

Gene Therapy Product "GT0003X" for Parkinson's Disease Designated Under Japan's "Regenerative Medical Products with SAKIGAKE Designation System"

June 8, 2026
ONODERA GT Pharma, Inc.

ONODERA GT Pharma, Inc. (hereinafter "the Company") is pleased to announce that today, the Ministry of Health, Labour and Welfare (MHLW) has designated GT0003X, the Company's gene therapy product under development for Parkinson's disease, as a target product under the "Regenerative Medical Products with SAKIGAKE Designation System."

1. Overview of the Designation

- **Designated Product:** Gene Therapy Product "GT0003X"
- **Target Disease:** Parkinson's Disease
- **Designation Number:** (R8-SAKIGAKE-Sai) No. 5

2. About GT0003X

GT0003X is a groundbreaking gene therapy product that uses an adeno-associated virus (AAV) vector to directly deliver three genes encoding enzymes essential for dopamine synthesis—AADC, GCH, and TH—to specific regions of the brain. Conventional treatments have faced challenges including "on-off phenomena*1" caused by levodopa ingestion, "wearing-off phenomena*2" associated with long-term use, and side effects such as involuntary movements. By restoring the brain's capacity for sustained dopamine synthesis, GT0003X has the potential to resolve these challenges and achieve a fundamental improvement in quality of life (QOL) not only for patients but also for their families, while also reducing the burden on caregivers.

3. About Regenerative Medical Products with SAKIGAKE Designation System

This system is designed to prioritize consultation and review by the Pharmaceuticals and Medical Devices Agency (PMDA) for innovative pharmaceuticals and medical devices that are being developed in Japan ahead of the rest of the world and for which early availability is eagerly anticipated, with the aim of shortening the time to approval.

4. Outlook

We are deeply honored that our proprietary technology—which simultaneously delivers three enzyme genes to enable autonomous dopamine synthesis in the brain—has been officially recognized through designation under the Regenerative Medical Products with SAKIGAKE Designation System. We regard this designation as an acknowledgment of the innovative nature of this product and its potential as a new treatment option for Parkinson's disease.

With this designation, the Company will work in close collaboration with the MHLW and PMDA to make the fullest use of the priority consultation and priority review framework and will accelerate development toward a marketing approval application for GT0003X as a regenerative medical product under the conditional and time-limited approval system for regenerative medical products. We will continue to pursue development in order to meet the expectations of patients and their families who are eagerly awaiting the earliest possible availability of this therapy.

5. About ONODERA GT Pharma, Inc.

With the Purpose of "Enriching the Future through Gene Therapy Innovation," the Company is a biotechnology company developing innovative gene therapy products for intractable diseases. Based in the Tonomachi district of Kawasaki City (King Skyfront), an international strategic hub, the Company advances its pipeline using proprietary AAV vector technology with the goal of contributing to the future of healthcare worldwide.

【Company Overview】

- **Company Name:** ONODERA GT Pharma, Inc.
- **Address:** 25-22 Tonomachi 3-chome, Kawasaki-ku, Kawasaki City, Kanagawa Prefecture, Japan
- **Representative:** Yosuke Sakuma, President and Representative Director
- **Founded:** May 16, 2014
- **Business:** Research and Development of Gene Therapy Products
- **URL:** <https://onodera-gtp.com/>

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■ Notes

*1 On-off phenomenon: A phenomenon in which the patient's condition suddenly switches between the "on" state (in which Parkinson's disease symptoms are suppressed by the drug's effect) and the "off" state (in which the drug's effect is not apparent).

*2 Wearing-off phenomenon: A phenomenon in which, as a result of long-term medication use, the duration of the drug's effect gradually shortens.

■ Inquiries regarding this announcement

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