Position on Early Access and Post-Trial Access to Investigational Therapy

Last updated March 5, 2025

Background

Astellas is committed to improving the health of people around the world through the provision of innovative and reliable pharmaceutical products.[1] Enrollment in clinical trials is the most effective way to access investigational Astellas therapies before they are approved in a country. However, we recognize that in cases of serious and life-threatening disease, this may not always be an option for patients and their families or caregivers.[2] With these patients in mind, Astellas evaluates and plans, when possible, for other appropriate access routes throughout the drug development process, including early access and post-trial access programs. For an understanding of how Astellas evaluates and plans for access to therapy at each phase of the product lifecycle, see Our Commitment to Improving Access to Medicines at *[*Access to Medicines | Astellas Pharma Inc.*]*.

Our Position

Early access

Early access programs enable access to an investigational Astellas therapy before marketing approval following an unsolicited request by a prescribing healthcare professional. We establish these programs as important access routes for patients with serious, life-threatening diseases who have exhausted all available therapeutic options and are unable to participate in a clinical trial. Astellas continually evaluates which diseases and therapies meet the criteria for providing early access and establishes selected programs before marketing authorization, typically to run until the therapy is commercially available. We strive to include countries conducting pivotal clinical trials for the investigational therapy and countries where commercial availability is planned. However, early access programs are not feasible in every country and there may be factors such as local regulations, diagnostics, manufacturing capabilities and product supply limitations that impact where early access programs are established. Early access programs that require physicians to follow an approved treatment protocol are registered, if required by regulation, on ClinicalTrials.gov, the EU Clinical Trials Information System and /or local registries.

Post-trial access

Astellas works to ensure patients with serious, life-threatening diseases, who have completed Astellas clinical trials and have no commercially available therapeutic alternatives, have uninterrupted access to the investigational therapy until it becomes commercially available in that patient's country of residence. When applicable, the clinical trial protocol describes post-trial access. In all cases, information related to post-trial access is communicated to relevant healthcare professionals and clinical trial participants before or during study participation.

Eligibility for post-trial access and early access

Astellas commits to establishing early and post-trial access programs for investigational therapies and patients that meet the following criteria:

Criteria established to implement early access and post-trial access programs

- The investigational therapy is actively being developed to treat a serious, life-threatening disease
- Safety and efficacy data that supports continued development with the dose selected and the intent to pursue marketing approval
- Clinical trials and registration processes will not be delayed or compromised
- The investigational therapy is not commercially available in the patient's country of residence
- There is sufficient product supply available and there are no barriers to obtaining, labeling and distributing the investigational therapy
- The program is permitted by health authorities under existing laws and regulations

Criteria established to determine patient eligibility

- The patient has a serious, life-threatening disease
- There are no commercially available therapeutic alternatives
- The benefit outweighs the risk to the patient, as determined by the patient's physician in consultation with the Astellas Early Access Medical Lead
- The patient meets product-specific eligibility criteria
- The appropriate local and national regulations are followed with the required approvals obtained to provide an investigational therapy
- Diagnostics, equipment and physician expertise required to administer the therapy are available to the patient
- The patient can continue therapy, if needed, following commercial availability by transitioning from early access or post-trial access to a commercial access mechanism

Early access requests

All requests for early access must be made by a patient's treating physician. Therefore, any patient seeking access to investigational therapy through an Astellas-sponsored early access program should contact their physician directly. Astellas will acknowledge receipt of all requests and begin discussing patient eligibility with the requesting physician within five calendar days. The decision to provide a patient with early access to therapy will be made by Astellas on a case-by-case basis in consultation with the patient's physician and the Astellas Early Access Medical Lead.

Healthcare professionals can submit early access requests on behalf of their patients using our Access to Medicines Request Platform. Our Access to Medicines Request Platform streamlines requests for support into one central and accessible location for healthcare professionals.

Click here for the Access to Medicines Request Platform.

Additional Information

Our guiding principles

The Access to Medicines philosophy at Astellas is guided by the principles of international ethical frameworks such as the Declaration of Helsinki, ICH Good Clinical Practices and other relevant international guidelines.[3,4] Astellas has controlled procedures in place to support prompt and fair decision-making, while ensuring a consistent approach for managing these programs throughout the world in compliance with applicable laws and regulations.

Access to cell and gene therapy

The access mechanisms implemented by Astellas are multifaceted, require close evaluation, and may be shaped by country specific regulations. This is especially true for more complex treatment forms such as cell and gene therapies where clinical and regulatory requirements vary and are rapidly evolving.

Additionally, cell and gene therapies typically require administration by highly trained physicians who have access to the diagnostics, equipment and resources needed to ensure the highest level of safety possible. For this reason, Astellas will continue to focus on bringing safe and effective cell and gene therapies to market as quickly as possible, with the quality controls required to develop these innovative therapies.

References

1. Astellas. Vision and strategy. Available at https://www.astellas.com/en/about/philosophy

2. FDA Code of Federal Regulations (21 CFR § 312.300). Expanded Access to Investigational Drugs for Treatment Use. Available at https://www.ecfr.gov/current/title-21/chapter-I/subchapter-D/part-312/subpart-I
3. WMA. Declaration of Helsinki. Available at https://www.wma.net/policies-post/wma-declaration-of-helsinki-ethical-principles-for-medical-research-involving-human-subjects/
4. ICH. Guideline for Good Clinical Practice E6(R2). Available at https://database.ich.org/sites/default/files/E6_R2_Addendum.pdf

Date of Disclosure: March 2025