Summary of Investigation Results

Onasemnogene abeparvovec

March 30, 2021

Non-proprietary name

Onasemnogene abeparvovec

Branded name (Marketing authorization holder)

Zolgensma Intravenous Infusion (Novartis Pharma K.K.)

Indications

Treatment of patients with spinal muscular atrophy (SMA, including those with genetically diagnosed presymptomatic SMA) who have tested negative for anti-AAV9 antibodies

Summary of revisions

1. Language concerning thrombotic microangiopathy should be added to the IMPORTANT PRECAUTIONS section.
2. “Thrombotic microangiopathy” should be added to the Clinically Significant Adverse Reactions section.

Investigation results and background of the revision

Cases of thrombotic microangiopathy have been reported in patients treated with onasemnogene abeparvovec in Japan and overseas. MHLW/PMDA in consultation with expert advisors concluded that revision of the package insert was necessary.

Pharmaceuticals and Medical Devices Agency

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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.

Number of cases and patient mortalities reported in Japan during the previous 3 fiscal years

1 case involving thrombotic microangiopathy has been reported to date (A causal relationship between the product and event was reasonably possible for this case.)
No patient mortalities have been reported to date.
(Japanese market launch: May 2020)

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).

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