Achondroplasia.expert literature review

Persistent growth-promoting effects of vosoritide▼ in children with achondroplasia are accompanied by improvements in physical and social aspects of health-related quality of life

Adapted from: Savarirayan R, Irving M, Wilcox WR, Bacino CA, Hoover-Fong JE, Harmatz P, Polgreen LE, Mohnike K, Prada CE, Kubota T, Arundel P, Leiva-Gea A, Rowell R, Low A, Sabir I, Huntsman-Labed A, Day J

Genet Med. 2024. doi: 10.1016/j.gim.2024.101274. Epub ahead of print.

▼This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions. The PI and AE reporting are available at the end of this slide deck.

Achondroplasia.expert is organised and funded by BioMarin. This material has been developed in conjunction with the Achondroplasia.expert Editorial Committee.

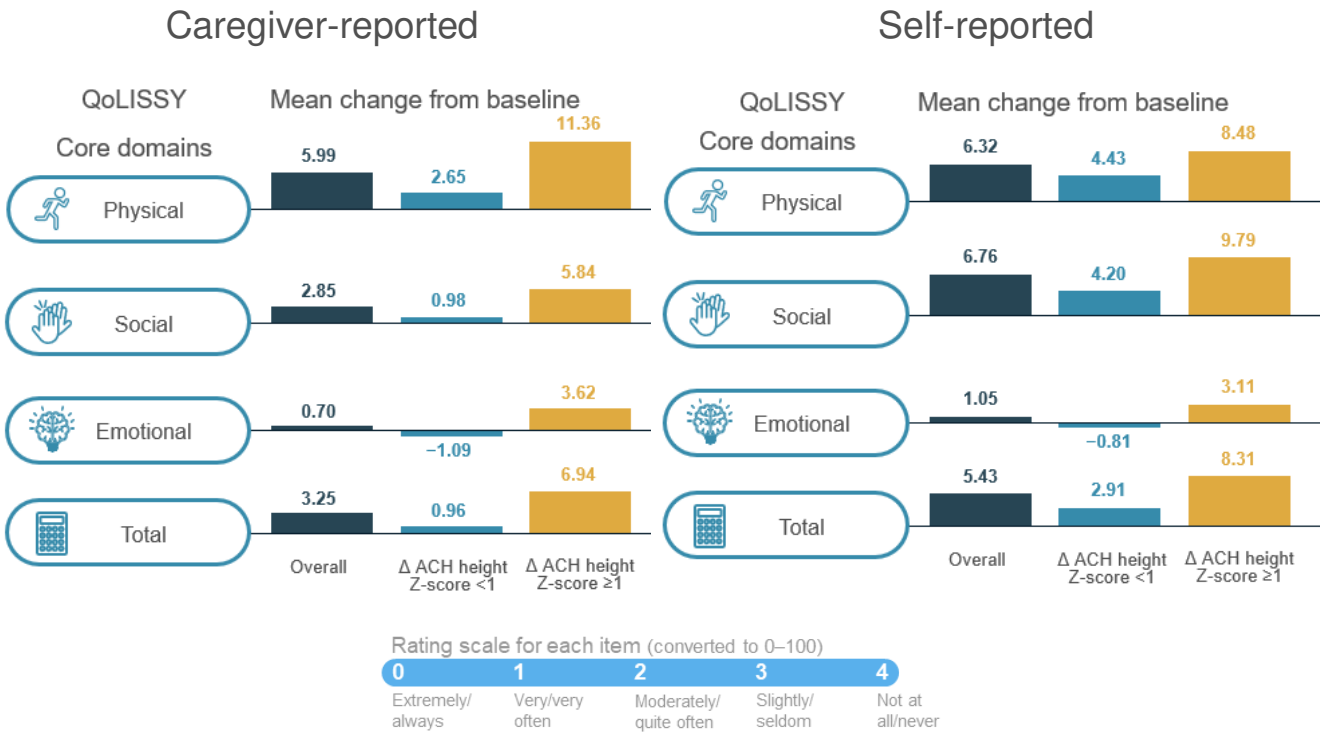
Persistent growth-promoting effects of vosoritide▼ in children with achondroplasia are accompanied by improvements in physical and social aspects of health-related quality of life

Increased AGV with vosoritide demonstrated in clinical trials

- It is unknown whether children with ACH derive HRQoL benefits through the increased height and improved body proportionality achieved with vosoritide
- This analysis focuses primarily on the results at 3-year follow up from an ongoing Phase 3 OLE study, with the aim of investigating the effect of vosoritide on HRQoL in children

OLE study following Phase 3 placebo-controlled study

Caregiver and self-reported QoLISSY change from baseline to year 3 (week 156)



Vosoritide may improve self-reported and caregiver-reported QoLISSY physical and social scores compared to baseline, with the greatest increases being seen in participants with a ≥1 SD increase in height Z-score

*Children were required to have a clinical diagnosis of achondroplasia confirmed with genetic testing to be eligible for enrolment. † Children and/or caregivers completed QoLISSY at baseline and at 6 monthly intervals. ACH, Achondroplasia; HRQoL, Health-related quality of life; OLE, Open-label extension; QoLISSY, Quality of Life in Short Stature Youth; SD, standard deviation. Savarirayan R, et al. Genet Med. 2024. doi: 10.1016/j.gim.2024.101274. Epub ahead of print.

Achondroplasia .expert

ACH.expert VOXZOGO®▼ (vosoritide) Prescribing Information Slide

Achondroplasia .expert

VOXZOGO®▼ (vosoritide) Prescribing Information

- Achondroplasia.expert may contain promotional material on BioMarin products, which is displayed based on the prescribing information approved by the European Medicines Agency – EMA

Access the latest API: VOXZOGO®▼ (vosoritide)

- Within your downloaded Zip File, you will find a copy of the latest Prescribing Information
  - The latest API can also be accessed on **Achondroplasia.expert – Prescribing Information**
  - The prescribing information must form part of the promotional material and must not be separate from it
- Achondroplasia.expert **Terms and Conditions**

Achondroplasia .expert