

- Title** Safety and effectiveness of LDL-apheresis in cerebrotendinous xanthomatosis.
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Aim

To assess the effectiveness and safety of LDL-apheresis for the treatment of cerebral xanthomatosis

Conclusions and results

Available evidence on the effectiveness of LDL-apheresis in cerebral xanthomatosis is practically nil and lacks clinical validity. The four studies found were descriptive analysis of case studies or very small series. In total, they included a total of 9 patients, with ages ranging from 24 to 54 years. All used the apheresis procedure in combination with QDCA and statins. Studies showed great heterogeneity with regards to the treatment schedule and contradictory results. One of the patients did not improve significantly, despite normalisation of cholesterol levels. A slight improvement in neurological symptomatology was reported in three patients, though objective scales were not used for their evaluation. Only one patient, which received LDL and HDL apheresis, displayed substantial neurological improvement. Evidence was restricted to patients with advanced disease, existing no evidence as to whether LDL-apheresis serves to slow disease progression and appearance of neurological symptoms.

Methods

A systematic search of the scientific literature was performed covering the main computerised medical databases (PubMed, Embase, CRD, Cochrane, etc.). Due to the scarce literature found, we considered all original studies published in scientific journals in Spanish, French, English, Portuguese or Italian, which provided primary data on effectiveness and safety of this procedure. The results were summarised in evidence tables and qualitatively synthesised.

Further research/reviews required

Adequately designed controlled clinical trials are needed in order to assess the effectiveness of LDL-apheresis

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