Supplemental material

The lipid-lowering effects of lomitapide are unaffected by adjunctive apheresis in patients with homozygous familial hypercholesterolaemia – a post-hoc analysis of a Phase 3, single-arm, open-label trial

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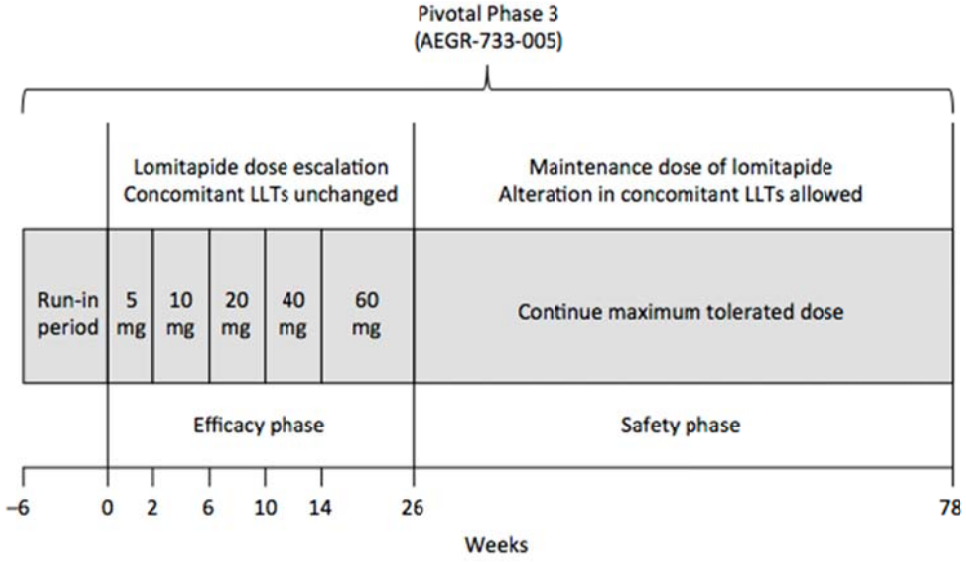
STUDY DESIGN

The study design (Figure 1), patient population and overall results for this study have been described previously.[1]

Patients underwent a 6-week run-in phase, during which concomitant lipid-lowering therapies, including apheresis, were stabilised. This was followed by a 26-week efficacy phase, which included a lomitapide dose titration period (5–60mg/day based on individual maximum tolerated dose), during which current lipid-lowering therapy and apheresis schedule was kept stable. Patients then entered a 52-week safety phase during which patients remained on their maximal tolerated dose of lomitapide reached in the efficacy phase. During the safety phase, statins and other lipid-lowering therapies (including frequency of apheresis) could be adjusted on a case-by-case basis at the physician and patient's discretion based on established protocol criteria.

Figure 1. Study design



LLTs, lipid-lowering therapies

References

[1] Cuchel M, Meagher EA, du Toit Theron H, Blom DJ, Marais AD, Hegele RA, Averna MR, Sirtori CR, Shah PK, Gaudet D, Stefanutti C, Vigna GB, Du Plessis AM, Propert KJ, Sasiela WJ, Bloedon LT, Rader DJ and Phase 3 Ho FHLSi. Efficacy and safety of a microsomal triglyceride transfer protein inhibitor in patients with homozygous familial hypercholesterolaemia: a single-arm, open-label, phase 3 study. *Lancet* 2013;**381**:40-6. DOI: 10.1016/S0140-6736(12)61731-0.