

Alpha₁-Antitrypsin (AAT) To Treat Emphysema In AAT-Deficient Patients

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| Title of the study: | Multi-Center, Randomized Trial With I.V. Prolastin® to Evaluate Frequency of Exacerbations and Progression of Emphysema by Means of Multi-Slice CT Scans in Patients With Congenital Alpha-1-Antitrypsin Deficiency |
| Principal Investigator: | Asger Dirksen, MD |
| Publications (references): | These results have not yet been published in a peer-reviewed journal. |
| Clinical phase: | 2 |
| Number of patients: | The study population consisted of 77 randomized adult Caucasian men (n=41) and women (n=36) aged between 35 and 74 years. All study drugs were administered under a double blind, 38 subjects received Prolastin and 39 received placebo. |
| Summary of efficacy: | <p>The main analysis of the primary endpoint was the slope analysis of total lung capacity (TLC)-adjusted 15th percentile of lung density. The annual decline in lung density estimated from the slope for the Prolastin group was -1.384 ± 0.320 g/L and -2.241 ± 0.333 g/L for the placebo group. The estimated difference (Prolastin minus placebo) was 0.857 ± 0.461 ($P = 0.068$).</p> <p>A statistically significant treatment effect was demonstrated when an alternative analysis of covariance for changes from baseline to endpoint in 15th percentile point of lung density with change in lung volume as covariate was performed ($P = 0.049$).</p> |
| Summary of safety: | <p>Over the mean treatment duration of about 2 years (and longer for a smaller cohort), nearly all subjects (97% of each group) experienced at least 1 treatment emergent adverse event (TEAE). In most cases, the TEAE was of mild or moderate severity.</p> <p>In both treatment groups, the most frequent TEAEs were nasopharyngitis (about 60%), pneumonia (about 30 to 40%), and headache (about 25%). Sixty-eight serious adverse events occurred during the study: 28 in the Prolastin group and 40 in the placebo group. These 68 SAEs occurred in 10 (26.3%) Prolastin subjects and 18 (46.2%) placebo subjects. The majority of SAEs in both treatment groups were related to the subjects' pre-existing disease of pulmonary emphysema such as exacerbations, pneumonia and other respiratory disorders of dyspnea and pneumothorax. No SAE was fatal and no subject died during the course of the study. Two subjects in the placebo group withdrew from the study because of adverse events.</p> |