

The UK ATMP landscape

Matthew Durdy,

Chief Executive Officer, Cell and Gene Therapy Catapult

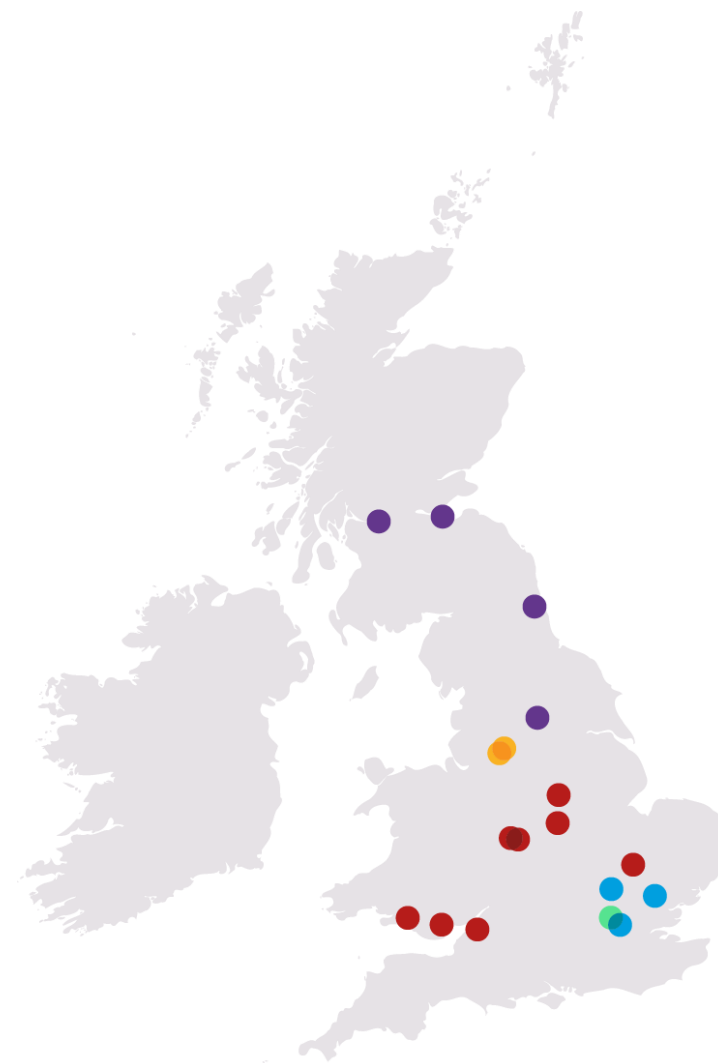
Chaired by Uta Griesenbach

Professor of Molecular Medicine, Imperial College London
President of the British Society for Gene and Cell Therapy

Funded by



**UK Research
and Innovation**



Coordinated by

CATAPULT
Cell and Gene Therapy

Who are LAT and the ATTCs?



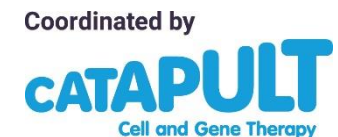
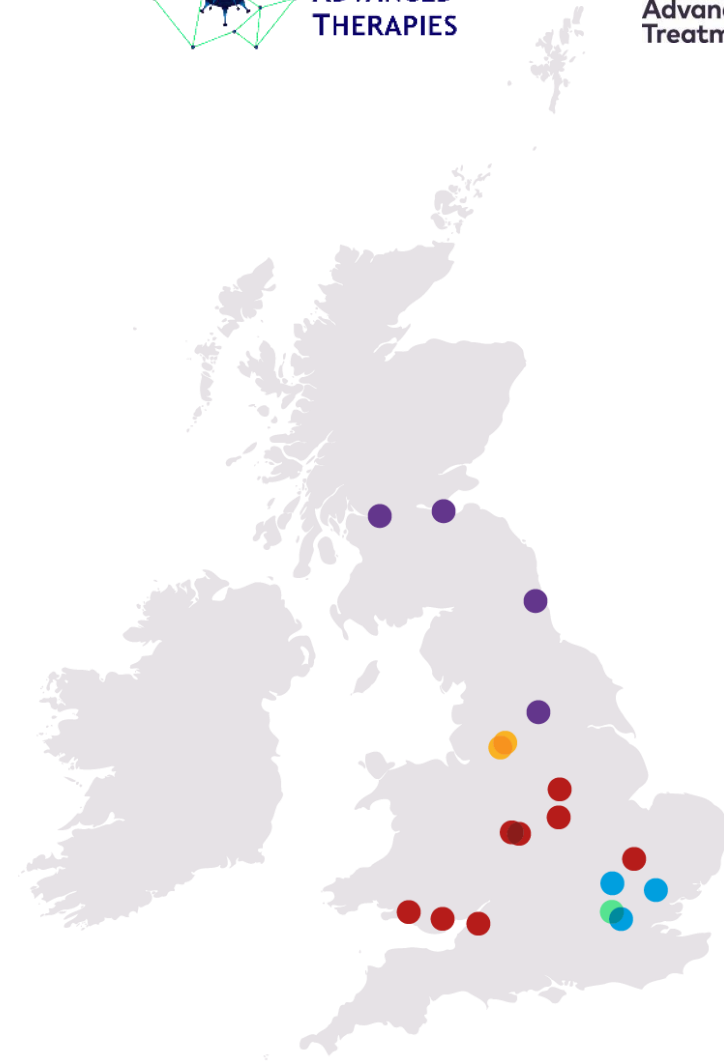
The ATTC (Advanced Therapy Treatment Centre) network is funded by Innovate UK and the Industrial Strategy Challenge Fund

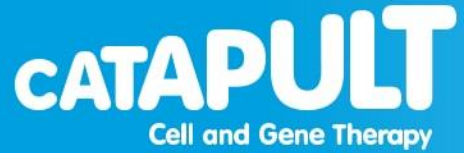
London Advanced Therapies (LAT) is funded by Research England

The centres are working together, along with the Cell and Gene Therapy Catapult to specifically look at the training requirements for the current workforce and what needs to be put in place for them to be ready to deliver the treatments that are currently being developed.

This series of webinars is designed to help increase the awareness of advanced therapies and their impact in the clinic

Find out more at <https://www.theattcnetwork.co.uk/>





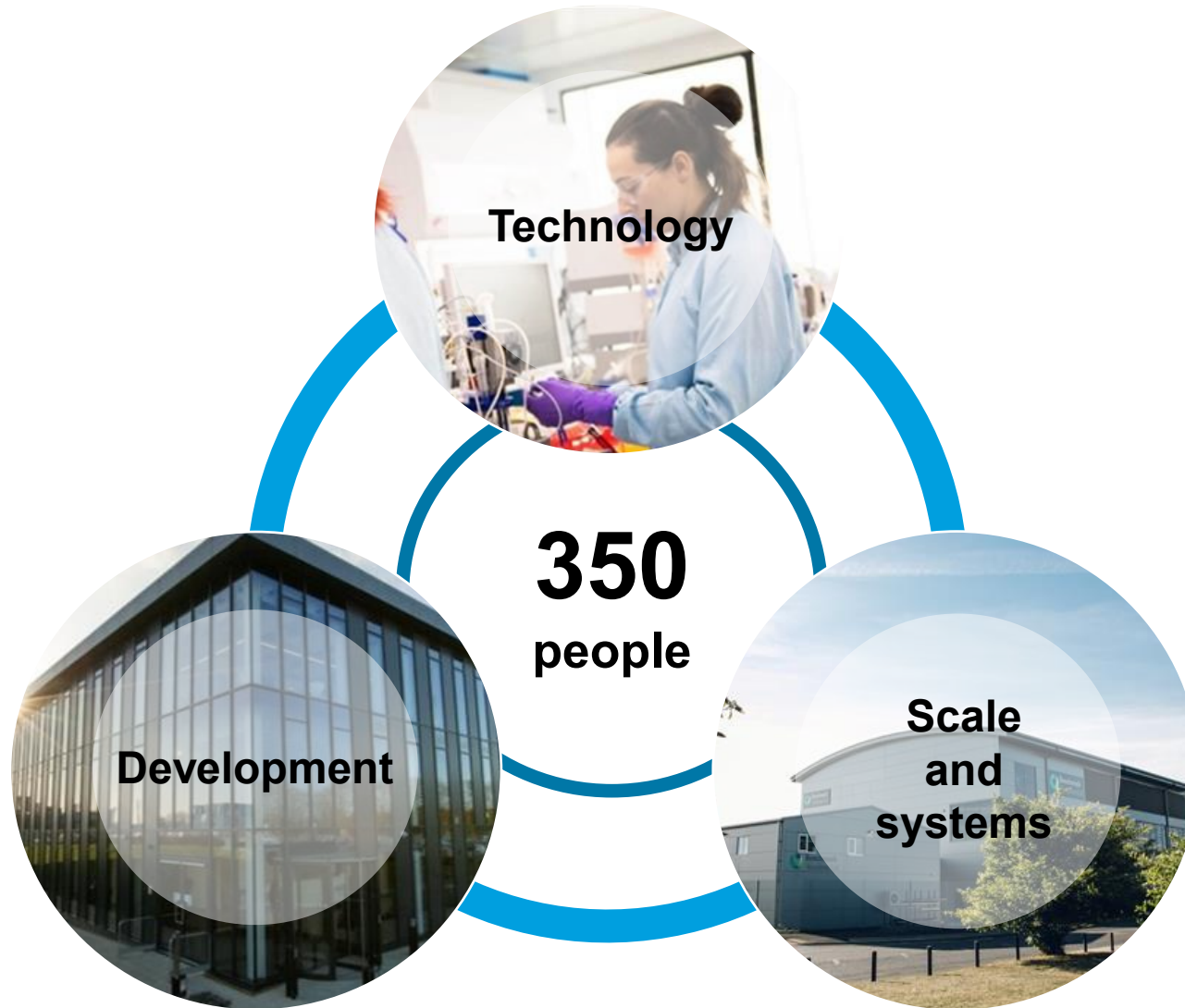
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Matthew Durdy, Chief Executive Officer
Cell and Gene Therapy Catapult

23 February 2020



The Cell and Gene Therapy Catapult perspective



Part of a **world-leading network** of technology and innovation centres



Provide access to unique technical **facilities** and **expertise** to help adopt, develop and exploit innovations



Bridge the gap between businesses and academic research



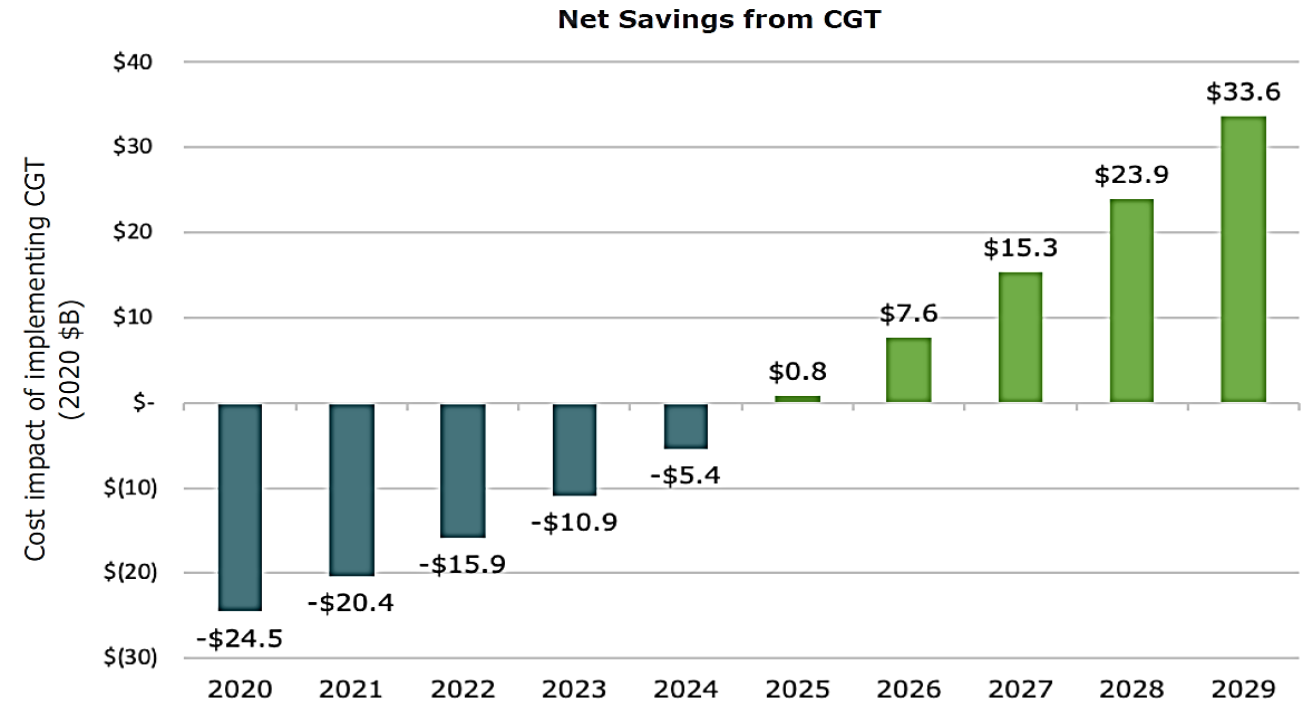
Established by Innovate UK as a **not-for profit**, independent centre

Not a better medicine.....



Emily Whitehead

ARM White Paper: A Transformative Therapy Value Model for Rare Blood Diseases, January 2020



Cost savings from a reduction in healthcare spend over time for patients with access to SCD, MM, and Hem A populations

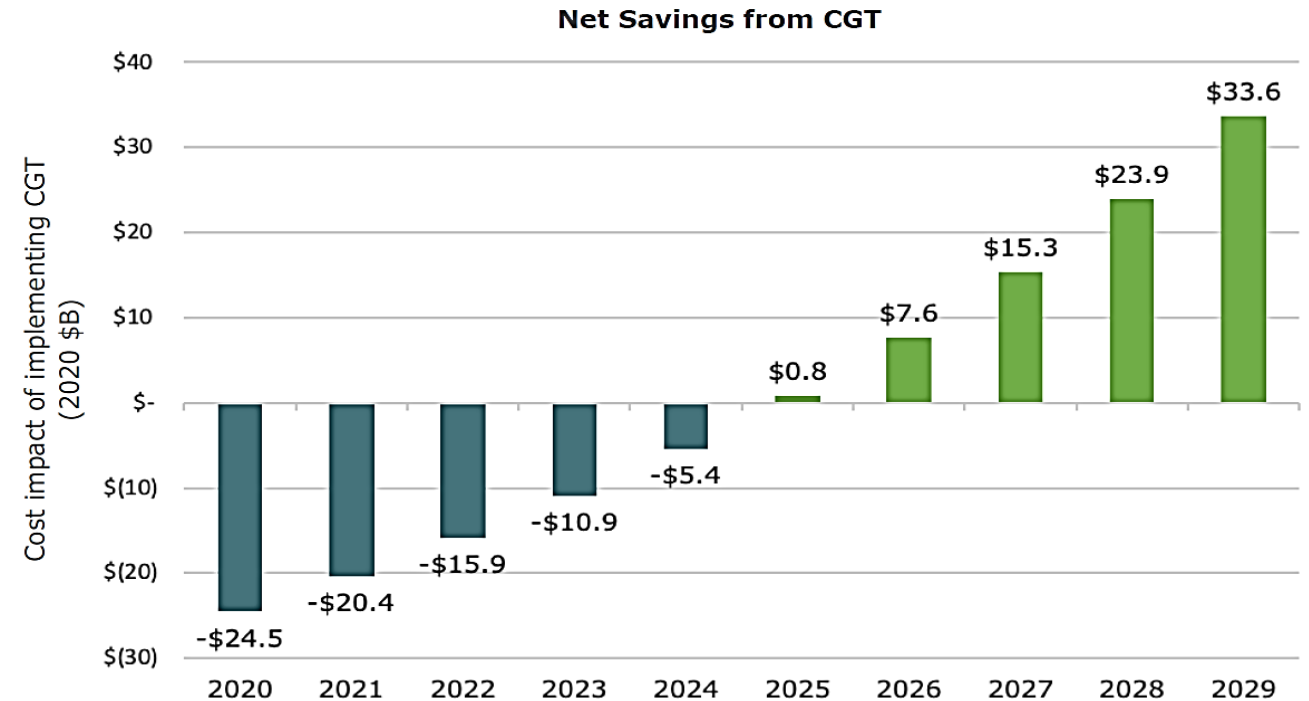
.....a new approach to healthcare

Not a better medicine.....



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Cost savings from a reduction in healthcare spend over time for patients with access to SCD, MM, and Hem A populations

.....a new approach to healthcare

What makes cell and gene therapies different?

Change	Treat	Example: haemophilia A
<ul style="list-style-type: none">■ Aim to address underlying cause■ Make fundamental change in disease state■ High initial intensity of treatment and cost■ Benefits span long periods■ Higher benefit-cost risk■ Lower healthcare system burden	<ul style="list-style-type: none">■ Treat the problems caused by the disease■ Modifies symptoms■ Costs distributed over life of treatment■ Costs synchronous with benefit■ Low benefit-cost risk■ High healthcare system burden	<p><i>Treat:</i></p> <ul style="list-style-type: none">■ <i>\$200k per annum treatment cost (Factor VIII)</i>■ <i>Average lifespan 65 years</i> <p><i>Change</i></p> <ul style="list-style-type: none">■ <i>Gene therapy to insert missing genetic material</i> <div></div> <p><i>"I never even think about haemophilia. It's just awesome."</i></p> <p><i>-Matthew Harrington</i></p>

10 licensed ATMPs approved in the EU

Product	Company	Indications
Zynteglo	bluebird bio	Beta thalassaemia
Luxturna	Spark Therapeutics	Hereditary retinal dystrophy
Yescarta	Kite/Gilead	Diffuse large B-cell lymphoma, primary mediastinal large B-cell lymphoma
Kymriah	Novartis	B-cell acute lymphoblastic leukaemia, diffuse large B-cell lymphoma
Alofisel	Takeda Pharma A/S	Complex perianal fistulas in adult patients with Crohn's disease
Spherox	CO.DON AG	Cartilage repair of the knee cap
Strimvelis	GSK	Severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)
Imlygic	Amgen Europe BV	Melanoma
Holoclar	Chiesi Farmaceutici S.p.A.	Limbal stem cell deficiency
Tecartus	Kite/Gilead	Adult patients with relapsed or refractory mantle cell lymphoma

US and Europe: two major healthcare markets

Companies by region

1,085 regenerative medicine companies worldwide, including cell and gene therapies

North America	543
South America	13
Asia & Oceania	295
Africa	25
Europe	209



1,220 ATMP focused clinical trials

383 Phase I trials

685 Phase II trials

152 Phase III trials

Total global financing:
\$19.9bn raised in 2020

103% increase from 2019

The UK's leading position in the global industry



10

Licensed ATMPs
approved



33%

Share of global ATMP
companies in Europe



10

Global pharmaceutical
companies



Largest

cell and gene therapy
cluster outside of the US



26

Manufacturing
facilities



3,000+

Jobs in the sector



12%

Global ATMP trials
in the UK



90+

Therapy developers



£2.5bn

Investment received by UK
companies since 2012



£1.7bn

Total financing for UK ATMP
companies in 2018-2020



3

Advanced Therapy
Treatment Centres



Global **1st** CGT specific
apprenticeship programme
and industry driven training
network



2025 vision

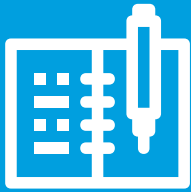
- **4,000+** jobs
- **£2bn** in revenue

2035 vision

- **18,000+** jobs
- **£10bn** in revenue

The UK: an attractive ATMP destination

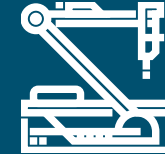
World-class research



12% of global clinical trials



Growing manufacturing footprint



Unique initiatives

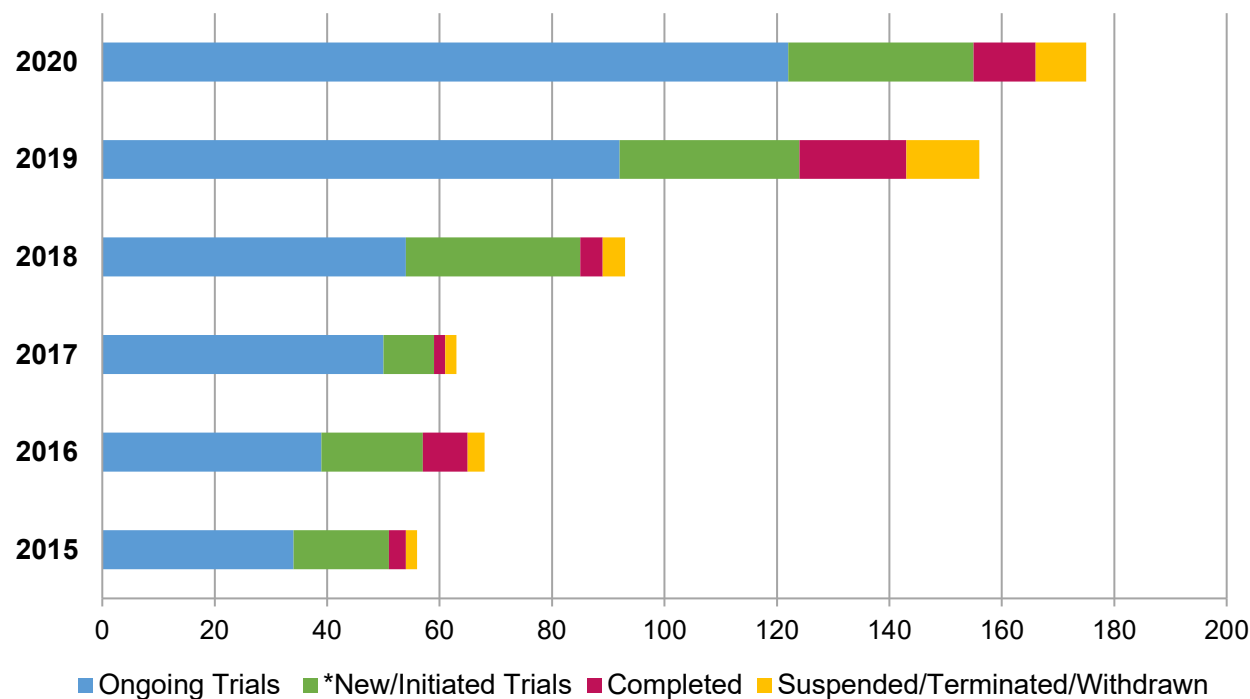


Catapult network

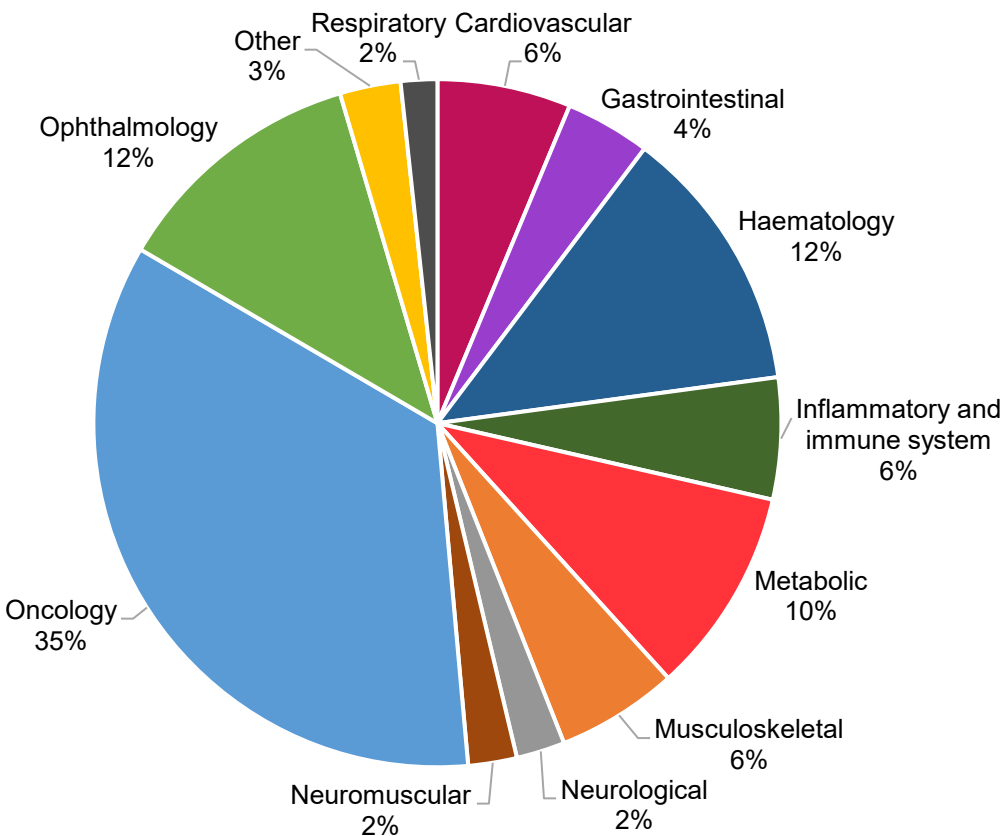
Clinical adoption centres

Skills programmes

Number of ongoing, initiated, completed, and closed trials 2015-2020



Distribution of UK ATMP clinical trials by therapeutic area in 2020



Data from CGT Catapult Clinical Trials Database, Feb 2021

Research and manufacturing facilities

Research hotspots throughout the UK



Distribution of MHRA licensed GMP facilities



26 facilities



159 cleanrooms



~11,756m²
cleanroom footprint



1,310
full-time employees

Data from CGT Catapult 2020 GMP manufacturing report

Impact of CGT Catapult: Stevenage facility



£1.6bn

total investment into the campus since 2013

£1.13bn

investment into cell and gene therapy companies

30%

campus tenants are cell and gene therapy companies

5

cell and gene therapy developers located at the CGT Catapult facility (out of 8 on campus)

Enhancing the growth and productivity of UK companies

Example:

Supported Autolus to grow significantly since 2018, raising £140m in public offerings since its IPO and increasing its workforce by 80%

Attracting international companies to the UK

Example:

The first US-based company to set up manufacturing in the UK is TCR² Therapeutics at the Stevenage facility

Growing the third largest CGT cluster globally

Example:

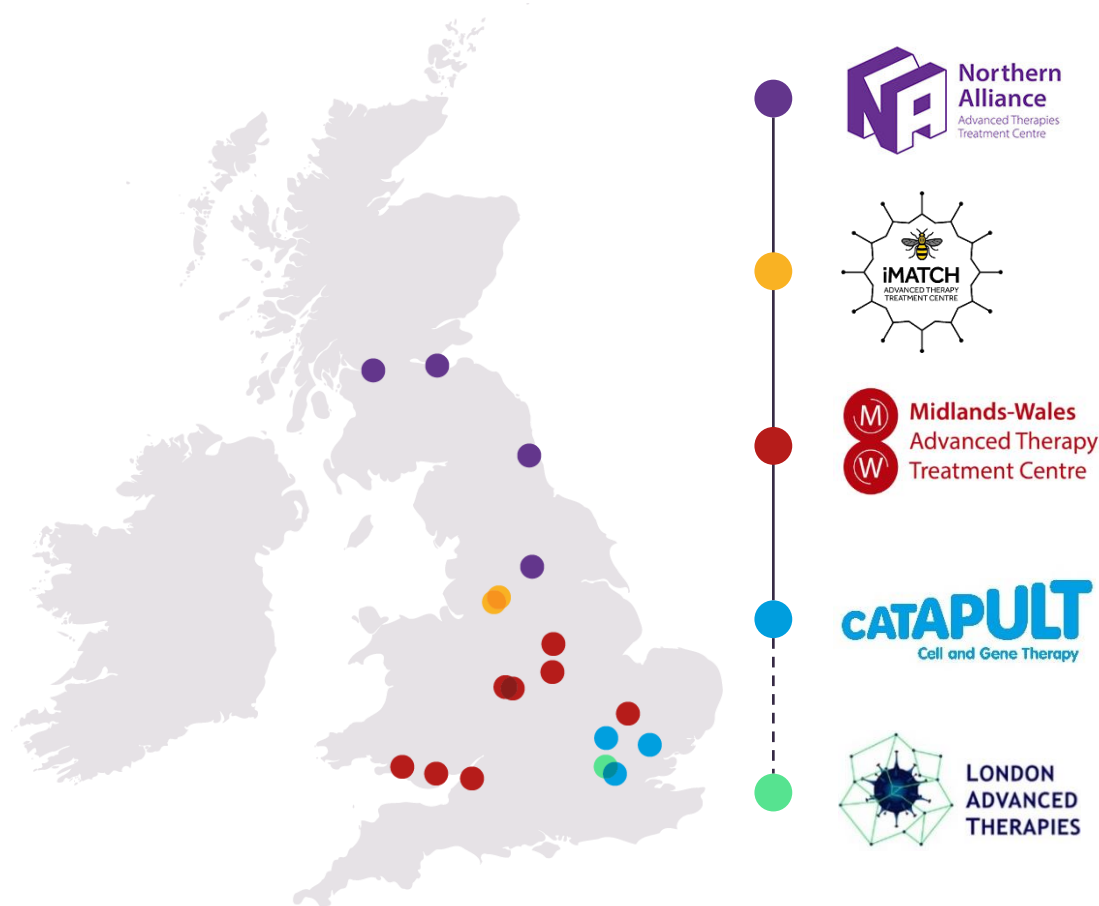
Driven 2/3 of the investment into the cell and gene therapy companies based at the Stevenage Advanced Therapies Campus (£630m of which £548m in equity)

Accelerating patient access

Example:

Clinical material produced by our collaborators at the facility is reaching patients nationally and in the US

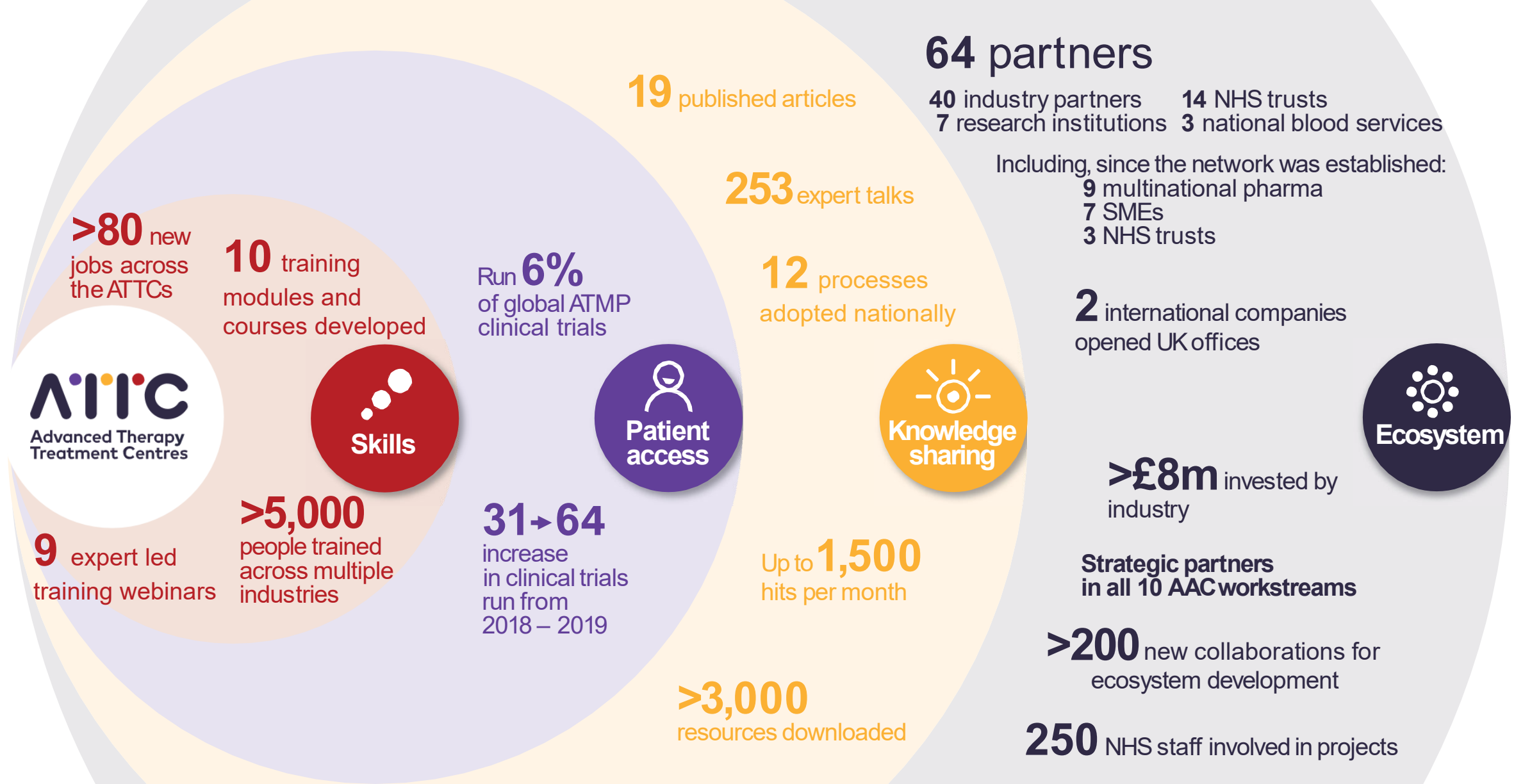
ATTC network



- Develop and deliver systems for the routine delivery of these disruptive therapies
- Disseminating the learnings and systems from the ATTC clinical centres to enable more widespread adoption of these innovative therapies across the NHS
- **64 partners** – true collaboration between NHS, industry and academia
- **200 – 250 people** working on the programme
- Working in collaboration with London Advanced Therapies

Coordinated by

cATAPULT
Cell and Gene Therapy



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Securing the advanced therapies talent pipeline

ATAC Advanced Therapies Apprenticeship Community

- Established by CGT Catapult and MMIP
- £1.5m award from ISCF
- First advanced therapies apprentice programme
- Aims to provide the skills required to fuel industry growth

137
apprentices
have now been
employed

36
companies have
hired ATAC
apprentices

10
programmes
have been
developed

ATSTN Advanced Therapies Skills Training Network

- £4.7m funding from BEIS to establish an online training platform and national training centres
- Career Converter tool to attract talent from outside the industry
- Training for industry skills in advanced therapy and vaccine manufacturing
- Will develop skills and experience in aseptic techniques for GMP to the rapidly growing industry



Potential impact

- Access to funding – more risk-averse and lower valuations
- Slowing of clinical trial system delays data generation and progress
- Disruption to market
- Manufacturing capability/capacity
- Potentially a shift from global to more localised plans



Potential opportunities

- Potential use of ATMPs in COVID-19 treatment
- More multifunctional facility usage
- Prioritisation of long-term/curative treatments, such as ATMPs, to reduce burden on healthcare systems
- Potentially a shift from global to more localised plans



The role of ATMPs moving forward

World-leading healthcare for patients

Transformative therapies to address unmet medical need



Growth in economy and productivity

Bringing investment and high-value employment



Clusters of activity driving regional growth

And potential for further clusters to accelerate industry progress



Tackling the increasing cost of healthcare

Long-term treatments can reduce the healthcare disease burden



Reducing healthcare inequality

Developing life-changing and personalised treatments



What do we need to do to realise this?

Manufacturers

- Keep up a strong flow of high impact therapeutics
- Keep the data and evidence flowing
- Innovate to increase scale and bring down cost of manufacture

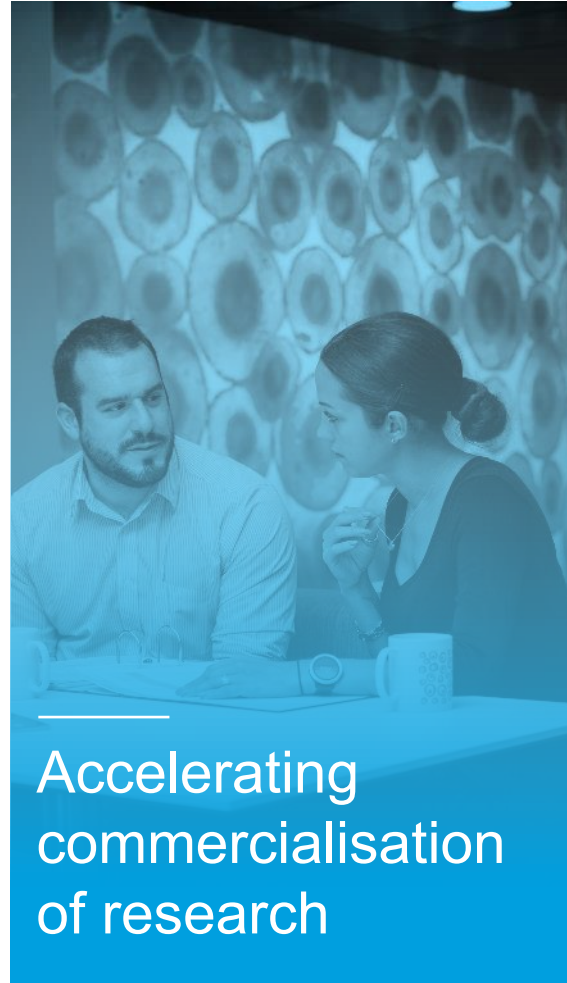
Healthcare system

- Adapt to adopt
- Create environment for higher volume use and ability to switch
- Break out of the short-term return paradigm

Government

- Continue to invest in research to feed the pipeline
- Continue to invest with the industry to make the UK a world leader in manufacturing
- Promote clusters

Thank you



CATAPULT

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Cell and Gene Therapy Catapult is committed to ensuring high standards of research integrity and research best practice in the activities we carry out. We subscribe to the principles described in the UK concordat to support research integrity.

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Q&A



Please add any question you have into the Q&A box

Please fill in feedback survey, your input is really valuable to us

Next webinar,

The unusual case of a rare disease, Spinal Muscular Atrophy,

4pm 9th March, Rafael Yanez (Professor of Advanced Therapy, Royal Holloway)