News Release

Implementation of Safety Measures for Elevidys

TOKYO, September 4, 2025 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it has implemented measures based on safety evaluations for ELEVIDYS® Intravenous Infusion [generic name: delandistrogene moxeparvovec] (hereinafter, "ELEVIDYS"), a regenerative medical product for Duchenne muscular dystrophy (DMD) discovered by Sarepta Therapeutics and in-licensed from Roche.

In Japan, ELEVIDYS obtained regulatory approval from the Ministry of Health, Labour and Welfare (MHLW) under the conditional and time-limited approval pathway for ambulatory DMD patients aged 3 to under 8 years on May 13, 2025. Following two reports of acute liver failure with fatal outcomes in non-ambulatory DMD patients who were treated with ELEVIDYS overseas, we have been in discussion with MHLW and the Pharmaceuticals and Medical Devices Agency (PMDA) regarding safety measures for this product.

Based on our evaluation of the currently available safety information, we have agreed to add acute liver failure as a serious adverse reaction, implement tests for early detection of liver dysfunction and acute liver failure during pre- and post-administration monitoring, and additionally added precautions regarding infections associated with corticosteroid administration. We implemented these revisions to the electronic package insert on August 28, 2025. We will continue to work closely as we provide this information to healthcare professionals and those considering this treatment in the future. With patient safety as our highest priority, we will continue to collect and evaluate safety information and implement additional safety measures as needed.

DMD is a progressive disease with limited treatment options. We are committed to delivering new treatment options as soon as possible to meet the urgent needs of patients with DMD and their families. We will proceed with appropriate discussions with the MHLW regarding National Health Insurance coverage and subsequent product launch.

[Reference]
Roche Announces New Safety Information of Elevidys for Non-Ambulatory Duchenne Muscular Dystrophy
https://www.chugai-pharm.co.jp/english/news/detail/20250616153000_1164.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

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girls is very rare.[1] Everyone who has DMD will lose walking ability, upper limb, lung and cardiac function,[1-3] and will lead to fatal outcomes. A diagnosis of DMD will require full-time caregiving which is most often provided by parents,[1-3] 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 muscle cells 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.[2,3]

About ELEVIDYS

ELEVIDYS is an adeno-associated virus (AAV) based gene therapy for Duchenne muscular dystrophy (DMD) and is designed to address the underlying cause of DMD by expressing a shortened form of dystrophin in targeted skeletal, respiratory and cardiac muscles. ELEVIDYS received an orphan regenerative medical product designation for DMD in Japan. It received approval as the first gene therapy for DMD in the US in June 2023, and is currently approved in 9 countries.

Sources

1. 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
2. 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
3. The Japan Neurosurgical Society, General Incorporated Association. Clinical Practice Guidelines for Duchenne muscular dystrophy. https://neurology-jp.org/guidelinem/dmd.html (Accessed September, 2025) (Japanese only)

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