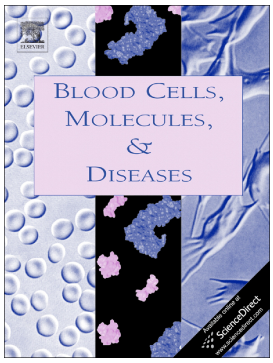

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TALIGLUCERASE ALFA IN GAUCHER DISEASE: DESCRIPTION OF A BRAZILIAN EXPERIENCE

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Abstract:

.We evaluated retrospectively, efficacy and safety of taliglucerase alfa for Gaucher Disease in a Brazilian population. Thirteen patients were included for efficacy analysis only one of them naïve to enzyme replacement therapy. All the parameters evaluated remained stable throughout treatment (mean duration 3,5 years). Only three patients (out of 35) had to discontinue treatment due to a serious adverse event. In conclusion, treatment with taliglucerase alfa was found to be safe and efficient.

Keywords: Gaucher disease; lysosomal storage diseases; enzyme replacement therapy; taliglucerase alfa; imiglucerase; velaglucerase

Abbreviations: GD, Gaucher disease; ERT, enzyme replacement therapy; FDA, Food and Drug Administration, AST, aspartate aminotransferase; ALT, alanine aminotransferase; SOC, System Organ Classification;