Associations between height and health-related quality of life (HRQoL) and functional independence in children with achondroplasia

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Background and Objectives

- Achondroplasia, the most common skeletal dysplasia, is characterized by severe short stature with a height deficit of approximately -6 SDS compared to average stature and is associated with a reduced quality of life.1–5

- Several studies have assessed the impact of short stature on health-related quality of life (HRQoL).3–5

- Our objective was to evaluate the impact of height deficit on HRQoL and functional independence in children with achondroplasia

Methods

- Height Z-score, HRQoL, and functional independence data were obtained from children with achondroplasia who enrolled in the VoroPhase 3 trial (study 111-301; EudraCT number, 2015-003383-11). Subjects completed at least six months of a baseline observational growth study (study 111-901; ClinicalTrials.gov number, NCT01930695) prior to enrolling in the Phase 3 trial and the subsequent open label extension study (study 111-302; ClinicalTrials.gov number NCT03424018)

- Height Z-scores were assessed every 6 months – Height Z-scores were derived using age-sex specific reference data (means and standard deviations) in reference to Centers for Disease Control and Prevention (CDC) normative data on average stature children.

- The Pediatric Quality of Life Inventory (PedsQL®), the Quality of Life in Short Stature Youth (QoLISSY®), and the Functional Independence Measures in Children (WeeFIM®) questionnaires were completed by the children and/or their caregivers every 6 months – For both PedsQL and QoLISSY, self-reporting started at age 8 years – The WeeFIM considers the child’s functional performance from the perspective of caregivers, and is therefore only completed by caregivers

- All visit matched pairs of height Z-score and HRQoL data pools from studies 901, 301 and 302 were analyzed using a mixed effect model to assess the relationship of a 1 SDS change in height deficit on domain scores of the PedsQL, QoLISSY, and WeeFIM, without reference to assignment to treatment or timepoint

- All pairs of assessments (height Z-scores and HRQoL scores) were categorized according to the height Z-score classification: 1.5 to < 4, ≤ -4 ≤ -6, -6 to ≤ -5

- Mean HRQoL scores were calculated for each category

Results

Participant characteristics

- 121 children (64 boys, 57 girls) were included in this exploratory analysis

- Age at the time of individual assessments ranged from 4–15 years

- For all instruments, mean scores were generally lower in subjects with greater height deficit (≤-6 SDS) compared to taller subjects with height z-score greater than -4 SDS

- Clear linear relationships between height Z-scores and several domain scores were observed, most notably in the PedsQL Physical domain, the height-specific QoLISSY Physical domain, and the WeeFIM Self-care and Mobility domains

- These data corroborate other findings that suggest height z-score is a predictor of physical functioning and overall HRQoL.

Conclusions

- These data suggest that height deficit in achondroplasia impacts HRQoL and function

- Therapies that have the potential to reduce height deficit in achondroplasia may improve HRQoL and functional independence

References


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