

Homozygous Familial Hypercholesterolemia

What to Expect: HoFH Treatment

Goal of Treatment

Homozygous Familial Hypercholesterolemia (HoFH) is the rare and most severe form of Familial Hypercholesterolemia (FH). HoFH can cause heart disease (including heart attacks and aortic valve disease) beginning in early childhood, if untreated. Treatment begins at diagnosis, regardless of the person's age.

HoFH is treatable. In fact, the same treatments used to lower Low-Density Lipoprotein Cholesterol (LDL-C) in FH are used to treat HoFH, though they may not be as effective. In addition, there are treatments specifically approved only for HoFH.

The goal of therapy is likewise similar to FH.

- **< 100 mg/dL** for adults who **do not** have cardiovascular disease or **< 130 mg/dL** for children.
- **< 70 mg/dL** for adults and children who **already have** cardiovascular disease.

A combination of treatments along with a healthy diet and exercise are needed to adequately lower LDL-C. It is especially important to work with a specialist who understands HoFH to find the right treatment plan for you.

HoFH Treatments



Statins

First line treatment for cholesterol lowering.

Statins decrease production of cholesterol in the liver and increase the function of LDL receptors that remove LDL-C from the bloodstream. It is an oral pill taken daily.



Ezetimibe

Ezetimibe works in the small intestine by decreasing cholesterol absorption and increasing the function of LDL receptors that remove LDL-C from the bloodstream. It is an oral pill taken daily.



PCSK9 Inhibitors

PCSK9 inhibitors help improve the function of LDL receptors that remove LDL-C from the bloodstream by inactivating PCSK9, a protein that destroys LDL receptors. It is a drug given by injection at home once or twice a month.

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Information provided for educational purposes only. Please consult your healthcare provider regarding your specific health needs.

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HoFH Treatments



Bempedoic Acid

Bempedoic acid is a non-statin treatment that lowers the level of cholesterol in the blood by inhibiting an enzyme in the liver (ATP citrate lyase). It is an oral pill taken daily.



Lipoprotein Apheresis

Lipoprotein apheresis is a procedure, usually done every two weeks, to remove lipids, including LDL-C and Lp(a) from the blood. It can lower LDL-C by 20-40%. It is approved for use in individuals with HoFH on maximum tolerated LDL-C lowering therapy.



Lomitapide*

Lomitapide is a microsomal triglyceride transfer protein (MTP) inhibitor that works in the liver by partially blocking the production of LDL-C and by reducing the absorption of cholesterol from the intestines. It can lower LDL-C by 40-50%. It is an oral pill taken daily and is approved as treatment taken in addition to a low-fat diet and other lipid lowering treatments for adults with **HoFH only**.



Evinacumab*

Evinacumab is a monoclonal antibody therapy that inhibits angiopoietin-like 3 (ANGPTL3). Evinacumab is given monthly by infusion in the doctor's office. It can lower LDL-C by 45-50% for people with HoFH. It is approved as treatment taken in addition to other lipid lowering treatments for adults and children over the age of 12 who have **HoFH only**.



Liver Transplant

Liver transplant may be considered in rare circumstances as a surgical option for the treatment of HoFH and can lead to normal cholesterol levels.

*Approved for HoFH only