

# Common cholesterol drug safe, may improve learning disabilities in patients with neurofibromatosis

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Researchers at Children's National Medical Center have found that a cholesterol-lowering statin drug appears to be safe in children with neurofibromatosis type 1 (NF1) and may improve learning disabilities, including verbal and nonverbal memory. This is the first time that the drug lovastatin has been studied in children with NF1. The study, led by Maria T. Acosta, MD, a pediatric neurologist and researcher at Children's National and clinical director and cognitive director of the Gilbert Family Neurofibromatosis Institute, appears in the October 2011 issue of *Pediatric Neurology*.

Lovastatin is currently approved by the U.S. [Food and Drug Administration](#) in adults and children for the treatment of high cholesterol. The drug works by inhibiting a specific enzyme in cholesterol biosynthesis. Previous animal studies have found that lovastatin affects a related molecular pathway that may be linked to cognitive deficits in neurofibromatosis.

"While we originally set out to determine the safety of lovastatin in NF1 patients, we also saw statistical improvements in memory and visual attention, which is a big step towards helping improve our patients' quality of life and in evaluating [biologic agents](#) which may be effective therapies for NF1," stated Dr. Acosta. "While this is a relatively small study, we now have strong baseline information, and we are working with other institutions in the country and throughout the world to

perform a definitive study to replicate these findings on a larger scale."

This Phase I study looked at the safety and efficacy of lovastatin as a treatment for patients with [neurofibromatosis type 1](#), which accounts for the majority of NF cases. Over a period of three months, 24 patients between ages 10-17 years received treatment with lovastatin. Patients were given cognitive functioning tests before and after treatment. All patients maintained normal cholesterol levels throughout the study, and there were some cognitive improvements in memory, [visual attention](#), and efficiency following treatment.

"The implications for all children with learning disabilities – not only those with NF1 – are of interest to the greater pediatric community, so we hope to move forward quickly through the Consortium to advance this research in NF1 patients in a timely manner," commented Roger Packer, MD, Senior Vice President of the Center for Neuroscience and Behavioral Medicine, Director of the Gilbert Family Neurofibromatosis Institute at Children's National, and Group Chair of the Department of Defense Neurofibromatosis Clinical Trials Consortium.

**More information:** Lovastatin as Treatment for Neurocognitive Deficits in Neurofibromatosis Type 1: Phase I Study,  
[www.sciencedirect.com/science/.../ii/S0887899411003146](http://www.sciencedirect.com/science/.../ii/S0887899411003146)

Provided by Children's National Medical Center

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