P-053 - HEALTH-RELATED QUALITY OF LIFE: A FOLLOW-UP TO ENZYMATIC REPLACEMENT THERAPY IN ADULT PATIENTS WITH MUCOPOLYSACCHARIDOSIS

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**INTRODUCTION:** The impact of enzyme replacement therapy (ERT) evaluated in phase I and II clinical trials has clinical results measured on physical tests, which assess aspects such as exercise tolerance, lung capacity, among others. It is necessary that this clinical impact of ERT in patients with Mucopolysaccharidosis be evaluated under a view of quality of life related to health in its social, psychological and emotional dimensions. **OBJECTIVE:** To evaluate the impact of ERT on the quality of life related to health in patients with Mucopolysaccharidosis type IV A in a third level institution Pereira-Colombia

**MATERIALS AND METHODS:** A follow-up was carried out on a series of cases of patients with Mucopolysaccharidosis (MPS) type IV A (children and adults) confirmed by molecular tests. An evaluation of the quality of life related to health was carried out before the start of enzyme therapy, and after a year of treatment a second evaluation was performed. Descriptive statistics and nonparametric inference tests were applied. **RESULTS:** Four adult patients were followed up, with an average age of 32 years. At the beginning of the therapy the domains of quality of life show a moderate affectation (score 40 to 60), significant clinical changes are detected in all the domains with an improvement in the quality of life, Health domain (difference 4 points p = 0.875) psychological (difference 30 points p = 0.065) social (difference 32 0.0625) environment (difference 11 p = 0.31), however statistically this is probably not detected in the few patients in the sample. **CONCLUSIONS:** Enzymatic therapy for adults patients with mucopolysaccharidosis type IV A brings improvement in aspects of mobility, pain reduction, resulting in changes in the health, social and psychological domains.