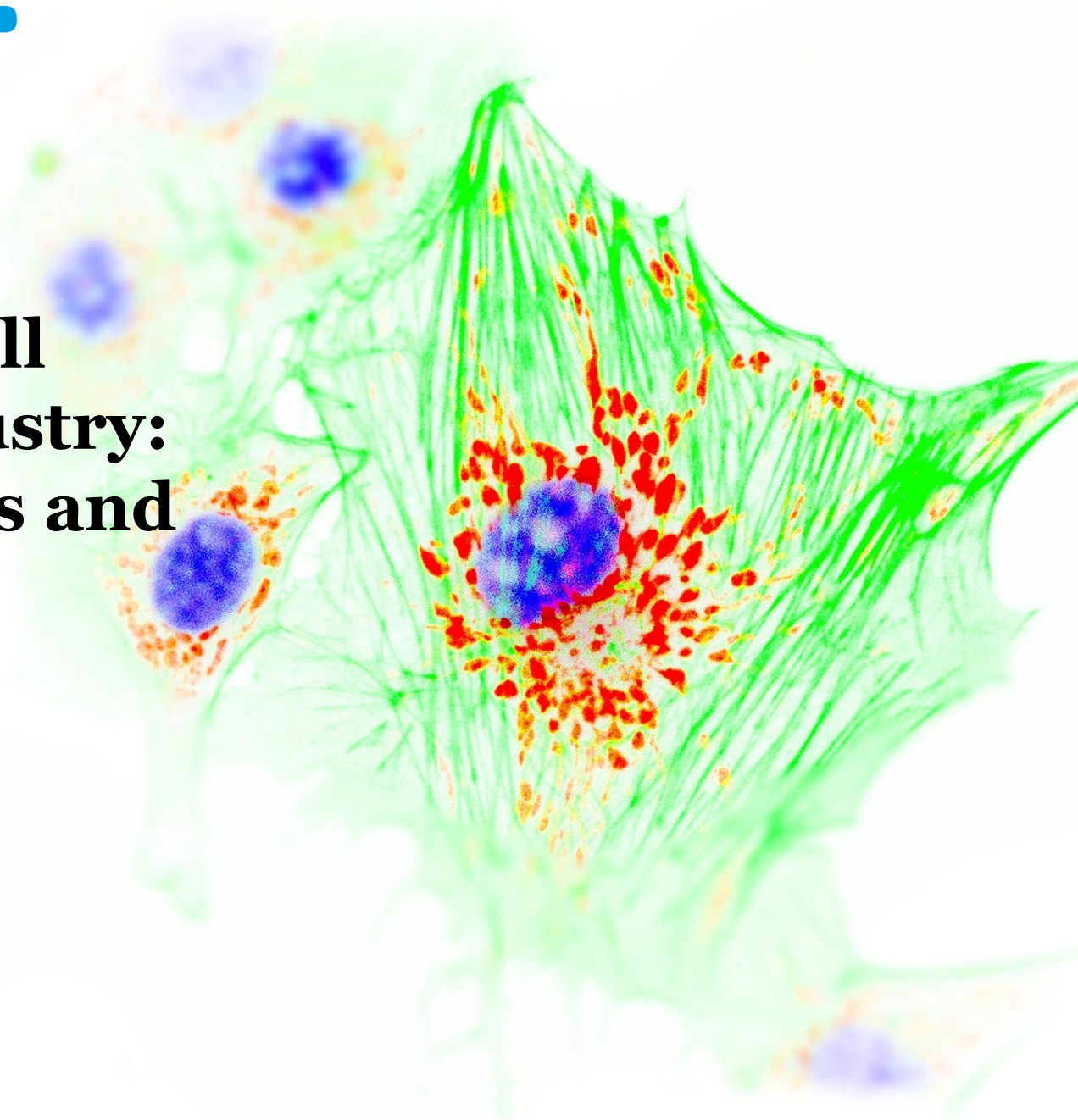


Growing a Cell Therapy Industry: Opportunities and Challenges

October 27th, 2014

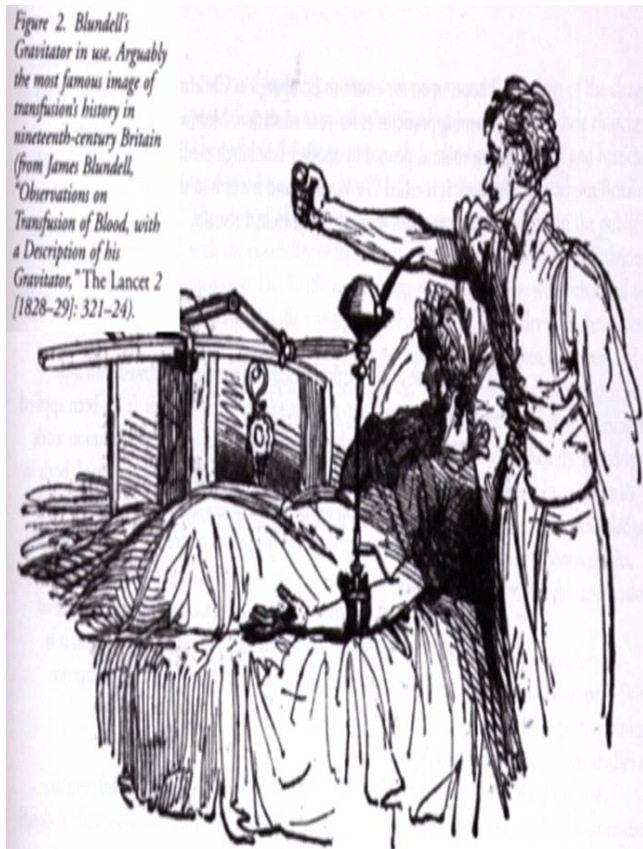
ct.catapult.org.uk



Cell therapy – New and not so new

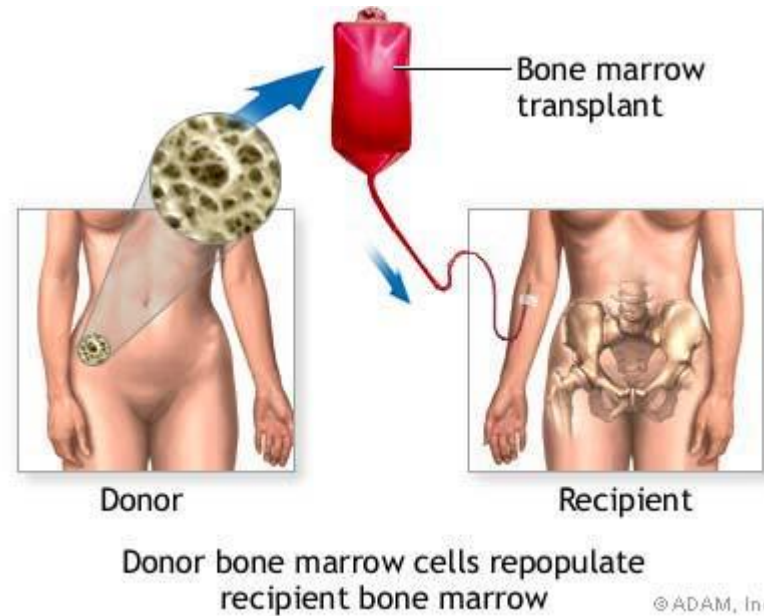
- Blood Transfusion

- The oldest cell therapy?

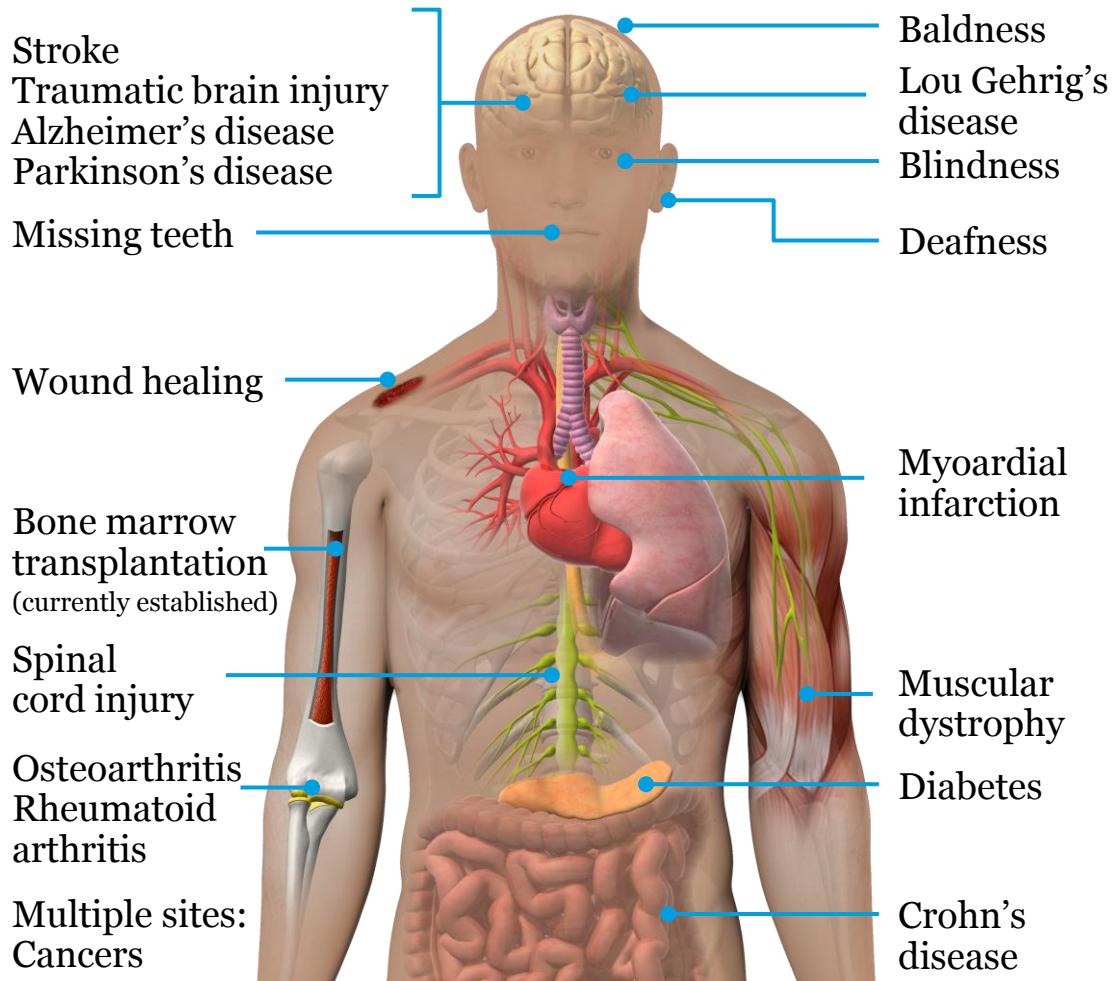


- Bone Marrow Transplant

- Till and McCulloch 1960s



Potential of Cell Therapies

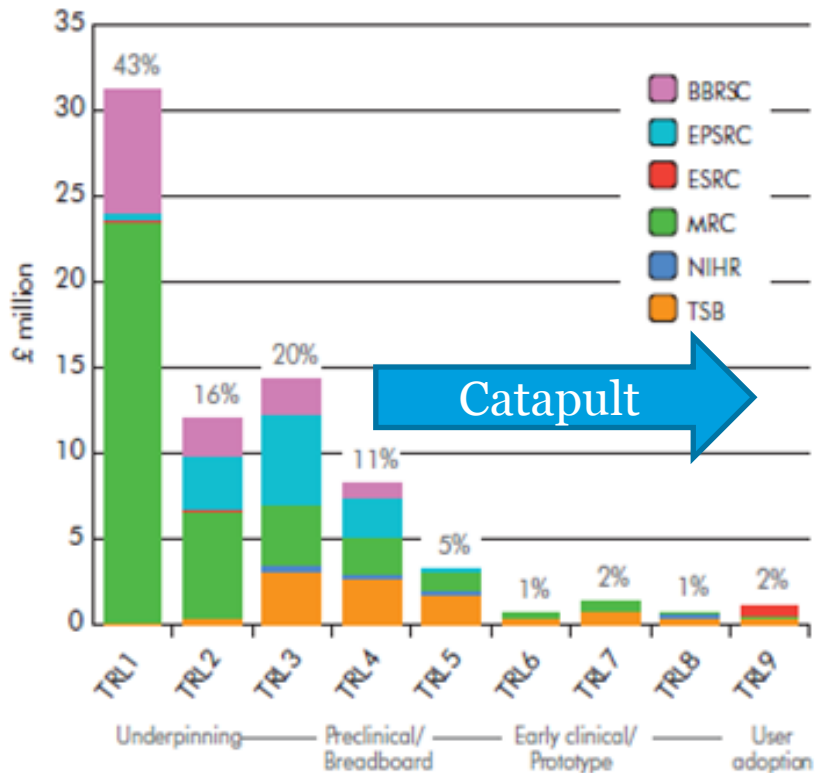


Potential cell sources include:

- Somatic cells
- Pluripotent stem cells
- Immune cells
- Gene modified cells

Cell Therapy-Translational gap

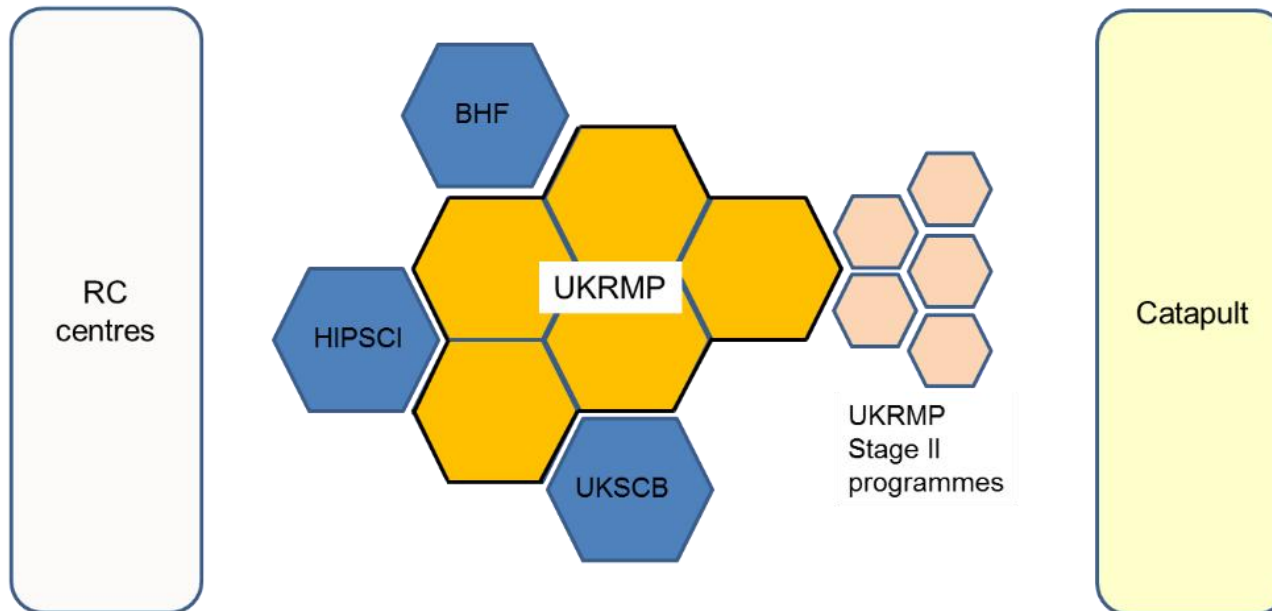
Spend by stage



- UK have a world leading research base in the area of Cell Therapies
- UK have many very strong clinician led innovative therapies but at early translation stage
- UK have a globally competitive environment in translation towards commercialisation and clinical reality.
- Limited commercial investment
- Working to help bring effective therapies to patients

The UK Cell Therapy environment: Regenerative medicine as an example

An integrated community



Working to help
bring effective
therapies to
patients.

Reproduced courtesy of the UK Regenerative Medicine Platform

The Cell Therapy Catapult

An independent, not for profit, company limited by guarantee, founded in 2012 as part of the Innovate UK/BIS Catapult program

Revenue is generated from:

- Public sector investment. **£70m** core government grant for first 5 years with a further **>£50m** for the coming 5 years
- Subcontract agreements with industry and academia
- Collaborative project grants

A personnel count of 90 (Sept, 2014) with in-depth expertise in business, manufacturing and clinical development

A 1200 m² state-of-the-art laboratory facility in central London enabling advanced process and analytical development, including translational GXP laboratories

In April, the UK Government announced it is investing **£55m** over 5 years in a late stage clinical trials and commercial manufacture facility under Catapult/Innovate UK governance.



Strategic Goals

- Goal
 - Build a £10bn industry
- Pipeline
 - Increase cell therapies in UK clinical trial and clinical use
- Value
 - Investible propositions created leading to cell therapy companies that succeed and stay in the UK
- Attractiveness
 - Demonstrating that the UK is the place to do this work, with increased inward investment

Addressing Barriers: Catapult Capabilities

Business

- Health Economics
- Business Models
- Reimbursement
- Partnering

Business Development

Manufacturing and Supply Chain

- Robustness & Reliability
- COGS & Scale up
- Characterisation & Analytical
- GMP
- CMC
- Delivery

Process Development

Clinical And Regulatory

- Complex Regulatory Landscape
- Pre Clinical Packages
- Clinical trial design
- NHS partnering

Clinical Operations & Regulatory Affairs

Project led approach

- Pathfind therapies into and through the Clinic

- Core projects with direct investment
- Rounded phase II data package
- Investible propositions



Imperial College
London

- Assist industry to progress to commercialisation

- Collaboration (also JV)
- Contract research
- UK as entry point into the EU



- Assist academia to progress to commercialisation

- Collaboration
- Contract research



KING'S
College
LONDON

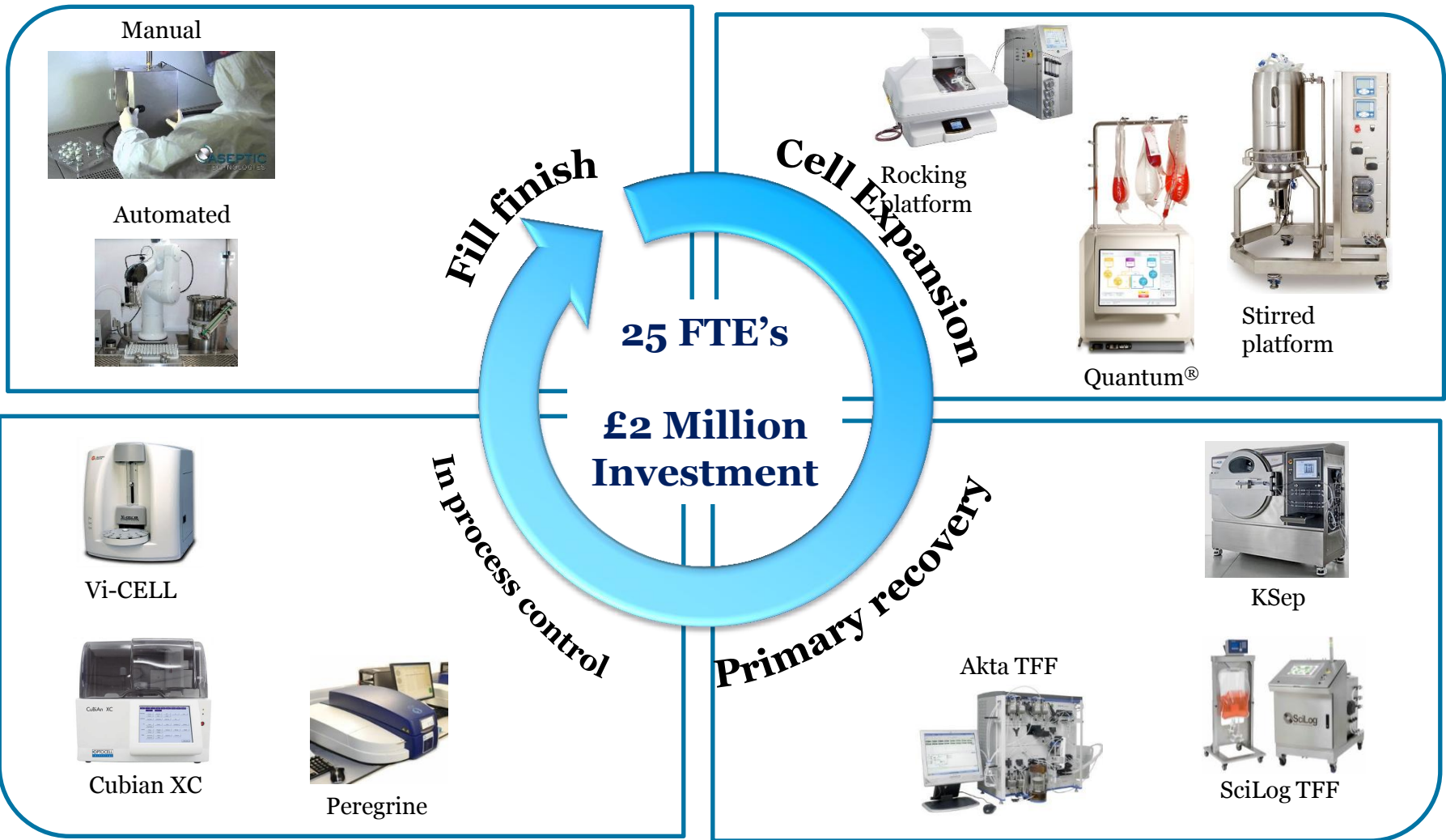
- Technology platforms

- Develop novel technologies and license to industry



CATAPULT
Cell Therapy

Process Development Capability all the way to GMP proving



Analytical Capability



UK preclinical and clinical stage cell therapies

Category	# 2014 (2013)	Comparison
Preclinical (<2 yrs from clinic)	45 (37)	A larger variety of cell types and a larger range of indications in preclinical stages
Clinical (UK trial ongoing)	41 (34)	Seven of the projects identified in the 2013 pre-clinical database have moved into the UK Clinical Trials Database along the timeline anticipated
Total	86 (71)	Trials in the UK Clinical Trials Database are mainly early stage

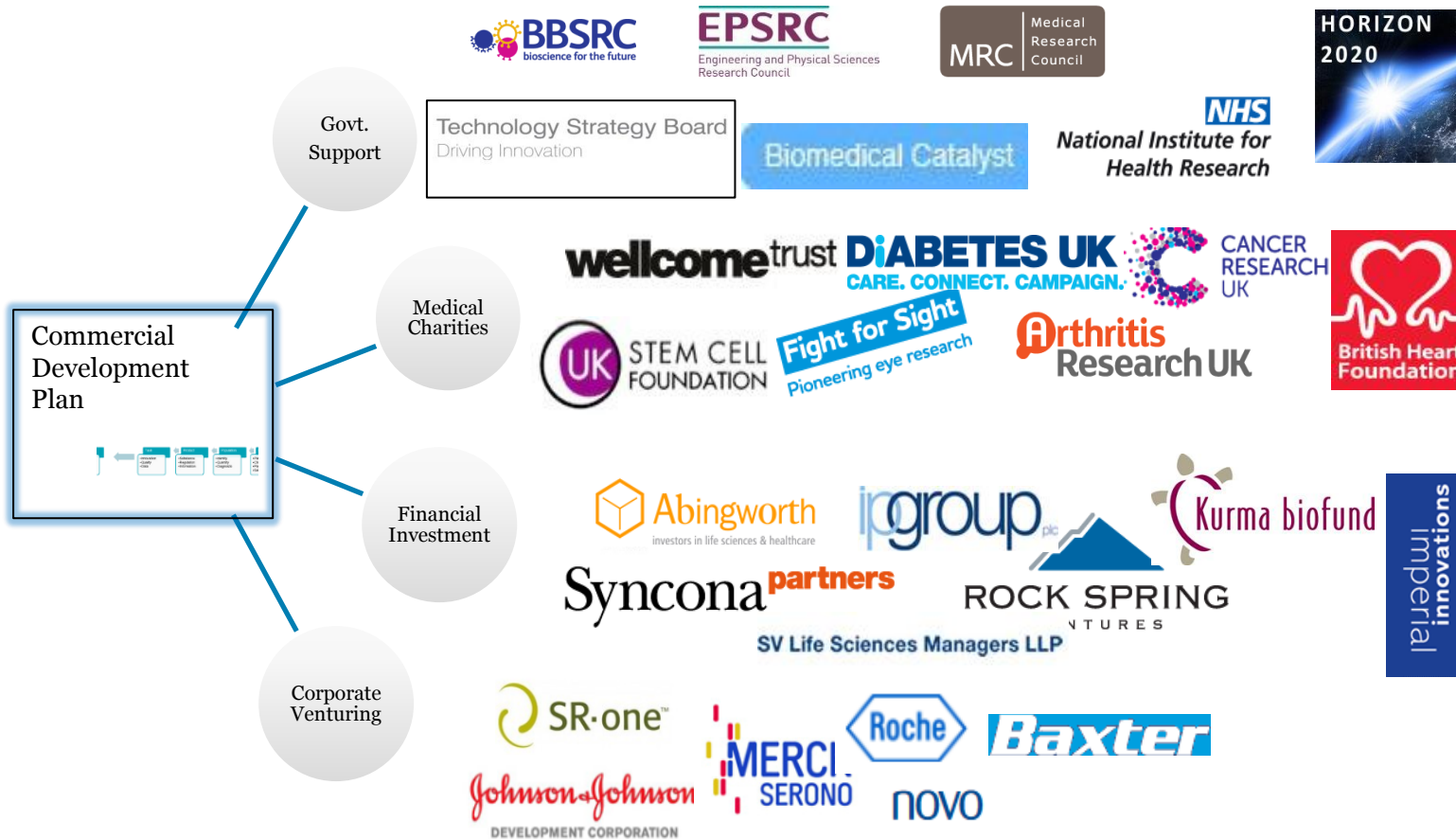
Large-scale Manufacturing Centre

£55m manufacturing hotel to support industry growth

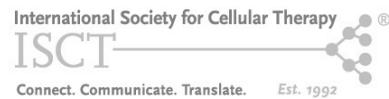
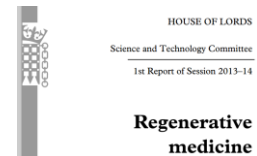
- Allowing CMOs **and** product development companies enable the manufacture **at large scale**
- Access large scale infrastructure without the capital outlay
- 5000m² **Flexible/modular** space
- Operated as a subsidiary of Cell Therapy Catapult sharing corporate services
- Capital, Revenue (Start up and initial operating costs and supporting TSB programmes)



Funding for Cell Therapy in the UK



Future in collaboration



- Contract or co-develop cell therapy products
- Next Generation process and technology development
- Solve generic industry barriers
- Driven by success of the industry

