

Novartis Gene Therapies Managed Access Program ^[1]

Novartis Gene Therapies is dedicated to developing innovative therapies for patients suffering from rare and life-threatening neurological genetic diseases. Before these therapies can be approved and commercially available to patients, they must undergo clinical trials to evaluate their safety and effectiveness.

While it is generally preferred that patients gain access to investigational therapies by participating in clinical trials, patients may not be eligible or able to take part in these studies. Patients with serious or life-threatening diseases or conditions sometimes seek therapies that are not yet approved or available in their country. The Novartis Gene Therapies "Managed Access Program" addresses this need by making certain investigational or unapproved therapies available to eligible patients.

Clinical Trials

Before an investigational therapy can be placed on the market, it must undergo well-controlled clinical trials to evaluate its safety and effectiveness, as well understand whether its potential benefit to patients outweighs the possible risks.

[More About Clinical Trials](#) ^[2]

Managed Access Program

The Novartis Gene Therapies "Managed Access Program" has been designed to provide patients seeking such treatment with additional opportunities, provided it is allowed by the applicable local laws and regulations.

The Novartis Gene Therapies "Managed Access" terminology covers all locally defined pre-approval access mechanisms and programs such as "Compassionate Use", "Expanded Access", "Named Patient Supply", "Special Access Schemes/Programs", "Autorisations temporaires d'utilisation (ATU)", and others.

Current Novartis Gene Therapies "Managed Access Program":

- Spinal muscular atrophy (SMA) Managed Access Program.

The global Managed Access Program (MAP) is available in countries where our treatment is not approved by local health authorities in compliance with local laws and regulations. For this global MAP, the physician will need to assess patient eligibility by determining whether the patient meets pre-defined medical and program criteria.

To participate in the global MAP, patients must meet all eligibility criteria outlined in the

treatment plan, including but not limited to:

- Patients under the age of two with genetically confirmed bi-allelic mutations in the SMN1 gene; regardless of type, copy number, or symptom onset.
- Geographically, the patient must be a citizen or legal resident of, and eligible for healthcare in, a country where our treatment is not approved by local health authorities.
- Implementation of the program will need to comply with the specific legal and regulatory framework that applies in every country.

Medical eligibility needs to be confirmed and Health Authority approval obtained in the country where the patient will be treated. In situations where the number of requests outweigh available supply, a third-party administers a blinded allocation. If Novartis Gene Therapies is not able to provide our treatment to the patient at that time, the patient automatically rolls over to the pool for the next allocation as long as he/she remains medically eligible and it is still permissible in the country.

Our one-time gene therapy will be provided free-of-charge. To ensure safe administration and adequate follow-up, treatment will be administered in trained treatment centers. If no such center exists in the patient's home country, Novartis Gene Therapies will determine the feasibility of alternative treatment center locations.

[More About Novartis Managed Access](#) ^[3]

Who should I contact for more information?

For general medical information queries, healthcare professionals may submit requests to [Novartis Gene Therapies Medical Affairs](#). ^[4]

An SMA-treating physician can submit a request for the global-MAP by contacting our third-party partner, [Durbin](#) ^[5].

Patients/parents/caregivers should contact their treating physician and ask him/her about this global MAP.

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[3] <https://www.novartis.com/our-focus/healthcare-professionals/managed-access-programs>

[4] <mailto:medinfo@avexis.com>

[5] <mailto:AveXisMAP@DurbinGlobal.com>