The Realisation of Research

Lowering LDL cholesterol levels by antisense oligonucleotide induced alternative splicing of apolipoprotein B

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60-064

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Category(s):
Gene/Cell Therapy

Description:

A new treatment for hypercholesterolaemia using antisense oligonucleotides

Available for: Collaborative research and licensing

Summary

We have invented an entirely novel method for lowering cholesterol levels as a treatment for the common genetic disease, familial hypercholesterolaemia. We have devised a treatment that uses exon-skipping antisense oligonucleotides (ASOs) that cause skipping of APOB exon 27. Removal of this exon reduces low-density lipoprotein cholesterol (LDLc) levels by truncating Apolipoprotein B, the protein that is essential for LDL assembly.

The Technology and its Advantages

We have synthesized and tested optimised RNA "skip" ASOs. These cause powerful exon 27 skipping in vivo when injected into transgenic mice expressing human APOB, which translates to an in vivo lowering of cholesterol.

Market Opportunity

Familial hypercholesterolaemia (FH) cases extremely high low-density lipoprotein (LDL) particle levels due to genetic defects in the hepatic LDL receptor. It is one of the most common genetic diseases, affecting 1 in 500 people. Patients with the most severe form, homozygous FH, suffer from early heart disease and die from heart attacks. Conventional treatment with the most powerful 'statin' drugs available is only partially effective in reducing LDLc by about 20% and patients are still vulnerable to early heart disease. There is an unmet health need for a drug for Familial Hypercholosterolaemia that is cost-effective, efficacious at reducing LDLc, and free of off-target side effects.

Intellectual Property Status

UK priority patent filed October 2011

Further Information

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