

Accelerating
patient access
to advanced
therapies



Introduction

The Cell and Gene Therapy Catapult (CGT Catapult) is a private sector research organisation with a mission to grow the UK cell and gene therapy industry, delivering health and wealth. We are part of a network of Catapult centres supporting the implementation of the UK Government's Industrial Strategy, bridging the gap between scientific research and full-scale commercialisation.

Our vision

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

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2018-2019 in numbers

180

cell and gene therapy experts

110

projects

partnered on

28

CR&D projects totalling £4.5m

£5.2m

invested into cell and gene therapy projects

13

research partners progressed towards commercialisation

5

companies manufacturing at the CGT Catapult manufacturing centre

3

advanced therapy treatment centres

45

SMEs engaged with us

32

advanced therapy apprentices

Chairman's statement

Patients are now receiving cell and gene therapies for indications such as cancer, blood disorders and blindness. CGT Catapult is proud to be supporting this industry and its development in the UK.



Dr John Brown CBE, FRSE
Chairman



Working with our collaborators we are making the UK an attractive place for advanced therapy development and manufacturing.

CGT Catapult have established a broad range of capabilities and it was fantastic to see these endorsed by UK Research and Innovation (UKRI) and Innovate UK when we signed our five-year grant funding agreement. This investment of £70.6m over the next five years will play a key role in the continued advancement of the UK as a global leader in the development of cell and gene therapies, delivering health and wealth.

This funding builds on a £3.36m funding from the European Regional Development Fund (ERDF) and a £12m Industrial Strategy Challenge Fund (ISCF) award for the expansion of the manufacturing centre in Stevenage, which began in 2018. Five companies are now operating in the centre, developing their manufacturing capabilities and systems. The awarding of the manufacturing licences from the Medicines and Healthcare products Regulatory Agency (MHRA) is another step forward for the collaborators manufacturing therapies for clinical trial and commercial supply. In March this year TCR² Therapeutics became the first US company to establish their manufacturing base in the UK, making the decision to produce their therapies at the CGT Catapult manufacturing centre.

This year has also seen the establishment of the three advanced therapy treatment centres (ATTC) where the NHS and industry are working to address the unique and complex challenges of bringing cell and gene therapies to patients on a large scale along with London Advanced Therapies and other centres of excellence.

On behalf of the Board I would like to thank the whole of the CGT Catapult team, as well as our industry, academic and Government partners for their hard work in building the industry in the UK to what it is today.

Chief Executive Officer's statement

The cell and gene therapy industry has seen vast changes over the last year, treatments that were once theoretical are now available for patients right here in the UK.



Keith Thompson
Chief Executive Officer



This strong Government commitment is allowing us to build a world-leading advanced therapies ecosystem in the UK.

The UK has built one of the world's best ecosystems for research, development, manufacture, clinical adoption and reimbursement of advanced therapies. The progress seen by the industry translating the outputs of the UK's outstanding science base is thanks to continued Government commitment in innovative industries such as ours, supportive regulators doing all they can to get these life-changing therapies to the patients that need them, and the important investment and technical advances that have been made by the industry.

In 2018 NHS England announced the availability of CAR-T treatments for cancer patients, marking the first time patients in Europe have had routine access to CAR-T therapies. This has been a major landmark in accessing these and other personalised cancer therapies, setting the scene for the expanding pipeline of cell and gene therapies.

In the UK there are currently over 85 clinical trials ongoing, and with 70 cell and gene therapy companies operating in the UK, we expect a large number of potentially curative therapies to be reaching patients in the UK over the next few years.

Of course, the struggle is not over. It is our vision for the UK to be a global leader in the development, delivery and commercialisation of cell and gene therapies. To achieve this, we must continue to anticipate the challenges that will face the industry and find ways to overcome them in order to lead the world in delivering advanced therapies at scale to patients.

The complete UK cell and gene therapy ecosystem

The UK has become the go-to place for cell and gene therapy development and boasts a complete cell and gene therapy ecosystem. CGT Catapult is facilitating the growth of the UK ecosystem by working with industry, the research base, Government, the NHS, industry associations and international organisations.

The UK recognises the potential of ATMPs to address significant and growing unmet healthcare needs and boasts established manufacturing, clinical adoption and supply chain. By working closely with and connecting all of the organisations that make up the ecosystem CGT Catapult is actively addressing challenges to accelerate ATMP development.

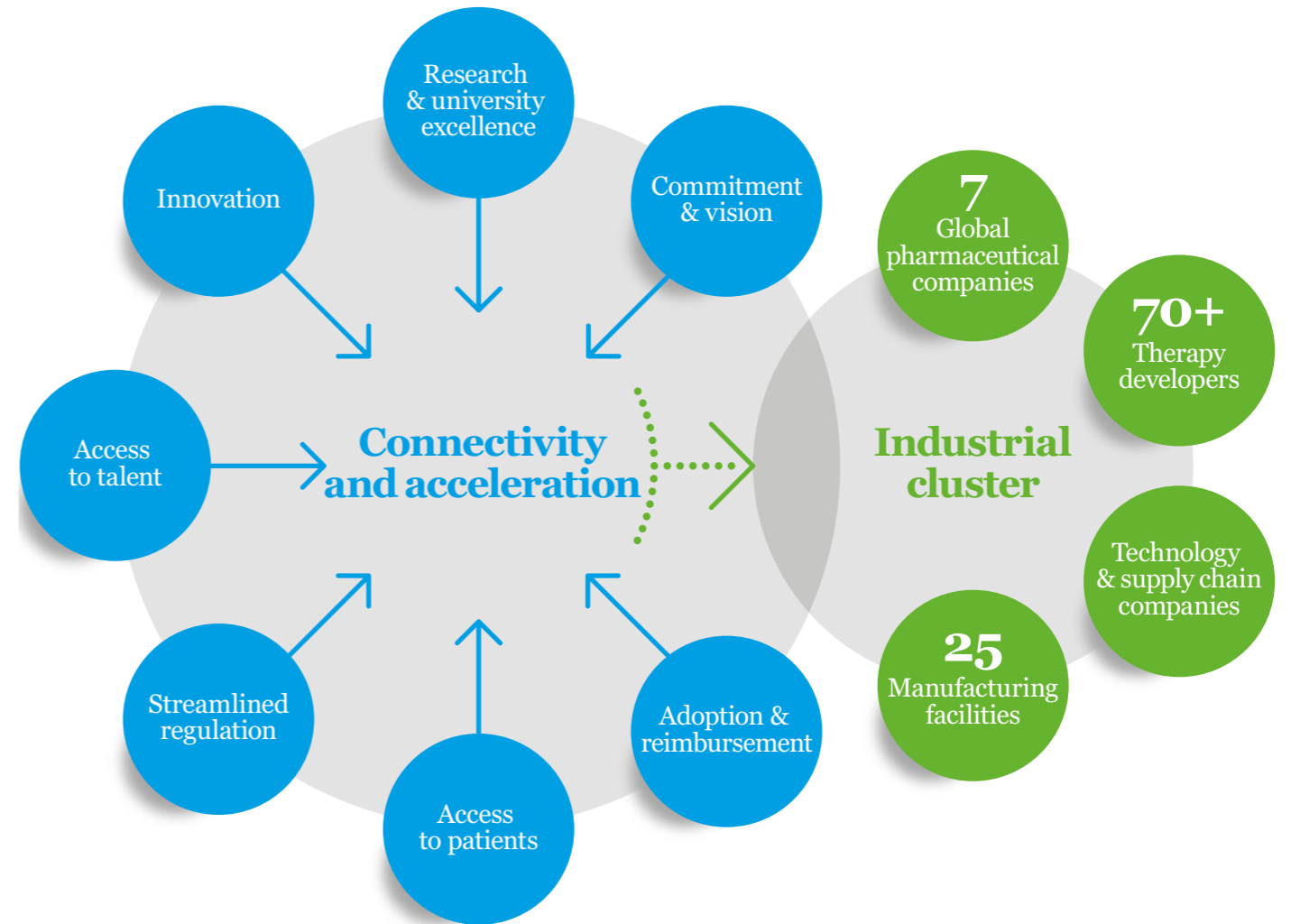
Organisations such as BEIS, UKRI, Innovate UK, BIA, NICE, NHS, and

initiatives such as the Government's industrial strategy, the MHRA's one stop shop, the ATTC Network and the CGT Catapult manufacturing centre, are examples of the commitment of the UK to innovation and to this field. The UK ecosystem provides research excellence through world leading universities and access to talent also through ATMP specific apprenticeship programs and with the industrial cluster providing industry expertise, services and support.



Through our Industrial Strategy we are determined to boost innovation, create new highly skilled jobs and use government investment to change people's lives for the better.

Rt Hon Greg Clark MP
Secretary of State for Business, Energy and Industrial Strategy



Helping develop systems for large scale manufacture and commercial supply

Providing the infrastructure and expertise to enable companies to develop their manufacturing capabilities and systems for large scale cell and gene therapy clinical studies and commercial supply.

In August 2018 the CGT Catapult manufacturing centre was awarded two licences from the MHRA, allowing collaborating companies to produce living medicines at scale to support clinical trials and commercial supply.

The centre is providing infrastructure and expertise to enable companies to develop new technologies, overcome barriers to manufacturing at industrial scale and shorten development timelines. Cell Medica have transferred the manufacture of their therapy to the UK, Adaptimmune will use the viral vector that they produce at the centre for the production of their T-cell therapies and Freeline will be supplying adeno-associated virus (AAV) for gene therapies.

At the centre, collaborating companies have access to good manufacturing practice (GMP) warehousing, quality systems, quality control (QC) provision, supply chain and healthcare provider links. Through the exploration and establishment of these new systems, expertise is being established in the UK, industrialising novel technologies and establishing large scale manufacturing systems.

Case study: Autolus

In March 2019, Autolus were the first collaborating company at the CGT Catapult manufacturing centre to have their manufacturing operations approved by the MHRA. They are now producing therapies at the centre for clinical trial.

Autolus have developed their large scale GMP and commercial manufacturing processes at the Stevenage-based manufacturing centre, including setting up raw material supply, qualifying equipment and developing a quality control strategy, all in collaboration with CGT Catapult.



Case study

5
companies manufacturing at the CGT Catapult manufacturing centre

100
highly skilled people employed by manufacturing centre collaborators

TCR² Therapeutics to manufacture at the Stevenage manufacturing centre

TCR² Therapeutics, a clinical-stage immunotherapy company pioneering the next generation of novel T-cell receptor (TCR)-based therapies for solid tumours and blood cancers, are the first U.S. company to develop their global manufacturing systems and establish operations at the CGT Catapult manufacturing centre.

TCR² Therapeutics highlighted the centre's flexibility and access to an established supply chain in their decision to utilise the CGT Catapult manufacturing centre and the UK ecosystem to support their immediate clinical trial needs.

“

The agreement with CGT Catapult enables us to meet our immediate clinical trials needs and have the flexibility of both our own dedicated manufacturing space and access to an established supply chain at one of the world's premier centres for cell and gene therapy development.

Garry Menzel, Ph.D.
President and Chief Executive Officer, TCR² Therapeutics



Anchoring global manufacturing in the UK

Actively facilitating the growth of the manufacturing and development cluster in the area surrounding the CGT Catapult manufacturing centre, anchoring this high value industry in the UK.

Research conducted by CGT Catapult showed major investment in the size and scale of the UK's manufacturing capability in 2018, supporting a significant growth in jobs and the production of cell and gene therapies. An increase in private and Government investment has led to a 60% increase in manufacturing space in the UK.

The CGT Catapult manufacturing centre has experienced exceptional demand for collaboration and as a result is doubling existing capacity with help from a £3.36m grant from the ERDF and a £12m ISCF award. Construction of a further six cleanroom modules is expected to be completed in 2019.

“

The go-ahead to make cell and gene therapies that could ultimately save lives is a great boost for the area, but also shines a light on the whole of the UK as an attractive place for this innovative industry to thrive. This project supports the ambition of our modern Industrial Strategy to maintain the UK's standing as a world leader in research and innovation.

Lord Henley
Life Sciences Minister



60%
increase in manufacturing space in the UK

1,500m²
manufacturing cleanroom space coming online in 2019

30%
increase in employment in the UK manufacturing industry

12
companies developing their manufacturing strategies in the UK

Data from CGT Catapult GMP manufacturing report 2018

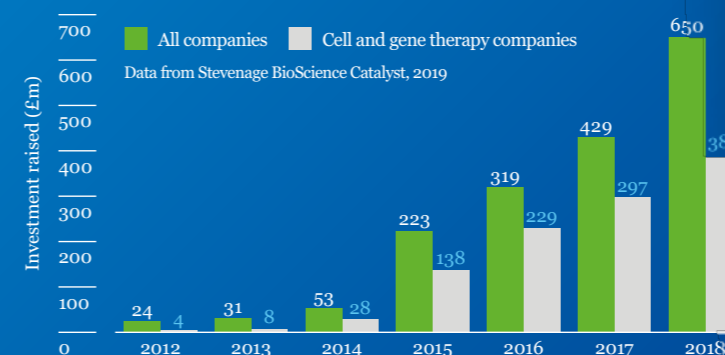
Case study

One of the largest cell and gene therapy clusters in the world

The Stevenage area is a globally recognised cluster that provides support for translation of cell and gene therapies and commercialisation within the UK and internationally. CGT Catapult decided to strategically locate its manufacturing centre in this area between London, Oxford and Cambridge in 2014.

Today, more than 10 cell and gene therapy companies operate out of the cluster in Stevenage, with five companies based at the CGT Catapult manufacturing centre, developing international production and supply chain systems, including developing skills. There are over 3,000 people employed in high-skilled jobs at the cluster. CGT Catapult's ongoing activity will indirectly support a further 4,000 new high-skilled jobs across the country, of which a quarter will be in the Stevenage area.

Companies at the cluster have experienced high growth, with cell and gene therapy companies raising a significant amount of investment, as shown in this data from Stevenage BioScience Catalyst:



Working together to accelerate patient access to advanced therapies

Coordinating NHS and industry to provide ready to use systems and solutions to accelerate adoption of advanced therapies.

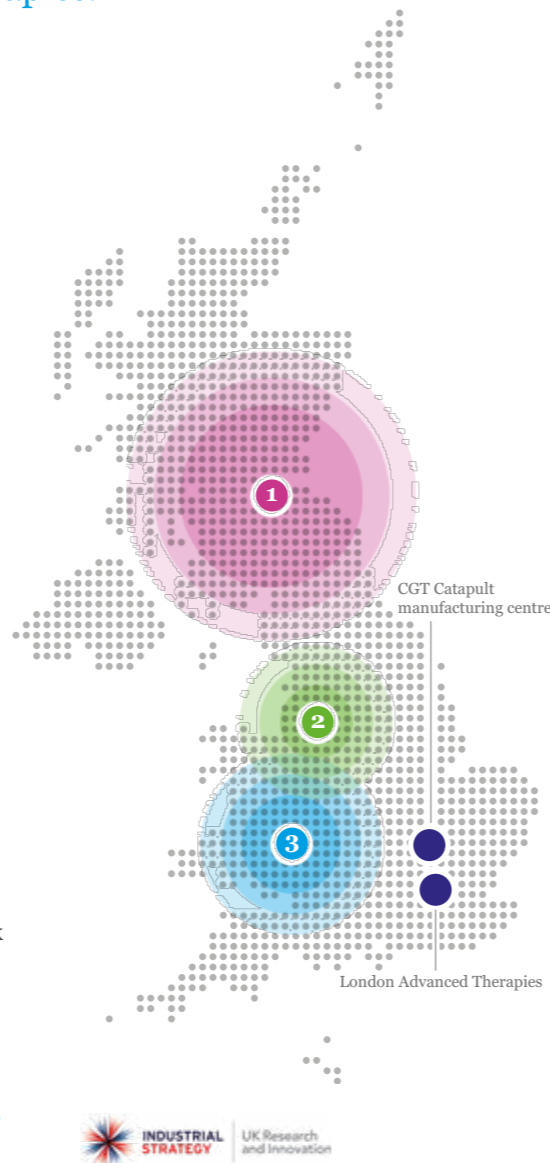
There are currently 85 clinical trials ongoing in the UK, with 2018 showing the largest number of phase III trials to date. A number of these commercially sponsored products are expected to be approved for clinical use over the next few years.

The ISCF have provided £30m to develop a network of advanced therapy treatment centres designed to develop systems and processes to support the routine supply of ATMPs by the NHS.

The network of ATTCs along with London Advanced Therapies and other centres of excellence, is addressing the unique and complex challenges of bringing these pioneering advanced therapies to patients. The ATTC network is coordinated by CGT Catapult.



Visit the ATTC website: www.theattnetwork.co.uk



This project has been funded by the ISCF, part of the Government's modern Industrial Strategy. The fund is delivered by UKRI.

Case study



1 Northern Alliance Advanced Therapies Treatment Centre
The Northern Alliance Advanced Therapies Treatment Centre (NA-ATTC) is a consortium of 20 industry, NHS and academic organisations led by Newcastle Hospitals and the Scottish National Blood Transfusion Service. This centre is focused on developing the systems and infrastructure required to support the delivery of cell and gene therapies.

2 Innovate Manchester Advanced Therapy Centre Hub
Innovate Manchester Advanced Therapy Centre Hub (iMATCH) is a Manchester-based consortium focused on scale-up of activity in ATMPs, aiming to improve access to ATMPs for patients, through integration of sample collection; development of electronic sample traceability and tracking systems utilising novel digital implementation.

3 Midlands and Wales Advanced Therapy Treatment Centre
The Midlands and Wales Advanced Therapy Treatment Centre (MW-ATTC) consists of a large regional network with the necessary commercial and NHS infrastructure to facilitate the delivery of advanced therapy treatments to patients. This centre is focused on overcoming the current challenges in clinical delivery of ATMPs.

Tackling key challenges in adoption of advanced therapies by the NHS

In September 2018, in collaboration with the Association of the British Pharmaceutical Industry and the UK BioIndustry Association, we brought together a number of expert stakeholders who explored how the ATTC Network can drive adoption of cell and gene therapies.

Among the topics dealt with were: procurement, manufacturing scale-up, supply chain issues, front-line delivery to patients, health economics and reimbursement. Key actions were identified to facilitate the reimbursement and supply of these disruptive therapies by the NHS. A summary of the outputs can be found at ct.catapult.org.uk/ATTCworkshop.

Increasing productivity through innovation

Supporting companies to realise the cost, scale and productivity gains needed to allow for regular use of cell and gene therapies.

In order to develop world-leading cell and gene therapy manufacturing productivity in the UK, we are developing smarter automated manufacturing processes that can automatically adapt to changing environments and process requirements with minimal intervention. Driven by in-process controls and feedback sensing, these new systems will reduce the cost of goods, optimise processing times and reduce failure rates.

Control over cell and gene therapies, such as autologous T-cell therapies, starts as early as the first collection of patient material and continues until the re-administration to the patient. Decisions at each stage are accompanied by data from multiple sources and locations. In a step change towards adaptive manufacturing, we are creating automated, closed manufacturing processes that require minimal human intervention.

Boosting productivity is key to making the most of the potential of advanced therapies, this increase in productivity and reduced cost of goods will be a major driver of industry growth in the UK.

Case study: Developing and accelerating closed GMP processes

Working with the University of Birmingham and Cancer Research UK, we have developed a GMP process for a transient CAR-T treatment for solid tumours. Because this type of therapy only lasts for a short time, it offers improved safety by reducing the chance of unwanted inflammatory responses.

This process has been closed so the product is not exposed to the surrounding environment, increasing reliability and safety, as well as reducing cost. The processing time has been reduced to 3-5 days, compared to an industry average of 10 days for a CAR-T process.



Case study

14

Collaborative projects developing intelligent manufacturing solutions

Cell Medica

We have worked with Cell Medica to transfer the GMP manufacturing process for an allogeneic CAR NKT product from the US to the CGT Catapult manufacturing centre in Stevenage. Cell Medica plans to start clinical trials in the UK with product manufactured at the centre in 2020.



Overcoming challenges in viral vector production

Working with industry to overcome the challenges of producing viral vectors at the required scale and cost to support late stage clinical trials and commercial supply.

Viral vectors are the most widely used system to deliver genetic material into cells. 65% of cell and gene therapy clinical trials ongoing in the UK use viral vectors to modify cells. This system is experiencing major clinical success, whereby both Kite's Yescarta and Novartis' Kymriah, which are now approved for use in a number of countries, use this technology to introduce genes into patients' own T-cells and generate immunity to cancer.

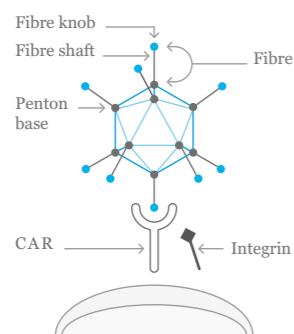
CGT Catapult established a dedicated viral vector laboratory in 2016 in response

to the expansion of these therapies, and industry concerns that the manufacturing of viral vectors could be a barrier to large scale and commercial supply of gene therapies. We are now working with a number of industry experts to overcome this barrier and our collaboration with Cobra Biologics and Pall Corporation to investigate continuous manufacturing of AAV is one example of this.

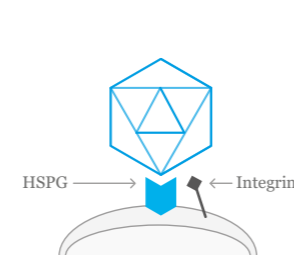
One way to achieve more potent, cost-effective and robust gene therapy products is to produce viral vectors at

Common viral vectors used in gene therapy

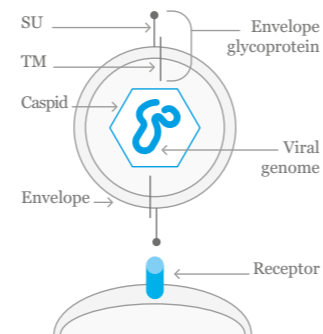
a. Adenovirus 5



b. AAV2



c. Retrovirus (lentivirus)



Nature Reviews | **Genetics** volume 8, pages 573–587 (2007)

65%

of clinical trials ongoing in the UK use viral vectors for genetic modification

lower cost with higher yield. We are developing technologies, along with the accompanying processes and analytics, to deliver high-yield and low-cost viral vector manufacturing. This will remove limitations on the growth and productivity for the UK advanced therapy industry.

Case study: Scaling-up viral production to clinical scale

Adaptimmune's TCR therapy uses a lentiviral vector to deliver genes to the cells. Adaptimmune's existing lentiviral process was successfully transferred into the CGT Catapult facility, with CGT Catapult supporting that transfer. Adaptimmune has implemented a number of process improvements, including scale-up of virus production and analysing different single use assemblies for the closed addition of reagents to the process. Adaptimmune aims to manufacture the first GMP lentiviral batches by end of 2019 for use in ongoing and future clinical trials.

Case study



Developing next generation viral vector analytics

As part of an ongoing collaboration with King's College London, a world-renowned supplier of viral vectors, CGT Catapult have improved the commercial scale production of AAV available to industry in the UK. By assessing and developing a suite of assays, viral vectors can be characterised supporting process improvements through increased process knowledge and understanding.

In a continuation of this collaboration, CGT Catapult is investing in second generation assays that will be more reproducible, more accurate and more sensitive. The aim of this work is to ultimately integrate with smart processing technologies.

Investing in the development of non-viral gene delivery systems

Working with the research base to support the emergence and development of the next generation of non-viral gene delivery technologies.

Non-viral gene delivery systems are another emerging method of delivering genetic material into cells. Non-viral gene delivery systems use organic or inorganic compounds or physical forces to deliver genetic material and can offer a low toxicity method of gene delivery with lower risk of immune reaction.

CGT Catapult have developed a unique in-house capability for the development of non-viral gene delivery technologies to support the industry. We have established a number of collaborations exploring different non-viral gene delivery technologies.

These disruptive technologies of the future have the potential to offer ease of scale-up with increased consistency, better safety profiles and lower cost than traditional viral vector delivery systems. The UK's capability in this area will ensure it is competitive on a global scale.

Case study: Using non-viral gene delivery capabilities to develop therapies for immune conditions

Regulatory T (Treg) cells are a promising new therapeutic approach for immune-related diseases which require non-viral methods of gene delivery. As part of a Horizon 2020 consortium of 10 industrial and academics partners, CGT Catapult is investigating a number of promising non-viral approaches for gene transfer, that will be compared and validated for use in Treg-based therapies.

Case study

pHion Therapeutics

CGT Catapult formed its first non-viral gene delivery collaboration with pHion Therapeutics, a spin-out from Queen's University Belfast. The synthetic protein RALA, produced by pHion Therapeutics, is designed to transport genetic material into cells and CGT Catapult is investigating the ability of the protein to transfect clinically relevant cell types.



We are excited to continue to work with CGT Catapult to develop pHion's manufacturing processes to GMP standard and to work with CGT Catapult's network of companies to develop new CAR-T or TCR-based therapeutics utilising the RALA technology.

Professor Helen McCarthy
Founder and Chief Executive Officer of pHion Therapeutics



Supporting UK researchers in reaching commercialisation

Supporting cell and gene therapy researchers commercialise their research in order to increase the flow of new technologies that will feed the industry and leveraging UK investment.

The UK has a strong position in the global cell and gene therapy industry thanks to sustained and targeted investment in research. New companies are now increasingly being formed through successfully financed spin-outs from the research base.

To increase this flow of new technology, CGT Catapult is working with several leading university groups in order to increase spin-out readiness by accelerating and supporting the development of therapies and advancing technologies into clinical use.

To support early stage researchers, CGT Catapult have hosted a series of seminar events at UK universities. We have been able to advise, assist and accelerate development through educative seminars and one-to-one feedback meetings.

CGT Catapult is actively engaging with the investor community on a level not seen in previous years and is establishing collaborations in order to increase the visibility of the UK research base to this audience.

Case study: Bringing SMEs together with investors

To increase the visibility of the UK research base to industry and investors, CGT Catapult, in collaboration with Eversheds Sutherland, FUJIFILM Diosynth Biotechnologies, Mathys & Squire and Stifel, held an investor day which brought together early stage researchers and venture capital investors, along with other stakeholders that are fundamental to the commercialisation process including legal, intellectual property and investment banks.

This enabled 11 SMEs to increase their visibility with investors and pitch their technology-driven products, therapies, services and processes.



Case study

Supporting translation of research

We have been engaging with academic centres of excellence to disseminate expertise and knowledge of commercial translation to speed up the adoption of technologies emerging from UK research.

Our seminar series has allowed researchers to benefit from expertise in key components of the pathway to commercialisation, including intellectual property, regulation, health economics and market access and process scaling and manufacturing. Researchers have also been able to receive one-to-one feedback on their projects from CGT Catapult subject matter experts through advice clinics and workshops on specific topics.

- Seminars
- Advice clinics
- Workshops



Developing novel reimbursement practices

Working to develop new approaches to reimbursement, including risk sharing and novel payment mechanisms, to allow healthcare systems to accommodate new therapies.

Cell and gene therapies are different to traditional medicines and existing reimbursement practices do not easily accommodate the possible outstanding value that these potentially curative and disease modifying drugs could deliver.

CGT Catapult works with companies in the UK, Europe and the US, ranging from SMEs to larger biotechs, and also academics, to encourage them to think about reimbursement during early stages of development and helping them to develop their strategies. We also collaborate with agencies in the UK including NHS England, NHS Scotland, NHS Wales, NICE, Scottish Medicines Consortium and the All Wales Medicines Strategy Group, as well as internationally to develop reimbursement risk sharing and payment mechanisms as well as prototyping these new approaches.

Performance-based reimbursement can improve patient access; however, this requires specific data collection mechanisms to calculate patient benefit and determine levels of payment. Through research projects with industry stakeholders, we have concluded that current data collection infrastructure in several indications is not sufficient to facilitate outcomes-based reimbursement.

Outcomes-based reimbursement requires a universal data collection infrastructure, however this implementation is associated with considerable challenges. In developing solutions to these challenges, the network of ATTCS will become an exemplar specialist ecosystem for reimbursement of cell and gene therapies.



Case study

3 publications in high impact journals

Performance-based, managed entry agreements

Managed entry agreements are mechanisms by which companies and payers share financial and clinical risks associated with the introduction of new medicines.

The use of these methods is increasingly considered as an option to enable adoption and patient access to advanced therapies. We have investigated the requirements of implementing performance-based pricing schemes, identifying the infrastructure needed to ensure streamlined use of performance-based pricing schemes within the NHS.

Read the paper in full at ct.catapult.org.uk/hema.



Fuelling growth through apprenticeships

The Advanced Therapies Apprenticeship Community has been established to develop the first apprenticeship programme designed specifically to train and upskill individuals to develop, manufacture and deliver advanced therapies at scale.

The skilled scientists who are needed to develop, manufacture and deliver these innovative therapies underpin the success of the industry. As the industry grows and companies progress towards manufacturing and delivery, more new and unique skills are required. Recruitment and retention of these individuals is becoming increasingly challenging, potentially limiting the growth of the industry.

CGT Catapult have been awarded £1.5m by the ISCF to establish the Advanced Therapies Apprenticeship Community (ATAC) developed in partnership with the Medicine Manufacturing Industry Partnership (MMIP) with support from the Gatsby Foundation. The purpose of the community is to create a ready supply of skilled talent ranging from manufacturing operatives to technical experts and researchers to fuel the growth of the UK advanced therapies industry.

CGT Catapult is working with industry to develop a series of bespoke apprenticeship standards and continuing professional development programmes that train and upskill individuals to develop, manufacture and deliver advanced therapies at scale.

Visit the website:
<https://www.advancedtherapiesapprenticeships.co.uk/>

32 apprentices have been employed with a total of 65 apprentices employed over the first 12 months of the programme

11 companies have hired apprentices

5 programmes have been developed

- Science Manufacturing Technician (Level 3)
- Technician Scientist (Level 5)
- Senior Leader (Level 7)
- Regulatory Specialist (Level 7)
- Modern Apprenticeship in Life Sciences (Scotland)

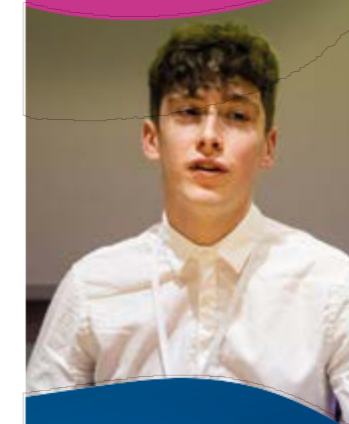


Jodie Patterson
 Advanced Therapies Apprentice,
 Cobra Biologics



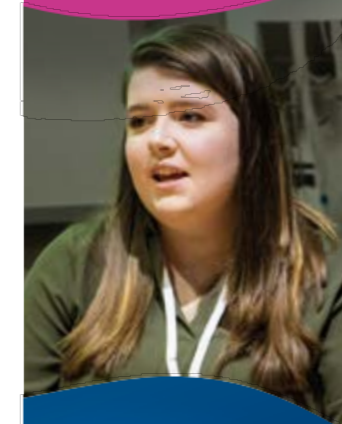
“ An apprenticeship seemed like a perfect opportunity to gain access to the industry and build my skills and understanding in the field.

Mike Devey
 Advanced Therapies Apprentice,
 Allergan Biologics



“ The skills I have gained have already set me up for a career within the biotech industry.

Emily Bird
 Advance Therapies Apprentice,
 Replimune



“ I get to study a subject I am passionate about whilst working amongst, and learning from, a brilliant team of experienced professionals.

Richard Shortland
 Senior Organisational Manager, NHS Blood and Transplant



“ Adding apprenticeships to our advanced therapies area was really important for us to help meet our workforce needs.

“ By funding skills, research and infrastructure in this sector we can help develop the solutions to tackle debilitating illnesses and conditions and boost the UK's prime position in clinical development and commercialisation.

Dr Ian Campbell,
 Interim Executive Chair, Innovate UK for UKRI.



Thank you to the MMIP and Gatsby Foundation for their contribution to this project.

This project has been funded by the Industrial Strategy Challenge Fund, part of the Government's modern Industrial Strategy. The fund is delivered by UKRI.

Informing, shaping and influencing

Our environment shaping activities are aimed at continuously improving and creating an advantageous environment in the UK for therapeutic developers, supply chain providers and manufacturers so that they can deliver therapies to patients at scale.



National

CGT Catapult continually engages with UK Government at many levels in order to encourage support for the sector. We have hosted delegations at the CGT Catapult manufacturing centre in Stevenage and the development centre in London. Government departments that we have interacted with include Department for Business Energy and Industrial Strategy, HM Treasury and Department of Health and Social Care.

Local

This year has seen exceptional growth of the cell and gene therapy cluster in Stevenage. This is thanks to the work of companies in the area including Stevenage Bioscience Catalyst, GSK, GE Healthcare Thermo Fisher and CGT Catapult, as well as the support of local councils and funding from the Hertfordshire Local Enterprise Partnership.



Global

Health economics has been a hot topic over the year, as organisations start to think about how these transformative therapies will be reimbursed by healthcare systems. CGT Catapult have been involved in several events with industry experts and policy makers. In September 2018 we gathered expert stakeholders together to explore how the NHS will cope with the high upfront cost of cell and gene therapies, and in March 2019, along with the New Statesman and bluebird bio, we brought together a group of experts to discuss the evolving challenges in access to gene therapy.

Case study

Accelerating patient awareness

The ARM Foundation for Cell and Gene Medicine is a global foundation dedicated to providing the education, information, and research needed to accelerate patient awareness of and access to transformative therapies. CGT Catapult is engaged with ARM Foundation in an international, multi-stakeholder project to quantify the potential impact of advanced therapies as a class, upon healthcare and healthcare systems worldwide. CGT Catapult sit on the advisory panel of the project providing general modelling and economic expertise and are representing the interests and perspectives of the single payer systems typical of Europe. The aim is to produce information that will allow and assist major healthcare providers, governments and politicians to understand the long-term opportunity that the sector presents and to facilitate the decisions needed to prepare for its impact.



We are delighted to have CGT Catapult participating on our Health and Economic Impact Model development team. CGT Catapult's insight and experience in working with European stakeholders to understand and quantify disease burden and the potential value of a durable and effective CGTx therapy that can be measured accurately is a key contribution to our efforts."

Morrie Ruffin
ARM Foundation for Cell and Gene Medicine

The advanced therapies industry is seeing great successes with therapies moving from clinical trial to licensed products for larger numbers of patients. Over the coming years patient treatment is expected to increase significantly from thousands to tens of thousands of therapies being delivered to patients in the UK each year.

To achieve this scale and embed advanced therapies in the health system for routine delivery, many barriers still need to be addressed. CGT Catapult will work with industry to identify barriers to growth, and continue to develop and exploit capabilities to address them.

Current barriers identified include:

- High cost of goods and low productivity
- Supply chain
- Clinical adoption and uptake
- Reimbursement
- Skills shortages

Developing a portfolio of technologies to support industry and academia

We will develop a portfolio of technologies that industry and academia can access to accelerate the commercialisation and industrialisation of new cell and gene therapies. This includes developing new manufacturing technologies and systems, as well as building our capabilities and technology platforms in quality control that accommodate the convergence of analytics, bio-informatics, genomics and cell and gene therapies.

Expansion of CGT Catapult manufacturing centre

The expansion of the CGT Catapult manufacturing centre will double its existing capacity. We will work with our collaborators to identify new manufacturing system and scale related challenges and we will address these collaboratively with industry.

Growth of cell and gene therapy cluster in Stevenage

The cell and gene therapy cluster in Stevenage is rapidly growing and we expect it to become the leading cluster for cell and gene therapy research, development and manufacturing in Europe. This will require continued investment into the site to allow new manufacturing technologies to be cultivated, and a mature integrated supply chain created.

The growth of the cluster in Stevenage will provide high value jobs and support the network of ATTCs.

Development of ATTC network

CGT Catapult will continue to support and coordinate activities to create an effective ATTC Network working with the three existing centres, London Advanced Therapies and other centres of excellence. We hope to see companies quickly establish proof to market using exemplar models and systems developed through the network, with licensed advanced therapies being delivered to patients. The first patients are already being treated through clinical trial and licensed products in these centres.

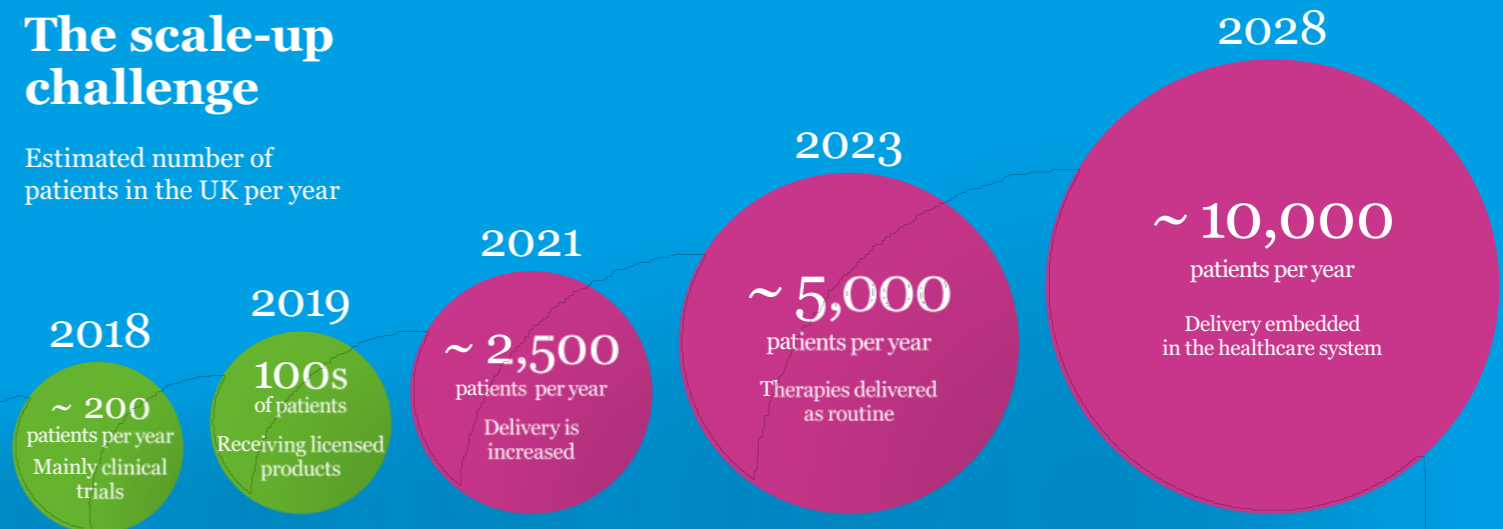
Cell and gene therapies approved for use in the European Union

Product	Company	Product type	Indication
Zynteglo	bluebird bio	Gene therapy	Beta thalassaemia
Luxturna	Spark Therapeutics	Gene therapy	Hereditary retinal dystrophy
Yescarta	Kite/Gilead	Gene therapy	B-cell lymphoma
Kymriah	Novartis	Gene therapy	B-cell acute lymphoblastic leukaemia
Alofisel	Takeda Pharma A/S	Cell therapy	Complex perianal fistulas in adult patients with Crohn's disease
Spherox	CO.DON AG	Tissue engineered product	Cartilage repair of the knee cap
Zalmoxis	MolMed	Gene therapy	Haematological malignancies
Strimvelis	GSK	Gene therapy	Severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)
Imlygic	Amgen Europe BV	Gene therapy	Melanoma
Holoclar	Chiesi Farmaceutici S.p.A.	Tissue engineered product	Corneal diseases

Adapted from Tracy T. et al 2018

The scale-up challenge

Estimated number of patients in the UK per year



Financial highlights

The financial information in this review is extracted from the consolidated statutory accounts for The Cell Therapy Catapult Limited for the year ended 31 March 2019.

Turnover

	2019 £'000's	2018 £'000's
For the year ended 31 March 2019		
Innovate UK core revenue grant funding	15,276	12,580
Collaborative research and development and other grant income	4,518	4,825
Commercial income	7,029	3,660
Total	26,823	21,065

Capital funding

	2019 £'000's	2018 £'000's
For the year ended 31 March 2019		
Innovate UK capital grant funding	11,521	5,851

Balance sheet

	2019 £'000's	2018 £'000's
As at 31 March 2019		
Fixed assets	53,157	44,341
Net current assets	9,758	2,704
Creditors	7,017	(337)
Provisions for liabilities	8,689	(7,316)
Net assets	47,209	39,392
Capital and reserves	47,209	39,392

Cell and Gene Therapy Catapult is a trading name of Cell Therapy Catapult Limited (limited by guarantee), registered in England and Wales under company number 07964711 with registered office at 12th Floor Tower Wing, Guy's Hospital, Great Maze Pond, London SE1 9RT.

Corporate governance

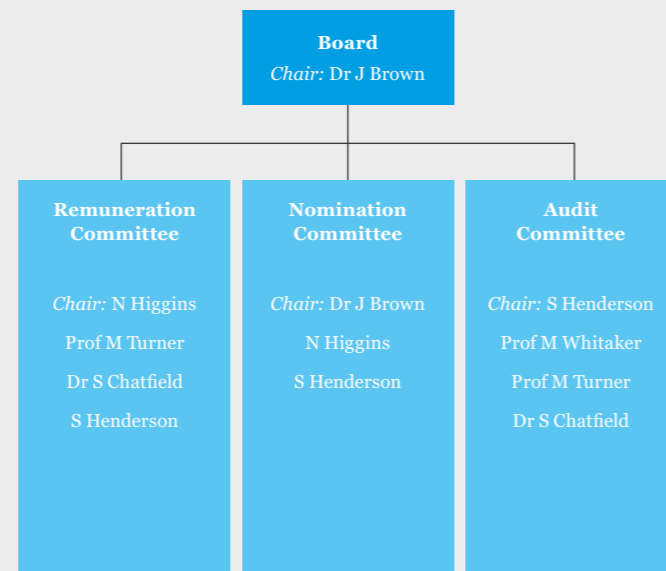
The Cell Therapy Catapult Limited is an independent private company limited by guarantee incorporated as a not-for-profit research organisation. CGT Catapult receives substantial grants from Innovate UK and works in coordination with them while remaining independent and is self-governing.

Our subsidiaries

- Cell Therapy Catapult Services Limited
- Cell and Gene Therapy Catapult
- Chimeric Therapeutics Limited

Our committees as of April 2019

- We have established three committees that meet independently and make recommendations to the Board



Non-Executive Directors



Dr John Brown CBE, FRSE
Chairman



Nick Higgins
Director



Professor Marc Turner
Director



Professor Michael Whitaker
Director



Dr Steven Chatfield
Director



Stuart Henderson
Director



Sir Bruce Keogh
Director



Hilary Newiss
Director



Professor Uta Griesenbach
Director

Executive team



Keith Thompson
Chief Executive Officer



Matthew Durdy
Chief Business Officer



Dr Jacqueline Barry
Chief Clinical Officer



Dr Stephen Ward
Chief Manufacturing Officer



Dr Jonathan Appleby
Chief Scientific Officer

Advisory panel

Professor Paul Fairchild
University of Oxford

Stuart Forbes
University of Edinburgh

Dr Regina Fritsche-Danielson
AstraZeneca

Michael Hunt
ReNeuron

Dr Edward Hodgkin
Syncona Partners

Professor Graham Lord
Kings College London

Professor Chris Mason
AvroBio

Professor Petra Reinke
Charité – Universitätsmedizin Berlin

Professor Adrian Thrasher
University College London

Martin Westberg
GE Healthcare

Dr Mark Zimmerman
J&J Innovation

CATAPULT

Cell and Gene Therapy

Thank you to the people we have worked with over the year, including:

Funders and investors



National and international organisations



National and international companies



National health services



Regulators



Researchers



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We work with
Innovate UK