FDA approves first gene therapy for spinal muscular atrophy

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(HealthDay)—The first gene therapy has been approved to treat children

younger than 2 years with spinal muscular atrophy (SMA), the U.S. Food and Drug Administration announced Friday.

Zolgensma (onasemnogene abeparvovec-xioi), an adeno-associated virus vector-based gene therapy, targets the cause of SMA by delivering a fully functional copy of the human *SMN1* gene into the target motor neuron cells. According to the FDA, a one-time intravenous administration of the drug yields expression of the SMN protein in a child's motor neurons, which improves movement, function, and survival. Dosing is based on patient weight, with a recommended dosage of 1.1×1.0^{14} vector genomes per kilogram of body weight. Zolgensma is administered as an intravenous infusion for 60 minutes.

Approval was based on two clinical trials, one ongoing. The completed clinical trial involved 36 children with infantile-onset SMA who were 2 weeks to 8 months old at study initiation. In the ongoing clinical trial, which initially included 21 patients, the 19 remaining patients are 9.4 to 18.5 months old. These patients have demonstrated significant improvement in reaching developmental motor milestones compared with the natural history of patients with infantile-onset SMA.

Commonly reported side effects include elevated liver enzymes and vomiting. A boxed warning included with the prescribing information indicates the risk for acute serious liver injury and warns that patients with preexisting liver impairment may be at higher risk. Before initiating treatment with Zolgensma and for at least three months after administration, clinicians should assess patients' liver function through clinical examination and laboratory testing. Patients should also be administered an oral corticosteroid before and after Zolgensma infusion; because certain vaccines are contraindicated for patients taking a corticosteroid, caregivers should consult with clinicians to determine if adjustments to the child's immunization schedule are necessary.

Approval of Zolgensma was granted to AveXis

More information: More Information

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