For children with severe hypercholesterolemia who fail to respond to dietary modifications, drug therapy may be necessary. Pharmacologic therapy is recommended for children older than 10 years old who have LDL cholesterol greater than 190 mg/dl without other risk factors or over 160 mg/dl in patients with two or more other risk factors or with a family history of early heart disease in a first degree relative. In young people who are considered to have individual risk conditions (such as diabetes, chronic kidney disease, Kawasaki disease with aneurysms, or heart transplant recipients), the threshold for medication is lower and may be considered when LDL values are greater than 130 mg/dl.

The use of medication in a child/adolescent needs to be a cooperative decision with the parents. Parents and patients should understand the available data related to statin use in young people particularly because prospective, long-term evidence-based practice is not practical or available for this population. Options for lipid lowering medications include bile acid-binding resins, 3-hydroxy-3-methylglutaryl coenzyme A (HMG-CoA) reductase inhibitors (statins), ezetimibe, and fibrates. Nicotinic acid is generally not used in children/adolescents.

The most recent guidelines on lipid abnormalities in children recommend treatment with statins if pharmacologic therapy is indicated after lifestyle modification has been attempted (Expert Panel on Integrated Guidelines for Cardiovascular Health and Risk Reduction in Children and Adolescents; and National Heart, Lung, and Blood Institute, 2011). Statins are effective in lowering LDL cholesterol. To a lesser degree, they also help lower triglycerides levels and can raise HDL cholesterol somewhat. Statins work by inhibiting the enzyme necessary for cholesterol synthesis. Statins are most effective when taken in the evening and are started at the lowest possible dose in young people. Blood work should be followed closely in children and adolescents and usually includes a fasting lipid profile, liver function tests, and creatinine kinase repeated a month or so after initiation, and then twice yearly as well as with any dosage changes.

Patients beginning therapy with a statin should be counseled regarding rare but potentially serious side effects (such as rhabdomyolysis) as well as more minor potential side effects. Patients should discontinue their medication and contact their