

Abstract

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A double-blind, parallel, multicenter comparison of L-acetylcarnitine with placebo on the attention deficit hyperactivity disorder in fragile X syndrome boys.

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BACKGROUND: Attention deficit hyperactivity disorder (ADHD) is a frequent behavioral problem in young boys with fragile X syndrome (FXS), and its treatment is critical for improving social ability. The short-term efficacy of stimulant medications like methylphenidate (MPH) is well established in children with ADHD. FXS boys treated with MPH have improved attention span and socialization skills; however their mood becomes unstable at higher doses. Therefore, alternative pharmacological treatment of ADHD symptoms is desirable. A recent study showed that carnitine has a beneficial effect on the hyperactive-impulsive behavior in boys with ADHD without side effects. Our previous placebo-controlled trial indicated that L-acetylcarnitine (LAC) reduces hyperactivity in FXS boys.

OBJECTIVE: The objective of this study was to determine the efficacy of LAC in a larger sample of FXS boys with ADHD.

METHODS: The study design was randomized, double blind placebo controlled, parallel, and multicenter (with eight centers involved in Italy, France, and Spain). Sixty-three FXS males with ADHD (aged 6-13 years) were enrolled; 7 patients dropped out, 56 completed the one-year treatment, and 51 were included in the statistical analysis.

RESULTS: Both groups improved their behavior, showing that psychosocial intervention has a significant therapeutic effect. However, we observed a stronger reduction of hyperactivity and improvement of social behavior in patients treated with LAC, compared with the placebo group, as determined by the Conners' Global Index Parents and the Vineland Adaptive Behavior Scale.

CONCLUSIONS: Our results show that LAC (20-50 mg/kg/day) represents a safe alternative to the use of stimulant drugs for the treatment of ADHD in FXS children.

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