Approval Signatures

Name: VOXZOGO QoL Infographic (achondroplasia.expert)

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Document Approvals	
Review & Certify (AQP Signatory) Completion of this task indicates that you have conducted approval and sign off for "VOXZOGO QoL Infographic (achondroplasia.expert)".	Executive Director, Head of Marketing, Europe and Canada Alan Harding (aharding@bmrn.com) Marketing - Approved 10-Feb-2025 12:57:49 GMT+0000
Review & Certify (Medical Signatory) Completion of this task indicates that you have conducted approval and sign off for "VOXZOGO QoL Infographic (achondroplasia.expert)".	Senior Medical Director Swati Mukherjee (sw898266@bmrn.com) Medical - Approved 12-Feb-2025 12:42:01 GMT+0000

VOSORITIDE▼

DEMONSTRATES A POSITIVE EFFECT ON PHYSICAL AND SOCIAL FUNCTIONING AMONG CHILDREN WITH ACHONDROPLASIA[1]

THE GREATEST EFFECTS ARE SEEN IN THE CHILDREN WHO GREW THE MOST[1]

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]

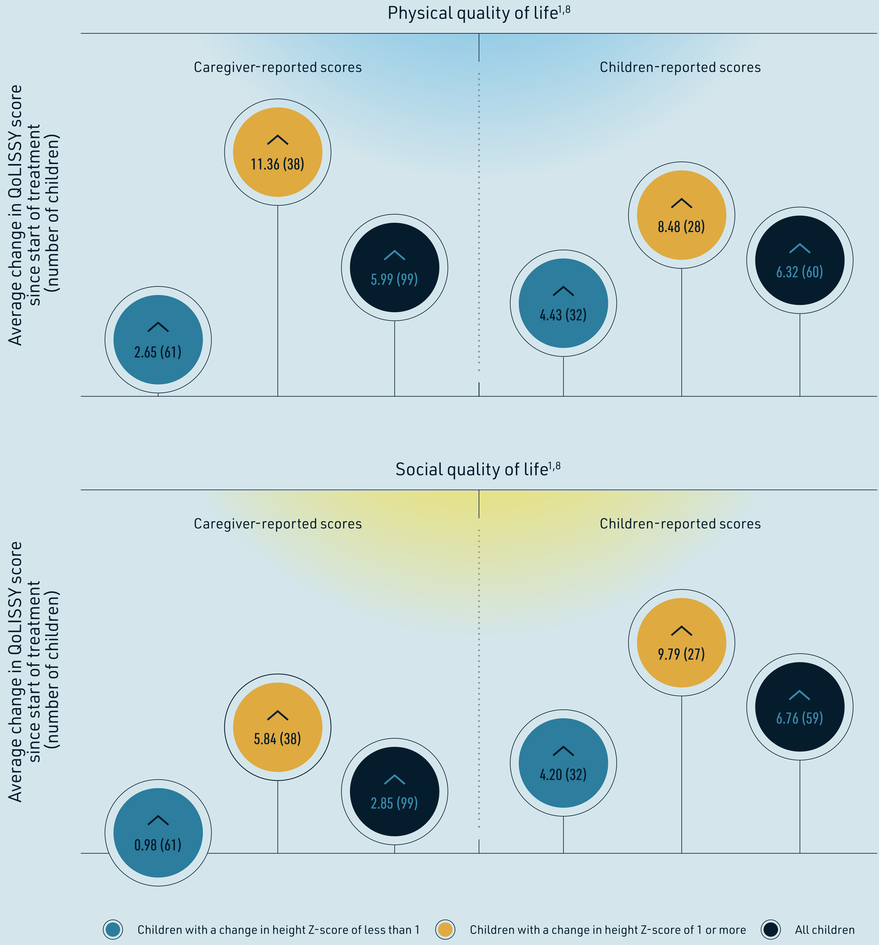
It is completed by both the **children and their caregivers.**[6]

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 TREATMENT[1]

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]

What do these results potentially mean?[2]

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

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.** Shediac 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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