Compliance and tolerability of statin treatment in children with familial hypercholesterolemia: preliminary results of a 10-year follow-up study

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Background: Statins are increasingly considered for certain pediatric conditions to prevent atherosclerosis. In adults, compliance to cholesterol-lowering drugs in clinical practice is a significant problem, partly due to the occurrence and severity of side effects. In this study, we evaluated the compliance and tolerability of 10-year statin treatment in children with familial hypercholesterolemia (FH).

Methods: All 214 FH children, who were randomized between 1997 and 1999 (8-18 years) into a placebo-controlled trial evaluating the 2-year efficacy and safety of pravastatin, were eligible. After the study, all children were continued on statins and were followed for 10 years. Questionnaires including the MARS and MASRI tools on compliance were taken.

Results: Follow-up was successful in 90% of subjects (age 18 to 30 years). After 10-years of follow-up, 85.9% reported to use lipid-lowering drugs, mostly statins. The main reasons to discontinue therapy were unwillingness to take medication (6.5%), pregnancy (3.8%) and side effects (3.7%). Of the patients who remained on treatment, 83% achieved ≥ 80% compliance. During follow-up, 16.4% of subjects experienced side effects, mostly muscle and joints symptoms or gastro-intestinal complaints. Rhabdomylosis or major adverse events were not reported.

Conclusions: Subjects with FH who started statin therapy in childhood with intensive follow-up in a academic pediatric lipid center, showed excellent compliance after ten years. Furthermore, statin therapy was well-tolerated; only a small minority discontinued therapy because of side effects and reported side effects were mild.

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