3-years Height Outcome during rh-Growth Hormone Therapy in Subjects with Achondroplasia and Hypochondroplasia

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Background. Achondroplasia (ACH) and hypochondroplasia (HCH) are the most common forms of chondrodysplasia. ACH is characterized by rhizomelic short stature, macrocephaly and lumber lordosis. Because HCH is clinically milder, HCH is often goes unrecognized in childhood but is diagnosed in adult life when disproportionate short stature becomes obvious.

Objectives. Although episodic reports showed the recombinant human growth hormone (rhGH) treatment may improve short-term height (Ht) in HCH or ACH subjects, poor data were available for long-term outcome.

Method. After thorough search of pertinent English-language literature, meta-analysis evaluation of the efficacy of 3-yrs rhGH treatment in patients with HCH or ACH were performed.

Conclusions. *The rhGH treatment progressively improved Ht outcome of short children with ACH and HCH though with minor catch-up growth. Future studies using carefully titrated rhGH protocols are need to optimize treatment protocols.*

Results. In total ACH patients (n=498), mean Ht at rhGH therapy start was subnormal [Ht –5.069 SDS (95%CI –5.109 to –5.029)] in all the studies. For ACH subjects, Ht progressively improved during 12 months of rhGH treatment with low catch-up growth [n=494; Ht –4.325 SDS (95%CI –4.363 to –4.287); $P<0.0001$]. Then, Ht trend of ACH subjects appeared constant until 36-months [n=60; –4.124 SDS (95%CI –4.190 to –4.059)]. From baseline [n=494; SHtR –4.325 SDS (95%CI –4.363 to –4.287); $P<0.0001$], Sitting Ht Ratio (SHtR) was clinically unchanged in ACH subjects after 2-yrs [n=45; SHtR 64.637% (95%CI 59.931 to 69.344); $P<0.0001$]. In HCH sample [n=74, pre-treated Ht –3.071 SDS (95%CI –3.779 to –2.362)], rhGH therapy progressively produced similar catch-up growth of ACH group at 12 months [n=74; –2.654 SDS (95%CI –3.043 to –2.264); $P<0.0001$]: Then, rhGH-induced Ht was progressively improved until 36 months [n=22; –2.493 SDS (95%CI –2.870 to –2.116); $P<0.0001$].

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