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Managing the Challenging Homozygous Familial Hypercholesterolemia Patient: Academic Insights and Practical Approaches for a Severe Dyslipidemia, a National Lipid Association Masters Summit

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Abstract:

The following article represent material presented and discussed at a symposium hosted by the National Lipid Association hosted entitled; "Managing the Challenging Homozygous Familial Hypercholesterolemia Patient – Academic Insights and Practical Approaches for a Severe Dyslipidemia" on November 7, 2015 in Orlando, FL. Presenters included G. Kees Hovingh, MD, PhD, Anne C. Goldberg, MD, and Patrick M. Moriarty, MD.

The diagnosis and genetic causes of extremely high LDL -cholerol which has become known as Homozygous Familial Hypercholesterolemia were discussed. This disorder in adults manifest often by LDL cholesterol in excess of 500occurs in several populations with an prevalence of one in 300,000. In more sequestered areas, the frequency may be much greater due to founder effects of specific settlers carrying the responsible alleles. Although the great majority of these patients have a variant sequences in the LDL receptor gene, variants in the apolipoprotein B (*APOB)*, *PCSK9*, or LDL receptor adaptor protein (*LDLRAP*) gene loci can also be causative. Some individuals have additional genetic abnormalities which have not been fully revealed. In most studies, the diagnosis has depended on predefined clinical findings in association with the very elevated LDL-cholesterol.

Standard lipid lowering drugs such as statins, ezetimibe or bile acid binding resins are usually only partially beneficial and leave the patients at high risk. Lipoprotein apheresis has been a more effective therapy and is a mainstay in treatment of many pateints. New therapies such as mipomersen and lomitapide have reduced LDL dramatically in some but are often ineffective in others. Inhibitors of PCSK9 can reduce LDL dramatically but in those with null genes for the LDL receptor, they are also ineffective. The availability of this battery of drugs has markedly improved the potential of pharmacotherapy to control LDL values and prolong the life of these patients.

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