Personalized Stem Cell Therapies for Diseases of the Central Nervous System – the Translational March Forward

21.02.2017 – Cambridge Innovation Technologies Consulting (CITC) aims to establish a method to prepare neural stem cells in order to develop future therapies for patients with neurological illnesses and injuries. PROvendis has successfully negotiated a licensing contract on behalf of the University of Bonn.

In complex neurological diseases such as multiple sclerosis, stroke or spinal cord injuries, a treatment with stem cells may be the sole remaining choice of cure: stem cells are able to replace damaged cells or profoundly mitigate host immune responses – even in the central nervous system. Successful clinical studies are yet not existing, since researchers are facing a number of hurdles in stem cell therapies: the use of embryonic stem cells is ethically problematic; adult stem cells are either difficult to collect or hard to be expanded in a therapeutically sufficient amount. A gleam of hope was provided by the Nobel Prize-winning invention to reprogram adult cells of the body to pluripotent-like stem cells – this approach however came along with high risk of cancer development so far. Researchers of the University of Bonn have now modified this approach and made it safer. PROvendis mediated a contract for a non-exclusive license between the University and CITC in January 2017: The British company intends to use the technique of directly reprogramming neural stem cells for clinical research and application.

The invention to convert adult cells of the body into so-called induced pluripotent stem (iPS) cells by genetic engineering has opened new perspectives for stem cell therapy: those cells resemble naturally occurring stem cells, and they can subsequently be differentiated into various tissues, e.g. blood, liver, or nervous cells. Shinya Yamanaka and John Gurdon have been awarded the Nobel Prize for physiology and medicine in 2012 for this option of an ethically unproblematic generation of stem cells. The therapeutic breakthrough held off thus far, since this technique has the risk of aberrant development and uncontrolled proliferation of the induced pluripotent stem cells.

Scientists of the University of Bonn have modified the technique of the Nobel Prize-awardees in a way that brain stem cells equivalent to those present in the central nervous system can be generated with high efficiency and preventing the development of tumor cells. This is possible because, unlike the approach of Yamanaka and Gurdon, stem cells obtained with this new technique overleap a preliminary state of differentiation, so that the adult cells are directly transformed to neural stem cells, designated as induced neural stem cells (iNSCs). They are only transiently genetically engineered, so that cultivation and proper differentiation proceed with much better control.

However, before this technique can be applied in routine use, clinical studies are required. In the forthcoming years, CITC seeks to establish the new methods, test the efficacy of induced neural stem cells (iNSCs) and apply it clinically. This provides a strong incentive for future developments in regenerative medicine and for the treatment of an increasing number of patients with diseases of the nervous system.

Prof. Frank Oliver Stefan Edenhofer, inventor, says: “The induced neural stem cells (iNSC) technology carries tremendous potential for the study and cure of neurological disorders. After years of cumbersome basic research and successful patenting, now, we have to bring it from the lab bench to bed-side. Thus, we are happy to have with CITC a potent partner who will elaborate on the clinical realization of our induced neural stem cells (iNSC) technology.“

Dr. Luigi G. Occhipinti, CITC's Founder and Director of Engineering, says: "We are very happy of the collaboration with PROvendis and with Prof. Edenhofer in securing an opportunity for CITC to exploit this technology, which may allow us to establish a unique pathway towards future stem-cell based therapies of incurable neurological diseases, and to add this fundamental breakthrough to our portfolio of Intellectual Property in the fields of nanomedicine and advanced therapies."

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