Human growth hormone for the treatment growth failure in children (review)

Appraisal Consultation Document

Overall I believe that the recommendations are reasonable, appropriate and pragmatic given 1) the relative lack of high quality relevant evidence, 2) the difficulty in determining effects on the clinically most relevant outcomes – and, therefore, 3) the major assumptions that have to be made about what constitutes 'effectiveness', and thus 'cost-effectiveness' and cost per QALY, but 4) the clinical value placed by patients and their representatives on the benefits of treatment with somatropin for growth failure, and which are poorly captured by the extant studies.

I would like to see a further recommendation that there should be 'compulsory' post-marketing surveillance of all patients treated, at whatever age and for whatever indication, with somatropin. This should be a joint responsibility of manufacturers and clinicians (through the British Society for Paediatric Endocrinology and Diabetes and the Society for Endocrinology) and should, in particular, be extended to the surveillance of adults no longer treated with somatropin but who were treated as children and/or adolescents. This is important for all brands of somatropin and particularly so for Omnitrope, the 'biosimilar' preparation where the medium- and long-term safety profile is less clear.

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