AveXis Statement on Access to Zolgensma® (onasemnogene abeparvovec-xioi)

July 25, 2019

The FDA's May 2019 approval of Zolgensma®, a gene therapy for spinal muscular atrophy (SMA) in pediatric patients less than 2 years of age, marked an important milestone within the SMA community. Zolgensma offers a new treatment option for children with SMA and their families. Understandably, many families are actively interested in accessing Zolgensma as soon as possible and we are working with health plans to support access for eligible children.

The introduction of a one-time gene therapy like Zolgensma has raised important questions about how our healthcare system defines the value of treatments, and manages the diagnosis, treatment, care and associated costs for patients with rare diseases. FDA approval—even for a breakthrough therapy like Zolgensma—does not create immediate access. For any newly approved therapy, it takes time to set up agreements with commercial and government-based health plans and it is common for there to be an appeal process while insurers put coverage and utilization policies and procedures in place. We also recognize that, as there is no data to support combination use, insurers may deny coverage when families are deciding to pursue Zolgensma in addition to the available chronic therapy.

"In the short amount of time that Zolgensma has been available, we have seen important progress in establishing coverage policies, which moves us closer to our mission of bringing this transformative one-time gene therapy to more children facing this rare and devastating disease," said Dave Lennon, president of AveXis. "We remain committed to partnering to accelerate coverage decisions for this important SMA therapy."

Since FDA approval, a wide range of patients with different SMA types, weights and ages, up to 2 years old, have been approved across a number of payer types, including Medicaid. We are actively partnering with insurers to accelerate coverage decisions that will support access for eligible patients that are seeking treatment including offering pay-over-time options up to 5 years through a third-party and outcomes-based agreements up to 5 years through AveXis. In the two months since FDA approval, more than 20 insurance plans, representing 40% of people with commercial insurance, as well as 4 Medicaid plans, have established coverage policies.

We encourage patients' families to talk to their physicians to understand their treatment options and if prescribed Zolgensma, reach out to our OneGene Program, which helps connect families with the resources they need throughout their treatment journey.

We are proud of the transformational innovation that Zolgensma is bringing to the SMA community and are committed to partnering across the healthcare system to support access. We also remain dedicated to bringing Zolgensma to older patients with SMA. Later this year, we will present additional data on intrathecal dosing of Zolgensma in older patients with SMA and will begin discussions with the FDA on a regulatory pathway.

Indication
Zolgensma (onasemnogene abeparvovec-xioi) is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patient less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (*SMN1*) gene.

Limitation of Use:

The safety and effectiveness of repeat administration of Zolgensma have not been evaluated.

The use of Zolgensma in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator-dependence) has not been evaluated.

Important Safety Information

Acute serious liver injury and elevated aminotransferases can occur with Zolgensma. Patients with pre-existing liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase and alanine aminotransferase], total bilirubin and prothrombin time). Administer systemic corticosteroid to all patients before and after Zolgensma infusion. Continue to monitor liver function for at least 3 months after infusion.

Thrombocytopenia
Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were observed at different time points after Zolgensma infusion. Monitor platelet counts before Zolgensma infusion and on a regular basis afterwards.

Elevated Troponin-I
Transient increases in cardiac troponin-I levels (up to 0.176 mcg/L) were observed following Zolgensma infusion in clinical trials. The clinical importance of these findings is not known. However, cardiac toxicity was observed in animal studies. Monitor troponin-I before Zolgensma infusion and on a regular basis for at least 3 months afterwards.

Adverse Reactions
The most commonly observed adverse reactions (incidence >=5%) were elevated aminotransferases and vomiting.

Please read full Prescribing Information for Zolgensma, including Boxed Warning for Acute Serious Liver Injury.