

Real-World Effectiveness, Safety, and Caregiver-Reported Outcomes of Vosoritide in Latin American Children with Achondroplasia

The EVOLAC Multinational, Multicenter Real-World Study

Julieta De Víctor¹ · Eduardo D. Gil⁴ · Florencia Pablich³ · Rocío Rabosto Moleón⁶ · Carolina A. Dellamea⁷ · Mónica Fernández² · Norma C. Serrano⁸ · Sílvia Maradei² · Gisel Gordillo-González⁹ · Pablo Rosselli² · Norma Elena de León Ojeda¹⁰ · Beatriz E. De la Fuente-Cortez¹¹ · Nancy Unanue^{13,14,15} · María Dora Lacarrubba-Flores¹² · Rosario Gueçaimburu⁵

1 Hospital Interzonal General de Agudos Eva Perón, San Martín, Buenos Aires, Argentina · 2 Fundación Cardioinfantil, Bogotá, Colombia · 3 Hospital Privado Universitario de Córdoba, Córdoba, Argentina · 4 Hospital Interzonal Especializado Materno Infantil "Victorio Tetamanti", Mar del Plata, Argentina · 5 National Reference Center for Congenital Defects and Rare Diseases, Montevideo, Uruguay · 6 Santa Fe, Argentina · 7 Hospital Pediátrico "Avelino Castellán", Resistencia, Chaco, Argentina · 8 Fundación Cardiovascular de Colombia – Hospital Internacional de Colombia, Bucaramanga, Colombia · 9 FUSA IPS, Barranquilla, Colombia · 10 CRIT Occidente, Fundación Teletón, Guadalajara, Jalisco, Mexico · 11 Hospital Universitario "Dr. José Eleuterio González", UANL, Monterrey, Mexico · 12 Medical Genetics and Neuropediatrics Unit, Hospital Pediátrico Niños de Acosta Ñu, San Lorenzo, Paraguay · 13 IDIMI – Universidad de Chile · 14 Hospital Clínico San Borja Arriarán · 15 Clínica Las Condes, Santiago, Chile

01 BACKGROUND

Achondroplasia, the most common skeletal dysplasia, is caused by a gain-of-function pathogenic variant in FGFR3 that impairs endochondral ossification and produces disproportionate short stature.^{1,2}

Although vosoritide has demonstrated efficacy and safety in clinical trials, Latin American patients represented <10% of participants in the pivotal studies.^{3,4} Real-world evidence from the region is essential to evaluate treatment outcomes in routine clinical practice across diverse healthcare settings. EVOLAC was designed to generate this regional evidence.

02 METHODS

DESIGN
Multicenter retrospective cohort study across Argentina, Colombia, Uruguay, Mexico and Chile.

ELIGIBILITY
Molecularly confirmed achondroplasia (FGFR3 c.1138G>A (p.G380R)); open growth plates; ≥6 months of vosoritide; ≥2 anthropometric assessments. Follow-up to 36 months.

ANALYSIS
Paired comparisons versus baseline and Linear Mixed Models, adjusted for age and sex.

03 BASELINE & EXPOSURE

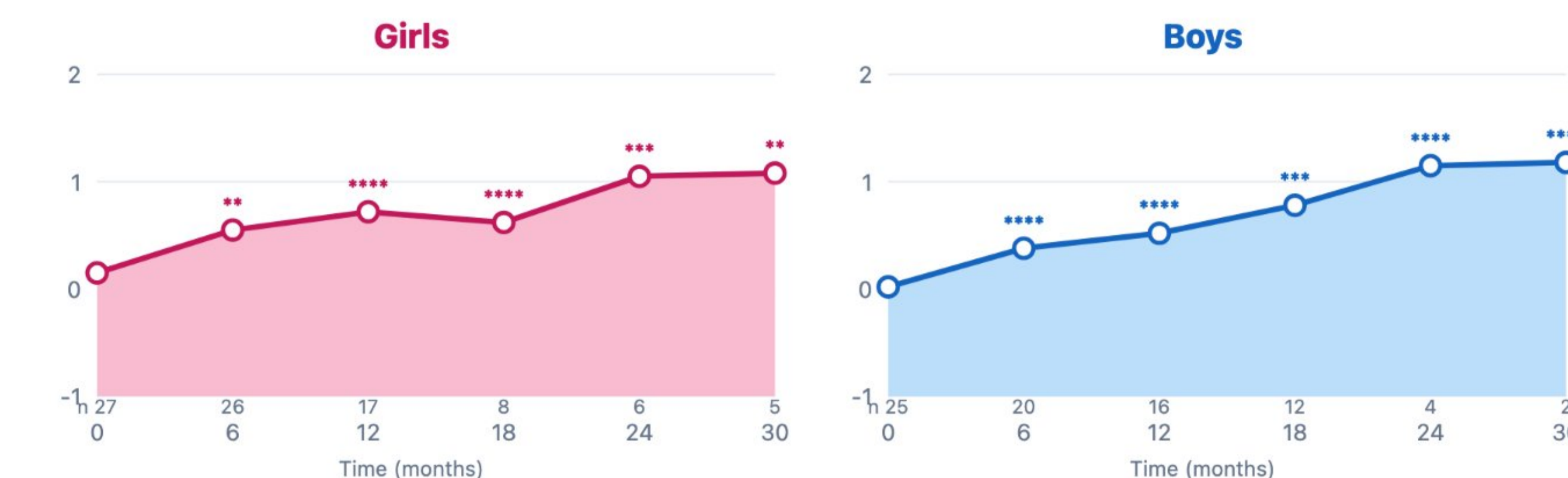
BASELINE CHARACTERISTICS (N = 52)	
Mean age at initiation, years (SD)	5.9 (2.6)
Female, n (%)	27 (51.9%)
Male, n (%)	25 (48.1%)
Age at treatment start, n (%)	
< 2 years	10 (19.2%)
2 – 5 years	10 (19.2%)
5 – 11 years	27 (51.9%)
> 11 years	5 (9.6%)
Mean treatment duration, days (SD)	539 (308)
Time without interruption, % (SD)	93.0 (10.1)



52 children (51.9% female), mean age 5.9 yrs at initiation; treatment averaged 539 days with 93.0% continuity.

52 patients enrolled across 5 Latin American countries; Argentina contributed the majority (37, 71%).

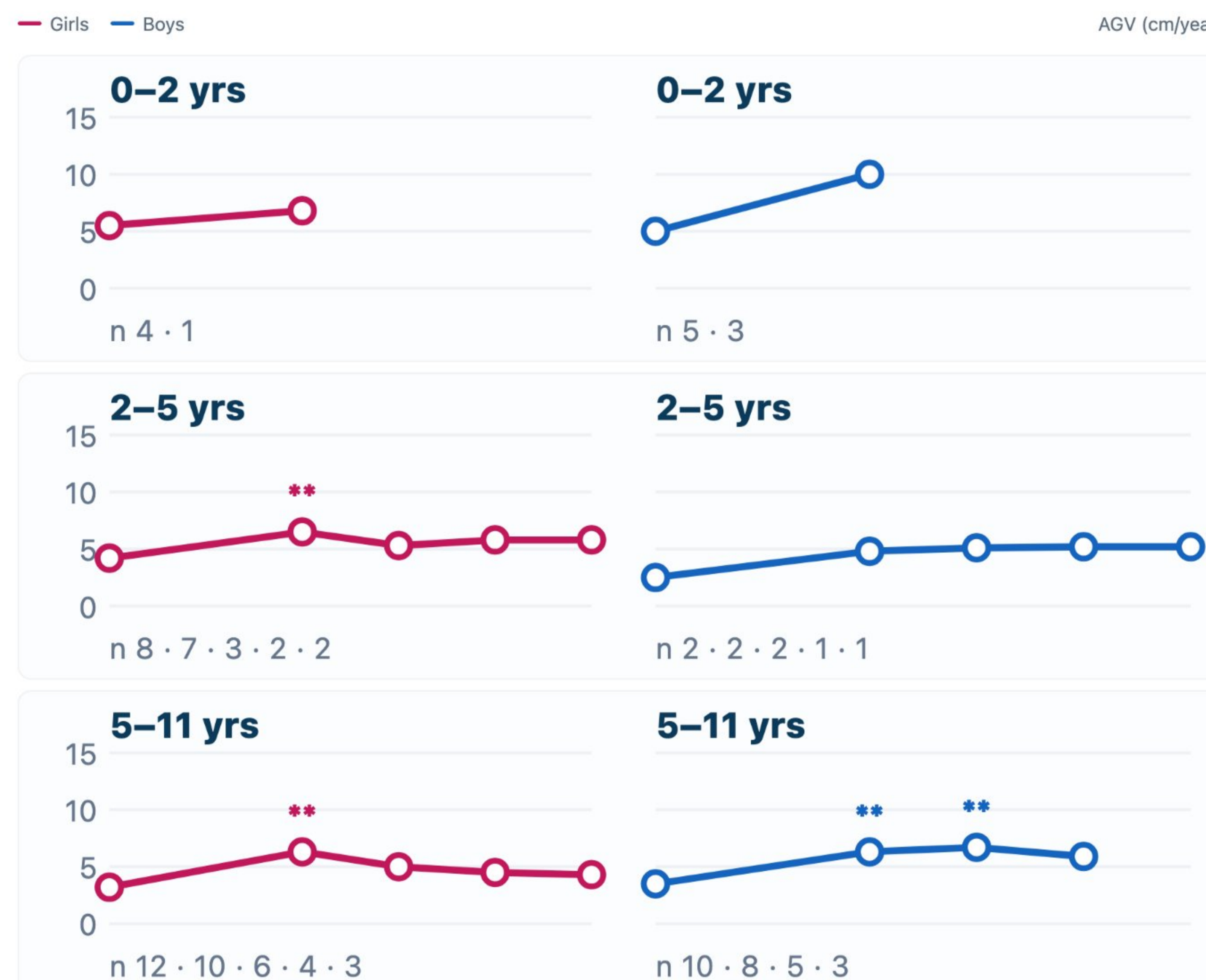
FIGURE 1 Height Z-score over time



Height Z-score rose significantly in both sexes, reaching about +1.0 by 24–30 months.

Paired t-test vs baseline · * p<0.05 · ** p<0.01 · *** p<0.001 · **** p<0.0001

FIGURE 2 AGV by sex & age group



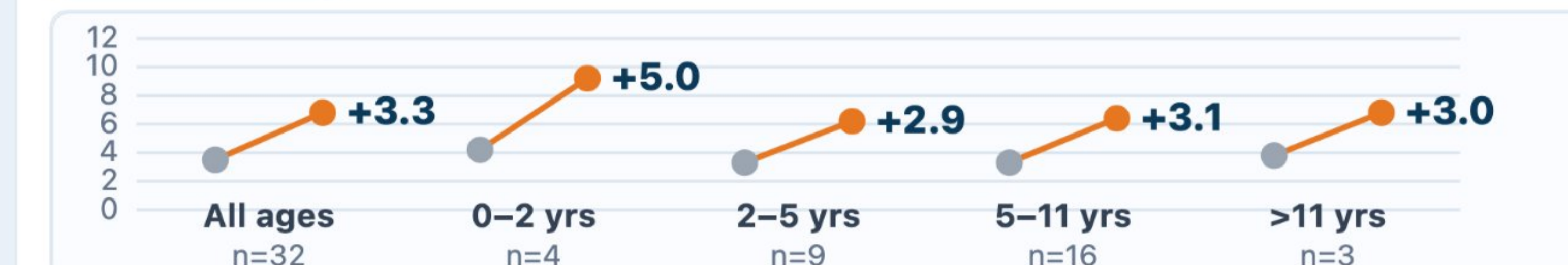
AGV rose over baseline at 12 months in all groups; gains largely sustained at 24 months where data available.

FIGURE 3 Observed vs expected AGV

cm/year vs reference (Savarirayan 2022)

Expected (grey dot) Observed (orange dot)

a · 12-month follow-up



b · 24-month follow-up



Observed AGV exceeded reference-expected values in every age group (+1.4 to +5.0 cm/year).

CAREGIVER OUTCOMES

N = 52

% OF PATIENTS

100%
Would recommend treatment

98.1%
Overall positive perception

- Improved mobility: 71.2%
- Greater independence: 65.4%
- Greater energy / vitality: 61.5%

Non-validated caregiver questionnaire.

Nearly all caregivers reported benefit: 100% would recommend and 98.1% positive perception.

SAFETY

59 events

PATIENTS WITH ADVERSE EVENTS

- Any adverse event: 43 (82.7%)
- Injection site reactions: 39 (75.0%)
- Hypertrichosis: 9 (17.3%)
- Hypotension: 6 (11.5%)

BY SEVERITY · 59 EVENTS TOTAL

- 98.3% Mild (58 events)
- 1.7% Moderate (1 event)
- 0% Severe (0 events)

Adverse events were common but 98.3% were mild, with no severe events.

CONCLUSIONS

In real-world Latin American practice, vosoritide produced significant, sustained gains in height Z-score and growth velocity above expected trajectories, with high treatment continuity (93.0%) and a favorable safety profile (98.3% mild events, none severe).

KEY FINDINGS

52 children treated across 5 countries

+3.3 cm/year above expected growth velocity at 12 months

93.0% treatment continuity



View poster online

Made with Quantus

REFERENCES

- Chamathi VS, et al. Achondroplasia. StatPearls. 2026.
- Pauli RM. Orphanet J Rare Dis. 2019;14(1):1.
- Savarirayan R, et al. Lancet Child Adolesc Health. 2024;8(1):40–50.
- Savarirayan R, et al. Lancet. 2020;396(10252):684–692.
- Savarirayan R, et al. Genet Med. 2022;24(12):2444–52.

ACKNOWLEDGEMENTS

The authors thank Pieralessandro Lasalvia and Jimena García (Quantus) for their support; analyses were performed using Quantus.

DISCLOSURES

The authors declare that they did not receive fees or financial compensation for conducting this study. Some authors have previously participated as speakers or academic advisors in educational activities sponsored by BioMarin, with no current contractual relationship and no influence on the study design, data analysis, or manuscript preparation.

FUNDING

The study received partial financial support from BioMarin, limited to medical editing and publication costs. The company had no role in study design, data collection, analysis, interpretation, or writing. Authors retained full scientific and editorial independence.