ALPHA-1 ANTITRIPSIN THERAPY - SINGLE INSTITUTION EXPERIENCE

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Introduction: Alpha-1-antitrypsin deficiency (AATD) is a rare genetic disorder associated with the development of liver and lung disease. It is defined as a low serum alpha-1-antitrypsin concentration. Exposure to smoke is the major risk factor for the development of lung disease most commonly characterised as chronic obstructive lung disease (COPD), emphysema or/and bronchiectasis. Intravenous substitution therapy (regular infusion of purified human AAT) has been recommended for individuals with established fixed airflow obstruction.

Methods: We retrospectively analyzed post-bronchodilator FEV1 (ml) and gas transfer DLCO (% predicted) during the therapy with human AAT (at the begining, after 12, 24 and 36 months of therapy) in our cohort of 13 patients with alpha-1-antitrypsin deficiency to determine whether there is a significant rates of decline (for FEV1 ≥200ml and for DLCO ≥10%).

Results: There were 13 patients, 10 males and 3 females, with median age of 51 years (38 to 65 years), 11 were former smokers (19 packs/years) and 2 were non-smokers. For 11 patients genotype was Pi*ZZ, one had Pi*MZ and one Pi*00. At the begining of the treatment, 12 patients had FEV1/FVC <70% (mean 42,74; SD 14,29), and one had >70% (76,49%). According to our results, post-bronchodilatator spirometry showed no statistically significant changes in FEV1 (mean 1,49 vs 1,39; p=0,108) and DLCO (mean 41,46 vs 38,55; p=0,242) after 12 months of therapy. Statistically significant change was not shown for FEV1 (mean 1,42 vs 1,29; p=0,122) even after 24 months of therapy while for DLCO (mean 43,16 vs 36,59; p=0,01) there was a significant decline.
Statistical significant decline was recorded after 36 months for FEV1 (mean 1.42 vs 1.20; p=0.007) and for DLCO (mean 43.16 vs 35.58; p=0.023).

Conclusion: Our data did not show any significant changes in FEV1 and gas transfer during the first year of therapy with human AAT, but after 24 months we had a significant decline in DLCO. Despite a regular application of human AAT, after 36 months we recorded a significant decline in both, FEV1 and DLCO. For more detailed statistical analysis our group of patients is too small. However, randomised studies have reported the efficacy of human AAT therapy on mortality, FEV1 decline and the frequency of exacerbations and have demonstrated the efficacy in preventing the loss of lung density.