

SUPPLEMENTARY MATERIALS

Real-World Safety and Effectiveness of Vosoritide in Achondroplasia: Results From a Single Center in Portugal

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SUPPLEMENTARY TABLES

Table S1 Detailed characteristics of three patients who discontinued vosoritide due to a lack of significant response

	Case A (F)	Case B (F)	Case C (M)
Treatment duration, months	18	24	24
Age, years			
At treatment initiation	13.5	12.5	14.2
At final treatment	15.1	14.5	16.2
Height, cm			
At treatment initiation	113.2	118.0	129.7
At final treatment	118.2	124.2	137.7
Height SDS (compared with WHO standard curves) [1, 2]			
At treatment initiation	-6.46	-5.20	-5.51
At final treatment	-6.35	-5.31	-4.63
Change from baseline	+0.11	-0.11	+0.88
AGV, cm/year			
At treatment initiation	2.86	6.0	5.46
At final treatment	2.50	3.17	2.33
Tanner stage			
At treatment initiation	4	3	3
At final treatment	5	4	5

AGV annualized growth velocity; *F* female; *M* male; *SDS* standard deviation score; *WHO* World Health Organization

SUPPLEMENTARY FIGURES

Fig. S1 Absolute mean height SDS in children ($n=27$) treated with vosoritide for up to 24 months, referenced to the untreated ACH-specific growth curve. Box plots display the lower (Q1) and upper (Q3) quartiles (box edges), the median (midline), the mean (cross symbol), and min/max (whiskers). $**P\leq 0.01$ compared with the ACH reference growth curve [3]. ACH achondroplasia; SDS standard deviation score; Q quartile

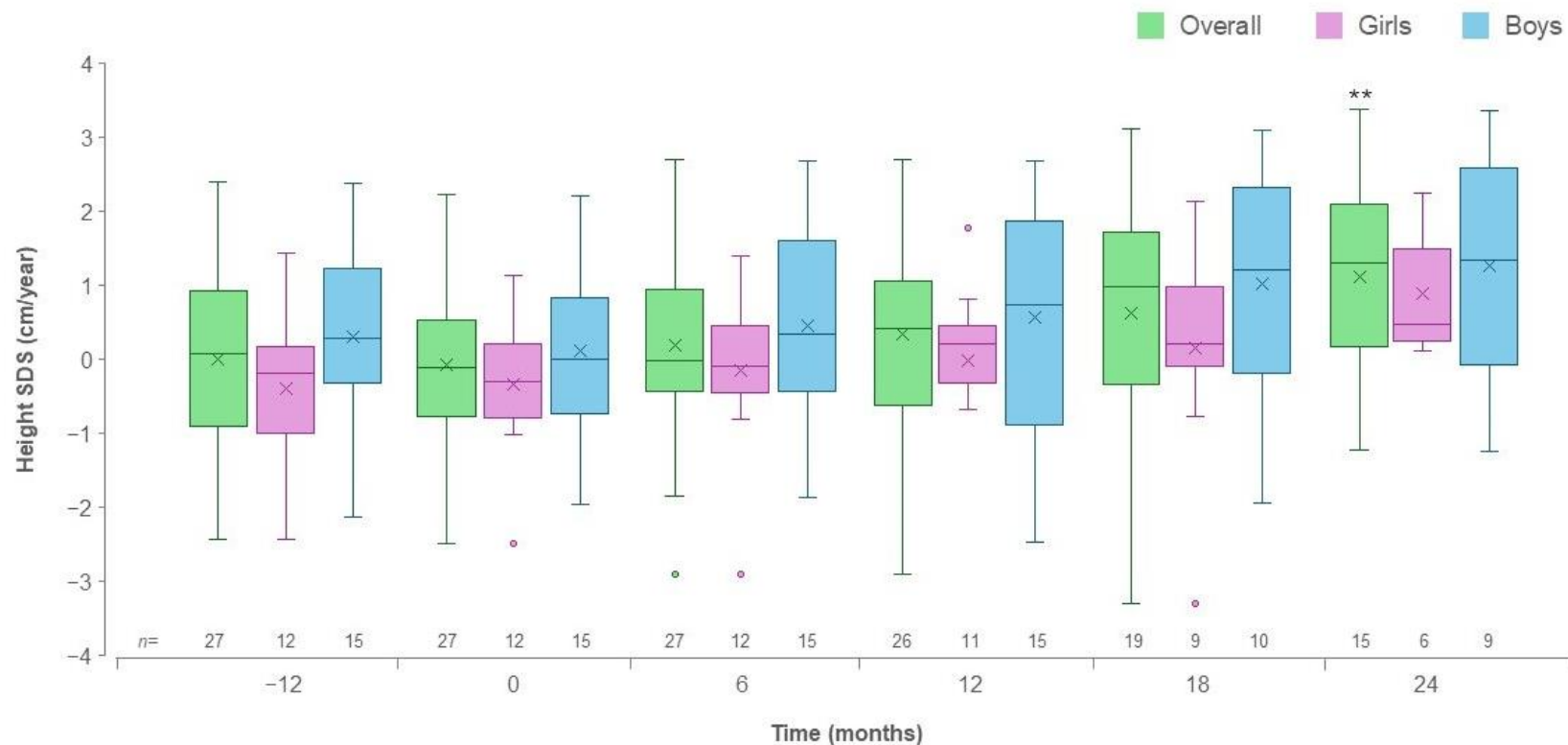


Fig. S2 Absolute mean height SDS in children ($n=27$) treated with vosoritide for up to 24 months, referenced to WHO standard growth curves. Box plots display the lower (Q1) and upper (Q3) quartiles (box edges), the median (midline), the mean (cross symbol), and min/max (whiskers). **** $P \leq 0.0001$ compared with the WHO reference growth curves [1, 2]. SDS standard deviation score; WHO World Health Organization

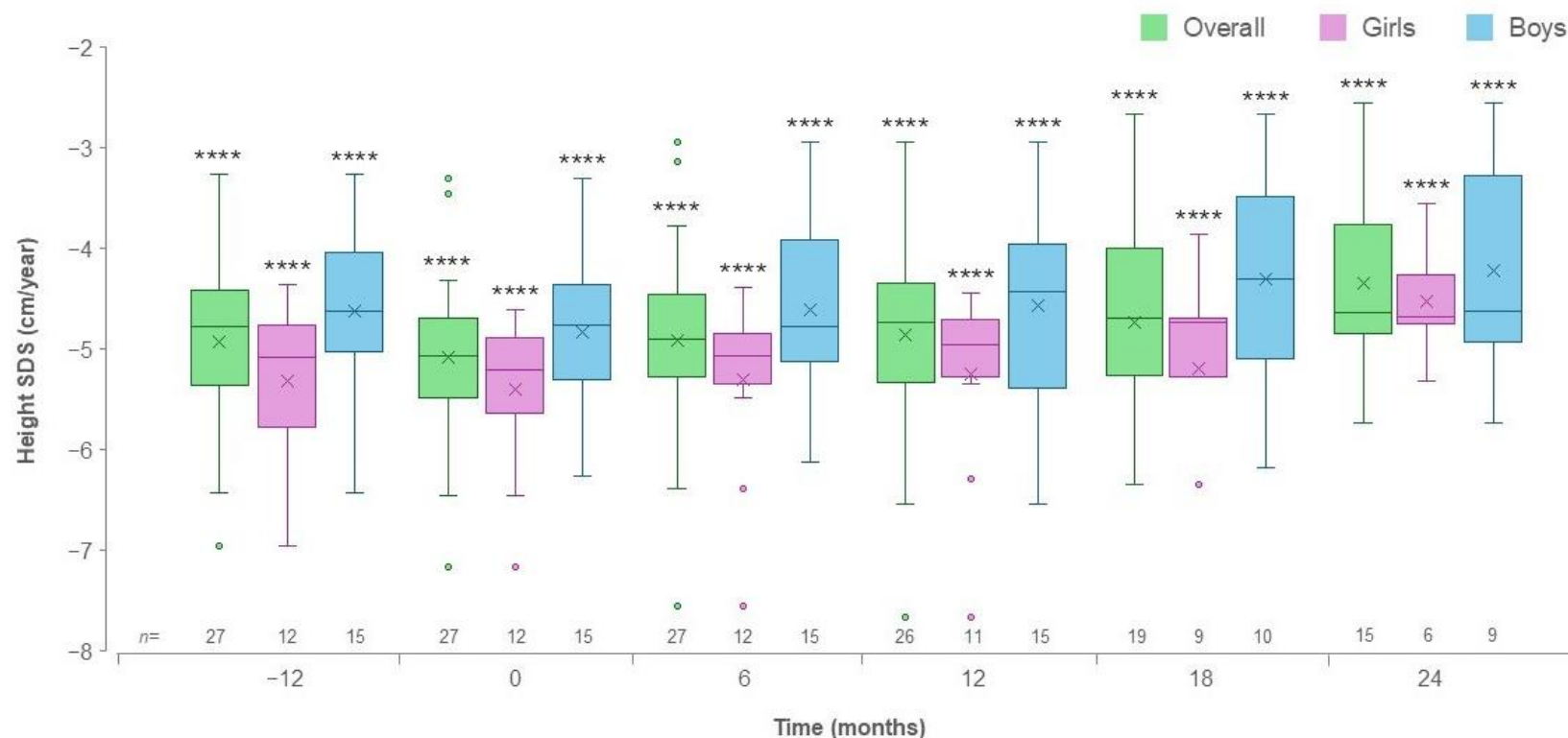


Fig. S3 Absolute mean AGV in children ($n=27$) treated with vosoritide for up to 24 months. Box plots display the lower (Q1) and upper (Q3) quartiles (box edges), the median (midline), the mean (cross symbol), and min/max (whiskers); the ranges on the x-axis represent the time points between which AGV was calculated. *** $P\leq 0.001$ and **** $P\leq 0.0001$, compared with baseline AGV. AGV annualized growth velocity; Q quartile

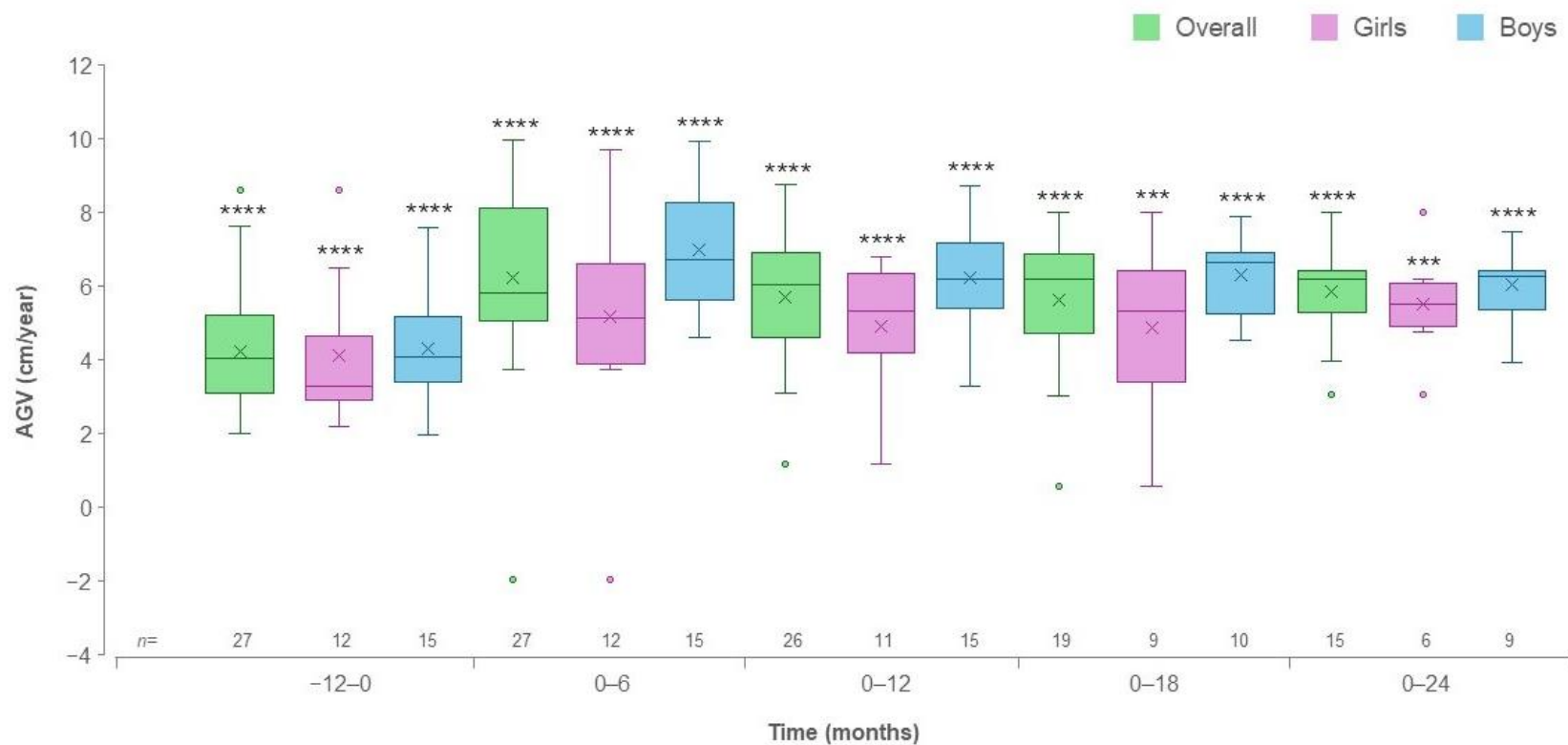


Fig. S4 Absolute mean arm span SDS in children ($n=27$) treated with vosoritide for up to 24 months. Box plots display the lower (Q1) and upper (Q3) quartiles (box edges), the median (midline), the mean (cross symbol), and min/max (whiskers). SDS standard deviation score; Q quartile

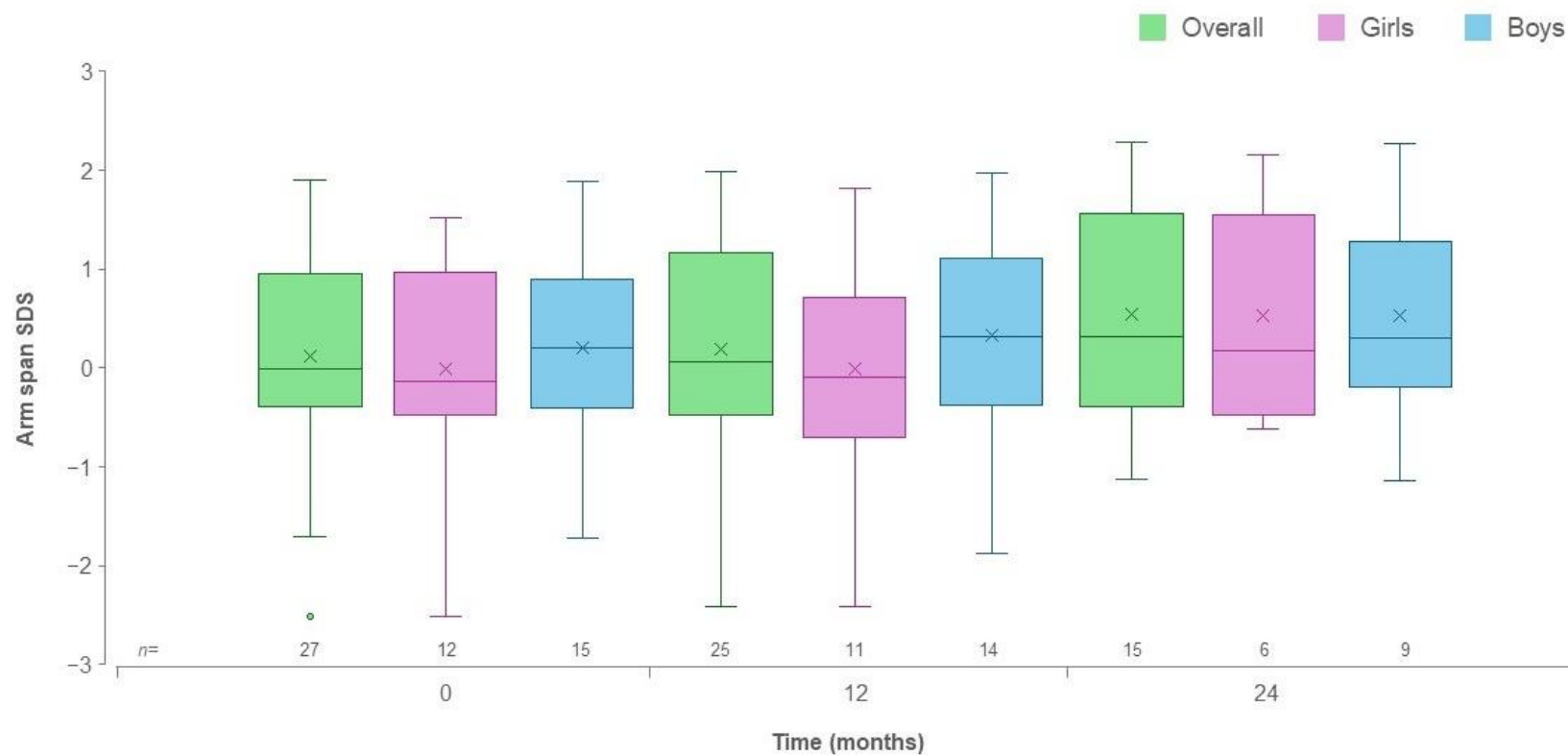


Fig. S5 Absolute mean sitting height SDS in children ($n=27$) treated with vosoritide for up to 24 months. Error bars show 95% confidence intervals. * $P\leq 0.05$ compared with the achondroplasia reference growth curve [4]. SDS standard deviation score

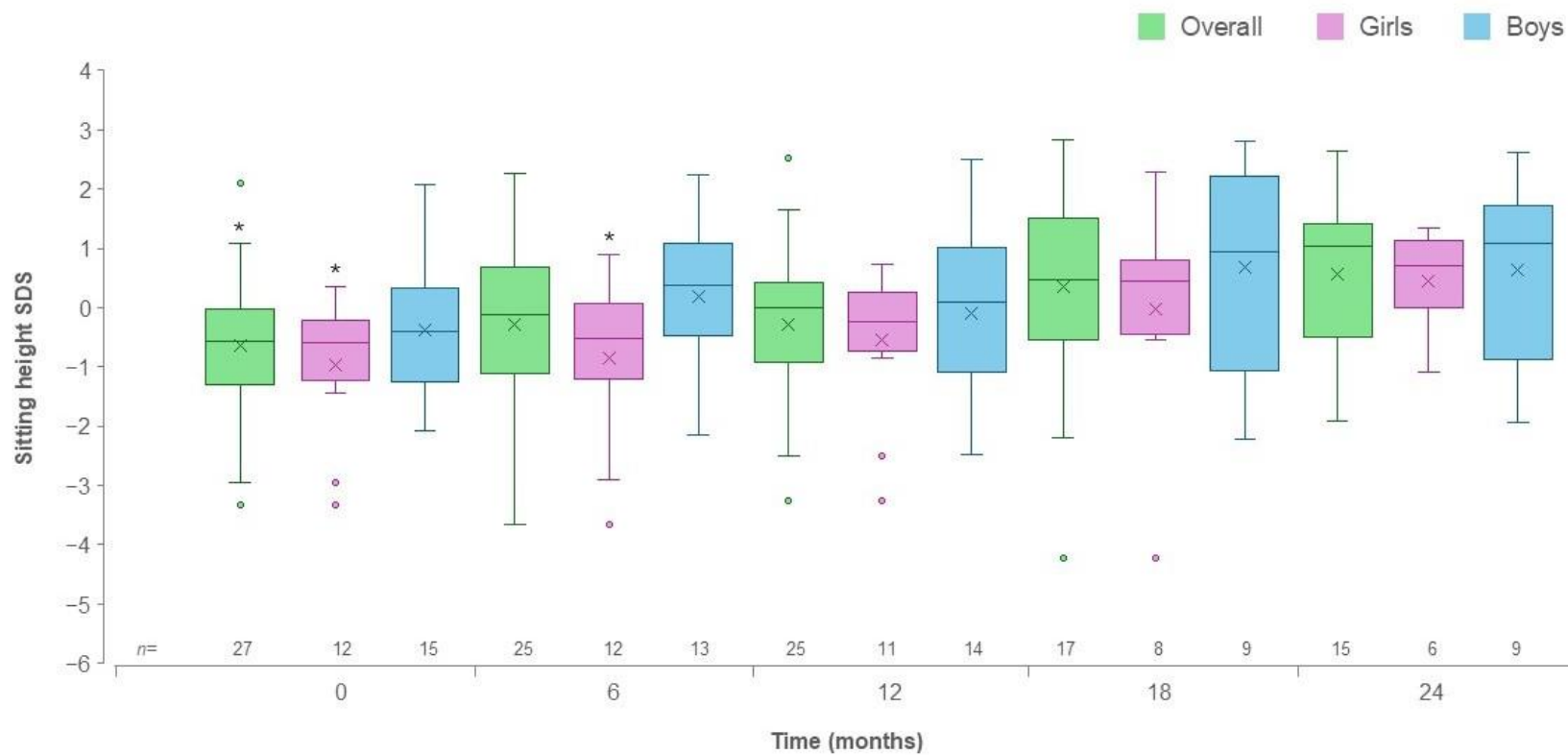


Fig. S6 Upper to lower segment ratio in individual patients following treatment with vosoritide for up to 24 months

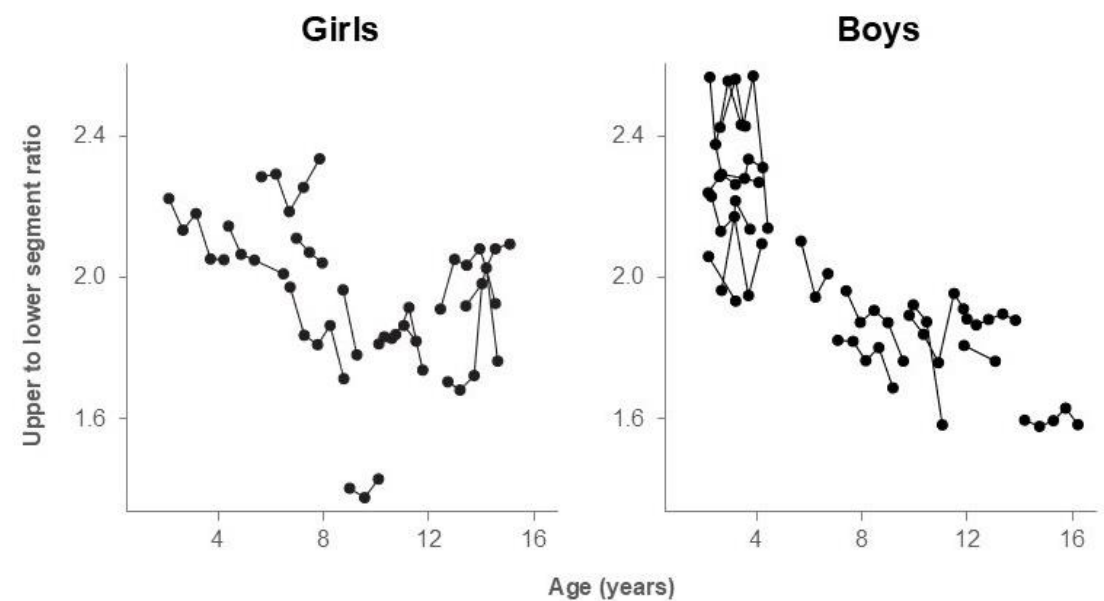


Fig. S7 Hypertrichosis at the dorso-lumbar region and buttocks of a 4-year-old male, coincident with a weight-related dose increase in vosoritide, 18 months after treatment initiation.



SUPPLEMENTARY REFERENCES

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