TransCon™ CNP (navepegritide)
ApproaCH Trial Topline Results

September 16, 2024

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This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation, such as statements regarding our expected timing for submission of certain regulatory filings related to TransCon CNP; our expectations regarding TransCon CNP's potential to meet the need for a treatment addressing the health and quality-of-life complications of achondroplasia; our development plans for TransCon CNP; our ability to apply our TransCon technology platform to build a leading, fully integrated biopharma company, particularly in the treatment of skeletal dysplasias and growth disorders; plans and objectives of management for future operations and commercialization activities; and future results of current and anticipated products and product candidates, are forward-looking statements. These forward-looking statements are based on our current expectations and beliefs, as well as assumptions concerning future events. These statements involve known and unknown risks, uncertainties and other factors that could cause our actual results to differ materially from the results discussed in the forward-looking statements. These risks, uncertainties and other factors are more fully described in our reports filed with or submitted to the Securities and Exchange Commission (SEC), including, without limitation, our most recent Annual Report on Form 20-F filed with the SEC on February 7, 2024, particularly in the sections titled "Risk Factors" and "Operating and Financial Review and Prospects." In light of the significant uncertainties in our forward-looking statements, you should not place undue reliance on these statements or regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all.

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Executive Summary

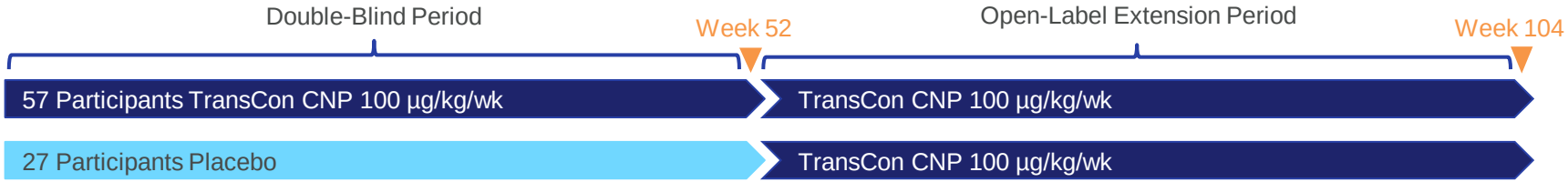
- The pivotal ApproaCH Trial in children aged 2-11 years achieved primary objective
  - TransCon CNP demonstrated AGV superior to placebo with LS mean treatment difference of 1.49 cm/year at Week 52 ($p<0.0001$)
  - For children aged 5-11 years TransCon CNP demonstrated a change from baseline AGV superior to placebo with LS mean treatment difference of 1.78 cm/year at Week 52 ($p<0.0001$)
  - Children with achondroplasia treated with TransCon CNP exceeded growth rate of general population, providing catch-up growth without accelerated bone age
- Other endpoints supportive that TransCon CNP may provide benefits beyond linear growth
  - Treatment with TransCon CNP resulted in numerical improvements in health-related quality of life compared to placebo as observed in several ACEM domains
  - Patients dosed with TransCon CNP demonstrated statistical improvement in body proportionality compared to baseline
- TransCon CNP was generally well-tolerated, with low frequency of mild injection site reactions
  - Continued to show safety results similar to placebo and was well-tolerated, with generally mild TEAEs
  - No evidence of hypotensive effect
  - Injections were generally well tolerated, with low frequency of injection site reactions (0.41 events per patient year), all mild

Once-weekly TransCon CNP has potential to address the needs for an efficacious, safe, tolerable, and convenient treatment

Data on file, Ascendis Pharma 2024.

ApproaCH Pivotal Trial Design: TransCon CNP in Children with Achondroplasia Ages 2 to 11 years

Double-blind, placebo-controlled trial with an open-label extension period
84 children (ages 2 to 11 years) with achondroplasia randomized 2:1 (TransCon CNP:Placebo)



Primary Objective
Evaluate efficacy of TransCon CNP on annualized growth velocity (AGV)

Secondary Objective
Evaluate efficacy of TransCon CNP on height Z-score
Evaluate impact of TransCon CNP on health-related QoL

Safety Objective
Evaluate safety & tolerability of TransCon CNP

Sample Size and Stratification
84 randomized participants, stratified by age and sex (aged < 5 years, aged ≥ 5 years and female, aged ≥ 5 years and male)

Countries
United States, Canada, Denmark, Ireland, Spain, United Kingdom, Australia, New Zealand

Primary Endpoint
Annualized growth velocity (AGV) at Week 52

Secondary Endpoints
Change from baseline in height Z-score at Week 52
Change from baseline in SF-10 physical summary score at Week 52
Change from baseline in ACEM-PF, ACEM-DF, ACEM-OSM scores at Week 52

Safety Endpoint
Incidence of treatment emergent adverse events (TEAEs) and safety assessments

Selected Other Endpoints
Bone age
Upper- to lower-body segment ratio (proportionality)
ACEM-EW
Muscle functionality test

Demographics and Baseline Characteristics (1/2)

Full Analysis Set	TransCon CNP (n=57)	Placebo (n=27)	Overall (N=84)
Age (years), mean (SD)	5.6 (2.6)	6.0 (2.7)	5.7 (2.6)
Age (years) group, n (%)			
2-4	21 (36.8)	10 (37.0)	31 (36.9)
5-7	26 (45.6)	10 (37.0)	36 (42.9)
≥8	10 (17.5)	7 (25.9)	17 (20.2)
Sex, n (%)			
Female	26 (45.6)	13 (48.1)	39 (46.4)
Male	31 (54.4)	14 (51.9)	45 (53.6)
Strata (sex, years), n (%)			
<5 years	21 (36.8)	10 (37.0)	31 (36.9)
≥5 years and female	17 (29.8)	8 (29.6)	25 (29.8)
≥5 years and male	19 (33.3)	9 (33.3)	28 (33.3)
Region, n (%)			
United States	14 (24.6)	8 (29.6)	22 (26.2)
Europe	29 (50.9)	14 (51.9)	43 (51.2)
Rest of world	14 (24.6)	5 (18.5)	19 (22.6)

Data on file, Ascendis Pharma 2024.

Demographics and Baseline Characteristics (2/2)

Full Analysis Set	TransCon CNP (n=57)	Placebo (n=27)	Overall (N=84)
Height (cm), mean (SD)	88.9 (12.9)	89.1 (11.5)	89.0 (12.4)
ACH-specific height Z-score*, mean (SD)	0.18 (0.92)	-0.11 (0.73)	0.09 (0.87)
CDC (general population) height Z-score**, mean (SD)	-4.90 (0.98)	-5.21 (0.93)	-5.00 (0.97)
Weight (kg), mean (SD)	17.8 (6.9)	16.8 (4.6)	17.5 (6.3)
BMI (kg/m^2), mean (SD)	21.7 (2.7)	20.9 (2.8)	21.4 (2.7)
Genetic variant causing achondroplasia, n (%) 1138G>A 1138G>C Other	 54 (94.7) 3 (5.3) 0	 24 (88.9) 0 3 (11.1)	 78 (92.9) 3 (3.6) 3 (3.6)
Age at ACH diagnosis (years), n (%) Pre-birth At birth ≥0 to 6 months ≥6 to 12 months ≥12 months	 15 (26.3) 20 (35.1) 18 (31.6) 2 (3.5) 1 (1.8)	 9 (33.3) 6 (22.2) 9 (33.3) 3 (11.1) 0	 24 (28.6) 26 (31.0) 27 (32.1) 5 (6.0) 1 (1.2)

Well balanced between key baseline demographics such as height, sex, and age

*Hoover-Fong JE, et al. *US. Orphanet J Rare Dis*. 2021;16(1):522. **https://www.cdc.gov/nccdphp/dnpao/growthcharts/index.htm

Data on file, Ascendis Pharma 2024.

Overview of Treatment Emergent Adverse Events (TEAEs)

Safety analysis set	TransCon CNP (n=57) n (%)	Placebo (n=27) n (%)
Any Adverse Event	**52 (91%)**	**26 (96%)**
Grade 1	52 (91%)	25 (93%)
Grade 2	16 (28%)	11 (41%)
Grade 3	4 (7%)	1 (4%)
Grade 4	0	0
Grade 5	0	0
Any treatment-related adverse events	12 (21%)	7 (26%)
Serious adverse events	3 (5%)	3 (11%)
Adverse events of special interest	11 (19%)	4 (15%)
Injection site reactions	11 (19%)	4 (15%)
Fractures	0	0
Symptomatic hypotension	0	0
AE leading to discontinuation of study drug	0	0
AE leading to withdrawal from trial	0	0
AE leading to death	0	0

Safety and tolerability results comparable to placebo, with generally mild TEAEs

Data on file, Ascendis Pharma 2024.

TransCon CNP Safety and Tolerability Results

- TransCon CNP showed safety results comparable to placebo and was generally well tolerated, with generally mild TEAEs
- Majority of AEs were mild (Grade 1) or moderate (Grade 2) and typical for children of these ages
- No AEs led to discontinuation of TransCon CNP or withdrawal from the trial and no SAEs were assessed as related to TransCon CNP
- No deaths were reported
- No fractures or other bone-related safety events observed
- No evidence of hypotensive effect with TransCon CNP
- Injections were generally well tolerated, with low frequency of injection site reactions (0.41 events per patient year), all mild

Data on file, Ascendis Pharma 2024.

Primary Endpoint: Annualized Growth Velocity at Week 52

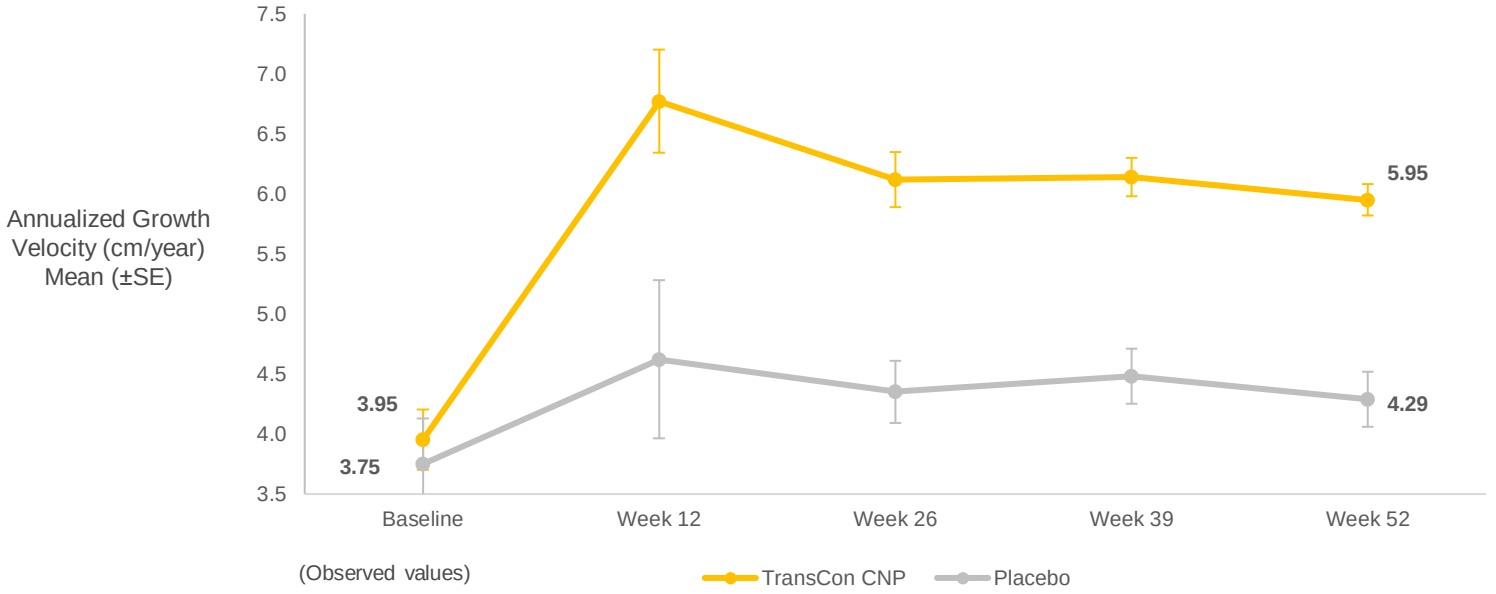
	TransCon CNP (n=57)	Placebo (n=27)
LS Mean AGV (cm/yr) at Week 52	5.89	4.41
LS Mean Difference [95% CI] (TransCon CNP vs. Placebo)	1.49 [1.05, 1.93]	
p-value (TransCon CNP vs. Placebo)	p <0.0001	

ANCOVA model.

TransCon CNP achieved the primary objective, demonstrating superiority over placebo

Data on file, Ascendis Pharma 2024.

Observed Annualized Growth Velocity Over 52 Weeks



Children treated with TransCon CNP increased their AGV 2.00 cm/year over 52 weeks

Data on file, Ascendis Pharma 2024.

Sub-Group Analyses: Primary Endpoint by Age Groups

Sub-group analyses by age	2 to <5 years		5-11 years	
	TransCon CNP (n=21)	Placebo (n=10)	TransCon CNP (n=36)	Placebo (n=17)
LS Mean AGV (cm/yr) at Week 52	6.07	5.06	5.79	4.02
LS Mean Difference [95% CI], p-value (TransCon CNP vs. Placebo)	1.02 [0.29, 1.74]		1.78 [1.22, 2.33]	
p-value (TransCon CNP vs. Placebo)	p=0.0084		p<0.0001	

ANCOVA model.

TransCon CNP demonstrated superior AGV compared to placebo in both age groups

Data on file, Ascendis Pharma 2024.

Sub-Group Analyses: Change from Baseline AGV by Age Group

Sub-group analyses by age	2 to <5 years		5-11 years	
	TransCon CNP (n=19)	Placebo (n=10)	TransCon CNP (n=35)	Placebo (n=17)
Change from Baseline AGV at Week 52, LS Mean	1.57	0.43	2.29	0.52
Treatment Difference, [95% CI], (TransCon CNP vs. Placebo)	1.15 [0.40, 1.89]		1.78 [1.20, 2.35]	
p-value (TransCon CNP vs. Placebo)	p=0.0047		p<0.0001	

ANCOVA model.

TransCon CNP demonstrated significant change from baseline AGV compared to placebo by age group

Data on file, Ascendis Pharma 2024.

Secondary Endpoint:
Change from Baseline in Height Z-score at Week 52

ACH Height Z-Score	TransCon CNP (n=57)	Placebo (n=27)
LS Mean CFB at Week 52	0.30	0.01
LS Mean Difference [95% CI] (TransCon CNP vs. Placebo)	0.28 [0.18, 0.39]	
p-value (TransCon CNP vs. Placebo)	<0.0001	

CDC Height Z-score	TransCon CNP (n=55)	Placebo (n=27)
LS Mean CFB at Week 52	0.15	-0.15
LS Mean Diff [95% CI] (TransCon CNP vs. Placebo)	0.30 [0.14, 0.45]	
p-value (TransCon CNP vs. Placebo)	0.0003	

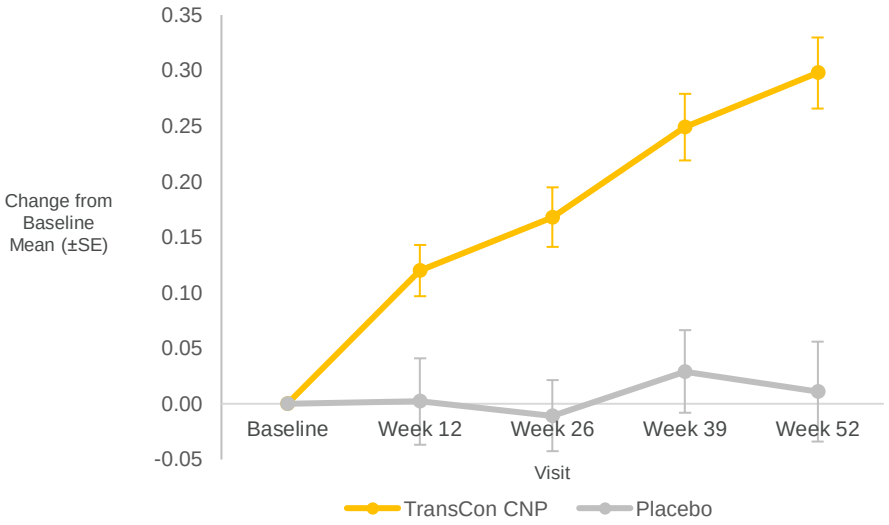
ANCOVA model.

TransCon CNP achieved secondary objective demonstrating superiority over placebo

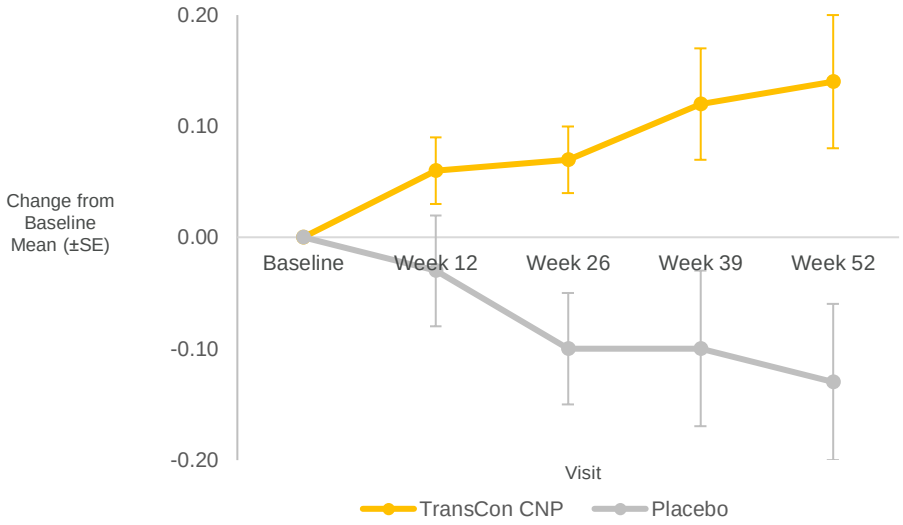
Data on file, Ascendis Pharma 2024.

Change from Baseline in Height Z-score Over 52 Weeks

ACH* Height Z-score



CDC Height Z-score**



Children with achondroplasia treated with TransCon CNP exceeded growth rate of general population, providing catch-up growth without accelerated bone age

*Hoover-Fong JE, et al. *US. Orphanet J Rare Dis*. 2021;16(1):522. **https://www.cdc.gov/nccdphp/dnpao/growthcharts/index.htm

Data on file, Ascendis Pharma 2024.

Results Beyond Linear Growth

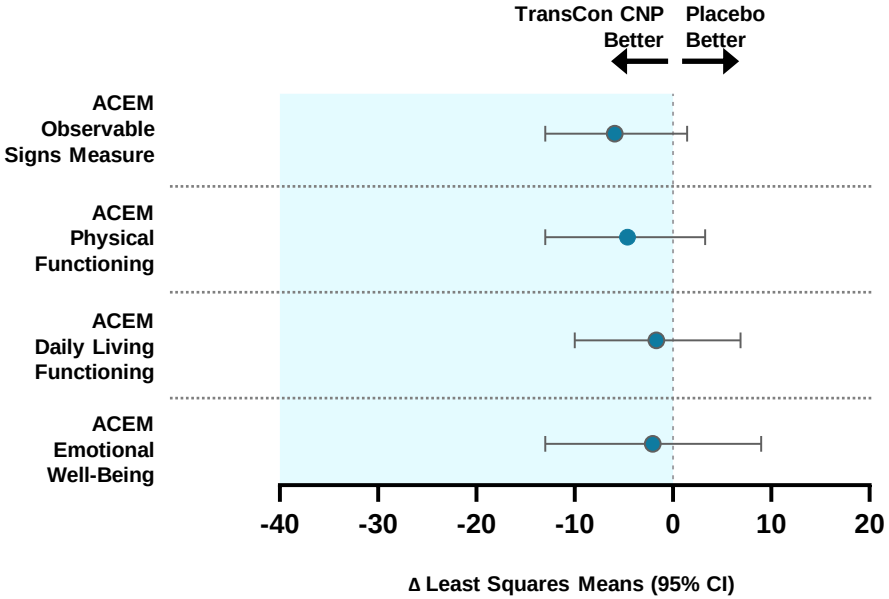
- In the total trial population, treatment with TransCon CNP resulted in numerical improvements in health-related quality of life compared to placebo as observed in several ACEM domains
  - At baseline, parents of children generally reported lower burden of health-related quality of life compared to the ACcomplisH Trial
- Selected endpoints beyond linear growth:
  - Health-related signs and symptoms and quality of life measures: ACEM-OSM, -PF, -DL, -EW and SF-10 PHS
  - Muscle functionality testing (exploratory endpoint)
- Predefined sub-group analyses of ACEM-Physical Functioning demonstrated potential treatment effect, supported by muscle functionality test results

For children with health-related QoL burden at baseline a potential treatment effect was observed across several HRQoL domains of the ACEM measures

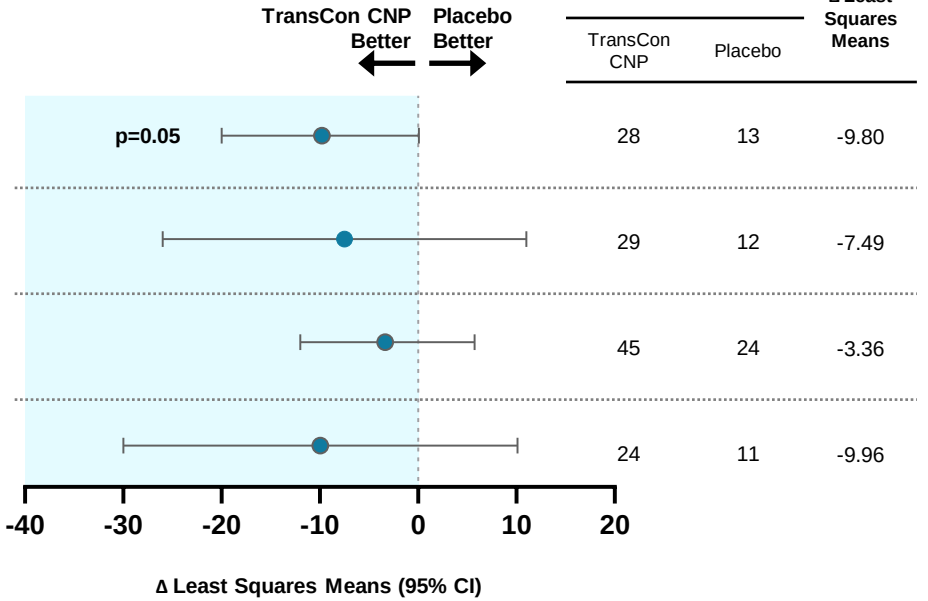
ACEM: Achondroplasia Child Experience Measure, -OSM: Observable Signs Measure, -PF: Impact Physical Functioning, -DL: Impact Daily Living Functioning, -DW:Impact Emotional-Wellbeing, SF-10 PHS: SF-10 Health Survey for Children Physical Summary Score

Results in Patients with Reported Health-Related QoL Burden at Baseline*

Total Trial Population



Patients with HRQoL Burden ≥20 at Baseline*

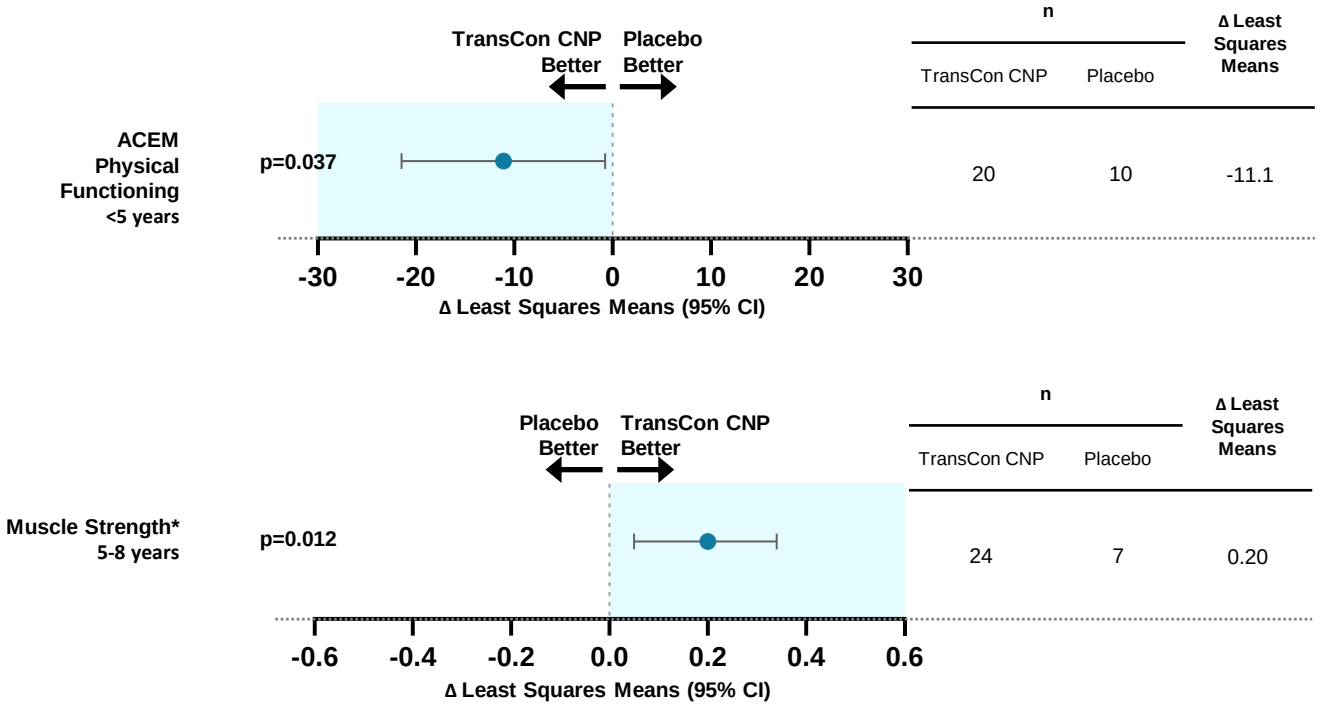


	n		Δ Least Squares Means
	TransCon CNP	Placebo	
ACEM Observable Signs Measure	28	13	-9.80
ACEM Physical Functioning	29	12	-7.49
ACEM Daily Living Functioning	45	24	-3.36
ACEM Emotional Well-Being	24	11	-9.96

*Post hoc analysis of data with baseline scores at or above 20. Nominal p-value.

Data on file, Ascendis Pharma 2024.

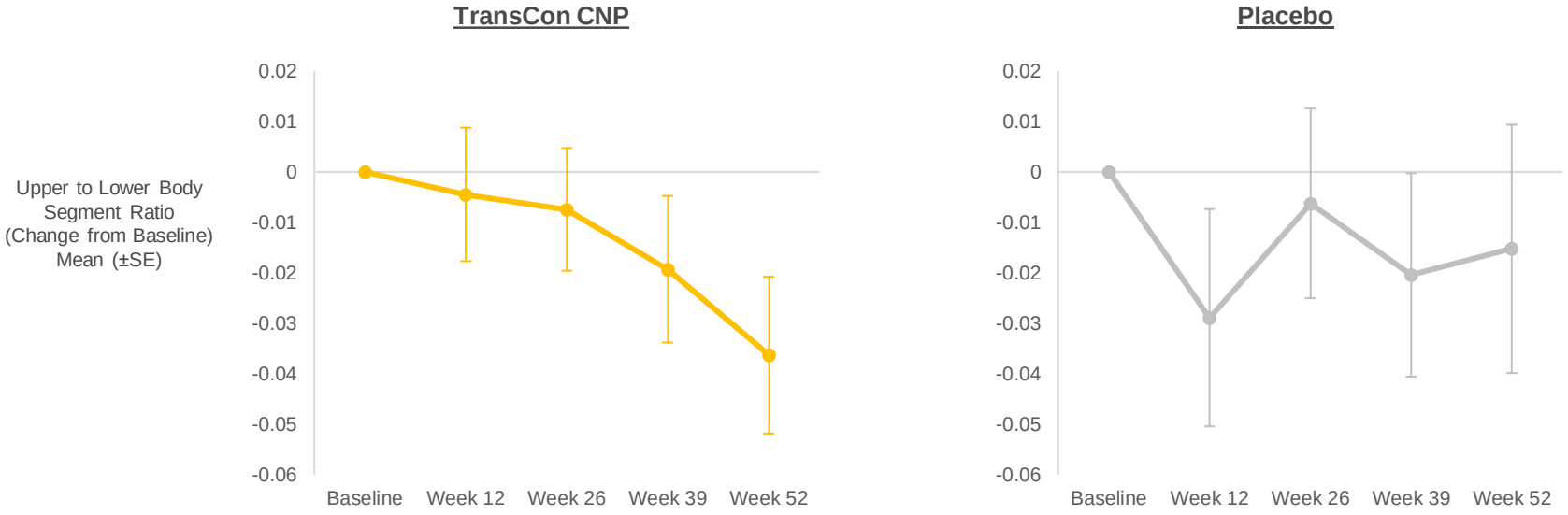
Results in ACEM-PF Predefined Sub-Group Are Supported by Exploratory Muscle Functionality Endpoint



*Week 52 Body-weight normalized torque in Knee Extension test [N*m/kg]; Exploratory end-point in trial subjects ≥5years at testing. Sub-group age at testing. Nominal p-values.

Data on file, Ascendis Pharma 2024.

ApproaCH Body Proportionality



Patients dosed with TransCon CNP demonstrated statistical improvement in body proportionality compared to baseline (p=0.02) while placebo was unchanged from baseline (p=0.43)

Nominal p-value. Data on file, Ascendis Pharma 2024.

Summary & Next Steps

- The pivotal ApproaCH Trial results support the desired target product profile of once-weekly TransCon CNP delivering continuous exposure of CNP
- Plan to submit New Drug Application to the U.S. FDA for TransCon CNP for treatment of children with achondroplasia in the first quarter of 2025
- Plan to submit a Marketing Authorisation Application for treatment of children with achondroplasia to the European Medicines Agency during the third quarter of 2025
- Comprehensive development plans continue with ongoing and planned trials to support TransCon CNP in additional patient populations
  - Strong retention with all 82 children who completed the double-blind period are continuing in the open-label extension of ApproaCH

With once-weekly SKYTROFA and TransCon CNP, Ascendis is uniquely positioned to become the leader in treatment of skeletal dysplasias and growth disorders

Vision 2030

Achieve blockbuster status for multiple products and expand our engine for future innovation

- **Be the Leading Endocrinology Rare Disease Company**
  - Achieve blockbuster status (>$1B) for TransCon PTH, TransCon hGH, and TransCon CNP through worldwide commercialization
  - Be the leader in Growth Disorders and Hypoparathyroidism, pursuing clinical conditions, innovative LCM and complementary patient offerings
  - Expand pipeline with Endocrinology Rare Disease blockbuster product opportunities
- **Create Value in Additional Therapeutic Areas through Innovative Business Models**
  - Obtain accelerated approval in oncology with registrational trials ongoing
  - Pursue TransCon product opportunities in >$5B indications
  - Maximize value creation of these product opportunities through collaboration with therapeutic area market leaders
- **Differentiate with Ascendis Fundamentals**
  - Outperform industry drug development benchmarks with Ascendis' product innovation algorithm
  - Remain independent as a profitable biopharma through lean and flexible ways of working
  - Let our values Patients, Science, Passion drive our decisions to success