

Gene Therapies Overview

Novartis Gene Therapies, the world leader in gene therapy, is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are working to turn promising gene therapies into proven treatments, beginning with our transformative gene therapy for spinal muscular atrophy (SMA). We are driven by the impact our therapy has on patient families.

Fast Facts



1st & only
gene therapy
approved for the
treatment of SMA



40+
countries
where our therapy
is approved and
reaching patients



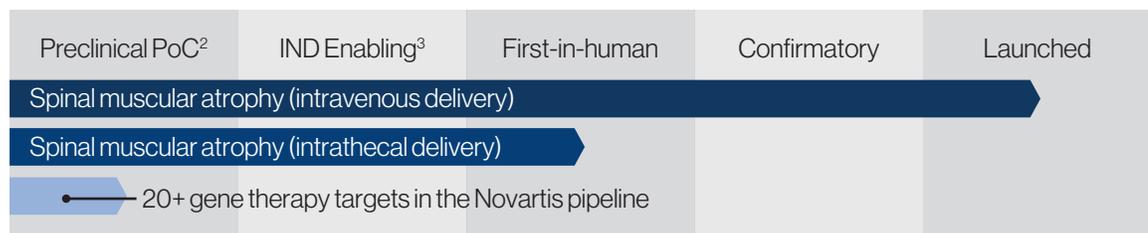
2,300+
patients
with SMA treated
globally in clinical trials,
managed access
programs and in the
commercial setting¹

Our Presence

Novartis Gene Therapies leverages the Novartis network around the world. Our global headquarters is located in **Bannockburn, IL** and our EMEA headquarters in **Zurich, Switzerland**. Other key locations include a research site in **San Diego, CA** and manufacturing sites in **Libertyville, IL** and **Durham, NC**.

Innovative Research Fuels Pipeline Development

The goal of Novartis Gene Therapies' cutting-edge science is to address the underlying, genetic root cause of diseases. With an end-to-end gene therapy approach, Novartis Gene Therapies pioneered foundational research, establishing AAV9 as a vector for gene transfer in diseases affecting the central nervous system. Pairing scientific expertise with proven manufacturing and market access capabilities laid the groundwork to build a transformational gene therapy pipeline.



[Novartis.com](https://www.novartis.com) [@NovartisGene](https://twitter.com/NovartisGene) [@Novartis Gene Therapies](https://www.linkedin.com/company/novartis-gene-therapies)

¹As of June 30, 2022 ²Preclinical Proof of Concept ³Investigational New Drug Enabling

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