

Dear Friends,

I am writing to inform you of some very important developments in the management of the shortage of Cerezyme which is so severely affecting Gaucher patients globally.

An Emergency meeting of the European Working Group on Gaucher Disease (EWGGD) was held in Bad Honnef Germany on the 10<sup>th</sup> September. The meeting was called at the request of Professor Hans Aerts, Chairman of the EWGGD and members of the board of the European Gaucher Alliance (EGA) as well as at the request of many senior members and physicians of the EWGGD. The meeting was Chaired and coordinated by Professor Timothy Cox (Vice Chairman of the EWGGD) and Professor Carla Hollak and hosted by Professor Stephan Vom Dahl who secured support for the meeting from the German Working Group on Inborn Errors of Metabolism in Adults.

Physicians from Europe presented details of the availability of treatment in their countries and Pharmaceutical companies (Genzyme, Protalix, Shire and Actelion) were invited to present updated information on their products to the meeting.

It was universally agreed that there should be a European strategy to address the current crisis and two important outcomes of the meeting were:

1. The establishment of a EWGGD Cerezyme Emergency Treatment Protocol
2. This has been created for patients with type 1 and III Gaucher disease who are at high risk of developing progressive disease or complications during the Cerezyme shortage. An emergency supply of enzyme is to be made available for the most urgent untreated cases in Europe to which physicians can apply on behalf of their patients. Applications will be reviewed by a Medical Advisory Board made up of European Gaucher Experts.

**Full details of the qualification criteria and an application form for this emergency supply can be found on this link.**

3. A paper will be published in "Blood Cells, Molecules and Diseases". This will be published online in the next 1 – 2 weeks. The paper will include a position statement based on the findings of the meeting, key recommendations for the identification and monitoring of at-risk patients threatened by the abrupt withdrawal of treatment, suggestions for the equitable distribution of the available Cerezyme, and access to other possible treatments including those that have completed phase 3 clinical trials but have not yet been licensed..

**A link to this paper will be posted here once it has been published.**

The importance of this EWGGD meeting and its outcomes cannot be underestimated. The working group has a long history of collaborative activity

for the benefit of Gaucher patients and the experts at this meeting have established by their actions concrete steps to help the most needy of patients at a European level at this most difficult time.

Yours truly,

Jeremy Manuel  
Chairman European Gaucher Alliance