

# 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.

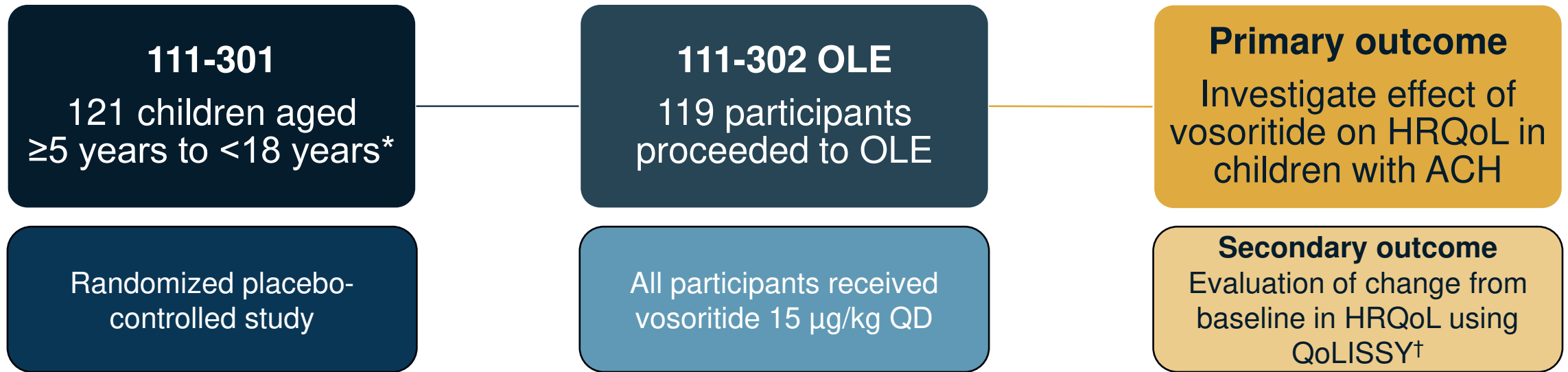
Epub ahead of print.

▼ 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.

# Background

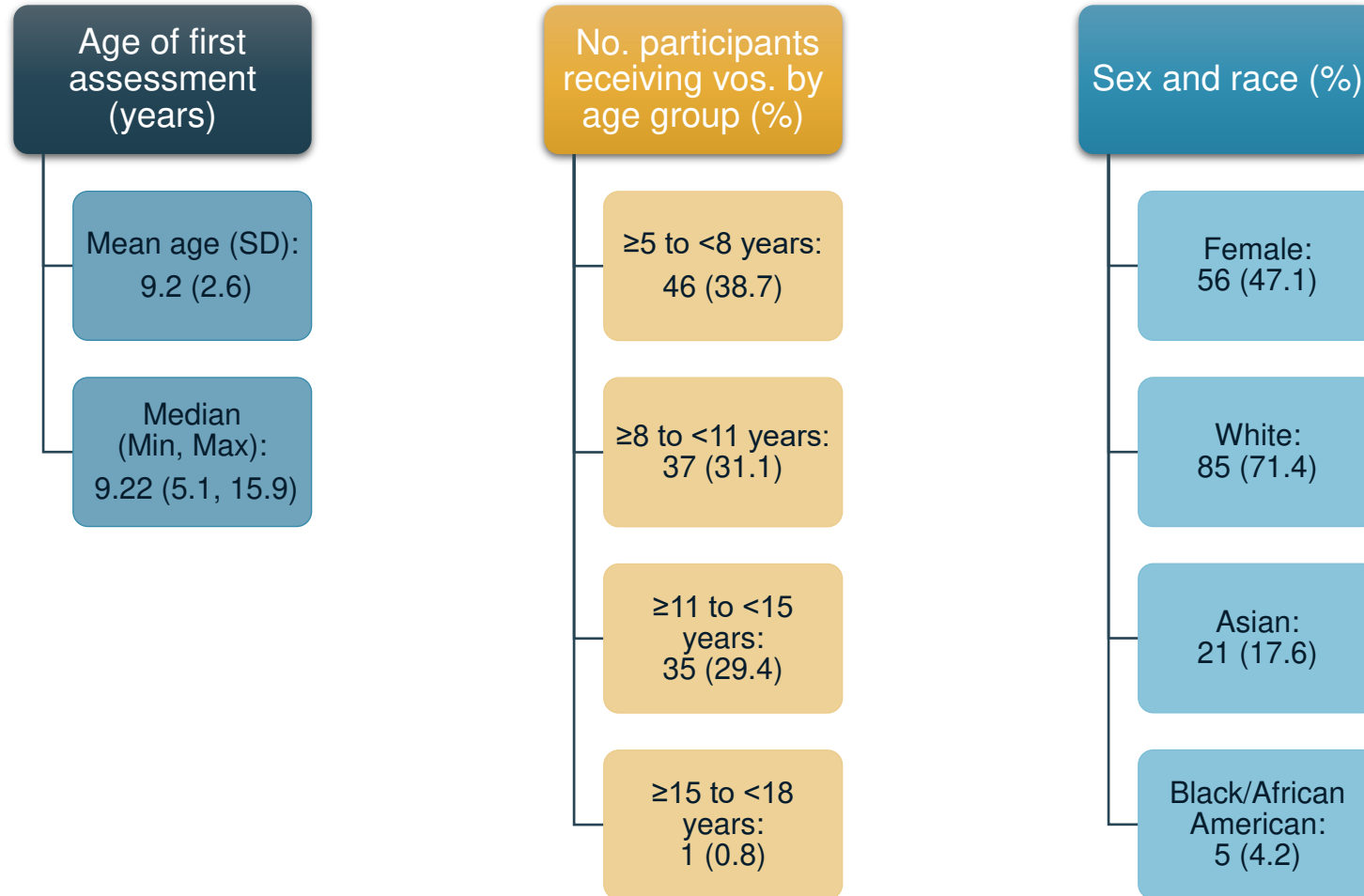
- ▶ Evidence from Phase 2 and 3 clinical trials and extension studies demonstrated that vosoritide treatment lead to an improvement in AGV after 1 year in children with ACH compared to a placebo
  - This improvement in AGV is maintained at 3 years
- ▶ It is unknown whether children with ACH derive HRQoL benefits through the increased height and improved body proportionality achieved with vosoritide
  - These benefits take time to manifest, indicating the need for early initiation of vosoritide to achieve the maximum benefits from increased stature and proportionality
- ▶ 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

# Methods: OLE study following Phase 3 placebo-controlled study

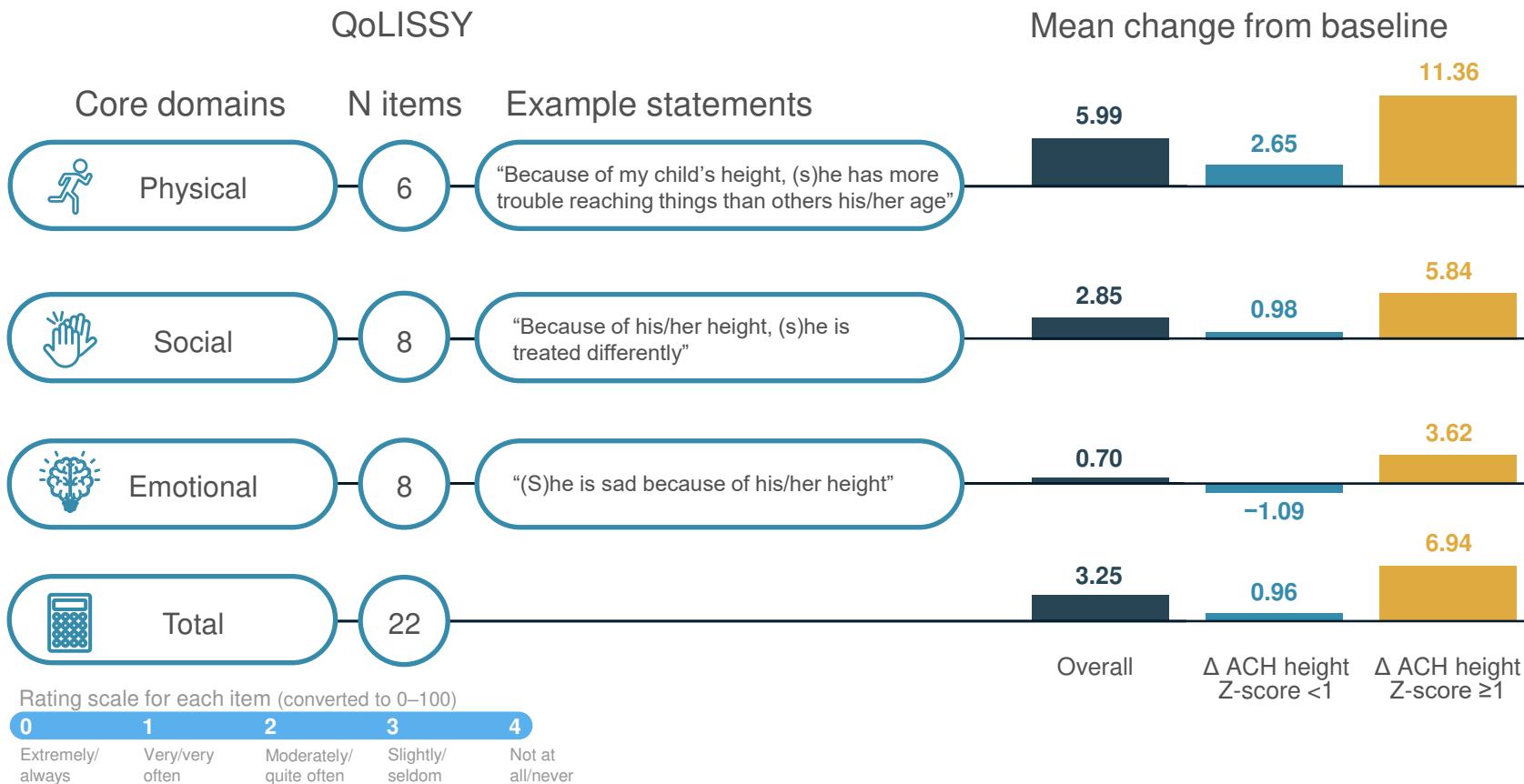


\*Children were required to have a clinical diagnosis of achondroplasia confirmed with genetic testing to be eligible for enrolment. †Children and/or caregivers completed QoLISSY at baseline and at 6 monthly intervals  
ACH, achondroplasia; HRQoL, Health-related quality of life; OLE, Open-label extension;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

# Results: Patient characteristics



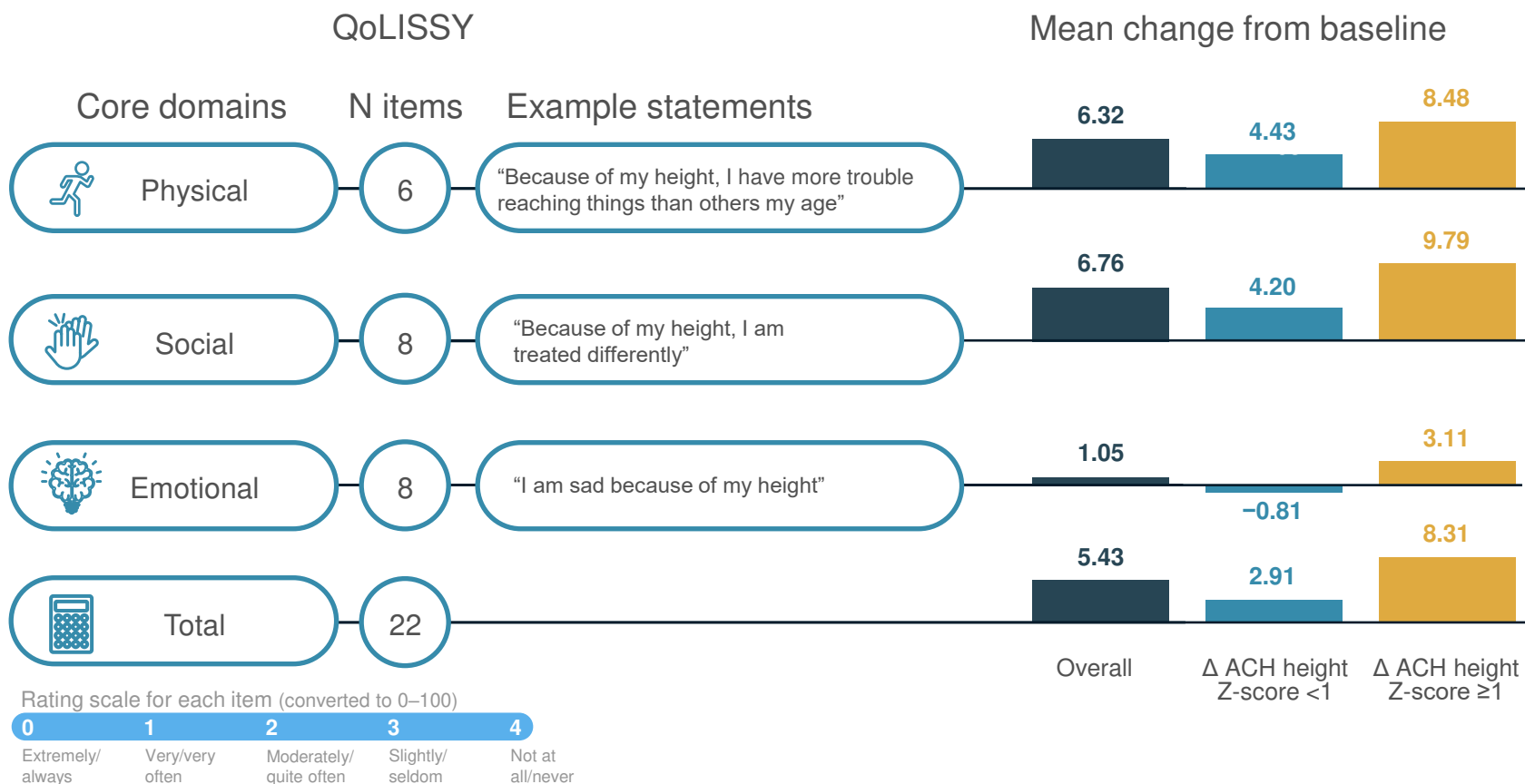
# Results: Caregiver-reported QoLISSY change from baseline to year 3 (week 156)



Vosoritide demonstrated a **positive effect on physical and social functioning**, particularly in children with more pronounced changes in height Z-score

Changes were greatest in participants with  $\geq 1$  SD increase in height Z-score

# Results: Self-reported QoLISSY change from baseline to year 3 (week 156)



Vosoritide demonstrated a **positive effect on physical and social functioning**, particularly in children with more pronounced changes in height Z-score

Changes were greatest in participants with  $\geq 1$  SD increase in height Z-score

# Conclusions

- ▶ Results from this study suggest that 3 years of treatment with vosoritide may translate into improvements in the HRQoL of children with ACH, as shown by increased QoLISSY scores over time
- ▶ Changes were most marked in physical and social domain scores
- ▶ Greatest improvements were reported in patients with  $\geq 1$  SD increase in height Z-score

**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  $\geq 1$  SD increase in height Z-score**

**ACH.expert VOXZOGO<sup>®</sup> ▼ (vosoritide)  
Prescribing Information Slide**



# VOXZOGO<sup>®</sup> ▼ (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<sup>®</sup> ▼ (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](#)

## **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**

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.

<https://pubmed.ncbi.nlm.nih.gov/39305160/>

Savarirayan et al. assessed the impact of vosoritide on growth-associated health-related quality of life (HRQoL) in children with achondroplasia. Data were gathered using the Quality of Life in Short Stature Youth (QoLISSY) questionnaire. Vosoritide improved self-reported and caregiver-reported QoLISSY physical and social scores, with the greatest increases being seen in participants with a  $\geq 1$  SD increase in height Z-score.

A total of 121 children aged 5 to <18 years were enrolled in the 111-301 study. Of these, 119 proceeded to the 111-302 open-label extension study. All participants received vosoritide at a daily dose of 15  $\mu\text{g}/\text{kg}$ . At Year 3, improvements were noted in QoLISSY physical and social scores, with the greatest improvements observed in participants with  $\geq 1$  standard deviation increase in height Z-score. These results indicate that there are quality of life benefits related to improved height deficit in children with achondroplasia treated with vosoritide.

▼ 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. For Healthcare Professionals Only.

© 2024 BioMarin International Ltd.  
All Rights Reserved. EUCAN-VOX-00720 09/24