Managed Access to Investigational Drugs

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 next-generation therapies for people living with rare diseases. And with the development of medicines designed to satisfy their unmet medical needs, comes the promise that these medicines will be fairly priced and broadly accessible. There are different pathways to gain access to medicines, both pre- and post-approval. The primary focus here is on pre-approval access.

The best way to provide broad access to our medicines is through marketing authorization, which respects the sovereignty of each country's health authority to conduct timely review, and to approve or reject the investigational medicine based upon supporting data and relevant inspections. The approval path enables access to as many people as possible.

However, given the long development and reimbursement timelines necessary to reach marketing authorization, as well as the inability to achieve marketing authorization in all geographies, we recognize there may be a need for access to our medicines before or outside of marketing authorization.

Amicus is committed to carefully designed and recognized pathways to access, especially through clinical research. In limited circumstances, we may be able to provide access in countries that do not have clinical study sites or will not soon provide market authorization. However, as a growing rare disease company there are several medical, regulatory, supply, and cost-based factors that we must consider, as explained below. And these factors may be different when applied to different technologies and/or different diseases.

Paths to Access

The first of the different paths to access 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 in collaboration with regulatory authorities, including the criteria necessary for volunteer participants, and by also considering insights shared by rare disease patient community advisors. Studies are closely monitored and can be spread out over many years. People wanting to help advance disease knowledge and who meet the proper entry criteria may participate in these clinical studies.

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

Managed Access: Prespecified

Sometimes, however, someone may not qualify for a clinical study or there may be no studies available from Amicus or another sponsor. Depending upon where a person lives and the local health authority, there may be other paths toward access. These options to access may have slightly different names or approaches: some are prespecified, such as Named Patient Program, Named Patient Use or Authorization for Temporary Use (ATU), and others are not prespecified, such as Expanded Use, Compassionate Use or Early Access, depending upon geography and what the respective regulatory authority may allow. Regardless of the name, Amicus has reporting requirements to health authorities about those who receive access under all plans.

Managed Access: **Expanded**

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 *any* medically appropriate clinical studies. For individual access requests not falling under a prespecified approved pathway, Amicus is committed to careful review of all physician-submitted requests for expanded access on a case-by-case basis. For such a review, there are several factors taken into consideration, including but not limited to: a person's current medical condition based upon information provided by their requesting physician; currently available treatment options, including clinical studies or approved medicines; the known safety and efficacy of the Amicus investigational medicine; available supply and distribution of medicine; applicable regulations, if any, among other pre-determined criteria for the patient and guidelines for the company. Expanded access is also considered based upon anticipated marketing authorization timing.

In collaboration with regulatory authorities, a patient may receive expanded access to Amicus investigational and pre-reimbursed products before such products have received regulatory approval, provided that specific requirements are met. These requirements are discussed in detail with the requesting physician or healthcare professional (HCP) providing medical to the individual.

As a growing rare disease company, Amicus must be able to potentially provide a lifetime supply of free medicine for a chronic condition, fully considering the manufacturing costs associated with that medicine, ongoing distribution costs of getting the medicine to a country where Amicus does not operate, and other related costs.

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 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. Once all necessary information has been received,

requests will be reviewed and decisions will be made as quickly as possible, and the requesting physician notified.

Requests for expanded 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