Skeletal Dysplasia and GH-therapy: Data on Final Height.

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Background

Skeletal Dysplasias (SD) are characterized by bone and cartilage tissues involvement and a severe impairment of linear growth and body proportions.
GH doesn't have an effective role in the pathogenesis of growth failure in skeletal dysplasias.
Reports of the benefits of GH treatment are difficult to evaluate having few final height data in groups with and without GH deficit

Objectives

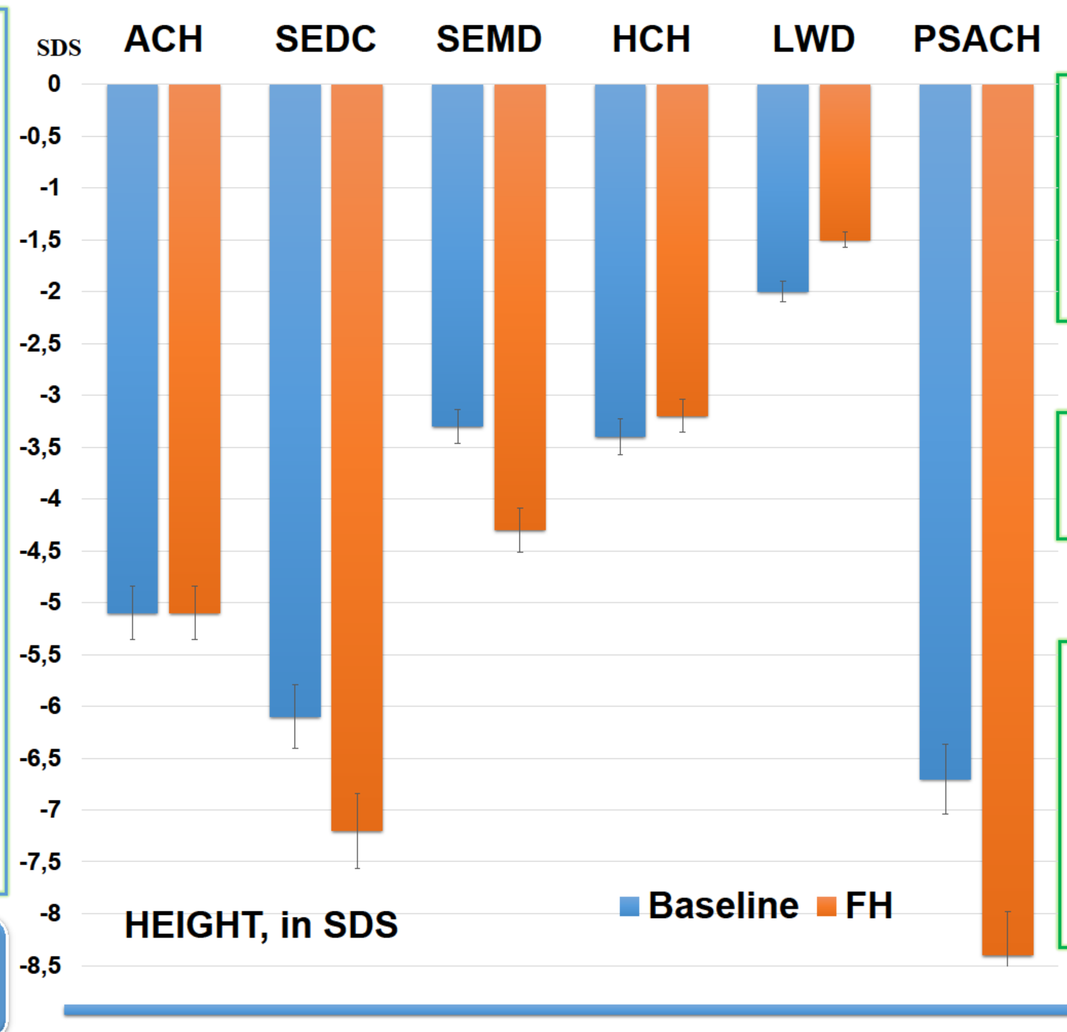
Final Height (FH) and body proportions after long term GH-treatment in subjects with SD (confirmed at molecular analysis) and GHD.

Methods

We studied 24 pts at FH after 6.5±3 yrs of GH-therapy (25-30 µg/kg/day): 6 pts with achondroplasia (**ACH**), 4 with hypochondroplasia **(HCH**), 4 with pseudoachondroplasia (**PSACH**), 3 with spondylo-epiphyseal dysplasia congenital (**SEDC**), 4 with spondylo-epi-metaphyseal dysplasia **(SEMD**) and 3 with Leri-Weill dyschondrosteosis (**LWD**).

Anthropometric measurements are expressed as SDS of Prader standards and were detected at baseline (7.3±3.0 yrs) and at FH (16.3±1.6 yrs)

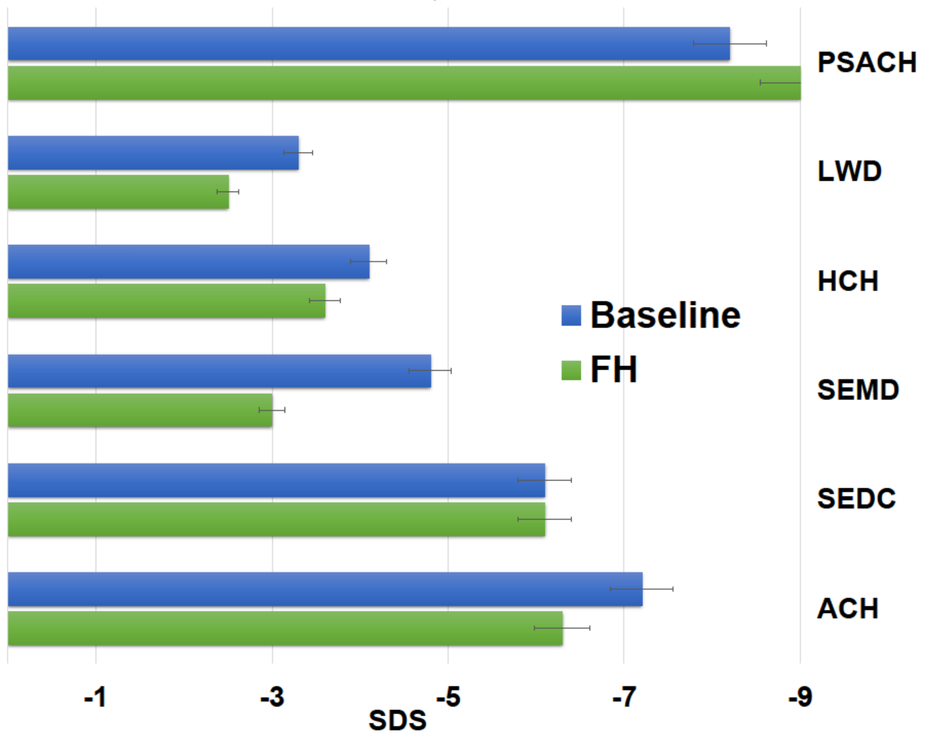
Results



At FH mean **Height Gain** vs pre-therapy height **was positive** in **HCH** (+0.2±1,1 SD), even lower than Hertel's data

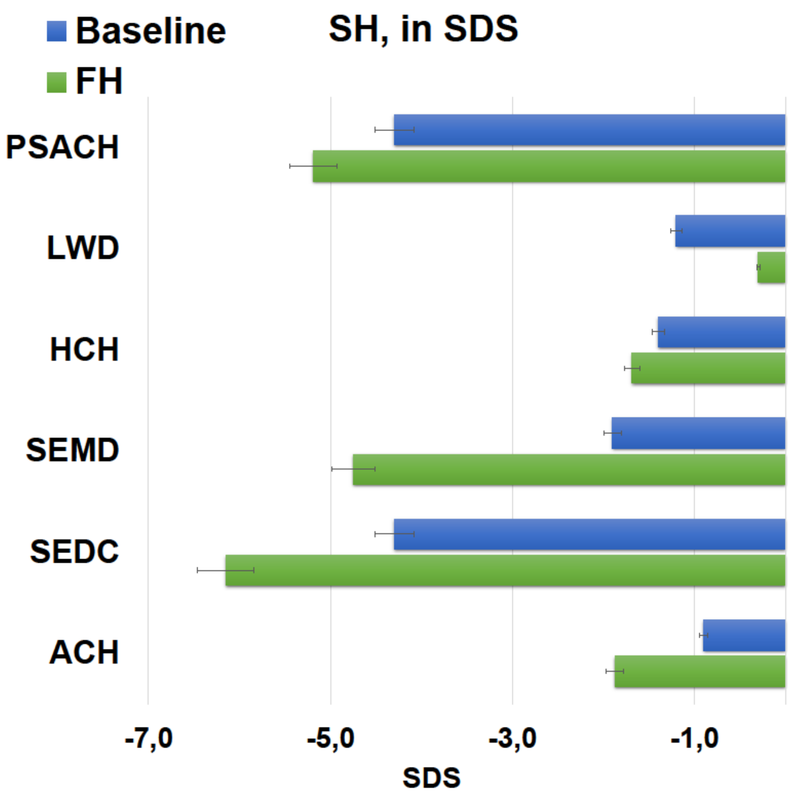
LWD subjects showed a **positive gain** (+0.5±0.75 SD)

The height gain was related to **treatment duration** (T 5.592, P<0,0001) and **Growth Velocity during 1st year of therapy** (T 2.967, P 0,009)



At FH mean **Subischial Leg Lenght** (SLL) was significantly **higher** vs pretherapy in **SEMD** (+1,8±1,8 SD), **ACH** (+0.9±1,8 SD), **LWD** (+0.7±1.9 SD) and in **HCH** (+0.5±1,4 SD) subjects.

At FH mean **Sitting Height** (SH) was significant **lower** vs pretherapy in **ACH** (-1±1,3 SD) and in **HCH** (-0.3±1,4 SD) subjects.



Conclusions

GH therapy in **HCH enhances** mildly the **FH** and **body proportion**, in **LWD enhances FH**, in **ACH** only **body proportions were improved**. **SEMD**, **SEDC** and **PSACH** pts, also with GHD, **should be excluded** from the opportunity to receive **treatment with GH**.

References

Prader et al, 1989; Hertel et al, 2007, Horm Res; Superti-Furga A, Unger S, 2011, Am J Med Genet; Krakow D, Rimoin D, 2010, Genet Med.

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GH and IGF Treatment

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