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Title **Low Density Lipoprotein Apheresis for the Treatment of Familial Hypercholesterolemia**

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Aim

To present evidence on the safety and efficacy/effectiveness of using apheresis to lower the concentration of low density lipoprotein (LDL) cholesterol in patients with familial hypercholesterolemia.

Conclusions and results

The report focused on two selective LDL apheresis systems: dextran sulfate cellulose (DSC) Liposorber and heparin induced LDL precipitation (HELP). Six controlled studies compared a combined LDL apheresis (DSC system) and drug therapy with drug therapy alone. Two further studies compared the DSC or HELP system with other apheresis systems. Weak evidence suggested that the DSC Liposorber system, combined with lipid lowering drug therapy, lowered LDL cholesterol in patients >50 years of age with severe familial hypercholesterolemia when treated at least once every 2 weeks for at least 1 year. The mean decrease in LDL cholesterol ranged from 34% to 81%. In combined therapy, the contribution of LDL apheresis to the treatment effect was unclear. Two studies concluded that all of the systems (Immunoadsorption, Liposorber, HELP, Lipidifiltration) decreased the levels of LDL cholesterol (mean decrease ranged from 54% to 65%). Adverse effects associated with DSC and HELP were hypotension, nausea, and vomiting. These effects were transient.

Recommendations

Information from the reviewed studies must be considered cautiously. Generalization of the results to a local context is challenging since none of the studies were conducted in Canada, and most used only the DSC system. For economic and ethical reasons, the decision to include LDL apheresis in the service package for patients with familial hypercholesterolemia is difficult. Planning processes must weigh costs and access against the gravity of the disease, the poor quality of life, and the life expectancy of patients with homozygous familial hypercholesterolemia. A national registry for patients with familial hypercholesterolemia and severe hyperlipidemia would be useful.

Methods

All original comparative studies published in English were identified by systematically searching (Jan 1998 to Mar 2004) PubMed, EMBASE, HealthStar, the Cochrane Library, Science Citation Index, and the websites of health technology assessment agencies, research registers, and guideline sites.

Further research/reviews required

Multicenter, concurrently controlled studies with long-term followup should assess whether LDL apheresis is more effective than drug therapy or plasmapheresis (alone or in combination with standard care) in treating patients with familial hypercholesterolemia. The effectiveness and safety of LDL apheresis in certain groups, eg, children or pregnant/lactating women, should also be evaluated. Randomized controlled crossover studies would be appropriate since they avoid the ethical problem of withholding LDL apheresis from patients who may need it. Cost-benefit and cost-effectiveness analyses are needed to assess the economic consequences of LDL apheresis versus alternative treatments, eg, drug therapy or plasmapheresis. The use of valid quality-of-life measures in these analyses is essential.

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