

## **United Therapeutics Corporation Expanded Access Program for an Investigational Drug**

Expanded access, also known as compassionate use, may be an option for a limited number of individuals with serious or life-threatening conditions, who have exhausted all available medical options and do not qualify for a clinical trial. In such rare cases, regulators may grant permission for United Therapeutics to provide a treating physician with an unapproved drug for that patient. It is important, however, to remember that investigational drugs have not yet received local regulatory authority approval; potential risks and benefits have not yet been established. Doctors and patients should consider all possible benefits and risks when seeking expanded access to an unapproved product.

In making decisions about expanded access, United Therapeutics considers the patient's case and the available medical and scientific information about the investigational medicine, while ensuring that expanded access is administered ethically, maintaining fairness, promoting good, and minimizing the risk of harm for both current and future patients.

In order to be considered under the United Therapeutics Expanded Access Program, a treating physician, on behalf of a specific patient, must submit a request to United Therapeutics on their own accord. This request should include the information identified below and should be emailed to [druginformation@unither.com](mailto:druginformation@unither.com). Once United Therapeutics has received the request and all requested information, we will acknowledge receipt of the request within seven (7) business days. Request will then be routed for review by the United Therapeutics Expanded Access Review Committee. If the request is approved by United Therapeutics, the country regulatory authority will need to grant final approval before the investigational medicine can be shipped to the requesting physician.

The following general criteria must be met for United Therapeutics to consider granting expanded access to an investigational medicine:

- The patient has a sufficiently serious or immediately life-threatening illness with no comparable or satisfactory alternative therapies.
- The patient's physician has determined that there is no comparable or satisfactory therapy, either approved or investigational, available to diagnose, monitor, or treat the patient's disease or condition (e.g., due to geographical or entry-related constraints).
- The patient has a disease for which there is sufficient evidence that a clinically meaningful benefit may be expected (i.e., available scientific evidence taken as a whole, provides a reasonable basis to conclude that the drug may be effective) from the use of the investigational medicine, and the benefits outweigh the known or anticipated risks.
- Sufficient evidence of safety indicating that patients in the specific patient population would not be exposed to unreasonable risk if provided the medicine.
- The investigational medicine is currently in clinical development - that is, it is currently being studied in humans and the patient is unable to obtain the investigational drug under an IND or participate in a clinical trial.
- Providing the investigational medicine for the requested use will not interfere with the initiation, conduct, or completion of clinical trials or with the sustainable and equitable access to other patients who do not have alternative treatment options.

For more information about our Expanded Access Program, physicians may contact United Therapeutics at (phone/fax) 1-877-522-2950 or (email) [druginformation@unither.com](mailto:druginformation@unither.com).