uniQure

uniQure is committed to delivering transformative gene therapy products to patients with serious unmet medical needs. Our policy on responding to requests to provide expanded access to our investigational therapies to patients is outlined below.

Expanded access is defined as use of an investigational therapy outside of a clinical trial. A clinical trial is an approved research study conducted by trained physicians where comprehensive safety and efficacy data are collected on an investigational therapy to determine the therapy's risk/benefit profile for patients. uniQure encourages patient participation in our clinical trials to access our investigational gene therapies.

Rarely, physicians may identify patients with serious diseases or conditions who cannot participate in our clinical trials but who might benefit from access to an unapproved uniQure investigational therapy. When that occurs, uniQure supports expanded access and compassionate use programs when there is substantial scientific evidence to support both the safety and the efficacy of an investigational therapy for an indication, when it is logistically practicable, and when it makes good sense for the individual patient.

Consistent with the US Food and Drug Administration (US FDA) and other regulatory agencies' guidelines, uniQure considers several factors when evaluating a request for expanded access to investigational therapies being developed by uniQure. They include:

- 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. (Generally, safety information to support expanded access would be obtained from a completed Phase I study.)
- 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.

- 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 programs 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.

A qualified treating physician must make the request for expanded access and/or compassionate use and will be referred to an internal team at uniQure for evaluation. The physician must agree to comply with all applicable uniQure and local regulatory requirements, including 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 the administration of the investigational therapy. uniQure limits expanded access to 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 or contact expandedaccess@uniqure.com . uniQure will acknowledge receipt of emails sent to this address within five business days. uniQure will review all requests according to uniQure procedures and notify the requestor of the determination. uniQure reserves the right to revise this policy at any time and to accept or deny any request for expanded access in its sole discretion.