独立行政法人 医薬品医療機器総合機構
Pharmaceuticals and Medical Devices Agency

This English version is intended to be a reference material for the convenience of users. In the event of inconsistency between the Japanese original and this English translation, the former shall prevail.

Summary of Investigation Results

Delandistrogene moxeparvovec

August 28, 2025

Non-proprietary name

Delandistrogene moxeparvovec

Brand name (marketing authorization holder)

Elevidys for Intravenous Infusion (Chugai Pharmaceutical Co., Ltd.)

Japanese market launch

Before market launch

Indications

Duchenne muscular dystrophy

Elevidys should be used only in patients meeting all of the following criteria:

・Patients who test negative for anti-AAVrh74 antibodies

・Ambulatory patients

・Patients aged 3 years to under 8

Summary of revisions

1. Test items related to acute hepatic failure and instructions to perform imaging tests, as well as instructions to take appropriate measures, such as postponing administration in the event of observing any abnormalities, should be added to the cautionary statement regarding hepatic function tests in 8. IMPORTANT PRECAUTIONS.
2. A cautionary statement regarding infections related to the administration of corticosteroids should be added to 8. IMPORTANT PRECAUTIONS. The cautionary statement for patients complicated with infections should be moved to the 9.1 Patients with Complication or History of Diseases, etc. section in 9. PRECAUTIONS CONCERNING PATIENTS WITH SPECIFIC BACKGROUNDS.

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3. “Acute hepatic failure” should be added to the 11.1.2 Hepatic impairment subsection in the 11.1 Clinically Significant Adverse Reactions section. Language should be added stating that cases of acute hepatic failure resulting in death have been reported overseas after administration of this product to non-ambulatory patients.

Investigation results and background of the revision

Cases involving acute hepatic failure resulting in death were evaluated. Cases for which a causal relationship between delandistrogene moxeparvovec and acute hepatic failure resulting in death was reasonably possible have been reported. As a result of consultation with expert advisors regarding the causality assessment of the cases and the necessity of revision of PRECAUTIONS, the MHLW/PMDA concluded that revision of PRECAUTIONS was necessary.

Of note, it was confirmed that tests for infections after administering this product had not been performed adequately in one of the evaluated cases involving acute hepatic failure. At the time of the marketing approval review in Japan, no autopsy results had been obtained for this cases. Although it was judged difficult to reach a conclusion on the necessity of additional precautions for acute hepatic failure and infections, it was determined necessary that precautions for both events be included in the package insert. It was deemed necessary to collect information continuously also after marketing and to provide information to clinical settings properly when new information is obtained. This time, since additional information on the autopsy results, etc. of the case was obtained, the risk of infections related to corticosteroid use in association with administration of this product was evaluated based on the information. As a result of consultation with expert advisors, the MHLW/PMDA concluded that revision of PRECAUTIONS was necessary as follows: A precaution regarding the occurrence of infections related to the use of corticosteroids should be added, and a cautionary statement regarding patients complicated with infections should be moved to the 9.1 Patients with Complication or History of Diseases, etc. section.

Reference: Number of cases* and patient mortalities involving acute hepatic failure resulting in death reported in Japan and overseas

No cases have been reported in Japan to date.

A total of 2 cases have been reported overseas to date. (A causal relationship between the

product and the event was reasonably possible for these cases.)

A total of 2 patient mortalities have been reported overseas to date. (A causal relationship between the product and the death following the event was reasonably possible for these cases.)

*Cases collected in the PMDA's safety database for regenerative medical products

The expert advisors present at the Expert Discussion regarding the current investigation were nominated based on their conflict of interest declarations concerning the relevant products, pursuant to the “Rules for Convening Expert Discussions, etc., by the Pharmaceuticals and Medical Devices Agency” (PMDA Administrative Rule No. 20-8, dated December 25, 2008).