Results of Competition: Developing Regenerative Medicines & Cell Therapies

Competition Code: 1506_CRD2_HEALTH_CELL

Total available funding for this competition was £8M from Innovate UK

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
Touchlight Genetics Ltd	Next generation DNA constructs for the rapid and safe manufacture of AAV vectors for Regenerative Gene Therapy	£507,600	£318,106

Project description - provided by applicants

Gene therapy is becoming an increasingly important method of treatment for a variety of major unmet medicalneeds especially in the areas of inherited and rare diseases and diseases of the eye, conditions which are lifethreatening or significantly diminish quality of life. Adeno-associated virus (AAV) vectors are currently thedelivery vehicle of choice for gene therapy treatments but the advancement these treatments into clinical trialsis currently hampered by the time and expense required to manufacture these vectors. The proposed collaboration between Cobra and Touchlight will develop a fast and less expensive route tomanufacture of AAV vectors, which will enable the acceleration of more potential products into clinical testing. This in turn will increase the chances of treatment being developed for a whole range of these currently intractable diseases.

Note: you can see all Innovate UK-funded projects here

https://www.gov.uk/government/publications/innovate-uk-funded-projects Use the Competition Code given above to search for this competition's results

Results of Competition: Developing Regenerative Medicines & Cell Therapies

Competition Code: 1506_CRD2_HEALTH_CELL

Total available funding for this competition was £8M from Innovate UK

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
Control for 1 100000 minovation Eta	Development of an Industrial Manufacturing Platform for Adeno- Associated Virus (AAV) production to support Regenerative Gene Therapy products	£1,796,783	£1,403,616

Project description - provided by applicants

Gene therapy is becoming an increasingly important method of treatment for a variety of major unmet medicalneeds especially in the areas of inherited and rare diseases and diseases of the eye, conditions which are lifethreatening or significantly diminish quality of life. Adeno-associated virus (AAV) vectors are currently thedelivery vehicle of choice for gene therapy treatments but the advancement of these treatments into clinicaltrials is currently hampered by the lack of scalabilty needed to manufacture these vectors. The proposed collaboration between Cobra and CPI will develop the scientific understanding to allow scalableflexibile process to be developed to manufacture AAV vectors. This will enable the acceleration of morepotential products into clinical testing and ultimately new medicinces. This in turn will increase the chances oftreatment being developed for a whole range of these currently intractable diseases.

Results of Competition: Developing Regenerative Medicines & Cell Therapies

Competition Code: 1506_CRD2_HEALTH_CELL

Total available funding for this competition was £8M from Innovate UK

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
Synpromics Ltd Cell Therapy Catapult Ltd	Enhanced Vector Bioprocessing Technologies for Cell and Gene Therapies	£1,963,206	£1,577,724

Project description - provided by applicants

Evolving therapeutic approaches of cell and gene therapy are harnessing the power of viruses in order tomodify genomes of cells to produce a therapeutic effect. Such therapies are starting to show efficacy in theclinic, but one of the key challenges to their widespread use is the ability to make large quantities of virus at alow cost. This project seeks to address this challenge by creating new methods of producing large quantities of virus at a low cost. It brings together Synpromics, a synthetic biology company based in Edinburgh, and the CellTherapy Catapult, one of the UK's network of Catapult centres focused on developing and growing a cell andgene therapy industry in the UK.

Note: you can see all Innovate UK-funded projects here

https://www.gov.uk/government/publications/innovate-uk-funded-projects_Use the Competition Code given above to search for this competition's results

07 December 2015 3

Results of Competition: Developing Regenerative Medicines & Cell Therapies

Competition Code: 1506_CRD2_HEALTH_CELL

Total available funding for this competition was £8M from Innovate UK

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
ReNeuron Ltd	Stem cell-derived exosomes for	£2,498,924	£2,104,240
University College London (UCL)	regenerative medicine		
Cell Therapy Catapult Ltd			

Project description - provided by applicants

ReNeuron is a leading UK regenerative medicine company currently conducting clinical trials with a novelhuman neural stem cell (hNSC) product for the treatment of stroke and limb ischaemia. These stem cells alsoproduce small parcels of biologic material called exosomes. CTX-derived exosomes have been found to havefunctional properties in laboratory models of cancer, particularly glioblastomas. It may therefore be feasible touse the exosomes as an alternative class of regenerative medicine that has a long shelf-life and application to awide range of diseases. In this project, we will tackle how to purify exosomes in large enough quantities cost-effectively at scale and then complete preclinical studies for a clinical trial application in glioblastoma. Ifsuccessful, our project will benefit patients with the target disease as well as the UK regenerative medicine industry.

Note: you can see all Innovate UK-funded projects here

https://www.gov.uk/government/publications/innovate-uk-funded-projects_Use the Competition Code given above to search for this competition's results

Results of Competition: Developing Regenerative Medicines & Cell Therapies

Competition Code: 1506_CRD2_HEALTH_CELL

Total available funding for this competition was £8M from Innovate UK

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
Autolus Ltd	CAR Therapy for T Cell	£2,175,539	£1,682,111
University College London (UCL)	Malignancies		

Project description - provided by applicants

T-cells are immune cells in our bodies whose function is to "seek and destroy" cells which are infected byviruses. Since T-cells actively move around our bodies looking for infected cells, medical science has long triedto make these T-cells attack cancer cells. Because cancer cells usually come from normal cells in our bodieswithout virus infections, T-cells don't normally attack them. By taking T-cells from a blood sample and "reprogramming" them using genetic engineering, medical science has found a way of using T-cells to attackcancer. These engineered T-cells are called "CAR T-cells" and they can be given back to the patient as a drip.Once back in the patient, they "seek and destroy" cancer cells as if they were virus-infected cells. CAR T-cellsseem to be a very effective new form of cancer treatment. Cancers can come from any cells in our bodies. EvenT-cells can become cancer cells and some of these cancers are called T-cell lymphomas. This project deals withthe particular problem of CAR T-cells attacking T-cell lymphomas. Scientists working on this project have found away of making CAR T-cells that attack the cancerous T-cells but leave plenty of normal healthy T-cells alone.

Note: you can see all Innovate UK-funded projects here

https://www.gov.uk/government/publications/innovate-uk-funded-projects Use the Competition Code given above to search for this competition's results