Sumitomo Pharma

Press Release

June 23, 2023

Sumitomo Pharma Co., Ltd.

Start of 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: Hiroshi Nomura; hereinafter, "Sumitomo Pharma") announced today that, after the completion of the 30-day review by the Pharmaceuticals and Medical Devices Agency (PMDA) regarding the protocol, submitted to the PMDA on May 24, 2023, of the Phase 1/2 study (hereinafter referred to as "the clinical study") in patients with retinal pigment epithelium tear, the preparation of the clinical study of allogeneic iPS cell-derived retinal pigment epithelial (RPE) cells (development code: HLCR011), being jointly developed in Japan with HEALIOS K.K. (Head Office: Chiyoda-ku, Tokyo, Chairman and CEO: Hardy TS Kagimoto; hereinafter "Healios"), has been completed.

The clinical study is a multicenter, unmasked, randomized study. Sumitomo Pharma is now selecting clinical study sites. Subjects will be enrolled immediately after the completion of the preparation, including conclusion of contracts with the clinical study sites.

To utilize the techniques and know-how in the field of regenerative medicine and cell therapy for patients as soon as possible, Sumitomo Pharma will collaborate with Healios to confirm the safety and efficacy of HLCR001 in patients with retinal pigment epithelium tear early in the clinical study, while promoting its commercialization in this field.

[Outline of the clinical study]

Test product	HLCR011: iPS cell-derived retinal pigment epithelial (RPE) cells suspension
Development stage	Phase 1/2
Subjects	Patients with retinal pigment epithelium tear
Design for the clinical study (Target number of cases)	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

* A submission of this clinical study notification was disclosed on May 25, 2023.

https://www.sumitomo-pharma.com/news/20230525.html

Reference

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 age-related macular degeneration (AMD) 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 cells come into contact with photoreceptors, and exert physiological functions to maintain and protect the functions of the photoreceptors. Since RPE cells with a single-layer structure do not regenerate, visual functions will be permanently impaired if they are damaged. Therefore, RPE cells have recently attracted attention in the research of regenerative medicine for compensating for a loss or dysfunction due to age-related macular degeneration.

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 through division of somatic cells, can differentiate into endodermal, mesodermal, and ectodermal cells with a 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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