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FDA Approves Zolgensma, Landmark AAV-Delivered Gene Therapy to Treat Spinal Muscular Atrophy

Zolgensma (Novartis, AveXis), an AAV-delivered gene therapy used to treat spinal muscular atrophy (SMA) type 1 also known as AVXS-101, was approved for clinical use in the United States by the Food and Drug Administration today.

The treatment is now both the second virally-delivered gene therapy approved to treat inherited genetic disorders in the United States and the second-ever approved treatment for SMA type 1.

FDA granted priority review status to developer AveXis in December 2018, The therapy previously received Breakthrough Therapy designation in the US and remains in the European Medical Agency's PRIority MEdicines (PRIME) program, both designed to facilitate efficient development of medicines.

"More than another clinical landmark for translational science in gene therapy, the approval of Zolgensma provides hope for thousands of families affected by SMA type 1 in the United States," Guangping Gao, Ph.D., president of the American Society of Gene and Cell Therapy and director of the Horae Gene Therapy Center at the University of Massachusetts Medical School says. "The road from the lab to the clinic is always long and arduous, but we expect to see accelerating approvals in gene and cell therapy as the basic and translational science proves safe and effective. Congratulations to all involved in the development of Zolgensma and thank you to the patients and families who have participated in all levels of tests and trials."

In clinical trial updates presented at the American Society of Gene & Cell Therapy's 22nd Annual Meeting on April 29, all 12 infants treated with Zolgensma had event-free survival and 92 percent demonstrated Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHIP-INTEND) scores over 40 six months following treatment compared with just 38.5% of patients treated with the sole alternative treatment for SMA type 1. Totals for the CHIP-INTEND test range from 0 to 64 with higher scores indicating better motor skills.

In clinical trial data supporting Zolgensma's priority review application, all 15 patients infused with Zolgensma were alive and without the need for permanent ventilation at 24 months, and 11 of 12 patients could sit unassisted for at least 5 seconds. Patients monitored as part of an ongoing observational long-term follow-up maintained their developmental motor milestones and some achieved additional motor milestones.

About SMA

Spinal Muscular Atrophy (SMA) is a rare genetic disease that affects the motor nerve cells in the spinal cord and is the leading genetic cause of infant mortality. It is caused by an inherited faulty SMN1 gene. The SMN1 gene helps provide instructions to cells on how to produce the SMN (survival motor neuron) protein. If there isn't enough correct SMN protein produced, it leads to degeneration, meaning decline

or deterioration, of motor neurons. This creates problems with a person's physical strength, often taking away the ability to walk, eat or even breathe. ASGCT has created educational resources for families of patients affected by SMA at asgct.org/education/spinal-muscular-atrophy.

About ASGCT

The American Society of Gene & Cell Therapy is the primary professional membership organization for scientists, physicians, patient advocates, and other professionals with interest in gene and cell therapy. Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, biotechnology and pharmaceutical companies. ASGCT advances knowledge, awareness, and education leading to the discovery and clinical application of gene and cell therapies to alleviate human disease to benefit patients and society.