P-083 - ELIGLUSTAT FOR ADULT PATIENTS WITH GAUCHER DISEASE TYPE 1: EFFECTIVENESS AFTER 6 YEARS OF TREATMENT EVALUATED IN THREE ARGENTINEAN CENTERS

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**INTRODUCTION:** One option currently available for the treatment of type 1 Gaucher disease (GD) is substrate reduction therapy (SRT) with Eliglustat. This therapy has recently been approved and data on its effectiveness in Argentina is not available. **OBJECTIVES:** to evaluate the effectiveness of Eliglustat used during 6 years in adult patients with GD in three centers of Argentina. **METHODS:** Twenty-two patients ≥18 years with GD admitted to the phase III clinical trial ENCORE (GZGD012607) and followed for at least 6 years were included. For the analysis, patient follow-up was separated into three stages: 1) Stage 1 comprised the period of patient randomization and follow-up during 12 months. 2) stage 2, comprised the time between the 12 months and the 4 years of follow-up (extension of the ENCORE study); 3) Stage 3, “real life”, included the follow-up of patients who continued for 2 years in treatment with Eliglustat and obtained data recorded by treating physicians during the follow-up outside the clinical trial. The main outcome evaluated consisted in the maintenance of therapeutic goals (TG). **RESULTS:** Previous to the inclusion to the ENCORE study 95.5 % of the patients had different degree of bone lesion: bone marrow infiltration (95.5%), Erlenmeyer flask deformity (59%), chronic bone infarcts (59%) and chronic avascular necrosis (50%). At the end of stage 1 eliglustat was not inferior to imiglucerase regarding TG accomplishment. In Stage 2, all patients continued to fullfill AT TG. There were no new bone lesions. Compliance to Eliglustat was 95 %. At the end of Stage 3, all patients still reached TG. There were no new bone lesions but a decreased in compliance to Eliglustat was found 65% (p=0.001). **CONCLUSION:** In our experience Eliglustat is a first line treatment of choice that can reach TG, but it requires a strict control of patients compliance to reach TG. With a follow-up time of 6.3 years (2 years out of the ENCORE study), all patients under treatment with Eliglustat maintained the TG. The bone lesions after 6 years of follow-up remained stable without new acute lesions or pain crises.