Evolocumab: No hint of added benefit

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Evolocumab (trade name: Repatha) has been approved since July 2015 for two therapeutic indications: on the one hand, for hypercholesterolaemia or mixed dyslipidaemia, and on the other, for homozygous familial hypercholesterolaemia. The drug is an option for patients whose cholesterol levels are not adequately lowered by diet and other drugs.

The German Institute for Quality and Efficiency in Health Care (IQWiG) examined in a dossier assessment whether evolocumab offers an added benefit over the appropriate comparator therapy. Due to a lack of suitable data, no such added benefit can be derived from the dossier for any of the two therapeutic indications.

The G-BA distinguished between a total of six treatment situations

Hypercholesterolaemia is diagnosed when the LDL cholesterol levels in the blood are too high. In mixed dyslipidaemia, triglyceride levels may also be elevated. Homozygous familial hypercholesterolaemia is a very rare hereditary form. If left untreated, all of these conditions can lead to cardiovascular diseases (coronary heart disease, arteriosclerosis); in hereditary homozygous hypercholesterolaemia, cardiovascular disease may already occur in childhood.

Standard treatment options include diet together with other lipid-lowering drugs or, if this is no longer effective, LDL apheresis, a procedure similar to dialysis. The Federal Joint Committee (G-BA)

differentiated between a total of six treatment situations and research questions for the assessment, and specified an appropriate comparator therapy for each of them.

Twelve-week studies are far too short to investigate long-term treatment

The manufacturer presented no studies in its dossier for one treatment situation in one of the two therapeutic indications and for two treatment situations in the other therapeutic indication. The dossier contained data from randomized controlled trials for the remaining three treatment situations. In these studies however, the patients were treated and observed for only twelve weeks.

Since these are chronic diseases and evolocumab is used in long-term treatment, however, studies with a minimum duration of one year are required to assess benefit and harm. Hence there is no hint of an added benefit in comparison with the appropriate comparator therapy for any of the therapeutic indications and for any of the treatment situations.

Provided by Institute for Quality and Efficiency in Health Care