Follow-up shows benefit of statin therapy for children with inherited cholesterol disorder

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Ten-year follow-up of children who have been taking statin therapy for an inherited cholesterol disorder showed benefit on a measure of atherosclerosis, although levels of low-density lipoprotein suggested that stronger or earlier initiation of statin therapy may be warranted, according to a study in the September 10 issue of *JAMA*.

Familial hypercholesterolemia (FH) is a prevalent (1:500 individuals) inherited disorder that strongly predisposes to premature atherosclerosis and subsequent cardiovascular disease. In children with FH, atherosclerosis progression is observed before puberty. Consequently, guidelines for FH treatment advocate initiation of statins in children as young as 8 years. However, long-term efficacy and safety data for statin therapy initiated during childhood do not exist, according to background information in the article.

D. Meeike Kusters, M.D., of the Academic Medical Center, Amsterdam, the Netherlands and colleagues followed up a group of children with FH receiving statin therapy until adulthood. The study included 214 children heterozygous (possessing two different forms of a particular gene, one inherited from each parent) for FH, living in the Netherlands, ages 8 to 18 years, who were randomly assigned between 1997 and 1999 into a 2-year, placebo-controlled trial of pravastatin. After the trial, all children received pravastatin and were followed up until March 2011 along with 95 unaffected siblings. After 10 years, all participants underwent a physical examination, fasted blood sample, assessment of family and medical history, including the occurrence of adverse events, and

measurement of carotid intima-media thickness (IMT; thickness of a wall of an artery), a validated marker of atherosclerosis.

Ten-year follow-up was achieved in 194 (91 percent) patients with FH and 83 (87 percent) siblings, all ages 18 to 30 years. The researchers found that statin treatment initiated during childhood in patients with FH was associated with normalization of carotid IMT progression. Moreover, earlier statin initiation was associated with thinner carotid IMT at follow-up. No serious adverse events were reported during follow-up.

The low-density lipoprotein levels of patients with FH at follow-up did not meet current treatment standards and carotid IMT was thicker than in unaffected siblings.

"More robust lipid-lowering therapy or earlier initiation of statins may be required to completely restore arterial wall morphology and avert cardiovascular events later in life in this high-risk population," the authors write.

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