

CATAPULT

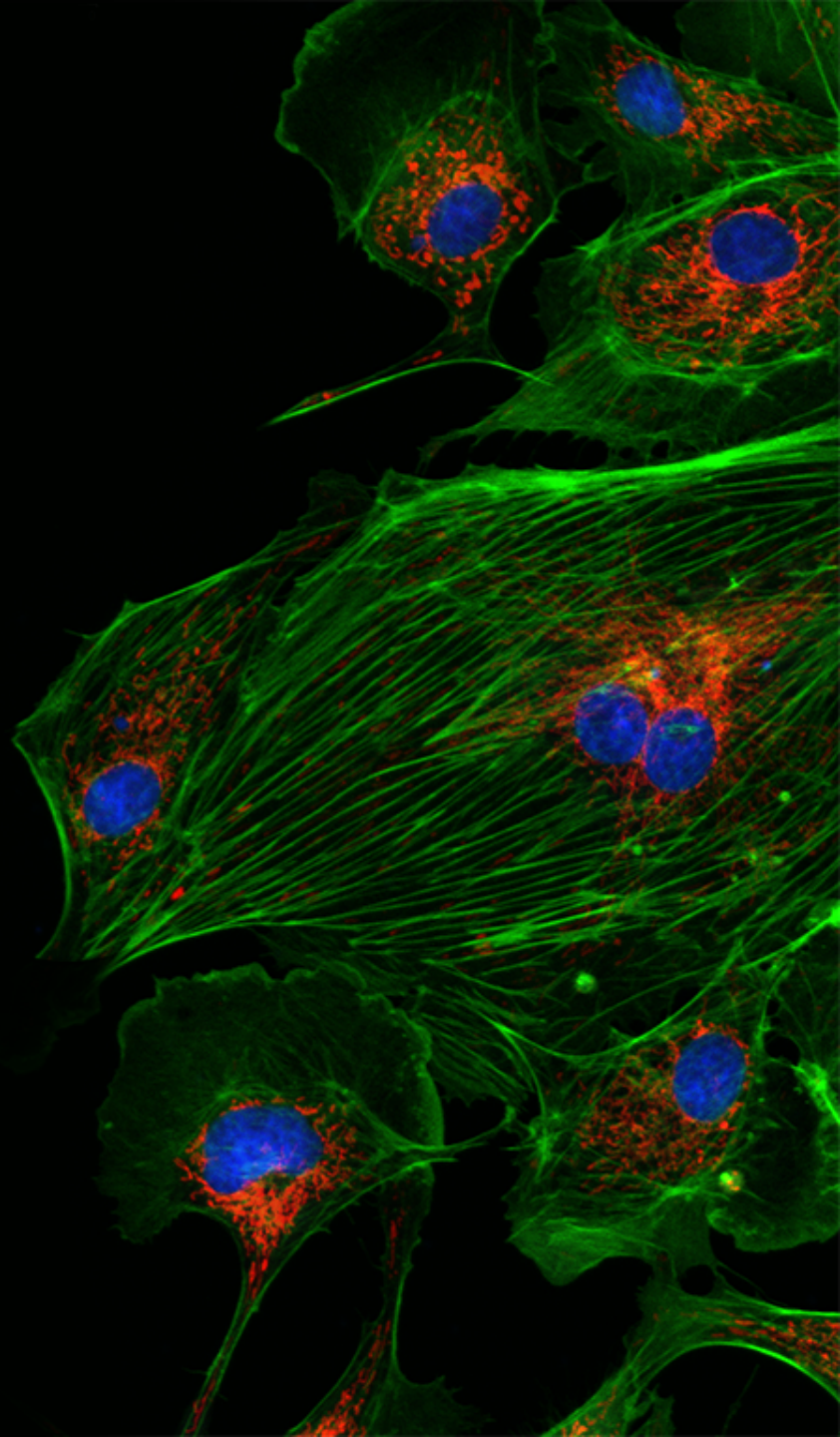
Cell Therapy

The Emerging Industry

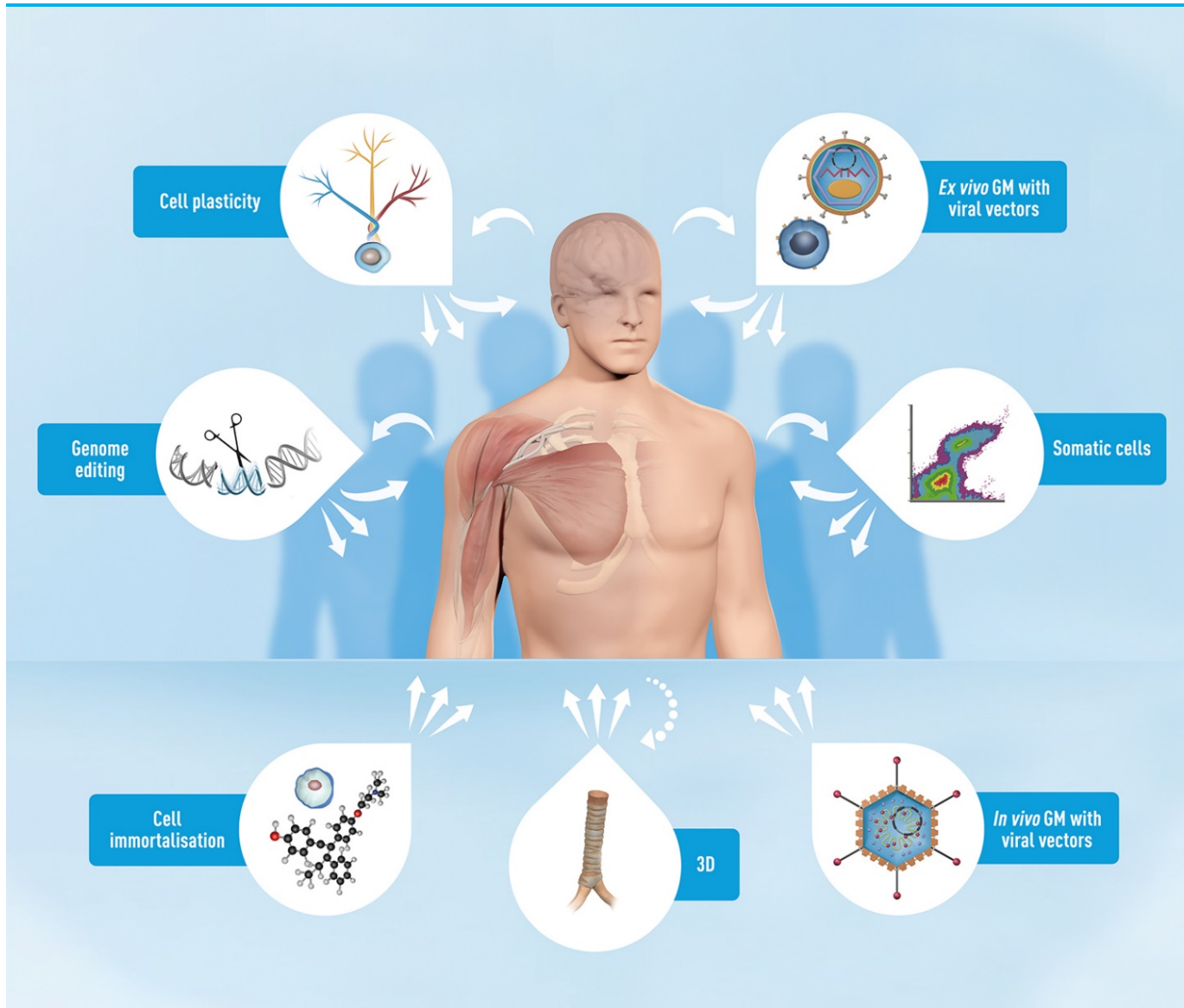
The Products

The Collective Challenge

Keith Thompson, CEO
Cell Therapy Catapult



Cell and Gene Therapy: A revolution in medicine



Global Performance: Q3 2015

1 Total financings:

\$2.8Bn Q3

\$9.3Bn YTDQ3

Up 163%
YTDQ3 2014

Gene & Gene Modified Cell Therapy:

\$1.0Bn Q3

\$5.7Bn YTDQ3

Up 137%
YTDQ3 2014

Cell Therapy:

\$1.9B Q3

\$6.3Bn YTDQ3

Up 198%
YTDQ3 2014

Tissue Engineered:

\$340M Q3

\$631M YTDQ3

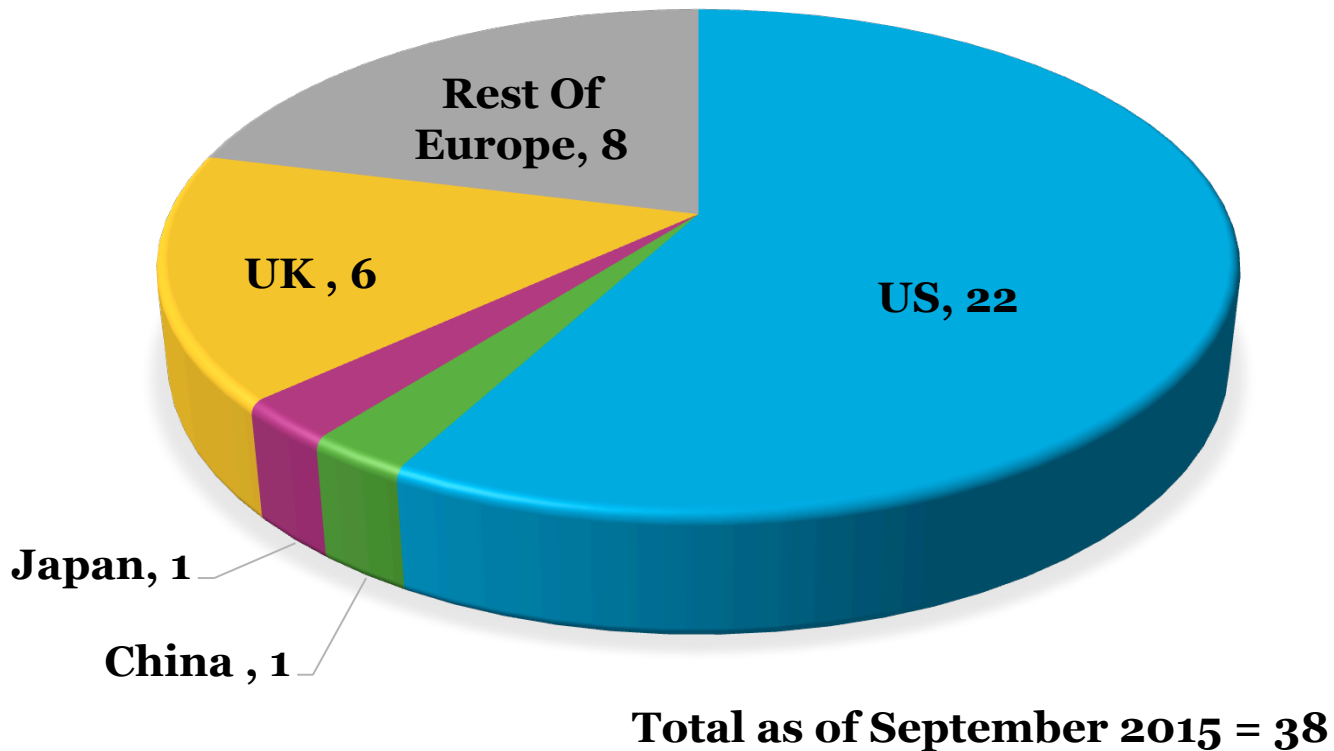
Up 258%
YTDQ3 2014



Source: ARM Q3 2015 Report

¹ Some companies are active in more than one technology group. As a result, the total amount raised does not equal the sum of the raises of the individual technology groups.

Genetically Modified Immune Cell Therapies – Active Companies Worldwide



Single Therapy
Class Companies
= 24

Multiple Therapy
Class Companies
(CAR and TCR) =
8

Single Therapy
Class Multiple
Cell Types (CAR)
= 6

Refs: Delvinsite – Chimeric Antigen Receptor T cell Immunotherapy – Competitive Landscape, Technology and Pipeline Analysis, 2015
Internet search
Internal Immunotherapy Monthly Reports – Monthly Analysis of Business activity

The Cell Therapy Catapult

Background:

Independent, not for profit company, founded in 2012 as part of the Innovate UK/BIS Catapult program to bridge the gap between universities and commercialisation.

£70m core government grant for first 5 years with a further **£50m** for the coming 5 years.

£55m for large scale manufacturing facility

Approach:

- **Core** projects to tackle industry-spanning issues
- **Collaborative R&D** projects with academia and industry
- **Fee-for-service** projects

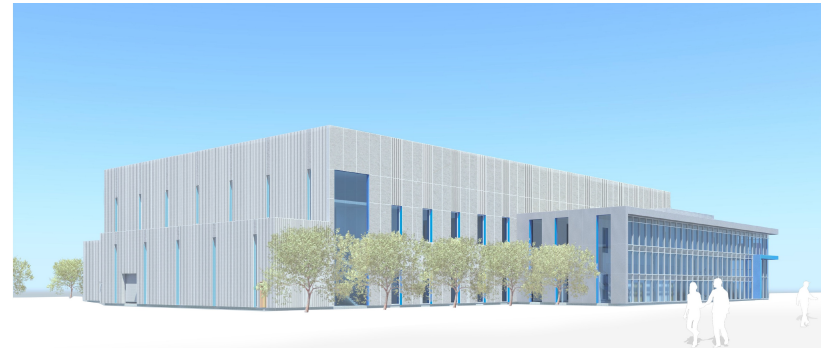
Structure:

- **>100** cell & gene therapy experts
- **1200 m²** development laboratory
- **7200 m²** large scale manufacturing centre **(2017)**



Large-scale manufacturing centre

- 7,200m² world-leading GMP manufacturing centre
- Located north of London
- Logistics – Global and European Gateway, inbound and outbound
- 12 modules to host companies
- Highly-skilled workforce
- Cluster development
 - GSK, J&J Innovation Centre, GE Healthcare, Tokyo Electron, Plasticell, Cambridge University, UCL, CRISPR Therapeutics



Built a team to address barriers

Business

- Health Economics
- Business Models
- Reimbursement
- Partnering

Manufacturing and Supply Chain

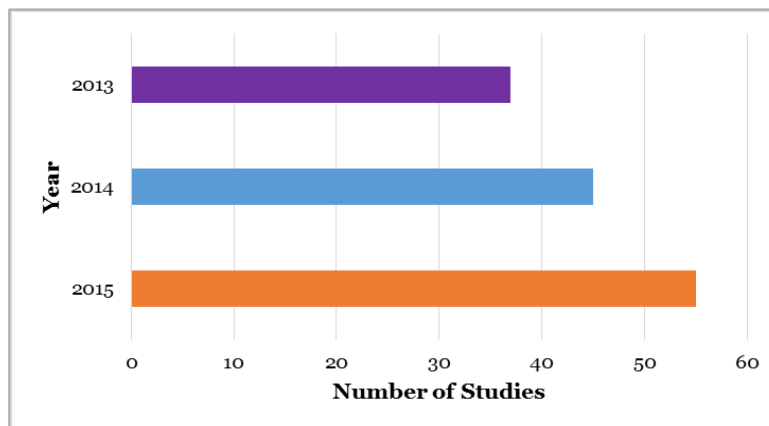
- Robustness & Reliability
- COGS & Scale up
- Characterisation & Analytical
- 2D/3D
- Autologous
- Shelf life and transport
- Delivery Device

Clinical And Regulatory

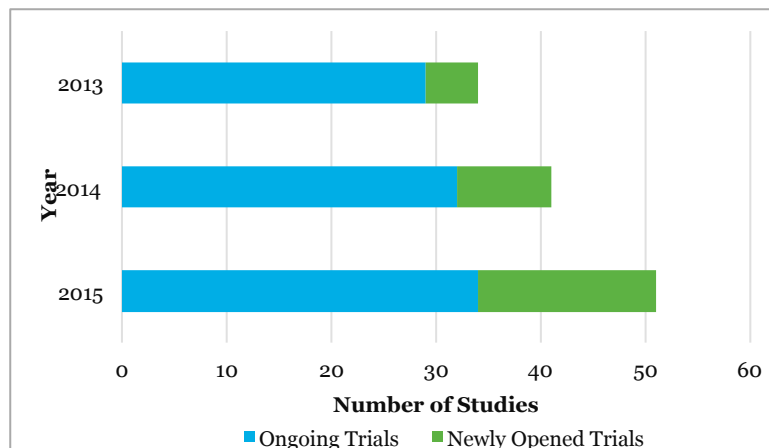
- Regulatory Pathway
- Pre Clinical Pathway
- Clinical trial design and delivery
- Clinical pathways
- Near patient automation/closed device

Science translating into therapies

- UK pipeline increasing year on year

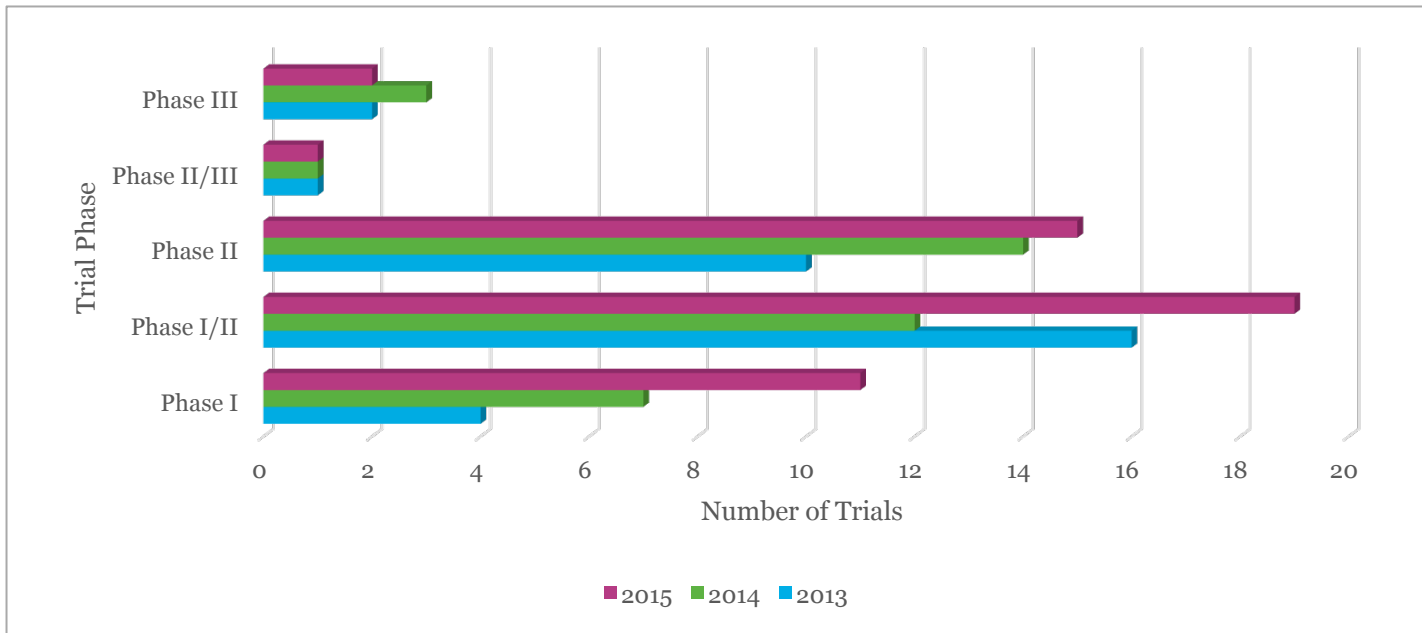


49% increase in preclinical projects 2013-15



47% increase in clinical trials 2013-15

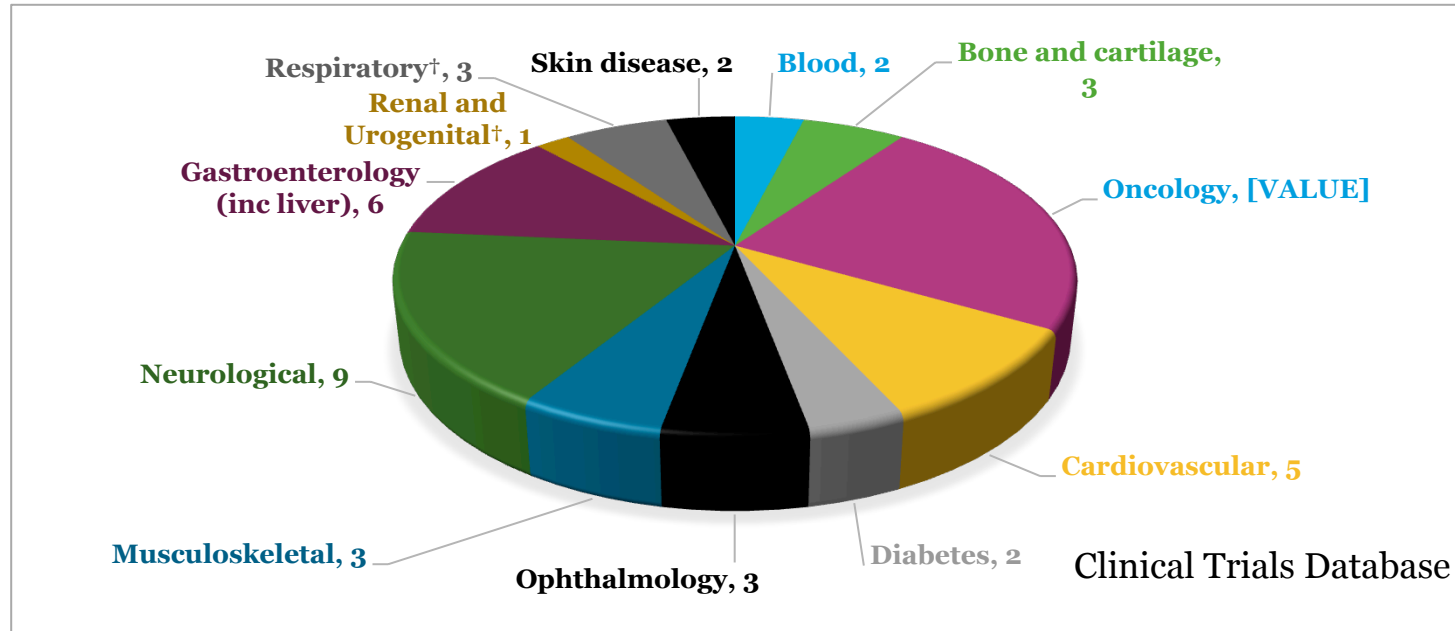
Majority of clinical trials in early phases



High proportion of autologous therapies

Still dominated by Academic trials

Disease area: Oncology remains the dominant



Clinical: WT1 - Catapult Therapy TCR Ltd

Aim: Development and commercialisation of gene modified T cells directed against WT1 expressed in haematological conditions, e.g. AML, MDS

Involvement: Catapult Therapy TCR Ltd has been set up to capture IP and know-how from UCLB and Imperial Innovations along with core investment from Cell Therapy Catapult to provide:

- Ph1/2a clinical trials
- Process development
 - Process simplification and automation
 - Gammretrovirus
- Commercialisation strategy

Finances: £10m CTC core funded subsidiary

Outcome: advanced speed to clinic and expanded the trial to Europe. Improved Process
Creation of investible proposition



Harvest
T Cells



Genetically
engineer to
modify specificity



T Cell Receptor
Therapy



Infuse back into
patient



Anti-Tumour
Activity



Clinical: ReNeuron – Process & Analytical Development

Aim:

- Develop and refine the manufacturing and analytical processes of the CTX cell line in preparation for clinical phase III trials and commercialisation.
- The project is currently in clinical phase II trials for treatment of ischemic stroke.

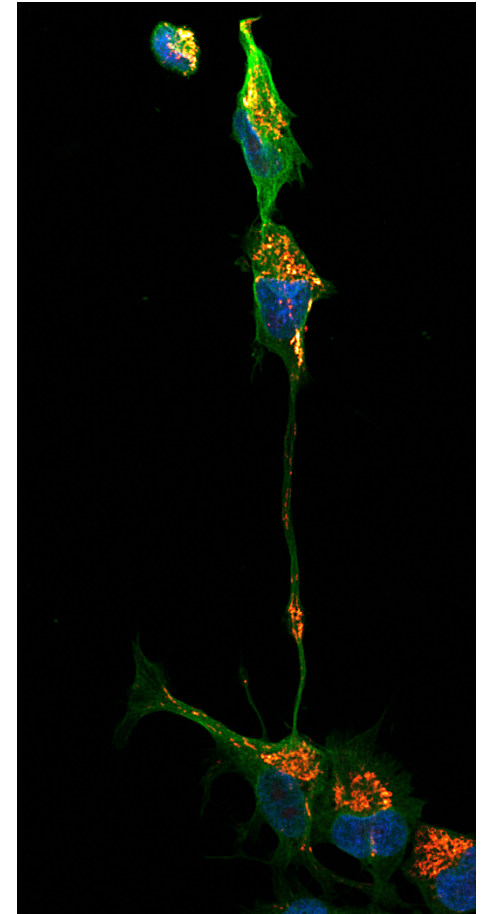
Involvement:

- Process Development, including scale up for phase III clinical manufacture
- Analytical Development (potency assays)

Finances: The collaboration has a combined budget of £3.3 million

Outcome: Improvement to manufacturing process and development of analytical assays.

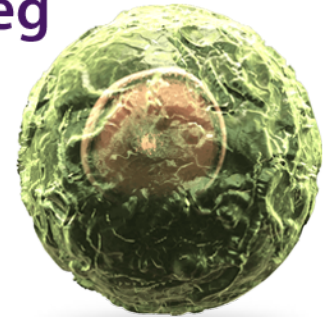
ReNeuron has subsequently raised £100M



Clinical: TxCell process development

- **Aim:** Development and optimisation of a new and improved manufacturing process for TxCell's OVA-specific Treg Therapy as a treatment of Crohns Disease
- **Involvement:** Catapult are providing a service to TxCell including:
 - Tech transfer and current process analysis (FMEA, COGs, BSD)
 - Acquisition and testing of GMP cell sorter
 - Process and Analytical Development
 - GMP proving
 - Tech transfer back to TxCell
- **Finances:** Commercially funded by TxCell
- **Outcome:** To enable TxCell manufacturing and supply development

Treg



Clinical: Athersys – ARDS trial

Aim:

Support a Phase I/II clinical study evaluating the administration of Athersys' MultiStem therapy to Acute Respiratory Distress Syndrome patients.

Involvement:

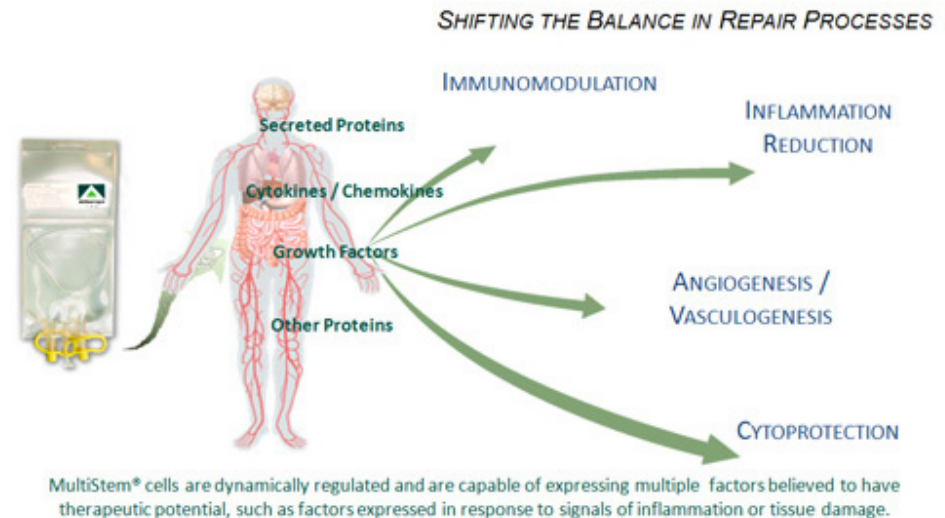
- Clinical site selection
- Trial execution

Finances:

Innovate UK Collaborative R&D grant of **£2.2m**

Outcome:

- Enables trial to be conducted in UK sites with Athersys.
- Opening of UK Subsidiary



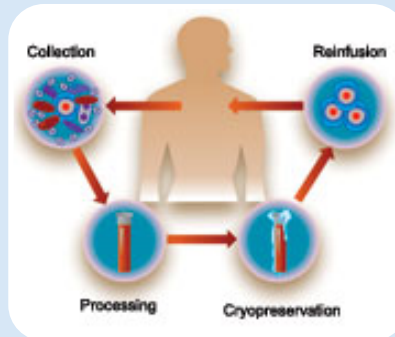
Summary- a paradigm shift



Clinical Needs

Strong clinical results

Clinical Development Velocity



Autologous

Clinical Pathways

Integrated healthcare



Supply chains

Future role of Pharmacy

Near patient automation

CATAPULT

Cell Therapy

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Innovate UK

Technology Strategy Board

Catapult is an Innovate UK programme.

