

A microscopic image of cells, likely oocytes, showing their internal structure and nuclei. The cells are arranged in a grid-like pattern, with some showing prominent nuclei and others showing more granular cytoplasm. The colors are primarily purple, blue, and green, with some yellow and orange highlights.

CATAPULT

Cell and Gene Therapy

Response to the House of Lords Inquiry – Life Sciences and the Industrial Strategy

September 2017

ABOUT THE CELL & GENE THERAPY CATAPULT

The Cell and Gene Therapy Catapult (CGT Catapult) was established in 2012 to grow the UK cell and gene therapy industry by bridging the gap between scientific research and full-scale commercialisation.

Our purpose

Growing a UK cell and gene therapy industry delivering health and wealth.

Our vision

Our vision is for the UK to be a global leader in the development, delivery and commercialisation of cell and gene therapy; where businesses can start, grow and confidently develop advanced therapies, delivering them to patients rapidly, efficiently and effectively.

Our mission

Our mission is to grow the industry in the UK to substantial and sustainable levels by:

- **Taking products into clinical trial**, de-risking them for further investment;
- Developing **Platform Technologies** that tackle industry issues;
- Developing **Infrastructure projects** to catalyse the development of manufacturing and anchoring those benefits in the UK;
- **Influencing stakeholders** to improve the environment for innovation and adoption;
- Providing **clinical expertise** and access to NHS clinical partners;
- Providing **technical expertise** and infrastructure to ensure products can be made to GMP and delivered cost effectively;
- Providing **regulatory expertise** to ensure that products can get to the clinic safely, in the shortest amount of time;
- Providing **reimbursement expertise** which accelerates the pathway to economic return;
- Providing **opportunities for collaboration**, both nationally and globally; and
- Providing access to **business expertise**, facilitating access to finance so that commercially viable products are progressed and investable propositions are generated.

SUMMARY

The UK is globally recognised as a world leader in the life sciences, which is a major contributor to the UK economy generating £64 billion in turnover and supporting more than 233,000 jobs. The UK has long identified the development of cell and gene therapies as an area where the science base is particularly strong. A priority area for the UK, the industry has been supported by the CGT Catapult and targeted investment through Innovate UK competitions. Cell and gene therapies provide a unique opportunity for the UK and continued Government investment leveraging Catapult assets and expertise, as a central part of the Life Sciences Industrial Strategy (LSIS), will ensure the UK captures a much larger share of the value chain and global revenues.

CGT Catapult welcomes the Government's commitment to build a modern LSIS that actively backs business, builds on the UK's strategic strengths and raises productivity to meet tomorrow's challenges and build a stronger and fairer economy that works across the UK. We believe it is vital that the UK maintains and develops an effective life science translational infrastructure to be globally competitive in the field of cell and gene therapy.

CGT Catapult has proactively engaged in the consultation on the LSIS launched by Professor Sir John Bell, to increase government investment in science research and innovation, particularly in the field of leading edge healthcare and medicine. We have also provided our views on the short and long term areas of focus for the £270 million allocation to the LSIS Challenge Fund announced in the Spring Budget. We believe continued investment in the cell and gene therapy industry will make the UK the "go to" place for the development of cell and gene therapies for supply to the global market.

The UK Cell and Gene Therapy Industry

Cell and gene therapies offer novel treatments by repairing, replacing, regenerating or re-engineering genes, cells and tissues. They offer a radical change in the treatment of often-incurable conditions and diseases but also represent a revolutionary new paradigm in science and in the way that these advanced treatments are turned into medicines that can be used in healthcare systems such as the NHS.

Cell and gene therapy is an important example of a sector, which was nascent until only a few years ago. Thanks to more than twenty years of investment in basic science by the UK research community and now complemented with innovation infrastructure such as the CGT Catapult, the UK has a critical mass of world leading research scientists in the field of cell and gene therapy. Over the last four years, the UK industry has grown rapidly and now has over 60 therapy developers predominately spinouts from UK academic institutions, some of which have received investment from patient capital investors. This rate of growth is demonstrably faster than our European neighbours. The UK's disproportionate share of current global activity has been achieved by a collaborative and co-ordinated network of interested institutions and bodies that recognise the importance of cell and gene therapy to UK patients and UK industrial growth.

In contrast to 2012 when CGT Catapult was launched, companies such as GSK and AstraZeneca are now quoting cell and gene therapy as the next pillar in their healthcare offering and a source of future growth and profitability. A recent cell and gene therapy market forecast prepared by the Association of the British Pharmaceutical Industry (ABPI) estimates a global market size that ranges from \$14bn - \$21bn per annum by 2025. We believe that with continued investment in the sector the UK can increase its disproportionate share of the global market to 15% which equates to £10bn of revenue (predominately exports) and 18,000 jobs in 2035.

Recommendations

Despite the UK's early successes there remain several barriers to commercialisation of cell and gene therapy technologies and continued intervention is required to ensure the cell and gene therapy industry takes off quickly and becomes firmly established in the UK, to yield a return on government's investment to date and to drive maximum economic impact. On this basis, CGT

Catapult highlights the importance of Government, through its LSIS, acting on the series of recommended actions set out in the Advanced Therapies Manufacturing Action Plan to retain and attract advanced therapies manufacture in the UK. In response to this inquiry, we make the following recommendations:

- **Science and innovation**

- Government should maintain science and innovation funding to support industry developing cutting-edge technologies.
- The UK cell and gene therapy industry is supported by a small pool of knowledgeable specialist investors and there is a lack of scale-up capital to support the growth of SMEs commercialising cell and gene technologies. This situation poses a risk to the long-term stability of the industry.
- UK life sciences research is globally competitive and highly productive and compared to European countries has a strong fiscal climate. However, Government needs to increase access to venture capital funding making it comparable to the US. This could be achieved through the British Business Bank and the proposed National Investment Fund providing access to scale-up capital through established and up-and-coming specialist fund managers.
- Government needs to strengthen and secure an internationally competitive fiscal landscape to attract investment to the UK.
- Government and industry can do more to support the efforts of University Technology Transfer Offices (TTOs).

- **Industrial Strategy**

- CGT Catapult supports the LSIS' vision to build the UK "life sciences industry into a global hub", which echoes our vision for the UK to be a global leader in the development, delivery and commercialisation of cell and gene therapy, where businesses can start, grow and confidently develop therapies.
- Cross-departmental alignment, ministerial leadership, NHS buy-in, and investment is required for the recently published LSIS to be successful.
- Government should target and capture internationally mobile investments through a proactive and simplified process of engagement.
- We welcome the focus on SME growth as a core aim of the LSIS as these businesses are key to the establishment of the cell and gene therapy industry.
- We highlight the importance of the LSIS leveraging the expertise and assets within Catapult Centres to accelerate the commercialisation and technology enablement of SMEs.
- The generation of measurable economic, health and well-being impacts should be the basis on which success of the LSIS is measured.

- **NHS procurement and collaboration**

- The LSIS correctly identifies the Government's privileged position as a purchaser to create a pull for healthcare innovation and support the supply chain.
- Despite Government initiatives, NHS uptake of new treatments remains low and slow. This situation has to be addressed by developing robust accountability structures in government and the NHS.
- Ring fenced time limited funding should be made available to stimulate the adoption of innovative advanced therapies by the NHS.

- **Responsibility and accountability**

- Ministerial leadership, alignment and buy in across-departments and the NHS, and investment from both government and industry is required for successful implementation of the LSIS.

- **Brexit**

- Government needs to ensure that post Brexit there are mechanisms in place to:
 - Secure long-term, predictable funding for scientific research and collaborations at scale internationally
 - Access export markets and allow easy movement of goods and capital across borders
 - Establish a globally competitive regulatory environment in the UK and for the Medicines & Healthcare Products Regulatory Agency (MHRA) to lead in global standards
 - Support the scientific activities and international outreach of the National Institute for Biological Standards and Control (NIBSC)
 - Access the best European and global talent

RESPONSE

A. Science and Innovation

1. *How can investors be encouraged to invest in turning basic life science research into new innovations in treatment? Why has investment been lacking in this sector? Does the research base have the necessary infrastructure to be world-leading?*
 - Public funding of research and innovation in universities and businesses is key to securing continued strength in the UK life sciences industry. Government working alongside industry should continue to invest in innovation in manufacturing technology development and capability through existing mechanisms such as Innovate UK Collaborate Research and Development Grants, Biomedical Catalyst and UK Research Council funding and. The Biomedical Catalyst and similar collaborative R&D models are a proven highly effective mechanism for supporting SMEs and should feature prominently in the LSIS.
 - Research shows that two – thirds of Gross Expenditure on R&D (GERD) in the UK is by businesses therefore incentives for private investment should be a strong focus for the LSIS.
 - Companies particularly small and micro-enterprises in the UK cell and gene therapy industry face several challenges:
 - Accessing funding to develop their products and technologies is difficult
 - A long and complex development pathway from early research and development work through to a final product
 - Complying with complex regulatory requirements as well as difficulties in getting therapies adopted by the healthcare system
 - A limited number of knowledgeable investors who understand cell and gene therapies and have the skills to support a SME throughout its growth
 - The combination of factors mean that investors may be reluctant to support untested or unproven approaches until they are relatively close to market and more easily valued.
 - The need to overcome the imperfections in capital markets that inhibit the availability of finance for emerging industries such as cell and gene therapy, justifies the provision of support for R&D expenditure to both address financial market constraints and de-risk projects.
 - In this context, CGT Catapult welcomes the Patient Capital Review that was announced in November 2016. We look forward to seeing any recommendations that could improve the availability of long-term capital for growing innovative cell and gene therapy firms, and state that our industry needs to see a step-change in the quantum of patient capital available to businesses.
 - We support the full implementation of the recommendations in the Advanced Therapies Manufacturing Action Plan, which promote R&D tax credits, investor tax incentives and venture capital corner stoning by the Government as key aspects of public support for research and innovation.
 - We also encourage Government to adopt a flexible approach in applying and interpreting State Aid rules.
 - Creating the right institutions to bring together sectors and places. This is where the Catapults as a network are at their strongest; in some sense, it is their very raison d'être. Catapults are located across the UK, building on clusters of scientific and industrial expertise to strengthen local innovation ecosystems.
 - It is important that Catapults remain focused on sectors, which offer the greatest opportunities to deliver long-term sustainable high value economic growth.

- Catapults are a complex intervention and require long-term, sustained and, most importantly, consistent investment by Government. They should be given time to achieve impact.
- 2. *Why has the UK underperformed in turning basic research in the life sciences into intellectual property? What needs to be done to address this historic weakness in the UK and grow new companies to commercialise new research and related technologies in the life sciences?*
 - There are many excellent Technology Transfer Offices (TTOs) in the UK, but there is inconsistency across the ecosystem, and only a handful have expertise in cell and gene therapy technologies. There is a need for greater sharing of best practice and adoption of a more open market model. We believe that the measurement of TTOs and the university system should be appropriately balanced towards both short and long-term measures that reflect both income to the universities and impact on the economy. We encourage the interaction of TTOs with CGT Catapult to enable spinouts to accelerate or become investable earlier.
 - The innovation budget targeted at industrial uptake and development should be increased as part of the new science and innovation funding settlement. Elements of that funding should be targeted to address barriers and market failures within sectors such as the advanced therapy industry.
- 3. *What can be done to ensure the UK has the necessary skills and manpower to build a world-class life sciences sector, both within the research base and the NHS?*
 - The growth of the cell and gene therapy sector in the UK will critically rely on a commensurate growth in the available talent pool.
 - A conservative estimate is that 400-600 additional skilled staff will be required over the next two years. Currently many cell and gene therapies are in an experimental phase and manufacturing processes are largely underdeveloped and small-scale. As a result, the skills and knowledge requirements currently associated with cell and gene therapies are highly specialised (often post-doctoral) and multi-functional.
 - As the industry matures and begins to commercialise products, the highest growth in skills and knowledge demand will not be in this highly expert group but increasingly in competent technicians or operators capable of reliably running routine manufacturing operations. In addition, specialised roles such as Qualified Person (QP) and regulatory professionals will grow.
 - In response to the immediate needs of industry, CGT Catapult has adopted the process of “training through immersion” whereby a 2-3 year stint in CGTC makes the individual highly valuable to industry, especially in our high growth sector where skill deficits are very acute.
 - CGT Catapult currently employs 140 people but has already had over 40 people from it move into industry. This turnover of staff covers the full range of expertise from scientists and regulatory experts through programme managers and senior managers. Whilst this has been difficult for us to manage, it is creating a reputation in industry that CGT Catapult is a place to recruit industry ready experts. CGT Catapult’s staff turnover is insufficient to feed the 1,500 new jobs we expect the cell and gene industry to create by 2023.
 - The longest established parts of the Catapult network exemplified by the High Value Manufacturing Catapult (HVMC) have a deep skills agenda around the development of advanced manufacturing skills (advanced apprentices, degree apprentices and re-skilling).

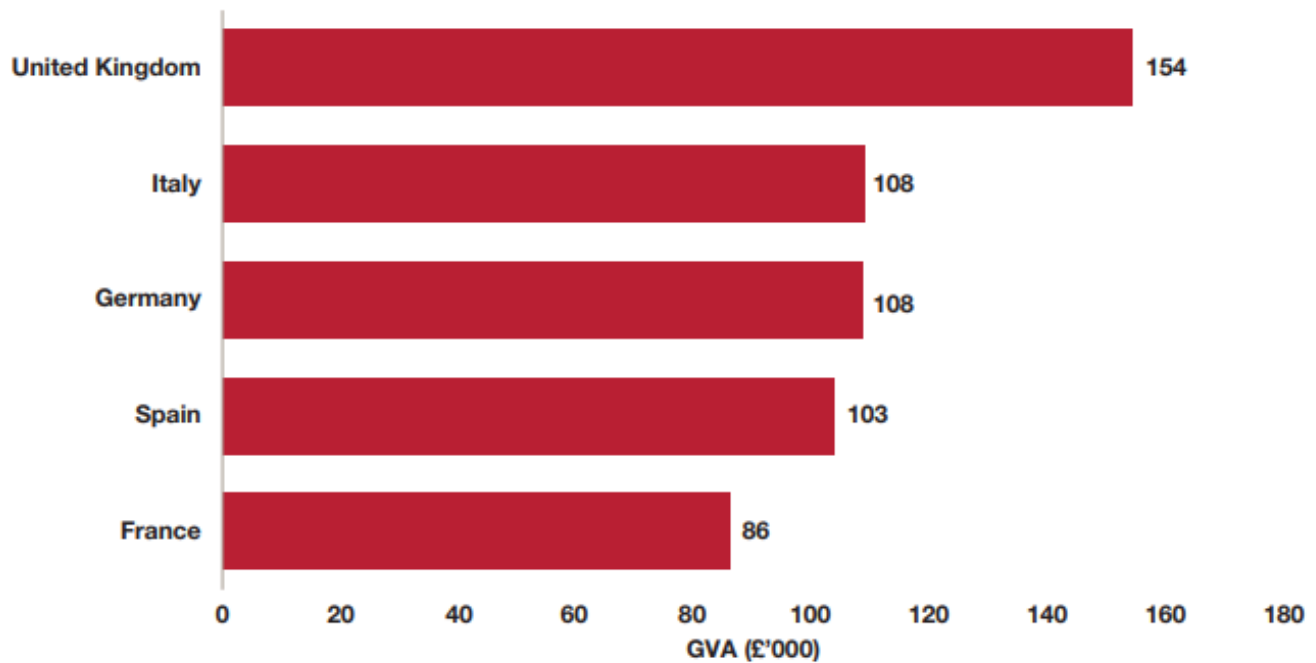
HVMC are working on this with industry to deliver impactful schemes. HVMC has two major training centres of excellence so far with 750 apprentices in training. This model seems ripe for duplication, and CGT Catapult is looking at how it can learn from and apply what has worked so well at HVMC in the cell and gene therapy sector.

- We recommend Government implement the Advanced Therapies Manufacturing Action Plan on skills and training with seed funding of £1.5 million to catalyse the successful set-up and coordination of an end-to-end talent management plan.

4. How does the UK compare to other countries in this sector, for example Germany and the United States?

- There are an estimated 822 regenerative medicine companies in the world: 455 are in North America, 214 in Europe and Israel, of which +60 (30%) advanced therapy companies are in the UK. (Source: Alliance for Regenerative Medicine Q2 2017 report and CGT Catapult database)
- The global regenerative medicine industry attracted \$20.6bn (£13.6Bn) of investment between 2014 and 2016. The lion's share of this investment has gone to companies in the US. (Source: Alliance for Regenerative Medicine full year reports 2014 – 2016)
- Between Jan 2013 – June 2017 advanced therapy companies in the UK received an estimated £1.3Bn in investment. (Source: CGT Catapult and Crunchbase databases)
- The UK has long identified the development of cell and gene therapies as an area where the science base is particularly strong. A priority area for the UK, the sector has been supported by the CGT Catapult and targeted investment through Innovate UK competitions. Data from Grant Thornton on the number of cell therapy manufacturing facilities shows that the UK holds the single largest share of the cell therapy manufacturing market with 44% of European facilities. This data also suggests that in Europe, UK Life Sciences companies are responsible for a higher proportion of high-value deals than their European counterparts, between 2006 and 2016, 81 UK companies were involved in deals compared to only 37 in Germany and 27 in France.
- The unique status of CGT Catapult as a private, independent business led by industry professionals is the critical differentiator from the broader network of academic institutions, as it ensures that we operate at the demand end of technology commercialisation. This is still a new mechanism for the UK, and indeed the world, but it is already clear it makes us very complementary partners for the existing actors in the landscape, including those linked to universities and research councils. Catapult centres are unique delivery vehicles in the UK, they provide valuable world beating facilities that many SMEs could not afford on their own. It is important that their services are maintained as world leading and relevant to the needs of industry and promoted widely to SMEs.
- The LSIS provides detailed evidence of the UK strength in basic life sciences where the nation has a strong international reputation. The report also notes that despite lower overall investment in R&D in life sciences than either Germany or the US the research community in the UK is twice as productive as in the US and almost three times greater than in Germany.
- The commercial life sciences sector in the UK remains significant and is one of the most productive sectors of the UK economy. Data from PWC shows the average direct GVA per employee in UK life sciences (£104k) is over twice the UK average (£49k) and UK pharmaceutical manufacturing has the highest GVA per employee of the major European nations.

Direct GVA per employee of manufacture of pharmaceutical products, current prices, 2014 £'000'



Sources: PwC analysis, Eurostat. Note this data is also presented in Figure 3.10 of the ABPI UK Biopharma R&D Sourcebook 2016, it has been converted from euros to pounds. <http://www.abpi.org.uk/our-work/library/industry/Pages/Open-for-Innovation-ABPI-Sourcebook-2016.aspx>

B. Industrial Strategy

5. *What can be learnt from the impact of the 2011 UK Life Sciences Strategy? What evidence is there that a strategy will work for the life sciences sector? How can its success be measured against its stated objectives?*

- We are half way through the present ten-year 2011 Strategy for UK Life Sciences. Since launching the Life Sciences Strategy in 2011, the Government policy agenda has continued to evolve.
- Collaborative R&D funding through mechanisms such as the Biomedical Catalyst was a key part of the 2011 UK Life Sciences Strategy and has been hugely successful. Government should understand why this intervention is successful.
- The success of the 2011 Life Sciences strategy and current LSIS will ultimately depend on demonstrable evidence of the economic, health and well-being benefits generated and successfully captured within the UK. The impact of these strategies will hinge on clear, measurable objectives, with a framework for base-lining and tracking progress against those objectives. The development of the strategic objectives must be undertaken in a transparent manner, preferably as part of an overarching framework.

6. *(If published) Does the strategy contain the right recommendations? What should it contain/what is missing? How will the life sciences strategy interact with the wider industrial strategy, including regional and devolved administration strategies? How will the strategies be coordinated so that they don't operate in 'silos'?*

- We welcome the publication of the LSIS and the recognition of the cell and gene therapy industry and CGT Catapult. We have proactively engaged in the consultation on the Life Sciences Industrial Strategy launched by Professor Sir John Bell, to increase government investment in science research and innovation, particularly in the field of leading edge healthcare and medicine. We have provided our views on the short and long-term areas of focus for Life Sciences Industrial Strategy. We believe continued investment in the cell and gene therapy industry will make the UK the "go to" place for the development of cell and gene therapies for supply to the global market.
- We believe the strategy amplifies the importance of smart specialisms such as advanced therapies; it calls for the full implementation of the Advanced Therapies Manufacturing Action Plan. We believe the LSIS should make deep use of Catapult Centres as instruments of policy in the sector.
- The Catapults are important in supporting the sector by continuing to help academics and the commercial sector develop new and important partnerships. Their understanding of the broad innovation landscape is important to supporting the development of new applications for a wide range of emerging technologies in the Life Sciences sector.

7. *What opportunities for small and medium sized enterprises (SMEs) are there/should there be in the strategy? How can they be involved in its development and implementation?*

- The pace of development in healthcare in the next few years is likely to favour small and medium enterprises. Smaller organisations are more likely to be sufficiently agile to be able to identify and exploit the opportunities that will develop from the ongoing transformation of healthcare. The number of SMEs in the UK cell and gene therapy industry has grown to +60 companies. These businesses struggle to navigate a complex and apparently fragmented

landscape of facilities and funding opportunities. Conversely, they possess the capacity to absorb the outputs of policy through collaborative R&D, Bio-Medical Catalyst and investment in manufacturing infrastructure.

- The Catapult network provides key elements of the strategy to support SMEs to find partners and identify funding opportunities. The Catapults also provide an important function in providing the critical evaluation of potential markets and the potential routes to adoption.
- SMEs engagement within the cell and gene therapy industry should focus on opportunities to accelerate the creation of spinouts from universities and the scaling up of businesses through technology enablement.
- CGT Catapult will continue to support the development of enabling technologies that are required to underpin developments in the advanced therapy sector, for example, the ability to automate manufacturing processes and to analyse complex data in real-time as part of a diagnostic and quality control system.

8. Where should the funding come from to support the implementation of the strategy?

- Funding to support the LSIS should come from central government in the form of collaborative R&D funding through the Industrial Strategy Challenge Fund (ISCF) or an equivalent mechanism. This funding must:
 - Be matched by industry
 - Address market failures and encourage industry to deliver market-leading commercial products and services that are aligned with the LSIS
- Collaborative R&D funding must remain nimble in nature and able to respond to the challenges faced by new emerging sectors. The LSIS includes an ambitious call for the establishment of 2-3 entirely new industries based on innovation in the Life Sciences. These industries of the future will have their foundations built upon a technology currently emerging from the research base. Funding for these new technologies needs be made available without compromising investment in existing successful programmes.

9. How do the devolved administrations and city regions fit into the strategy? Scotland has its own life sciences strategy, how will the two interact?

- CGT Catapult's remit is to grow a UK cell and gene therapy industry that delivers health and wealth to the nation. We have projects across the UK including all the devolved nations.
- We acknowledge the devolved nations have local priorities and important existing life sciences clusters. We believe clustering plays a key role in the life sciences industry and we place importance on building on peaks wherever they are in the UK and not filling in troughs.

C. NHS procurement and collaboration

10. How can public procurement, in particular by the NHS, be an effective stimulus for innovation in the Life Sciences Sector? Can it help support emerging businesses in the Life Sciences sector?

- The LSIS correctly identifies the Government's privileged position as a purchaser to create a pull for healthcare innovation and support the supply chain.
- The NHS could become a powerful and unique asset for patients and the UK cell and gene therapy industry through the combination of world-leading R&D capabilities and procurement processes that promote early adoption, reimbursement and proof of market.
- To encourage NHS uptake it may be necessary to provide ring-fenced funds for the clinical adoption of new technologies and products in cell and gene therapies.

11. How can the recommendations of the Accelerated Access Review (AAR) be taken forward alongside the strategy? Will the recent changes to the NHS England approval process for drugs have a positive or negative effect on the availability of new and innovative treatments in the NHS? How can quick access to new treatments and the need to provide value for money be reconciled?

- Industry perceives the NHS as being slow and reluctant to adopt innovation due to its sharp focus on cost-containment. This situation is likely to be exacerbated by the net budget impact threshold of £20M p.a. as well as the newly introduced QALY threshold for Highly Specialised Technologies. These could potentially influence the adoption of cell and gene therapies particularly those with ultra-orphan indications.
- We believe the NHS and regulators can clearly set out a swift, predictable and viable route to market for innovative cell and gene therapies giving industry confidence that the UK is a progressive global hub. We support the recommendations of the Advanced Therapies Manufacturing Action Plan, which are:
 - The creation of ISCF competitions to support industry to investigate opportunities to launch advanced therapy-focused pilots in the Accelerated Access Pathway that has been proposed by the AAR
 - The creation of a ring fenced time-limited reimbursement fund specifically for advanced therapies to help initiate the market for these products
 - The creation of a network of Advanced Therapy Treatment Centres (ATTCs) with new Government funding (£30 million) delivered through a competitive process managed by Innovate UK with widespread industry involvement to develop and implement the new systems needed to allow these Centres to operate.

12. How can collaboration between researchers and the NHS be improved, particularly in light of increased fiscal pressures in the NHS? Will the NHS England research plan help in this regard? How can the ability of the NHS to contribute to the development of and adopting new technology be improved?

- The uptake of new treatments in the NHS is low and slow, despite numerous government initiatives aimed at improving the adoption of innovation by the NHS. The Life Sciences Competitiveness indicators, published by Office of Life Science (OLS) in April 2017, show that for NICE-approved medicines launched between 2011 and 2015 the UK rate of uptake in

the first 12 months after launch was 18.2% of the median usage in comparable countries. The LSIS rightly explains that to deliver outstanding patient outcomes and to create an innovation-led health system, innovative products, such as cell and gene therapies, that generate patient benefits should be adopted at a rate that places the UK in the top quartile of comparator countries. CGT Catapult welcomes this target. We are concerned that without NHS buy in this will not be achieved.

- Research commissioned by the BIA in March 2017 reveals that staff across the NHS are generally unaware of the challenges around the adoption of innovation and have little to no awareness of previous government initiatives aimed at addressing these challenges and improving the uptake of new treatments.
- The LSIS outlines measures for overcoming what it describes as ‘the issue of diffusion and widespread adoption within the NHS,’ including shared assessments of UK uptake for NICE approved medicines and audited reports from healthcare providers. We believe that for such initiatives to succeed NHS leadership will need to be aware of and embrace the LSIS.

D. Responsibility and Accountability

- 13. Who should take responsibility for the implementation of the Life Sciences Industrial Strategy and to whom should they be accountable? What should the UK Government's role be? What should the role of the academic, charitable and business sectors be?*
- 14. What is the role of companies within the sector, particularly the large pharmaceutical companies, in the implementation of the strategy? How are they accountable for its success?*

- The Government working with industry is the architect of the LSIS; as such, Department for Business, Energy and Industrial Strategy (BEIS), Department of Health (DH) and the OLS should be responsible and accountable for its full implementation, with industry playing its part.
- The implementation plan should not affect the general healthcare budget, which has to remain protected, and funding for the LSIS should be additional ring-fenced investment.
- For the LSIS to succeed, there needs to be ministerial leadership, alignment and buy in across departments and the NHS.

- 15. Does the Government have the right structures in place to support the life science sector? Is the Office of Life Sciences effective? Should the Government appoint a dedicated Life Sciences Minister? If so, should that Minister have UK-wide or England-only responsibilities?*

- A dedicated Life Sciences Minister would act as a useful focal point to work the interface between the Life Sciences industry and the NHS.

E. Brexit

16. What impact will Brexit have on the Life Sciences sector? Will the strategy help the sector to mitigate the risks and take advantage of the opportunities of Brexit?

Impact of Brexit on access to collaborations and funding

- We welcome Government's stated position that "the UK must find a way to continue to collaborate with the EU, in the interests of public health and safety", and Government's aim of maintaining "deep, broad and dynamic co-operation" with the EU after Britain exits.
- Ultimately, the impact of Brexit on the advanced therapies industry in the UK will depend on the deal that the UK achieves with the EU. Whatever settlement is reached with the EU should ensure the continued ability to collaborate at scale and secure long-term, predictable funding for scientific research.
- The UK cell and gene therapy industry has worked in close collaboration with many European countries for many years. The industry actively participates in important pan-European support mechanisms such as Horizon 2020. Establishing structures that allow such collaboration to continue after EU-exit would benefit the UK research community and support the more rapid development of new advanced therapies.
- Consideration has to be made to the loss of the European Investment Bank, which poses a threat to investment funding and that should be addressed, potentially via the British Business Bank.

Impact of Brexit on Exports

- Advanced therapies are intrinsically an export-focused sector – the UK is a small proportion of the global drugs market so companies must look overseas to recoup high R&D costs.
- Continued access to European and global markets is critical to the success of the industry.
- Brexit risks creating regulatory uncertainty or divergence. Unless an agreement is reached which acknowledges the UK as equivalent, the UK being viewed as a third country (i.e. not within the EU) would mean products manufactured in the UK would need to be tested and QP certified from within an EU member state.

Impact of Brexit on Foreign Direct Investment (FDI)

- Brexit puts at risk the UK's ability to capture internationally mobile inward investment that would grow the nation's advanced therapy industry.
- We support the full implementation by Government of recommendations set out in the Advanced Therapies Manufacturing Action Plan. The action plan sets out a series of interventions that aim to capture £350 million of advanced therapies manufacturing investment in the UK over the next three years.

Examples of how CGT Catapult supports inward investment

- CGT Catapult has a strong international reputation and has built relationships with businesses in over 24 countries.
- We work closely with UK Trade and Investment (UKTI) and now the Department for International Trade (DIT) to support exports and drive FDI. We have contributed to trade missions to China, Japan, Singapore & South Korea and work with OLS to target inward investment from North American cell and gene therapy companies.

- In March 2017, Fisher BioServices announced a collaboration with CGT Catapult to provide therapy developers with both the manufacturing capability and the distribution, logistics, and storage capacity needed to create a seamless supply chain to accelerate cell and gene therapy development and commercialisation. As part of the collaboration, Fisher BioServices will expand its CryoHub solution by co-locating it with CGT Catapult's new large-scale cell and gene therapy manufacturing centre based in Stevenage UK, due for completion in summer 2017. Combining the two offers centralised manufacturing, storage, distribution and logistics, resulting in a seamless supply chain. The co-location of the CryoHub with the CGT Catapult will support collaborators within the manufacturing centre and the broader UK community. Combined with, Fisher BioServices' existing capabilities in Asia and the US, the Stevenage CryoHub enables Fisher BioServices to more easily support its customers' global trials as they develop and commercialise their therapies in Europe.

Impact of Brexit on access to global talent

- The UK cell and gene therapy industry needs access to global talent to fuel its rapid growth. We anticipate UK industry creating 1,500 new high skilled jobs by 2023.
- The immigration system plays a key role in facilitating access to talent of all levels in the cell and gene therapy industry. As the UK leaves the EU the system will likely change. Continued ability to secure the most talented people for UK science and industry can be delivered through an immigration system, which facilitates ease of movement for talented students, researchers and workers. This should be straightforward, and rapid – providing certainty of outcome. It should project a welcoming and open Britain.

17. How should the regulatory framework be changed or improved after Brexit to support the sector?

- The MHRA, NIBSC and the British Pharmacopoeia (BP) should lead a series of stakeholder engagement meetings with industry, SMEs and academic innovators to identify current gaps in advanced therapies standardisation and address different aspects of cell, gene and viral vector materials, as well as their manufacturing processes.
- We ask that Government enable and resource MHRA and NIBSC, to work through the challenges of standardising complex ATMP production with relevant parties and ensure that it be properly resourced with funding and expertise to take this critical work forward.
- CGT Catapult will work with regulators to develop a long-term regulatory strategy and plan for the MHRA to lead in global standards and support the scientific activities and international outreach of NIBSC.
- We believe Brexit provides the UK with the opportunity to develop a globally competitive national regulatory pathway for advanced therapies.

18. To what extent should the UK remain involved with and contribute to agencies such as the EMA post Brexit?

- The two globally significant regulators, the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA) cover markets comprising 32% and 25% of global pharmaceutical sales respectively and together with Japan, two thirds of the global market.
- Currently the UK benefits from a common regulatory framework and market with the EU, to which UK expertise has materially contributed.

- Brexit forces change on the UK regulatory system for all medicines, including Advanced Therapeutic Medicinal Products (ATMPs). The regulatory environment for ATMPs is rapidly evolving and Brexit provides the opportunity for the UK to re-position itself advantageously by creating a regulatory environment, which will accelerate clinical trials and the ultimate approval of ATMPs in the UK, and increase the possibility of greater international harmonisation.
- If the UK leaves the single market as expected and can no longer remain part of the centralised procedures, the UK will no longer retain any say in the regulation of medicinal products in Europe.
- If the UK can negotiate a role to remain within the centralised procedure, the level to which it interacts with the EMA and other EU national agencies will be limited by the terms of the agreement.
- The latter situation remains highly unlikely, in which case the UK will have to maintain dialogue with the European agencies based on ensuring that mutual recognition of good manufacturing practices and other relevant legislation is agreed and that the UK maintains a regulatory environment that is sufficiently harmonised with the evolving EU regulatory landscape.

REFERENCES

1. [Advance Therapies Manufacturing Action Plan](#)
2. ABPI sponsored PWC report: ['The Economic contribution of the UK Life Sciences industry](#)
3. [Alliance for Regenerative Medicine 2016 Regen Med and Advanced Therapies State of the Industry Briefing and Q2 2017 Report](#)
4. [Cell and Gene Therapy Catapult Clinical Trial Database](#)
5. Cell and Gene Therapy Catapult Internal Databases and Market Research
6. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/635714/strength-and-opportunity-2016-bioscience-technology-accessible-revised.pdf
7. https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/606651/life-science-competitiveness-indicators-report-2017.pdf
8. <https://www.gov.uk/government/publications/patient-capital-review>
9. [https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/600023/Biomedical Catalyst Baseline Evaluation Report.pdf](https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/600023/Biomedical_Catalyst_Baseline_Evaluation_Report.pdf)
10. Analysis by Grant Thornton for Innovate UK on European cell manufacturing facilities and investment in Europe vs the UK
11. Dods (March 2017), The adoption of innovation in the NHS: A survey of healthcare professionals on behalf of the BioIndustry Association

Some of the universities, businesses, Government institutions and research organisations CGT Catapult has worked with across the UK

