Technology Strategy Board
Driving Innovation

Collaboration nation

Regenerative medicine projects

Introduction

This directory of projects we helped to fund provides an overview of the opportunities highly innovative companies can offer across the UK through their developing technologies. It can also be used to help link those companies to the wider funding community to develop their ideas into new products, processes and services.

Regenerative medicine therapeutics feasibility studies

We funded 31 projects totalling a £3m investment as the first part of our £18m Regenerative Medicine programme. Twenty-eight of those projects are highlighted at Collaboration Nation.

Each six-month project received up to £100k to explore the feasibility of developing previously identified therapeutic candidates, that can replace or regenerate human cells, tissues or organs to restore or establish normal function, into regenerative medicines.

Some projects were conducted by single companies whilst others were carried out by collaborative consortia.

Regenerative medicine collaborative R&D projects – value systems and business models

We funded three projects (with additional funding from the Scottish Government and the Economic and Social Research Council) to develop a better understanding of where and how value will be created in the regenerative medicine value chain, and to develop business models that will enable businesses to best capture that value.

These projects are being carried out by business-led consortia.

This directory provides a snapshot of the winning projects so that potential future collaborators, investors and companies interested in open innovation can get to know the companies involved.

Feasibility projects

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Lead partner
AedStem Ltd

Disposable device to produce a serum-based chondrogenic reagent from a patient's own serum sample

The problem our feasibility study project is trying to solve

AedStem Ltd aims to improve the efficacy and cost-benefits of biologically-based cartilage repair therapies by developing highly cost-effective methods for converting stem cells (eg, mesenchymal stem cells) into functionally mature cartilage cells.

Our products could potentially be used in both research laboratories and as an adjunct to a variety of next generation cell-based cartilage repair therapies.

What our study is aiming to achieve

The study aimed to:

- optimise the composition of biologically-based reagents to stimulate MSC into cartilage cells
- develop methods to produce these reagents.

The potential benefits

Cartilage injuries do not heal and, if left untreated, tend to further degenerate until total joint replacement becomes unavoidable. Our products aim to assist in the development of stem cell-based therapies for these injuries.

What we need to do next

We have recently obtained additional funding to continue development of the clinical applications of our technology. We are currently seeking:

- laboratory research groups who are interested in the biology of biomaterial and cell-based cartilage repair
- clinical groups using human MSC for regenerative orthopaedic medicine.

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Aqix RS-I: regeneration of organs otherwise marginal for transplant

The problem our feasibility study project is trying to solve

How to utilise more organs that have been donated for transplant and thus reduce the transplant waiting list.

What our study is aiming to achieve

The underlying patented product, AQIX RS-I, is a synthetic interstitial fluid, designed to restore homeostasis and normal activity in cells, tissues, and living systems. We investigated the feasibility of a GMP version of the product and determined how to take it through the regulatory process.

The potential benefits

There is a US$150m+ market for regenerative medicine for donor kidneys. The savings to the NHS alone could be US$500m over 10 years.

What we need to do next

We are developing a transplant model in conjunction with the University of Newcastle. However, there are many other potential uses of AQIX RS-I and its GMP status makes it regulator friendly. We are looking at roles in transport of regenerative medicine therapeutics and other therapeutic applications.

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CellBeads for the prevention of vein graft disease

The problem our feasibility study project is trying to solve

The saphenous vein remains the most commonly used conduit for coronary artery bypass grafting. Up to 50% of grafts fail within 10 years however, due to vein graft disease (VGD) caused by atherosclerosis, resulting in recurrent angina, heart attacks and death. VGD occurs as a result of adaptive changes in the veins when subject to arterial pressures and flow and disruption of the microvasculature within the vein wall during harvesting. We are investigating the local application of genetically-modified stem cells encapsulated in alginate microspheres to prevent VGD by local release of proangiogenic and other regenerative factors into the vessel wall.

What our study is aiming to achieve

We have developed genetically-modified stem cells which secrete a variety of proangiogenic and regenerative factors. These cells have been immortalized and then encapsulated in an immuno-protective alginate coating to produce microspheres (CellBeads) that can be implanted at various sites within the body where healing and regeneration are desired. The CellBeads are manufactured to GMP and are currently in clinical evaluation in the area of haemorrhagic stroke. In this project we are evaluating the feasibility of local application of CellBeads to the vein wall immediately post implantation in order to encourage angiogenesis and prevent the occurrence of VGD.

The potential benefits

There is no effective treatment of VGD apart from repeat surgery, which carries additional risk. With over 30,000 patients undergoing coronary artery bypass grafts (CABG) in the UK at an estimate of £8-10K per procedure, an effective treatment for VGD represents both a significant health benefit and financial benefit to the healthcare system.

What we need to do next

Significant further funding is required for more extensive pre-clinical testing to demonstrate unequivocal safety and efficacy for the treatment and provide the regulatory data necessary to allow the initiation of clinical studies. Randomised clinical data would be ultimately required to establish the treatment as a routine procedure during CABG.

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Formulation and processing of cell-containing regenerative medicine products to enable long-term storage

The problem our feasibility study project is trying to solve

Unlike other interventional approaches in healthcare, many regenerative medicine products rely on the delivery to patients of products that incorporate live cells. This presents a significant challenge since cells are living material, and so are highly perishable and require costly and hazardous storage (eg, liquid nitrogen). This project seeks to address these challenges by demonstrating the feasibility of preserving human cells through freeze drying.

What our study is aiming to achieve

Our feasibility study focused on the freeze-drying of red blood cells as a simple but sensitive model system, prior to studying nucleated cells. Several variables were examined, in order to enable an understanding of what factors are critical in achieving cell survival and minimising haemoglobin oxidation, namely: methods for concentrating cells iso-osmotically, pH buffer type, potential 'protectant' molecules, employing a novel polymer to modulate cell membrane permeability, cooling rate, freezing temperature, drying temperature and rehydration conditions. The study of red blood cell stabilisation in itself has significant value in enabling possible reduction in the wastage of donor blood for medical use.

The potential benefits

The capability of long-term storage of cells with minimal refrigeration would have a direct impact not only on current high value regenerative products and associated services (eg, blood, bone marrow and cord blood) but also completely alter the market dynamics of emerging therapies that incorporate live cells.

What we need to do next

The next steps will be to achieve successful scale-up and technology transfer, enabling the handling of larger volumes of human blood (or blood components) for long-term storage, and the application of knowledge gained in this project to address how the same approach can be applied to nucleated cell-based therapeutics.

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Novel T cell immunotherapy to treat adenovirus infections in paediatric patients following allo-HSCT

The problem our feasibility study project is trying to solve

Adenovirus infections develop in up to 30% of paediatric patients following allogeneic haematopoietic stem cell transplant (allo-HSCT). Infection is associated with a high level of mortality and at present, there are no effective antiviral drugs available. This feasibility study would allow Cell Medica to validate a process for the reliable generation of adenovirus-specific T-cells from donors for adoptive transfer to patients. It will facilitate the launch of a Phase I/II clinical trial to investigate the effectiveness of a novel cell therapy to treat adenovirus infections in children following allo-HSCT.

What our study is aiming to achieve

Validation of the Rapid Expansion System for the reliable generation of an adequate adenovirus-specific T cell dose from donors will facilitate the launch of a Phase I/II study investigating the use of this promising cell therapy for immune reconstitution in paediatric patients who are at risk of untreatable adenovirus infections.

The potential benefits

Successful performance of this feasibility project will progress the first of Cell Medica's 'second generation' products and will validate the company's business strategy demonstrating an active research portfolio. The Rapid Expansion System may provide platform technology that can be easily adapted for other low frequency antigen-specific cells, hence shortening development times for subsequent cell therapy products.

What we need to do next

Following completion of this feasibility study, we need to formalise the design of a Phase I/II study with clinical collaborators. In parallel, adenovirus specific T-cells will be progressed through the regulatory approval process to obtain a CTA. This will require input from regulatory experts and research focussed paediatricians.

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Determining the efficacy of candidate cell therapy products

The problem our feasibility study project is trying to solve

Method of testing efficacy of cellular products for QC

What our study is aiming to achieve

Develop fast in vitro assay to evaluate wound healing

The potential benefits

- QC for cell therapy
- Testing system for drugs and cosmetics

What we need to do next

- Validate against a library of chemicals
- Scale-up

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Improving bioprocessing of therapeutic cells via dead-cell removal

The problem our feasibility study project is trying to solve

Cell death is a major problem in production and delivery of populations of therapeutic cells in which the viability of the cells administered to patients needs to be high. We have developed technology that specifically removes dead cells from cell populations, thereby improving their quality. The aim of this project is to adapt the technology so that it can be applied to clinical grade cells.

What our study is aiming to achieve

We have developed a dead-cell removal system that works well with cells for research purposes. What we are now developing in this project is a dead-cell removal system that can be used in the manufacture, storage and delivery of therapeutic cells. The aim is to be able to manufacture a clinical-grade dead-cell removal system that will satisfy all regulatory issues.

The potential benefits

Loss of cell viability causes significant loss of product (50% or more in certain cell therapies). Our technology is set to provide a step change in capacity to improve therapeutic cell quality which will benefit potentially all companies producing cells for therapeutic purposes.

What we need to do next

We need now to collaborate with therapeutic cells manufacturers - producing clinical grade cells now - to demonstrate that our technology improves their products at one or more stages:

- manufacture
- storage/supply
- clinical efficacy.

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Allogeneic fibroblast therapy for epidermolysis bullosa

The problem our feasibility study project is trying to solve

Dystrophic Epidermolysis Bullosa is a rare, inherited disease with devastating consequences to the sufferer and their family. The disease causes severe blistering and patients are sometimes referred to as butterfly babies as the slightest touch can cause skin damage. Based on pioneering work at King's College London, we are aiming to develop the first clinically approved treatment for this disease. This is based on injecting normal human dermal fibroblasts into damaged areas of the skin. From initial studies on patients, these cells seem to stimulate the rapid regeneration of the patient's skin.

What our study is aiming to achieve

The study aims at getting the regulatory and clinical elements in place to start a Phase II clinical trial in 2010. This involves developing GMP manufacture, obtaining Orphan designation from the EMA and FDA, obtaining input from the MHRA concerning the format of a clinical trial and creating appropriate clinical protocol and other documentation needed to carry out a clinical study.

The potential benefits

The potential benefits extend beyond potentially providing the first treatment for these patients. It will also provide the basis of a new, self sufficient company that will manufacture and distribute this therapy, initially in the UK and then in additional countries around the world. This could eventually lead to exports of over £100m/year.

What we need to do next

We now need to carry out the clinical programme started in this project. Treatments for this Phase II study will begin by the end of 2010. Following successful completion of this study Phase III clinical trials will have to be carried out and additional funds will be needed to support this.

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Allogeneic cell therapy for acute liver failure

The problem our feasibility study project is trying to solve

Acute liver failure affects approximately 300 patients annually in the UK, and at any one time there are approx. 300-400 individuals awaiting liver transplant. For the majority of affected individuals the outlook is poor as treatment options are limited and patients require a significant degree of artificial liver support until there is adequate tissue regeneration or transplant.

What our study is aiming to achieve

The project aims to address a number of challenges facing LiverCyte as it moves an allogeneic cell therapy for treatment of acute liver failure into the clinic. The project output will:

- identify major regulatory, quality, manufacturing and logistic hurdles
- propose solutions to problems identified
- provide a 'generic roadmap' to clinic for other companies active in the sector.

The potential benefits

The core product to arise out of this project would be a cell-based treatment - initially developed for acute liver failure, and subsequently as a treatment for individuals awaiting a transplant. The product will be a number of allogeneic cells potentially supplied as a cryopreserved vial which could be shipped to a specialist liver clinic, and cells resurrected prior to administration. It is envisaged that patients with acute liver failure may require several treatments over a 5-10 week period in order to build and maintain liver function.

What we need to do next

To move this technology forward LiverCyte needs to:

- manufacture allogeneic cell banks
- establish efficacy in clinical studies
- obtain funding to progress to phase 2/3 clinical trials.

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EktoTherix: development of a regenerative tissue scaffold for repair of surgical excision wounds

The problem our feasibility study project is trying to solve

EktoTherix has been developed for the repair of excision sites of which wounds resulting from the surgical removal of non-melanoma skin cancers are the principal indication. Skin cancer incidence has been growing worldwide at around 5% pa for the past 40 years. Removal by surgical excision is the treatment of choice but does leave a site that must be repaired. This feasibility study addresses whether EktoTherix is a viable option to simplify and improve the repair pathway whilst reducing overall healthcare costs.

What our study is aiming to achieve

We have developed two prototype tissue repair scaffolds for evaluation of their efficacy in a preclinical wound repair model. We have also assessed the feasibility of developing a GMP manufacturing process capable of scale-up and have commissioned advice on the regulatory status of the EktoTherix scaffold device.

The potential benefits

The estimated global market for this product is £360m pa. There are benefits to the clinician and health service (simplification and reduction of time spent re-dressing wounds leading to cost savings) and the patient (an expected improvement in the cosmesis of healed wounds).

What we need to do next

Neotherix now needs to continue the development towards full commercialisation by conducting confirmatory preclinical testing for efficacy and safety, performing full GMP production trials and exploring the patient pathway. On completion of these stages, our aim is to conduct a clinical evaluation of the product, with an estimated market launch in late 2012.

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High strength regenerative tissue scaffolds for large lesion articular cartilage repair

The problem our feasibility study project is trying to solve

Each year around 740,000 people - often young - suffer traumatic cartilage injuries. Many of these patients will go on to develop osteoarthritis of the knee as a result of the damage to the cartilage, often requiring eventual total knee replacement (TKR). TKR now costs the NHS over £1bn - more than hip replacement and is only appropriate for older patients, leaving a large number of young patients with a crippling knee condition. Orthox has developed Spidrex® - a high strength, resilient, silk-based biomaterial with the potential to effect both a functional and regenerative repair of damaged cartilage tissue.

What our study is aiming to achieve

Using Spidrex®, Orthox has developed FibroFixT: a device for repair of the shock-absorbing meniscal cartilage in the knee. The objective of this feasibility study has been to adapt the properties of FibroFixT for repair of the smooth articular cartilage which covers the ends of the thigh and shin bone in the knee joint, and then evaluate the resulting devices. This required development of new casting methodologies to replicate the contoured surfaces of the knee joint. Then, the manufacturing conditions were adapted to achieve appropriate mechanical properties and porosity to enable the devices to act as functional replacements and regenerative scaffolds

The potential benefits

There is a pressing, unmet, clinical requirement for early intervention knee cartilage repair products. They allow patients to resume healthy, active lifestyles and prevent knee joint deterioration and the enormous expense associated with TKR. With an ageing, obese population, TKR frequency is increasing rapidly and costs around $65bn in the US.

What we need to do next

Follow-on funding has been secured from the Technology Strategy Board and Wellcome Trust to further develop this promising prototype product and conduct additional evaluatory trials. This will require development of a reliable fixation methodology, include consultation with the surgical community and involve collaboration with a major industry partner.

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Therapeutic ES cells: a map for sustainable supply

The problem our feasibility study project is trying to solve

This project will address a key challenge to sustaining regenerative cell products through clinical trials to approval. It will identify critical points in the product development path based on 'just-in-time' delivery of a candidate human ES cell (HES) therapy for age-related macular degeneration (AMD) and prescribe alternative, regulatory compliant approaches to product supply. The clinical candidate is a combination cells and device therapy. AMD is a disease which has no significant symptom-modifying treatment for the dry form or current cures to enhance quality of life and the therapeutic candidate is planned for clinical entry beginning with initial international regulatory body discussions.

What our study is aiming to achieve

Generic recommendations based on critical path challenges will be exemplified and an evidence-based deliverable for critical path specifications and general points-to-consider will benefit the wider community. Defining raw material issues for the AMD candidate sooner will bolster process design, strengthening likelihood of trial completion and market approval.

The potential benefits

There are currently no approved options for dry AMD (85% cases) and equivocal evidence for effectiveness of main treatments for wet AMD (15% cases) (Lucentis, Avastin). Thus cell therapy is a strong, competitive alternative to current AMD treatment. The ultimate beneficiaries will be the patients, if a successful cell product is developed. Pfizer, the lead project partner will develop a valuable knowledge of developing HES cell based regenerative medicines.

What we need to do next

Pfizer will continue with the clinical development plan integrating the important project learnings to develop contingency plans for the manufacturing process to ensure regulatory authoritisation. The organisation will be better able to understand the likelihood of clinical success once the first trials have been performed.

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Animal component-free bioprocess development for MSC-derived osteoblasts

The problem our feasibility study project is trying to solve

Current bone graft treatments for spinal fusion and fracture repair, for example, are unsatisfactory. Stem cell derived therapies could offer an alternative approach to overcome the associated problems such as pain and non healing at the graft collection site, immune-rejection and insufficient supply of material. However, before stem cell therapies can become routine alternative treatments, several technical difficulties need to be overcome; primarily concerning the consistent generation of high quality cell populations at scale.

What our study is aiming to achieve

Having previously discovered a highly efficient protocol for generation of bone cells from mesenchymal stem cells (MSCs) that could form the basis of a bone-graft substitute product, this study aimed to address the issues of reproducing the protocol at a scale that would allow performance of pre-clinical studies and clinical trials. We aimed to develop scale-up methods so as to optimise differentiation efficiency, reduce the cost of production and allow simple quality control processes.

The potential benefits

A stem cell derived bone graft substitute giving improved, consistent, more cost-effective treatment for patients. The commercial benefits to Plasticell and the UK economy from development of this product could be considerable given the billion dollar bone graft market.

What we need to do next

The therapeutic potential of scaled-up cell populations in combination with bioscaffold constructs needs to be ascertained, requiring collaboration with bioscaffold experts to assess different materials and performance of animal model studies and in longer term clinical trials, to assess efficacy of cell/bioscaffold combinations.

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Development of an allogeneic articular cartilage stem cell therapy

The problem our feasibility study project is trying to solve

Articular cartilage is the smooth white tissue that covers the surface of the joints in the human body. Articular cartilage damage due to traumatic injury or degeneration is a very common problem. The most common method of treating traumatic injury - 'microfracture' - is suboptimal and prone to long-term complications. Modern cell-based therapies have been developed but these are complex, expensive and inconvenient. There is no current cell-based therapy for cartilage degeneration. Progenteq was established in 2009 to explore the feasibility of creating a universal cell-bank for cartilage repair using a proprietary cell type isolated from mature cartilage.

What our study is aiming to achieve

This novel cell type was discovered at Cardiff University. The objective of the study was to assess whether or not the cells and the process used to isolate and grow them have the potential to be developed into a commercially-viable product. The work involved was two-fold. Firstly a paper-based 'roadmap' exercise sought to set out how to modify the process to allow a cell-bank to be established. Secondly, a laboratory-based technology transfer of the process would demonstrate that the process could be established in the facilities of a commercial manufacturing company (Angel Biotechnology plc).

The potential benefits

Over two million people suffer from acute cartilage damage in the US and Europe each year. Successful development of this product would transform the way in which articular cartilage damage can be treated. It has the potential to provide an improved therapy at a lower cost than current cell-based options.

What we need to do next

Progenteq has received funding until Q1 2011 from Technology Strategy Board and corporate investors. Thereafter it will require a further ~£1-2m in funding. This money will be used to further characterise the cells to comply with regulatory requirements, to commence cGMP manufacture and to conduct a first-in-man clinical study.

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Scale-up and manufacture of an injectable regenerative bone matrix

The problem our feasibility study project is trying to solve

Each year around 1.6 million bone graft procedures are performed in the US alone. The present gold standard of fracture repair, autografting, is hampered by the need for a harvesting operation, which in turn often provides limited substitute tissue and has a high incidence of post-operative morbidity. Treatments relying on donor tissues are subject to supply and pose risk of rejection and infection. Other current treatment options all have inherent significant deficiencies. Our product, Injectable Bone, aims to advance the state of the art due to ease of use, its post-hardening physicochemical properties, versatility, and ready supply.

What our study is aiming to achieve

The objective of this feasibility study was to advance manufacturing development work for our product. We had previously identified a scaleable two-stage manufacturing process that can be operated to GMP standards and demonstrated that small batches of particles can indeed be generated by this approach.

The main aim was to demonstrate that larger scale batches can be reproducibly processed using this strategy. We additionally sought to investigate the potential to adjust the presentation of our material in order to extend its shelf life.

The potential benefits

A platform-to-product company, RegenTec aims to build a portfolio of products to treat a wide range of clinical disorders. Injectable Bone is unique as its characteristics open new applications that cannot be accessed using competitor products (eg, drug and cell delivery). Our ability to secure joint development deals for proprietary actives is significantly enhanced by the data.

What we need to do next

Follow-on funding will now be sought to complete a 15-month work package (which includes performance testing, validation, sterility assurance, stability, labelling activities) to enable the US market approval and EU clinical investigation of our first generation product.

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cGMP manufacturing, formulation and delivery challenges for a proprietary clinical grade stem cell product for peripheral arterial disease

The problem our feasibility study project is trying to solve

One major uncertainty in the regenerative medicine field relates to how manufactured product can be readily presented in a commercial form. Currently, most stem cell products being developed for the clinic are not readily marketable due to potential manufacturing limitations, lack of batch to batch consistency or poor shelf life. All of these issues are making stem cells seem uncompetitive in the marketplace. What is needed for high market penetration of stem cell products is a consistent, stable product with a long shelf life and relatively low cost of goods.

What our study is aiming to achieve

A fully GMP manufactured and tested stem cell product at high scale that can be shipped worldwide, stored within hospital or clinic pharmacies and able to be used locally by the clinician on demand with minimal manipulation on site and by the clinical team themselves. This study is designed to take our frozen stem cell product, CTXcryo, to cGMP outsourced manufacture, thereby demonstrating its true product potential.

The potential benefits

A novel proprietary product as well as a standardisable formulation of stem cells that can be developed for other products as the market develops. If stem cell therapies do develop as is forecast, then the market for this kind of formulation could be a significant component of a >US$5bn pa market in the next 5-10 years.

What we need to do next

Over the next three years we will take our product, CTXcryo, to a clinical trial programme for at least two indications. On clinical proof of concept, we will partner this product for later stage clinical development and eventual marketing as well as out-license the technology for other applications.

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The Glasgow stroke disability stem cell trial

The problem our feasibility study project is trying to solve

ReNeuron has received regulatory and ethical approval for a first-in-man clinical trial to treat stroke disability with a stem cell therapy injected directly into the patient's brain. This trial requires procedures for monitoring patient safety, a special needle for implanting the cells, and manufacture of the cell product to treat a number of patients. The feasibility study has supported the development and provision of these specialist activities to allow the start of the clinical trial.

What our study is aiming to achieve

We are developing a cell therapy product with the potential to treat stroke disability. This product has been manufactured in a way that will make it available to the stroke community at large, with the possibility of providing a novel therapy for an unmet clinical need to hundreds, if not thousands, of patients. This first clinical trial will be a milestone achievement and will lead the way to future treatment of brain disease with stem cell therapies. Following this, and subsequent trials, it is anticipated that the product will be commercialised through licensing to larger biotech or pharmaceutical companies.

The potential benefits

Within the healthcare industry the value of the regenerative medicine business could be billions of dollars per year. A successful cell therapy product such as ReNeuron's will provide further impetus for investment in this industry in the UK. This in turn will lead to growth in product development with associated growth in employment in this sector over the coming years.

What we need to do next

The next stage of product development will require large scale investment in further clinical trials and optimisation of distribution and supply of product.

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Skin regeneration and scar reduction following injury

The problem our feasibility study project is trying to solve

RN1005, a protein, was identified as a potential new therapeutic protein drug candidate for the promotion of tissue regeneration and scar free healing. However, processes to make sufficient quantities of RN1005 needed to be identified to progress this promising drug candidate into formal product development.

What our study is aiming to achieve

The feasibility study addressed key developmental challenges to identify the best routes forward:

- effectiveness of methods for improving the secretion of RN1005 from a recombinant mammalian cell line
- screening methods to aid the development of a suitable fermentation and purification strategy for the provision of RN1005 protein.

The potential benefits

Success with the feasibility programme has highlighted novel methods for improving RN1005 secretion from cells. This, coupled with the many protein recovery and purification techniques screened have informed a more efficient route to the development of cell lines and production processes for RN1005.

This research can shorten the time to market of a scar reduction pharmaceutical drug allowing Renovo and the UK economy to capitalise on this huge, unmet market need.

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NPL
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Bioceramic therapeutic's porous bone scaffold – manufacturing and pre-CE technical file project

The problem our feasibility study project is trying to solve

The main technical challenge is taking the RepRegen proprietary glass composition and scaling-up production of porous granules. This is performed using the patented gel-casting process developed at Imperial College to form large-scale consistent foams for sintering with the end goal to produce porous bioactive glass granules. The glass comes into contact with water during the process so glass chemistry must be monitored during processing. The formed gel-cast foams must then be sintered at high temperature to form a pore structure similar to cancellous bone and broken up into granules to meet RepRegen's specification.

What our study is aiming to achieve

The aim of the project is to develop the first porous bioactive glass synthetic bone graft. Bioactive glass compositions currently on the market (NovaBone and BonAlive) are not suitable for porous processing as they crystallise on heating which reduces bioactivity in vivo. The advantage of porous granules is that the granule size can be varied depending on the indication (site of implantation or size of defect) giving the surgeon improved product handlability; as the product performance in vivo (bioactivity) is determined by the pore surface area, varying the granule size does not alter degradation characteristics, which would be the case for dense granules.

The potential benefits

Recent in vivo studies have shown RepRegen's proprietary strontium bioactive glass synthetic bone graft materials show faster formation of higher quality bone than conventional bioceramics. The presence of strontium and other osteostimulatory ions results in an environment that promotes up-regulation of bone forming cells (osteoblasts) and down-regulation of bone resorbing cells (osteoclasts).

What we need to do next

We plan to run the full set of biocompatibility tests on this product (ISO10993) as well as in vivo studies to compare it to conventional Bioglass and RepRegen's dense granular strontium bioactive glass synthetic bone graft - StronBone. We would also like to run gene array profiling on this family of materials to determine the cellular mode-of-action of strontium bioactive glasses.

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Imperial

Bioceramic therapeutic's porous hybrid bone substitute – technical file and quality design control

The problem our feasibility study project is trying to solve

The main technical challenge is to scale-up the hybrid material to achieve a consistent validated pore-structure, which controls material degradation and mechanical properties in vivo at kilogram batch levels. This product can be made successfully on a small-scale with microstructure and mechanical properties in the region of cancellous bone. Any toxic precursors must be driven off during processing to give a chemically homogeneous product batch-to-batch to ensure that the material degrades in a controlled fashion in clinical use.

What our study is aiming to achieve

The aim of the project is to scale-up a hybrid (polymer-ceramic) material for tissue regeneration. This product has the potential for use as a synthetic graft in load-bearing/load-sharing indications which is the 'holy-grail' in orthopaedics. The material is a nano-composite, which degrades congruently post-implantation. The data and standard operating procedures gathered in this project will form the basis of the product specification and be used to populate the design history and technical files for future regulatory submissions (EU and FDA).

The potential benefits

This product would be revolutionary in the orthopaedic field as there are no synthetic graft materials on the market with load-bearing claims/indications. This is due to the poor mechanical properties of synthetic ceramic materials when implanted. They are typically augmented with metal hardware. This product could potentially eliminate the need for this metalwork, subsequent operation to remove prosthesis and eliminate complications associated with metals such as stress shielding.

What we need to do next

Following on from the toxicity studies performed for the feasibility study, we plan to test this product with advanced cellular assays and immunofluorescent staining to determine the influence of dissolution products on human osteoblast cell lines. We also plan to test the product in a challenging in vivo model (eg, critical size or segmental defect).

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GMP master cell bank for red blood cell production

The problem our feasibility study project is trying to solve

The project will address a critical challenge in the development of the capacity to produce red blood cells (RBCs) from human embryonic stem cells (hESCs). That is, how to produce sufficient cells to establish a master cell bank (MCB) which is capable of supplying the quantities of RBCs needed to undertake clinical trials. It is essential to address this issue now as any change in the techniques used to create the MCB may have a direct impact on an application to the MHRA for a manufacturing licence or the EMA for the clinical trial.

What our study is aiming to achieve

The project will evaluate cell line expansion technologies and their suitability for use in GMP processes to generate clinical MCBs. We will establish the most suitable process to generate GMP MCB of suitable size and validate/test this process for robustness/stability with different cell lines. We will generate significant intellectual property in terms of the procedures and batch records required to execute the process. We will refine our existing QC capabilities to be tailored to production techniques. Finally, we will conduct a gap analysis to determine further work required before an application can be made to the MHRA.

The potential benefits

While this project is focussed on creation of MCBs for red blood cell production, the capabilities developed can be applied to the development of other therapies. In vitro RBC production will address the demand for publically donated blood which is increasingly problematic due to the increasing numbers of the population eliminated from donation due to clinical concerns and the lack of donors in developing countries.

What we need to do next

Further funding is required to develop relationships with research partners, prove the MCBs utility with other clinical grade cellular therapeutics and develop the in vitro differentiation protocols required (~£60k). Funding is also required to develop the extensive quality control testing that will be required (~£50k).

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Peptide amphiphile technology for the repair of cartilage lesions in osteoarthritis patients

The problem our feasibility study project is trying to solve

Total joint replacements have limited life and require revision surgeries to be carried out after 15-20 years. Today patients are living for much longer and face undergoing a number of revision surgeries. With each surgery the replacement of the joint becomes more difficult and the patient is faced with significantly limiting their lifestyle. We are trying to bridge the gap between current technologies and joint replacement with the aim of increasing the time before a patient faces joint replacement and therefore limiting the number of revisions that patient may be faced with in their life time. The feasibility study we are currently undertaking is to test this technology in a relevant preclinical model to investigate whether this technology can repair cartilage better than the most relevant current surgical procedure.

What our study is aiming to achieve

We have identified an early development stage technology which is known as a peptide amphiphile. This peptide has the ability to self-assemble into a peptide hydrogel which is capable of binding and concentrating growth factors. The feasibility study takes this technology and tests it against the current most relevant surgical procedure, microfracture. Within this feasibility study we are aiming to show that repair of cartilage lesions within our chosen model is better than that of microfracture alone.

The potential benefits

This technology would be an important addition to our company portfolio if proved successful. This technology provides a minimally invasive treatment for the repair of cartilage defects in patients suffering from osteoarthritis.

What we need to do next

Our next steps would be to carry out further product specification studies which would culminate in a final GLP preclinical study for presentation to the FDA. This would enable us to gain approval to enter into clinical trials for this product. These types of studies are a big commitment for the company requiring the input of many resources including clinical, regulatory, manufacture and R&D.

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Towards GMP production of RAFT tissues for cornea regeneration

The problem our feasibility study project is trying to solve

The outermost cornea layer is essential for vision and is maintained by stem cells which reside at the edge of the cornea. If these stem cells are lost, by injury or disease, the cornea becomes cloudy and blindness occurs. Current treatment is by transplant of cells cultured on natural materials causing variable outcomes. Our Real Architecture for 3D Tissue (RAFTT) technology aims to provide an improved therapy based on a reproducible biomimetic collagen scaffold that supports corneal surface regeneration. The tuneable properties of the RAFT scaffold offer broad applicability in regenerative medicine.

What our study is aiming to achieve

Transitioning a therapy from the lab to the clinic is a complex, lengthy and costly process and we need to determine the key steps and documentation required for this complex cell therapy. The feasibility study will outline the requirements for taking a cell therapy through clinical trials to market; including an understanding of the appropriate regulatory pathway to a first-in-man clinical trial. In addition we will develop and document methods for scalable, reproducible manufacturing methods, and compare cost of production with the existing therapy.

The potential benefits

Our RAFT scaffolds have major advantages over current materials in terms of safety, consistency, cost and superior function and could restore the sight of substantial numbers of people globally. The technology is based on UK intellectual property and could therefore benefit the UK economy substantially.

What we need to do next

With £275k we can undertake further work to transition towards a commercial product including; identify sources of cells (other than the patient's own), assess product safety, optimise the production process, and develop quality standards (potency and reference standards) and a robust shipping solution.

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Collaborative partners
The RAFT Institute

Design & development of an automated process design for controlled (GMP) manufacture and final verification of a fibrin-based biomaterial tissue regeneration scaffold

The problem our feasibility study project is trying to solve

The RAFT Institute has developed an artificial bio-material that has shown to significantly help in the healing of full thickness skin loss injuries. The problem facing the RAFT team is how to manufacture this material in such a way that ensures that the product is manufactured repeatably, and to the required standards to allow the product to move forward to the ultimate goal of human use.

What our study is aiming to achieve

We are aiming to develop an automated system that will repeatably and reliably manufacture the RAFT product. This system will be able to cleaned and installed in such a way that it meets all of the required standards for the manufacture of medical products.

The potential benefits

Benefits:- Patients: 2,400 full-thickness burn injury patients in the UK. 300 in-hospital mortalities from burns injury. Half of cases are children. 200,000 patients with chronic wounds, 350 deaths. £2-3 billion in care of chronic wounds. 100 amputation operations per week.

What we need to do next

The next stages of the project are as follows:

- enhance the existing prototype machine to optimise the performance so far
- extend the machine to include the final drying & packaging steps
- produce a batch of product and use this batch in a pre-clinical study.

We estimate this process would require a further £250k, and we are actively seeking funding for this.

Lead partner

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Development of a multi-centre trial and patient registry for a cartilage regeneration natural implant product

The problem our feasibility study project is trying to solve

TiGenix has developed an innovative scaffold implant product, Chondromimetic, for first line regenerative repair of cartilage damage. Since 2007, it has undergone extensive development and was recently awarded a CE Mark of Approval. To maximise its commercial potential, wider evidence on the product's clinical efficacy is required, for which a 200 patient pan-European Patient Registry is being established. The company is now addressing two critical development activities for the study, namely, completion of all regulatory and contractual requirements for engaging the clinical centres and ensuring continuity of supply of clinical grade material through the duration of the study.

What our study is aiming to achieve

The study is aiming to bring the Patient Registry to a state of full readiness by completing an agreed plan for execution of a multi-centre study involving orthopaedic surgeons in EU, a fully scoped Chondromimetic Cartilage Register defining the clinical information and data capture process and manufacture and supply of clinical grade material for supply to the different centres

The potential benefits

An earlier controlled but limited first-in-human trial involving 15 patients at a single centre has provided important information on the surgical technique, product safety and clinical outcome. The larger study will generate more conclusive evidence of the treatment's healthcare benefits and enable the product's commercial value to be realised.

What we need to do next

The next step will be to conclude the preparatory work ahead involving detailed planning with the clinical partners and production of an appropriate level of product stock to be followed by launch of the multi-centre Chondromimetic Registry.

Lead partner

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Process development and optimisation for large scale GMP manufacture capacity for the production of a clinical grade cartilage regeneration product

The problem our feasibility study project is trying to solve

An innovative biomaterial based scaffold for first line surgical intervention of cartilage damage has been developed to respond to the growing need for treatment of articular cartilage lesions. The manufacturing process involves a freeze drying step, critical in imparting the characteristics of the product for its role in regenerative repair. Full commercial potential is however limited due to the current scale of the manufacturing operations. The feasibility study will create a long-term solution to the manufacturing scale-up issue of this high value product to enable demand for clinical evaluation and commercial sales to be adequately met.

What our study is aiming to achieve

The study aims to develop an optimised and scalable freeze drying process that is the critical step in the manufacture of the novel biomaterial scaffold.

The potential benefits

The most significant benefit will be the ability of TiGenix to offer its regenerative scaffold product to a much wider market satisfying the growing demand by surgeons and patients for a cost effective treatment for cartilage damage and generate wider clinical evidence of a novel regenerative tissue repair product.

What we need to do next

The next step is to further develop the proof of concept to a fully validated manufacturing process.

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Tissue Regenix Group plc

Development of dCELL Meniscus

The problem our feasibility study project is trying to solve

The complex organisation of collagen in meniscal tissue means the structure is virtually impossible to replicate with synthetic materials, a problem that is overcome by using meniscus as the starting material to manufacture an implantable scaffold.

What our study is aiming to achieve

The dCELL® Meniscus is a device made from porcine meniscus which possesses the biomechanics and structure of human meniscus to assist in restoring normal function.

The potential benefits

Key benefits of the dCELL® Meniscus include that it is acellular and biocompatible and the dCELL® process results in a cell friendly scaffold which regenerates with the patient's own cells and remaining tissue.

What we need to do next

Development of the dCELL® Meniscus to secure regulatory approval for marketing.

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Implementation and certification of an ISO 13485:2003 quality system

The problem our feasibility study project is trying to solve

Without a CE mark the dCELL® Vascular Patch may not be sold in the EU and would be unacceptable to the health authorities of most countries. For the CE mark to be awarded, Tissue Regenix must achieve certification of its quality system to ISO 13485:2003 - the route chosen to meet the essential requirements of the Medical Device Directive - 93/42/EEC and its amendment 2007/47/EC.

What our study is aiming to achieve

This project will implement and achieve certification of Tissue Regenix's quality system to ISO 13485:2003 which will subsequently enable Tissue Regenix to be awarded a CE mark for the dCELL® Vascular Patch and place the product for sale on the European market in 2010.

The potential benefits

Award of a CE mark for the dCELL® Vascular Patch, Tissue Regenix's first product, would be a historic moment for Tissue Regenix. As well as providing access to some of the world's largest medical device markets, this achievement would validate the dCELL® technology platform.

What we need to do next

Tissue Regenix will now be simultaneously working towards receiving FDA approval for the US market.

Regenerative medicine collaborative R&D projects – value systems and business models

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Quy Biosciences, NHS Blood and Transplant,
NHS Technology Adoption Centre,
Pfizer, Tigenix, Loughborough University

Regenerative medicine value systems: navigating the uncertainties

The problem our feasibility study project is trying to solve

Regenerative medicines (RM) have the potential to cure chronic disorders and thereby revolutionise healthcare and address costs; they will become a high value manufacturing industry for the UK. However, RM companies have been unable to attract any significant level of investment, due to a lack of commercially successful trailblazers and uncertainties that impact the value system and business strategy and thus knowledge of how to manage investment risk.

What our study is aiming to achieve

This is a two year study executed by a 10 partner consortium (including BUPA, Cell Medica, Consulting in Advanced Biologics, Quy Biosciences, NHS Blood and Transplant, NHS Technology Adoption Centre, Pfizer, Tigenix and Loughborough University) which brings together the expertise across the value system required to clarify the four major uncertainties: regulatory, market, technological and finance strategies impacting development of the RM industry.

The potential benefits

The project will deliver the models, processes and guidance that enable a better understanding of where and how value will be created in the RM value chain, and to ensure businesses capture that value to the benefit of both the RM company and the UK.

What we need to do next

The consortium is committed to a wider communication of its generic understanding in order to grow the industry and its marketplace. The principles learned from the project will be disseminated to the RM industry and associated stakeholders including government, investors and insurers via briefing forums and White Papers during the project lifetime.

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NHS National Technology Adoption Centre

British regen industry tool set

The problem our feasibility study project is trying to solve

The ultimate goal of the BRITS project is to create and integrate detailed process economics models with higher level business models (inc. NHS interactions) in order to identify successful routes to market for cell-based regenerative medicine products and services. Outputs will include an integrated tool set with an appropriate graphical user-interface to allow all the stakeholders (manufacturers, researchers, clinicians, investors, healthcare buyers, regulators and policy makers) to better understand where and how the value of their potential cell-based therapy can be created and captured.

What our study is aiming to achieve

'Cells-as-therapies' represent a paradigm shift in patient care and requirements for manufacture, distribution, clinical deployment and reimbursement. These essential factors have never been adequately addressed, let alone in an integrated manner. This is THE fundamental challenge facing the commercial translation of cell-based therapies into therapies for use in routine clinical practice. In particular, investors are acutely aware that very little is known about how to create and capture value in regenerative medicine. Solve this dilemma with the appropriate evidence-based tool set and there is the real potential for the creation of a competitive and sustainable UK cell-therapy healthcare sector.

The potential benefits

Given the size of the unmet medical indications targeted by potential cell-based therapies, including neurodegeneration, cardiovascular disease and diabetes (three of the world's top four disease groups), it is not unrealistic to expect that if the commercial opportunity can be properly captured, that a multibillion pound UK industry could result.

What we need to do next

Additional resources will be sought to establish a BRITS spin-off consulting service at the end of the technology Strategy Board funding. This would pump-prime sustainability and continue to give expert advice and support to the UK stakeholders for the tools that are developed plus update and refinements to meet changing market conditions.

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A therapy realisation pathway tool (TRPT) applied to three representative regenerative medicine therapeutic products (REALISE)

The problem our feasibility study project is trying to solve

There is no guide to the translation of ideas in stem cell biology into commercially viable products. The project aims to provide such a template by uncovering the issues associated with developing these novel products and testing the processes on three products currently in development.

What our study is aiming to achieve

The provision of a guide and software tool which highlights the principal decision areas and provides solutions for problem solving across the value chain. The tool will be exemplified with three regenerative medicine therapeutic candidates.

The potential benefits

The streamlining of processes and investment based on an improved understanding of the value chain, increased business planning abilities with supporting processes and procedures to guide and inform product development which will increase the rate of success whilst shortening the timeframe.

What we need to do next

Continue information collection and validation and build the knowledge base to populate the tool. Test the tool functionality with the prototype and develop the commercialisation model across the regenerative medicine sector to include licensing, outsourcing and market development activities.

Disclaimer

The entries in this directory were provided by the individual companies. The Technology Strategy Board cannot guarantee the accuracy or completeness of any of the information about the winning projects.

The Technology Strategy Board is a business-led executive non-departmental public body, established by the Government. Its role is to promote and support research into, and development and exploitation of, technology and innovation for the benefit of UK business, in order to increase economic growth and improve quality of life.

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