Treatment with A1-PI slows the progression of emphysema in Alpha-1 antitrypsin deficiency

May 21 2013

Treatment with an Alpha-1 proteinase inhibitor (A1-PI), a naturally occurring protein that protects lung tissue from breakdown and protects the lung's elasticity, is effective in slowing the progression of emphysema in patients with Alpha-1 antitrypsin deficiency (AATD), a life-threatening genetic disorder, according to a new study presented at the 2013 American Thoracic Society International Conference.

The study showed the efficacy of A1-PIin preventing the loss of lung tissue as measured by computed tomography (CT) scan lung density at full inspiration (TLC), which is a more sensitive measure of disease progression than conventional parameters.This is the first prospective study to demonstrate the efficacy and safety of augmentation therapy in a randomized, placebo-controlled trial using this parameter.

"Our experience of the last quarter century has been that augmentation therapy is associated with better preserved lung function and reduced mortality, " said lead author Kenneth R. Chapman, M.D., director of the Asthma and Airway Centre of the University Health Network, in Toronto. "This randomized, placebo-controlled trial using a sensitive measure of lung density adds the most rigorous evidence to date that augmentation therapy slows the progression of emphysema in patients with Alpha-1 antitrypsin deficiency. The effect of A1-PI seen in this trial was both clinically and statistically significant, finally confirming its benefit in preventing the loss of lung tissue in patients with this

potentially debilitating disease."

Chapman added that preliminary data from an extension trial suggest that early treatment with A1-PIshows persistent efficacy in patients with AATD and emphysema. In both the A1-PI and placebo groups who elected to continue treatment withA1-PI 60 mg/kg weekly, the benefit in CT scan lung density decline continued.

AATD is a hereditary condition that can severely affect a patient's lung function. The condition is marked by a low level or absence of A1-PI, a natural protein that protects the lung from breakdown by inhibiting neutrophil elastase, and protects lung elasticity. AATD can lead to emphysema at a young age (