Repatha® (evolocumab) – New indication and expanded indication

- On September 24, 2021, Amgen announced the FDA approval of Repatha (evolocumab), as an adjunct to diet and other low density lipoprotein-cholesterol (LDL-C)-lowering therapies in pediatric patients aged 10 years and older with heterozygous familial hypercholesterolemia (HeFH), to reduce LDL-C.

- In addition, the FDA approved Repatha as an adjunct to other LDL-C-lowering therapies in adults and pediatric patients aged 10 years and older with homozygous familial hypercholesterolemia (HoFH), to reduce LDL-C.

    — It was previously approved for this indication in patients 13 years and older.

- Repatha is also approved:

    — In adults with established cardiovascular disease to reduce the risk of myocardial infarction, stroke, and coronary revascularization.
    — As an adjunct to diet, alone or in combination with other LDL-C-lowering therapies, in adults with primary hyperlipidemia, including HeFH, to reduce LDL-C.

- The approval of Repatha for the new indication was based on HAUSER-RCT, a 24-week, randomized, placebo-controlled, double-blind study in 157 pediatric patients aged 10 to 17 years with HeFH. Patients were randomized to Repatha or placebo.

    — The difference between Repatha and placebo in mean percent change in LDL-C from baseline to week 24 was -38% (95% CI: -45, -31; $p < 0.0001$).

- In addition, the approval of Repatha for the expanded indication was based on HAUSER-OLE, an 80-week, open-label, single-arm study in 12 pediatric patients aged 10 to 17 years with HoFH.

    — The median (Q1, Q3) percent change in LDL-C from baseline to week 80 was -14% (-41, 4)

- In pediatric patients aged 10 years and older with HeFH, the recommended dose of Repatha is either 140 mg every 2 weeks OR 420 mg once monthly administered subcutaneously (SC).

- In adults and pediatric patients aged 10 years and older with HoFH, the initial recommended dosage of Repatha is 420 mg once monthly administered SC. The dosage can be increased to 420 mg every 2 weeks if a clinically meaningful response is not achieved in 12 weeks.

    — Patients on lipid apheresis may initiate treatment with 420 mg every 2 weeks to correspond with their apheresis schedule. Repatha should be administered after the apheresis session is complete.

- Refer to the Repatha drug label for dosing for its other uses and indications.