About Novartis Gene Therapies

Rare diseases don’t feel rare to the families devastated by them. They create daily challenges that must be faced head-on, often without the help of readily available treatment.

Since 2013, Novartis Gene Therapies (formerly AveXis) has had one focus: bringing change to those devastated by genetic diseases.

The Novartis Gene Therapies culture embraces this mission. As a compassionate and dedicated team, we are enthusiastic about the science behind our work, breaking barriers, and finding answers to difficult questions. We are dedicated to communities affected by rare diseases, and these patients and families are the motivation for everything we do.

We have built a team with exceptional depth of experience, unified by a common vision; to develop gene therapies with the potential to positively impact the lives of the patients and families devastated by rare and life-threatening neurological genetic diseases. Though we are proud of what we have achieved to date, we remain relentlessly focused on making that vision a reality.

With cutting-edge technology, we are making progress in the treatment of rare and life-threatening neurological genetic diseases. Our initial gene therapy for spinal muscular atrophy (SMA) has been approved in more than 45 regions and countries, with access pathways in place across more than 30 countries and has been used to treat more than 3,400 patients worldwide.
