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

Onasemnogene abeparvovec

May 20, 2025

Non-proprietary name

Onasemnogene abeparvovec

Brand name (marketing authorization holder)

Zolgensma Intravenous Infusion (Novartis Pharma K.K.)

Japanese market launch

May 2020

Indications or performance

Treatment of patients with spinal muscular atrophy who have tested negative for anti-AAV9 antibodies

Summary of revisions

1. A cautionary statement regarding infusion reaction should be added to the Important Precautions section.
2. “Infusion reaction” should be added to the Clinically significant adverse reactions section.

Investigation results and background of the revision

Cases involving infusion reaction were evaluated. Cases for which a causal relationship between onasemnogene abeparvovec and infusion reaction 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.

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Reference: Number of cases* and patient mortalities involving infusion reaction reported in Japan and overseas

One case has been reported in Japan to date. (A causal relationship between the product and the event could not be established for this case.)

No patient mortalities have been reported in Japan to date.

A total of 10 cases have been reported overseas to date (including 8 cases for which a causal relationship between the product and the event was reasonably possible).

One instance of patient mortality has been reported overseas to date. (A causal relationship between the product and the death subsequent to the event could not be established for this case.)

*Cases collected in the PMDA's database for defects or adverse events, etc. reports

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).