

NEWS RELEASE



NIPPON SHINYAKU CO., LTD.

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SAKIGAKE Pharmaceutical Product (Pioneering Drug) Designation Granted to NS-035 for the Treatment of Fukuyama Congenital Muscular Dystrophy

KYOTO, Japan, August 26, 2026 – Nippon Shinyaku Co., Ltd. (Nippon Shinyaku; Headquarters: Kyoto; President: Toru Nakai) announced that the Minister of Health, Labor and Welfare (MHLW) has granted SAKIGAKE Pharmaceutical Product (Pioneering Drug) Designation to NS-035 for the treatment of Fukuyama congenital muscular dystrophy (FCMD).

SAKIGAKE System aims to facilitate earlier patient access in Japan to innovative medical products for serious diseases. A drug is eligible for designation if it is determined to meet all four of the following criteria: (1) innovativeness of the therapeutic agent, (2) severity of the target disease, (3) extremely high efficacy for the target disease, and (4) the intention and capability to develop and file for approval in Japan ahead of the rest of the world. Designated products are eligible for various support measures, including a shortened review period.

FCMD is a congenital muscular dystrophy that is particularly prevalent in the Japanese population. It is a genetic disorder characterized by degeneration and necrosis of muscle fibers, leading to progressive muscle weakness, and as the disease progresses, only a limited number of patients retain the ability to walk. Most patients lose motor function during childhood, and many become bedridden at around 10 years of age. Patients often develop aspiration pneumonia or respiratory failure, and the prognosis is poor. Current managements are primarily supportive, including rehabilitation, and symptomatic treatment. As no disease-modifying treatments have been approved either in Japan or globally, there remains a significant unmet medical need for new therapeutic options.

NS-035 is a novel antisense oligonucleotide designed to address abnormal protein production caused by mutations in the fukutin gene, the causative gene of FCMD. By promoting the production of functional fukutin protein, NS-035 is expected to suppress disease progression and improve muscle function.

Nippon Shinyaku has been working actively with a sense of mission to develop agents for the treatment of intractable and rare diseases, with a view to launching products for FCMD patients as soon as possible.

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