Veritas In Silico

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Announcement of Patent Grant for a Novel Nucleic Acid Therapeutic for Amyotrophic Lateral Sclerosis (ALS)

The Jikei University School of Medicine, Research Center for Medical Science, Division of Regenerative Medicine (Minato-ku, Tokyo; Professor Hirotaka James Okano), and Veritas In Silico Inc. (headquartered in Shinagawa-ku, Tokyo; Representative Director and CEO: Shingo Nakamura; hereinafter "VIS") have been conducting joint research (the "Joint Research") aimed at developing a novel treatment for amyotrophic lateral sclerosis ("ALS"). On January 19, 2026, The Jikei University School of Medicine and VIS announced the publication of a patent application covering nucleic acid therapeutic compounds arising from the Joint Research in a release entitled "The Jikei University School of Medicine and Veritas In Silico announce Patent Publication for Novel Nucleic Acid Therapeutic Targeting Amyotrophic Lateral Sclerosis (ALS)." VIS is now pleased to announce that, following examination, a patent covering one of these compounds has been granted in Japan.

- Patent Title: Nucleic Acid Therapeutics and Their Use
- Japanese Application Number: JP2025-575964
- International Application Number: PCT/JP2025/025230
- Gene name: TDP-43 MP13
- Target disease: Amyotrophic Lateral Sclerosis (ALS)
- Novelty: First-in-class (no existing approved drug)
- Modality: Nucleic acid drug
- Target population: Approximately 300,000 worldwide, approximately 10,000 in Japan
- Future Development Policy: Envisioning clinical development by partner companies collaborating on this new drug project

ALS is a disease in which motor neurons in the brain and spinal cord to control muscles become impaired. This results in a gradual loss of movement in the muscles of the limbs, throat, tongue, and those necessary for breathing. This disease generally progresses rapidly, and many cases result in death within two to five years after onset. While ALS most commonly affects individuals in their 40s and older, with the highest number of patients in their 60s and 70s, cases of younger onset have also been reported. At the time of the survey, the global patient count was approximately 300,000, with about 10,000 in Japan. The male-to-female ratio among patients is approximately 1.3 to 1.5 times higher for males than for females. In most cases, ALS is not inherited. However, hereditary onset within families has been observed in approximately 5% of all cases. Furthermore, cases of frontotemporal dementia (FTD), which frequently co-occurs with ALS, share genetic, clinical, and pathological commonalities with ALS. It is believed that these conditions form a disease spectrum with shared etiology and pathophysiology.

Numerous genes associated with ALS have been identified. Pathological abnormalities involving TDP-43[*1], an RNA/DNA-binding protein normally located in the cell nucleus, are found in most ALS cases. Recent research has demonstrated that TDP-43 plays an important role in ALS pathogenesis. Emerging evidence suggests that the loss of TDP-43 function in ALS may be associated not simply with the amount of TDP-43, but with qualitative changes, including an imbalance between normal TDP-43 and inhibitory TDP-43 variants[*2].

Based on the ongoing Joint Research, it has been assumed that reducing inhibitory TDP-43 variants expressed in the brain and spinal cord with nucleic acid drugs, thereby restoring normal TDP-43 function, could represent a novel therapeutic strategy for ALS.

● Outlines of the Patent Grant

Evidence suggesting that inhibitory TDP-43 variants may play a significant role in the onset of ALS has raised expectations for a therapeutic strategy that reduces such variants and thereby improves TDP-43 function. Findings related to this approach were published in a scientific research article in October 2025 (see the following section for details). In parallel with the publication of that research article, VIS used its AI-driven drug discovery platform, aibVIS, to design and optimize two nucleic acid drugs that knock down inhibitory TDP-43 variants. VIS subsequently acquired the rights to these two nucleic acid drugs from The Jikei University School of Medicine and became the patent applicant. Examination of the patent application for one of these two compounds has resulted in a patent grant in Japan.

● Research article Related to the Joint Research

Title of Journal｜Journal of Cell Biology

Title of the Joint Research Article｜Dominant-negative isoform of TDP-43 is regulated by ALS-linked RNA-binding proteins

Author｜Minami Hasegawa-Ogawa, Asako Onda-Ohto, Takumasa Nakajo, Arisa Funabashi, Ayane Ohya, Ryota Yazaki, and Hirotaka James Okano

●Roles and Responsibilities in the Joint Research

ALS is a serious disease affecting a substantial number of patients, for which no curative treatment has yet been established. Developing an effective treatment has long been an important challenge in medicine.

The Jikei University School of Medicine has a long-standing commitment to ALS research and has accumulated extensive knowledge of disease mechanisms and causative genes, particularly through medical studies involving human induced pluripotent stem (iPS) cell models and pathological tissue sections. Since 2017, VIS has collaborated with The Jikei University School of Medicine to contribute to ALS treatment development, undertaking the design of nucleic acid therapeutics targeting genes believed to be involved in the disease.

VIS, as a biotechnology company, has supported the research from an early stage. This collaborative approach brings together academia, which seeks to elucidate disease mechanisms and discover therapeutic agents, and biotechnology companies, which aim to improve and optimize the properties of drug candidates. Such synergy is expected to facilitate efficient and effective drug discovery.

Under the Joint Research, The Jikei University School of Medicine is responsible for evaluating drug efficacy using the relevant assay systems and validating the therapeutic concept using human iPS cells or the relevant animal models. VIS is responsible for designing and synthesizing candidate therapeutics for proof-of-concept studies and conducting in vitro evaluations[*3].

● Comments from Hirotaka James OKANO M.D., PhD., Division of Regenerative Medicine, Research Center for Medical Science, The Jikei University School of Medicine

ALS is an intractable disease for which no curative treatment has yet been established. We are very pleased that the nucleic acid therapeutic developed on the basis of our research into disease mechanisms has now been granted patent protection. This is an important step toward realizing a new treatment. We will continue our joint research with Veritas In Silico in the hope of bringing new treatment possibilities to patients living with ALS.

● Comments from Jun SASAKI, PhD., Executive Officer and General Manager of Research Strategy Division of VIS

This Joint Research has involved a distinctive approach in which VIS has pursued nucleic acid therapeutic research in parallel with advanced academic research. Building on this patent grant, we intend to continue our research in close collaboration with the researchers at The Jikei University School of Medicine, while exploring partnerships with companies that could support further development. Our goal is to advance the work toward future clinical development and, ultimately, make a new treatment available to patients.

● Impact on Financial Position and Future Business Performance of VIS

The Jikei University School of Medicine and VIS each bear their own research expenses under the Joint Research, and no transfer of funds or other consideration between the parties is currently planned. The costs of the Joint Research have already been incorporated into VIS's

budget for the fiscal year ending December 31, 2026. Accordingly, there is no change to the earnings forecast for that fiscal year announced on November 11, 2026.

VIS owns the rights to the patent granted in Japan. We expect that further progress in research, development and commercialization will contribute to enhancing our corporate value over the medium to long term.

In case any matters requiring disclosure arise in the future, they will be promptly disclosed.

● Glossary for Reference

*1 TDP-43: TAR DNA-binding protein 43; normally present in the cell nucleus as an RNA/DNA-binding protein, it becomes abnormal in neurodegenerative diseases such as amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD).

*2 Variant: A form of a protein that differs somewhat in amino acid sequence or structure from other forms produced from a single gene or gene family

*3 In vitro evaluation: An evaluation method conducted in an artificial environment, such as a culture vessel or test tube, using cells or tissues derived from living organisms. It can be used as an alternative to in vivo animal studies, including for investigating mechanisms of action and screening drug candidates efficiently and at lower cost.

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For Further Information, Contact:

● Veritas In Silico Website Inquiry Form : https://www.veritasinsilico.com/en/contact/