ApproaCH Trial Open-Label Extension: Week 104 Results in Children with Achondroplasia Aged 5 Years or Older

Presented at PES on May 2, 2026

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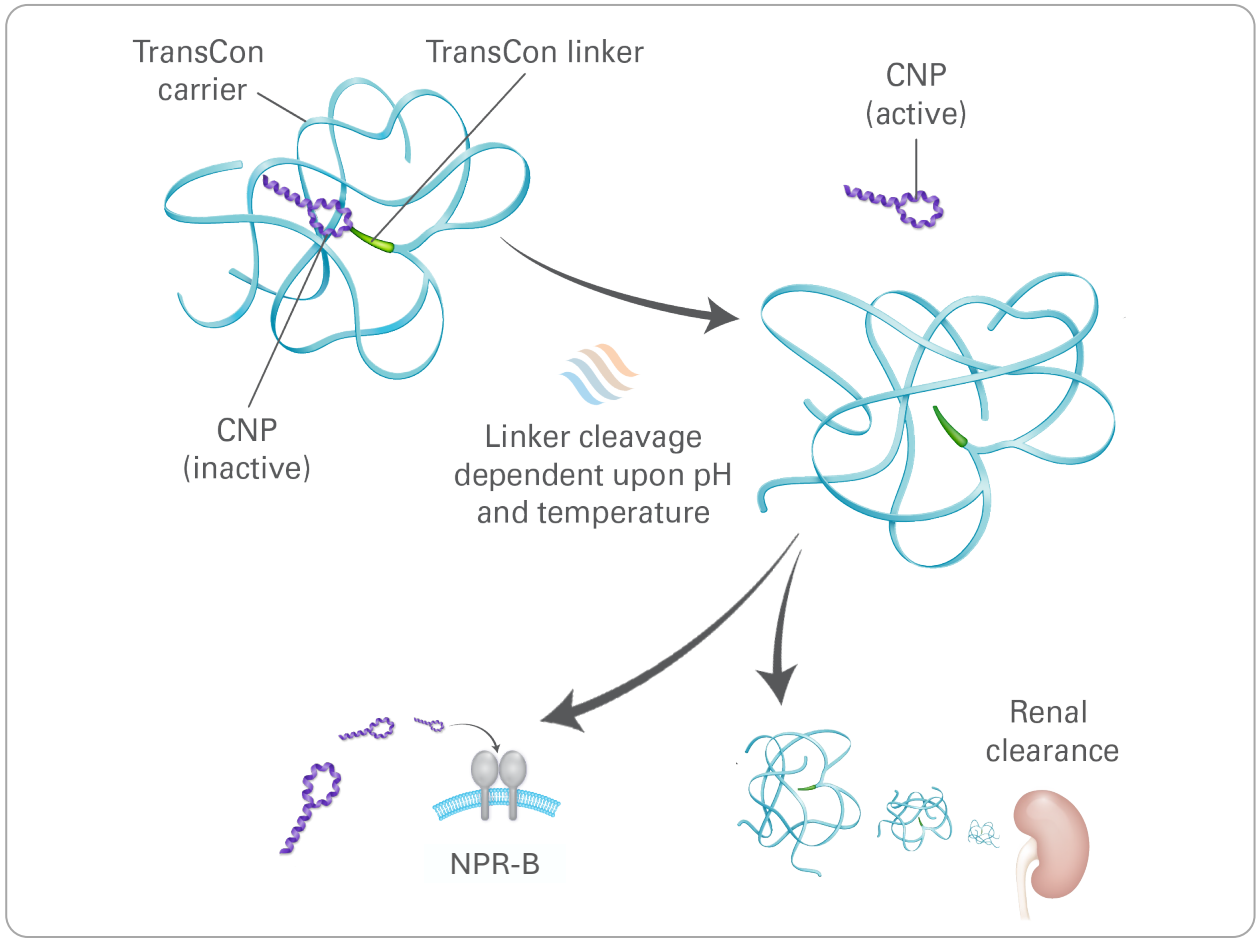
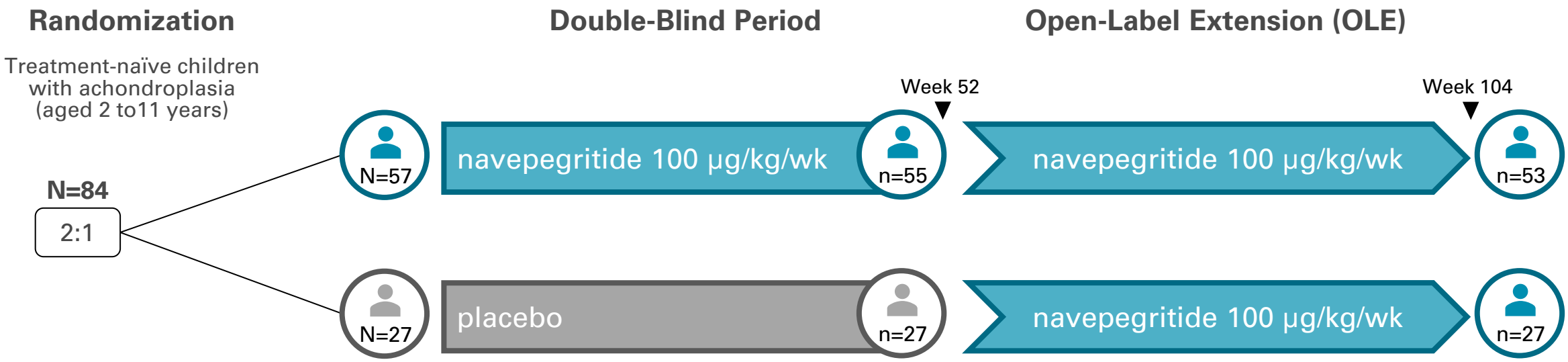
TransCon® CNP (navepegritide)



- Navepegritide 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 navepegritide 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 achondroplasia[1]
- Navepegritide is approved by the U.S. FDA to increase linear growth in children 2 years and older with achondroplasia with open epiphyses[2]

CNP = C-type natriuretic peptide; FDA = Food and Drug Administration; FGFR3 = fibroblast growth factor receptor 3; NPR-B = natriuretic peptide receptor B
[1] Breinholt VM, et al. *J Pharmacol Exp Ther* 2019;370(3):459-471. [2] YUVIWEL (navepegritide). Package Insert. Ascendis Pharma 2026.

Trial Design and Disposition



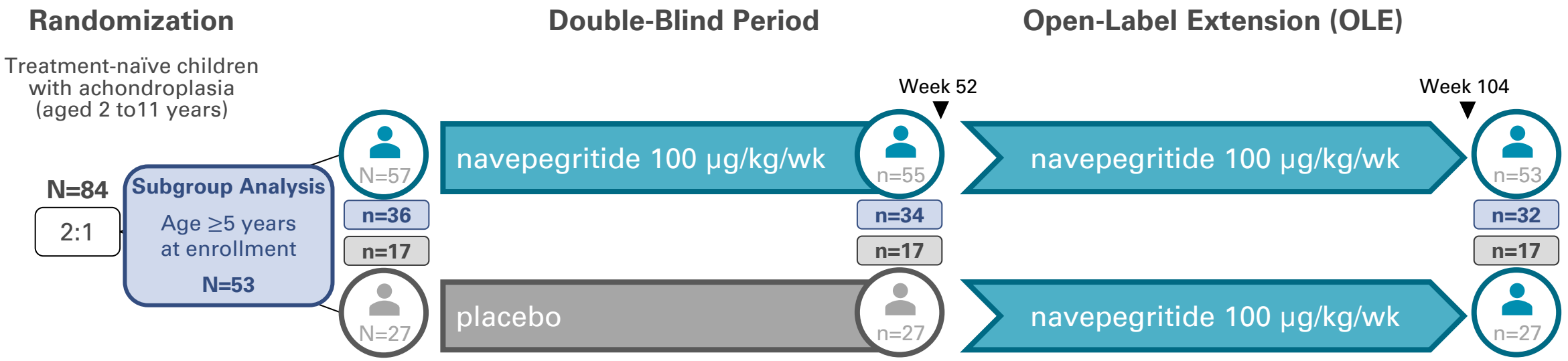
Primary Endpoint: Annualized growth velocity (**AGV**) at Week 52

Additional Efficacy Assessments: Change from baseline in **height Z-scores**
AGV at Week 104

Safety and Tolerability Assessments: Treatment-emergent adverse events (AEs)
Bone age

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

Subgroup Analysis: Children Aged ≥5 Years



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

Demographics at Baseline

	All children		*Subgroup analysis* Children aged ≥5 years at enrollment	
Treatment Group:	**navepegritide N=57**	**placebo N=27**	**navepegritide n=36**	**placebo n=17**
Age (years), mean (SD) [min, max]	5.6 (2.6) [2.0, 11.9]	6.0 (2.7) [2.1, 12.0]	7.0 (2.1) [5.0, 11.9]	7.6 (1.9) [5.0, 12.0]
Sex, Male, n (%)	31 (54.4)	14 (51.9)	19 (52.8)	9 (52.9)
Height (cm), mean (SD)	88.9 (12.9)	89.1 (11.5)	96.2 (9.3)	96.5 (6.7)
Achondroplasia-specific height Z-score, mean (SD)	0.18 (0.92)	-0.11 (0.73)	0.27 (0.90)	-0.10 (0.72)
CDC-based height Z-score, mean (SD)	-4.90 (0.98)	-5.21 (0.93)	-4.96 (0.99)	-5.40 (0.93)
AGV (cm/year), observed mean (SD)	4.0 (1.9)	3.8 (2.0)	3.5 (1.7)	3.7 (1.3)

CDC = Centers for Disease Control and Prevention; AGV = annualized growth velocity.

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.

	Primary Endpoint analysis **All children**	
AGV at Week 52, cm/year	**navepegritide n=57**	**placebo n=27**
LS Mean [95% CI]	**5.89** [5.66, 6.13]	**4.41** [4.04, 4.77]
LS Mean Difference [95% CI] Navepegritide vs. Placebo	**+1.49** [1.05, 1.93] p <0.0001	

Navepegritide was superior to placebo for the primary endpoint of AGV at Week 52

Note: The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.
Note: ANCOVA model includes treatment, stratification factor, baseline age and baseline achondroplasia-specific height Z-score as covariates. Missing height data at Week 52 for 2 participants in the navepegritide group were imputed using the multiple imputation method; CI = confidence interval; LS = least squares.

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.

	Primary Endpoint analysis **All children**		*Subgroup analysis* **Children aged ≥5 years at enrollment**	
AGV at Week 52, cm/year	**navepegritide n=57**	**placebo n=27**	**navepegritide n=36**	**placebo n=17**
LS Mean [95% CI]	**5.89** [5.66, 6.13]	**4.41** [4.04, 4.77]	**5.79** [5.51, 6.07]	**4.02** [3.54, 4.49]
LS Mean Difference [95% CI] Navepegritide vs. Placebo	**+1.49** [1.05, 1.93] p <0.0001		**+1.78** [1.22, 2.33] p <0.0001	

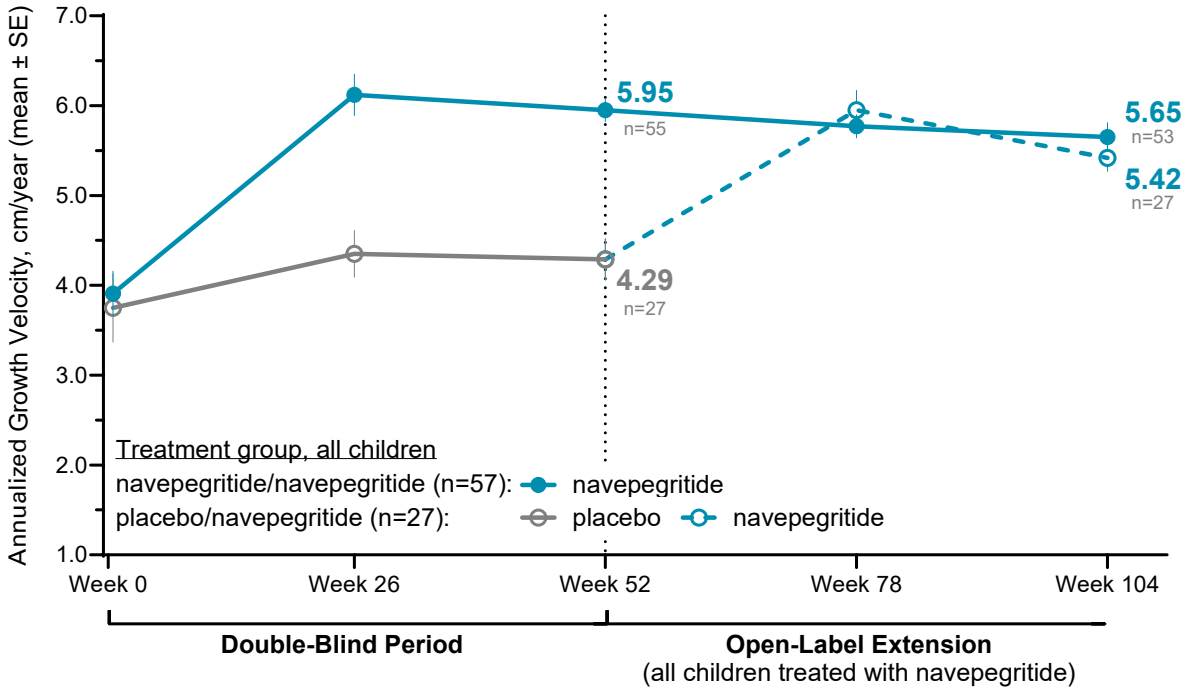
Navepegritide was superior to placebo for the primary endpoint of AGV at Week 52

Navepegritide-treated children aged ≥5 years also had significantly higher AGV at Week 52

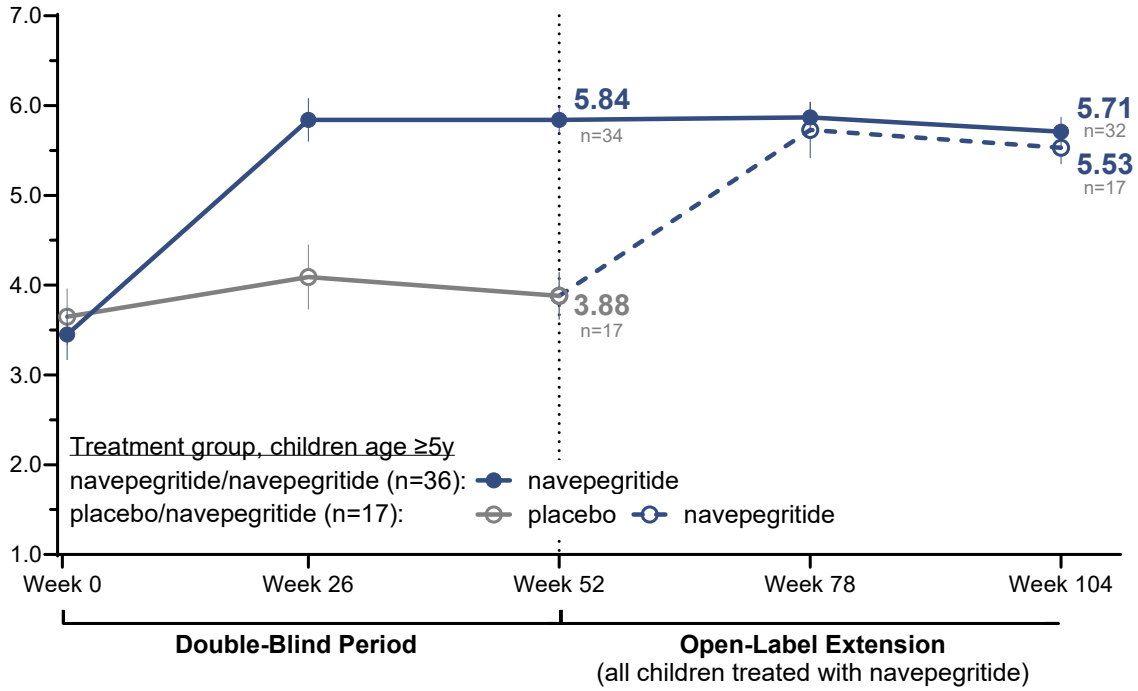
Note: The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.
Note: ANCOVA model includes treatment, stratification factor, baseline age and baseline achondroplasia-specific height Z-score as covariates. Missing height data at Week 52 for 2 participants in the navepegritide group were imputed using the multiple imputation method; CI = confidence interval; LS = least squares

Observed AGV Through Week 104

All children



Children aged ≥5 years at enrollment

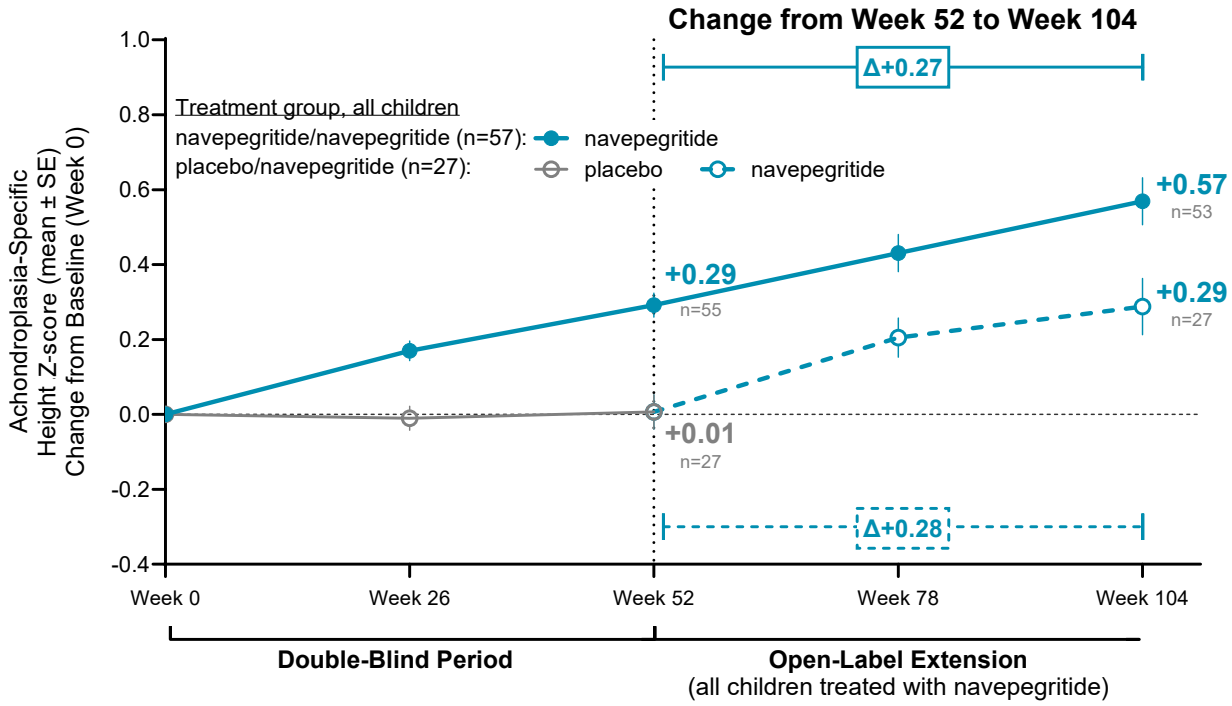


Improvements in AGV with navepegritide treatment were maintained through Week 104 of the OLE

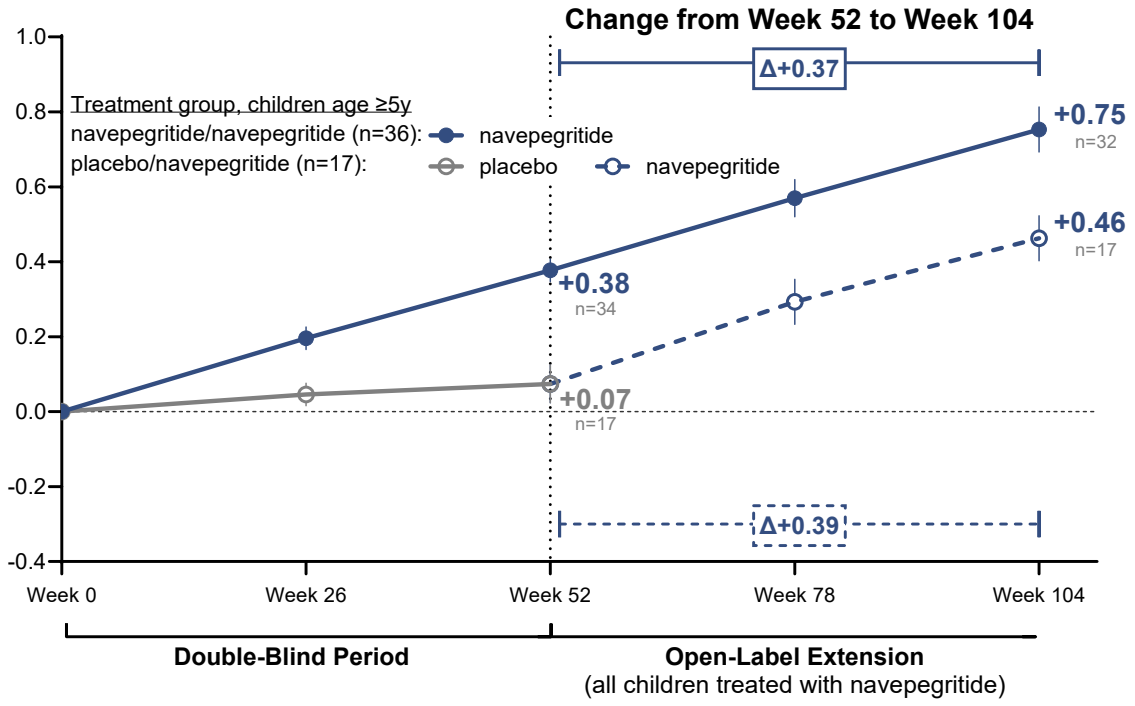
Note: The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.
AGV = annualized growth velocity; OLE = open-label extension; Note: Data shown are observed mean (±SE) AGV

Change in Achondroplasia-Specific Height Z-score

All children



Children aged ≥5 years at enrollment

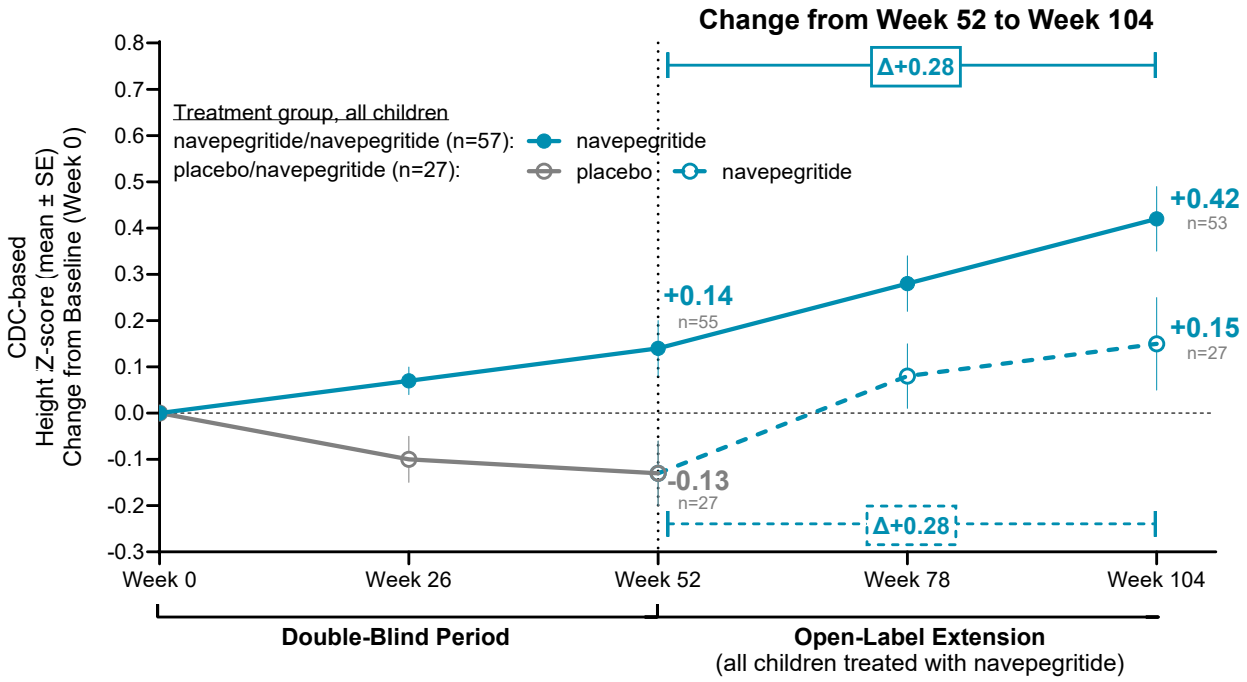


Achondroplasia-specific height Z-scores continued to improve with navepegritide in the OLE, with greater gains observed in children ≥5 years of age relative to the overall population

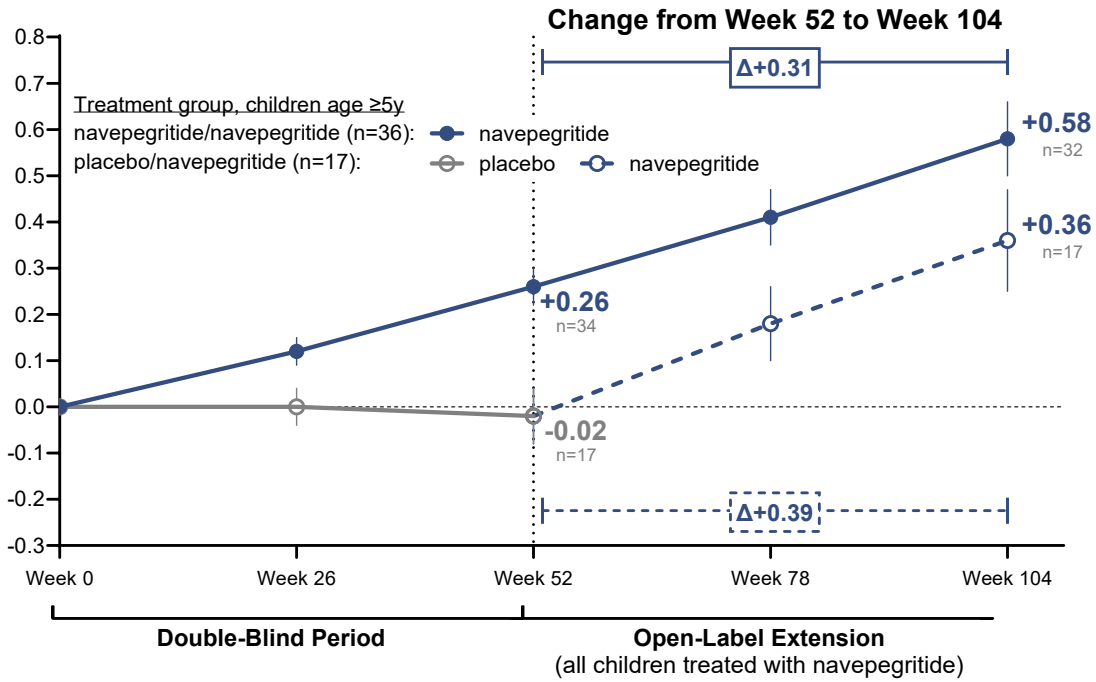
Note: Data shown are observed mean (±SE) change from trial baseline. The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.
OLE = Open-label extension

Change in CDC-Based Height Z-score

All children



Children aged ≥5 years at enrollment



Improvements in CDC-based height Z-scores with navepegritide treatment were more pronounced in children ≥5 years of age

Note: Data shown are observed mean (±SE) change from trial baseline. The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.
CDC = Centers for Disease Control and Prevention

Safety Through Week 104

	All navepegritide-treated children (N=84)	Navepegritide-treated children aged ≥5 years at enrollment (N=53)
Treatment exposure	**137.1 person-years**	**84.9 person-years**
Any treatment-emergent AE, n (%)	76 (90.5)	47 (88.7)
Most frequent AEs (≥15% of participants), n (%)		
Otitis media	31 (36.9)	18 (34.0)
Nasopharyngitis	29 (34.5)	20 (37.7)
Pyrexia	29 (34.5)	13 (24.5)
Upper respiratory tract infection	19 (22.6)	9 (17.0)
Headache	17 (20.2)	12 (22.6)
Vomiting	16 (19.0)	8 (15.1)
Influenza	13 (15.5)	7 (13.2)
Arthralgia	10 (11.9)	9 (17.0)
Injectionsite reaction (ISR), n (%)	17 (20.2)	11 (20.8)
Exposure-adjusted ISR rate[1]	0.35	0.39

The safety profile in children ≥5 years was similar to the overall population, with a low rate of ISRs (all mild), no symptomatic hypotension, and no acceleration of bone age

[1]Events per person-year of exposure
N = number of participants with observation; AE = adverse event; ISR = injection-site reaction

Conclusions

- Results from the ApproaCH Trial support the efficacy and safety of navepegritide through 104 weeks of treatment
- Navepegritide-related improvements in AGV were maintained through Week 104
- Height Z-scores continued to increase through Week 104 with ongoing navepegritide treatment
- The safety profile in children ≥5 years was similar to the overall population, with a low rate of ISRs, no symptomatic hypotension, and no acceleration of bone age

Children aged 5 years or older at enrollment experienced more pronounced gains in growth outcomes relative to the overall population

AGV = annualized growth velocity.
ISRs = Injection site reactions

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