Familial hypercholesterolemia (FH) is an inherited autosomal dominant disorder which is characterized by substantially increased Low-Density Lipoprotein Cholesterol (LDL-C) levels. Timely reduction of LDL-C is of paramount importance to ameliorate the risk for CV disease as patients with FH have a significantly higher risk for Cardiovascular (CV) events. Pro-protein Convertase Subtilisin/Kexin type 9 (PCSK9) inhibitors have emerged as a very promising class of drugs for the management of such patients, among the available lipid-lowering novel therapies.

To discuss the potential differences between the two drugs, this review presents the available data on the efficacy and safety of the two available PCSK9 inhibitors in patients with FH. To identify available data from clinical studies evaluating the impact of evolocumab or alirocumab on lipid and CV parameters in patients with FH, a comprehensive literature search was performed.

In patients with FH, several studies have assessed the lipid-lowering profile of PCSK9 inhibitors. Furthermore, data also support a lower rate of lipid apheresis in FH patients receiving a PCSK9 inhibitor. Both evolocumab and alirocumab were found to significantly reduce LDL-C by more than 50-60% in FH patients. However, alirocumab reduced all-cause mortality, as well, a finding not observed with evolocumab. In terms of CV outcomes, both drugs were found to possess CV-ameliorating effects of the same extent in patients with CV disease. Several differences in the study population characteristics might explain this and other mild differences observed in the CV trials of these drugs.


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