

Epirium Bio Statement On Compassionate Use

Epirium Bio is committed to developing safe and effective therapies for intractable neuromuscular and neurodegenerative diseases associated with mitochondrial depletion, as well as primary mitochondrial disorders, and providing those therapies to the broadest group of patients as quickly as possible. We also recognize that there are many diverse conditions in which mitochondrial depletion or dysfunction are a key component of the disease process. As part of our commitment to the rare disease community, we will support compassionate use / expanded access programs* when we have substantial scientific evidence to support both the safety and the potential efficacy of an investigational medical product for a given indication, and when it is logistically practicable.

Epirium has developed a process for determining whether the company will provide an experimental therapy under compassionate use.

In the first step, the company will evaluate whether:

- there is substantial scientific evidence to support both the safety and the efficacy of an investigational medical product for a particular indication;
- it has been established that access on a compassionate use basis will not compromise clinical trials or the regulatory pathway for an investigational medical product;
- there is adequate supply of the investigational medical product; and
- the investigational medical product can be administered – and it is logistically feasible to make it available – outside of the clinical trial setting.

If the company decides that, under the first step, availability of the investigational medical product on a compassionate use basis is possible, then the company will evaluate an individual's request for access.

This second step uses the following criteria:

- the patient has a serious disease or condition, or whose life is immediately threatened by their disease or condition;
- there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition;
- patient enrollment in a clinical trial is not possible;
- potential patient benefit justifies the potential risks of treatment;
- providing the investigational medical product will not interfere with investigational trials that could support a medical product's development or marketing approval for the treatment indication, and;
- all necessary regulatory/institutional approvals have been obtained to allow the administration of the investigational medical product.

Requests for access to investigational medical products must be made by a qualified and licensed physician and will be evaluated by Epirium. Patients interested in seeking expanded access to an Epirium investigational medical product should talk to their physician. Qualified and licensed physicians may

make compassionate use / expanded access requests by contacting Epirium by e-mail at info@epirium.com. Epirium anticipates it will acknowledge receipt of such requests within five business days of their receipt

* Compassionate use programs include requests for access under section 561(b) of the Federal Food, Drug, and Cosmetic Act, as well as 'Right to Try' legislation.