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Sumitomo Pharma Co., Ltd.
Kyoto University Center for iPS Cell Research and Application
CiRA Foundation

Start of Investigator-Initiated Clinical Study of iPS Cell-Derived Dopaminergic Progenitor Cells for Parkinson's Disease in the United States

Sumitomo Pharma Co., Ltd. (Head Office: Osaka, Japan; Representative Director, President and CEO: Hiroshi Nomura, "Sumitomo Pharma"), Kyoto University Center for iPS Cell Research and Application (Headquarters: Kyoto; Director: Professor Jun Takahashi, "CiRA") and CiRA Foundation (Headquarters: Kyoto, President: Shinya Yamanaka, "CiRA_F") announced today the clearance of Investigational New Drug Application (IND) by the U.S. Food and Drug Administration (FDA) for an Investigator-initiated clinical study of iPS cell-derived dopaminergic progenitor cells for the treatment of Parkinson's disease ("this clinical study") to be conducted by the Sanford Stem Cell Institute CIRM Alpha Clinic at University of California San Diego School of Medicine ("UC San Diego"). The IND application, submitted in October 2023, received approval by the FDA after a 30-day review.

In Japan, Kyoto University Hospital has been conducting an investigator-initiated clinical study since 2018 to confirm the safety and efficacy of the therapy by a research group led by Professor Jun Takahashi of CiRA. The researchers and physicians involved in the clinical study have exchanged information with UC San Diego's personnel involved in this clinical study.

In this clinical study, as with the clinical study at Kyoto University Hospital, Sumitomo Pharma will produce and provide iPS cell-derived dopaminergic progenitor cells. These cells will be produced in Japan from QHJI donor-derived iPS cells provided by the iPS Cell Stock Project of CiRA_F. Sumitomo Pharma will also provide financial support for the conduct of this clinical study. The data from this clinical study will be used for further development of the therapy in the United States.

In order to make technologies and know-how in the regenerative medicine/cell therapy area available to patients worldwide as soon as possible, Sumitomo Pharma, CiRA and CiRA_F are collaborating to commercialize this novel therapy. In addition to this clinical study, Sumitomo Pharma is preparing to initiate a company-sponsored clinical study outside Japan.

[Overview of this clinical study]

Test product	CT1-DAP001
Development stage	Phase 1/2
Target disease	Parkinson's disease
Study design (Target number of patients)	Single center, open, non-placebo-controlled (Seven patients)

Primary endpoint	Safety: Frequency and severity of adverse events
Secondary endpoints (Efficacy)	Motor symptoms and others

(Reference)

Parkinson's disease

Parkinson's disease is a progressive neurodegenerative disease that is thought to be caused by a marked decrease in striatal dopamine levels due to neuronal degeneration and loss, which results in an imbalance in the function of the basal ganglia circuitry, which controls the motor function in the brain, leading to the development of motor symptoms.

The four most common motor symptoms of Parkinson's disease are tremor, muscle stiffness or rigidity, slow movements, and impaired postural reflexes. Motor symptoms often begin as tremor followed by slow movements and muscle rigidity occurring in an upper or lower limb on one side, gradually spreading to the other side. In more advanced cases, impaired postural reflexes or posture instability may occur. In addition to motor symptoms, which gradually progress from the limbs to the trunk, non-motor symptoms, including autonomic symptoms, psychiatric symptoms, and sleep disturbances, may occur.

Dopaminergic progenitor cells

Dopaminergic neurons produce the neurotransmitter dopamine. In Parkinson's disease, these cells progressively degenerate, resulting in decreased dopamine production. Dopaminergic progenitor cells are precursor cells in the final stage of differentiation into dopaminergic neurons. Studies in animal models of the disease have shown that transplanted dopaminergic progenitor cells are efficiently engrafted and differentiate into mature dopaminergic neurons in the brain.

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.

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