News Release

Chugai Receives Regulatory Approval for “ELEVIDYS” as a Gene Therapy Product for Duchenne Muscular Dystrophy in Japan

- Duchenne muscular dystrophy is a rare genetic muscle disorder with poor life prognosis, which is difficult to cure and significantly reduces independent living and quality of life due to muscle weakness
- Approval based on results from clinical studies including a global Phase III clinical study (EMBARK)
- Approval for ambulatory patients with DMD from 3 years to less than 8 years of age who are negative for anti-AAVrh74 antibodies

TOKYO, May 13, 2025 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it obtained regulatory approval from the Ministry of Health, Labour and Welfare (MHLW) for ELEVIDYS® Intravenous Infusion [generic name: delandistrogene moxeparvovec] (hereinafter, "ELEVIDYS"), as a regenerative medicine product for the treatment of Duchenne muscular dystrophy (DMD) under the conditional and time-limited approval pathway in Japan. The approved patient population are ambulatory patients with DMD who do not have a deletion of any portion or the entirety of exon 8 and/or exon 9 in the DMD gene, are negative for anti-AAVrh74 antibodies, and are 3 years to less than 8 years of age. For the confirmation of anti-AAVrh74 antibody negativity prior to administration, the Elecsys anti-AAVrh74 assay can be used. Roche Diagnostics K.K. has obtained a regulatory approval for this assay in Japan on March 5, 2025, as a companion diagnostic to aid in determining eligibility for ELEVIDYS treatment in DMD patients.

“We are very pleased that ELEVIDYS has been approved as a gene therapy product for DMD, this rare and intractable disease. ELEVIDYS is a one-time foundational treatment designed to address the absence of dystrophin function, the cause of DMD. While treatment options for DMD remain limited, we will proceed with preparations for the launch to deliver ELEVIDYS to patients in Japan as soon as possible,” said Dr. Osamu Okuda, Chugai’s President and CEO.

This approval is based on the results from clinical studies in this product including a global Phase III clinical study (EMBARK) that evaluated the efficacy and safety of ELEVIDYS for up to 2 years[1,2] in ambulatory boys with DMD aged 4 to 7 years. Based on the results of the EMBARK study, while the primary endpoint of motor function assessed by the North Star Ambulatory Assessment (NSAA) did not show statistical significance

CHUGAI PHARMACEUTICAL CO., LTD. Corporate Communications Dept.
1-1 Nihonbashi-Muromachi 2-chome, Chuo-ku, Tokyo 103-8324 Japan
Media Relations Group TEL: +81-(0)3-3273-0881 E-mail: pr@chugai-pharm.co.jp
Investor Relations Group TEL: +81-(0)3-3273-0554 E-mail: ir@chugai-pharm.co.jp

compared to placebo, clinically meaningful improvements were observed in key secondary endpoints (time to rise from the floor, 10-meter walk time, next to stride velocity 95th centile [SV95C] and time to ascend 4-steps). [1]

Approval Information

Product Name: ELEVIDYS® Intravenous Infusion

Generic Name: delandistrogene moxeparvovec

Contraindication and Prohibition (partial excerpt):

Patients do not have a deletion of any portion or the entirety of exon 8 and/or exon 9 in the dystrophin gene

Efficacy or Effects:

Duchenne muscular dystrophy

Indicated for patients who meet all of the following criteria:

- Patients who are negative for anti-AAVrh74 antibodies
- Ambulatory patients
- Patients aged 3 years or older and younger than 8 years

Dosage and Administration:

For patients weighing 10 kg or more and less than 70 kg, the usual dose is 1.33×10^{14} vector genomes (vg)/kg administered as a single intravenous infusion over 60 to 120 minutes. For patients weighing 70 kg or more, the usual dose is 9.31×10^{15} vg administered as a single intravenous infusion over 60 to 120 minutes. This product should not be readministered.

(Table of dosage by body weight is omitted)

Approval Conditions and Time Limit:

[Approval Conditions]

- During the period until the reapplication for marketing approval of this product after conditional and time-limited approval, post-marketing approval condition evaluation shall be conducted through clinical trials aimed at confirming the long-term efficacy and safety of this product, as well as post-marketing surveillance targeting all cases in which this product is used.
- Necessary measures shall be taken, including dissemination of proper use guidelines developed in cooperation with relevant academic societies, to ensure that physicians with sufficient knowledge and experience in Duchenne muscular dystrophy use this product in accordance with the "Efficacy or Effects" and "Dosage and Administration" after thoroughly acquiring knowledge of the clinical trial results and adverse events of this product, at medical institutions with established systems for treating Duchenne muscular dystrophy.
- Necessary measures shall be taken, including dissemination of the usage regulations, to ensure that this product is used in compliance with the Type 1 Use

regulations approved under the "Act on the Conservation and Sustainable Use of Biological Diversity through Regulations on the Use of Living Modified Organisms (Act No. 97 of 2003)."

[Time Limit]

3 years

[Reference]

Chugai Files Elevidys (SRP-9001) as a Gene Therapy Product for Duchenne Muscular Dystrophy in Japan (Press release by Chugai issued on August 14, 2024)
https://www.chugai-pharm.co.jp/english/news/detail/20240814150000_1091.html

Roche Announces Outcomes for EMBARK Study of Elevidys in Duchenne Muscular Dystrophy (Press release by Chugai issued on October 31, 2023)
https://www.chugai-pharm.co.jp/english/news/detail/20231031083000_1022.html

About Duchenne muscular dystrophy (DMD)

DMD is a rare, genetic, muscle-wasting disease that progresses rapidly from early childhood. Approximately one in 5,000 boys worldwide are born with DMD, while DMD in girls is very rare.[4] Everyone who has DMD will lose walking ability, upper limb, lung and cardiac function,[4-6] and leads to fatal outcomes. A diagnosis of DMD will require full-time caregiving which is most often provided by parents,[4-6] the majority of whom will find it difficult to carry out usual work or household activities and suffer from depression, physical pain and discomfort.

DMD is caused by mutations of the DMD gene, which affects the production of the muscle protein, dystrophin. Dystrophin is a critical component of a protein complex that strengthens muscle fibers and protects them from injury during muscle contraction. Due to a genetic mutation in the DMD gene, people with DMD do not make functional dystrophin; their muscle cells are more sensitive to injury and muscle tissue is progressively replaced with scar tissue and fat.[5,6]

About ELEVIDYS

ELEVIDYS is the gene therapy product for Duchenne muscular dystrophy (DMD) and is designed to address the underlying cause of Duchenne through targeted skeletal, respiratory and cardiac muscle expression of shortened dystrophin produced by delandistrogene moxeparvovec. Delandistrogene moxeparvovec received an orphan regenerative medical product designation for DMD in Japan. It received approval as the

first gene therapy product for DMD in the US in June 2023 and was filed in EU in May 2024.

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Sources

1. Roche announces new results from EMBARK demonstrating significant sustained benefits of Elevidys in ambulatory individuals with Duchenne muscular dystrophy (DMD). Available from: https://www.roche.com/media/releases/med-cor-2025-01-27
2. [Ad hoc announcement pursuant to Art. 53 LR] Roche announces EMBARK trial in Duchenne muscular dystrophy (DMD) did not reach primary endpoint, but shows positive efficacy outcomes on all timed functional key endpoints. Available from: https://www.roche.com/media/releases/med-cor-2023-10-30
3. A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants With Duchenne Muscular Dystrophy (DMD) (EMBARK). Available from: https://clinicaltrials.gov/study/NCT05096221?intr=delandistrogene%20moxeparvovec&rank=7(Accessed May, 2025)
4. Salvatore Crisafulli et al, Global epidemiology of Duchenne muscular dystrophy: an updated systematic review and meta-analysis. Orphanet J Rare Dis. 2020 Jun 5;15(1):141
5. David J Birnkrant et al, Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018 Mar;17(3):251-267
6. The Japan Neurosurgical Society, General Incorporated Association. Clinical Practice Guidelines for Duchenne muscular dystrophy. https://neurology-jp.org/guidelinem/dmd.html (Accessed May, 2025) (Japanese only)

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