

hydroxylysine and glucosylgalactosyl hydroxylysine were significantly higher in MPS patients compared to age-matched controls. The correlation between changes in glycosylated lysine and GAG expression levels post treatment was found only in 10 out of 19 patients. Conclusion: Whilst modified lysine expression was increased similarly to GAG expression pre-treatment, only in approximately half of the patients, post-treatment modified lysine was reduced. As MPS bone disease has limited response to ERT, our findings suggest that the level of free glycosylated collagen could assist in understanding the underlying mechanism, and in the search for new therapies. Further longitudinal studies are needed to confirm if this marker can be used in prognosis and treatment monitoring.

#### 645 - Quality of Life of a Group of Patients With Mucopolysaccharidosis Type IVA and VI of COLOMBIA and Argentina in Enzyme Replacement Therapy

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**Objective:** To assess the quality of life of a group of patients with Mucopolysaccharidosis type IVA and VI of Colombia and Argentina in enzyme replacement therapy. **Materials and methods:** A cross-sectional descriptive study was carried out in Pereira Colombia and Rosario Argentina. All the patients with clinical and biochemical diagnosis of Morquio and Maroteaux Lammy syndrome were included in this study. A trained assistant applied the following instruments: for adults, the WHOQOL-BREF questionnaire, developed and validated by the Group WHOQOL of the World Health Organization (WHO). This questionnaire accounts for 4 domains that are: Physical, Psychological, Social, Environment. For children (under 18 years of age), their quality of life was assessed using the Stanford Health Assessment Questionnaire (SHAQ). The SHAQ measures eight domains as follows: dressing and grooming, getting up, walking, eating, hygiene, reaching, grabbing, and activities. **Results:** A total of 18 patients, 12 children and 6 adults were evaluated. Children: Consolidated results of the CHQ according to their domains, presents significant affectation in dressing, hygiene, followed by walking domains and other activities. A negative relationship between age and quality of life was evidenced. In relation to the total score of the quality of life, it has a rather important affectation (mean score 2 of a score of 1-4) accompanied by an assessment on a visual analog scale of pain (1-10 points) On average is 3 Points, showing a slight of slight intensity pain. In some patients

their mobility, as evidenced by the difficulty in, and activities for personal hygiene, increases with age. There is a moderate correlation between age and the degree of pain reported by the patients. Having specific instruments for Mucopolysaccharidosis, a sure quality of life is necessary to better objective quality of life. Available WHO surveys are implemented for matoid arthritis and other chronic diseases. The instrument used was easy to apply, logical response correlation with the clinically state of the patients. The results are similar to those obtained in cohorts of patients with chronic pathologies. However further research

#### 646 - Use of Methylphenidate for the Treatment of Hyperactivity in Children With Hunter Syndrome

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Hunter syndrome (MPSII) is an X-linked chromosomal disorder due to deficiency of the lysosomal enzyme iduronate 2-sulfatase. It is clinically divided into neuronopathic form (about 2/3 of cases) and non-neuronopathic phenotype. Hyperactive behavior is a very common clinical feature in MPSII due to the neuronopathic form. However, literature evidence about treatment of hyperactive agents for hyperactivity in MPSII is scarce. We evaluate methylphenidate (MPH) safety and efficacy in a cohort of pediatric patients with Hunter syndrome. Seven MPS II patients (mean age 5 years, range 3-16) were put on MPH between 2010 and 2016. Mean treatment was 28 months (range 3 to 50 months). All patients received weekly infusions of IV iduronate. Cognitive assessments were done in all the patients. A retrospective chart review obtained comorbid symptoms, medication, vital signs, side effects, and MPH efficacy. **Results:** Five of seven MPSII neuronopathic involvement defined for the patient group. Progressive decline over time, all of them still with hyperactive behavior. Additional comorbidities included seizures (1), atrial arrhythmia (1). Four patients reported MPH side effects: appetite suppression (2) anxiety (1) and obsessive behaviors (2) marked rebound effect (1). There were no statistically significant changes in EKG or blood pressure 12 and 24 months after initiation. Hyperactivity improvement was reported by parents in 4/7 patients for at least 6 months. Risperidone was used as concomitant medication in 3/7 patients. Three of seven patients remained