## **RIKEN Center for Developmental Biology (CDB)**

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## Launch of new clinical research using allogeneic iPSCs

*February 17, 2017*–At a press conference held on February 6, RIKEN CDB's Masayo Takahashi, project leader of Laboratory for Retinal Regeneration, Kobe City Medical Center General Hospital's Yasuo Kurimoto, director of Department of Ophthalmology, and Osaka University's Kohji Nishida, professor of Department of Neural and Sensory Organ Surgery (Ophthalmology), Graduate School of Medicine/ Faculty of Medicine and director of Ophthalmology at Osaka University Hospital announced the launch of a new clinical research study aimed at establishing a new treatment for a retinal disease using allogeneic induced pluripotent cells (iPSCs). The new study will be led by Kobe City Medical Center General Hospital (Kobe General Hospital), and carried out in collaboration with Osaka University, Kyoto University's Center for iPS Cell Research and Application (CiRA), and RIKEN. RIKEN will be responsible for generating the retinal pigment epithelial (RPE) cells from iPSCs stored in CiRA's iPSC stock bank established for regenerative medical purposes, and the Kobe General Hospital and Osaka University Hospital will carry out the transplantation of RPE cells into patients. Their clinical research proposal has passed the all required reviews by the ethics committees of each organization as well as the relevant councils under the Japanese Ministry of Health, Labour and Welfare.



(From left) Yasuo Kurimoto, Masayo Takahashi, and Kohji Nishida at the press conference

Like the previous clinical research study launched by Takahashi and Kurimoto in 2013, the new study will continue to target exudative (wet-type) age-related macular degeneration (AMD), an eye disease that primarily affects the elderly causing loss of visual acuity. What is different in this clinical research study, is the use of an allogeneic (donor) iPSCs rather than autogeneic (patient-derived) iPSCs, as well as using cell suspensions as opposed to cell sheets. The team will be testing the safety of transplanting cell suspensions of RPE cells generated from iPSCs derived from a donor, and plans to carry out transplants in at least five patients over a two year period. Allogeneic transplantation is expected to significantly reduce the long timespan and high costs involved in manufacturing the cells for transplantation, two major issues associated with autogeneic transplantation that needed to be addressed when considering trying to make the treatment affordable and accessible to patients.

"While the main aim of this research study is to examine the safety of the intervention, at the same time, we plan to evaluate the efficacy of transplanting the allogeneic cells and consider the feasibility of using this method as a routine treatment in the future, accessible to the wider society," said Takahashi at the press conference. "We are just beginning to heighten our senses for this study as the schedule will move forward at a faster pace than the pilot safety study using autologous RPE cells sheets."

The team also plans to submit proposals for clinical research using allogeneic RPE cell sheets, autologous RPE cell suspensions and cell sheets in the near future, as potential alternative treatment methods for wet-type AMD, thereby expanding the patient spectrum.

Patient recruitment for the study began February 6 through Kobe General Hospital and Osaka University Hospital, but will be limited to residents of Japan.