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Title **The Clinical Effectiveness and Cost Effectiveness of Treatments for Children with Idiopathic Steroid-Resistant Nephrotic Syndrome: A Systematic Review**

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Aim

To systematically review the clinical and cost effectiveness of treatments for children with idiopathic steroid-resistant nephrotic syndrome.

Conclusions and results

The systematic review of clinical effectiveness included 2 systematic reviews and 11 trials. Seven different therapies were included, but only ciclosporin and cyclophosphamide were assessed by more than one study. The quality of reporting and methodology of the included studies was generally poor. No economic evaluations were identified. Ciclosporin significantly increased remission rates compared with placebo or supportive treatment (RR 7.66, 95% CI 1.06, 55.34), but not for a subgroup with FSGS (RR 5.83, 95% CI 0.75, 45.09). No significant difference in remission rates was found with cyclophosphamide plus prednisone versus prednisone, but the time to response was significantly less with cyclophosphamide (38.4 days [range 6-80] versus 95.5 days [range 61-129], $p<0.05$). No significant differences were found between azathioprine versus placebo, 6-month versus 18-month regimen of methylprednisolone, intravenous dexamethasone versus intravenous methylprednisolone, or tuna fish oil versus placebo. A difference in the urine albumin to creatinine ratio reduction percentage between high-dose and low-dose enalapril was found, but this was statistically significant in the period before crossover only. Studies varied in the extent of reporting adverse events. Limited literature was found on costs associated with steroid-resistant nephrotic syndrome in children. The cost of pharmacotherapy varies considerably, eg, an 8-week course of cyclophosphamide costs less than 6 pounds (GBP) while a course of ciclosporin costs almost GBP 900 per year. Children who fail to respond to treatment are at high risk of developing end stage renal failure, the costs of which are considerable. No published evidence on the cost effectiveness of treatments for children was identified. Subsequent searches were undertaken to identify economic evaluations and economic evidence for treatments in adults. Data are sparse, and modeling the cost effectiveness of current treatments is not feasible at present.

Recommendations

Evidence is limited as regards the clinical and cost effectiveness of treatments for idiopathic steroid-resistant nephrotic syndrome in children. The evidence suggests a beneficial effect of ciclosporin on remission rates and of cyclophosphamide on time to remission; but poor-quality studies limit the strength of the conclusions. No economic evaluations were identified. Data on costs and outcomes are sparse and do not permit reliable modeling of the cost effectiveness of treatments for steroid-resistant nephrotic syndrome. A modeling framework is suggested should more relevant data become available.

Methods

See Executive Summary link at www.ncchta.org/project/1497.asp.

Further research/reviews required

See Executive Summary link at www.ncchta.org/project/1497.asp.

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