INTRODUCTION

Familial hypercholesterolemia (FH) is a rare genetic disease that results in high levels of circulating LDL-C. Untreated it can cause premature coronary artery disease and stroke. FH is especially severe when the gene for FH is inherited from both parents, instead of just one - a condition referred to as homozygous familial hypercholesterolemia (HoFH). HoFH is characterized by defective or deficient LDL receptors. HoFH can result from negative/defective (<2% of normal) LDL receptor activity (<95% of normal LDL receptor activity) LDL receptor activity. HoFH with negative/defective (50%) LDL receptor activity is considered mild to moderate HoFH, whereas HoFH with negative/defective (<30%) LDL receptor activity is considered severe (HoFH with negative <50% LDL receptor activity).

INTESTRO
tion

Figures

1. Effect of Gemcabene and Atorvastatin on Plasma Total and LDL Cholesterol in Cholesterol Deficient Mice (Week 16).
2. Effect of Gemcabene on Hepatic and Plasma Triglycerides in LDLr-/- Mice.
3. Effects of Gemcabene and Atorvastatin on Plasma Total and LDL Cholesterol in HoFH Patients.
4. Effect of Gemcabene on Hepatic and Plasma Triglycerides in LDLr-/- Mice.
5. Effect of Gemcabene on Plasma Total and LDL Cholesterol in HoFH Patients.
7. Effect of Gemcabene on Plasma Total and LDL Cholesterol in HoFH Patients.
8. Effect of Gemcabene on Hepatic and Plasma Triglycerides in LDLr-/- Mice.
11. Effect of Gemcabene on Plasma Total and LDL Cholesterol in HoFH Patients.
15. Effect of Gemcabene on Plasma Total and LDL Cholesterol in HoFH Patients.

RESULTS

The authors acknowledge the contributions to this work by Karen T. Lott, Christopher P. Johnson, and John C. Staub.

REFERENCES


CONCLUSIONS

Gemcabene is a novel oral drug candidate in clinical development and may be a potential drug of choice for patients with hypercholesterolemia. Gemcabene's mechanism of action and its potential utility for reducing LDL-C levels and the rate of atherosclerosis progression in HoFH patient population.

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