



Compassionate or Expanded Access To AMO's Investigational Medicines

AMO Pharma is focused on developing medicines with potential to advance the treatment of serious and debilitating diseases, including rare genetic disorders. Our goal is to identify and advance a pipeline of promising treatments for patient populations with significant areas of unmet need.

Investigational medicines are treatments for a particular disease that are not yet approved for that use by regulatory authorities, such as the FDA or EMA. In order to evaluate investigational medicines, companies such as AMO conduct clinical trials to assess safety and efficacy. At the conclusion of the trials, companies then provide the results and other supporting information to regulatory authorities responsible for making the decision about approvals of new medicines.

In some cases, patients who are not part of a clinical trial but who meet certain criteria may qualify for access to an investigational medicine. In most cases, the request for access must be submitted by the patient's physician. Any requests for access to AMO's investigational medicines prior to their regulatory approval should be made by a physician and should be based on the following conditions:

- the patient has a serious or immediately life-threatening condition;
- the patient does not qualify for any ongoing AMO clinical trials;
- the patient has no other viable approved treatment options available;
- all other relevant medical criteria that would allow for compassionate or expanded access use of the investigational product are met; and,
- clinical data, such as early-stage (Phase 1 or 2) clinical trial results in the same indication, demonstrate there may be potential benefit and acceptable risks associated with the proposed use in this patient.

If the criteria listed above are met, AMO will assess whether compassionate use or special access can be considered and approved based on any national, regional or local legal and regulatory guidelines that may apply. AMO will also assess whether the supply of the investigational product is adequate for distribution to patients through compassionate use. In addition, AMO will confirm whether a treating physician has or will obtain approval for compassionate use of our drugs from their institution by an ethics or independent review committee.

If approved, the patient (or his or her guardian) must provide informed consent that complies with the safety and monitoring requirements defined by AMO and/or the regulatory authorities. The treating physician must also agree to comply with all relevant safety and monitoring requirements. Compassionate use or special access will discontinue if, as a result of clinical trials, the investigational medicine does not demonstrate effectiveness or an acceptable benefit/risk balance for use by patients.

For patients that meet AMO's criteria for compassionate use of our investigational medicines, treating physicians can make a request or send an inquiry to info@AMO-pharma.com.

At AMO we are continually evaluating opportunities to provide eligible patients access to our investigational medicines, particularly as our clinical programs advance and there is evidence of potential benefit and acceptable safety. Please check back at <http://www.AMO-pharma.com> for updates.