Catapult is an Innovate UK programme

The Cell Therapy Catapult
First Review to March 2014
Our vision

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

Our purpose

Growing a UK cell therapy industry delivering health and wealth.

Our mission

Our mission is to grow the industry in the UK to substantial and sustainable levels by:

- taking products into clinical trial, de-risking them for further investment;
- being a source of clinical expertise and access to NHS clinical partners;
- providing technical expertise and infrastructure to ensure products can be made to Good Manufacturing Practice and delivered cost-effectively;
- providing regulatory expertise to ensure that products can get to the clinic safely, in the shortest amount of time;
- creating opportunities for collaboration, both nationally and globally; and
- being a source of business expertise and knowledge of routes to grants and investment finance, so that commercially viable products are progressed and investable propositions are generated.
Highlights

- Designed, built and opened world beating collaborative office and laboratory space including the innovative manufacturing pod concept.
- Expanded our team of experts to over 70, on track for our plan of 100 after three years.
- Awarded £55m to create a large-scale manufacturing centre for the UK.
- Became a clinical trial sponsor enabling us to take clinical trials across Europe.
- Brought together a portfolio of 10 projects meeting a range of critical industry needs.
- Engaged with 189 contacts within the academic community and over 297 small- and medium-sized enterprises.
- Published clinical trial databases in 2013 and 2014 showing an encouraging increase in the number of trials.
- Demonstrated industry leadership at 10 conferences throughout the world.
- Supported the Royal Institution on its Christmas lectures, reaching the research community and general public.
- Established a leading position in the UK cell therapy community, including participation in the Regenerative Medicine Expert Group.
- Started major projects including:
  - a strategic translation award from the Wellcome Trust to make stem cell-derived red cells;
  - collaborated with Videregen and others to bring tissue engineered trachea into clinical trial;
  - worked with ReNeuron on a manufacturing challenge;
  - established a joint venture with UCL and Imperial on a cancer immunotherapy; and
  - initiated a major collaboration with Roslin cells to establish Good Manufacturing Practice iPS cell bank.

“Not just world class, but world beating.”

Kieran Murphy
President and CEO,
GE Healthcare Life Sciences
Chairman’s statement

To be tasked with helping build a multi-billion pound cell therapy industry in the UK, generating health and wealth for all, is a big responsibility – and one which the Cell Therapy Catapult is proud to accept.

With strong support from the UK Government, a world-leading academic base and an attractive tax treatment for the commercialisation of intellectual property, the UK is the most fertile environment to build and grow cell therapy companies. Commitment from Innovate UK (formerly the Technology Strategy Board), Department for Business, Innovation and Skills, the Department of Health, National Health Service, universities, and research councils such as the Medical Research Council, plus biotech and pharmaceutical companies has created a strong cell therapy network in the UK for us to collaborate with. This has been of enormous benefit in the Cell Therapy Catapult’s first full year of operation, and will continue to be so.

We are extremely fortunate to have attracted high-calibre Non-Executive Directors to the Board. They are working well together to guide and constructively challenge the Cell Therapy Catapult, ensuring it is making maximum impact across all areas of operation. I thank them for their valuable contributions, and they join me in congratulating the executive team on their exceptional achievements in this start-up period.

The Cell Therapy Catapult is proud to be part of Innovate UK’s Catapult network of elite centres of technical excellence. The seven-strong family is working collaboratively in many areas.

Looking ahead, the Cell Therapy Catapult believes that the environment for cell therapy is improving rapidly, and the UK is establishing itself as the go-to place for this important work. The industry is growing with more funds being invested into the sector. Alongside the effect we’ve had on the industry, there is positive clinical data emerging that reinforces our strategy to accelerate growth in the sector. We will be focusing on translating our pioneering work for use in the UK, as well as helping the wider cell therapy community develop the products that patients need. Thank you for your continued support in this task.

Dr John Brown CBE FRSE, Chairman
Chief Executive’s statement

Making the UK a centre of excellence in cell therapy – our task here at the Cell Therapy Catapult.

Looking back over the year’s exciting developments, we’ve made great progress towards that goal by working on what we believe to be the main barriers to development of the sector. This focus on business; clinical and regulatory; manufacturing and supply chain issues has enabled us to build the innovation-centric organisation and environment required for a UK centre of excellence.

The House of Lords enquiry into regenerative medicine, which reported in July 2013, also recognised the importance of making the UK a cell therapy leader. This led to the formation of the Regenerative Medicine Expert Group and we are pleased to be deeply involved in all of its workstreams, notably on the regulatory and licensing groups.

What’s been the highlight of 2013/14? We met all of our corporate objectives and it’s hard to single one out, but we can point to three achievements that illustrate our approach. Firstly, we’ve recruited 70-plus cell therapy specialists across our teams, to help you move your cell therapy closer to the market... and the patient. Secondly, we’ve developed a UK and international portfolio of collaborations showing how we are working in a pathfinding, project-led manner, to tackle industry challenges. We also initiated a major platform project to establish a Good Manufacturing Practice grade iPS haplobank.

Lastly, delivery of our fantastic state-of-the-art facilities, 1,200m² of laboratory and office space built for innovation and collaboration, was an enormous achievement.

In 2014/15, we’ll be pushing ahead with expansion and progression of the UK cell therapy portfolio. We’ll be building on our international reach beyond North America.

Finally, the recent news that long-term UK manufacturing needs are to be met via a large-scale Cell Therapy Manufacturing Centre was a culmination of our work and that of others in the industry. It will operate as our subsidiary, and we’re already working to ensure its doors open as a manufacturing hotel in 2017.

The Cell Therapy Catapult is where it is today as a result of the hard work of its staff and Board, and the support of the UK and international cell therapy communities. Many thanks are due to you all.
## Project portfolio by therapeutic area

<table>
<thead>
<tr>
<th>Project details</th>
<th>Description</th>
<th>Our contribution</th>
<th>Rationale for our involvement</th>
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<tbody>
<tr>
<td><strong>OPHTHALMIC</strong></td>
<td>Transplant of own stem cells onto eye</td>
<td>Regulatory path</td>
<td>Pathfinding for orphan indication with approximately 140 limbal transplants undertaken per year in the UK.</td>
</tr>
<tr>
<td><strong>RESPIRATORY</strong></td>
<td>Scaffold plus own cells as organ replacement</td>
<td>Clinical trial sponsor, regulatory pathway</td>
<td>Pathfinding complex 3D manufacture and business models, and the US market is estimated to be US$600m per year.</td>
</tr>
<tr>
<td><strong>GASTROINTESTINAL</strong></td>
<td>Scaffold plus own cells as organ replacement</td>
<td>Project management, non-clinical, clinical and regulatory advice</td>
<td>Developing new non-clinical procedures for complex cell therapy which has the potential to heal more than 3,000 patients.</td>
</tr>
<tr>
<td><strong>DERMATOLOGICAL</strong></td>
<td>Human dermal fibroblasts</td>
<td>Novel delivery system</td>
<td>Creating new delivery systems and developing device expertise. Epidermolysis bullosa is a rare condition affecting approximately 5,000 people in the UK.</td>
</tr>
<tr>
<td><strong>CENTRAL NERVOUS SYSTEM</strong></td>
<td>Therapy based on neural cell line</td>
<td>Manufacturing development and scale-up</td>
<td>New manufacturing and cryopreservation technology that could benefit approximately 200,000 patients in the UK per year.</td>
</tr>
<tr>
<td><strong>TRANSFUSION</strong></td>
<td>Conversion of stem cells into red blood cells</td>
<td>Manufacturing scale-up, advice on non-clinical and clinical studies, market access</td>
<td>Tackling large-scale cost of goods. Beta thalassemia affects approximately 16,000 people in Europe.</td>
</tr>
<tr>
<td><strong>MULTIPLE DISEASE AREAS</strong></td>
<td>Adult progenitor cells</td>
<td>Market access analysis</td>
<td>Bringing investment to the UK and building an understanding of EU reimbursement. For example, more than 150,000 UK patients who have suffered a stroke stand to benefit from advances in these technologies.</td>
</tr>
<tr>
<td><strong>ONCOLOGY</strong></td>
<td>T-cell therapy targeting cancer cells</td>
<td>Investment, manufacturing development, regulatory and clinical trial sponsorship</td>
<td>Driving success in immune therapy. There are more than 25,000 patients diagnosed with haematological malignancies in England every year.</td>
</tr>
<tr>
<td><strong>IMMUNOLOGICAL</strong></td>
<td>Expansion of levels of tolerant immune cells in organ recipients</td>
<td>Clarification of clinical, regulatory and business aspects of route to market</td>
<td>Pathfinding in a poorly served market area. There are about 4,200 organ transplantsations performed in the UK every year.</td>
</tr>
<tr>
<td><strong>STEM CELL BANK</strong></td>
<td>iPS cell lines isolated and banked under Good Manufacturing Practice, for use in early stage and clinical research</td>
<td>Project definition, oversight and cell line distribution</td>
<td>Meeting strong industry need for clinical grade cells to accelerate therapy development. The European market is growing rapidly and was worth nearly £200m in 2012.</td>
</tr>
</tbody>
</table>
**Objectives**
To build the business and drive the delivery of investable propositions, we set out with three broad aims: to establish a network covering the industry; to create a portfolio of activities that tackled the industry issues; and to build our reputation and awareness of our capabilities.

**Our achievements**
In delivering on those aims, we put together an experienced and specialist business development team covering the areas of: intellectual property, law, healthcare economics and reimbursement, business models, finance and marketing.

We have engaged with cell therapy companies, large healthcare companies, companies covering all aspects of the supply chain and investors. Through an orchestrated series of speaker events and conferences we are now recognised in the largest cell therapy market, North America.

This is exemplified by US companies such as Athersys contracting us to assist with the expansion of their UK and European business. Perhaps most importantly, we have developed a portfolio of projects (page 6) ranging from tackling of immediate needs, such as those of ReNeuron (page 19); to long-term issues for the industry, as in the BloodPharma project (page 17).

**SME engagement**
We deliberately designed an operating model that would be as accessible to Small- and Medium-Sized Enterprises (SMEs) as it is to larger industry participants. Our cornerstone activity of a short initial study to develop the route to commercialisation (Suitability Study) has provided in-depth understanding of pioneering SMEs, like Azellon. At the same time it has left the SMEs with a tangible benefit and identified future collaboration opportunities. Our preclinical and non-clinical databases identify the pipeline of forthcoming SME opportunities and enable us to ensure that all cell therapy developers are aware of the capability that we bring. Our ability to help SMEs win Collaborative Research and Development (CR&D) funding has been demonstrated in the case of Videregen (page 18). Activities such as the creation of a Road Map to Reimbursement are bringing down the barriers for SMEs by increasing access to the rewards of cell therapy development in the UK.

**Looking ahead**
In the coming year, we will continue to build our portfolio of projects around the diverse needs of the industry. We have had early successes in developing the CR&D and contract income streams, in addition, to core funding. We will continue building these streams towards each being a third of our income. In addition we will increase our global reach with planned development activities in Japan, Korea and China. At the same time we will continue to enable the industry through the building of a series of platform projects which tackle generic industry issues, by working to improve the reimbursement environment and facilitating growth through access to investment.
Navigating the clinical environment

Objectives
Cell therapies have the potential to treat diseases that cannot be adequately addressed by traditional medicines. In order to realise this benefit for patients and achieve commercial success, researchers, whether from academia or industry, need to conduct high quality clinical development programmes. This can seem complex and daunting but at the Cell Therapy Catapult, we have built a group of experts whose aim is to help our stakeholders navigate the route into robust clinical trials.

Achievements
We have collaborated with academics, clinicians and companies to provide acceleration paths for promising therapies. A particular example is our recent collaboration with University College London and Imperial College, on a project previously funded by Leukaemia and Lymphoma Research, which will see the team providing expertise to accelerate the clinical development of a new T-cell-based treatment for haematological malignancies including acute myeloid leukaemia. Clinical trials under our sponsorship are getting under way in late 2014 and early 2015 (such as the trachea project on page 18). In addition, companies developing cell therapies outside the UK recognise that they also need to deliver trials within the EU, both to recruit patients and to secure their future regulatory path towards licensing. We enable these inward investors to use the UK as an entry point and base to grow EU operations.

Stakeholder engagement and support
Another important part of our mission is to positively influence the UK environment and infrastructure for development of cell therapies. Over the past year we have developed a strong network in the UK, the EU and in the US and had particular early success in working with stakeholders to address regulatory overlaps, and responses to US Food and Drug Administration and European Medicines Agency consultations, as well as running workshops for the cell therapy community in areas including preclinical safety, quality requirements and international regulation. Our publications on topics of clinical, non-clinical and regulatory interest have been well received, and we will continue to release these on a regular basis.

Looking ahead
In the next year, we will be running a busy portfolio of clinical development projects. We expect to be progressing clinical trials in immune-based cell therapies, stem cell therapies and also complex tissue engineered products as well as regulatory interactions at UK and EU level. In addition, our work to further position the UK as a leader for clinical development of cell-based therapies will continue through the work we are doing with the Regenerative Medicine Expert Group and stakeholders such as Medicines and Healthcare products Regulatory Agency, the Human Tissue Authority, Health Research Authority and the National Institute for Health Research.

Dr Natalie Mount, Chief Clinical Officer
Connecting with the UK science network

Objectives
We set out to engage with both the academic researchers and the technology transfer offices in order to understand and address the challenges to commercialisation of intellectual property in the cell therapy sector. In doing so we aimed to build a network and partnership with universities.

Achievements
Over the past two years we have actively reached out to investigators and have held meetings with the major universities active in the development of cell therapies. In addition, we have been active contributors to the organisation and delivery of UK and global conferences. These efforts have resulted in 17 projects with universities, collaboration within a project submission to the first Horizon 2020 call, and a pipeline of 29 collaborative projects with universities.

University and stakeholder engagement
We have created a network of relationships with stakeholders across universities, research councils, charities and trade associations. An example of this is our close relationship with the Regenerative Medicine Hubs funded by the research councils. We have also established a regular meeting agenda with three research councils that provide significant support in the field, the Medical Research Council, Biotechnology and Biological Sciences Research Council and the Engineering and Physical Sciences Research Council.

Our work on building links with universities across the UK is progressing well. Individual universities are visited and screened for early and late stage projects. Since there is no centralised mapping of projects in the UK, this is a key activity.

Furthermore, we are expanding access and understanding of the sector through our preclinical and clinical databases, which aim to capture and publicise all advanced UK activity in the sector and are updated annually. Charities are an essential part of the network, both in terms of their funding but also their often deep understanding of a healthcare issue. We have formed relationships with key players such as Cancer Research UK, the British Heart Foundation and UK Stem Cell Foundation as well as the Wellcome Trust, Arthritis UK, British Heart Foundation and Fight for Sight.

Looking ahead
In the next year, we will look beyond the UK and continue to build an extensive international scientific network to increase inward investment, and to gain early stage awareness of new developments within the industry.

Professor Johan Hyllner, Chief Scientific Officer

University engagement

Connect with the UK science network

189 43 7
Leads Suitability studies University projects

We will continue to interact with active UK universities and other organisations as well as take part in international conferences. In addition, a high-level scientific advisory panel is in development to further enhance our access to knowledge and experience.
Developing new technologies and processes

Dr Stephen Ward, Chief Operating Officer

Objectives
Our goal was to build a cell therapy development team with commercial manufacturing experience to work within our world-leading facility. To do this we would also need to develop an infrastructure of people and relationships to support the activity in the lab. A key part of this would be a team of highly talented managers to steer projects through to commercialisation. In addition, we set out to develop a close relationship with the manufacturing community in the UK.

Achievements
In March 2014, we relocated the organisation to our new facilities on the 12th floor of Guy’s Hospital in London. We turned around the fit-out of the office and lab space in nine months from receiving access to the floor.

The facility has been designed to operate flexible manufacturing pods for core processes, developed at the Catapult. We’re already engaged in three major development projects and collaborations, as well as run a successful cell therapy manufacturing workshop with University College London and the advanced therapy medicinal products manufacturing community.

Some of the key projects we will be focusing on in our new facility include process and analytical development of neural stem cells for stroke, process and analytical development of a T-cell immune therapy and scale-up and Good Manufacturing Practice (GMP) validation of stem cell-derived red blood cells.

Core manufacturing process, assay and know-how will be gained through this work which will further enhance technical capabilities. Having identified that, as it grows, the UK cell therapy industry will require appropriate commercial-scale manufacturing capacity. We were delighted when we were awarded funding to create a new large-scale manufacturing centre, here in the UK. This Cell Therapy Manufacturing Centre, scheduled to open in 2017, will fill a gap that we identified in large-scale manufacturing in the UK (page 11).

Stakeholder engagement
The team also continues to actively engage with external stakeholders through a variety of channels such as gathering survey information to produce the cell therapy GMP manufacturing in the UK: capability and capacity analysis May 2014; and holding workshops and training sessions to further support the industry by covering topics that address key industry challenges such as how to deliver commercially viable cell therapy products. We also take part in key exhibitions and conferences such as International Society for Cellular Therapy (ISCT) and the World Stem Cells Regenerative Medicine Congress and participate in numerous committees such as the Ministerial Industry Strategy Group, The BioIndustry Association Cell Therapy and RegenMed Advisory Committee and the ISCT process development and commercialisation committees.

Looking ahead
Over the course of the next financial year, the overall theme is one of transition from a start-up to becoming fully operational using the pod concept and GMP proving labs. The team will be focusing on delivery of world-class innovation through the projects and collaboration. We will continue to develop our expert team across analytical development, process development, manufacturing development, facility and programme management.
In April 2013, we conducted a survey of the Good Manufacturing Practice (GMP) manufacturing capability and capacity in the UK.

The findings were clear – showing that although there were many high-quality Phase I and Phase II facilities, there was a lack of resource that would support larger scale Phase III clinical trials and onward into commercialisation.

This, coupled with the recent inquiry into regenerative medicine by the House of Lords Science and Technology Committee (which determined late stage manufacturing capacity in the UK should be supported and invested in) and similar findings by the Ministerial Industry Strategy Group (MISG), led us to develop a proposal that supported a vision of creating a centre for Phase III clinical trial and commercial supply. This was progressed through Innovate UK and Department of Business, Innovation and Skills (BIS).

One year after the start of our initial survey, the chancellor, George Osborne, announced in the March 2014 budget an award of £55m to create a large-scale manufacturing centre that will feature a 5,000m² GMP facility that provides a seamless transition from the smaller-scale facilities. This new centre, operating as a ‘manufacturing hotel’ for companies, will position the UK firmly in the international market for inward investment and supply the European marketplace, quickly and effectively, with cell therapies. It also ensures that we are in a position to tap into the wealth of knowledge and research available in the UK supporting the whole of its growth and development. In addition, it will compliment the great UK network of high-quality Phase I and II facilities with large-scale manufacturing capacity.

Our role in delivering and managing the facility, as well as training and supporting its 100+ staff, reinforces our aim of becoming a leading enabler in the global cell therapy industry.
Our developmental milestones

Q2
- Keith Thompson sets out vision for the Cell Therapy Catapult at the World Stem Cells and Regenerative Medicine Congress

Q3
- Five-year strategic and operational plan approved

Q4
- Facility conceptual design and pod concept finalised

2012
- First full independent Board meeting

Q1
- Collaboration with ReNeuron on manufacturing technologies

Events

Projects

People
- Keith Thompson appointed CEO
- Natalie Mount appointed Chief Clinical Officer
- John Brown appointed Chairman
- Matthew Durdy appointed Chief Business Officer

UK cell therapy activity

12  The Cell Therapy Catapult First Review to March 2014
Keith Thompson sets out vision for the Cell Therapy Catapult at the World Stem Cells and Regenerative Medicine Congress

Five-year strategic and operational plan approved

Facility conceptual design and pod concept finalised

Innovative manufacturing collaboration with Loughborough University

Collaboration agreement with Canadian Centre for Commercialization of Regenerative Medicine

Agreement to work with UK Stem Cell Foundation on stem cell therapies

Innovative manufacturing collaboration with GlaxoSmithKine to explore joint working on cell therapies

Regenerative Medicine report from UK House of Lords

Supporter of Christmas Lectures at Royal Institution

The Cell Therapy Catapult T-cell therapy white paper

Move into new facilities

£55m UK large-scale manufacturing facility announced

Training course on cell therapy manufacturing led by the advanced therapy medicinal products manufacturing community, the Cell Therapy Catapult and UCL Biochemical Engineering

Agreement to work on acellular scaffolds with Leeds University

Joint venture with UCL and Imperial on leukaemia cell therapy

The Cell Therapy Catapult First Review to March 2014

£55m UK large-scale manufacturing facility announced

Training course on cell therapy manufacturing led by the advanced therapy medicinal products manufacturing community, the Cell Therapy Catapult and UCL Biochemical Engineering

Agreement to work on acellular scaffolds with Leeds University

Joint venture with UCL and Imperial on leukaemia cell therapy

Employees in May 2013 37 Total

Employees in June 2013 40 total

Employees in August 2013 57 total

Employees in December 2013 68 total

Stephan Ward appointed Chief Operating Officer

Johan Hyllner appointed Chief Scientific Officer

UK preclinical research projects

UK cell therapy clinical trials

Total activity
Raising the profile of cell therapy in the UK

- Wellcome Trust awards BloodPharma team £5m for red blood cell transfusion project 14 April 2014
- Budget unveils £55m large-scale Cell Therapy Manufacturing Centre for the UK 19 March 2014
- The Cell Therapy Catapult takes possession of new state-of-the-art premises 24 February 2014
- LifeSciences UK report highlights positive impact of the Cell Therapy Catapult 23 January 2014
- The Cell Therapy Catapult and Roslin Cells to create clinical grade stem cells to accelerate research into new treatments 11 September 2013
- The Cell Therapy Catapult and Regenerative Medicine Centres in Canada and UK collaborating to accelerate commercialisation of new medicines for patients 31 May 2013
- The final piece of the jigsaw – Why the UK is building cell therapy capacity MedNous, April 2014
- Scientists ready to make artificial blood in next step to re-engineer human body The Times, Science, 14 April 2014
- Realising the potential of cell therapies PMLiVE, 18 December 2013
- The Cell Therapy Catapult future prospects looking up Manufacturing Chemist, April 2013
- Going to market Research Fortnight, 17 October 2012

Key:
- The Cell Therapy Catapult news
- The Cell Therapy Catapult press coverage and articles
Developing the environment for cell therapy

Over the period, we engaged in a variety of activities aimed at improving the environment for the development of cell therapies.

**Preclinical and clinical databases**

Now in their second release, these annual surveys aim to showcase the UK cell therapy pipeline.

**Reimbursement road map**

For the first time it is possible to see in one place, the actions, institutions and process involved with achieving reimbursement in the UK for cell therapy-related products.

**Manufacturing survey**

The manufacturing survey collects information to provide an overall picture of the Good Manufacturing Practice (GMP) capability and capacity in the UK, providing a gateway and vision for cell therapy.

**Regenerative Medicine Expert Group (RMEG)**

The Cell Therapy Catapult has taken a key role in the grouping of industry leaders whose aim is to develop an NHS regenerative medicine strategy to deliver innovative treatments and assess the effect of regulation on the development of therapies in the UK.
**Healthcare need and technical challenge**

Blood transfusions play a critical role in current clinical practice, with over 90 million red blood cell transfusions taking place each year worldwide. While many developed economies can meet their routine blood transfusion needs via donated blood, many thousands of lives are lost elsewhere annually as the result of a lack of a safe blood supply.

Recognising this need, the BloodPharma consortium, led by the Scottish National Blood Transfusion Service, has been working on the development of red blood cells for transfusion from stem cells for around four years. Recently, funding of £5m has been awarded by the Wellcome Trust for scale-up of the manufacturing and move into clinical trials.

**Working in collaboration towards a solution**

With the ultimate aim of producing a safe and effective blood substitute that can be produced in the significant volumes required for routine healthcare use, we have joined the consortium to help meet these challenges.

**The Cell Therapy Catapult expertise**

Our process development, manufacturing, analytical development, regulatory, clinical and health economics teams are working on this exciting project.

**Generating health and wealth**

The widespread availability of a safe and effective blood substitute would bring significant healthcare benefits worldwide, saving thousands of lives. In addition, the insights and technical developments arising from the project will be of value to other researchers working on the development of cellular therapies.

“Improving health through research is a priority for the Wellcome Trust, and this interdisciplinary stem cell-derived red blood cells programme is expected to have a significant impact in the field.

The range of expertise the Cell Therapy Catapult brings to this project consortium, particularly in the health economics and market access areas, makes it a compelling proposition.”

**Richard Seabrook.**

Director Business Development, Wellcome Trust
Stem cell bioengineering for airway diseases

The challenge
Severe Structural Airway Disease causes airway obstruction, leading to poor health and a 50% mortality rate if not treated successfully. Current surgical treatment has a high incidence of failure and there is an urgent need for a more effective and long-lasting solution.

To meet this need, an Innovate UK-funded consortium led by Videregen Ltd, a UK SME, is developing a technique, which involves repopulating a decellularised trachea scaffold with the patient’s own stem cells and epithelial cells. The other project partners are NHS Blood and Transplant, Royal Free Hospital, University College London and the Cell Therapy Catapult.

Working in collaboration to find a solution
As an alternative to conventional tracheal transplantation, this project represents a step change approach to the treatment of airway disease. Working towards and undertaking clinical trials requires an innovative approach to the regulatory and clinical hurdles. The Cell Therapy Catapult will be using its expertise in these areas.

The Cell Therapy Catapult expertise
Our clinical and regulatory teams are working with the consortium on this groundbreaking project.

Generating health and wealth
This innovative project addresses an unmet medical need, as well as opening up other opportunities in organ replacement. The expertise gained throughout the project will remove roadblocks for future development of similar products, assisting the cell therapy industry as a whole. In addition, the project gives a UK SME the potential to become a leader in the field.

“Partnership with the Cell Therapy Catapult has been instrumental in securing the Innovate UK grant (£1.9m) and Videregen’s recent fundraising (c £1.2m). It’s provided valuable expertise in regulatory and clinical development for our novel technology platform, enabling us to accelerate our development plans.”

Dr Steve Bloor
CEO of Videregen

The Cell Therapy Catapult gives us an expert resource that would be difficult for us to access otherwise, and we consider it part of the company’s team. We consider the partnership to be an important validation of Videregen and its technology.”
Working with one of the leading UK cell therapy companies on its flagship product

Healthcare need and technical challenge
ReNeuron is a leading UK cell therapy company with two products in clinical trials – ReN001 for stroke, which is soon to enter Phase II clinical trials, and ReN009, which has entered Phase I for limb ischaemia. The Cell Therapy Catapult worked with the company on making the manufacturing processes for the CTX stem cell line commercially ready. In doing this, we utilised our analytical, process development and manufacturing expertise.

Working in collaboration towards a solution
Combining ReNeuron’s product knowledge and our manufacturing expertise, the collaboration focused on scaling-up manufacture of the cell line, automated manufacture and potency assays. Work on developing a cryogenically preserved product that can be stored for long periods of time – and to be used in clinical trials – was also undertaken. Manufacturing skills at Roslin Cells in Edinburgh and the University of Loughborough also formed part of the collaboration.

The Cell Therapy Catapult expertise
This exciting project enabled us to put our novel process technology, assay and Quality by Design thinking to good use.

Following the initiation of the collaboration, ReNeuron received a £33m financing package from a group of funders and institutional investors, enabling it to position itself as a global leader in stem cell development. Commercial supplies of its products will be made at a new manufacturing factory in Wales.

Generating health and wealth
The benefits of this collaboration include the development of new expertise at the Cell Therapy Catapult that can be used to accelerate the growth of the industry. In addition, it has helped a leading cell therapy company cement its position in the UK, assisted in development of its new medicines and enhanced its commercial and competitive edge.

“Important support for the financing was provided by the validation of the Cell Therapy Catapult collaboration...”

Michael Hunt
CEO of ReNeuron
New joint venture creates an innovative approach to targeting cancer cells

The challenge
A novel approach to the treatment of acute myeloid leukaemia and myelodysplastic syndrome has been developed by scientists at Imperial College and University College London (UCL), initially funded by the charity Leukaemia & Lymphoma Research. Patients with these diseases often have limited treatment options and a poor prognosis, meaning new treatment options are much needed. A new multiparty approach was needed in order to accelerate the development through clinical trials of the treatment, a T-cell therapy targeting overexpression of the WT1 antigen.

Working in collaboration to find a solution
The Cell Therapy Catapult, UCLB and Imperial Innovations worked together to form a new joint venture company, the Catapult Therapy TCR Ltd, to manage the development of the WT1 cell therapy. The new company will accelerate the start of clinical trials (expected in late 2014 and early 2015), as well as facilitate the future development of the therapy and allow everyone to share in its eventual success. Core funding of up to £10m for early-stage trials is coming from the Cell Therapy Catapult.

The Cell Therapy Catapult expertise
The Cell Therapy Catapult is using its expertise in many aspects of this project, including manufacturing and process development, clinical trial sponsorship and the commercialisation strategy.

Generating health and wealth
This T-cell therapy project is addressing the clear unmet medical needs of acute myeloid leukaemia and myelodydysplastic syndrome, for patient benefit, as well as showing the UK to be a leader in the development of this novel, growing and important area of medicine.

“Seeing patients in clinic every week serves as a stark reminder of how urgent it is to have new treatment options for diseases such as acute myeloid leukaemia. The involvement of the Cell Therapy Catapult in the WT1 T-cell therapy work, and the range of expertise it brings across the board, has accelerated project progress and put it on a much stronger commercial footing.”

Dr Emma Morris
UCL Clinical Academic,
Chief Investigator and Co-inventor
The challenge
Induced pluripotent stem (iPS) cells, which were discovered in 2006, are a highly versatile source of cells for new therapies based on their ability to differentiate into any cell type. As a result, they are being extensively investigated in preclinical research. In this project, in order to meet the requirement for GMP cells for clinical trials once a therapy has been developed, a clinical grade iPS cell bank is being developed. With research grade lines derived therefrom to be made available for early-stage work, the bank will enable acceleration of therapies into clinical trials.

The Cell Therapy Catapult expertise
The Cell Therapy Catapult is using its expertise to manage this project, including negotiation of appropriate licences, and will ultimately be responsible for distribution of the cell lines.

Generating health and wealth
The establishment of the clinical grade iPS cell bank puts the UK at the forefront of the development of new treatments based on this versatile cell type, ultimately meeting patient need.

Working together to develop a GMP grade iPS cell bank

“iPS cells have vast therapeutic potential across a range of unmet medical needs. We anticipate that the provision of a high-quality cell bank such as this will mean a step change in the speed at which iPS-based cell therapies can enter the clinic and ultimately reach the market for patient benefit.”

Chris Mason
Professor of Regenerative Medicine, University College London
Chair, Cell Therapy & Regenerative Medicine Advisory Committee, BioIndustry Association (BIA)
Financial highlights

The Cell Therapy Catapult is an independent private company limited by guarantee incorporated as a not-for-profit research organisation. The financial information in this review is the consolidated results from the statutory accounts for the Cell Therapy Catapult for the year ended 31 March 2014.

Turnover

For year ended 31 March 2014

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 £</th>
<th>13 month period to 31 March 2013 £</th>
</tr>
</thead>
<tbody>
<tr>
<td>Innovate UK (formerly the Technology Strategy Board) core revenue grant funding</td>
<td>7,769,951</td>
<td>1,724,808</td>
</tr>
<tr>
<td>Innovate UK core capital grant funding</td>
<td>8,802,782</td>
<td>251,962</td>
</tr>
<tr>
<td>Other grant funding</td>
<td>24,642</td>
<td>–</td>
</tr>
<tr>
<td>Commercial income</td>
<td>83,239</td>
<td>–</td>
</tr>
<tr>
<td><strong>Total turnover</strong></td>
<td><strong>16,680,614</strong></td>
<td><strong>1,976,770</strong></td>
</tr>
</tbody>
</table>

Balance Sheet

As at 31 March 2014

<table>
<thead>
<tr>
<th>Description</th>
<th>2014 £</th>
<th>2013 £</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fixed assets</td>
<td>8,924,523</td>
<td>248,220</td>
</tr>
<tr>
<td>Net current assets</td>
<td>4,615</td>
<td>1</td>
</tr>
<tr>
<td>Provisions for liabilities – deferred tax</td>
<td>(1,772,172)</td>
<td>(43,879)</td>
</tr>
<tr>
<td><strong>Net assets</strong></td>
<td><strong>7,156,966</strong></td>
<td><strong>204,342</strong></td>
</tr>
</tbody>
</table>

| Capital and reserves                                 | 7,156,966  | 204,342    |
| **Company information**                              | 7,156,966  | 204,342    |

Directors

Dr J.R. Brown CBE FRSE (Chairman)
K.J. Thompson (Chief Executive)
Dr Z. Latif (Non-Executive Director)
T.P.W. Edwards (Non-Executive Director)
N.A. Higgins (Non-Executive Director)
Professor M.L. Turner (Non-Executive Director)
Professor M.J. Whitaker (Non-Executive Director)

Company Secretary

S. Crossley

The 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