

0075 - A RANDOMIZED CONTROLLED TRIAL OF ORAL INFIGRATINIB IN CHILDREN WITH ACHONDROPLASIA: RESULTS FROM THE PROPEL 3 STUDY

Mode: Research paper

Thematic Unit: Rare Diseases

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Background/Objectives: Achondroplasia (ACH) is characterized by disproportionate short stature and can be associated with medical complications. Infigratinib is an oral FGFR1–3 tyrosine kinase inhibitor in development for ACH. In this phase 3 trial (PROPEL 3, NCT06164951) in children with ACH aged 3–

Methods: Children were randomized to receive infigratinib at 0.25 mg/kg or placebo once daily for 52 weeks. Exploratory analyses investigated the severity of sleep apnea, rates of otitis media, and changes in body composition using previously described methods.

Results: In total, 114 participants underwent randomization in a 2:1 ratio to receive infigratinib or placebo. Mean CFBL in obstructive apnea-hypopnea index (AHI) and central AHI were smaller with infigratinib vs placebo (obstructive AHI, 0.25 events/hour vs 0.91, respectively; central AHI, 0.06 vs 0.24). In children aged



Conclusion: In children with ACH, treatment with oral infigratinib for 52 weeks resulted in directionally favorable trends in clinically significant exploratory endpoints, suggesting that infigratinib has the potential to provide meaningful benefits beyond growth.

