Sumitomo Pharma

Press Release

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Sumitomo Pharma Co., Ltd.

First Transplantation in Phase 1/2 Study of Allogeneic iPS Cell-Derived Retinal Pigment Epithelial Cells

Sumitomo Pharma Co., Ltd. (Head Office: Osaka, Representative Director, President and CEO: Toru Kimura; "Sumitomo Pharma") announced today that the first patient transplantation in the Phase 1/2 study ("the clinical study") of allogeneic iPS cell-derived retinal pigment epithelial (RPE) cells (development code: HLCR011) for patients with RPE tear, being jointly developed in Japan with HEALIOS K.K. (Head Office: Chiyoda-ku, Tokyo, Chairman and CEO: Hardy TS Kagimoto; "Healios"), has been conducted at Kyushu University Hospital (Principal Investigator: Professor Koh-Hei Sonoda, M.D., Ph.D., Department of Ophthalmology). Moving forward, after a certain observation period without any safety concerns, the clinical study will proceed to its randomized phase (Part 2).

Sumitomo Pharma is promoting commercialization in the field of regenerative medicine and cell therapy by utilizing the unique techniques and know-how in this field. Sumitomo Pharma aims, in collaboration with Healios, to expedite patient enrollment and to confirm the safety and efficacy of HLCR001 in patients with RPE tear early in the clinical study, and to provide this new treatment option to the patients as soon as possible.

* Start of the clinical study was disclosed on June 23, 2023.
https://www.sumitomo-pharma.com/news/20230623.html

Reference

Outline of the clinical study

Test product	HLCR011: iPS cell-derived retinal pigment epithelial cells suspension
Development stage	Phase 1/2
Subjects	Patients with retinal pigment epithelium tear
Design for the clinical study (Target number of cases)	Multicenter, double-blind (active and sham), randomized study Part 1: Unmasked, uncontrolled (One HLA-mismatched subject) Part 2: Unmasked, randomized (treatment/observation groups, 10 subjects/group, total 20 subjects)
Primary endpoint	Safety of subretinal administration of HLCR011 in patients with retinal pigment epithelium tear (Number and ratio of subjects observed adverse events)
Secondary endpoint (Efficacy)	Visual function evaluation

About Retinal pigment epithelium (RPE) tear

RPE tear is a condition in which the RPE cell layer is torn, contracted, and partially defective due to AMD (age-related macular degeneration) or other causes. It causes visual field defects and vision loss, but currently no treatment for this condition has been established. If RPE cells are missing but photoreceptor function is preserved, RPE cell transplantation can be expected to maintain or restore visual function.

About Retinal pigment epithelial (RPE) cells

RPE cells form the retinal pigment epithelium outside the neural retinal layer. RPE is a single-layer structure that comes into contact with photoreceptors, and exert physiological functions to maintain and protect the functions of the photoreceptors. Dysfunction or deficiency of RPE cells induce diseases such as age-related macular degeneration and RPE tear, leading to impairment or decline in visual function. Therefore, therapies that transplant stem cell derived RPE cells have garnered attention in the field of regenerative medicine.

About iPS cells (induced pluripotent stem cells)

iPS cells, generated through artificial reprogramming of somatic cells by gene transfer, protein transfer, drug treatment, etc., or proliferation of these reprogrammed cells. iPS cells have the potential to differentiate into endodermal, mesodermal, and ectodermal cells, as well as the capacity for self-renewal. In this clinical study, RPE cells, induced to differentiate from iPS cell stocks (from QHJI donors) provided by the CiRA Foundation, are used.

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