

Purpose & Scope

AstraZeneca's purpose is to discover and develop new medicines that make a meaningful difference to patient's lives, primarily for the treatment of diseases in three main therapy areas: oncology, cardiovascular and metabolic disease, and respiratory. We are also selectively active in autoimmunity, neuroscience and infection.

In our endeavour to bring innovative medicines to patients as fast and safely as possible, we conduct clinical trials to establish the safety and efficacy of an 'Investigational Medicine', which is a potential medicine that is in active clinical development but has not yet received marketing approval by health authorities.

The clinical trials and the subsequent generation of safety and efficacy data are the most effective way of ensuring timely review and decision making by Health Authorities on a given medicine. This will ultimately result in access to new, safe and effective approved medicines for the broadest patient population. For this reason, AstraZeneca prioritises access to an investigational medicine through clinical trials and encourages patients to be enrolled in such studies. For information on available clinical trials visit: www.ClinicalTrials.gov.

However, we recognise that there are circumstances wherein patients with serious or life-threatening diseases have exhausted other comparable or satisfactory alternative therapeutic options and may not be eligible for, or otherwise unable to participate in, one of our trials. In such circumstances, subject to the criteria set forth below and country specific regulations, patients may be eligible for Early Access to AstraZeneca's investigational medicines.

This document summarises our policy on access to one of our investigational medicines outside of a clinical trial, which AstraZeneca refers to as Early Access.

Outside of a clinical trial, any use of an AstraZeneca investigational medicine must be overseen by a licensed and appropriately qualified physician, in accordance with local laws and regulations governing such programmes, as well as AstraZeneca policies and procedures.

The Early Access programme does not apply to the use of an approved medicine for an indication beyond the authorised prescribing information (often referred to as "off-label use").

Acquisition and use of marketed medicines outside of approved indications is at the discretion of the treating physician and the patient and is neither facilitated nor endorsed by AstraZeneca.

Early Access programmes will terminate around the time of the investigational medicine receiving regulatory (i.e., marketing) approval authorising its general availability for physicians and patients.

Types of Early Access Programmes

AstraZeneca operates two distinct types of Early Access programmes – Individual Named Patient Supply (NPS) and Multiple Patient Early Access Programmes (MPEAP). It is important to note that terminology used can vary from country to country and in some instances different countries use identical terminology to describe different approaches.

Multiple Patient Early Access Programmes (MPEAP)

Multiple Patient Early Access programmes (MPEAP) known as Expanded Access Programmes in the US or Compassionate Use in Europe, describes the process by which an investigational medicine is made available to a group of patients under a specific treatment protocol. This may be undertaken on AstraZeneca's initiative and conducted following discussions with and approval by the relevant regulatory authorities in a given country. These programmes are conducted in accordance with the proposed prescribing information for the investigational medicine and is aligned to the indication and use for which AstraZeneca has submitted an application for marketing approval. These programmes may begin enrolment only after sufficient data are available to support benefit/risk profile and the regulatory submission for marketing approval.

Individual Named Patient Supply (NPS)

Where there is no MPEAP, AstraZeneca may make an investigational medicine available to a single patient in accordance with country-specific regulations and for use in accordance with the intended indication. Terminology and requirements of named patient access schemes vary globally; in the US, for instance this includes the FDA's guidelines for treatment of individual patients with an Investigational New Drug (IND).

Eligibility Criteria

An Early Access programme for a specific investigational medicine may be opened if AstraZeneca determines that all the following criteria are met:

Investigational Medicine Eligibility:

1. The investigational medicine is the subject of an active clinical development program and is not approved in any indication in the country concerned
2. An adequate supply of the investigational medicine exists to perform necessary clinical studies as well as to provide Early Access to patients who do not have alternative treatment options
3. Early Access does not impede or compromise the clinical development or regulatory approval of the medicine under investigation
4. There are sufficient clinical data available with respect to both the investigational medicine and the disease condition for which the application is being sought, to anticipate that any potential benefits from treatment are likely to outweigh any associated risks to the patient

Patient eligibility:

5. The patient is suffering from a serious or life-threatening disease, has exhausted other comparable or satisfactory alternative therapeutic options and is not eligible to enrol in a clinical study for medically valid reasons or is otherwise unable to participate in one of our clinical trials.
6. The patient will be receiving treatment in a country or jurisdiction where AstraZeneca plans to seek marketing approval for the investigational medicine.
7. The patient's medical status is deemed appropriate to receive the investigational medicine.

Treating Physician eligibility:

8. The treating physician(s) is appropriately qualified to administer the investigational medicine and follow all relevant safety requirements and procedures.

Request Process

Requests for Early Access to an AstraZeneca investigational medicine outside of a clinical study must be made by a patient's treating physician. Requests must not include the patient's name or specific identifying information.

Physicians seeking information about how to apply for Early Access, or who wish to submit a request may contact the AstraZeneca Medical Affairs team in the local country as follows:

- US: Medical Information Call Center: 1800-236-9933
- Outside the US: an overview of websites of the individual countries where AstraZeneca has subsidiaries can be found at "[Local AstraZeneca Affiliates](#)".
- Alternatively, an e-mail can be submitted to: EarlyAccess@AstraZeneca.com.

How Do We Use the Information Submitted:

The information submitted is only used to evaluate requests for Early Access to our investigational medicines

How We Protect Information Submitted to Our Early Access Programme

We have in place appropriate privacy and security policies which are intended to ensure, as far as reasonably possible, the security and integrity of all our information, including information submitted by you, whether the information is your personal information or pertains to another individual (e.g., the patient).

Evaluation of Requests

AstraZeneca's willingness to consider requests for Early Access to an investigational medicine is based on its review of the patient information submitted by the requesting physician to support the request as well as satisfactory evidence having been provided to AstraZeneca that the physician responsible for treating the patient is appropriately qualified in accordance with local laws and regulations.

To ensure a fair review of Early Access requests, decisions will be based on the above criteria. Requests will be considered on a first come first served basis for all patients dependent on drug availability.

In considering and administering requests for Early Access, our patient-focused principles are based on a commitment to be fair, impartial and transparent. Our decisions are guided by the best clinical and scientific evidence currently available for any given IMP.

Please note that submission of a request does not guarantee that access to the investigational medicine will be provided. Eligibility will be determined by AstraZeneca based on the established Early Access policy and eligibility criteria for the investigational medicine concerned. We may not be able to provide IMP in response to all requests received.

Treating Physician Criteria and Responsibilities

The physician(s) treating the patient receiving an investigational medicine through Early Access must be properly licensed and fully qualified to administer the product.

The physician will be responsible for collecting data throughout the treatment and for submitting those to AstraZeneca, where applicable and in accordance with local laws and regulations. In addition, the physician will be responsible for ensuring all local legal, regulatory and Health Authority requirements are met, including Investigational Review Board or Ethics Committee approval and reporting of Adverse Events.

The physician must agree in writing to comply with the following terms:

1. To use the product only for the patient specified.
2. Any applicable country-specific legal and regulatory requirements related to providing an investigational medicine under Early Access.
3. Any AstraZeneca requirements applicable to patient confidentiality and data privacy, medical criteria, safety reporting, drug supply handling and use, and protection of intellectual property and confidential information.
4. Obtaining informed consent from the patient approved by an Institutional Review Board. This must include a statement that the investigational medicine will be provided free of charge, until regulatory approval has been secured. However, any associated costs of treatment, including but not limited to drug administration costs, costs associated with laboratory or radiological monitoring, travel, physician and hospital fees will be the responsibility of the insurer, health care system, and/or patient.

AstraZeneca will send to the treating physician:

- Letter of Acceptance (including a request for confirmation of the patient's informed consent)
- Instructions for Use for the investigational medicine
- Adverse Event Report Form.

By signing a Letter of Acceptance the treating physician agrees to fulfill all the responsibilities outlined in the letter.

Early Access Programme Closure

Early Access may be discontinued at any time at the discretion of the treating physician or the patient. Early Access may also be discontinued by the treating physician, the patient or AstraZeneca when:

1. The patient is no longer receiving any clinical benefit, typically documented by progression or deterioration of the underlying disease or adverse events that are deemed by the treating physician or patient to outweigh the potential benefit of the investigational medicine
2. An alternative effective medicine is available
3. The patient becomes eligible to enroll in a clinical trial for the investigational medicine
4. The product receives marketing approval
5. The benefit/risk profile of the investigational medicine has been determined by AstraZeneca to no longer support further use of the investigational medicine
6. The local Health Authority rejects the application for marketing approval
7. Unanticipated shortage of investigational medicine.