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Force Majeure: Therapeutic measures in response to restricted supply of imiglucerase (Cerezyme) for patients with Gaucher disease☆

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Abstract

Gaucher disease is the first lysosomal disorder for which clinically effective enzyme replacement therapy has been introduced. Lifelong treatment with imiglucerase, the recombinant glucocerebrosidase manufactured by the Genzyme Corporation (MA, USA), is administered intravenously — usually at biweekly intervals. An acute shortage of imiglucerase (to 20% of prior global supply) has occurred as a result of viral contamination of the production facility; production was halted, and a full supply of imiglucerase is not anticipated until January 2010. An urgent meeting of physicians, researchers, and patients was convened through the agency of the European Working Group for Gaucher Disease; this was instigated by patients internationally represented by the European Gaucher Alliance. Here we present a position statement based on the findings of the group, with key recommendations about identification and monitoring of at-risk patients threatened by the abrupt withdrawal of treatment, the equitable distribution of residual imiglucerase — and access to alternative treatments including those that have completed phase III clinical trials but have not yet been licensed.

Keywords: Gaucher disease; Cerezyme; Imiglucerase; Vesivirus; Europe

Abbreviations: CEAP, Cerezyme Emergency Access Program; ECAP, European Cerezyme Access Program; EGA, European Gaucher Alliance; EMEA, European Medicines Agency; ERT, enzyme replacement therapy; EWGGD, European Working Group on Gaucher Disease; ESGLD, European Study Group on Lysosomal Diseases; FDA, Food and Drug Administration; GD, Gaucher disease; h-GCD, human cell-derived glucocerebrosidase (velaglucerase); ICGG, International Collaborative Gaucher Group; MPS II, mucopolysaccharidosis type II, Hunter disease; NP-C disease, Niemann-Pick disease type C; pr-GCD, plant-cell derived human glucocerebrosidase (taliglucerase); SRT, substrate reduction therapy