Background: alirocumab is a human monoclonal antibody that inhibits PCSK9. PCSK9 increases LDL-C levels by binding LDL receptors.

Purpose: evaluation of the efficacy and safety of alirocumab in patients with heterozygous familial hypercholesterolemia (hFC) who are not controlled on maximally tolerated statin or other lipid-lowering therapy.

Trial Design: Phase 3, randomized, double-blinded, placebo-controlled, efficacy and safety study; n=735; alirocumab 75-150 mg SQ Q2W (1 ml, self-administered) vs. placebo for 78 weeks in hFC patients not adequately controlled on their current lipid medication.

Primary Endpoint: percent change in LDL-C from baseline to 24 weeks

### Trial Results

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<td>alirocumab vs. placebo (% change from baseline @ 24 weeks)</td>
<td>-57.9% (P&lt;0.0001)</td>
<td>-51.4% (P&lt;0.0001)</td>
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Conclusions: LDL-C levels were lowered at 24 weeks with the addition of alirocumab and the results were maintained over 52 weeks. Safety was similar to placebo.

Presented by: FARNIER, M., ESC Congress 2014, Barcelona, Spain

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