Novartis Public Affairs

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 normally not available between the completion of clinical trials and regulatory approval or commercial availability. 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 commercial availability. 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 and before regulatory approval.

Novartis Position

We are committed to providing patients who have no comparable or satisfactory alternative therapy a Novartis product prior to regulatory approval[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 physician[2]

[1] The Novartis Commitment to Patients and Caregivers
[2] In some instances from Health Authorities, Institutions or Governments

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  - The patient to be treated has a serious[3] or life threatening[4] 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 product[5]and providing the investigational product will not interfere with ongoing clinical trial(s) or with the overall development program[6]
  - 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 commercially available, 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. Requests can be submitted via our webpage

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[3] Serious disease or condition means a disease or condition associated with morbidity that has substantial impact on day-to-day functioning. Short-lived and self-limiting morbidity will usually not be sufficient, but the morbidity need not be irreversible, provided it is persistent or recurrent. Whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one. (FDA Expanded Access: Information for Physicians, 2019).

[4] 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, 2019 Note: FDA definition is for immediately life-threatening)

[5] There may be situations when Novartis is 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.

[6] In instances where there are supply constraints, access for patients in countries where regulatory approval has been obtained for the product will be prioritized over managed access provision to new patients in countries without regulatory approval.