Novartis Gene Therapies Managed Access Program

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

Managed Access Program

The Novartis Gene Therapies global Managed Access Program (MAP) is designed to provide a potential pathway for patients seeking such a treatment, provided the required eligibility criteria are met and in accordance with 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”, and others.

Current Novartis Gene Therapies “Managed Access Program”:

- Spinal muscular atrophy (SMA) Managed Access Program.

This global Managed Access Program is available to eligible patients with spinal muscular atrophy (SMA) in countries where Zolgensma® (onasemnogene abeparvovec) treatment has not received regulatory approval or formal access approval. For this global MAP, the physician will need to assess patient eligibility by determining whether the patient meets pre-defined medical and program-specific 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 Zolgensma has not received regulatory approval or formal access approval.
- 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 is provided free-of-charge. To ensure safe administration and adequate follow-up, treatment can only be administered in trained treatment centers. If no such center exists in the patient’s country of residence, Novartis Gene Therapies determines the feasibility of alternative treatment center locations.

More About Novartis Managed Access

Who should I contact for more information?

For general medical information queries, healthcare professionals may submit requests to the Novartis Gene Therapies Medical Information team in the **US**, **Europe, Middle East and Asia**, **Latin America & Canada**, or **Asia-Pacific**.

How do I submit a request for Managed Access?

A request must be submitted by the treating physician on behalf of the patient. Requests can be submitted via Novartis Gene Therapies request portal [here](mailto:medinfo.gtx@novartis.com). Guidance on using the portal is also available.

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

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