

Ambroxol Therapy for Patients With Type 1 Gaucher Disease and Suboptimal Response to Enzyme Replacement Therapy

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Ambroxol hydrochloride, an over-the-counter antitussive available in many markets , was identified as an interesting pharmacological chaperone. In addition to a mucolytic action, ambroxol has antioxidant and anti-inflammatory properties. Importantly, ambroxol therapy was found safe when given to pregnant women for prevention of neonatal respiratory distress syndrome .

Thus, ambroxol, an oral available drug on the market, may be a safe option for GD patients with potential disease-specific efficacy and should be expanded into a clinical trial using higher doses and placebo-controlled design. The investigators propose to start with a phase II study for patients with type 1 GD and suboptimal response to ERT. In addition the investigators plan to open an international registry of patients with GD currently receiving ambroxol (off study).