Generic Name:
Onasemnogene abeparvovec-xioi
Trade Name:
Zolgensma
Company:
AveXis
Notes:

FDA has approved onasemnogene abeparvovec-xioi, the first gene therapy approved to treat children younger than 2 years who have spinal muscular atrophy (SMA). SMA is the leading genetic cause of infant mortality.

SMA is a rare genetic disease caused by a mutation in the survival motor neuron 1 (SMN1) gene. SMA caused by mutations in the SMN1 gene is generally classified into several subtypes that are based on age of onset and severity. Infantile-onset SMA is the most severe and most common subtype. Children with this condition have problems holding their head up, swallowing, and breathing. These symptoms may be present at birth or may present by the age of 6 months.

A one-time I.V. administration of onasemnogene abeparvovec-xioi results in expression of the SMN protein in a child’s motor neurons, which improves muscle movement and function, and survival of a child with SMA. Dosing is determined on the basis of the patient's weight.

Safety and effectiveness of the drug are based on an ongoing clinical trial and a completed clinical trial involving a total of 36 pediatric patients with infantile-onset SMA between the ages of approximately 2 weeks and 8 months at study entry. The primary evidence of effectiveness is based on results from the 21 patients treated with onasemnogene abeparvovec-xioi a in the ongoing clinical trial.

The drug's most common adverse effects are elevated liver enzymes and vomiting. A boxed warning cautions that acute serious liver injury can occur. Patients with preexisting liver impairment may be at higher risk of experiencing serious liver injury.

Certain vaccines are contraindicated for patients on a substantially immunosuppressive steroid dose. Therefore, caregivers should consult with their health professional to determine if adjustments to the patient’s vaccination schedule are necessary to accommodate concomitant corticosteroid administration.

Medication Monitor Categories:
New Drug Approvals