

Radiographic Data from Week 104 of the Pivotal ApproaCH Trial of TransCon CNP in Children with Achondroplasia

July 2026

Cautionary Note on Forward-Looking Statements

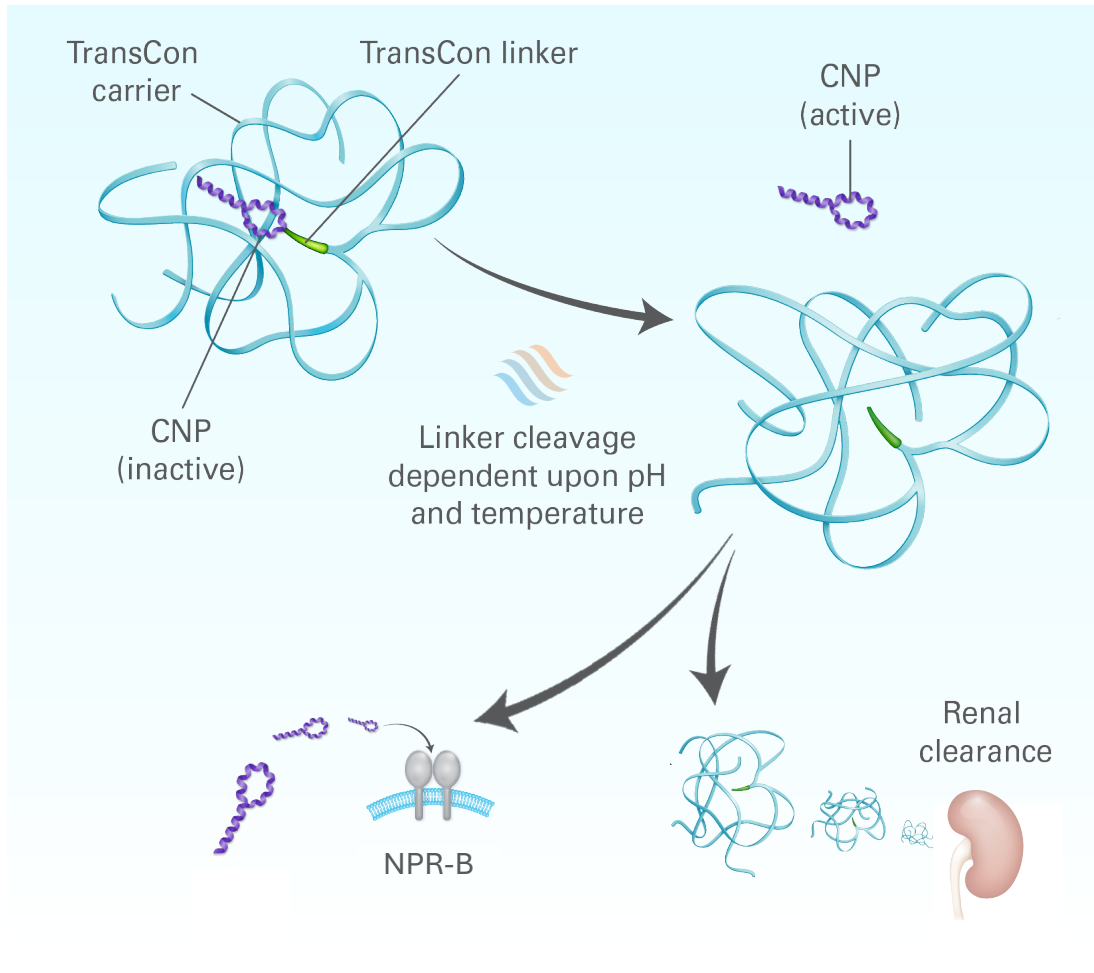
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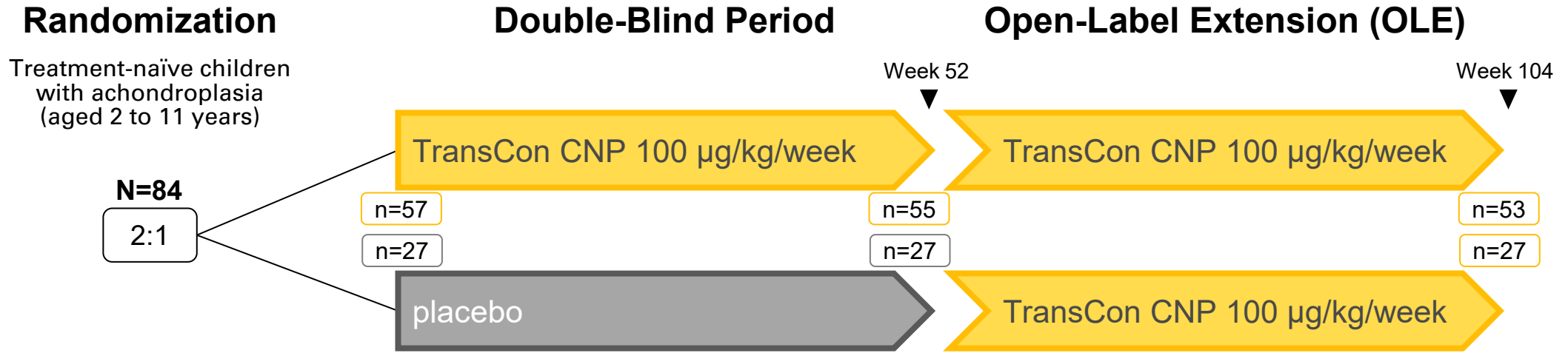
TransCon[®] CNP (Navepegritide)



- TransCon CNP is a prodrug of C-type natriuretic peptide (CNP) administered once weekly and designed to provide continuous exposure to active CNP
- Active CNP released from TransCon CNP has an amino acid sequence identical to endogenous CNP [89-126] and binds to NPR-B throughout the body to counteract the constitutively active FGFR3 signaling in achondroplasia¹
- TransCon CNP is approved by the FDA under the trade name YUVIWEL[®] to increase linear growth in children 2 years and older with achondroplasia with open epiphyses²

CNP, C-type natriuretic peptide; FDA, Food and Drug Administration; FGFR3, fibroblast growth factor receptor 3; NPR-B; natriuretic peptide receptor B
¹Breinholt VM, et al. *J Pharmacol Exp Ther* 2019. ²YUVIWEL (navepegritide). Prescribing information. 2026. Accessed 10 March 2026. <https://www.accessdata.fda.gov>

ApproaCH Trial Design, Disposition, and Demographics¹



Key Assessments

Growth

- AGV through Week 104
- Change from baseline in ACH-specific height Z-score

Safety

- Treatment emergent adverse events (AEs)
- Tolerability

Exploratory

- Change from baseline in key orthopedic features of skeletal dysplasia
 - Radiographs collected at baseline, Week 52, and Week 104 were assessed by blinded, expert central readers

Baseline Characteristics

Age, years;
mean (SD) [min, max]

Sex, male;
n (%)

ACH-specific height Z-score;
mean (SD)

	TransCon CNP N=57	placebo N=27
Age, years; mean (SD) [min, max]	5.6 (2.6) [2.0, 11.9]	6.0 (2.7) [2.1, 12.0]
Sex, male; n (%)	31 (54.4)	14 (51.9)
ACH-specific height Z-score; mean (SD)	0.18 (0.92)	-0.11 (0.73)

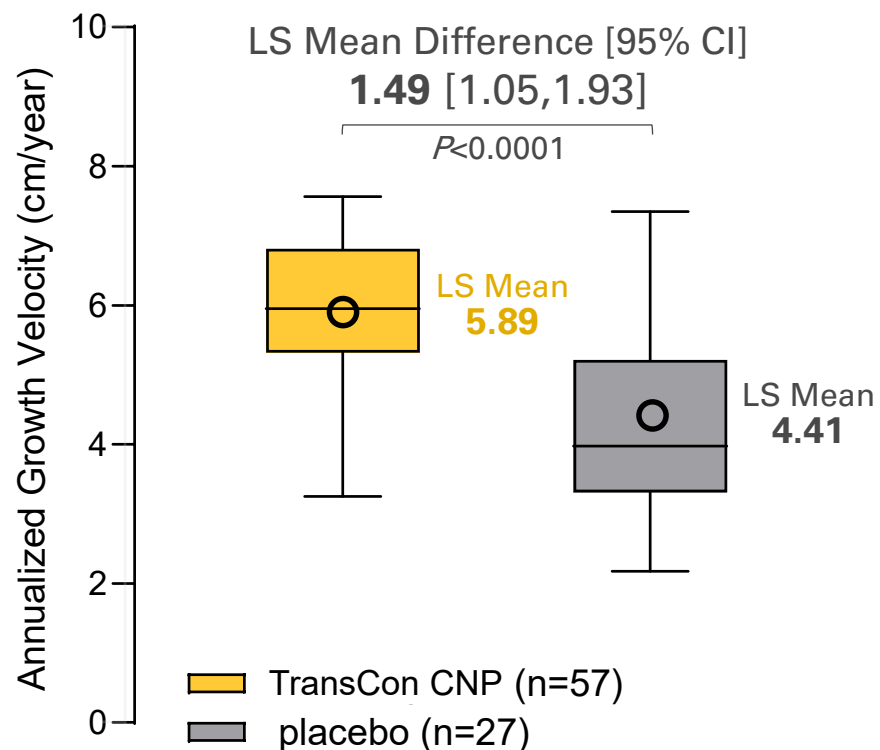
N = number of children that received at least one dose of investigational medicinal product.

ACH, achondroplasia; AGV, annualized growth velocity

¹For an overview of the trial design see Savarirayan R, et al. *JAMA Pediatr.* 2026;180(1):18–25.

ApproaCH Primary Endpoint: Annualized Growth Velocity (AGV) at Week 52

Savarirayan R, et al. Once-Weekly Navepegritide In Children With Achondroplasia: The APPROACH Randomized Trial. *JAMA Pediatrics* 2026;180;(1):18-25.

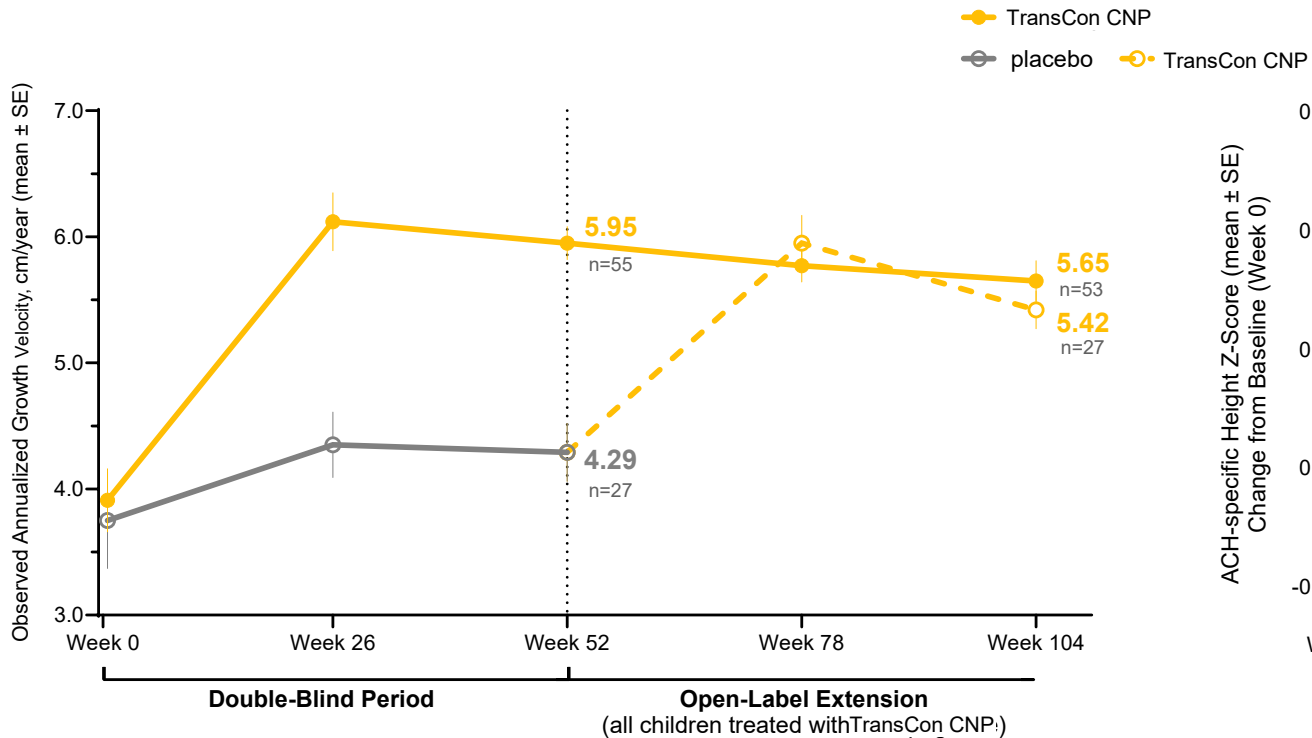


Results from the double-blind period demonstrated superiority of once-weekly TransCon CNP over placebo for AGV at Week 52

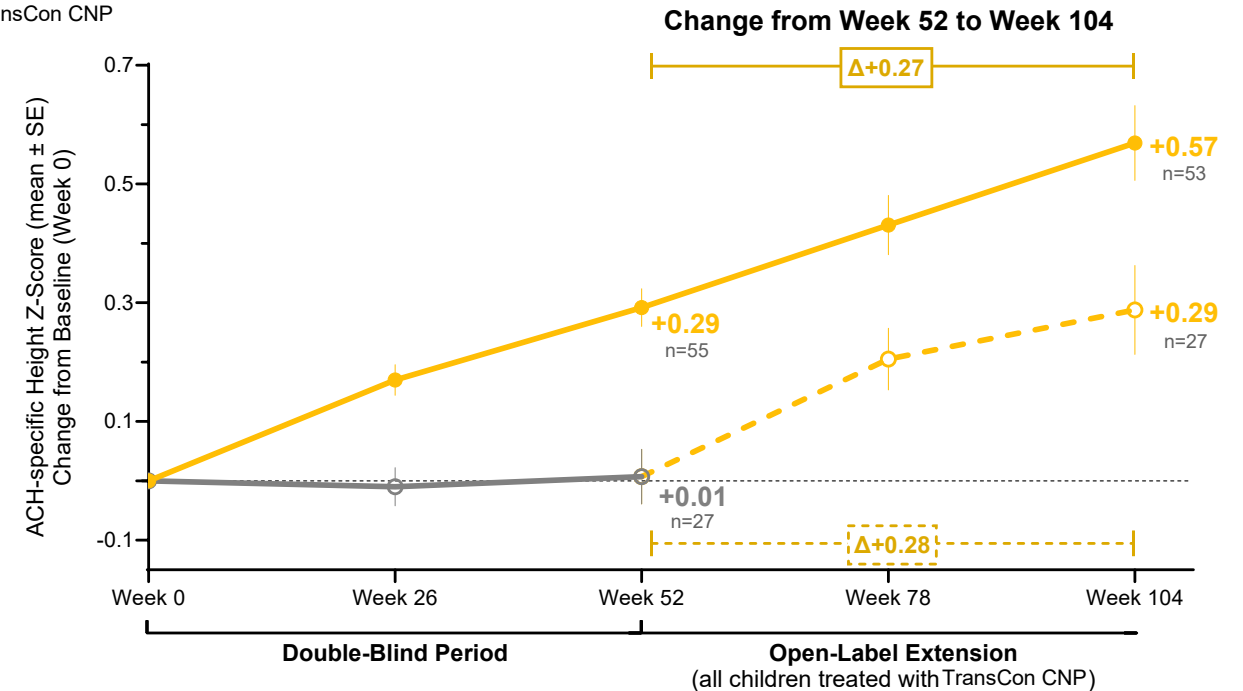
Note: Box-and-whisker plots derived using observed data at Week 52 and display the 75th and 25th percentile (box edges), IQR (colored area), median (midline), and minimum and maximum observed values; labeled circles represent least squares mean values. Missing height data at Week 52 for 2 participants in the TransCon CNP group were imputed using the multiple imputation method. AGV, annualized growth velocity; CI, confidence interval; IQR, interquartile range; LS, least squares

Improvements in AGV were maintained and ACH-specific height Z-score increased with TransCon CNP through Week 104

Annualized Growth Velocity (AGV)



Change in ACH-specific Height Z-score

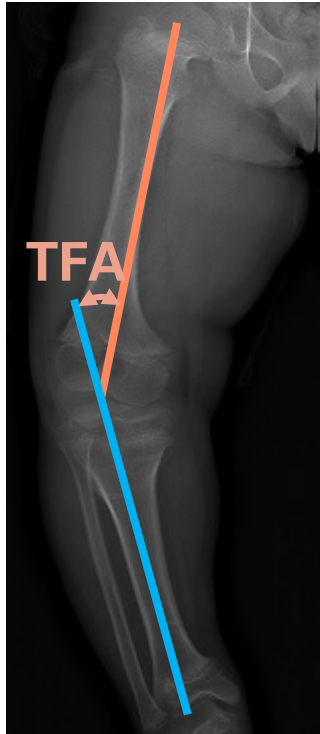


ACH, achondroplasia; AGV, annualized growth velocity; OLE, open-label extension

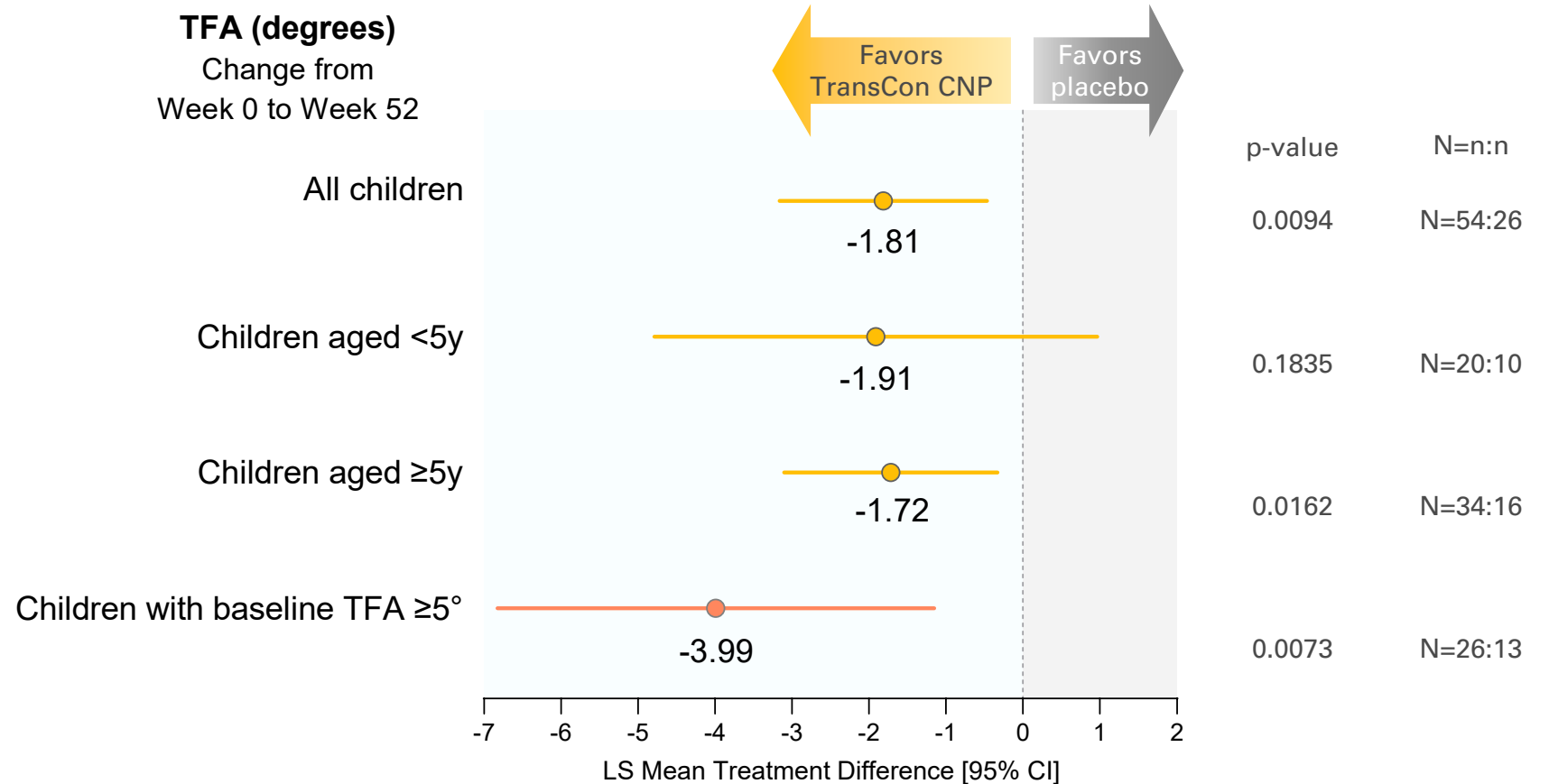
TransCon CNP improved lower extremity alignment compared with placebo at Week 52

TFA: Tibial-Femoral Angle

Angle between the mechanical axes of the femur and the tibia



TFA (degrees)
Change from Week 0 to Week 52



N=n:n refers to number of children for TransCon CNP:placebo; age subgroups determined by age at time of enrollment. Figure shows TFA with dot indicating LS mean difference between TransCon CNP and placebo; error bar indicates 95% CI. ANCOVA model included treatment, stratification factor, baseline age and baseline value as covariates. With the exception of the subgroup of children with baseline TFA ≥5°, change from baseline represents absolute change from Week 0.

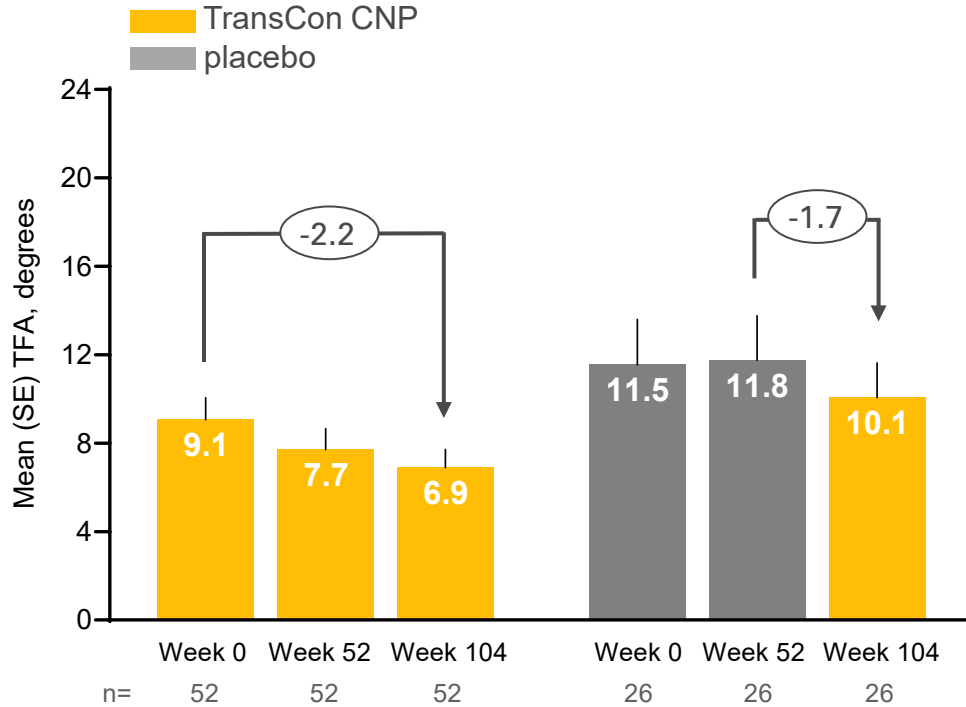
ANCOVA, Analysis of covariance; CI, confidence interval; LS, least squares; TFA, Tibial-femoral angle

TFA decreased with TransCon CNP treatment through Week 104

All children

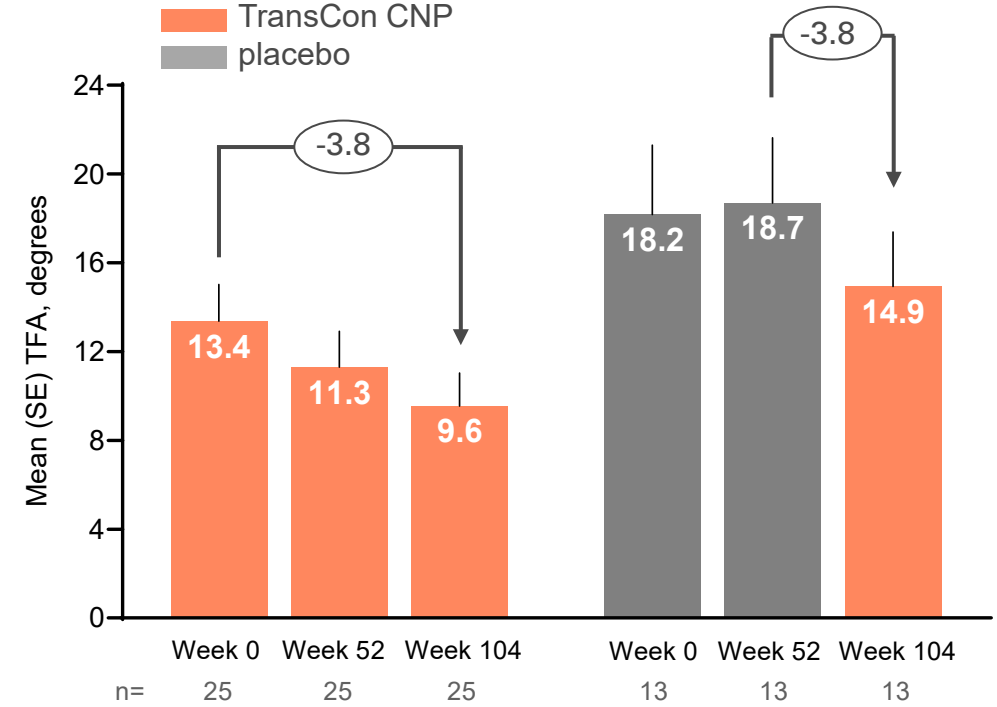


Improved alignment



Fibula-to-tibia length ratio remained stable, reflecting proportional growth of the lower leg

Children with baseline TFA $\geq 5^\circ$



Greater effects observed in children with preexisting genu varum

Data reported are paired analyses for children with data available at all timepoints; error bars indicate standard error. TFA is reported as the average of the absolute values (deviations from straight alignment) from each leg; change from baseline represents absolute change from Week 0. TFA, tibial-femoral angle

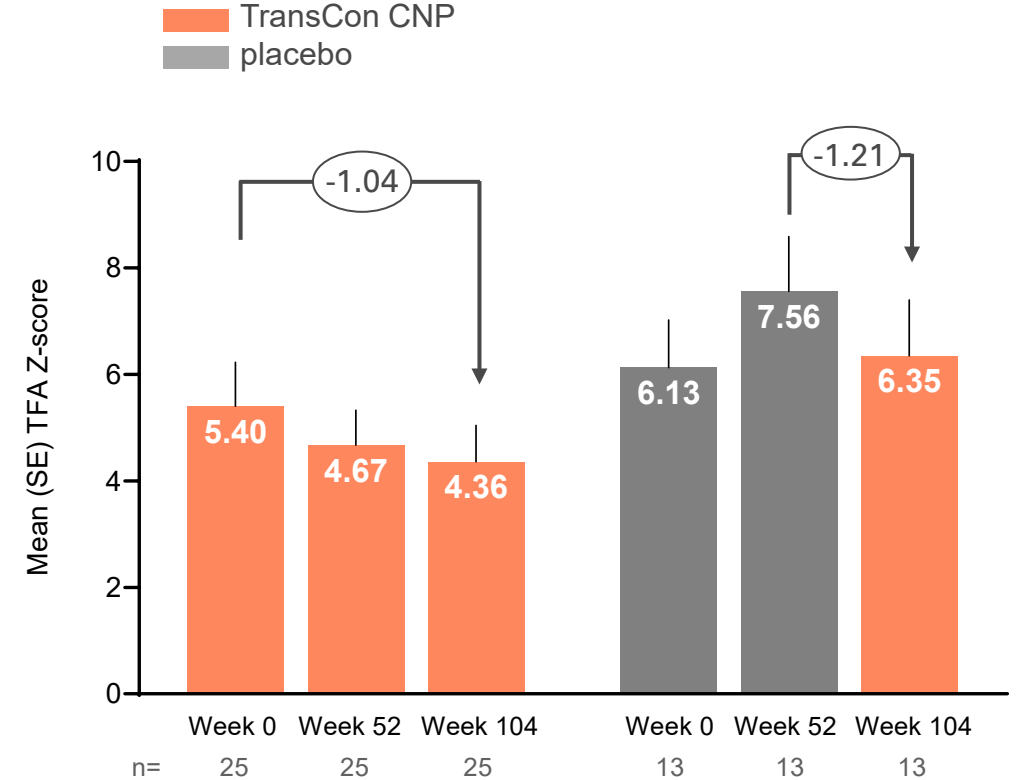
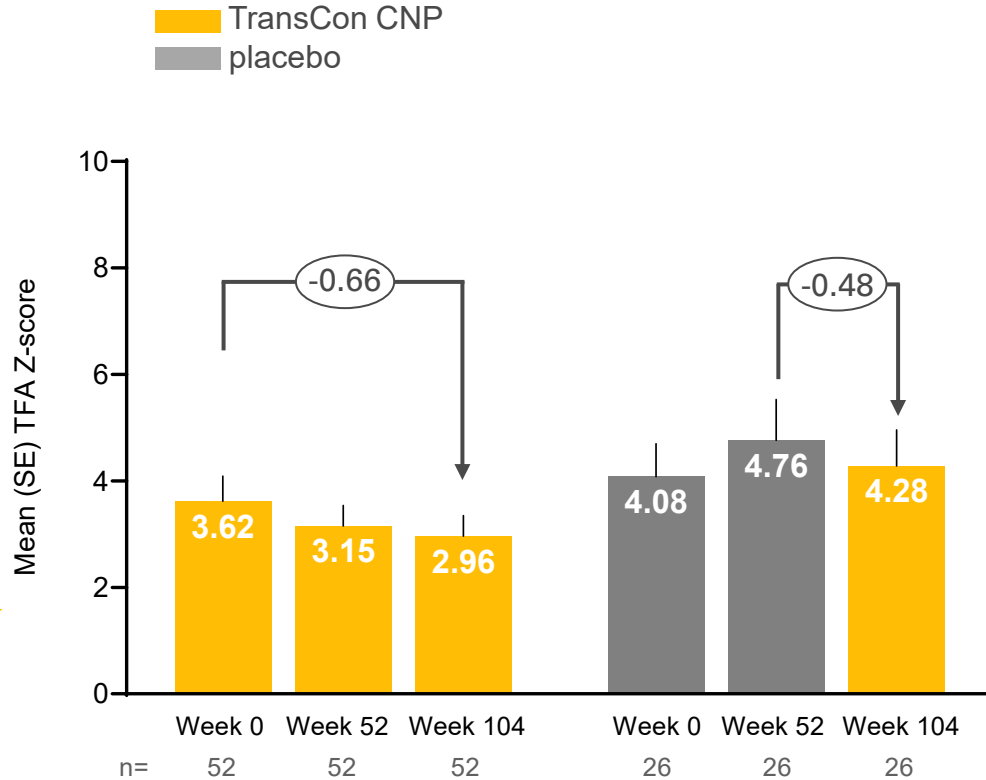
TFA Z-scores continued to decrease through Week 104 with TransCon CNP treatment

All children

Children with baseline TFA $\geq 5^\circ$



Improved alignment



Data reported are paired analyses for children with data available at all timepoints; error bars indicate standard error. TFA Z-scores were calculated as the average of the absolute values of the TFA Z-scores from both legs, using reference data from children of average stature (Sabharwal S and Zhao C, *J Bone Joint Surg Am* 2009); change from baseline represents absolute change from Week 0. TFA, tibial-femoral angle

Most AEs with TransCon CNP treatment were mild or moderate, with none leading to treatment discontinuation or trial withdrawal through Week 104

	TransCon CNP-treated children N=84
Treatment exposure	137.1 person-years
Any treatment-emergent AE, n (%)	76 (90.5)
Most frequent AEs (≥15% of children)	
Otitis media	31 (36.9)
Nasopharyngitis	29 (34.5)
Pyrexia	29 (34.5)
Upper respiratory tract infection	19 (22.6)
Headache	17 (20.2)
Vomiting	16 (19.0)
Influenza	13 (15.5)
Injection site reaction (ISR), n (%)	17 (20.2)
ISRs per person-year of exposure	0.35 [†]
Symptomatic hypotension, n (%)	0 (0.0)

- Bone age advanced consistently with chronological age
 - Mean bone age to chronological age ratio remained below 1 at Week 104
- In the OLE, two children experienced fractures associated with trauma
 - Neither fracture (1 clavicle, 1 forearm) included the growth plate
- No osteonecrosis or slipped capital femoral epiphysis (SCFE)

n = number of children with observation.

[†]Equivalent to approximately 1 ISR for every 3 years of treatment
AE, adverse event; ISR, injection site reaction; OLE, open-label extension

Conclusions

In the ApproaCH Trial

- Treatment with once-weekly TransCon CNP in children with achondroplasia was generally well tolerated and resulted in gains in AGV that were maintained through 104 weeks
- Once-weekly TransCon CNP continued to improve lower limb alignment in children with achondroplasia through Week 104
- Greater improvement in lower limb alignment was evident in children with preexisting genu varum (baseline tibial-femoral angle $\geq 5^\circ$) compared to the overall study population

TransCon CNP may address features of skeletal dysplasia that contribute to major health-related complications associated with achondroplasia

Thank you

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