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Japan approves Novartis' SMA drug

19 March 2020 | News

Novartis receives approval from Japanese Ministry of Health, Labour and Welfare for Zolgensma the only gene therapy for patients with spinal muscular atrophy (SMA)

Novartis Pharma has announced that the Japanese Ministry of Health, Labour and Welfare (MHLW) approved Zolgensma (onasemnogene abeparvovec) for the treatment of spinal muscular atrophy (SMA) in patients under the age of two, including those who are pre-symptomatic at diagnosis. Patients must be negative for elevated anti-AAV9 antibodies. A rare, genetic neuromuscular disease caused by a lack of a functional *SMN1* gene, SMA results in the rapid and irreversible loss of motor neurons, affecting muscle functions, including breathing, swallowing and basic movement. Approximately 60% of all SMA is

Type 1.

Zolgensma is a one-time gene therapy designed to address the genetic root cause of the disease by replacing the function of the missing or nonworking *SMN1* gene. Zolgensma is administered during a single intravenous (IV) infusion, delivering a new working copy of the SMN gene into a patient's cells, halting disease progression. Approximately 15-20 SMA patients in Japan are expected to be eligible for treatment each year. Reimbursement with MHLW is expected by the end of 1H20 and, pending agreement, Zolgensma will be available at that time.

Approval is based on the Phase 1 START, START Long-term follow-up, Phase 3 STR1VE-US, Phase 3 SPR1NT and Phase 1/2 STRONG (intrathecal injection) trials. START and STR1VE-US were designed to evaluate the efficacy and safety of a one-time IV infusion of Zolgensma in symptomatic SMA Type 1 patients <6 months of age at dosing, who had one or two copies of the *SMN2* backup gene, or two copies of the *SMN2* backup gene, respectively.

Zolgensma demonstrated rates of survival never seen in the natural history of the disease; rapid motor function improvement, often within one month of dosing; and milestone achievement, including the ability to sit without support, a milestone never achieved in untreated patients.