

# 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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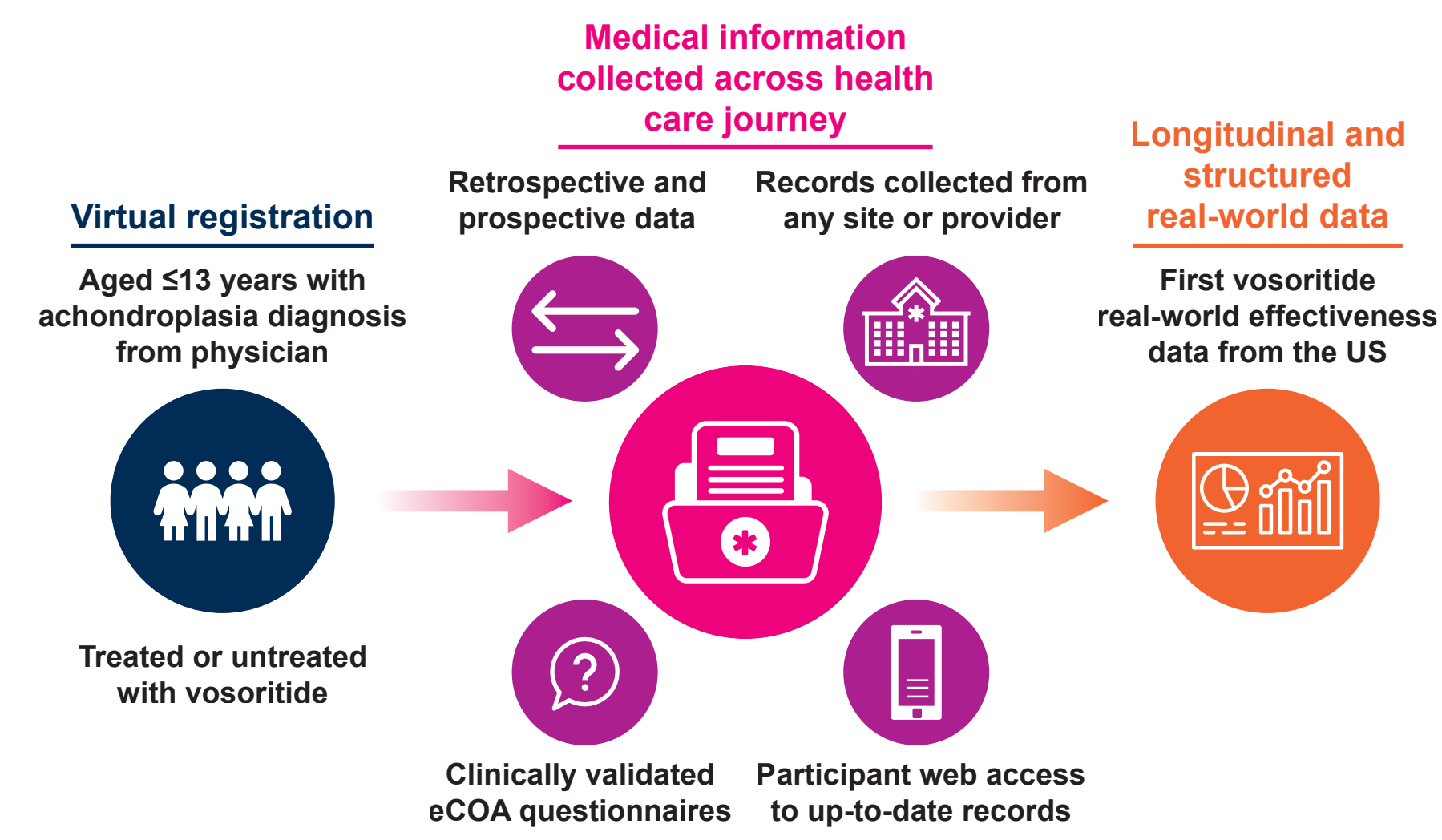
## Introduction

- Achondroplasia is a rare skeletal dysplasia that impairs endochondral ossification by inhibiting chondrocyte proliferation and differentiation, often resulting in medical complications and functional impairment<sup>1,2</sup>
- 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<sup>1-7</sup>
  - 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)<sup>4</sup>
- Although real-world evidence for vosoritide safety and effectiveness is growing, particularly from Europe and Japan, data from the US are limited<sup>8-11</sup>
- 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

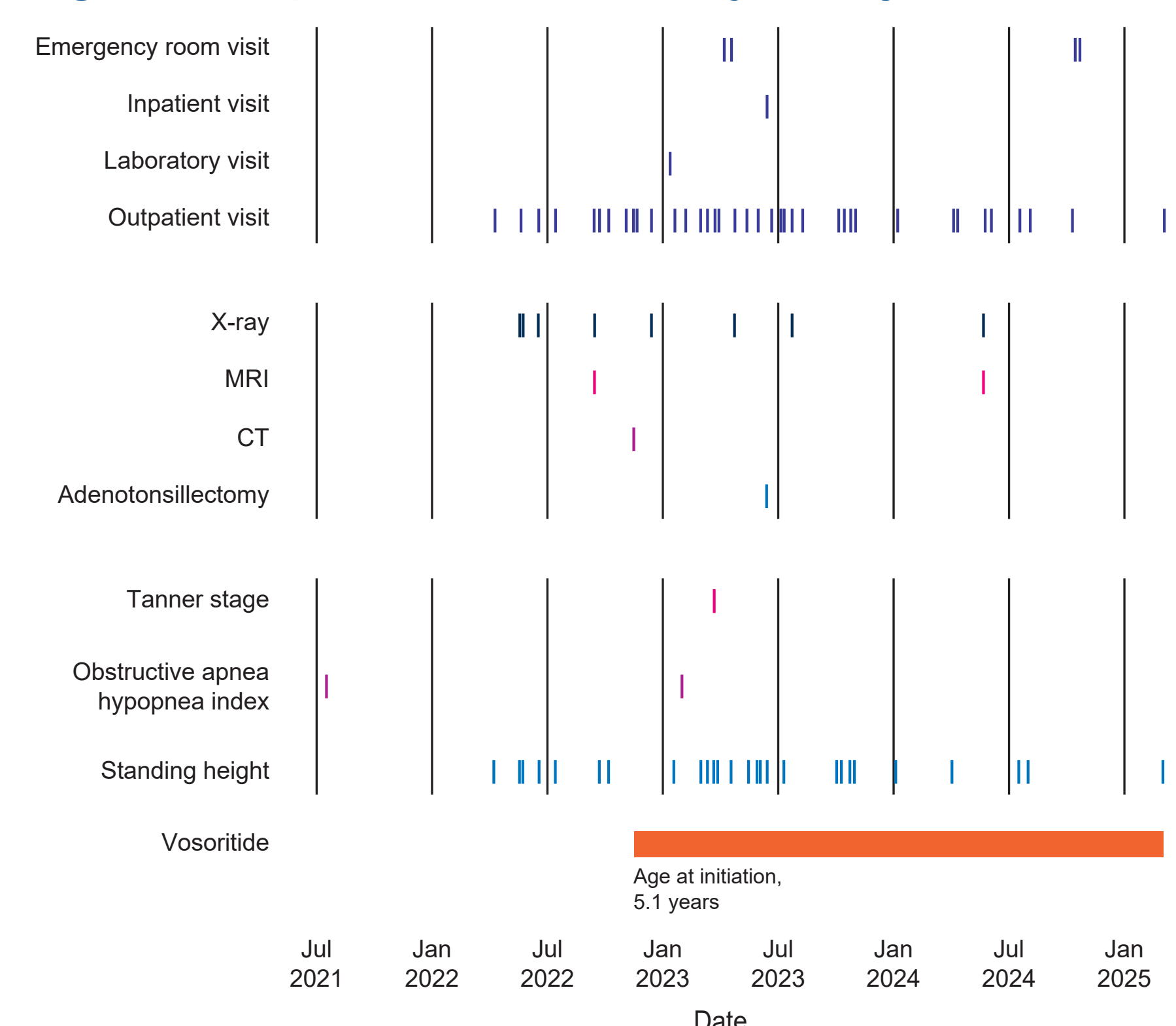
- VISTA is an ongoing, decentralized, virtual, observational cohort study of individuals with achondroplasia in the US treated or untreated with vosoritide
  - Enrollment began February 21, 2023, and focused on children who were ≤13 years of age starting October 2024
- Participant-mediated access to 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



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

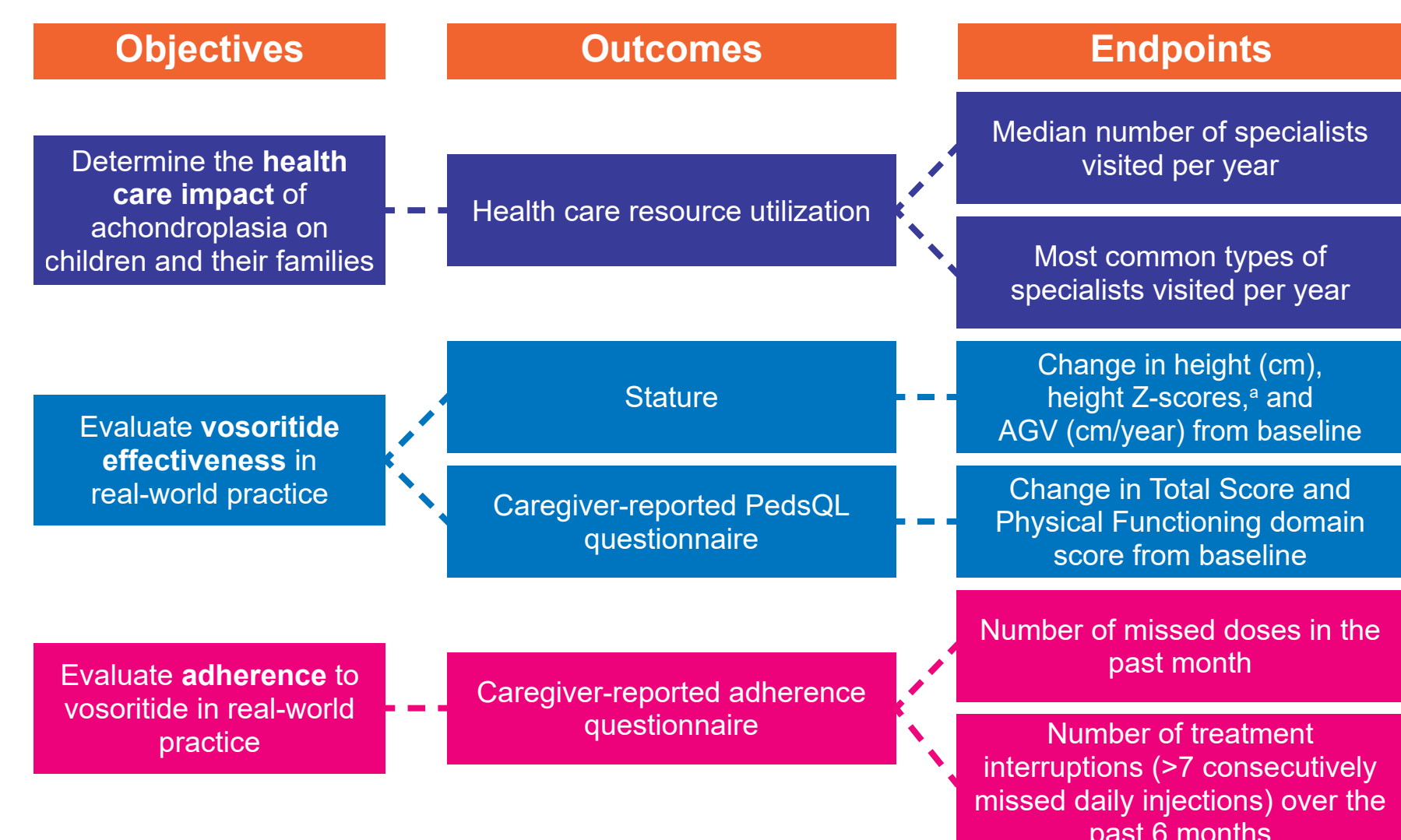
Figure 2. A patient health care journey



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



\*Referenced to average-stature population (CDC) and untreated children with achondroplasia (CLARITY<sup>12</sup>). 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

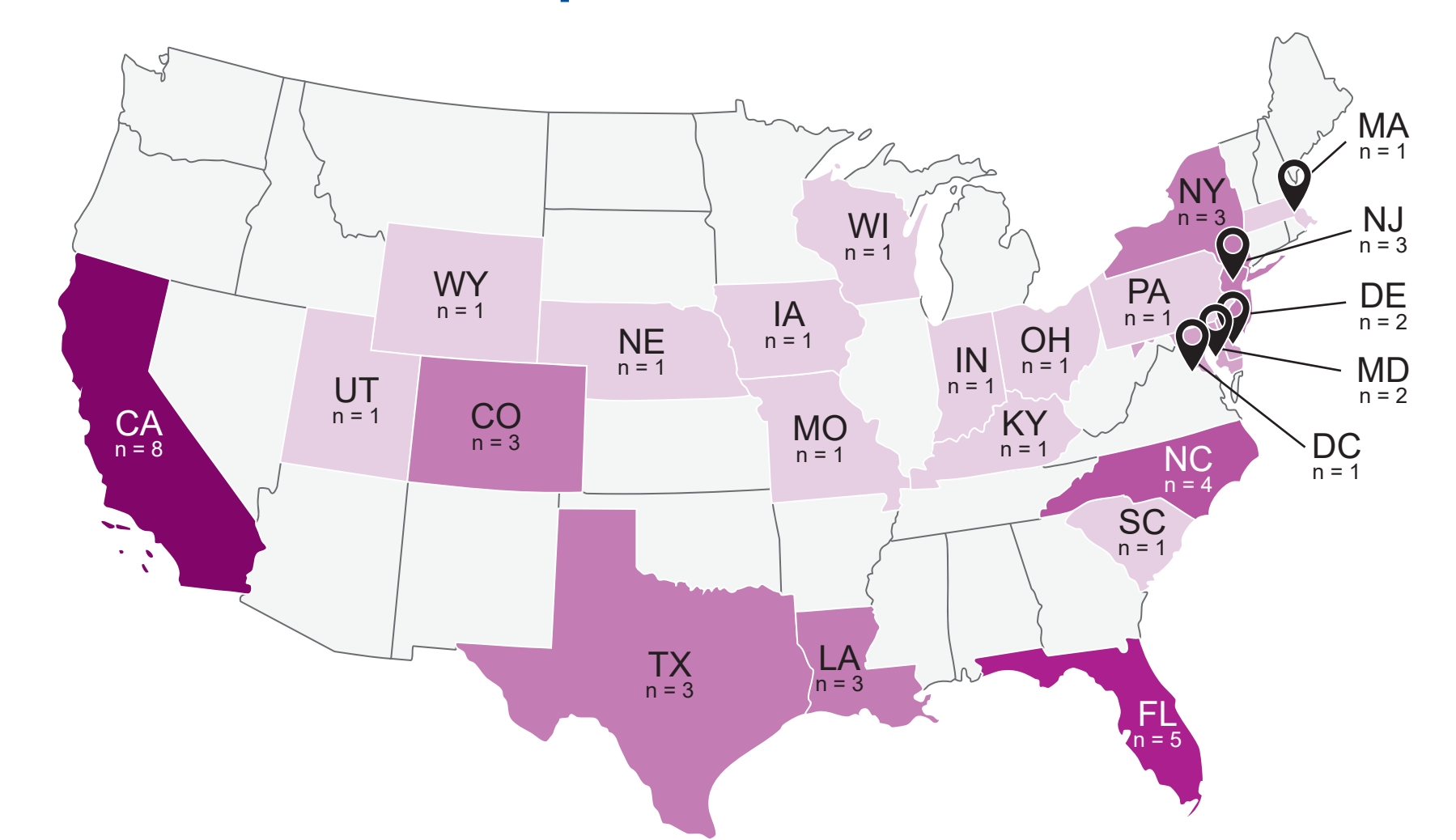


Table 1. Baseline characteristics for participants treated with vosoritide

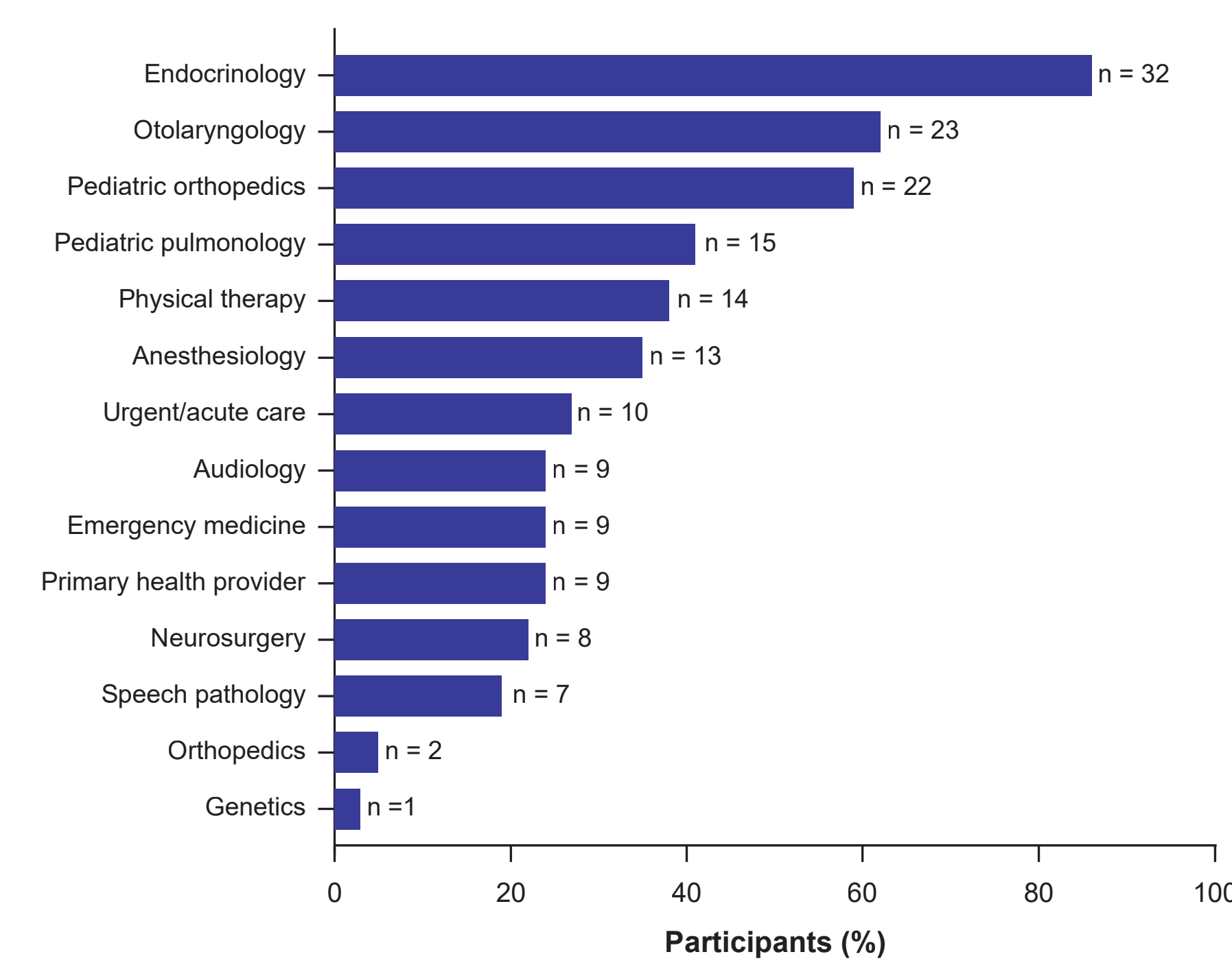
	Vosoritide n = 37
<b>Age at vosoritide initiation, years</b>	
Mean (SD)	4.4 (3.6)
Min, max	0.1, 12.1
<b>Age at enrollment, years</b>	
Mean (SD)	4.9 (4.1)
Min, max	0.1, 12.8
<b>Sex, n (%)</b>	
Male	19 (51.4)
Female	18 (48.6)
<b>Race,<sup>a</sup> n (%)</b>	
White	26 (70.3)
Asian	1 (2.7)
Black or African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

<sup>a</sup>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

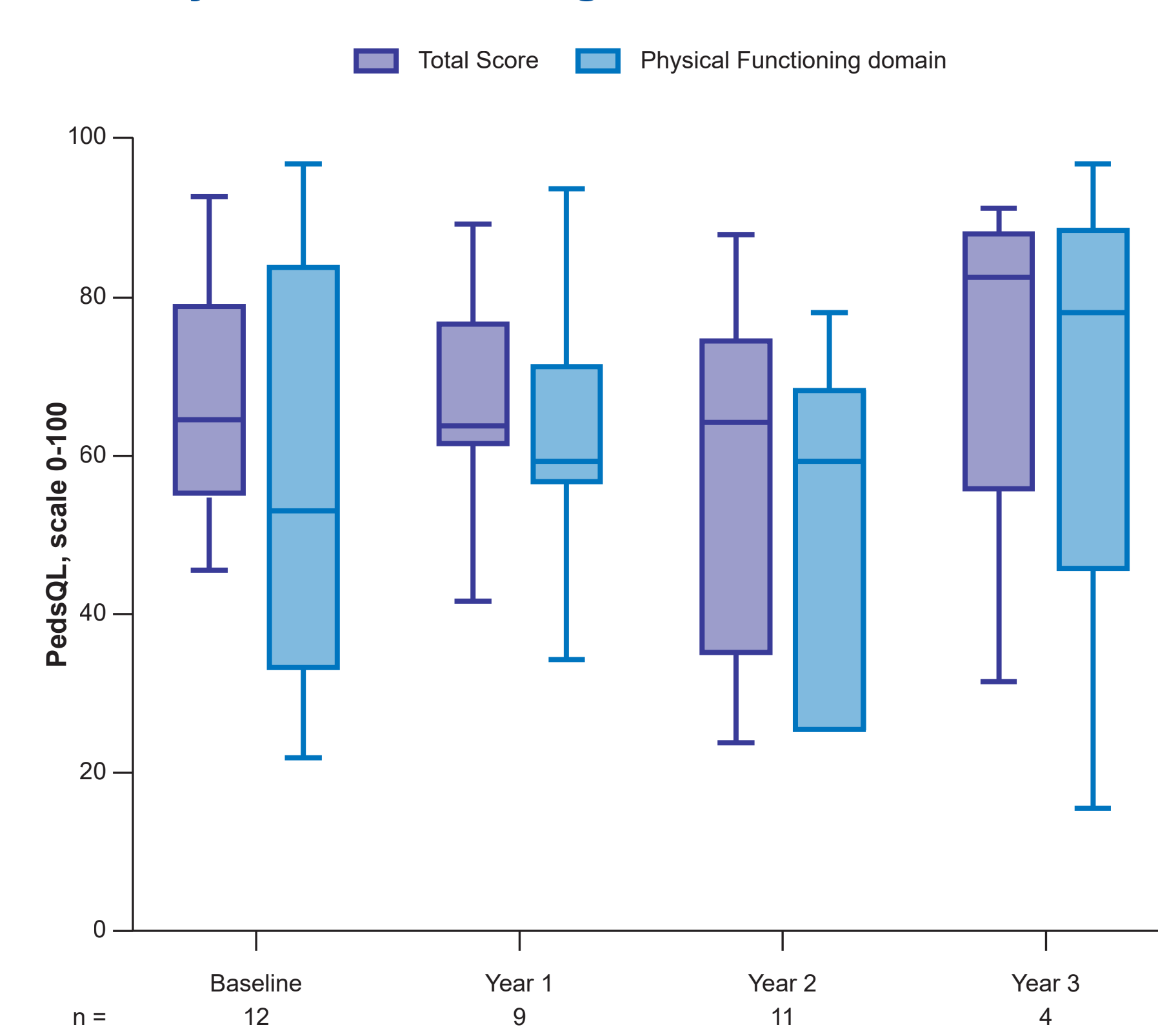
Table 2. Height, height Z-score, and AGV for participants with 1 year (±60 days) of vosoritide exposure

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 <sup>12</sup> )	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

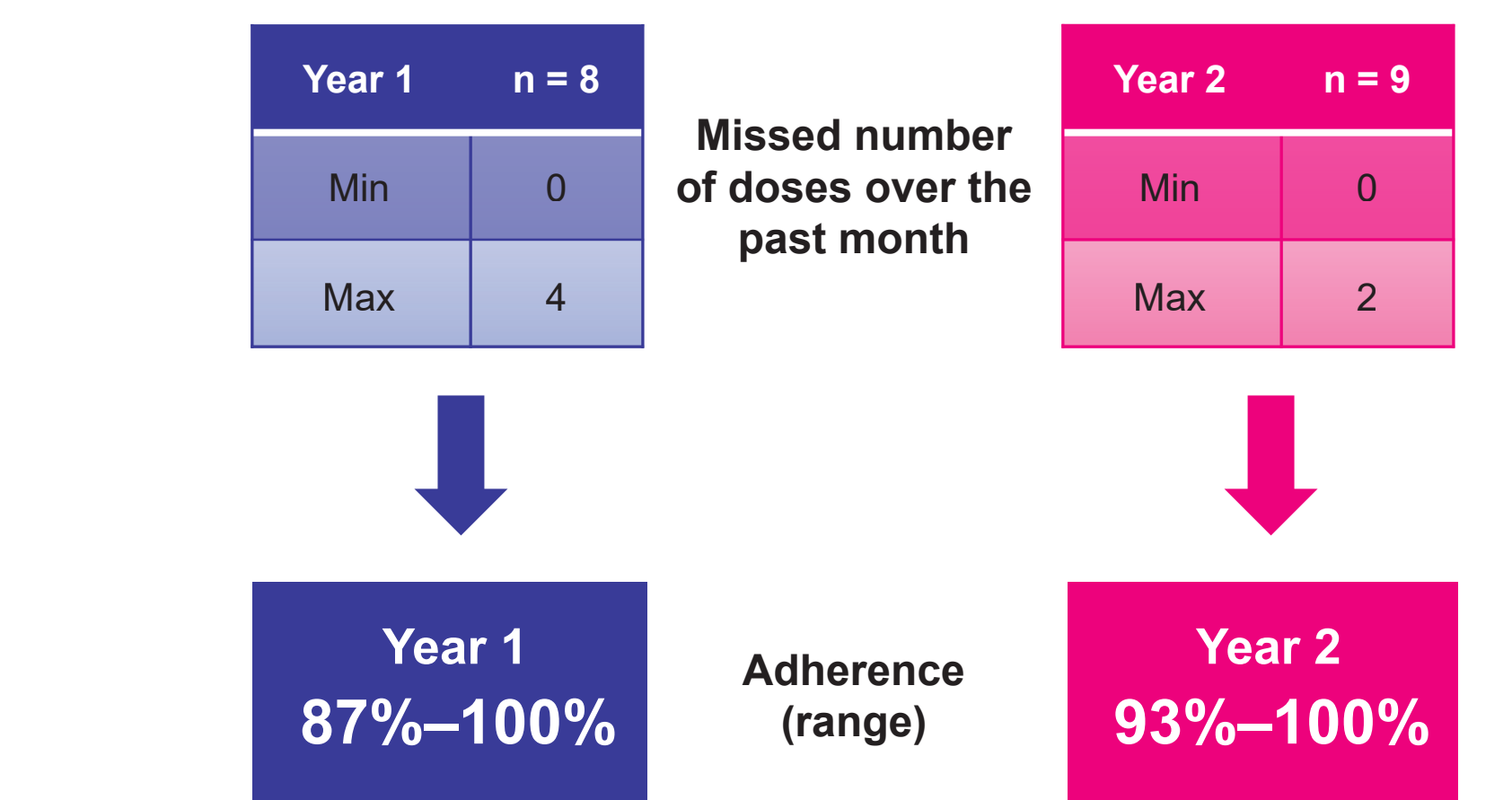


Horizontal lines represent medians. Boxes and whiskers represent IQR and min/max, respectively. IQR, interquartile range; PedsQL, Pediatric Quality of Life Inventory.

### 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<sup>5-11,13</sup>
  - 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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## Acknowledgments

We thank all study participants, their families, study-site staff, and investigators. Funding for this study was provided by BioMarin Pharmaceutical Inc. Medical writing support was provided by Rachel Corrigan, PhD, of Red Nucleus, and funded by BioMarin Pharmaceutical Inc. Project management support was provided by Justin Potuzak, PhD, of BioMarin Pharmaceutical Inc.

## Disclosures

NM is on advisory boards for Alexion, Ascendis, BioMarin Pharmaceutical Inc., BridgeBio, Catalist, Kyowa Kirin, Novo Nordisk, Pfizer, and UltraGenyx. JC, SA, and SK are employees of PicnicHealth. JMP and VH are employees and shareholders of BioMarin UK Ltd. SR, MC, DC, and AD are employees and shareholders of BioMarin Pharmaceutical Inc. JML has received grants or contracts from BioMarin Pharmaceutical Inc., QED Therapeutics/Bridge Bio, and Tyra Biosciences and payment or honoraria for lectures/presentations from Ascendis Pharma and BioMarin Pharmaceutical Inc.

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