

June 23, 2026

Orphan Drug Designation granted to NS-035 for the Treatment of Fukuyama congenital muscular dystrophy in Japan

KYOTO, Japan, June 23, 2026 - Nippon Shinyaku Co., Ltd. (Headquarters: Kyoto, Japan; President: Toru Nakai) announced that the Ministry of Health, Labor and Welfare (MHLW) has granted Orphan Drug Designation to NS-035 for the treatment of Fukuyama congenital muscular dystrophy (FCMD).

Under Japan's Orphan Drug Designation system, drug candidates are reviewed and designated if they target fewer than 50,000 patients domestically and address serious diseases with high medical needs. This designation provides various incentives to support development.

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 only a limited number of patients are able to walk. Most patients lose motor function during childhood, and many become bedridden around 10 years. 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.

Contact

Public Relations & Investor Relations Dept., Nippon Shinyaku Co., Ltd.

[e_mail kouhou@po.nippon-shinyaku.co.jp](mailto:kouhou@po.nippon-shinyaku.co.jp)