Policy on Expanded Access to Investigational Therapies

Background

Oftentimes, patients who are suffering from severe or life-threatening conditions may seek access to an investigational therapy prior to those therapies being approved by a regulatory body. In these cases, patients may make a request directly to a company to obtain access to a therapy through a program called expanded access, which is sometimes also referred to as compassionate use. Expanded access is defined as use of an experimental drug outside of a clinical trial and requires the approval of a regulatory body like the Food and Drug Administration in the United States, or the European Medicines Agency in the European Union.

uniQure Policy

uniQure is committed to the promise of delivering transformative gene therapy products to patients with serious unmet medical needs and feels that the best way to protect the interests of these patients is to preserve the integrity of the clinical trials assessing the safety and efficacy of its investigational therapies. In certain cases, physicians may identify patients who, for various reasons, cannot participate in clinical trials but who they believe might benefit from access to an unapproved uniQure investigational therapy. In considering the provision of expanded access, uniQure considers factors regarding the risk/benefit, such as whether or not there is substantial scientific evidence to support both the safety and efficacy of an investigational therapy for a particular indication. Typically, investigational therapies need to reach phase 3 clinical trials before such a level of substantial safety and efficacy data exists.

Given uniQure's focus on the development of gene therapy products, there are additional considerations for expanded access. Gene therapy is a relatively new technology, whereby therapy is often given in a single dose during a patient's lifetime. Clinical trials evaluating gene therapy seek to address long term safety, durability of treatment response, and the potential for loss of some treatment effects over periods of time.

After careful consideration, uniQure has decided not to provide expanded access to any of our investigational gene therapies at this time. We understand that there is a high unmet medical need in many of the therapeutic areas in which we work, where these gene therapies have the potential to be transformative treatment options. Given the significance of the concerns associated with providing expanded access in a safe, equitable, and appropriate way, uniQure has made the decision to focus on completing ongoing clinical trials with the hope of ensuring wider and faster patient access.

The United States 21st Century Cures Act requires companies to publicly provide their policies and procedures for processing and assessing requests for expanded access. For any future expanded access programs uniQure might establish, the following principles will be used to evaluate requests for expanded access under those programs:

- The request must come from a qualified treating physician.

- The patient's illness must be serious or life-threatening with no other alternate treatment options for the patient (including approved therapies or enrolling in a clinical trial).
- Sufficient scientific/medical evidence must exist to support the appropriate dosing of the investigational therapy and that the potential benefit to the patient would likely outweigh the potential risks, based on available safety and efficacy data, as recommended by the treating physician and further assessed by uniQure.
- The patient's underlying medical conditions must not pose safety risks that have not been sufficiently studied.
- Delivery and administration of the investigational therapy outside of the clinical trial setting must be feasible, including ensuring that there are appropriate healthcare facilities available to administer the investigational therapy, manage potential side effects, and ensure ongoing follow-up as needed.
- uniQure must have an adequate supply of the investigational therapy and the ability to provide the investigational therapy fairly and equitably, ensuring adequate availability for ongoing clinical trials.
- Expanded access requests cannot compromise the scientific validity of ongoing uniQure clinical development, interfere with or delay current or anticipated clinical studies or regulatory submissions due to uniQure's mission to provide approved gene therapies to as many patients as possible as soon as possible.

By law, requests for expanded access and/or compassionate use must be made by a treating physician on behalf of a patient. These requests are referred to an internal team at uniQure for evaluation. uniQure will review the requests according to these procedures and may approve or deny any request for expanded access in its sole discretion. If a request for expanded access were to be approved, the physician must agree to comply with all applicable uniQure and local regulatory requirements, including obtaining appropriate informed consent, safety reporting, adverse event collection, and long-term follow-up consistent with local health authority requirements for Gene Therapy. Further, all necessary regulatory and institutional approvals must be obtained to allow for the administration of the investigational therapy. Expanded access would be considered only in countries where expanded access is permitted by local authorities and where uniQure has adequate resources, including, but not limited to, safety monitoring, to comply with all local regulatory requirements.

uniQure encourages patient education and participation in the process for determining whether expanded access to investigational gene therapies is appropriate for a particular patient. For additional information, you should speak with your physician, who can contact expandedaccess@uniqure.com on your behalf. uniQure will acknowledge receipt of emails sent to this address within five (5) business days. In accordance with the 21st Century Cures Act, uniQure reserves the right to revise this policy at any time.