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A Correspondência deve ser encaminhada para: Editor da Revista HCPA - Largo Eduardo Zaccaro Faraco - Rua Ramiro Barcelos, 2350
90035-903 - Porto Alegre, RS - Tel: +55-51-2101.8304 - www.hcpa.ufrgs.br

MAINTAINING CLINICAL EFFICACY WITH COST-REDUCTION IN THE TREATMENT OF GAUCHER DISEASE: AN EXAMPLE OF A SUCCESSFUL EXPERIENCE IN THE SOUTH OF BRAZIL.

BÁRBARA CORRÊA KRUG; IDA VANESSA DOEDERLEIN SCHWARTZ; PAULO DORNELLES PICON; ROBERTO GIUGLIANI; JOÃO GABBARDO DOS REIS

Introduction: Gaucher disease (GD) is a lysosomal storage disorder treated with imiglucerase one of the world most expensive drugs. In 2004, total cost of this treatment was U\$ 48.56M (425 patients) for the Brazilian Ministry of Health. Brazilian guidelines (BG) were developed to improve cost-effectiveness of this enzyme replacement therapy. According to this evidence-based guidelines GD patients should be seen at local Reference Centers (RC) and imiglucerase should be started and or maintained with the lowest clinically-effective dose. Methods: First Brazilian local RC created was in Porto Alegre-RS in July 2003. All 20 patients have already been treated with imiglucerase given i.v. every 15 days. After RC creation dose was adjusted according to the BG severity-score. Clinical and lab evaluation were performed regularly and dose adjusted every 6 months. Results: Mean age was 18,7y (6-51). Seventeen patients presented type I and 3 type III GD. Before RC the mean imiglucerase dose was 47.4 (type I) and 75.3 (type III) U/kg. After 18 months number of patients was 23, the mean imiglucerase dose was 22.4 (type I) and 60 (type III) U/kg. All clinical parameters showed continuous improvement as the hemoglobin increased from 10.75 to 12.17 g/dl. Patients declared to be highly satisfied with the RC. The RC treatment of this sample saved U\$ 2.200.000.00 in 18 months. Conclusion: implementation of Brazilian Guidelines in a RC is a cost-effective intervention for GD. All regional local health state authorities should follow that example. This intervention might be useful for other countries.