Betaine, a promising new agent for patients with nonalcoholic steatohepatitis: results of a pilot study.

Abstract

OBJECTIVES: No effective therapy currently exists for patients with nonalcoholic steatohepatitis (NASH). Betaine, a naturally occurring metabolite of choline, has been shown to raise S-adenosylmethionine (SAM) levels that may in turn play a role in decreasing hepatic steatosis. Our aim was to determine the safety and effects of betaine on liver biochemistries and histological markers of disease activity in patients with NASH.

METHODS: Ten adult patients with NASH were enrolled. Patients received betaine anhydrous for oral solution (Cystadane) in two divided doses daily for 12 months. Seven out of 10 patients completed 1 yr of treatment with betaine.

RESULTS: A significant improvement in serum levels of aspartate aminotransferase ($p = 0.02$) and ALAT ($p = 0.007$) occurred during treatment. Aminotransferases normalized in three of seven patients, decreased by >50% in three of seven patients, and remained unchanged in one patient when compared to baseline values. A marked improvement in serum levels of aminotransferases (ALT -39%; AST -38%) also occurred during treatment in those patients who did not complete 1 yr of treatment. Similarly, a marked improvement in the degree of steatosis, necroinflammatory grade, and stage of fibrosis was noted at 1 yr of treatment with betaine. Transitory GI adverse events that did not require any dose reduction or discontinuation of betaine occurred in four patients.

CONCLUSIONS: Betaine is a safe and well tolerated drug that leads to a significant biochemical and histological improvement in patients with NASH. This novel agent deserves further evaluation in a randomized, placebo-controlled trial.