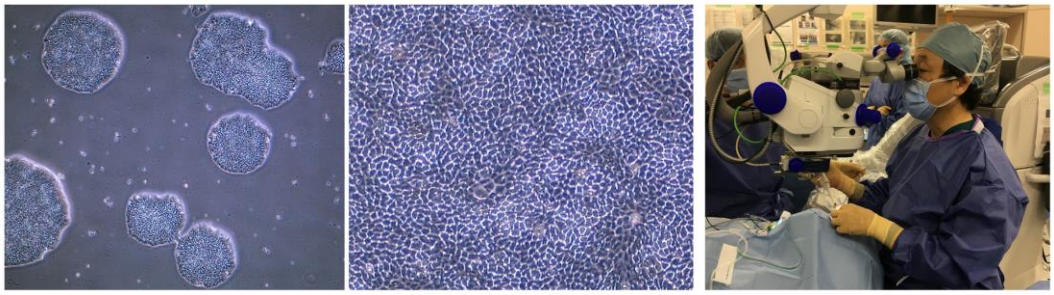
RIKEN Center for Developmental Biology (CDB)
2-2-3 Minatojima minamimachi, Chuo-ku, Kobe 650-0047, Japan

Donor iPSC-derived RPE cells transplanted into first AMD patient in new clinical study

April 4, 2017– On March 28, a man in his 60s diagnosed with wet-type age related macular degeneration (AMD) received an experimental transplant of retinal cells made from induced pluripotent stem cells (iPSCs) that were originally derived from a healthy donor. The patient was the first subject to undergo the procedure as part of the recently launched clinical study examining the safety and feasibility of transplanting donor (allogeneic) iPSC-derived retinal pigment epithelial (RPE) cells for wet-type AMD, which is led by Kobe City Medical Center General Hospital (hereafter Kobe General Hospital) and carried out in collaboration with Osaka University Hospital, RIKEN and Kyoto University's Center for iPSC Cell Research and Application (CiRA). At the press conference held on the same day, Yasuo Kurimoto, director of Kobe General Hospital's Department of Ophthalmology, and Masayo Takahashi, project leader of RIKEN CDB's Laboratory for Retinal Regeneration, reported that the surgery took about one hour as expected, with no major complications.



Donor iPSCs (left) used to make RPE cells (middle) that were transplanted in male patient. Kurimoto performed the surgery (right; credit: Kobe City Medical Center General Hospital).

The current clinical study uses iPSCs from CiRA's iPSC bank for regenerative medicine, which collected cells from healthy donors, instead of using patient-derived iPSCs. While using cells from donors carries the risk of triggering an immune reaction (rejection), the team will minimize this risk by closely matching the human leukocyte antigens (HLA) type—cell surface proteins which can trigger immune reactions—of donor cells with that of the patients. The use of donor iPSCs as the original cell source is expected to help markedly reduce costs and the time associated with preparing transplantable RPE cells, two major concerns with using a patient's own cells.

The surgical procedure involved transplanting RPE cells suspended in solution, in the narrow space between the retina and choroid layer of the eye. This method is different from the previous clinical study using autologous iPSCs, which included removal of abnormally formed blood vessels and damaged RPE followed by transplantation of an RPE cell sheet.

"I am relieved that the transplant surgery went smoothly," commented Kurimoto at the press conference. "We will continue to stay on our toes as this is an important first step in assessing whether or not using this new approach will be feasible as treatment in the future."

The collaborating team will continue to recruit for patients who meet the eligibility criteria for their study through Kobe General Hospital and Osaka University Hospital, with the aim of performing the procedure in at least five patients within two years. As HLA-type of iPSC line used for the study is more commonly found in the Japanese population, enrollment in the study will be limited to residents of Japan.

Contact: sciencenews@cdb.riken.jp Fax: +81-78-306-3090
RIKEN CDB, Office for Research Communications