



PRODUCT:  
**Zolgensma®**

COMPANY: Novartis Gene Therapies

THERAPEUTIC AREA & INDICATION:  
One-time gene replacement therapy for children  
under age 2 with spinal muscular atrophy

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#### ABOUT THE PRODUCT

Novartis Gene Therapies imagined a world in which a gene therapy – with just one treatment – could address the root cause of spinal muscular atrophy. This devastating disease, caused by the lack of a functional survival motor neuron 1 gene, can result in irreversible loss of motor neurons that control breathing, swallowing, and basic movement. Today, we have delivered on this vision. Physicians have the option to treat children under two years old with a one-time, intravenous infusion called Zolgensma® (onasemnogene abeparvovec-xioi).