Stem cell research opens door to new diabetes treatments

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Type 1 diabetes is a chronic insulin deficiency condition that is triggered by massive destruction of beta cells – pancreatic cells that normally produce insulin. This type of diabetes can be diagnosed at all ages but is the most frequent form when clinical onset occurs under the age of 40 years. While the administration of insulin can make up for shortages within the body, mimicking the tight hormonal control of a normal beta cell mass – and thus avoiding the risk of complications – is difficult to achieve.

The BETACELLTHERAPY programme has been developing and implementing new therapies to restore a functional beta cell mass in patients with this disease. This involves finding ways to replace lost beta cells and protect existing beta cells against disease. The programme was supported by an EU-funded FP7 project until June 2015.

'Significant progress has been made on the roadmap towards beta cell replacement in diabetes patients,' says project coordinator Daniel Pipeleers from the Free University of Brussels, Belgium. 'During the project's final 6-month extension, we completed preclinical assessments, comparisons of encapsulated cell therapy products and used human pancreatic beta cell preparations as references.'

This project built upon biology-driven tracks towards beta cell replacement, first in preclinical models and then in patients. The track to therapeutic beta cell implants sought to develop large-scale sources for biologically defined grafts and addressed the current shortage in metabolically adequate beta cell grafts prepared from human donor organs.

Human pluripotent stem cells – undifferentiated biological cells that can differentiate into specialised cells – were used to generate pancreatic precursor cells that can differentiate to insulin-producing cells, following transplantation in animal models. Human embryonic stem cell generated cells were pre-clinically validated as cell therapy product for clinical development and trials. Parallel studies defined markers for safety and efficacy, and examined ways for minimising immune and inflammatory reactivity in recipients.

Preparatory steps were also taken towards the submission of a clinical trial protocol for the transplantation of encapsulated human embryonic stem cell -derived progenitor cells.

The BETACELLTHERAPY consortium plans to continue the development of cell therapy products and protocols for beta cell replacement in diabetes, through further collaborations with the research and clinical departments of industrial partners. 'Our consortium included clinical reference centres and clinical diabetology units where novel forms of diagnosis and treatment are prepared, evaluated and implemented,' says Pipeleers. 'The results have already led to benefit for patients with Type 1 diabetes, but the duration and scale of implementation must now be increased.'

Taken together, the successful findings of the BETACELLTHERAPY study represent a significant step forward in the development of innovative new

approaches for treating diabetes. These have the potential to improve the quality of life of patients, and greater improve scientific understanding of how chronic diseases can be treated less invasively. 'Our annual reports received high scores from the project's scientific advisory board with an overall rating of excellent,' adds Pipeleers.

More information: For further information please visit BETACELLTHERAPY project website: www.betacelltherapy.org/

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