Sumitomo Pharma

Press Release

November 29, 2024

Sumitomo Pharma Co., Ltd.

Initiation of Phase1/2 Study on Allogeneic iPS Cell-derived Retinal Sheet for Retinitis Pigmentosa in the United States

Sumitomo Pharma Co., Ltd. (Head Office: Osaka, Japan; Representative Director, President and CEO: Toru Kimura) announced today the clearance of an Investigational New Drug (IND) Application by the U.S. Food and Drug Administration (FDA) for the Phase 1/2 study ("the clinical study") on allogeneic iPS cell-derived retinal sheet (3-dimensional [3D] retina, development code: DSP-3077) for the treatment of retinitis pigmentosa. The IND application, submitted on October 25, 2024, received FDA approval after a 30-day review, and preparations for initiating the clinical study are being finalized. Fresh non-frozen 3D tissue/organoid will be used in the clinical study.

To start the clinical study, Sumitomo Pharma has been conducting discussions with Massachusetts Eye and Ear in Boston, Massachusetts, USA (MEE) with the aim to begin transplantation in patients in fiscal 2025. In addition, the Company has already started a prospective, observational study (NCT06517940) at MEE to search for optimal ophthalmic endpoints for retinitis pigmentosa, planning to use the obtained data in the clinical study as well as future clinical development.

Preceding the clinical study, allogeneic iPS cell-derived retinal sheets manufactured and provided by Sumitomo Pharma have been transplanted to two patients for the first time in the world at Kobe City Eye Hospital in the clinical research, "Safety study using allogeneic iPSC-derived retinal sheets for patients with retinitis pigmentosa", which began in 2020. Kobe City Eye Hospital has published research confirming engraftment and safety of retinal sheets over a 2-year period after transplantation[*1]. A part of the clinical research was referred for planning of the clinical study. The clinical study being conducted by the Sumitomo Pharma is independent with Kobe City Eye Hospital.

The technology for the treatment is based on the self-organizing cell culture technique, named SFEBq method, an efficient differentiation method from pluripotent stem cells into 3D neural tissues originally developed by Dr. Yoshiki Sasai's research group at RIKEN. Sumitomo Chemical Co., Ltd. ("Sumitomo Chemical") had conducted joint research with RIKEN from 2010 to 2014 to improve the technology[*2]. Aiming at the commercial application, Sumitomo Pharma took over the joint research[*3] with RIKEN from 2013, and has established a manufacturing process to generate iPS cell-derived retinal sheet. Having completed the joint research, Sumitomo Pharma is independently conducting basic research for the aims of further improving the technology and expanding its application.

Sumitomo Pharma will conduct the clinical study with the objective of offering a new treatment option to retinitis pigmentosa patients as early as possible.

*1 https://www.cell.com/cell-stem-cell/fulltext/S1934-5909(23)00396-X

*2 http://www.cdb.riken.jp/en/news/2015/researches/0421_6395.htmlh
*3 https://www.cell.com/stem-cell-reports/fulltext/S2213-6711(18)30058-4

[Overview of the clinical study**]**

Test product	DSP-3077: allogeneic iPS cell-derived retinal sheet
Development stage	Phase 1/2
Target disease	Retinitis pigmentosa
Study design (Target numbers of subjects)	Unmasked, single-arm, dose-escalation study (12 subjects)
Primary endpoint	Safety and tolerability
Secondary endpoints	Engraftment, immune response, efficacy, etc.
Company conducting study	Sumitomo Pharma America, Inc. (Sumitomo Pharma U.S. subsidiary)

Reference

About Retinitis pigmentosa

Retinitis pigmentosa (RP) is a group of inherited retinal disease caused by the loss or dysfunction of light-responsive photoreceptors or retinal pigment epithelial cells. The causative genes are diverse, and there are individual differences in the disease progression. However, the common pathology includes progressive degeneration (cell death) of rod photoreceptors (cells responsible for scotopic vision) followed by progressive degeneration of cone photoreceptors (cells responsible for photopic vision, visual resolution, and color vision). After a long period of progression, RP often leads to severe visual impairment. RP is the second leading cause of blindness in Japan, therefore the development of new treatments for this serious condition is highly desired.

About iPS cells (induced pluripotent stem cells)

In 2006, Professor Shinya Yamanaka's research group at Kyoto University was the first in the world to generate induced pluripotent stem (iPS) cells, by introducing genes into somatic cells such as skin cells. iPS cells are artificially created stem cells and have the ability to differentiate into almost all cell types in the body except the placental cells, as well as the capacity for self-renewal and unlimited proliferation.

About iPS cell-derived retinal sheet

Human allogeneic iPS cells are differentiated into 3D retinal organoid (3D-retina) using SFEBq method, and then processing 3D-retina into the retinal sheet (DSP-3077) with multilayered retinal tissue structure. Retinal sheet includes abundant photoreceptor precursors.

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