News Release

Chugai Launches “ELEVIDYS” as Japan's First Regenerative Medical Product for Duchenne Muscular Dystrophy

- Launched as Japan’s first regenerative medical product for Duchenne muscular dystrophy (DMD), a rare, genetic and difficult-to-treat muscle-wasting disease
- Eligible patients are ambulatory patients with DMD aged 3 years to less than 8 years who are negative for anti-AAVrh74 antibodies
- ELEVIDYS is a one-time therapy designed to address the absence of dystrophin function, the underlying cause of DMD, before irreversible muscle loss

TOKYO, February 20, 2026 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it launched ELEVIDYS® Intravenous Infusion [generic name: delandistrogene moxeparvovec] (hereinafter, "ELEVIDYS"), which received conditional and time-limited approval on May 13, 2025, following its listing on the national health insurance (NHI) reimbursement price list today, as a regenerative medical product for the treatment of Duchenne muscular dystrophy (DMD) in Japan. Eligible patients for treatment 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.

“DMD is a progressive disease that begins in early childhood and significantly impacts daily life due to gradual muscle weakness. We are very pleased to be able to deliver ELEVIDYS to patients diagnosed with DMD and their families who have been eagerly awaiting new treatment options. Putting patient safety as our highest priority, we will work to drive proper use of Elevidys. We will also conduct post-marketing clinical studies and all-case post-marketing surveillance to confirm the long-term efficacy and safety of ELEVIDYS,” 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 at 52 week 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). In addition, two fatal cases of acute liver failure in non-ambulatory DMD patients treated with ELEVIDYS have been

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reported overseas. In response to these reports, we have reinforced safety measures by revising the electronic package insert and educational materials for healthcare professionals and patients, with patient safety as our top priority. Specifically, we have implemented the following measures:

• Establishing a framework for promoting appropriate use through industry-government-academia collaboration

• Developing and publishing materials for healthcare professionals and patients (Appropriate Use Guide, Patient Handbook)

• Establishing a specialist consultation framework: following facility certification by the Japanese Society of Child Neurology, internet-based consultation through an expert panel (BRIDGE-NMD*) comprised of specialists will be available after domestic market launch

*BRIDGE-NMD (Building Recommendations in Developing Gene Therapy for Neuromuscular Disorders) is a multidisciplinary expert team comprising physicians in pediatric neurology, pediatric hepatology, pediatric hepatobiliary surgery, pediatric cardiology, pediatric immunology, pediatric nephrology, and pediatric hematology.

For the detection of anti-AAVrh74 antibody negativity prior to administration, use the Elecsys anti-AAVrh74 assay. Roche Diagnostics K.K. has launched this assay in Japan following its listing on the NHI reimbursement price list on February 1, 2026, as a companion diagnostic to aid in determining eligibility for ELEVIDYS treatment in DMD.

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

Date of approval: May 13, 2025
Date of NHI reimbursement price listing: February 20, 2026
Date of launch: February 20, 2026
Drug price: JPY 304,972,042 per patient
The NHI price is determined based on official rules following discussions at the Central Social Insurance Medical Council.

[Reference]

Chugai Receives Regulatory Approval for “ELEVIDYS” as a Gene Therapy Product for Duchenne Muscular Dystrophy in Japan (News release by Chugai issued on May 13, 2025)
https://www.chugai-pharm.co.jp/english/news/detail/20250513181500_1160.html

Roche Announces New Safety Information of Elevidys for Non-Ambulatory Duchenne Muscular Dystrophy (News release by Chugai issued on June 16, 2025)
https://www.chugai-pharm.co.jp/english/news/detail/20250616153000_1164.html

Implementation of Safety Measures for Elevidys (News release by Chugai issued on September 4, 2025)
https://www.chugai-pharm.co.jp/english/news/detail/20250904183000_1178.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,[3-5] and leads to fatal outcomes. A diagnosis of DMD will require full-time caregiving which is most often provided by parents,[3-5] 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.[4,5]

About ELEVIDYS

ELEVIDYS is the regenerative medical 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. ELEVIDYS was originated by Sarepta Therapeutics and has been co-developed by Sarepta and Roche. Chugai in-licensed ELEVIDYS from Roche and, as the marketing authorization holder in Japan, holds the exclusive marketing rights for ELEVIDYS in Japan.

In the US, ELEVIDYS was approved in June 2023 as the first gene therapy product for DMD. After two fatal cases of acute liver failure in non-ambulatory DMD patients treated with ELEVIDYS were reported, the indication for non-ambulatory patients has been removed. In Europe, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) recommended against granting conditional marketing approval, and the European Commission (EC) endorsed the recommendation in

September 2025. ELEVIDYS is currently approved for ambulatory DMD patients in nine countries around the world.

About EMBARK study

EMBARK is a multinational, Phase 3, randomised, double-blind, two-part crossover, placebo-controlled study assessing the safety and efficacy of Elevidys in ambulatory boys with a confirmed mutation in the *DMD gene*, aged between 4 and 7 years. Eligible participants received a single dose of Elevidys during either Part 1 or Part 2 of the study. The study is ongoing. We now have 3 year follow-up data for Part1, and patients will continue to be followed.[6]

Participants (n=125) received $1.33x10^{14}$ vg/kg of delandistrogene moxeparvovec and placebo. In Part 1, participants were randomised according to age (4-5 or 6-7) or NSAA total score at screening (≤22 or >22) to receive either Elevidys or placebo, with a follow-up period for 52 weeks. In Part 2, participants crossed over - meaning, those who were previously treated with placebo in Part 1 received Elevidys and participants who were previously treated with placebo received Elevidys, with a follow-up period for 52 weeks. The primary endpoint of the trial was change from baseline in NSAA total score at week 52.

Sources

1. AAV gene therapy for Duchenne muscular dystrophy: the EMBARK phase 3 randomized trial. Nature Medicine. 2025 Jan Volume31 332-341
2. Two-Year Outcomes Following Delandistrogene Moxeparvovec Treatment in Ambulatory Patients with Duchenne Muscular Dystrophy: Phase 3 EMBARK Trial. Neurol Ther.2026 Jan 10. Doi:10.1007/s40120-025-00879-8
3. 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
4. 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
5. The Japan Neurosurgical Society, General Incorporated Association. Clinical Practice Guidelines for Duchenne muscular dystrophy. https://neurology-jp.org/guidelinem/dmd.html (Accessed February, 2026) (Japanese only)
6. Sarepta Therapeutics to Report 3-Year Topline Data from EMBARK Study of ELEVIDYS Gene Therapy in Ambulatory Individuals with Duchenne Muscular Dystrophy.

Available from: https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-report-3-year-topline-data-embark-study (Accessed February, 2026)

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