



Title Systematic Review of Effectiveness of Different Treatments

for Childhood Retinoblastoma

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## Aim

To provide the evidence base on clinical effectiveness of different treatments for childhood retinoblastoma, building on previous work completed in October 2003.

#### Conclusions and results

The review included 31 individual studies from 42 publications. Apart from 1 non-randomized controlled trial, only comparative studies of observational design were available for any of the treatments. Four of the included studies were prospective, and the remaining 27 were retrospective. Most of the studies were of radiotherapy or chemotherapy, with few studies available on enucleation or focal treatments such as brachytherapy, photocoagulation, cryotherapy, and thermotherapy. Methodological quality was generally poor, with a high risk of bias in all included studies. The main problems related to how treatment was allocated and lack of consideration of potentially confounding factors, eg, initial disease severity, in the study design and data analysis. The evidence base for effectiveness of treatments for childhood retinoblastoma is extremely limited. Owing to the limited evidence, it was not possible to make meaningful, robust conclusions about the relative effectiveness of different treatment approaches for childhood retinoblastoma.

### Recommendations

In the authors' opinion, the evidence base for the effectiveness of treatments for childhood retinoblastoma is not sufficiently robust to provide clear guidance for clinical practice. While many of the studies reported high levels of treatment success, the relative effectiveness and adverse effects of treatment were unclear.

## Methods

Seventeen databases were searched, up to April 2004. Two reviewers independently assessed studies for inclusion. Studies of participants diagnosed with childhood retinoblastoma, any interventions, and all clinical outcomes were eligible for inclusion. Randomized and non-randomized controlled trials and cohort studies with clear

comparisons between treatment groups were included. Methodological quality was assessed, and a narrative synthesis was conducted. Where possible, studies assessing common interventions were grouped together, with prospective and retrospective studies grouped separately. Emphasis was placed on prospective studies.

# Further research/reviews required

Ideally, good-quality, randomized controlled trials (RCTs) assessing the effectiveness of different treatment options for childhood retinoblastoma are required. Research is required on all the treatments currently used for this condition. Where RCTs are not feasible for ethical or practical reasons, only high-quality, prospective, non-randomized studies should be given consideration, owing to the generally higher risk of bias in retrospective studies. To reduce the risk of confounding due to allocation by clinical indication, studies should compare patients with similar disease severity rather than compare patients of mixed disease severities. Standardized outcomes should be agreed for use in studies assessing the effectiveness of treatment. These outcomes should encompass the potentially important beneficial effects and adverse effects of treatment, eg, loss of visual acuity and cosmetic outcome.