Recombinant growth hormone therapy for cystic fibrosis in children and young adults

Study design (if review, criteria of inclusion for studies)
Randomized controlled trials and quasi-randomized trials

List of included studies (8)

Participants
Children or young adults aged up to 25 years diagnosed with cystic fibrosis, who have not received growth hormone therapy in the previous six months

Interventions
All preparations of recombinant growth hormone compared to either no treatment, or placebo, or each other at any dose (high-dose and low-dose) or route and for any duration

Outcome measures
Primary outcomes: FEV1, FVC, PImax, PEmax, height and weight (cm and SDS), height and weight velocity, lean body mass measured by DEXA, QoL

Main results
We included eight trials (291 participants, aged between five and 23 years) in the current version of the review. Seven trials compared standard-dose rhGH (approximately 0.3 mg/kg/week) to no treatment and one three-arm trial (63 participants) compared placebo, standard-dose rhGH (0.3 mg/kg/week) and high-dose rhGH (0.5 mg/kg/week). Six trials lasted for one year and two trials for six months. We found that rhGH treatment may improve some of the pulmonary function outcomes, but there was no difference between standard and high-dose levels (low-certainty evidence, limited by inconsistency across the trials, small number of participants and short duration of therapy). The trials show evidence of improvement in the anthropometric parameters (height, weight and lean body mass) with rhGH therapy, again no differences between dose levels. We found improvement in height for all comparisons (very low- to low-certainty evidence), but improvements in weight and lean body mass were only reported for standard-dose rhGH versus no treatment (very low-certainty evidence). There is some evidence indicating a change in the level of fasting blood glucose with rhGH therapy, however, it did not cross the clinical threshold for diagnosis of diabetes in the trials of short duration (low-certainty evidence). There is low- to very low-certainty evidence for improvement of pulmonary exacerbations with no further significant adverse effects, but this is limited by the short duration of trials and the small number of participants. One small trial provided inconsistent evidence on improvement in quality of life (very low-certainty evidence). There is limited evidence from three trials in improvements in exercise capacity (low-certainty evidence). None of the trials have systematically compared the expense of therapy on overall healthcare costs.

Authors’ conclusions
When compared with no treatment, rhGH therapy is effective in improving the intermediate outcomes in height, weight and lean body mass. Some measures of pulmonary function showed moderate improvement, but no consistent benefit was seen across all trials. The significant change in blood glucose levels, although not causing diabetes, emphasizes the need for careful monitoring of this adverse effect with therapy in a population predisposed to CF-related diabetes. No significant changes in quality of life, clinical status or side-effects were observed in this review due to the small number of participants. Long-term, well-designed randomised controlled trials of rhGH in individuals with CF are required prior to routine clinical use of rhGH in CF.

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See also

Keywords

Adult; Child; Hormones; pharmacological_intervention; Recombinant Proteins; Growth Hormone;