Real-World Health Care Utilization, Effectiveness, Health-Related Quality of Life, and Adherence to Vosoritide in Children with Achondroplasia in the United States

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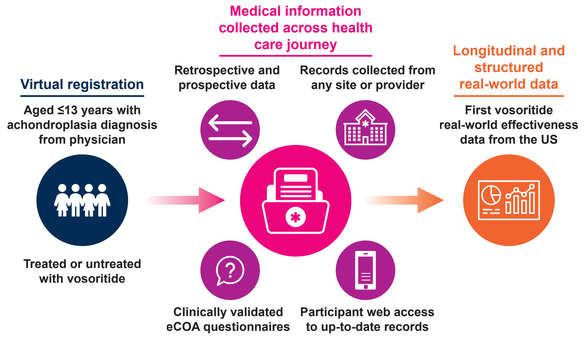
Introduction

- Achondroplasia is a rare skeletal dysplasia that impairs endochondral ossification by inhibiting chondrocyte proliferation and differentiation, often resulting in medical complications and functional impairment[1,2]
- Vosoritide, a C-type natriuretic peptide analog that stimulates endochondral bone growth, is the first approved targeted treatment for children with achondroplasia based on significant improvements in growth-related parameters (eg, annualized growth velocity [AGV] and body proportionality) and quality of life in clinical trials[1–7]
  - Vosoritide was first approved in the United States in November 2021 for children aged ≥5 years of age and since expanded to include treatment from birth (October 2023)[4]
- Although real-world evidence for vosoritide safety and effectiveness is growing, particularly from Europe and Japan, data from the US are limited[8–11]
- Here, we report interim effectiveness and electronic clinical outcome assessments (eCOAs) from children with achondroplasia ≤13 years of age after treatment initiation from the VIrtual STudy in Achondroplasia for the US (VISTA; NCT06168201)

Methods

- VISTA is an ongoing, decentralized, virtual, observational cohort study of individuals with achondroplasia in the US treated or untreated with vosoritide
  - Enrollment began February 21, 2023, and focused on children who were ≤13 years of age starting October 2024
- Participant-mediated access to retrospective and prospective medical records and eCOAs were collected via the virtual PicnicHealth Platform (**Figure 1**)
  - Caregivers completed eCOAs at enrollment and every 6 months thereafter

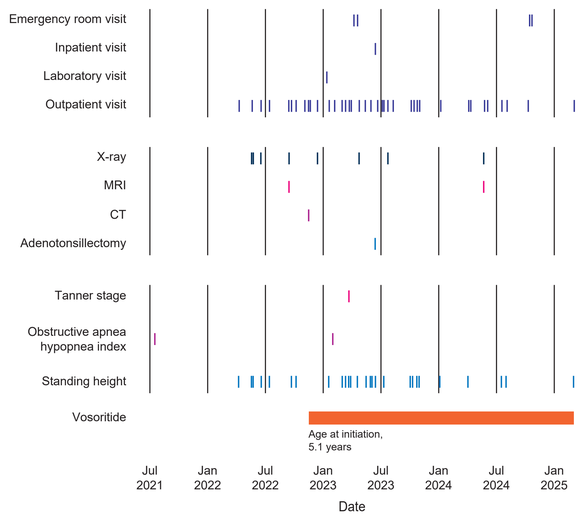
Figure 1. VISTA study design



eCOA, electronic clinical outcome assessment.

- VISTA enables a holistic view of individual health care journeys without the need for data entry by participants or investigators (**Figure 2**)

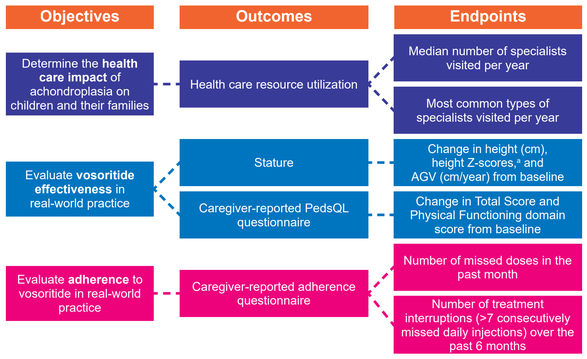
Figure 2. A patient health care journey



Data are from a male sample participant for illustrative purposes.
CT, computed tomography; MRI, magnetic resonance imaging.

- This interim analysis evaluated health care resource utilization and eCOAs at baseline and years 1–3 of study participation in vosoritide-treated participants (**Figure 3**)
  - Stature-related measurements were analyzed in participants with 1 year (±60 days) of vosoritide exposure

Figure 3. Interim analysis objectives and endpoints



[a]Referenced to average-stature population (CDC) and untreated children with achondroplasia (CLARITY[12]).
AGV, annualized growth velocity; CDC, US Centers for Disease Control and Prevention; PedsQL, Pediatric Quality of Life Inventory.

Results

Population

- As of April 17, 2025, 49 participants had enrolled across 23 states (**Figure 4**), 37 of whom were treated with vosoritide
  - 17 participants had ≥1 year of treatment
- Mean (standard deviation) age at vosoritide initiation was 4.4 (3.6) years (minimum, 0.1 years; **Table 1**)

Figure 4. Geographic distribution of VISTA enrollment as of April 2025

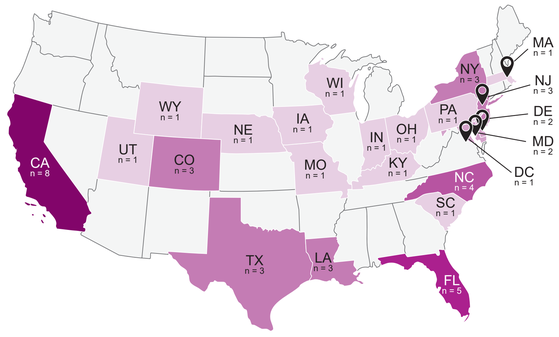


Table 1. Baseline characteristics for participants treated with vosoritide

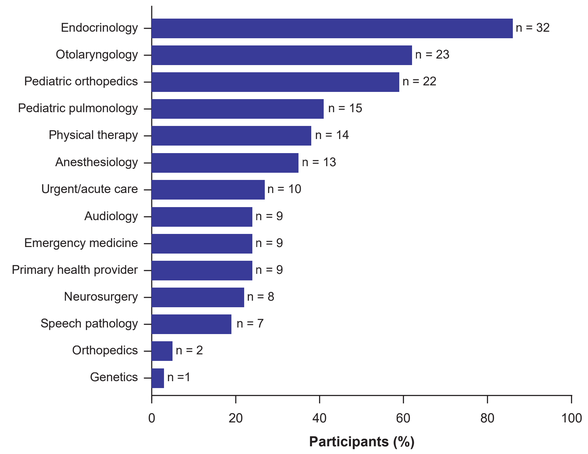
	Vosoritide n = 37
Age at vosoritide initiation, years	
Mean (SD)	4.4 (3.6)
Min, max	0.1, 12.1
Age at enrollment, years	
Mean (SD)	4.9 (4.1)
Min, max	0.1, 12.8
Sex, n (%)	
Male	19 (51.4)
Female	18 (48.6)
Race,[a] n (%)	
White	26 (70.3)
Asian	1 (2.7)
Black or African American	5 (13.5)
More than one race	3 (8.1)
Other	1 (2.7)
Ethnicity,[a] n (%)	
Not Hispanic or Latino	32 (86.5)
Hispanic or Latino	4 (10.8)
Treatment duration, years	
Mean (SD)	1.2 (0.9)
Min, max	0.0, 2.8
Height, cm	
Female, median (IQR)	82.5 (61.5, 100.0)
Male, median (IQR)	77.5 (62.2, 88.9)
Height Z-sore (CDC)	
Female, median (IQR)	−4.4 (−5.6, −2.8)
Male, median (IQR)	−4.5 (−5.5, −3.9)
Achondroplasia height Z-score (CLARITY[12])	
Female, median (IQR)	−0.2 (−0.8, 1.2)
Male, median (IQR)	−0.4 (−0.6, 0.8)

[a]One participant did not disclose
CDC, US Centers for Disease Control and Prevention; IQR, interquartile range; SD, standard deviation.

Health care resource utilization

- Across follow-up, participants visited a median (interquartile range) of 7 (5.0–11.0) different health care providers over the course of a year
- Participant care was multidisciplinary: across 14 specialties documented, the most common were endocrinology (86%), otolaryngology (62%), pediatric orthopedic (59%), and pediatric pulmonology (41%) (**Figure 5**)

Figure 5. Specialists visited by the highest proportions of participants



Effectiveness

- Effectiveness results for 17 participants with 1 year of vosoritide exposure are shown in **Table 2**
  - After 1 year of treatment, median AGV was 7.44 cm/year for male and 5.41 cm/year for female participants

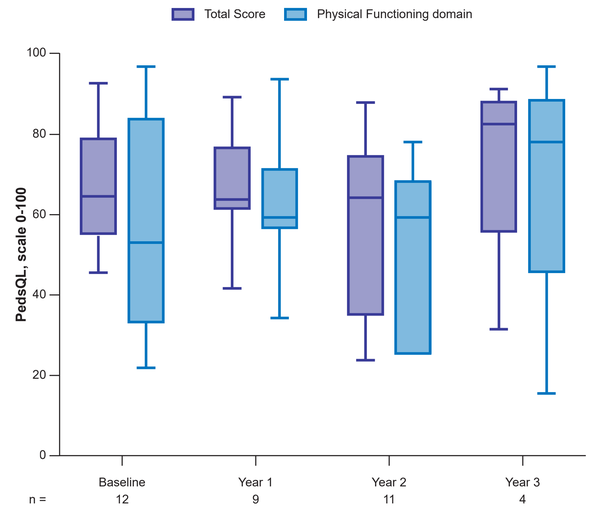
Table 2. Height, height Z-score, and AGV for participants with 1 year (±60 days) of vosoritide exposure

Median (IQR)	Males n = 8	Females n = 9
Height, cm	87.25 (71.25, 103.48)	96.50 (76.20, 106.00)
Height Z-score (CDC)	−4.22 (−4.66, −3.92)	−3.90 (−5.36, −3.50)
Achondroplasia height Z-score (CLARITY[12])	0.41 (0.05, 1.05)	1.19 (−0.01, 1.47)
AGV, cm/year	7.44 (6.21, 11.96)	5.41 (4.71, 10.16)

AGV, annualized growth velocity; CDC, US Centers for Disease Control and Prevention; IQR, interquartile range.

- On the Pediatric Quality of Life Inventory (PedsQL) at year 3 of follow-up (n = 4), caregivers reported improvements in the median Total Score and the Physical Functioning domain score from baseline (**Figure 6**)

Figure 6. Caregiver-reported PedsQL Total Score and Physical Functioning domain score over time

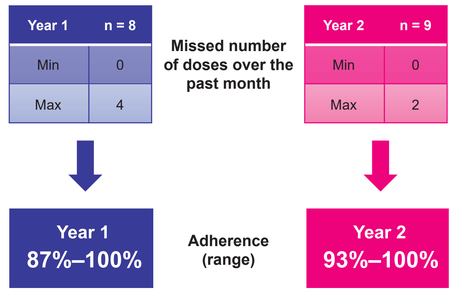


Horizontal lines represent medians. Boxes and whiskers represent IQR and min/max, respectively.
IQR, interquartile range; PedsQL, Pediatric Quality of Life Inventory.

Adherence

- After year 1 (n = 8) and 2 (n = 9) of follow-up, caregivers reported that vosoritide adherence was high in the past month (**Figure 7**)
- No participants had treatment interruptions in the past 6 months (defined as missing >7 consecutive daily injections) at years 1 and 2 of study follow-up

Figure 7. Caregiver-reported missed doses and adherence over the past month at years 1 and 2 of follow-up



Conclusions

- These retrospective and prospective data reveal the complex, multidisciplinary, and multidimensional nature of routine care for children with achondroplasia
  - Many participants reported visiting multiple health care providers across different specialties during routine clinical practice
- These interim results from VISTA are the first real-world data on vosoritide use and effectiveness in practice in the US
  - Effectiveness data, including linear growth and improvements in quality of life, were consistent with real-world data from other countries as well as clinical trial data[5–11,13]
  - Trends in improvements in physical functioning underscore the importance of early treatment initiation to maximize the clinical benefit of vosoritide
- VISTA is still ongoing, with >100 participants currently enrolled. Future VISTA analyses are designed to include comparisons with untreated participants

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Disclosures

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