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

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Summary of Investigation Results

Onasemnogene abeparvovec

November 26, 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

Spinal muscular atrophy

Only for patients who have tested negative for anti-AAV9 antibodies

Summary of revisions

A description regarding cardiac troponin I measurement in the IMPORTANT PRECAUTIONS section should be revised.

Investigation results and background of the revision

The relationship between increased cardiac troponin I following administration of this product and cardiac toxicity was evaluated. The expert advisors' opinions on the necessity of revision of the PRECAUTIONS concerning the review of the monitoring rules for cardiac troponin I were also heard. Based on the following reasons, it was considered not reasonable to maintain the rules for regular cardiac troponin I measurement. Therefore, the MHLW/PMDA concluded that it is necessary to delete the current rules regarding the period and frequency of cardiac troponin I measurement and revise the PRECAUTIONS so that cardiac troponin I levels will be measured before administration of this product and within approximately 1

month after administration of this product considering the timing when an increase in cardiac troponin I is observed:

• Although cardiac troponin I may increase, the increase is mild, and no case in which increased cardiac troponin I indicated decreased cardiac function has been reported.

• An increase in cardiac troponin I following administration of this product was observed, suggesting myocardial damage, and risks of cardiac toxicity observed in nonclinical and clinical studies of this product have not been ruled out.

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).