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## Press Release

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March 28, 2024

Sumitomo Pharma Co., Ltd.

### **Initiation of Company-sponsored Clinical Study on 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) announced today the clearance of an Investigational New Drug (IND) Application by the U.S. Food and Drug Administration (FDA) for a company-sponsored clinical study ("the clinical study") on iPS cell-derived dopaminergic progenitor cells for the treatment of Parkinson's disease.

The IND application, submitted in February 2024, received FDA approval after a 30-day review, and preparations for the clinical study are being finalized.

Cryopreserved cells will be used in the clinical study.

An investigator-initiated study for non-cryopreserved cells began at the University of California San Diego School of Medicine in November 2023, and in Japan, Kyoto University Hospital has conducted an investigator-initiated study for non-cryopreserved cells since 2018.

This is the first clinical study to be sponsored by Sumitomo Pharma in the U.S. using allogeneic iPS cell-derived differentiated cells in regenerative medicine/cell therapy field, a priority business area for Sumitomo Pharma, and is a major step toward developing a business in this area in the U.S.

#### **[Overview of the clinical study]**

Test cells	DSP-1083 Allogeneic iPS Cell-derived Dopaminergic Progenitor Cells (cryopreserved cells)
Development stage	Phase 1/2
Target disease	Parkinson's disease
Study design (Target numbers of subjects)	Multicenter, double-blind (active and sham), randomized study (dozens of subjects)
Primary endpoint	Safety: Frequency and severity of adverse events
Secondary endpoints (Efficacy)	Motor symptoms and others
Company conducting study	Sumitomo Pharma America, Inc. (Sumitomo Pharma U.S. subsidiary)

\*Details of investigator-initiated study in the U.S. were disclosed on December 26, 2023

<https://www.sumitomo-pharma.com/news/20231226.html>.

(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 are 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.

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