The clinical effectiveness and cost-effectiveness of pegvisomant for the treatment of acromegaly: a systematic review
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Record Status
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Authors' objectives
This review assessed the evidence about the clinical effectiveness and cost effectiveness of pegvisomant for the treatment of acromegaly in patients whose IGF-1 levels fail to normalise in response to other treatments.

Authors' conclusions
Pegvisomant treatment for acromegaly is highly effective for improving patients' IGF-1 level. Evidence is lacking about the long term effects of treatment in respect of improved signs and symptoms of disease, quality of life, patient compliance and safety. An economic evaluation using a simple decision tree model indicated that pegvisomant was very unlikely to represent good value for money according to currently applied standards. The prevalence of acromegaly (~58/106) falls just outside the definition for an ultraorphan disease (<20/106) but within the orphan criterion; as such pegvisomant might be considered by some wholly or partially exempt from usually applied value-for-money criteria and subject to other criteria as yet ill defined or incompletely applied by national or local reimbursement bodies.

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