Our Promise

Since the company's earliest days, extraordinary dedication to people affected by rare and orphan diseases has been a hallmark of Amicus Therapeutics. Our mission is to develop new medicines that improve the health and well-being of people living with rare diseases. With the development of medicines designed to satisfy unmet medical needs, comes the promise that these medicines will be broadly accessible. Our ultimate goal is to provide access through marketing authorization. However, before our product candidates might become available through these means, Amicus is committed to carefully designed and considered pathways to expanded access to our investigational drugs.

Paths to Access

The first of these paths is clinical research (also called clinical trials or clinical studies). As part of established processes for drug development, Amicus sponsors clinical research to best understand the safety and effectiveness, the risk and benefit of its investigational drugs. Parameters for these studies are determined together with regulatory authorities, including the criteria necessary for volunteer participants. Studies are generally multi-phased, closely monitored and can be spread out over many years. People wanting to help advance disease knowledge and who meet the appropriate criteria may participate in these clinical studies.

For those participants who complete an Amicus clinical study, it is the company's general plan to provide an open-label, long-term extension study, as appropriate to meet need. This gives study participants continued access to the investigational drug until it is approved and available in their respective geography. Long-term extensions also enable collection of additional data to support ongoing safety and, in some instances, potential efficacy of the still-experimental medicine.

Expanded Access

Sometimes, however, someone may not qualify for a clinical study or there may be no studies available. Depending upon where a patient resides and the local authorities, there may be other paths toward access. These approaches to expanded access may have slightly different names or approaches, such as Named Patient Program or Named Patient Use, Authorization for Temporary Use (ATU), Compassionate Use, Early Access or others, depending upon geography and what may be allowed by the respective regulatory authorities.

Amicus is dedicated to addressing the needs of those individuals who have a serious or potentially life-threatening disease for which Amicus has a potential therapy under investigation and who have exhausted all alternative treatment options, including enrolling in available clinical trials. For these patients, Amicus is committed to careful review of all physician-submitted requests for expanded access on an individual, case-by-case basis. Factors taken into consideration include: a person's current medical condition based upon information provided by their requesting physician; treatment options; the known safety and efficacy of the investigational medicine; available supply of medicine; applicable regulations, if any, among other pre-determined criteria for the patient and guidelines for the company.

EXPANDED ACCESS TO AMICUS INVESTIGATIONAL DRUGS

In collaboration with regulatory authorities, a patient may be given expanded access to Amicus investigational and pre-reimbursed products before such products have received regulatory approval, provided that the requirements listed below are met.

Criteria for Consideration of an Expanded Access Request

All of the following conditions must be met for a request to be considered.

The individual patient's situation must align with the following requirements:

- The individual must have a serious or life-threatening medical condition within the disease areas that Amicus is investigating.
- No standard treatments exist, or the individual has undergone and not responded to medically appropriate standard treatments, or the individual has declined medically appropriate standard treatments for safety considerations as documented by the referring healthcare professional for ethical (e.g., religious) reasons.
- The individual does not qualify to participate in any ongoing clinical study sponsored by Amicus in a reasonably accessible geographical location, as determined by Amicus.

The qualified physician or healthcare professional (HCP) providing medical care to the individual must:

- Voluntarily initiate the request for expanded access.
- Supervise administration of the investigational product in line with Amicus' defined access criteria (which in some countries may include a protocol for treatment use).
- Obtain informed consent and/or assent as necessary from the individual patient and/or his/her legal guardian(s) for treatment use of the investigational product, and collection of any patient data as appropriate. (Patient data will be determined for each individual investigational product prior to the initiation of the Expanded Access Program (EAP), in line with local regulatory requirements and guidelines).
- Carry out the protocol for treatment use, in countries where this applies, under appropriate regulatory and ethical standards, including regulatory agency, and/or Institutional Review Board (IRB)/Institutional Ethics Committee (IEC) review as applicable.
- Maintain and release to regulatory agencies, and to Amicus, all treatment records and data as specified on a local basis.

The Amicus cross-functional internal review process must confirm that:

- The Amicus product is an investigational product, is currently under investigation in a clinical study, and/or the patient does not qualify for that study, or all clinical trials of the product have been completed, or is unapproved in a particular geography.
- Sufficient clinical safety and efficacy data in humans with the disease are available and such data, including appropriate dose, indicate that the potential benefits of Expanded Access of the investigational product outweigh any known or potential risks, as determined by Amicus and the treating physician or qualified HCP.

- There is adequate supply of the investigational medicine to meet all needs for patients enrolled in ongoing clinical trials, and that designating supply for an Expanded Access treatment use will not compromise supply or otherwise postpone providing the new treatment, once approved, to the broader patient population.
- Providing expanded access to an investigational drug does not negatively influence an ongoing clinical development program.
- Any additional requirements by the relevant regulatory authority or institution have been met.

Making a Request for Expanded Access

Requests for expanded access to an Amicus investigational medicine must be made by the patient's treating physician or qualified HCP. Physicians or qualified healthcare providers may submit a request by completing the intake form found on Amicus' website or obtain additional information from the Amicus Medical Affairs representative in their area. As with any situation involving the care and concern for individuals living with rare and orphan diseases, all information submitted as part of a request will be maintained in the strictest of confidence, adhering to all applicable patient data privacy regulations, and used solely for the purpose of evaluating a patient's eligibility for expanded access. Confirmation of receipt of written requests will be made within three (3) working days. Decisions will be made as quickly as possible and the requesting physician notified.

Requests for early access cannot be made directly by an individual patient or a patient's parent/legal guardian or caregiver. Patients and caregivers seeking general information may reach out to Amicus' Global Patient & Professional Advocacy Department at patientadvocacy@amicusrx.com or call toll-free in the United States and Canada at 1-866-9AMICUS (1-866-926-4287).