C-0090

Caregiver perspectives on vosoritide treatment: meaningful HRQoL improvements in children with achondroplasia

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Background

Achondroplasia, caused by a gain-of-function mutation in *FGFR3*, leads to severe disproportionate stature, varying multisystem complications, and functional consequences that have a significant impact on health-related quality of life[1,2]

Vosoritide, a C-type natriuretic protein (CNP) analog, stimulates endochondral bone growth by counteracting overactive FGFR3 signaling; it is the first and only approved targeted treatment for children with achondroplasia from infancy[3,4]

In infants and children with achondroplasia, vosoritide significantly improves growth from birth, resulting in sustained increases in height over time[5]

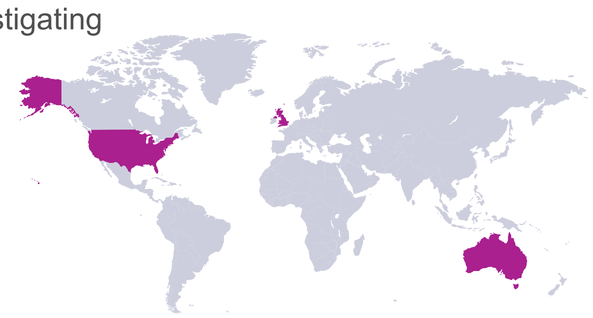
This qualitative analysis summarizes caregiver perspectives on their experience with achondroplasia, meaningful treatment goals, and the impact of vosoritide on their child's daily life

Conclusions

- **This work provides evidence to support emerging findings from clinical trials by qualitatively elucidating the benefits of vosoritide and how this treatment may improve many aspects of daily life for individuals with achondroplasia**
- Ongoing analysis of the HRQoL data collected from clinical trials aims to provide further evidence of the impact of vosoritide over time

Methods

- Written testimonials were collected from caregivers of children with achondroplasia (N=12) who were enrolled in clinical trials investigating the safety and efficacy of vosoritide (111-205, 111-206 and 111-301) in Australia, the United States, and the United Kingdom
- Caregivers were asked to provide a brief written narrative characterizing the impact of achondroplasia on their children's lives and any changes observed in their children while on vosoritide
- The statements were thematically analyzed using the qualitative research tool ATLAS.ti and a codebook based on a conceptual model of the lived experience of children and adolescents with achondroplasia, which included signs, symptoms and clinical presentation, impact on wellbeing, and impact on physical functioning[6]



Results

- Caregivers reported meaningful improvements in a range of signs, symptoms, and HRQoL impacts of achondroplasia after vosoritide treatment
- Overall, families' experiences with vosoritide were positive (n=9), with the benefits of treatment outweighing the inconvenience of daily injections

We have noticed some negatives associated with the injections, however, the positives far outweigh the negatives

The daily injection and preparation of such has minimal impact on our lives

We have found a bike with gears that didn't need special adjustments, allowing connection with peers

Her reach has improved to the point where she can brush her hair

Legs have straightened, legs no longer bowed

His confidence has soared. The increased independence has [also] decreased how often others see and treat him as if he is a baby

We have less issues and involvement with putting on school shorts with a button, going to the toilet no longer needs to be monitored

We have seen his spine straighten which has given him better posture

Her fingers have definitely grown

Her confidence has certainly grown when it comes to self-care

He can run fast and scores goals for his soccer team

…On her tenth birthday, we went to the sports shop where she was able to sit for the first time on a standard bike. Within two days she was cycling unaided…

Changes in ACH signs and symptoms (N=12)

Sign/symptom changes endorsed	Caregivers endorsing change N (%)
Height increase	N=7 (58)
Longer fingers	N=2 (17)
Straighter limbs	N=3 (25)
Straighter spine	N=2 (17)

Impact on wellbeing (N=12)

Impact endorsed	Caregivers endorsing change N (%)
Improved confidence	N=6 (50)
Improved social interactions	N=5 (42)

Impact on physical functioning (N=12)

Impact endorsed	Caregivers endorsing change N (%)
Higher reach	N=6 (50)
Improved self-care (toileting, showering, brushing hair, dressing)	N=5 (42)
Improved walking	N=2 (17)
Improved running	N=4 (33)
Improved balance	N=3 (25)
Able to ride bike	N=3 (25)

ACH, achondroplasia; FGFR3, fibroblast growth factor receptor 3; HRQoL, health-related quality of life

References

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