptive environment for the development and adoption of innovative and scientific advances in regenerative medicine, but the next Government should work with the NHS to: create the appropriate financial incentives to further stimulate regenerative medicine research and innovation; deliver a ‘fast track’ appraisal system for emerging therapies; and agree new reimbursement payment models which take greater account of the value of regenerative medicine therapies.

The next Government should work with UK Research & Innovation (UKRI), industry and researchers to develop a strategy for advanced therapies that covers the entire regenerative medicine value-chain specifically—from academic research, to commercial development and clinical application—and the NHS’s Personalised Medicine strategy should explicitly include regenerative medicine and cell therapies. These strategies 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.

# 1 Introduction

1. The House of Lords Science and Technology Committee reported in 2013 on regenerative medicine, defining it as “methods to replace or regenerate human cells, tissues or organs in order to restore or establish normal function. This includes cell therapies, tissue engineering, gene therapy and biomedical engineering techniques, as well as more traditional treatments involving pharmaceuticals, biologics and devices”.<sup>1</sup> It has been used, for example, in bone marrow transplants, and offers the prospect of more effective repairs for faulty hearts, skin burns and worn-out joints. The Government identified regenerative medicine in 2013 as one of the “eight great technologies”<sup>2</sup>, which would “propel the UK to future growth”.<sup>3</sup>

2. The Lords Committee’s report noted that the value and importance of regenerative medicine lies in its potential both to address unmet medical needs and “to cure or provide more effective treatments for a number of chronic diseases, which would be a major benefit to the UK public purse given the rising expenditure on healthcare associated with chronic disease management and related indirect costs”.<sup>4</sup> It called for further investment to support the growth of the sector and to enhance the UK’s global competitiveness.<sup>5</sup>

3. Following the Committee’s report, the Government set up a Regenerative Medicine Expert Group (RMEG) to “develop an NHS regenerative medicine strategy [and action plan] so that the NHS is fully prepared to deliver these innovative treatments, and also assess the effect of regulation on the development of regenerative medicines in the UK”.<sup>6</sup> The RMEG considered three key areas in its 2014 report: regulation and licensing, evaluation and commissioning, and delivery and adoption within the healthcare system. While it found significant progress in the first of these areas, there had been less improvement in understanding and addressing the challenges of trialling and delivering novel therapies.<sup>7</sup>

4. The then Life Sciences Minister, George Freeman MP, responded in February 2015 by asking the RMEG to liaise with industry, health and research sectors “to evolve a high-level group” to oversee the implementation of the Expert Group’s recommendations.<sup>8</sup> In March 2016, a Government/industry-based Advanced Therapy Manufacturing Taskforce was created to help “anchor advanced therapy manufacturing in the UK”.<sup>9</sup>

5. A number of significant therapeutic advances have been demonstrated for some conditions, including diabetes, liver disease and blood and neurodegenerative disorders. Success in recent small-scale clinical trials of cell-based cancer immunotherapies has

1 House of Lords, Regenerative Medicine, First Report of the Science and Technology Committee, Session 2013–14, [HL Paper 23](#), para 8

2 Department for Business, Innovation and Skills and Rt. Hon David Willetts, [Eight great technologies](#), Speech, 24 January 2013

3 BIS, [Eight Great Technologies](#) (January 2013)

4 House of Lords, Regenerative Medicine, First Report of the Science and Technology Committee, Session 2013–14, [HL Paper 23](#), para 21

5 House of Lords, Regenerative Medicine, First Report of the Science and Technology Committee, Session 2013–14, [HL Paper 23](#)

6 Gov.uk, ‘[Regenerative Medicine Expert Group](#)’, accessed 24 April 2017

7 Regenerative Medicine Expert Group, [Building on our potential: a UK pathway for regenerative medicine](#) (December 2014)

8 Department of Health and Department for Business, Innovation and Skills, [Letter from Minister for Life Sciences, George Freeman MP to Sir Mike Rawlins, Chair of the Regenerative Medicine Expert Group](#) (February 2015)

9 Medicines Manufacturing Industry Partnership, ‘[Industry taskforce launched to secure the future of manufacturing advanced therapy medicines in the UK](#)’, Press Release, 23 March 2016

encouraged increased commercial investment. Annual product sales in the global regenerative medicine and cell therapy market are currently in the region of £1 billion and are projected to exceed £5 billion by 2020.<sup>10</sup>

## Our inquiry

6. We decided to examine progress since the Lords Committee's inquiry by examining two main areas: research and commercialisation (Chapter 2) and the adoption of regenerative medicine in the NHS (Chapter 3). We received 31 written submissions and took oral evidence from 22 witnesses, including from the pharmaceutical industry; Government regulatory and advisory bodies; academia and the then Parliamentary Under-Secretary of State for Health, Lord Prior of Brampton. We also visited the UCL Institute of Ophthalmology at Moorfield's Eye Hospital and the Centre for Cell, Gene and Tissue Therapeutics at the Royal Free Hospital (see Annex). We would like to thank everyone who contributed to our inquiry, including Professor Sian Harding, professor of cardiac pharmacology at the National Heart and Lung Institute at Imperial College, for her expertise and guidance as our special adviser during the inquiry.<sup>11</sup>

10 Innovate UK, *Competition for funding—developing regenerative medicines and cell therapies* (2015)

11 Professor Harding declared her interests on 28 June 2016: Professor of Cardiac Pharmacology, National Heart and Lung Institute, Imperial College London; Board member of the British Society for Gene and Cell Therapy; Director of British Heart Foundation (BHF) Cardiovascular Regenerative Medicine Centre; Co-investigator in the UK Regenerative Medicine Platform Immunomodulation Hub; Co-ordinating Investigator of the Clinical Trial of Gene Therapy in Heart Failure; member of the Medical Research Council (MRC) Regenerative Medicine Research Committee; member of the BHF Chairs and Programme Grants Committee; on editorial board of Cardiovascular Research; on Scientific Advisory Board of the Stem Cells for Safer Medicines (PPP); Head of Division, Cardiovascular, National Heart and Lung Institute (NHLI); cardiovascular Theme Lead, Biomedical Research Centre, Imperial Academic Health Trust; member of Biomedical Research Unit Committee, Royal Brompton and Harefield Trust; member of the Tissue Governance Committee, Royal Brompton Hospital; Designated Individual for NHLI, Human Tissue Act Licence; Expert on Alternatives to Animals in Research, Imperial College; Fellow of the British Pharmacological Society, Society of Biology, International Society for Heart Research, European Society of Cardiology and American Heart Association; Until 2010 President of the European Section of the International Society for Heart Research; until 2013 member of the MRC Populations, Systems and Medicine Board, Nuffield Council for Bioethics, and European Society of Cardiology Programme Committee; recipient of grant awards from MRC, National Centre for the Replacement, Refinement and Reduction of Animals in Research, BHF, Rosetrees Trust, Leducq network of Excellence, Rectors Award for Excellence, Celladon, Wellcome Trust, GSK.

## 2 Research and commercialisation

7. In this chapter we look at the UK's clinical research landscape, examining basic and translational research and the regulation and the commercialisation of regenerative medicine and cell therapies.

### The research landscape

8. Regenerative medicine involves a wide spread of 'science', spanning tissue engineering, developmental and stem cell biology, gene therapy and cellular therapeutics, biomaterials such as 'scaffolds' and 'matrices', nanoscience, bioengineering and chemical biology.<sup>12</sup> It was clear from the written evidence we received that regenerative medicine is a developing research field that offers the potential to revolutionise patient care. Advances in regenerative medicine hold the promise of improving methods of disease diagnosis and prevention, and the development of innovative treatments for injuries and illnesses.

9. Cell-based therapies,<sup>13</sup> for example, have been approved in the UK for bone marrow transplants (for treating blood and disorders in the immune system). Other stem cell treatments being used, but not yet routinely, include emergency skin grafts (epidermal stem cells) and repairing the cornea of the eye using limbal stem cells.<sup>14</sup> Although many stem cell treatments are showing promise in clinical trials, Professor Paul Riley of the University of Oxford highlighted that "a knowledge gap" still exists.<sup>15</sup> Professor Anthony Hollander of the University of Liverpool described his research into developing a method of creating cartilage cells from stem cells, making possible the first successful transplant of a tissue-engineered trachea, utilising the patient's own stem cells.<sup>16</sup> His work provided evidence that using adult stem cells could offer solutions for other serious illnesses.

10. Research by scientists hinges on learning how different biological processes work and applying that knowledge to:

- understand better the causes and progression of different diseases, such as Parkinson's disease and multiple sclerosis, thereby facilitating better treatments;
- create new biological tools and technologies to accelerate research, drug discovery and medical testing, while also reducing costs and the use of animals; and
- design and develop novel treatments to both enhance natural repair processes and use the abilities of stem cells for replacing damaged, malfunctioning or diseased cells.<sup>17</sup>

11. Since 2012, research councils have provided £80 million for research on regenerative medicine and supporting technologies.<sup>18</sup> This included £25 million provided to support the establishment of the UK Regenerative Medicine Platform (UKRMP) specifically to

12 BBSRC, EPSRC, ESRC, MRC and Technology Strategy Board, [A Strategy for UK Regenerative Medicine](#) (2012)

13 Cell based therapies work via stimulation of endogenous repair through extracellular factors or differentiation and functional replacement of endogenous cell types, which include stem cell transplantation or infusion to treat hematopoietic diseases, cardiac conditions and Parkinson's disease.

14 Centre for Regenerative Medicine, ['What are stem cells?'](#), accessed 24 April 2017

15 Q19

16 Qq80–82

17 Centre for Regenerative Medicine, [What are stem cells?](#), accessed 24 April 2017

18 Department of Health ([REG0007](#))

address key ‘translational’ bottlenecks (paragraph 15) identified in the research councils’ *Strategy for UK regenerative medicine* in 2012.<sup>19</sup> In 2014, £11 million was provided by the Engineering and Physical Sciences Research Council (EPSRC) and the Medical Research Council (MRC) to create three centres for doctoral training.<sup>20</sup>

12. The UKRMP—a joint initiative by the MRC, the EPSRC and the Biotechnology and Biological Sciences Research Council (BBSRC)<sup>21</sup>—has established multi-science research hubs across the UK, which produce a variety of tools, reagents and protocols for use by the academic and commercial sectors.<sup>22</sup> This initiative has brought together research teams from across 20 universities to help develop new therapeutic approaches.<sup>23</sup> It has advanced ‘pre-clinical’ work in areas such as Parkinson’s disease, liver disease, eye disease (age-related macular degeneration), corneal and retinal surgery and joint disorders. The UK Regenerative Medicine Platform (UKRMP) is expected to receive further funding of £17 million from the Medical Research Council (MRC) in 2017 to support programmes reaching clinical testing stage within the next five years.<sup>24</sup>

13. Regenerative medicine is also an important element of the National Institute of Health Research’s (NIHR) research portfolio. Between 2012 and 2017, it will have provided over £44 million for regenerative medicine research in Biomedical Research Centres and Biomedical Research Units.<sup>25</sup> In 2014–15, these research centres had over 150 active regenerative medicine projects, part-funded by the private sector and charitable bodies.<sup>26</sup>

### **Basic and translational research**

14. Substantial progress has been made in recent years to understand better the underlying biology of cells and of the role of their surrounding micro-environment, which has led to more researchers moving into ‘translational’ regenerative medicine—“the process of applying ideas, insights, and discoveries generated through basic scientific inquiry to the treatment or prevention of human disease”, or ‘bench to bedside’.<sup>27</sup>

15. Parkinson’s UK highlighted that such research in regenerative medicine is “expensive and offers little in the way of scientific discovery, but is essential if the therapy is ever to go to trial”.<sup>28</sup> Translational research is a challenge because of knowledge gaps that remain and the high costs of developing complex and multi-disciplinary therapeutic approaches for which the commercial market is uncertain. The UK is well positioned, however, to undertake clinical development in this field, given the strength of its pre-clinical science

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19 Department of Health ([REG0007](#))

20 Department of Health ([REG0007](#))

21 UK Regenerative Medicine Platform, ‘[Future of regenerative medicine looks bright with new funding boost](#)’, Press Release, March 2017

22 UK Regenerative Medicine Platform, ‘[Future of regenerative medicine looks bright with new funding boost](#)’, Press Release, March 2017

23 UK Regenerative Medicine Platform, ‘[Future of regenerative medicine looks bright with new funding boost](#)’, Press Release, March 2017

24 UK Regenerative Medicine Platform, ‘[Future of regenerative medicine looks bright with new funding boost](#)’, Press Release, March 2017

25 Department of Health ([REG0007](#))

26 Department of Health ([REG0007](#))

27 Office for Translational Research, University of Cambridge, ‘[What is translational research?](#)’, accessed 24 March 2017

28 Parkinson’s UK ([REG0005](#))



and the potential to draw upon high quality research infrastructure (through NIHR) and established capacity in the procurement, processing and distribution of cells and tissues for human application (through NHS Blood and Transplant).<sup>29</sup>

16. The UKRMP was established specifically to address the translational research agenda. Dr Rob Buckle from the Medical Research Council, one of the Platform's sponsor bodies, explained that the translational research landscape had changed significantly:

There is a real opportunity around the interdisciplinary mix that has been brought together, and the tools and technologies are in large part now there [...] We should be optimistic that there will be good progress in the 10-year time-line but whether that translates to clinical products is an open question.<sup>30</sup>

The opportunities for translational research were also echoed by Dr Ruth McKernan, Chief Executive of Innovate UK, who told us:

With the continued investment in the UK of research and translation in stem cell science and regenerative medicine, we are one of the leading countries in the world. [...] We see more small and medium sized business growing very quickly [...] there is an opportunity for us to think globally about how we get ahead of the different steps that are required to really make us a global leading country for cell based therapies.<sup>31</sup>

17. Despite the focus on 'translational research', witnesses saw a continuing need for the more basic scientific research that underpins it. Professor Peter Andrews from the University of Sheffield told us that:

The research councils, the Government and various organisations have clearly put in place the mechanism for translation. Quite a lot of work has been put in place to begin to exploit these opportunities. The real problem is getting the balance between the basic biology that will feed the pipeline into the translation and how to take it forward.<sup>32</sup>

Professor Jeremy Pearson from the British Heart Foundation voiced a similar concern:

We are at a pivotal stage in the science, which is advancing rapidly, with the potential for real development into clinical efficacy, but [...] there is a lot more science yet to be learned to enable that transition to work fluidly across a wide range.<sup>33</sup>

Professor Paul Whiting, a member of the Medical Research Council's regenerative medicine research committee, told us that:

Within the UK, we have an outstanding science base in this area. For us to continue to be leaders within this field, we need to continue to maintain

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29 Q75 [Dr Rob Buckle]

30 Q54

31 Q151, Q153

32 Q10

33 Q57

that investment in the basic science and translational science—otherwise, our ability to stay in the vanguard in terms of moving that into commercial products is going to severely wane.<sup>34</sup>

Professor Paul Riley from University of Oxford wanted more emphasis on “a multi-disciplinary approach” which goes beyond the work of the UKRMP in linking together researchers in different fields:

While there is obvious pressure [...] to move down a translational pipeline in the context of both [research councils] and charities, [ ... ] leaving behind the basic science cannot be allowed to happen. You need something to translate first and foremost. More importantly, you need to understand the mechanisms of what you are actually doing when you go down these therapeutic routes.<sup>35</sup>

**18. Regenerative medicine provides a unique approach to treating diseases and disorders by providing the body itself with the means to repair, replace, restore and regenerate damaged or diseased cells, tissues and organs. Its continual development depends crucially on a strong foundation of basic scientific research. *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.***

## Commercialisation

19. The commercialisation of regenerative medicine research depends on the regulatory framework and industry’s capacity to scale-up the therapies involved.

## Regulation

20. Regulation of regenerative medicine is complex, reflecting the nature of the technologies involved and their risks. Many regenerative medicine products are governed by the EU Tissues and Cells Directives which outline mandatory standards for using tissues and cells for human applications.<sup>36</sup> In the UK, the Human Fertilisation and Embryology Authority and the Human Tissue Authority are responsible for implementing these standards. Other regenerative medicine products are governed by the Community Code for Medicinal Products. Authorisation for clinical trials of regenerative medicines (and of all medicinal products) is governed by EU Directives 2001/20/EC and 2005/28/EC, which are implemented by the Medicines and Healthcare products Regulatory Authority Agency (MHRA). The regulatory framework is intended to ensure that medicinal products used in a clinical setting comply with ‘Good Manufacturing Practice’ (GMP). Lawyers, clinicians, ethicists and other scientists evaluate research proposals to ensure that the use of stem cells is appropriate. If researchers want to carry out new experiments with already-produced stem cell lines, a new proposal must be submitted for approval.

34 Q101

35 Q13

36 [Safe human tissues and cells for transplantation—\(2004/23/EC, 2006/17/EC and 2006/86/EC\)](#), January 2016

21. Dr Ian Hudson, Chief Executive of the MHRA, explained that the Agency works with those developing products to ensure consistency, quality, safety and efficacy, so that products can be developed appropriately and be licensed.<sup>37</sup> The Regenerative Medicine Expert Group identified various aspects of the regulatory framework which required improvement. It called on the MHRA to consider a European classification scheme for regenerative medicine products, to make changes to the ‘hospital exemptions’ scheme which allows for the marketing of unlicensed medicinal products and to incorporate applications for clinical trials approval into the Health Research Authority’s existing Integrated Research Application Systems.<sup>38</sup> The MHRA subsequently established a one-stop shop advice service for regenerative medicine. The PHG Foundation (a health policy think tank) complained, nevertheless, that there was still “a lack of central coordination and oversight to streamline the regulatory process and share data, share ‘lessons learned’ from ongoing trials and a central vision driving forward the development of the most promising therapies”.<sup>39</sup>

22. PHG Foundation told us that “the regulatory environment was not being consistently applied in the field of ‘advanced therapy medicinal products’ (ATMPs)”.<sup>40</sup> These emerging technologies and therapies are based on genes (‘gene therapy’), human cells (‘cell therapy’) and tissues (‘tissue engineering’) and are regulated by the EU.<sup>41</sup> The EU Directive requires ATMPs to obtain authorisation from the European Medicines Agency before they can be placed on the market.<sup>42</sup> Because ATMPs are complex therapeutic products that require specialist manufacture, storage and distribution, there were only 20 approved ATMPs worldwide as at 2016.<sup>43</sup>

23. Current EU regulations allow for exemptions where products can be made on a non-routine basis without market authorisation. A ‘hospital exemption’ allows an ATMP to be manufactured for use by doctors in a hospital setting to meet the needs of particular patients. In the UK, there is also a so-called ‘specials’ exemption scheme which allows further flexibility. The regulatory landscape has changed in response to the development of new drugs. Through the MHRA’s Early Access to Medicines Scheme, drugs are made available to patients before they have gained full licence approval.<sup>44</sup> Such adaptive licensing also allows drugs that have received initial approval for use by a defined group of patients to then go on to be used on larger populations.

24. Although the exemption system is a potentially life-saving process for some patients, some witnesses had concerns about possible inconsistency and the implementation of the exemptions. Genetic Alliance UK told us that the “impact of these schemes on patient access to advanced therapies [...] is unclear”.<sup>45</sup> The Association of the British

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37 Q93

38 Regenerative Medicine Expert Group, [Building on our potential: a UK pathway for regenerative medicine](#) (December 2014)

39 PHG Foundation ([REG0017](#))

40 PHG Foundation ([REG0017](#))

41 The aim of ATMP regulation was to offer a consolidated regulatory framework for innovative medicines and it was designed to ensure the free movement of these medicines within the EU, to facilitate their access to the EU market, and to provide incentives to European pharmaceutical companies in the field, while guaranteeing the highest level of health protection for patients.

42 [EU Directive 2001/83/EC](#) as amended by the ATMPs Regulation 1394/2007

43 Department of Health ([REG0007](#))

44 Medicines and Healthcare products Regulatory Agency, [‘Apply for the early access to medicines scheme \(EAMS\)’](#), accessed 24 April 2017

45 Genetic Alliance UK ([REG0008](#))

Pharmaceutical Industry (ABPI) also raised concerns about the hospital exemption, arguing that “there was no uniform interpretation of the term ‘non-routine’, leading to different implementation of the exemption by different Member States”.<sup>46</sup>

**25. It is important that the regulatory environment for regenerative medicine remains flexible to accommodate new and diverse approaches while also maintaining robust review processes to ensure that the most promising, effective and safe therapies are made available to patients. The MHRA have taken forward the Regenerative Medicine Expert Group’s recommendations on providing a central focal point for regulatory advice, but there is more to be done.**

*26. 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.*

### **Opportunities for manufacturing**

27. There are also non-regulatory challenges for the manufacture and supply of regenerative medicine products, including the complexity of some manufacturing procedures and the difficulty in scaling-up processes. The Academy of Medical Sciences told us that:

Further infrastructure needs to be prioritised to enable affordable access to appropriate manufacturing facilities at GMP standards, particularly for academia and SMEs. Currently these facilities are not available at sufficient scale in the UK, and researchers need the source material from the EU and US manufacturing centres, generating further cost and regulatory challenges.<sup>47</sup>

28. Ian McCubin of GlaxoSmithKline, and co-chair of the Advanced Therapy Manufacturing Taskforce, explained that:

The nature of these products is that they are moving quickly and within one or two years, they will be much more common.[...] SMEs are growing, but they will hit a tipping point quite quickly where they have to make their mind up about whether they invest in manufacturing capability here or go somewhere else. We have to make sure that, when they reach that point, they do it here and the mechanisms are in place.<sup>48</sup>

University of Bristol told us that:

A major barrier to progress is the paucity of funding of GMP manufacture of novel therapies for use in Phase 1 clinical trials [...] A further imminent national problem will be that of Phase 3 trials. These multi-centre trials [...] are extremely expensive and far much beyond the scale of the MRC, NIHR or charities.<sup>49</sup>

46 Association of the British Pharmaceutical Industry ([REG0020](#))

47 Academy of Medical Sciences ([REG0014](#))

48 Qq158–159

49 University of Bristol ([REG0002](#))

29. To support the growth of regenerative medicine, Innovate UK established the Cell and Gene Therapy Catapult in 2012. In 2014, the Chancellor announced further funding to create a UK Cell Therapy Manufacturing Centre, scheduled to open in 2018. This will be managed by the Catapult.<sup>50</sup> Statistics published by the Cell and Gene Therapy Catapult show that investment in the cell and gene therapy industry in 2015 was over £400 million compared to £35 million in 2012.<sup>51</sup> Witnesses welcomed the Catapult's work in helping to bridge the gap between translational research and the manufacturing of cellular and regenerative medicine therapies. Dr Rob Buckle from the Medical Research Council believed that "the Catapult's role is to commercialise. It is a company and therefore the demarcation between academic research and how it crosses over into the Catapult is reasonable. [...] It is a good pipeline and model".<sup>52</sup> Professor Jeremy Pearson of the British Heart Foundation believed that the Catapult was "in the right space to assist that transition as and when it occurs, but it has not quite got there yet. It is a valuable piece of the ecosystem".<sup>53</sup> While the Academy of Medical Sciences told us that the Catapult provided helpful guidance, they believed that "on occasion, the cost implications and limitations in capacity for the [Catapult] to provide guidance can mean that further advice needs to be sought elsewhere".<sup>54</sup> Professor Anthony Hollander of University of Liverpool expressed similar views:

It is hugely valuable to us on the one hand as an expertise resource. On the other hand, I think that in future it will help us collectively to figure out how we scale up commercially and turn the discoveries into something that can reach larger numbers of people. I am not sure the model is perfect yet. The Catapult is still developing its own self vision, but we are getting there.<sup>55</sup>

30. The size of the advanced therapies market has been estimated at \$10 billion by 2025 for individual product classes such as Gene Modified Immune Therapies or Ex-vivo Gene Therapies, up to \$67 billion per annum by 2020 for the industry as a whole.<sup>56</sup> The key commercial challenge for companies seeking to tap that market is working out how Advanced Therapy Medicinal Products [ATMPs] will fit into European healthcare systems and how they will be paid for by healthcare funders or users.<sup>57</sup> Innovate UK told us:

ATMPs promise to be highly disruptive to the current clinical treatment setting and systems. Establishing 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.<sup>58</sup>

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50 Cell and Gene Therapy Catapult ([REG0026](#))

51 Cell and Gene Therapy Catapult, '[Investment in cell and gene therapy industry in the UK grows 10x since 2012 to £400m/per year](#)', Press Release, 21 September 2016

52 Q70

53 Q63

54 Academy of Medical Sciences ([REG0014](#))

55 Q63

56 Department of Health ([REG0007](#))

57 BioIndustry Association ([REG0022](#))

58 Innovate UK ([REG0013](#))

31. In September 2016, the Advanced Therapy Manufacturing Taskforce published an Action Plan, which included:

- securing an internationally competitive fiscal landscape to attract investment;
- capturing investments through a proactive and targeted marketing approach and simplifying the process of engagement between investors and Government;
- capturing internationally mobile investment through capacity and capability growth in the UK;
- setting out an end-to-end talent management plan to secure the relevant skills for emerging manufacturing technologies;
- setting out a swift, predictable and viable route-to-market for these innovative products and giving industry confidence that the UK is a progressive global hub; and
- developing a long-term regulatory strategy and plan for the MHRA to lead in global standards.<sup>59</sup>

Jo Johnson MP, minister for Universities, Science, Research and Innovation, believed that the action plan will provide valuable input to the Industrial Strategy, stating in November 2016 that “life sciences, and within that advanced therapies, hold great potential for the UK to develop new sources of economic competitiveness”.<sup>60</sup>

32. In April 2017, Business Secretary Greg Clark MP announced the Government’s commitment of over £1 billion to the Industrial Strategy Challenge Fund, which we examined in our recent report on the industrial strategy.<sup>61</sup> That new commitment included £197 million over the next four years to develop first-of-a-kind technologies for the manufacture of medicines that will speed up access to new drugs and treatments.<sup>62</sup>

**33. The UK life sciences sector is a pioneer in the clinical development of new regenerative medicine therapies and well placed to create new high-tech high-value manufacturing businesses around these advanced therapies. Regenerative medicine researchers, however, need manufacturing support for ‘translating’ their work, and the Cell and Gene Therapy Catapult is working to bridge the gap between ‘translational’ research and commercialisation. *The Catapult should nevertheless extend its support more widely, to make it available to both experienced and new innovators in the regenerative medicine sector.***

34. In order for regenerative medicine to attract commercial interest, companies must have the confidence that their investment will generate a return. Potential barriers to commercial investment remain, including uncertainty over the ability to patent cell-based products, and the need for expensive development of Good Manufacturing Practice (GMP)-

59 Medicine Manufacturing Industry Partnership, [Advanced Therapies Manufacturing Action Plan](#), (November 2016)

60 Association of British Pharmaceutical Industry, [‘Action plan for UK to capture the next generation of medicines manufacturing jobs’](#), Press Release, November 2016

61 House of Commons Science and Technology Committee, Thirteenth Report of session 2016–17, [Industrial Strategy: science and STEM skills](#), HC 991

62 Department for Business, Energy and Industrial Strategy, [Business Secretary announces Industrial Strategy Challenge Fund investments](#), Press Release, April 2017

quality products before testing on patients. The British Heart Foundation emphasised that these must be addressed if regenerative medicine is to have a wide-reaching impact for patients.<sup>63</sup> NHS market access and reimbursement arrangements are perhaps, however, the most critical factor for manufacturers being able to supply treatments which are both profitable for the supplier and affordable for the healthcare system.<sup>64</sup> Keith Thompson from the Cell and Gene Therapy Catapult explained that there are business-related barriers in understanding the influence of ‘health economics’:

It is no good saying “my product is special, give me a special price”. The price is only ever going to be the health benefit and therefore you have to be able to make that product within what the reimbursement price is.<sup>65</sup>

35. We discuss the barriers to the NHS adoption of regenerative medicine therapies in more detail in Chapter 3.

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63 British Heart Foundation ([REG0012](#))

64 BioIndustry Association ([REG0022](#))

65 Q156

## 3 Adoption in the NHS

36. In this chapter, we look at the adoption of regenerative medicine in the NHS, examining the current infrastructure, the role of the National Institute for Health and Care Excellence and NHS England in taking regenerative medicine forward. We also examine the opportunities for regenerative medicine following the Accelerated Access Review and the Government's Industrial Strategy.

### NHS infrastructure

37. According to REGenerableMED (an ESRC-funded regenerative medicine project) the wider adoption of regenerative medicine therapies will require major, long-term infrastructure commitment alongside a responsive clinical demand-dependent product supply.<sup>66</sup> At present, adoption by the NHS has been most rapid for clinician-led surgical procedures whose development may require only minor adjustments to existing clinical practices, such as limbal stem cell transplantation<sup>67</sup> to restore corneal function. A key determinant has been how well the administration of particular cell therapies aligns with existing clinical treatment pathways.

38. The Scottish National Blood Transfusion Service voiced similar concerns: "No matter how good the science and manufacturing, if these products do not complement current clinical practice and harness existing infrastructure, implementation will be tortuous".<sup>68</sup> REGenerableMed explained that the emergence of potential complex advanced therapies and products (including medical devices and diagnostics) which do not have a clear regulatory and commercial route to their clinical use, highlights a need for regulation that can evolve in tandem with emerging scientific knowledge and new technologies.<sup>69</sup> Professor Paul Whiting from the Medical Research Council's Regenerative Medicine Research Committee explained the particular benefits of the NHS facilitating such an approach:

The UK is uniquely positioned to do long-term follow up, because of the NHS, where everybody has their identifier and people can get tracked through the system and monitored for a long time. That is not necessarily true, or largely not true, in many other places. That is an opportunity for us. [...] The earlier you are getting the NHS familiar with these types of treatments, how they are dealt with, how they are processed at pharmacy and how they are then delivered to patients, the better, because that is what the NHS is going to have to square up to as these treatments get closer to market anyway, and the earlier we can promote that innovation in the NHS at a clinical trial level, the easier it is going to be further down the line when these treatments start to get approved [...] and adopted in the NHS for patient benefit. The NHS is a resource that we have that is not replicated anywhere else in the world, so why don't we use it for that purpose?<sup>70</sup>

66 REGenerableMED ([REG0004](#))

67 Limbal stem cell transplantation involves the grafting of stem cells that have been taken from donor eyes and grown in tissue culture, with the aim of improving vision and other symptoms such as eye irritation and dryness.

68 Scottish National Blood Transfusion Service ([REG0015](#))

69 REGenerableMED ([REG0004](#))

70 Q114



39. The expansion of regenerative medicine as a scientific discipline—with its core principles of rejuvenation, regeneration and replacement (‘the 3Rs’)—is helping to drive a shift in healthcare from symptomatic to curative treatments. This is seen in an increase in clinical trials of regenerative medicine therapies.<sup>71</sup> Our witnesses, nevertheless, described difficulties they have experienced in doing clinical research within the NHS. There were concerns about the current infrastructure and capacity of the NHS that does not lend itself to doing regenerative medicine clinical trials research. Parkinson’s UK told us that:

The research culture in the NHS needs to improve to embrace research. Capacity needs to be increased across the NHS to allow health professionals time to carry out research and develop their skills [...] Many clinicians’ workloads do not allow them to have capacity to carry out research.<sup>72</sup>

40. The University of Bristol believed that “the system is not clear to the majority of researchers in regenerative medicine”. It wanted “a formal national regenerative medicine network” to be created, comprising researchers, industry, NHS and regulatory bodies, “to enhance synergy, sharing and cross-fertilisation across an area of science that is highly interdisciplinary”.<sup>73</sup>

41. The Academy of Medical Sciences also expressed concerns about the preparedness of the current healthcare system to adopt regenerative medicine therapies:

We need to consider the preparedness of the UK healthcare system, in terms of technical and clinical capacity as well as capability for the complexity of such therapies (including when running clinical trials) taking into account the cost implications. The technologies required for regenerative medicine are not current available through NHS laboratories and there will be significant training needs for all healthcare staff [...] To maximise cost-effectiveness and appropriately manage demand in the shorter term, there may be a case for accreditation of centres that wish to deliver regenerative medicine therapies, so that the appropriate infrastructure is in place.<sup>74</sup>

42. REGenerableMED highlighted that although NHS England has policy responsibility for healthcare ‘innovation’, regenerative medicine has not been explicitly mentioned in any its recent strategies such as the NHS Five Year Forward View or ‘sustainability and transformation plans’<sup>75</sup> Adoption of regenerative medicine varies by individual NHS Trusts.<sup>76</sup> Nevertheless, one of our NHS England witnesses reassured us that:

Regenerative medicine links very much to our strategy [...].That has been developed, and now we are going into implementation. We feel it meets our approach to the health and wellbeing gap and the link to the Five Year Forward View around precision and personalisation. For that [...] we need clear clinically-led decision making about making the right products available and put into practice for our patients; that we work with industry in general to ensure we can get the best innovative pricing around; [...] that

71 Caplan A.I, Mason C., Reeve B, “[The 3Rs of Cell Therapy](#)”, Stem Cells Translational Medicine, vol 6 issue 1 (2016)

72 Parkinson’s UK ([REG0005](#))

73 University of Bristol ([REG0002](#))-

74 Academy of Medical Sciences ([REG0014](#))

75 REGenerableMED ([REG0004](#))

76 REGenerableMED ([REG0004](#))

we make sure that, where they are commissioned, we get the uniformity of access across England that we would expect our patients to want; and importantly that we continue to collate the data we need to make sure that the treatments are delivering in the way they are expected to.<sup>77</sup>

43. Dr Sven Kili, head of Gene Therapy development from GlaxoSmithKline, voiced concerns regarding the NHS's capacity to take forward regenerative medicine:

Very often, you approach an NHS hospital, they are under staffed. The nursing staff who are working to support are so busy just taking care of patients that they do not often have the time to look after a study [...] We need a mechanism to make them better to be able to do clinical trial work.<sup>78</sup>

Professor Paul Whiting from the Medical Research Council's Regenerative Medicine Research Committee similarly told us that:

It is not easy to interact with the NHS very often. It is not obvious where the portal is, the entry point, for some things you want to do, and people are enormously conscious of the perceived or otherwise 'stress' that the NHS is under. That is, if we are not careful, going to build a reluctance to interact with the NHS.<sup>79</sup>

44. In July 2016, NHS England announced the results of its "clinical prioritisation process", and confirmed that funding would not be allocated for the routine commissioning of stem cell transplants.<sup>80</sup> While providing second stem cell transplants was recognised by NHS England as being clinically effective, the treatment was considered unaffordable relative to other treatments. According to the Anthony Nolan, "this decision represents a backwards step for the health service as second stem cell transplants were routinely available to patients prior to 2013".<sup>81</sup>

### **NICE Health Technology Appraisals and NHS Specialised Commissioning**

45. Regenerative medicine presents particular difficulties for the National Institute for Health and Care Excellence's (NICE's) technology appraisal process because while such therapies potentially confer substantial health gains, they can also be expensive on a per-patient basis and be supported by a weak evidence base. In response to recommendations set out by the Regenerative Medicine Expert Group, NICE published in March 2016 the findings of an independent appraisal of regenerative medicines and cell therapies.<sup>82</sup> It included a 'mock' appraisal of a hypothetical product<sup>83</sup> based on new, unlicensed cell therapies for treating a type of leukaemia in children.<sup>84</sup> Their report concluded that

77 Q192 [Dr Jonathan Fielden] (see also NHS England, [Board Paper - Strategic Framework for Specialised Services](#), May 2016)

78 Q103

79 Q117

80 NHS England, '[NHS England announces provisional investment decisions for specialised services](#)', press release, July 2016

81 Anthony Nolan Trust ([REG0031](#))

82 CRD/CHE University of York, [Exploring the assessment and appraisal of regenerative medicine and cell therapy products](#) (March 2016)

83 Chimeric Antigen Receptor (CAR) T-cell therapy

84 CRD/CHE University of York, [Exploring the assessment and appraisal of regenerative medicine and cell therapy products](#) (March 2016)

NICE's health technology appraisal methods could be applied to regenerative medicine and cell therapies,<sup>85</sup> but Advanced Therapy Medicinal Products (ATMP) developers still had some concerns:

- Some cell and gene therapies will not meet NICE's criteria for appraisal because of NICE's cost threshold or the challenges of managing 'immature data';
- Some cell and gene therapies will be for rare conditions with patient populations that are very small, in which case they will not be assessed by NICE. In these cases, therapies would have to be considered under specialised commissioning routes rather than through NHS commissioning, and their adoption will be determined at local rather than national NHS levels; and
- There is a perception that NICE's data requirements to tackle the uncertainty around novel cell and gene therapies may be disproportionately burdensome compared to traditional small-molecule or biological medicines.<sup>86</sup>

46. Subsequently, NICE and NHS England consulted on proposals in January 2017 to change the arrangements for evaluating drugs and other health technologies through NICE's 'technology appraisal' and 'highly specialised technologies' programmes.<sup>87</sup> The proposals include introducing a 'budget impact threshold' of £20 million, linking NICE and NHS England processes for evaluating highly specialised technologies and the introduction of a new 'fast track' appraisal. Michael Hunt of ReNuron told us that "companies like us spend a lot of time thinking or worrying about the ability of payers to pay for these treatments: how are they going to be paid for, are the existing mechanisms appropriate, in terms of NICE evaluation, health technology appraisals and so on".<sup>88</sup>

### **Payment and reimbursement**

47. In the England and Wales NHS, new healthcare therapies may be paid for (or 'reimbursed') in accordance with decisions at a local level via individual NHS Foundation Trusts and regional Clinical Commissioning Groups, or at a national level through a NICE Technology Appraisal.<sup>89</sup> In addition, NHS England is able to commission 'specialised services'.

48. Securing reimbursement in these ways was identified by our witnesses as a major challenge. Michael Hunt from ReNuron told us that manufacturers of regenerative medicine therapies have struggled to obtain reimbursement consistently across EU countries.<sup>90</sup> There remains substantial inconsistency in the uptake of the Health Technology Assessment (HTA) across the EU, despite proposals to improve coordination.<sup>91</sup> Questions raised about the suitability of existing HTA methodologies led NICE to undertake a review of the technology appraisal of a regenerative medicine therapy on the NHS. REGenerableMED saw a need to extend this analysis to a wider range of regenerative medicine therapies.

85 Ibid

86 BioIndustry Association ([REG0022](#))

87 NICE and NHS England, '[Consultation on changes to technology appraisals and highly specialised technologies](#)', March 2017

88 Q142

89 Health and Social Care Information Centre, '[NICE Technology Appraisals in the NHS in England Innovation Scorecard to September 2015](#)' (May 2016)

90 Q96

91 REGenerableMED, '[Regenerative Medicine in the UK: Reimbursement policy briefing](#)' (April 2016)

It recommended that priority should be given to gathering information on Clinical Commissioning Groups' position on regenerative medicine in service contracts and for further support to be given for coordination between the MHRA, NICE's Office for Market Access and NHS England.<sup>92</sup>

49. Michael Hunt from ReNuron told us that:

The biggest challenge that now remains, beyond garnering clinical data for proof-of-concept in man is getting these treatments adopted in the UK through the NHS in routine practice and, of course, paid for—'reimbursed'. How are we going to pay for treatments, especially where there is the potential for very significant long-term healthcare savings, quite apart from patient benefits in the long term that may come at a considerable up-front cost compared with more conventional treatments? That is the difficult equation that the field now needs to tackle and solve.<sup>93</sup>

50. Keith Thompson, Chief Executive from the Cell and Gene Therapy Catapult, emphasised that:

The next step in the deconstruction of all the barriers is to get more rapid access to the NHS and to join up the reimbursement challenge. Compared with Japan as a for instance, Japan's regulatory regime changed, it's fairly complex, but they gave earlier conditional licensing plus reimbursement. They get early access to patients and they get paid for it. Although global pharmaceutical firms probably take a more global view, certainly SMEs are very attracted to that and it is SMEs that come in, do the early work and put their roots down into complex supply chains that simply cannot be uprooted and moved elsewhere.<sup>94</sup>

**51. Having a universal NHS for all patients provides a receptive environment for the development and adoption of innovative and scientific advances in regenerative medicine. *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.***

**52. *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.***

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92 REGenerableMED ([REG0004](#))

93 Q96

94 Q154

## A strategy for regenerative medicine

53. The Accelerated Access Review was commissioned by the Department of Health and the former Department for Business Innovation and Skills in November 2014.<sup>95</sup> It made recommendations in October 2016 to make it easier for NHS patients to access innovative medicines, medical technologies, diagnostics and digital products, to improve patient outcomes.<sup>96</sup> The review recommended the creation of a new ‘accelerated access partnership’ to speed up and simplify the process for getting the most promising new treatments and diagnostics safely from pre-clinical development to patients.<sup>97</sup> The review concluded that accessing innovation in the NHS had become increasingly challenging. Through the proposed new partnership of NHS England, NHS Improvement, NICE and the MHRA, innovators would be able to access joined-up help for clinical development, regulation and assessment of cost-effectiveness.<sup>98</sup>

54. Innovate UK hoped that the review would make further recommendations that would speed up the adoption of ATMPs (paragraph 30) so that patients will benefit from these treatments earlier.<sup>99</sup> We are still awaiting the Government’s response to the recommendations set out in the Accelerated Access Review.

55. There is no explicit mention of regenerative medicine in NHS England’s recently updated *Five Year Forward View*,<sup>100</sup> although there was a focus on innovation and the likely opportunities in the life sciences in the Government’s Industrial Strategy.<sup>101</sup> NHS England published its Personalised Medicine strategy in September 2016, in which personalised medicine was defined as “a move away from ‘one size fits all’ approach to the treatment and care of patients with a particular condition, to one which uses new approaches to better manage patients’ health and targets therapies to achieve the best outcomes in the management of patients’ disease or predisposition to disease”.<sup>102</sup> This strategy had a strong focus on informatics and genomics, but there was no mention of regenerative medicine. Dr James Palmer told us that regenerative medicine was “so interlinked [with personalised medicine] that it is right that the NHS has focused on the genomics side first, because the regenerative medicine side is still a way away from those big transformative curative treatments; they are not coming through thick and fast”.<sup>103</sup>

**56. The Regenerative Medicine Expert Group provided recommendations to Government to develop an NHS regenerative medicine strategy to deliver innovative treatments. While the Government has responded and made progress through investment in further research and in setting up the Cell and Gene Therapy Catapult, there is still more work to be done. A strategy is needed that covers the entire value**

95 Independent review with support from the Wellcome Trust, [Accelerated Access Review: Final Report, Review of innovative medicines and medical technologies](#), October 2016

96 Ibid

97 Independent review with support from the Wellcome Trust, [Accelerated Access Review: Final Report, Review of innovative medicines and medical technologies](#), October 2016

98 Ibid

99 Innovate UK ([REG0013](#))

100 NHS England, [Five Year Forward View](#) (March 2017)

101 Department for Business, Energy & Industrial Strategy, [Building our Industrial Strategy—Green Paper](#) (January 2017)

102 NHS England, [Improving outcomes through personalised medicine](#) (September 2016)

103 Q221

chain—academic research, commercial development and clinical application—if the UK is to respond to the challenges of our healthcare system as well as facilitate economic growth.

*57. 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.*

*58. 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.*

# Conclusions and recommendations

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## Research and commercialisation

1. Regenerative medicine provides a unique approach to treating diseases and disorders by providing the body itself with the means to repair, replace, restore and regenerate damaged or diseased cells, tissues and organs. Its continual development depends crucially on a strong foundation of basic scientific research. *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)
2. It is important that the regulatory environment for regenerative medicine remains flexible to accommodate new and diverse approaches while also maintaining robust review processes to ensure that the most promising, effective and safe therapies are made available to patients. The MHRA have taken forward the Regenerative Medicine Expert Group's recommendations on providing a central focal point for regulatory advice, but there is more to be done. (Paragraph 25)
3. *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)
4. The UK life sciences sector is a pioneer in the clinical development of new regenerative medicine therapies and well placed to create new high-tech high-value manufacturing businesses around these advanced therapies. Regenerative medicine researchers, however, need manufacturing support for 'translating' their work, and the Cell and Gene Therapy Catapult is working to bridge the gap between 'translational' research and commercialisation. *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)

## Adoption in the NHS

5. Having a universal NHS for all patients provides a receptive environment for the development and adoption of innovative and scientific advances in regenerative medicine. *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)

6. *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)*
7. The Regenerative Medicine Expert Group provided recommendations to Government to develop an NHS regenerative medicine strategy to deliver innovative treatments. While the Government has responded and made progress through investment in further research and in setting up the Cell and Gene Therapy Catapult, there is still more work to be done. A strategy is needed that covers the entire value chain—academic research, commercial development and clinical application—if the UK is to respond to the challenges of our healthcare system as well as facilitate economic growth. (Paragraph 56)
8. *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)*
9. *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)*



## Annex: Visit to UCL and Centre for Cell Gene and Tissue Therapeutics, Royal Free Hospital

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- 1) On Tuesday 29 November 2016, the Chair and Carol Monaghan visited the *UCL Institute of Ophthalmology*. The UCL Institute of Ophthalmology is working in partnership with Moorfields Eye Hospital.
- 2) Following a tour of the stem cell laboratories, they had the opportunity to speak to Professor Pete Coffey and Professor Robin Ali about their MRC-funded research in gene and stem cell therapy.
- 3) Robin Ali is Professor of Human Molecular Genetics at UCL Institute of Ophthalmology. The main focus of Professor Ali's research is the development of gene and cell therapy for the treatment of retinal disorders. As chief investigator, he established the world's first clinical trial of gene therapy for retinopathy. The results from this trial reporting an improvement in vision along with results from two other trials, established proof-of-concept for gene therapy for inherited retinal degeneration. His group has also provided the first proof-of-concept for effective transplantation of photoreceptors that has provided the basis for ES cell-derived photoreceptor transplantation, now a major programme in his laboratory.
- 4) Professor Coffey is director of the London Project to Cure Blindness and Professor of Cellular Therapy and Visual Sciences at the Institute of Ophthalmology, University College of London (UCL) and is an MRC funded researcher.
- 5) In September 2015, a pioneering trial of a new treatment derived from stem cells for people with 'wet' age-related macular degeneration (AMD) commenced at Moorfields Eye Hospital following a successful operation on a patient. This first operation was a major milestone in the London Project to Cure Blindness, which was established 10 years ago with the aim of curing vision loss in patients with wet AMD, and is the result of a partnership between the hospital, the UCL Institute of Ophthalmology, and the National Institute for Health Research (NIHR). Pfizer Inc. joined the partnership in 2009 with the goal of helping to turn the original idea into a potential therapy.
- 6) Professor Coffey's team are transforming cells taken from skin biopsies into stem cells. These stem cells will be converted into eye cells that will be transplanted back into patients' eyes to preserve their sight. In the recent clinical trial for the treatment of dry age-related macular degeneration, these cells have been grown on a membrane which is then inserted into the eye as a patch of cells.
- 7) On Tuesday 13 December 2016, the Chair visited the *Centre for Cell Gene and Tissue Therapeutics (CCGTT)* at the Royal Free Hospital in London. A tour of the CCGTT manufacturing suites and process development laboratories was provided by Professor Martin Birchall and Professor Mark Lowdell. The CCGTT is a manufacturing and development facility for Advanced Therapeutic Medicinal Products regulated by both the Human Tissue Authority and the MHRA. The cell manufacturing labs allow scientists at the hospital to create or modify cells which can be used to treat a range of conditions,

including lung cancer, haemophilia and macular degeneration, using state of the art platform technologies. The suite is utilised by both academic and industrial partners, bridging the gap between basic research and manufactured medicinal products.

8) In 2008, MRC-funded researchers at University College London carried out the first transplant of a human trachea (wind pipe) reconstructed using stem cells. By 2013, the group were ready to build on this success by developing the first clinical trials of a stem cell-derived larynx transplant in a project known as “RegenVOX”. The RegenVOX procedure involves preparing a reconstructed larynx made from the patient’s own stem cells and a donor larynx. The team removes the cells from the donor larynx, leaving behind a scaffold onto which the patient’s stem cells are grafted. This means that the new larynx will not be rejected by the immune system so patients do not need immunosuppressant medication.

9) On both visits, the challenges for securing funding for ‘translational’ research were raised. Such programmes require significant and sustained funding. Funding for research is available from the MRC and research charities, and Innovate UK supported developmental stages and industry-led work. Company and venture capital funding can be more difficult to secure than from other biomedical drug development activities due to uncertainties over the business model and challenges in product development. A major challenge is sustaining funding support for the critical GMP facilities needed to underpin product development and testing, which is expensive. Ideally, long term support needed to be made available to underpin facilities with costs recovered through individual projects, potentially with commercial partners.

# Glossary of terms/abbreviations used in report

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## General terms

**Cell therapy (cellular therapeutics, cell-based therapies):** administration of cells to the body to the benefit of the recipient

**Gene Modified Immune Therapies/Ex vivo gene therapy:** cells from the patient's blood or bone marrow are removed and grown in a laboratory

**Gene therapy:** deliberate introduction of genetic material into cells

**Limbal stem cell transplantation:** involves the grafting of stem cells that have been taken from donor eyes and grown in tissue culture, with the aim of improving vision and other symptoms such as eye irritation and dryness.

**Regenerative medicine:** replaces or regenerate human cells, tissues or organs to restore or establish normal function

**Tissue engineering:** use of a combination of cells, engineering, materials and methods ex-vivo living tissues and organs that can be implanted to improve or replace biological functions.

**Transplantation:** process of implanting cells, tissues or organs.

## Non-cellular components

**Biomaterial:** material intended to interface with biological systems to evaluate, treat, augment or replace any tissue, organ or function of the body.

## Manufacturing and production

**Good Manufacturing Practice (GMP)** is a system for ensuring that products are consistently produced and controlled according to quality standards. It is designed to minimise the risks involved in any pharmaceutical production that cannot be eliminated through testing the final product.

**Marketing authorisation:** authorisation by a European regulatory authority for a medicinal product to be placed on the market.

**Translation:** active turning of a basic science discovery into a safe and effective therapy deployed in routine clinical practice.

## Regulatory terms for products and therapies

**Advanced therapy medicinal product (ATMP):** medicinal product for human use that is a gene therapy product, medicinal product, a somatic cell therapy medicinal product or tissue engineered product.

**Health Technology Assessment (HTA):** refers to the systematic evaluation of properties, effects and/or impacts of health technology. It is a multidisciplinary process to evaluate the social, economic, organisational and ethical issues of a health intervention or health technology.

## Organisations/reviews

**Accelerated Access Review** was announced in November 2014 by the then Minister for Life Sciences, George Freeman MP. Its aim is to speed up access to innovative drugs, devices, diagnostics and digital products for NHS patients.

**Advanced Therapy Manufacturing Taskforce (ATMT)** was launched by the Medicines Manufacturing Industry Partnership to secure the future of manufacturing advanced therapies in the UK.

**Cell and Gene Therapy Catapult** was established in 2012 as an independent centre of excellence to advance the growth of the UK cell and gene therapy industry, by bridging the gap between scientific research and full scale commercialisation.

**Medicines Manufacturing Industry Partnership (MMIP)** represents the voice of medicine manufacturers in the UK. It was established jointly by Government and the biopharmaceutical industry in 2014 to ensure that the UK is recognised by the global medicines industry as a world-class, advanced centre for medicines manufacturing.

**Regenerative Medicine Expert Group (RMEG)** is an expert group formed following publication of the Lords Science and Technology Committee report on regenerative medicine. Their remit is to develop an NHS regenerative medicine strategy so that the NHS is fully prepared to deliver innovative treatments as well as also assess the effect of regulation on the development of regenerative medicines in the UK.

**UK Cell Therapy Manufacturing Centre:** the Cell and Gene Therapy Catapult will be building a £55 million large scale GMP manufacturing centre in Stevenage to help bring cell and gene therapies to market in the UK and internationally. The centre is due to be opened in 2018.

**UK Regenerative Medicine Platform (UKRMP):** a £25 million initiative to address regenerative medicine translational challenges. The UKRMP was established in 2013 by the Biotechnology and Biological Sciences Research Council, the Engineering and Physical Sciences Research Council and the Medical Research Council. The UKRMP provides academic expertise, innovation and knowledge, connected to commercial and clinical end-users.

# Formal Minutes

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**Wednesday 26 April 2017**

Members present:

Stephen Metcalfe, in the Chair

Victoria Borwick      Graham Stringer

Jim Dowd              Derek Thomas

Chris Green          Matt Warman

Carol Monaghan

Draft Report (*Regenerative medicine*), proposed by the Chair, brought up and read.

*Ordered*, That the draft Report be read a second time, paragraph by paragraph.

Paragraphs 1 to 58 read and agreed to.

Summary, Annex and Glossary agreed to.

*Resolved*, That the Report be the Fifteenth Report of the Committee to the House.

*Ordered*, That the Chair make the Report to the House.

*Ordered*, That embargoed copies of the Report be made available, in accordance with the provisions of Standing Order No. 134.

[The Committee adjourned

## Witnesses

The following witnesses gave evidence. Transcripts can be viewed on the [inquiry publications page](#) of the Committee's website.

### Tuesday 19 July 2016

*Question number*

**Professor Paul Riley**, British Heart Foundation Professor of Regenerative Medicine, University of Oxford, **Professor Stuart Forbes**, Professor of Transplantation and Regenerative Medicine, University of Edinburgh, and **Professor Peter Andrews**, Professor of Biomedical Science, University of Sheffield

[Q1–52](#)

**Dr Rob Buckle**, Director of UK Regenerative Medicine Platform, Research Councils UK, **Professor Jeremy Pearson**, Associate Medical Director, British Heart Foundation, **Professor Neil Hanley**, Professor of Medicine, University of Manchester, and **Professor Anthony Hollander**, Head of Institute of Integrative Biology and Professor of Stem Cell Biology, University of Liverpool

[Q53–91](#)

### Wednesday 19 October 2016

**Dr Ian Hudson**, Chief Executive, Medicines and Healthcare products Regulatory Agency, **Professor Paul Whiting**, Member, Medical Research Council Regenerative Medicine Research Committee, **Dr Sven Kili**, Head of Gene Therapy Development, GlaxoSmithKline, and **Michael Hunt**, Chief Financial Officer, ReNeuron Group

[Q92–145](#)

**Ian Trenholm**, Chief Executive, NHS Blood and Transplant, **Ian McCubbin**, Medicines Manufacturing Industry Partnership and co-chair of the Advanced Therapy Manufacturing Taskforce, **Keith Thompson**, Chief Executive Officer, Cell and Gene Therapy Catapult, and **Dr Ruth McKernan**, Chief Executive, Innovate UK

[Q146–190](#)

### Wednesday 7 December 2016

**Dr Nick Crabb**, Programme Director, Scientific Affairs, National Institute for Health and Care Excellence, **Dr Jonathan Fielden**, Director of Specialised Commissioning and Deputy National Medical Director, NHS England, and **Dr James Palmer**, Clinical Director of Specialised Commissioning, NHS England

[Q191–226](#)

**Dr Anthony Mathur**, Professor of Cardiology, Queen Mary's School of Medicine and Dentistry, **Professor Amit Nathwani**, Professor of Haematology, Royal Free Hospital, and **Professor Giovanna Lombardi**, Professor of Human Transplant Immunology, King's College London

[Q227–268](#)

**Lord Prior of Brampton**, Parliamentary Under-Secretary of State for Health, Department of Health

[Q269–312](#)

## Published written evidence

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The following written evidence was received and can be viewed on the [inquiry publications page](#) of the Committee's website.

REG numbers are generated by the evidence processing system and so may not be complete.

- 1 Anthony Nolan ([REG0021](#)) and ([REG0031](#))
- 2 AstraZeneca ([REG0024](#))
- 3 BioIndustry Association (BIA) ([REG0022](#))
- 4 British Heart Foundation ([REG0012](#))
- 5 Centre for Doctoral Training in Regenerative Medicine ([REG0006](#))
- 6 Centre for Doctoral Training in Regenerative Medicine, University of Manchester ([REG0033](#))
- 7 Charlotte Lozier Institute ([REG0023](#))
- 8 Department of Health ([REG0007](#)) and ([REG0032](#))
- 9 Dr Navid Malik ([REG0019](#))
- 10 Dr Paul Lewis ([REG0011](#))
- 11 Genetic Alliance UK ([REG0008](#))
- 12 GSK ([REG0027](#))
- 13 Human Tissue Authority (HTA) ([REG0016](#))
- 14 Innovate UK ([REG0013](#))
- 15 Mr Christopher Roy-Toole ([REG0001](#))
- 16 National Institute for Health and Care Excellence (NICE) ([REG0009](#))
- 17 NHS Blood and Transplant ([REG0003](#))
- 18 Parkinson's UK ([REG0005](#))
- 19 PHG Foundation ([REG0017](#))
- 20 REGenableMED ([REG0004](#))
- 21 Research Councils UK ([REG0028](#))
- 22 Scottish National Blood Transfusion Service ([REG0015](#))
- 23 The Academy of Medical Sciences ([REG0014](#))
- 24 The Association of the British Pharmaceutical Industry ([REG0020](#))
- 25 The Cell and Gene Therapy Catapult ([REG0026](#))
- 26 The Humanimal Trust ([REG0010](#))
- 27 The Medicines and Healthcare products Regulatory Agency ([REG0025](#))
- 28 University of Bristol ([REG0002](#))
- 29 Wellcome Trust ([REG0018](#))

## List of Reports from the Committee during the current Parliament

All publications from the Committee are available on the [publications page](#) of the Committee's website.

The reference number of the Government's response to each Report is printed in brackets after the HC printing number.

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First Report	EU regulation of the life sciences	HC 158
Second Report	Digital skills crisis	HC 270 (HC 936)
Third Report	Satellites and space	HC 160 (HC 830)
Fourth Report	Forensic Science Strategy	HC 501 (HC 845)
Fifth Report	Robotics and artificial intelligence	HC 145 (HC 896)
Sixth Report	Evidence Check: Smart metering of electricity and gas	HC 161 (HC 846)
Seventh Report	Leaving the EU: implications and opportunities for science and research	HC 502 (HC 1015)
Eighth Report	Setting up UK Research & Innovation	HC 671 (HC 1063)
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Second Special Report	Forensic Science Strategy: Government Response to the Committee's Fourth Report of Session 2016–17	HC 845
Third Special Report	Evidence Check: Smart metering of electricity and gas: Government Response to the Committee's Sixth Report of Session 2016–17	HC 846
Fourth Special Report	Digital skills crisis: Government Response to the Committee's Second Report of Session 2016–17	HC 936
Fifth Special Report	Robotics and artificial intelligence: Government Response to the Committee's Fifth Report of Session 2016–17	HC 896



Sixth Special Report	Leaving the EU: implications and opportunities for science and research: Government Response to the Committee's Seventh Report	HC 1015
Seventh Special Report	Setting up UK Research & Innovation: Government Response to the Committee's Eighth Report	HC 1063

### Session 2015–2016

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Second Report	Science in emergencies: UK lessons from Ebola	HC 469 (Cm 9236)
Third Report	Investigatory Powers Bill: technology issues	HC 573 (Cm 9219)
Fourth Report	The big data dilemma	HC 468 (HC 992)
First Special Report	Royal Botanic Gardens, Kew: Government Response to the Committee's Seventh Report of Session 2014–15	HC 454
Second Special Report	Current and future uses of biometric data and technologies: Government Response to the Committee's Sixth Report of Session 2014–15	HC 455
Third Special Report	Advanced genetic techniques for crop improvement: regulation, risk and precaution: Government Response to the Committee's Fifth Report of Session 2014–15	HC 519
Fourth Special Report	The science budget: Government Response to the Committee's First Report of Session 2015–16	HC 729
Fifth Special Report	The big data dilemma: Government Response to the Committee's Fourth Report of Session 2015–16	HC 992