



DEMONSTRATES A POSITIVE EFFECT ON PHYSICAL AND SOCIAL FUNCTIONING AMONG CHILDREN WITH ACHONDROPLASIA¹

THE GREATEST EFFECTS ARE SEEN IN THE CHILDREN WHO GREW THE MOST¹

Compared with children of average stature, children with achondroplasia experience poorer **health-related quality of life (HRQoL)**, especially when it comes to their physical and social well-being.²⁻⁵

The **'Quality of Life of Short Stature Youth' (QoLISSY)** questionnaire is a commonly used and reliable tool for measuring the HRQoL of children with short stature, including those with achondroplasia.^{2,4-6}

It is completed by both the **children and their caregivers**.⁶

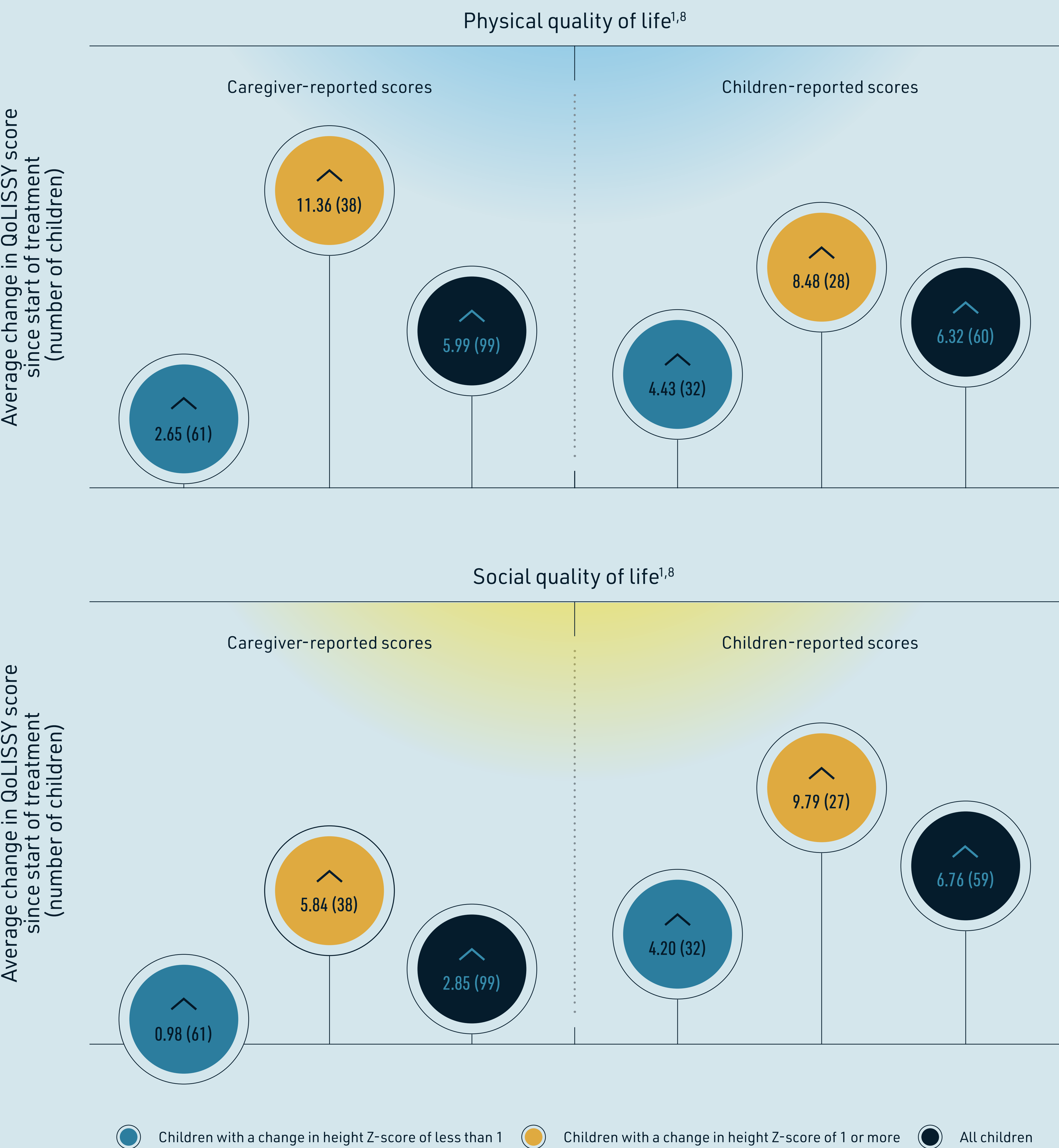


Vosoritide is indicated for the treatment of achondroplasia in patients 4 months of age and older whose epiphyses are not closed.⁷ The diagnosis of achondroplasia should be confirmed by appropriate genetic testing.⁷

The most common adverse reactions to vosoritide were injection-site reactions (85%), vomiting (27%) and decreased blood pressure (13%).⁷

USING QoLISSY, BOTH CHILDREN WITH ACHONDROPLASIA AND THEIR CAREGIVERS REPORTED IMPROVEMENTS IN PHYSICAL ABILITIES AND SOCIAL WELL-BEING AFTER 3 YEARS OF VOSORITIDE TREATMENT¹

The greatest improvements were seen in the children who grew the most^{*1}



*The amount of growth the children experienced was determined using height Z-score,¹ which measures how a child's height compares with the average height of children with achondroplasia.⁹ A Z-score of 0 means the child's height is average.⁹ A negative Z-score means the child is shorter than average.⁹ A positive Z-score means the child is taller than average.⁹

In QoLISSY, statements (e.g., 'I am sad because of my height') are used to measure the views of children and caregivers.⁶ A total of 6 and 8 statements are used in the physical and social sections of the questionnaire, respectively.⁶

The children who took part in the study were aged 5 to <18 years at start of treatment.¹ Children-reported data were limited to those aged 8 years or over at the start of treatment.¹

For patients



Better physical abilities



Enhanced independence



More social integration



Greater self-esteem

For caregivers



Emotional relief



Lighter caregiving burden



Less financial impact



Better family dynamics

What do these results potentially mean?²

AE, adverse event; HRQoL, health-related quality of life; QoLISSY, Quality of Life of Short Stature Youth.

References: 1. Savarirayan R, et al. *Genet Med.* 2024;26(12):101274. 2. Shediak R, et al. *Mol Genet Genomic Med.* 2022;10(4):e1891. 3. Witt S, et al. *Orphanet J Rare Dis.* 2019;14(1):194. 4. Llerena J, et al. *GIM Open.* 2024;2:100843. 5. Maghnie M, et al. *Orphanet J Rare Dis.* 2023;18(1):56. 6. Bullinger M, et al. *Health Qual Life Outcomes.* 2013;11:76. 7. VOXZOGO® Summary of Product Characteristics. 8. Savarirayan R, et al. *Genet Med.* 2024;26(12):101274. Supplementary Appendix. 9. Martinez-Millana A, et al. *PLoS ONE.* 2018;13(12):e0208362.

Access the **VOXZOGO® (vosoritide)** prescribing information and AE-reporting instructions.

▼ 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 AEs. The AE-reporting statement can be accessed from the link above.

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