

Novartis Position on Pre-Approval Access to Novartis Products through Novartis Managed Access Programs (MAPs)

The mission of Novartis is to discover ways to improve and extend people's lives. Using science-based innovation, Novartis aims to deliver better outcomes for patients and to address society's evolving healthcare needs.

Clinical trials are an essential part of the development and registration of innovative products. They enable the collection of robust safety and efficacy data on investigational products to support regulatory approval.

However, there are instances where a patient has a serious or life-threatening disease or condition for which all the currently available treatment options have been exhausted and enrollment into a clinical trial is not an option. Additionally, investigational products are not normally available between the completion of clinical trials and regulatory approval or launch. Delayed availability is particularly challenging when no alternative or satisfactory treatment is available to meet the urgent needs of patients.

For patients who have no options, there exist a variety of evolving local regulations and mechanisms to provide investigational products before regulatory approval or product launch. Different terms exist locally for these pre-approval access provisions including "Compassionate Use", "Expanded Access", "Named Patient Supply", "Special Access Schemes/Programs".

At Novartis, "Managed Access Programs" (MAPs) refer to the mechanisms to provide access to any of Novartis' products outside of clinical trials before approval or local product launch.

Novartis Position

We are committed to providing patients who have no comparable or satisfactory alternative therapy a Novartis product prior to regulatory approval or local product launch.¹

- Novartis considers granting managed access to investigational or pre-approval products when all the following criteria are met:
 - An independent request has been received from the treating physician²
 - The patient to be treated has a serious³ or life threatening⁴ disease or condition, and no comparable or satisfactory alternative therapy is available to monitor or treat the disease or condition
 - The patient is ineligible for enrolment into or unable to access ongoing clinical trials

- Sufficient information exists to believe the potential benefit of treatment outweighs the potential risk in the context of the disease or condition to be treated
- Novartis has an adequate supply of the investigational product⁵ and providing the investigational product will not interfere with ongoing clinical trial(s) or with the overall development program
- The patient meets any other important medical criteria established by the medical experts working on the product development program
- Such access provisions as described above are allowed as per local laws and regulations.
- Each request will be reviewed fairly and promptly by qualified Novartis medical experts with every effort made to provide a response within a maximum of five working days once all required medical information has been received from the treating physician. This timeline may be impacted by factors such as national and local requirements or government health authority feedback. Each request will be assessed in consideration of applicable local laws and regulations.
- Novartis will provide the product free of charge to patients in any MAP initiated prior to obtaining first major approval.
- Once the product is approved by regulatory authorities and launched, a MAP cannot be used as a mechanism for provision of medicine to patients. The product may be made available through local mechanisms, such as a patient support program, as allowed by local laws.
- Novartis commits to the timely provision of the product made available under a MAP to the treating physician.

The complex and ever-evolving nature of the Managed Access landscape warrants that we anticipate and plan for situations that may not fall within the norm. In order to address the unmet medical needs of patients as quickly as possible, Novartis has a process in place to support prompt and fair decision-making and ensure a consistent approach across the world in compliance with applicable local laws and regulations.

References

- For more information on managed access programs please refer to our [webpage](#).
- For Managed Access-related questions, please contact the medical team in your [Novartis local country office](#).
- A request must be submitted by the treating physician on behalf of the patient. Request forms can be accessed [here](#).

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¹ [Novartis Declaration for Patients, June 2015](#).

² In some instances, requests may be received from Health Authorities or institutions

³ A disease or condition associated with morbidity that has substantial impact on day-to-day functioning ([FDA Expanded Access: Information for Physicians, 2016](#)).

⁴ A disease or condition is at a stage of disease in which there is reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment ([FDA Expanded Access: Information for Physicians, 2016](#)).

⁵ There may be situations when we are faced with a limited supply of investigational products during the early phases of development while a MAP is open. In such cases, clinical trial patients will always be given priority in order to ensure full assessment of the safety and efficacy of the product within the development program.