Medicine Manufacturing Industry Partnership

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BIA UK BioIndustry Association

Innovate UK Knowledge Transfer Network

Advanced Therapies Manufacturing Action Plan

Retaining and attracting advanced therapies manufacture in the UK

Dedication to Richard Archer

Richard Archer, who died suddenly in September, made a significant contribution to the concept and draft of this paper. As with most bits of process engineering, Richard's work on this project wasn't immediately obvious to those who weren't intimately involved in it. However for those of us on the inside his role was vital. He made sure we all understood the prize, kept us honest to the timeframe, and brought his experience of how you get projects like this over the line. Like an imperfectly running petrol lawnmower pushed rapidly onto the cricket square to get the first pitch of the season ready – this document misses his wordsmithing and fine tuning. What I do know is that he would have been immensely proud to have seen it published – and we will miss him bugging us all over the next couple of years to turn it into something that actually happens.

by Steve Bates, BioIndustry Association,
on behalf of Advanced Therapies Manufacturing Taskforce.

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Foreword from Co-Chairs of ATMT

We have seen rapid growth and significant clinical success with advanced therapies in recent years and the UK has been at the forefront of this research and development. The sector is moving quickly and is now at a critical point, as it prepares to commercialise and scale up manufacture of these treatments. The Taskforce believes these recommendations will help anchor these investments in the UK and MMIP, with its partners the ABPI, BIA and KTN will continue to take this work forward with Government and its partners to capture this opportunity.

I would like to thank all of those that have been involved in the work of the taskforce and willingly given significant amounts of their energy, time, resource, and knowledge. We especially welcomed the early support of George Freeman, former Life Sciences Minster and the continuing engagement of Jo Johnson, Minister of State, BEIS. I would particularly like to thank Roger Connor, Jim Faulkner and Keith Thompson who led the critical workstreams and engaged their industry colleagues to pull together the recommendations. Special thanks must also go to the ATMT secretariat, led by Mark Bustard, KTN, who I know have worked tirelessly on behalf of industry to get this report completed.

The UK is very well placed to anchor manufacturing of these potentially curative therapies in the UK and secure its position as a global hub for advanced therapies but the time to act is now. The forthcoming Industrial Strategy gives us an opportunity to take forward this report and work together to secure investments, further the science, and contribute significant benefits to the UK economy and patients in the UK and worldwide.

Ian McCubbin
Chair of MMIP
Senior Vice President North America, Japan & Global Pharma Supply
GlaxoSmithKline

I warmly welcome this excellent report from the Advanced Therapies Manufacturing Taskforce which highlights the potential of these disruptive technologies to deliver real benefits to patients and, at the same time, to provide a new area for UK leadership in biopharmaceuticals. Your report sets out how the UK can build on the success of a number of companies including ReNeuron, Oxford BioMedica and Cobra Biologics who have spotted the growth opportunity and are now investing in manufacturing and in skilled staff. We want to see more of these investments so the UK becomes a leading location to manufacture this new class of medicines. It is clear from the work of the Taskforce that Industry and Government need to work together to make this happen.

Your report comes at an opportune time with development of the Government's Industrial Strategy. This is a critical part of the Prime Minister's ambition to build an economy that works for everyone. The strategy will set out how we will boost productivity, create good jobs, and ensure sustainable economic growth. Life Sciences, and within that, Advanced Therapies – hold great potential for the UK to develop new sources of economic competitiveness. Your report provides a valuable input as we develop the Industrial Strategy. I welcome continued commitment and engagement from your industry.

I would like to thank in particular Ian McCubbin who has kept the taskforce focussed in its work, Roger Connor, Keith Thompson and Jim Faulkner for their leadership, taskforce members for their ideas and commitment and George Freeman MP who co-chaired the taskforce during the critical kick-off and development phases. Finally, I would also like to recognise the hard work of the secretariat led by the Knowledge Transfer Network supported by the ABPI, BIA, Cell and Gene Therapy Catapult and the Office for Life Sciences.

Jo Johnson
Minister of State for Universities, Science, Research and Innovation
Department for Business, Energy & Industrial Strategy
UK Government

Executive Summary

Advanced therapy medicinal products (ATMPs), including breakthrough cell and gene therapies, offer unprecedented promise for the long-term management and even cure of disease, especially in areas of high unmet medical need. These advanced therapies are driving an exciting and revolutionary new paradigm in science and healthcare.

The UK took an early lead in capturing advanced therapy medical research and now has a recognised leading international position in the discovery and development of advanced therapies with world-class academics, innovation infrastructure including the Cell and Gene Therapy Catapult, investors, many SMEs, and now pharmaceutical companies, concentrating their research in the UK.

This emerging industry is at a critical stage in translating this research into manufactured products with the focus now on planning manufacturing scale-up in order to commercialise these therapies. In this context, the UK has the opportunity to secure its position as a global hub for researching, developing, manufacturing and adopting advanced therapies.

The Advanced Therapies Manufacturing Taskforce (ATMT) was set up to identify actions that the UK must consider taking in order to anchor manufacturing of advanced therapies in the UK and capture investments to secure the UK position as a world class hub. These investments are globally mobile and we know similar initiatives are taking place in countries such as Japan and the USA. The UK will lose out on its early lead and potential early investment if no action is taken.

The growth and potential of the cell and gene therapy market

- Global cell and gene therapy market is estimated to be worth between £9 to £14 billion ($14 to $21 billion) per year by 2025[1]
- The UK is expected to form 4% of this market size, which is higher than its normal share for small molecules and biologics[1]
- UK's market size is estimated between £0.4 to £0.6 billion ($0.6 to $0.9billion) per year by 2025, based on local uptake[1]
- Conservative growth estimates by 2030 were also calculated to be £21 to £32 billion per year ($31 to $48 billion) for the global market size and £0.9 to £1.3 billion ($1.3 to $2 billion) for the UK[1]
- UK companies attracted over £400 million investment in 2015[2]
- At least 48 ATMP developers in the UK - of which over 50% are actively growing - a 15% increase on the start of this work nine months ago[3]
- By the end of 2015 over 1,000 jobs had been created in the UK[2]
- Conservative estimate is for 400-600 additional skilled staff being required over the next two years[4]
- Over 50% growth in UK Clinical trials in UK since 2013[3]
- Investing now to build and grow the UK's operational base could enable local firms to capture a bigger share of globally accessible revenues, further shaping these market forecasts

The UK must sustain and continue to attract life sciences investment to maintain its strong position and capitalise on future opportunities. On this basis, the Taskforce has set out the following recommended actions for the Government and industry to act on and take forward through the forthcoming Industrial Strategy.

Recommended actions to make the UK a global hub for manufacturing advanced therapies:

1. Strengthen and secure an internationally competitive fiscal landscape to attract investment

- Through Industrial Strategy, the Government should strengthen the R&D tax credit system by reviewing and extending the definition of R&D to include advances in manufacturing beyond clinical manufacture

[1] Cell and Gene Therapy market data, ABPI, PWC October 2016
[2] Cell and Gene Therapy Catapult Annual Review 2016
[3] Source data from CGTC, KTN, OLS, Innovate UK, ABPI, BIA
[4] Outputs from the ATMT People, Skills and Training workstream

- Government should reintroduce capital allowances in the UK for commercial manufacturing assets, and in particular for Good Manufacturing Practice (GMP) facilities. The UK trails behind international competitors on the provision of these allowances
- Government should retain the value of the patent box, as an incentive, by reducing the patent box rate below 10%, as suggested in the 2016 Budget. A suggested rate of 7-8% would align with competitors such as Ireland (6.25%) and Belgium (6.98%)
- Support SMEs to help them understand and utilise existing incentives, which are competitive globally

2. Target and capture internationally mobile investments through a proactive and simplified process of engagement

- Government to set a call to companies to come forward with their investment plans for advanced therapy manufacturing in order to land these in the UK. This should be supported by a targeted programme that will run for two years (2017-2018) to capture these investments
- Government should set out a simple, effective mechanism to provide a single entry point for companies looking to invest in advanced therapy manufacturing and that the Department for International Trade (DIT) and the Life Sciences Organisation (LSO) recruit a Manufacturing Specialist that can engage investors while supporting continued growth of UK domiciled businesses
- DIT/LSO review and refresh its factsheet for the offer in medicines manufacturing and advanced therapies/regenerative medicine
- Industry to provide a number of Business Ambassadors that can support LSO efforts to promote the UK and help land advanced therapy manufacturing investments

ReNeuron

ReNeuron secured a £7.8 million grant package from the Welsh Government to establish a cell research, development and manufacturing facility in South Wales for late stage clinical and commercial product requirements. The company relocated its existing staff and operations to the new state of the art R&D facility in Pencoed in 2016, and will create further highly skilled jobs as the manufacturing facility comes on stream.

Global investments of significant scale are happening all around us. We must be constantly at the forefront of capitalising on our strengths in ATMPs so that we land similar investments for the UK.

GE Healthcare

GE's EUR 150 million investment in the creation of BioPark, Cork will create up to 500 new jobs, and according to IDA Ireland, address a large number of industry challenges such as cost pressure, increasingly expanding drug pipelines and flexibility, allowing companies to quickly invest in capacity while hedging risk of over-investment. It allows them to move more quickly and adopt the latest in manufacturing technology. GE is also in the process of setting up a training collaboration with National Institute for Bioprocessing Research and Training (NIBRT) to develop skills for Ireland's biologics sector, training 1,500 professionals per year.

3. Maintain science and innovation funding to support industry developing cutting-edge technologies

- The UK seeks to capture at least £350 million in advanced therapies manufacturing investment by making available competitive or loan/grant funding that could deliver in the range of £30 million per annum for at least three years to attract and anchor this strategically important and transformative sector in the UK. This

support could be outlined in the Government's forthcoming Industrial Strategy

- Government to establish competitive funding to support viral vector capability growth within two years. The Taskforce proposes the development of a specialist manufacturing operation leveraging where possible existing infrastructure. The operator(s) should be mandated to work with academic groups to supply viral vectors and therefore gain exposure to cutting edge developments, whilst at the same time industrialising the academic processes. The Taskforce will work with the funders by early spring 2017 on delivery and process
- Government and industry continue to invest in innovation in manufacturing technology development and capability through existing mechanisms such as Innovate UK CR&D, Research Council funding and Biomedical Catalyst
- Ensure that manufacturers of advanced therapies are eligible to apply for the New Innovative Financial Products that are being developed by BEIS to be offered through Innovate UK. Funding that can be competitively won by individual businesses or by collaborative partnerships, alongside research council investment, in the range from £25 to £35 million over the next three years will help to anchor manufacturing and technological development in the UK and secure new skill sets and expertise

Oxford BioMedica

Oxford BioMedica (OXB) is a leading gene and cell therapy company, which successfully secured £7.7 million funding from the Government's Advanced Manufacturing Supply Chain Initiative (AMSCI) in 2014. The AMSCI funding received has helped to catalyse the growth of the company and, together with a significant deal with Novartis, will result in more than 140 new jobs in the company, with the majority being in highly-skilled technical roles i.e. in manufacturing, supply chain, testing, quality and development. This investment in new jobs, from an original headcount of around 80, together with a major and concomitant investment in facilities and manufacturing capacity will result in significant growth in manufacturing capability in the UK, and help to deliver the company's potential to become a world leader for advanced therapy manufacture and supply chain expertise. The Novartis CTL019 product, which uses OXB lentiviral vector technology manufactured in the UK by OXB, is on track for approval in 2017 and if approved will be the first ATMP approved worldwide that utilises lentiviral vector technology.

4. Set out an end-to-end talent management plan to secure the relevant skills for emerging manufacturing technologies[5]

- Industry to lead with support from multiple stakeholders including academia, Higher Education, Innovate UK and research bodies, the creation and implementation of an end-to-end talent plan for the sector. The plan will encompass integrated strategies to develop the talent pool at multiple entry-points. These range from manufacturing technicians through to post-doctoral and professional levels
- Efforts should be concentrated on leveraging existing initiatives, with the expectation that the majority of funding will come from industry, either through direct subscription or through Apprenticeship Levy funds
- Seed funding of £1.5 million from Government is recommended to enable successful set-up and coordination of the plan

5. Clearly set out a swift, predictable and viable route to market for these innovative products and give industry confidence that the UK is a progressive global hub

- Establishment of a task and finish group with representation from industry, NHS, NICE, Department of Health and Office for Life Sciences is recommended to consider the market challenge and review existing initiatives including Accelerated Access Review (AAR), NICE modelling, NHS specialised commissioning, as well as new opportunities to create a sustainable funding model and pathway for adoption in the NHS
- Industry should investigate opportunities to launch advanced therapy-focused pilots in the

[5] http://bia.me/ATMTskills

Accelerated Access Pathway that has been proposed by the AAR

- To support ongoing discussions the Government should set out a time-limited reimbursement fund specifically for advanced therapies to help initiate the market for these products
- Establish a network of Cell and Gene Therapy Treatment Centres 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

6. Develop a long-term regulatory strategy and plan for the MHRA to lead in global standards, supporting the scientific activities and international outreach of NIBSC

- Government to recognise the inherent challenge in the standardisation of complex ATMPs and the importance of standardisation in supporting the development of manufacturing processes
- 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 and products
- Government must enable and resource MHRA with NIBSC, to work through the challenges of standardising complex ATMP production with relevant parties and ensure that it is properly resourced with funding and expertise to take this critical work forward

Report background and context

ATMPs, including breakthrough cell and gene therapies, offer unprecedented promise for long-term management and even cure of disease, especially in areas of high unmet medical need. The benefits of growing an advanced therapies global hub for manufacture in the UK would not only advance innovation and science but add to the growth of the UK economy, and ultimately improve the health of patients. The transformative potential of advanced therapies has been fuelling an impressive increase in investment.

The UK took an early lead by creating a supportive research environment for this platform, providing public sector funding and innovative infrastructure and this provided a foundation for the UK to develop its world-leading position in the discovery and development of advanced therapies.

The UK's world-renowned academic base is producing the necessary talent pool of high-quality graduates and post-graduates and spinning out companies and an innovation pipeline with new assets and players. According to the Alliance for Regenerative Medicine, over 550 clinical trials on advanced therapies were conducted worldwide in 2015, increasing to 669 in the first quarter of 2016 and nearly ten percent of these were done in the UK[6]. The UK is now in a strong position to capitalise on the establishment and growth of an emergent industry that will manufacture advanced therapies and deliver them to patients.

Having successfully captured advanced therapies research investment, the UK needs to accelerate translation and development of these complex high value products, secure manufacturing in the UK and avoid all opportunities to lose this expertise abroad. On the latter point, we have seen this experience with monoclonal antibodies, where despite the UK leading in research and development, the manufacture of these therapies went overseas and the UK effectively lost a significant commercial market share. A number of persistent challenges still test the confidence of advanced therapies developers. Many are currently setting out their manufacturing strategies and considering what investments are necessary to commercialise products, but there is uncertainty around routes of adoption into clinical practice and reimbursement of products.

In reality, concerns about funding availability, evidence of duration of effect required, and health system commissioning and adoption can significantly affect appetite and confidence to invest in these therapies. Countries that signal they are willing to address challenges will be perceived as attractive locations for investment. The UK needs to be ambitious and act quickly to get ahead. The USA, Canada and Japan are particularly active in this space and, although the UK is preeminent in Europe; Germany, Italy, France and Spain, in particular are rapidly reviewing how they can also capture these investments.

The Taskforce believes the UK can take action to address some of these challenges, capture manufacturing investment and ensure that the UK assumes a leading position in the global advanced therapies space to supply patients with these medicines at scale. The Government has recognised

[6] Alliance for Regenerative Medicine Releases Q3 2016 Data Report, Highlighting Sector Trends and Metrics

this ambition, and outlined regenerative medicine as one of the eight great technologies. Anchoring these technologies in the UK, through a future Industrial Strategy, will absolutely support the UK's agenda to drive productivity, exports and inward investment.

The UK needs to act with urgency and differentiate itself as a global destination for advanced therapies development and manufacturing in order to set the sustainable foundations for this emergent sector to root and grow. Life science has always been strong in the UK and as the Government sets out its forthcoming Industrial Strategy, we recommend that it aggressively targets future areas of growth – advanced therapies manufacturing being one of these areas.

Recommended actions to make the UK a global hub for manufacturing advanced therapies

1. Strengthen and secure an internationally competitive fiscal landscape to attract investment

A critical requirement for life sciences investment decisions is a supportive fiscal landscape. In particular, tax and the financial incentives available to companies in competitor countries will be reviewed carefully, as part of the decision-making process.

The industry has welcomed the UK's drive to be fiscally competitive internationally. The lowest corporation tax in the G7 – a legislated headline rate of 20% reducing to 17% by 2020, the introduction of the patent box, R&D tax credits are all incentives that have placed the UK in a competitive position. Going forward, and in the current context with the UK planning to exit the EU, it is vital that the UK makes all efforts to retain and continue to improve its fiscal offering in order to secure investments and anchor infrastructure in the UK and give confidence to investors.

The Taskforce recognises that following the UK referendum result there will be a more acute need to boost confidence and attractiveness and send strong signals around its commitment to attracting the best research and manufacturing infrastructure in the UK. Changes to UK Government tax legislation require extensive review and consultation but there is an opportunity to provide a range of further incentives.

As part of the UK's forthcoming Industrial Strategy, the Taskforce recommends that the following areas are assessed for impact and feasibility with relevant industry and industry bodies as interventions that will boost investment and growth in the UK economy:

- Through Industrial Strategy, the Government should consider strengthening the R&D tax credit system by reviewing and extending the definition of R&D to include advances in manufacturing beyond clinical manufacture

As products reach the point of commercial scale manufacture, it is the decision gate for many companies on where to locate manufacturing. A very strong influence on this decision is the availability of funding for the build of GMP (Good Manufacturing Practice) capacity.

- The Government should reintroduce capital allowance for manufacturing assets and GMP facilities for commercial manufacturing. The UK trails behind international competitors on the provision of these allowances
- The Government should retain and extend the value of patent box, as an incentive, by considering reducing the patent box rate below 10%, as suggested in the 2016 Budget. A suggested rate of 7-8% would align with competitors such as Ireland (6.25%) and Belgium (6.98%)

The Taskforce has found that larger pharmaceutical companies have the ability and capacity to understand and take advantage of the fiscal incentives the UK provides. However, there is an opportunity to do more to support SMEs, in particular, and potentially global companies with senior management based abroad, to ensure they understand the incentives available and can articulate these to relevant decision-makers. The Medicines Manufacturing Industry Partnership (MMIP) has set out these incentives to inform industry members and we would encourage all relevant parties seeking to attract investment to provide support to future investors, as part of its marketing efforts.

The Taskforce recommends that support is made available to SMEs to help them understand and utilise existing incentives. Through the MMIP, the industry will support these efforts by promoting its recently published fiscal guide to support the sector[7].

- Support SMEs to help them understand and utilise existing incentives, which are competitive globally

[7] http://bia.me/MMIPfiscal_briefing

2. Target and capture internationally mobile investments through a proactive and simplified process of engagement

All companies will seek ease and predictability in their efforts to do business. The Taskforce noted the strength of the UK's offering in advanced therapies and although organisations such as KTN successfully assist companies to understand the innovation landscape and funding mechanisms, industry representatives highlighted the challenge they had navigating and understanding the system and the offers available from UK Government. This was in stark contrast to international competitors, for example Ireland and Singapore, that have created strong account management capabilities and were able to work strategically with companies to broker offerings, deliver against national plans and ultimately capture investments. Government should consider benchmarking its account management offering against some key competitors for life sciences investment globally.

It is apparent that DIT/LSO has a clear remit to target and attract foreign investment and support British-based companies with export growth. However, the Taskforce noted the gap in support for home-grown SMEs, which are thriving in the UK. The industry identified that to boost ATMP manufacturing and development at full scale, more needs to be done to both maximize significant early overseas investment and give the home-grown companies, particularly SMEs, an appropriate level of attention and support.

To bolster the UK's global competitiveness, the Taskforce stressed the need to establish a defined resource with key expertise to strategically target and account manage emerging advanced therapies companies and suppliers looking to invest in UK based manufacturing and the supply chain. This single point of contact for senior managers is an approach taken forward by the Irish Development Agency (IDA) and the Economic Development Board (EDB, Singapore). Their tailored account management approach has made it very easy for companies to operate and invest in these countries and this point was reiterated by industry on several occasions.

Very constructive and positive discussion has taken place with the LSO and the Office of Life Sciences (OLS) to consider and address these concerns and a potential set of proposals on a way forward has been discussed. The Taskforce recommends that LSO targets advanced therapy companies with a call for investment opportunities, provides a single point of contact that can take companies through a defined process of engagement and dialogue and that a Manufacturing Specialist is recruited to work collaboratively with UK bodies to land investments.

The Taskforce understands that LSO and OLS are open to designing new support mechanisms and an advanced therapies-tailored delivery plan to improve company account management. These mechanisms should seek to capture eight to ten projects over the next three years.

There is a time-limited window to anchor advanced therapies manufacturing in the UK. Investment, whether by domestic or overseas companies, is increasingly mobile and attracted to locations offering the best incentives, biggest market opportunity, or a mix of other factors such as tax efficiency, strength of the science and technology base, regulatory framework, export potential, or skilled workforce. The UK can and should compete for this investment.

The Taskforce recommends that:

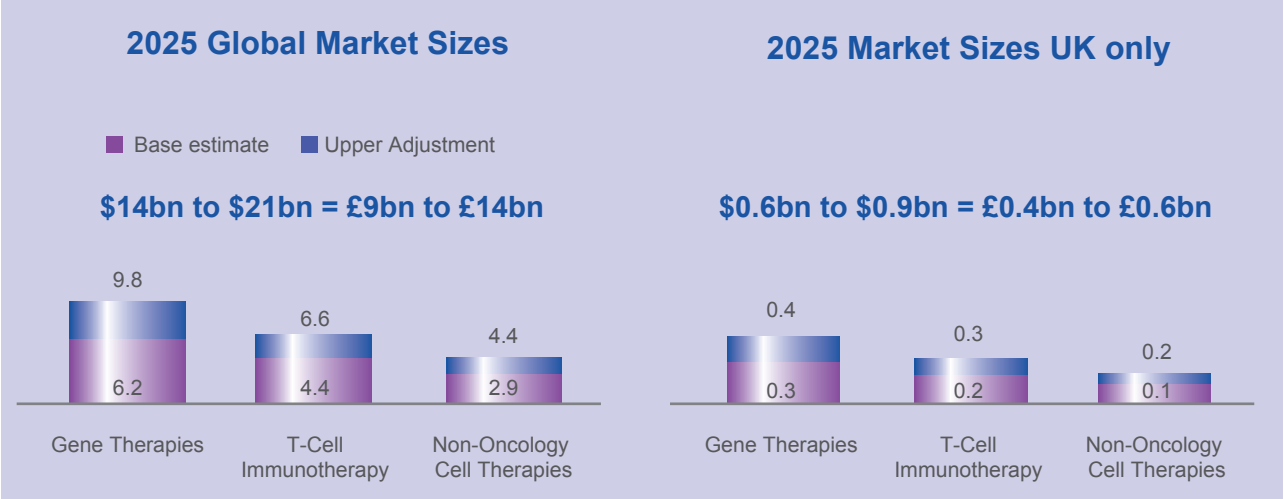
- Government to set a call to companies to come forward with their investment plans for advanced therapy manufacturing in order to land these in the UK. This should be supported by a targeted programme that will run for two years (2017-2018) to capture these investments.
- Government should set out a simple, effective mechanism to provide a single entry point for companies looking to invest in advanced therapy manufacturing and that the Department for International Trade (DIT) and the Life Sciences Organisation (LSO) recruit a Manufacturing Specialist that can engage investors while supporting continued growth of UK domiciled businesses
- DIT/LSO review and refresh its factsheet for the offer in medicines manufacturing and advanced therapies/regenerative medicine
- Industry to provide a number of Business Ambassadors that can support LSO efforts to promote the UK and help land advanced therapy manufacturing investments

3. Maintain science and innovation funding to support industry developing cutting-edge technologies

The Taskforce considered the size of the future investment prize and recommended that the UK seeks to capture at least £350 million in advanced therapies manufacturing investment by providing a substantial level of competitive funding in the range of £30 million per annum over three years to attract and anchor this strategically important and transformative sector in the UK. Alongside this, a need exists to invest in viral vector manufacturing infrastructure capacity and capability in the UK. The solution should meet the needs of academia and early stage business who are currently sourcing much of their requirements overseas, and ultimately move to significantly improve a viral vector manufacturing offering in the UK. As a result, the Taskforce recommends the following, as outlined below.

3.1 Securing investment in manufacturing capacity through flexible funding

Investment in advanced therapies is internationally mobile and we have already seen examples of companies leaving the UK to invest and anchor abroad. The Taskforce considered what impactful actions had kept company infrastructure in the UK and it was clear that examples of access to Government funding or loans had allowed some companies to remain in the UK and grow. More importantly, seed funding had given these companies the ability to leverage further private financing. Companies are being courted by global locations all vying to provide a range of incentives to capture this internationally mobile investment. The UK is in a good position to capture this investment and to support a strategic decision to create a global hub in advanced therapies, providing any access to capital will tip this over the edge.



2025 Market size estimates, based on Base and Upper Product Pricing Ranges: Sum totals rounded to nearest 1 billion. This study focuses exclusively on cell and gene therapy products within the broader sector of ATMPs, including gene therapies, T-cell immunotherapies and non-oncology cell therapies. It does not include tissue-engineered products or stem-cell transplants not involving any modification. The non-oncology cell therapies segment was calculated through a top-down methodology approach based on overall market rather than individual products. *(All values calculated in US dollars, with five year average rate used to account for current volatility (1.5 USD: 1 GBP), sum totals rounded to nearest 1 billion).*[8]

Reviewing the prospects for both UK based companies (48 developers and associated supply chain companies) and LSO states that around 250 overseas-based companies are ready to invest in the next two to three years, we anticipate significant investment is due to take place. The UK needs to target this investment capturing at least eight to ten investment opportunities bringing in £350 million of internationally mobile investment.

In order to reach this target, the Taskforce believes that there is a need for a substantial level of competitive funding to attract this strategically important and transformative sector. The Taskforce recommends that:

- The UK seeks to capture at least £350 million in advanced therapies manufacturing investment by making available competitive or loan/grant funding that could deliver in the range of £30 million per annum for at least three years to attract and anchor this strategically important and transformative sector in the UK. This support could be outlined in the Government's forthcoming Industrial Strategy

3.2 Invest in viral vector manufacturing capacity in the UK

The explosion in gene therapy in recent years has led to a short supply of viral vector manufacturing capacity, and both UK academics and industry are currently having to source viral vector from overseas which is a lost opportunity for the UK. There is a global shortage of capacity, skills and technology, and the UK has assets and expertise that can be built upon.

In addition to the small scale academic facilities manufacturing viral vectors, mostly manufacturing at capacity, the UK has a small number of industry manufacturers spanning both in-house supply and contract manufacturing to late clinical phase scale including; Oxford BioMedica, Cobra Biologics and BioReliance (Merck Group) all of whom have an excellent track record. The large scale manufacturing

[8] Cell and Gene Therapy market data, ABPI PWC October 2016

centre being built by the Cell and Gene Therapy Catapult will come on stream in 2017 and is designed to host companies manufacturing both cell and gene therapies, including those manufacturing viral vectors. In addition, the NHS Blood and Transplant strategy around advanced therapies remains under development. However, as part of their expansion, additional capacity for small-scale production for clinical trials will exist.

The Taskforce recommends that there is a need to build upon this base and support academics to have viral vectors manufactured effectively and at an appropriate cost in the UK, while also building a production service, which could be offered to industry. This service would provide leading technology innovation to offer a de-risked scalable runway to commercial scale production. Provision of a de-risked seamless runway from research and innovation through to scalable GMP production for viruses would be globally impactful. There is an opportunity to join up MRC-funded academics, relevant EPSRC activities and Catapults more effectively to work with industry so there is a continuum within the UK driving translation of viral vector technology and manufacturing needs.

Ultimately, the prize on offer is to grow UK industry and through strategic linking with UK-based CMOs, to enable clinical phase manufacture through to commercial scale. The Taskforce recommends:

- Government to establish competitive funding to support viral vector capability growth within two years. The Taskforce proposes the development of a specialist manufacturing operation leveraging where possible existing infrastructure. The operator(s) should be mandated to work with academic groups to supply viral vectors and therefore gain exposure to cutting edge developments, whilst at the same time industrialising the academic processes. The Taskforce will work with the funders by early spring 2017 on delivery and process

Cobra Biologics

Over the last three years, Keele-based CMO Cobra Biologics has been very successful in securing three Collaborative R&D grants, each one focusing on different aspects of the bioprocessing and biomanufacturing of viral vectors. Collaboration has involved both UK SMEs and a Catapult Centre. The total project costs have been £2.45 million of which Cobra has received £0.9 million in grant funding from Innovate UK. Even within a short timeframe the output has already resulted in capability and service provision enhancements for Cobra's global manufacturing business in the cell and gene therapy market.

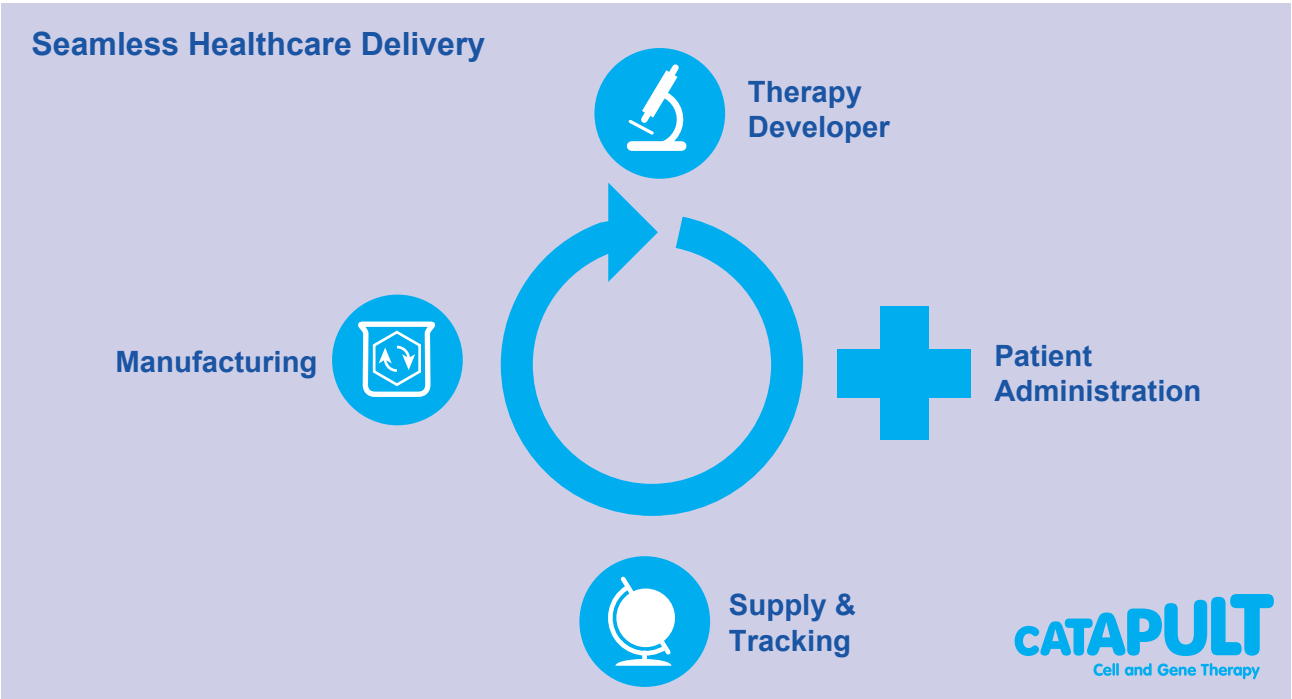
Driven by the significant global upturn for cell and gene therapy development and manufacturing services, and in combination with direct outputs from the projects, Cobra has seen a substantial six-fold growth in revenues for its service offerings in plasmid DNA and viral vector production. In addition, the increased business opportunity has resulted in the employment of an additional 30 full time employees to date.

3.3 Sustain the range of funding mechanisms to grow advanced therapies manufacturing technologies

Advanced therapies remain a fledgling therapy platform and the UK was quick to invest vital public sector funding to seize the opportunity to support and establish a strong research base, especially for cell and gene therapy in the UK. Over the last seven years, Innovate UK has provided £54 million in grants, which has supported 126 projects and has been pivotal in supporting business innovation. The establishment of the Cell and Gene Therapy Catapult at a cost of £70 million (2012-2017) provides additional support for academia and industry to translate research into commercially viable products. Anchoring manufacture of these products is the next crucial step to make the UK a global hub. The Taskforce is encouraged by the £55 million Government investments in the recently announced Innovate UK competition funding focused on cell and gene therapy manufacturing and the building of a manufacturing centre for cell and gene therapies to create UK capacity for companies looking to establish at scale and test supply chains and technologies.

The Taskforce also welcomes the recent decision to renew the Biomedical Catalyst funding to £100 million[9]. Similarly, continued funding from MRC, EPSRC and BBSRC is vital in propelling advanced therapies discovery, proof of principle, as well as the progression of manufacturing science and the development of new process, tools and technologies. The ongoing UK Regenerative Medicine Platform (UKRMP) review of challenge ideas is a timely opportunity to consider how the Government's Industrial Strategy will address funding research in underpinning manufacturing technologies.

Advanced therapies offer a new paradigm in the provision of healthcare. Existing systems and supply chains have evolved around the more traditional and well-established manufacture and delivery chains for small molecule medicines. For advanced therapies, entirely new systems will need to be designed and developed to enable cost-efficient manufacturing, assessment, and ultimately patient access. The challenge will be creating enabling and bespoke technologies and tools needed in this space.



The Taskforce discussed the need for the development of new manufacturing technologies covering manufacturing automation, advanced analytics, IT and data management and viral vector manufacturing technologies. A brief outline on the challenge and need for developing these areas is outlined below.

Automation: Large manual processes need to be automated to achieve consistency, lower cost of goods and ultimately bring the manufacture of therapies near to patients. These systems will be more than automating unit operations and bolting them together.

IT/data management: The management of data as patient derived materials flow to the factory and personalised therapies flow back is crucial to ensure that patients receive therapies that work. These systems do not yet fully exist and will need standards.

[9] https://www.gov.uk/government/news/220-million-for-cutting-edge-new-technology

Advanced analytics: Analytical methodologies and strategies are in their infancy, both off line and in situ. The way that they talk to automation and data management will be important and require both development and agreed standardisation.

Viral vectors: Current capacity is restrictive to perform both early and commercial scale trials from the UK due to constraints in upstream, downstream, fill finish, quality control, supply chain and access to scalable and industrialisable platforms. There is a need for new tools, technologies, assays and materials to be developed more rapidly.

Given the extent of these challenges, going forward we would recommend the UK maintains focus on providing funding tools and opportunities to support the vibrant pipeline of translatable discoveries and uphold UK's world-leading status in science and research.

The Taskforce recommends that:

- Government and industry continue to invest in innovation in manufacturing technology development and capability through existing mechanisms such as Innovate UK CR&D, Research Council funding and Biomedical Catalyst
- Ensure that manufacturers of advanced therapies are eligible to apply for the New Innovative Financial Products that are being developed by BEIS to be offered through Innovate UK. Funding that can be competitively won by individual businesses or by collaborative partnerships, alongside research council investment, in the range from £25 to £35 million over the next three years will help to anchor manufacturing and technological development in the UK and secure new skill sets and expertise

4. Set out an end-to-end talent management plan to secure the relevant skills for emerging manufacturing technologies

The growth of the advanced therapies 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 advanced therapies are in an experimental phase and manufacturing processes are largely under-developed and small-scale. As a result, the skills and knowledge requirements currently associated with advanced 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 and regulatory professionals will grow.

The UK is fortunate to have many strengths, including an academic base that is widely acknowledged as world-class, which makes it well positioned to grow the necessary talent pool and a supply of high-quality graduates and post-graduates. The UK must nurture this to avoid any skills shortages. The Science Industry Partnership (SIP), an industry-led group, recently published a report entitled Skills Strategy 2025[10] which made a number of proposals regarding increasing the capability and capacity of the UK science talent pool to meet the identified needs of the industry over the next decade.

There are a number of initiatives proposed within the SIP report that are relevant to the ATMT challenge. These include a proposed STEM Ambassador network, a system for facilitating placement of undergraduate work experience in the sector and mechanisms for promoting continuing professional development within the workplace. Included in the report is a recognition that the UK needs to attract young people into STEM careers, promote vocational training and link academia to industry needs. The Taskforce has aligned with the SIP where relevant and ensured that these broader initiatives are leveraged to suit advanced therapies manufacturing requirements.

Currently, the world-class UK academic science base attracts significant numbers of high-quality talent from other EU nations, who often come to the UK to study and subsequently move into the UK industry and the UK should continue to ensure this mobility of talent remains, while also extend its ability to provide unique and specialised skill sets in advanced therapies process innovation and manufacturing. Without an adequate and sustained supply of appropriately-skilled and knowledgeable talent, the UK advanced therapies manufacturing sector risks losing out to European and other overseas competitors. With its world-leading position on science and technology research and education, and legacy of top-ranking network of universities and research institutes, the UK is well placed to capitalise on and translate this academic advantage into skilled, experienced people to develop, manufacture, regulate and administer advanced therapy products and facilities. Considering the strengths and existing advantages present within the UK, taking no action in skills development would be an enormous missed opportunity and damaging to the home-grown development of new and innovative treatments. In order to ensure a sustainable supply of knowledge and skills necessary to fuel the growth of the sector, the Taskforce recommends:

[10] http://www.scienceindustrypartnership.com/media/529053/5202fd_sip_skills_strategy_2015_final_low.pdf

- Industry to lead with appropriate support from multiple stakeholders including academia, Innovate UK and the Research Councils the creation and implementation of an end-to-end talent plan for the sector. The plan will encompass integrated strategies to develop the talent pool at multiple entry-points. These range from manufacturing technicians through to post-doctoral and professional levels
- Efforts are concentrated on leveraging existing initiatives, with the expectation that the majority of funding will come from industry, either through direct subscription or through Apprenticeship Levy funds
- Seed funding of around £1.5 million from Government is recommended to enable successful set-up and coordination of the plan

5. Clearly set out a swift, predictable and viable route to market for these innovative products and give industry confidence that the UK is a progressive global hub

Advanced therapies will be transformative in the treatment of patients but the development of these revolutionary treatments have not been without significant investment risks and there are many anticipated challenges ahead that need to be worked through, not least in how these products will be adopted and reimbursed.

In order to anchor manufacturing investment in the UK, industry has been very clear that having clarity on a viable route to market will be essential. For advanced therapies this need is acute, as manufacturing costs of advanced therapies remain high and are further exacerbated by the small patient pools for rare or ultra-rare conditions. In addition, funding the lengthy clinical development of advanced therapies and the switch from research funded academic trials to private entities remains a risky undertaking. Regulatory risk can be reduced by early engagement with MHRA through the Innovation Office or the 'One Stop Shop'. Given the many SMEs working in this space, early reimbursement is critical to support reinvestment and return on investment.

Inevitably, investors will be most attracted to those countries and health systems that are ready to 'pull' through these products for early reimbursement and adopt these innovative therapies for the benefit of appropriate patients. The UK needs to send a very strong signal that it is serious about supporting these novel and innovative products and give industry confidence that the NHS will adopt these products. We do not believe this confidence currently exists. The AAR proposes an Accelerated Access Pathway for strategically important transformative products, making the ATMPs strong contenders for consideration in this pathway. Piloting the proposed Accelerated Access Pathway around selected advanced therapy assets would provide an opportunity for the Government to work alongside companies and the NHS to develop novel systems for assessment[11], commissioning and usage that can accelerate patient access.

NICE also conducted modelling to outline how its processes could support the value assessment of these products. In March 2016, the NICE report[12] concluded that NICE's technology appraisal methods and decision framework are applicable to advanced therapies. Industry has welcomed that NICE has been forward-thinking about the forthcoming challenges and accepts it is a step towards ensuring these products can be accessed by UK patients. However, outstanding concerns remain around how these therapies will meet cost threshold criteria and challenges around 'immature data' and data uncertainty.

Marrying the high cost of developing and manufacturing these treatments often for low patient numbers with the widespread need for affordable treatments in healthcare systems is a challenge. The UK must acknowledge the challenge and work constructively with all relevant partners - industry, NICE, NHS and Government to work through a sustainable and viable pathway for these products to move from bench to the bedside. Many of these products offer the potential of a lifetime cure instead of many years of chronic management, but the durability of the treatments will only be determined over time.

Potential considerations include the exploration of risk-sharing schemes that allow the quantification and management of immature evidence and allow the progressive collection of evidence (i.e. lifetime leasing, or annuity-based models). Part of this dialogue will need to involve the assessment of other attractive options currently on offer from global competitors, like Japan's Sakigake[13] and the French ATU schemes[14].

Although there is recognition that NHS and Government are subject to budgetary constraints, there would be value in discussing the potential for a dedicated and time-limited reimbursement fund for the early procurement by the NHS. This is proposed to be transitory in nature to start the market, secure early evidence on clinical efficacy and cost effectiveness, and most importantly secure patient accessibility while discussions on more innovative- and sustainable-funding models, as mentioned above, are initiated.

[11] Through the National Institute for Health and Care Excellence (NICE) in England, Scottish Medicines Consortium (SMC) in Scotland, All Wales Medicines Strategy Group (AWMSG) in Wales, The Department of Health, Social Services and Public Safety (HPSS) in Northern Ireland, the Joint Committee on Vaccination and Immunisation (JCVI) and Regional Medicines Optimisation Committees in the NHS in England)
[12] https://www.nice.org.uk/Media/Default/About/what-we-do/Science%20policy%20and%20research/Regenerative-medicine-study-march-2016.pdf
[13] http://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/140729-01.html
[14] http://agence-tst.ansm.sante.fr/html/pdf/5/atu_eng.pdf

Industry has made clear that NHS adoption and reimbursement is a critical issue. If the UK could be the first country to solve the cost conundrum then it would be particularly well placed to be the leading global hub for advanced therapies.

This also calls for the NHS to play a greater role as a partner for innovation, requiring greatest proximity and collaborations between industry developers, hospitals and patients to build capacity for clinical manufacturing at scale, develop the accompanying supply chains and logistics support. Following on from the Regenerative Medicine Expert Group report[15], Innovate UK has led a high-level proposal (£30 million) for funding the establishment of a national network of Cell and Gene Therapy Treatment Centres (CGTTCs). The proposal is that the CGTTCs, based in hospitals, will increase the UK capacity to deliver these treatments to patients and spearhead the creation of viable business models for hospitals to support commercial manufacture and formulation of therapies at scale in an in-hospital setting. The centres will be partnerships between industry and NHS trusts supporting clinical trials of therapies and delivering approved therapies to patients. This network of CGTTCs is envisioned to set the foundations for the early utilisation and specialised delivery of advanced therapies. Establishment of these centres will help supply chain development and create the necessary data infrastructure required to collect evidence on long-term patient outcomes.

In order for the UK to maintain a global presence in advanced therapies and secure investment the UK must outline how it will provide viable market for these products. The Taskforce recommends:

- Establishment of a task and finish group with representation from industry, NHS, NICE, DH and OLS is recommended to consider the market challenge and review existing initiatives including AAR, NICE modelling, NHS specialised commissioning, as well as new opportunities to create a sustainable funding model and pathway for adoption in the NHS
- Industry should investigate opportunities to launch advanced therapy-focused pilots in the Accelerated Access Pathway that has been proposed by the AAR
- To support ongoing discussions the Government should set out a time-limited reimbursement fund specifically for advanced therapies to help initiate the market for these products
- Establish a network of Cell and Gene Therapy Treatment Centres 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

6. Develop a long-term regulatory strategy and plan for the MHRA to lead in global standards, supporting the scientific activities and international outreach of NIBSC

The UK's MHRA is renowned for its progressive thinking and leading role in the regulatory space, both in Europe and internationally. This is epitomised by approaches like the Early Access to Medicines scheme (EAMS) that is paving the way for the accelerated adoption of innovation by the NHS and the establishment of the Innovation Office, which acts as a 'One Stop Shop' for regulatory queries.

The British Pharmacopoeia (BP), responsible for the development and publication of quality standards for medicines and medicinal products, is used in over 100 countries worldwide and is an acknowledged global leader in standards. NIBSC has a long history as the primary WHO physical standards laboratory and in the generation of collaborative R&D outputs for WHO and Pharmacopoeial physical standards.

Having a regulator that supports manufacturing innovation and is willing to engage early is a vital strength that the UK needs to capitalise on to support this still emerging platform. We know that there is industry interest in a global regulator to set gold standards for both quality and standards themselves - the UK is well positioned to lead on this. This is not without challenge, given the very personalised and unique approach to these products. In many instances, the process is the product and this is a new paradigm for regulators to contend with.

Industry is very encouraged by the fact that MHRA and NIBSC are already taking forward some valuable work on this area. The Innovation Office is the MHRA's first point of call for regulatory queries across medicines, devices and blood. For advanced therapies, the MHRA in collaboration with the other UK regulators, namely the Human Tissue Authority, Human Fertilization and Embryology Authority and the Health Research Authority, have formed the Regulatory Advice Service for Regenerative Medicine, also known as the 'One Stop Shop'. NICE is due to join the 'One Stop Shop'.

Through an active dialogue with MHRA, NIBSC and BP the Taskforce has been exploring how the UK could develop an enhanced and world-leading regulatory role in advanced therapies. For advanced therapies, the product is the process and therefore, again this is a new paradigm in regulatory approval. Novel manufacturing processes and supply chains need to be discussed at the earliest opportunity.

[15] https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/415919/build-on-potential.pdf

Current discussions have centred on investigating the practical arrangements that have to be in place around GMP assurance, standardisation and clinical assessment and devise support schemes for SMEs and other commercial innovators in the space. We note that a further current benefit is the MHRA's 'specials' that provides a flexible approach to allow supply of advanced therapies where suitable authorised products are not available.

There would be benefit in holding a series of technical meetings around the challenges of cell and gene therapies and vector systems to identify the inherent flexibilities of the current regulatory framework. It would also be worth canvassing different UK activities and groups that are developing new tools, processes, platforms and measurements.

The MHRA has a wide range of contact points and industry concluded it is worth having a single point that will take manufacturers through a process of engagement and dialogue. NIBSC could be well placed to take this work forward with industry and other regulators working through the challenges. It is currently developing and producing more than 90% of the international standards for biological products and carrying out foundational research into the safety, efficacy and quality of biologics. As a forward looking organisation, NIBSC could take a similar position for advanced therapies. To capture this opportunity though, the centre would need to be adequately resourced and supported to thoroughly review this work.

Cementing a good level of interaction between MHRA and NICE will also be key in establishing and strengthening a predictable and accelerated access pathway for advanced therapies. This link is endorsed by the AAR and its recommendation for an Accelerated Access partnership between the NHS-England, NICE and MHRA to offer a joined-up pathway from clinical and regulatory assessment to market access.

Building from this, the Taskforce recommends that:

- Government recognises the inherent challenge in the standardisation of complex ATMPs and the importance of standardisation in supporting the development of manufacturing processes
- MHRA, NIBSC and the BP 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 and products
- Government must enable and resource MHRA with NIBSC to work through the challenges of standardising complex ATMP production with relevant parties and ensure that it is properly resourced with funding and expertise to take this critical work forward

Concluding statement

This Action Plan is the summary of nine months of active consultation and engagement with industry, Government and relevant partners. From the outset, the Taskforce stated that the UK will need to act within two years or risk losing investment to other countries. Now is the time to act to capture what the Taskforce has indicated is sticky technology and infrastructure - highly technical and once anchored difficult to move. We know that manufacturing strategies are being developed and investment decisions are being made and the UK can be at the forefront of global consideration.

To that end, the Taskforce looks forward to swift response from Government regarding how we can work together on the timeline and delivery of these recommendations. We propose a Taskforce review meeting in early spring 2017. This work is even more timely given the Government focus on developing an Industrial Strategy. Capturing investment in this emerging sector should be a critical component in any future strategy and we look forward to seeing how the strategy will incorporate and take forward the Taskforce recommendations.

Image courtesy of FUJIFILM Diosynth Biotechnologies

Appendices

Appendix 1

Taskforce Membership

Chaired by

Ian	McCubbin	GlaxoSmithKline, Chair of MMIP
Jo	Johnson	UK Government

Members

Virginia	Acha	ABPI, MMIP
Richard	Archer	Institute for Manufacturing
Andy	Baker	British Society for Cell and Gene Therapy
Neil	Baker	Pfizer, MMIP
Steve	Bates	BioIndustry Association, MMIP
Ger	Brophy	GE Healthcare Life Sciences
Hannah	Brown	Office for Life Sciences
Mark	Bustard	KTN, MMIP
Peter	Coleman	Cobra Biologics
Roger	Connor	GlaxoSmithKline, MMIP
Catriona	Crombie	MRC
Natalie	de Lima	GlaxoSmithKline, MMIP
Janet	Downie	Roslin Cell Therapies
Annette	England	BioIndustry Association, MMIP
Jim	Faulkner	Autolus
George	Freeman	UK Government
Sarah	Goulding	KTN, MMIP
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Sharon	Grimster	ReNeuron, MMIP
Marc	Jones	AstraZeneca, MMIP
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Ruth	McKernan	Innovate UK
James	Miskin	Oxford BioMedica
Declan	Mulkeen	MRC
Nigel	Perry	Centre for Process Innovation
Magda	Papadaki	ABPI, MMIP
Ian	Rees	MHRA
Mike	Sullivan	Innovate UK
Keith	Thompson	Cell and Gene Therapy Catapult
Ian	Trenholm	NHS Blood and Transplant
Stephen	Ward	Cell and Gene Therapy Catapult

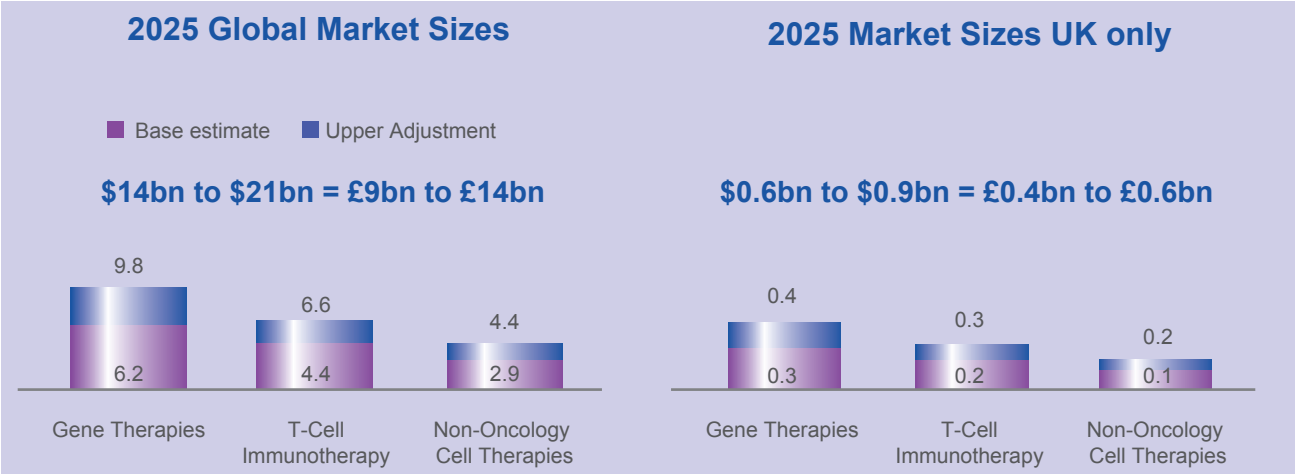
Appendix 2

The Taskforce would like to thank all the following contributing organisations for their input to this process

Association for the British Pharmaceutical Industry
Aggio Sergeant
Allergan Biologics
amc
Apprenticeship Trailblazer Group
AstraZeneca
Autolus
BioIndustry Association
Biomarin
BioReliance
British Pharmacopoeia
British Society for Cell and Gene Therapy
BSI
Business Environment, BEIS
Cell and Gene Therapy Catapult
Cell Medica
Cellular Therapeutics
Centre for Process Innovation
Clinical BioManufacturing Facility, University of Oxford
Cobra Biologics
Cognate
Collagen Solutions
Convatec
Covance
Department for Business, Energy & Industrial Strategy
Department for International Trade's (DIT) Life Sciences Organisation (LSO)
Department of Health
Doctoral Training Centre in Regen Med, Loughborough University
Eisai
EPSRC
eXmoor pharma concepts
Fisher BioServices UK
Freeline Therapeutics
FTI Consulting
FUJIFILM Diosynth Biotechnologies
GE Healthcare Life Sciences
GlaxoSmithKline
Institute for Manufacturing
Imperial College London
Innovate UK
King's College London
LGC

Loughborough University
Medical Research Council
Medicines Manufacturing Industry Partnership (MMIP)
MeiraGTx
MHRA
UK Government
Neotherix
Newcastle University
NHS Blood and Transplant
NIBSC
NICE
NightstaRx
Office For Life Sciences
Oxford BioMedica
Pall
Pfizer
Plasticell
PWC
Regener8
ReNeuron
Roslin Cell Therapies
Science Industry Partnership
Scottish Enterprise
Scottish Life Sciences Association
Scottish National Blood Transfusion Service
Skills Development Scotland
Stevenage Bioscience Catalyst
Stratophase
Syncona Partners
Synpromics
Teesside University
The Automation Partnership
The Knowledge Transfer Network (KTN)
Tokyo Electron
Two BC
UKTI
UKTI Life Sciences Organisation
University College London
University of Edinburgh
University of Manchester
University of Oxford
Welsh Government Life Sciences

Appendix 3



2025 Market size estimates, based on Base and Upper Product Pricing Ranges: Sum totals rounded to nearest 1 billion. This study focuses exclusively on cell and gene therapy products within the broader sector of ATMPs, including gene therapies, T-cell immunotherapies and non-oncology cell therapies. It does not include tissue-engineered products or stem-cell transplants not involving any modification. The non-oncology cell therapies segment was calculated through a top-down methodology approach based on overall market rather than individual products. *(All values calculated in US dollars, with five year average rate used to account for current volatility (1.5 USD: 1 GBP), sum totals rounded to nearest 1 billion).*

As part of this Taskforce, the ABPI with support by PwC completed a high-level, compressed market and pipeline analysis of the cell and gene therapy market globally and in the UK. Based on this work, the global cell and gene therapy market is estimated to be worth between £9 to £14 billion ($14 to $21 billion) per year by 2025. The UK is expected to form 4% of this market, which is higher than its normal share for small molecules and biologics. UK's share is thus estimated between £0.4 to £0.6 billion ($0.6 to $0.9 billion) per year by 2025. Conservative growth estimates by 2030 were also calculated to be £21 to £32 billion per year ($31 to $48 billion) for the global market and £0.9 to £1.3 billion ($1.3 to $2 billion) for the UK.

This high-level analysis represents a conservative estimate based on current pipeline, technical capabilities and manufacturing capacity. From a point in time, the pipeline analysis and 2030 forecasts were focused on all products currently in clinical development (e.g. expected to have launched by 2022). Anything currently in preclinical/early clinical development that may be launching earlier and with payment schemes in place, or additional indications for the existing pipeline of products, could increase forecasts. Deferred payment models, currently considered as a way to ease upfront costs, were also estimated to begin in 2025 and thus not representing revenues accumulated from sales in previous years, which could also increase the forecasts.

Regardless of the margins, the time for the UK to capture this emerging market share is now. Given the nature of these therapies, much of the manufacturing will be required to take place in the UK, for the benefit of its patients, as well as the country's GDP and skilled manufacturing workforce. Investing now to grow the UK's manufacturing infrastructure can influence its pipeline and market shares in the future, further shaping these market forecasts. Similarly, investment to address the technical challenges that continue to hinder manufacturing scale and efficiency can also improve operating costs and product risk profiles. These could in turn affect the potential for uptake within healthcare systems, and in the future allow manufacturers to scale-up production to cover indications with larger patient populations.

To gain a fuller understanding of the cell and gene therapy market potential, a detailed pipeline and economic analysis should also be completed, including a full horizon scanning exercise, with bottom-up feedback from companies and input from regulatory, pricing and clinical adoption decision makers. Estimated product prices range from £30,000 to £450,000 (per patient, per treatment), based on the size of the target population and disease burden. Arguably, these could require innovative payment models, most likely involving deferred payments or conditional reimbursement schemes. Specifically for the UK, more detailed product pricing estimates could be developed based on existing NICE methodologies, as well as the NHS England schemes available for some disease types, in addition to new potential schemes that could be launched in the future.

Medicine Manufacturing Industry Partnership

abpi Bringing medicines to life

BIA UK BioIndustry Association

Innovate UK Knowledge Transfer Network

Website:

http://bia.me/MMIP_info

http://www.abpi.org.uk/our-work/mmip/Pages/default.aspx

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