Technology Strategy Board
Driving Innovation

Results of competition: Supporting regenerative medicines and cell therapies

Total available funding for this competition was £8m from the Technology Strategy Board.

Note: These proposals have succeeded in the assessment stage of this competition. All are subject to grant offer and conditions being met.

Participant organisation names	Project title	Proposed project costs	Proposed project grant
Asymptote Limited (Lead) University College London	Development of a regulatory compliant cassette for large volume cell culture, cryopreservation, thawing and perfusion	£1,198,497	£922,413

Project description (provided by applicants)

This application relates to the development of the consumables and associated equipment to allow the widespread clinical delivery of a bioartificial liver (BAL). Since the liver is one of the few organs that can repair and regenerate, therapies enabling regenerative medicine, that is creating living functional tissues to repair or replace organ function lost due to damage, are expected to play a role in several areas of liver disease.

A bioartificial liver machine can temporarily replace the functions of the liver, allowing the damaged liver to regenerate whilst protecting the patient's other organs from the life-threatening damage that ensues during liver failure. If the toxicity can be mitigated, within 24 to 48 hours, the majority of liver cells will enter DNA synthesis, closely followed by mitosis enabling the liver to regenerate, restoring full function within a few days.

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Participant organisation names	Project title	Proposed project costs	Proposed project grant
Automation Partnership (Lead) Tap Biosystems Ltd The University of Cambridge Wellcome Trust	Personalised iPS cell culture system	£796,666	£522,974
Project description (provided by applicants)			
We plan to develop a novel, automated cell culture system specifically designed for iPSC and autologous cell culture. Early stage iPS cell culture is very labour intensive since cells must be monitored and fed daily for many weeks. For clinical applications cells must be cultured in a GMP clean room environment, with high staff and infrastructure costs. Our system will automate routine feeding, monitoring and incubation of cells in a 'closed', low cost unit, with remote monitoring and operation. It will maintain multiple plates of cells from one patient, to ensure segregation, within a small footprint, and be GMP compliant. We aim to deliver more consistent, higher quality cells, to minimise the cost, resource and 'hands on' time needed for iPSC and autologous cell culture, and to address a major barrier to wider adoption of iPSC for pre-clinical and clinical studies. The partners, TAP Biosystems, The Wellcome Trust Sanger Institute and University of Cambridge together have outstanding track records in developing innovative automation for cell culture and world-class stem cell research, with a broad focus on developing and translating iPSC-based research into novel therapies.			

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Oxford Biomedica (UK) Ltd (Lead)	Development of a novel therapeutic treatment to engineer corneas to resist graft rejection	£2,985,443	£1,791,265
Project description (provided by applicants)			
Corneal transplantation is one of the most successful transplant procedures, due mainly to the relatively immune-privileged status of the eye and the fact that the cornea is largely free of blood vessels. However there is a failure rate in the first year of 14% and much higher in high rejection risk patients (such as those with failed previous grafts) due to blood vessel formation in the patient's eye, and the replacement of failed grafts is the indication for corneal transplantation for a significant proportion of patients in several referral centres. The prognosis in these patients can be so poor that many are not offered the opportunity of a further transplant and are left blind. EncorStat® is a novel engineered donor cornea, modified prior to transplantation, to extend rejection-free survival or prevent this risk entirely by suppressing blood vessel formation into the cornea post-transplant. We propose to use Technology Strategy Board funding to complete non-clinical safety studies and gain regulatory approval for clinical evaluation, to produce clinical grade vector and to use this to evaluate EncorStat® in a Phase I/IIa clinical trial.			

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Participant organisation names	Project title	Proposed project costs	Proposed project grant	Proposed other funders project grant
Regentec Limited (Lead)	A Targeted and Orchestrated Signalling Matrix for Clinically Challenging Defects	£2,321,508	£1,392,904	

Project description (provided by applicants)

Our rapid increase in the understanding of the biology underlying many bodily repair processes has led to new perspectives in the design and use of materials to address disease or injury. For restorative stem cells to fully participate in tissue regeneration they need to be packaged in a delivery material that provides an optimal environment for them to function.

This project will commercially develop a multi-functional tissue matrix that precisely locates an array of regenerative signals to service the requirements of tissue regeneration. The technology will be first directed towards bone repair applications, where earlier materials-based approaches are insufficient to heal large defects and in cases where more potent stimuli are required (e.g. for non-healing bone fractures and some spinal surgeries). The flexibility of the technology means that combinations of healing signals can be introduced into the matrix to make it more efficient and yield greater quantities of regenerated tissue. Further, a biologically complex, highly active product can be produced with a low-cost formulation due to the re-purposing of existing, market-approved constituents.

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Participant organisation names	Project title	Proposed project costs	Proposed project grant
ReNeuron Limited (Lead)	A Phase II Simon Two-Stage Efficacy Study of Intracerebral CTX-DP in Patients with Stable Paresis of the Arm Following an Ischaemic Stroke	£2,486,825	£1,492,095

Project description (provided by applicants)

Disability affects approximately 50% of stroke survivors due to permanent loss of brain tissue. ReNeuron is testing the effectiveness of a unique stem cell product, called CTX, in disabled stroke patients.

ReNeuron has developed CTX from human brain stem cells, with cell banks established to provide the large quantities needed for clinical use. The safety of CTX has been assessed in stroke-disabled patients in Scotland. Treatment of all patients in this Phase I clinical trial has been completed, with no reports to date of any serious side effects or worsening in disability in the patients.

Sustained reductions in neurological impairment and spasticity have also been observed in most patients compared with their stable pre-treatment baseline performance. Technology Strategy Board support is needed to conduct a Phase II study in up to 41 patients with upper limb paralysis at 2-4 months after their strokes. This proposed clinical trial will determine if CTX produces clinically meaningful improvements in the use of the paralysed arm at 6 months post-treatment in a sufficient number of patients treated. Success in this project will represent a significant milestone for ReNeuron and for the wider stroke field.

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Participant organisation names	Project title	Proposed project costs	Proposed project grant
Smith and Nephew UK Ltd (Lead)	BMAC (Bone Marrow Apsirate Concentrator)	£1,303,072	£325,768
Project description (provided by applicants)			
Iliac crest autograft, the gold standard therapy for bone defects, frequently causes long term morbidity at the site where the bone is harvested. Aspiration is an alternative way to collect bone marrow (BM) which preserves the bone structure, reducing procedure severity and patient suffering. However, BM aspirates (BMA) are often diluted with peripheral blood potentially reducing the stem cell concentration below therapeutic levels. This applied research project provides an opportunity to develop a stand-alone, portable BMA concentrator ("BMAC") based on innovative technology to remove contaminating blood from the BMA and concentrate the stem cells to levels above the therapeutic threshold for effective healing. Grant funding of £652K will be matched by internal funding to conduct the pivotal pre-clinical studies over 2 years. This novel transformational technology addresses a pressing healthcare need in terms of improved patient outcomes, and reduced health costs associated with the procedure and secondary interventions. The project will help promote a viable UK regenerative medicine therapy by fast-tracking the development of the BMAC device.			

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Videregen (Lead) University College London Cell Therapy Catapult Limited	Stem cell-based 3D tissue engineered organ for tracheal replacement	£2,429,246	£1,943,386
Project description (provided by applicants)			
This project seeks to create a niche, highly defensible, innovative regenerative medicine product with a global commercial potential of an estimated minimum £70M annually This is aimed at patients with a life-threatening disease which would otherwise be unattractive had the same team of experts not already tested a clinical prototype which has shown promise to be a one-off cure for severe structural airway diseases. This project brings together these experts with other partners within a consortium to progress this clinically successful prototype into a commercially ready stem cell-based tissue-engineered trachea for the treatment of adults and children with life-threatening severe structural airway disease. We will deliver a clinical trial providing robust quality data, validated manufacturing processes and economic assessment to support an accelerated path to market for commercial exploitation in this indication and will open up market potential for similar organ replacement products in larger disease indications.			