Growing a Cell Therapy Industry: Opportunities and Challenges

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ct.catapult.org.uk

Catapult is a Innovate UK programme.
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

- Stroke
- Traumatic brain injury
- Alzheimer’s disease
- Parkinson’s disease
- Missing teeth
- Wound healing
- Bone marrow transplantation (currently established)
- Spinal cord injury
- Osteoarthritis
- Rheumatoid arthritis
- Multiple sites: Cancers
- Baldness
- Lou Gehrig’s disease
- Blindness
- Deafness
- Myocardial infarction
- Muscular dystrophy
- Diabetes
- Crohn’s disease
- Diabetics
Cell Therapy - Translational gap

- 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

- Manufacturing and Supply Chain
  - Robustness & Reliability
  - COGS & Scale up
  - Characterisation & Analytical
  - GMP
  - CMC
  - Delivery

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

Business Development
Process Development
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

- 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

- Technology platforms
  - Develop novel technologies and license to industry
Process Development Capability all the way to GMP proving

- 25 FTE’s
- £2 Million Investment

Manual
Automated

Vi-CELL
Cubian XC
Peregrine

Rocking platform
Stirred platform

Quantum®
KSep
SciLog TFF
Akta TFF
Analytical Capability

10 FTE’s

£1.5 Million Investment
UK preclinical and clinical stage cell therapies

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

- Govt. Support
- Medical Charities
- Financial Investment
- Corporate Venturing

Technology Strategy Board
Driving Innovation

Biomedical Catalyst

HORIZON 2020

National Institute for Health Research

UK Stem Cell Foundation

Wellcome Trust

Diabetes UK

Cancer Research UK

Arthritis Research UK

Fight for Sight
Pioneering eye research

STEM Cell Foundation

Abingworth

iGroup

Kurma Biofund

Syncona partners

Rock Spring Ventures

SV Life Sciences Managers LLP

Merck Serono

Roche

Johnson & Johnson

BAM Capital

Baxter

Novo
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