

Request of Expanded Access to Investigational Medicinal Products_Version 01/February 2018

Kedrion's mission is to discover, develop and distribute innovative therapies to treat people suffering from rare and debilitating conditions. To do this, we carry out clinical trials to evaluate the safety and efficacy of Investigational Medicinal Products (IMPs) with the aim of collecting clinical data to support the approvals from regulatory authorities and, therefore, to make the product commercially available.

However, for patients with serious or life-threatening diseases or conditions, for which no satisfactory alternative approved therapies are available and participation in an on-going clinical trial is not an option, Kedrion may consider providing an investigational drug outside the frame of a clinical trial in compliance with applicable laws and regulations. Such use of IMP outside a clinical trial is known as "Expanded Access" or "Pre-Approval Access".

Kedrion will consider granting Expanded Access to an investigational medicinal product only if all the following criteria are met:

- the disease/condition being treated is life threatening or seriously debilitating and no approved effective treatment options with specific indication exists and is commercially available in the specific country;
- the patient is ineligible or is unable to participate in any on-going clinical trial, also due to geographic limitations;
- data from clinical trials point towards a favorable benefit/risk ratio;
- no possibility to interfere with or compromise any related clinical trial or regulatory pathway;
- the request must be unsolicited and made by the Health Care Professionals (HCPs) in charge of patient.

In addition, the following aspects will be taken into account:

- there is evidence of a potential benefit for the individual specific patient requesting the treatment (context of disease or condition to be treated);
- the patient must meet all other medical criteria established by the medical experts working on the product development program;
- the proposed Expanded Access will be in a country where appropriate medical oversight exists;
- the clinician in charge of the patient confirms that all medical criteria are met and commit to report any adverse event;
- an adequate supply of IMP is allowed.

HCPs treating patients who meet Kedrion's criteria, can submit their request [by clicking on this link](#). Each request will be considered individually. Kedrion will not grant Expanded Access to identical requests: all the requests will be evaluated case by case.

Kedrion cannot guarantee that an Expanded Access Program will be available. In the event an Expanded Access Program is offered, due to the inclusion criteria and IMP availability, Kedrion cannot guarantee that all requests will be granted. After the confirmation that a patient meet all the criteria defined above, it will be Kedrion's discretion to include the patient in a Cohort or provide the treatment as individual.

Any pre-approval access to an investigational medicinal product must always comply with the applicable country-specific laws and regulations, including medical importation requirements, and approval by applicable regulatory authorities, by Institutional Review Board (IRB) or Ethics Committee.

When a request is acknowledged, the informed/data handling consent to comply with the safety and monitoring requirements defined by Kedrion (e.g. outcome, baseline and historical data) must be provided; the data will be treated anonymously. If allowed by country-specific laws and regulations, an Agreement with treating center or hospital will be also signed for the Expanded Access management.

When a product is approved and becomes available commercially, all existing expanded access programs in that country will be phased out.