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Symptomatic versus Substitution Growth Hormone Therapy in Short Children: From Auxology Towards a Comprehensive Multidimensional Assessment of Short Stature and Related Interventions

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ABSTRACT

There is an ongoing debate as to whether symptomatic growth hormone treatment (GHT) in short children without growth hormone deficiency (GHD) is justified, since there is no substitutional indication. The increasing evidence that final height cannot be normalized in these patients (e.g. in Ullrich-Turner syndrome) reinforces this controversy. We have focused on the empirical evidence on the psychosocial and physical meaning of being short in childhood as well as on the underlying assumptions of the different GHT indications. The indication for GHT in patients with non-GHD may be seen as a pharmacotherapeutic intervention in order to prevent the developmental, physical and psychosocial risks associated with short stature. This requires a qualitative shift in methodological assessment with respect to the psychosocial and physical impact of being short as well as of the potential benefit of new treatment indications in terms of a more comprehensive evaluation including health-related quality of life for these patients.

KEY WORDS

short stature in childhood, quality of life, assessment strategies, growth hormone therapy, non growth hormone deficiency

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INTRODUCTION

In this paper we focus on the multidimensional psychosocial and physical aspects of short stature in childhood and have drawn conclusions for a more comprehensive approach concerning the evaluation of short stature-associated risks for health and development and for the assessment of treatment benefit in children with various growth disorders.

Indication for growth hormone therapy (GHT) in short children with growth hormone deficiency (GHD) is clearly medically founded because GHT represents a substitution therapy in these patients[1-5]. During therapy, auxological and endocrinological parameters as well as possible medical side effects are monitored. Auxological improvement in terms of catch-up growth under GHT has been defined as the most relevant clinical criterion concerning the evaluation of therapeutic success[2,3]. In contrast, GHT in short children without GHD (e.g. in Ullrich-Turner syndrome) does not represent substitution therapy[5]. It therefore has to be seen as a medical symptomatic intervention in those conditions that share the symptom of short stature but not the pathogenesis of GHD.

THE CRITICAL ISSUE OF SYMPTOMATIC GROWTH HORMONE THERAPY: TREATMENT OF DEVELOPMENTAL RISKS?

The indication for GHT in short patients without GHD is primarily based on the implicit assumption that great psychosocial risks/physical restrictions due to short stature are to be expected and that these problems can be prevented via auxological improvement under GHT. Figure 1 shows the essential assumptions regarding the indication as