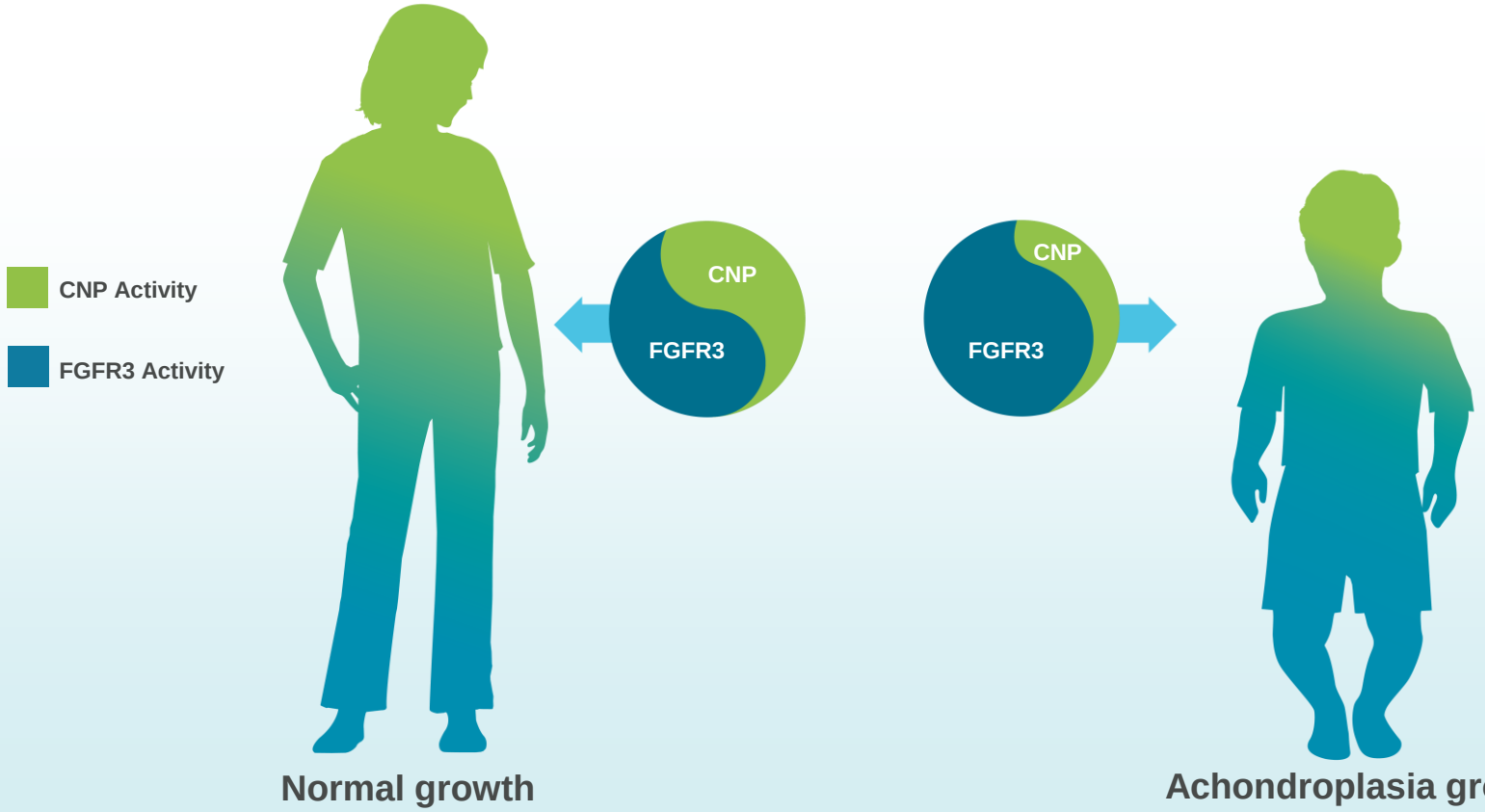
TransCon CNP:
Preliminary Phase 1 Data

November 28, 2018

Normal Growth Depends on Balanced Pathways

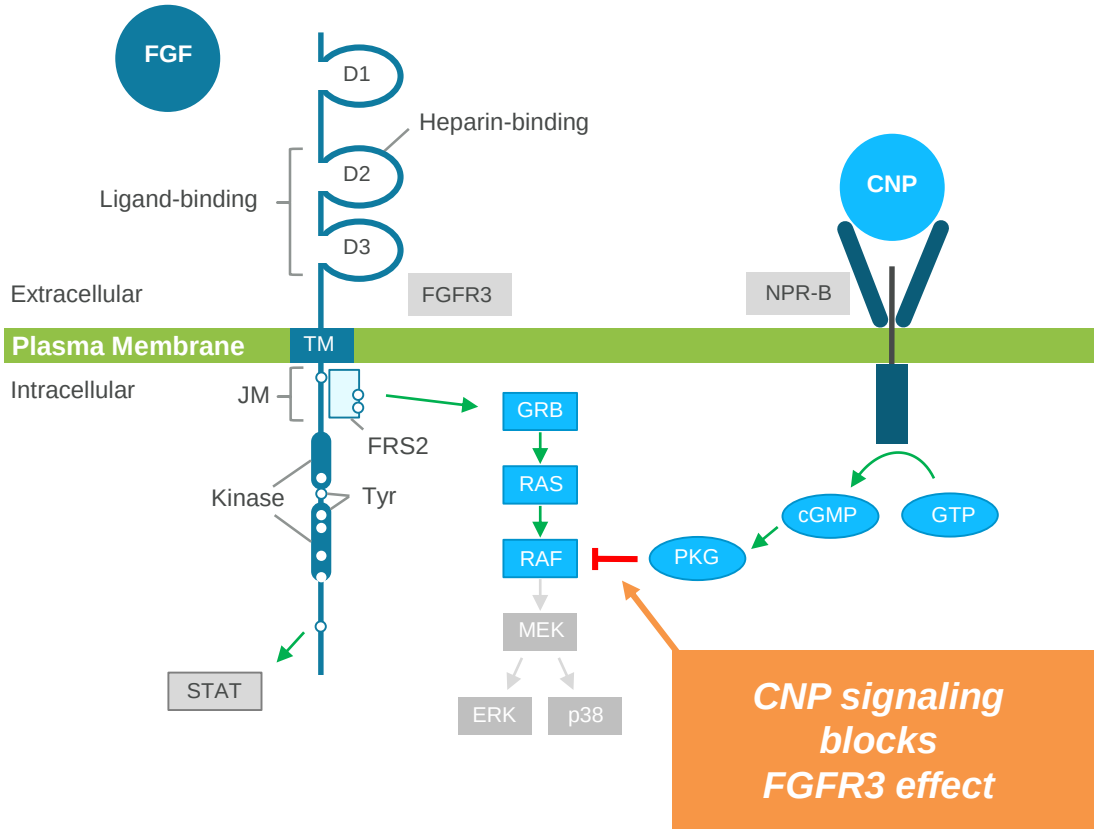


TransCon CNP is designed to provide continuous exposure to CNP to optimize efficacy with a well-tolerated and convenient once-weekly dose

Achondroplasia Signaling Defect is Well Understood

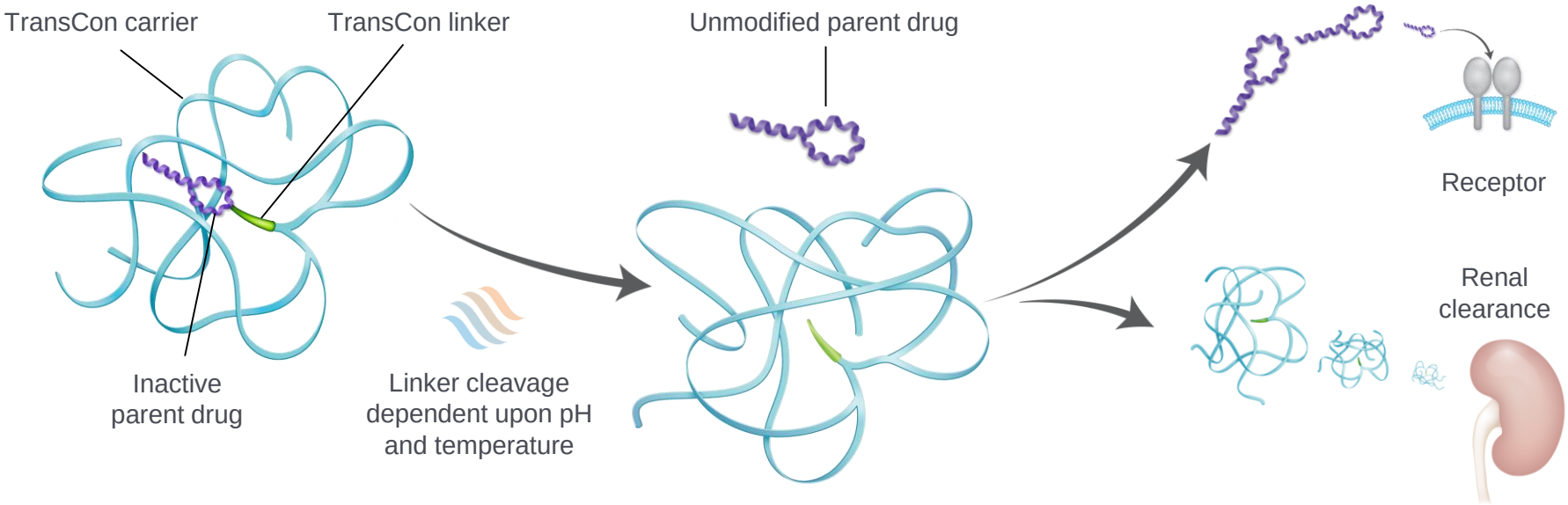
- **FGFR3 negatively regulates chondrocyte proliferation and differentiation and hence bone growth**
- **Achondroplasia results from a mutation in FGFR3 which leaves the receptor constitutively activated**
- **CNP inhibits the FGFR3 pathway and thereby promotes proliferation and differentiation of chondrocytes to restore bone growth**

FGFR3 Signaling Pathway[1]



[1]Adapted from Current Opin Pediatrics 2010; 22:516-523

TransCon Technology Offers Potential Solution



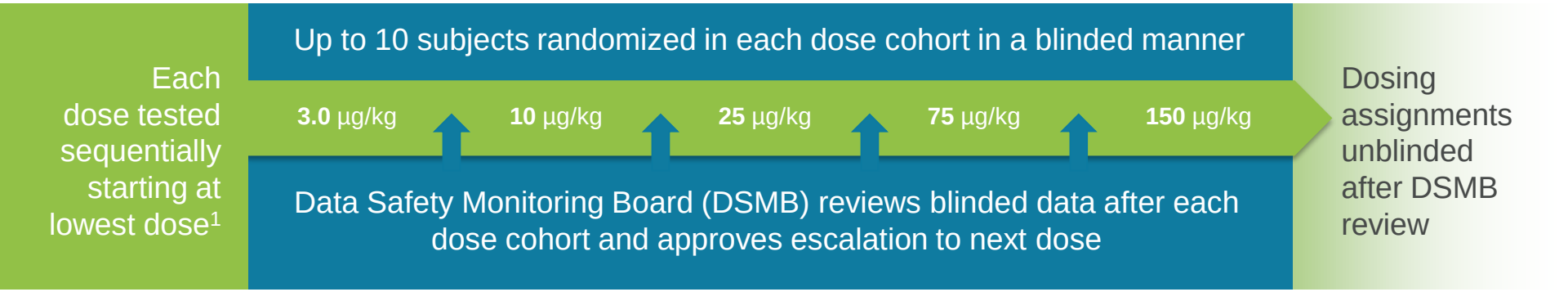
- TransCon technology provides effective shielding of CNP:
  - From neutral endopeptidase degradation in subcutaneous tissue and blood compartment
  - Minimize binding of TransCon CNP to the NPR-C clearance receptor
  - Reduce binding of TransCon CNP to the NPR-B receptor in vasculature to avoid hypotension
- Unmodified CNP liberated from TransCon CNP maintains small enough size to allow penetration into growth plates

TransCon CNP: Phase 1 Trial

A Phase 1, Double-Blind, Randomized, Placebo-Controlled, Dose Escalation Trial Evaluating Safety, Tolerability and Pharmacokinetics of Subcutaneous Single Doses of TransCon CNP in Healthy Adult Male Subjects

Phase 1 Trial Design

45 healthy adult male subjects enrolled at two study centers in Australia
TransCon CNP vs. placebo (4:1 randomization)



Primary Endpoint

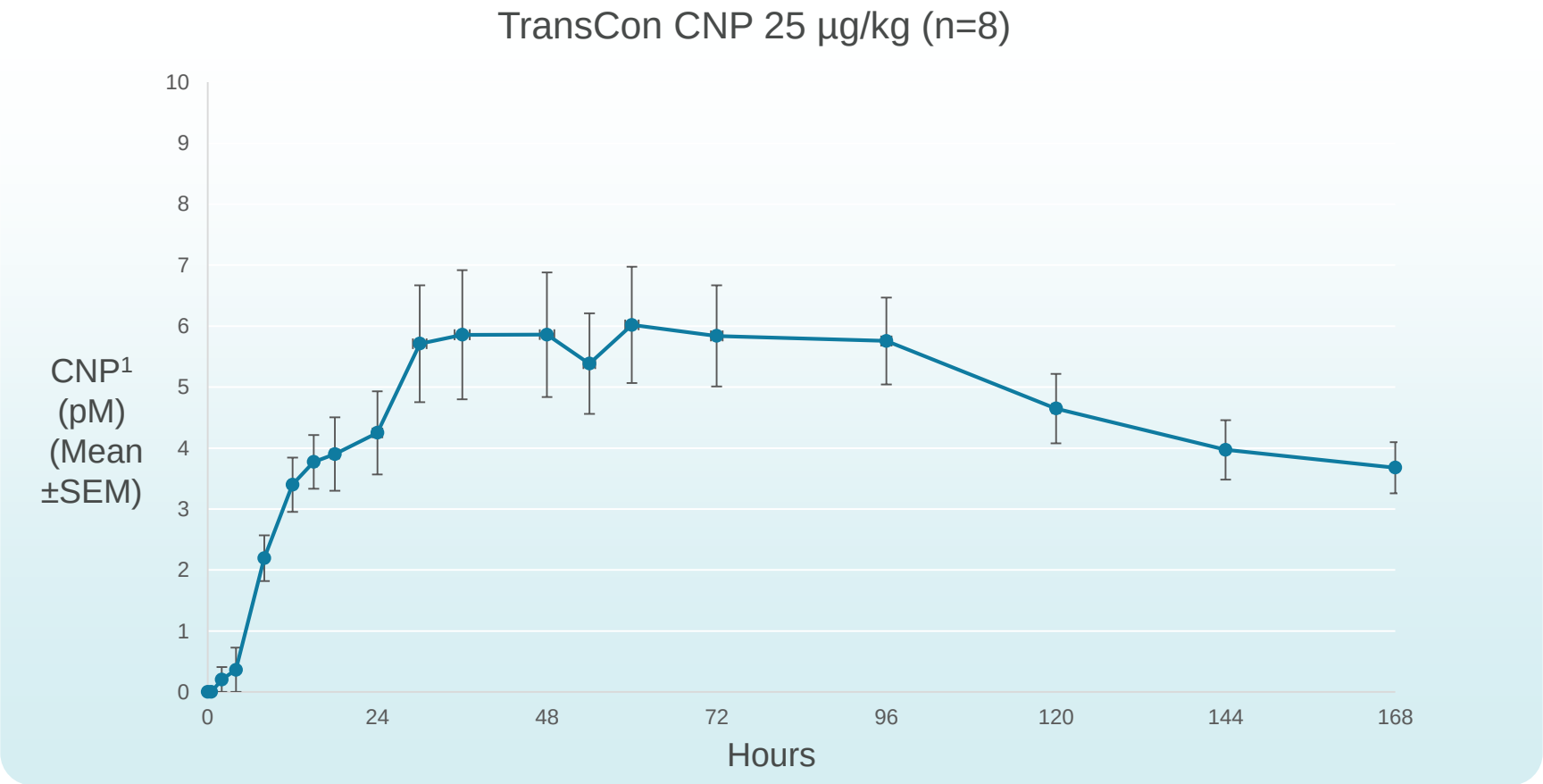
- Frequency of adverse events (AEs) reported after administration of TransCon CNP

Secondary/Exploratory Endpoints

- Safety parameters and local tolerability assessment
- Pharmacokinetic parameters
- Other exploratory endpoints

[1] 300 µg CNP/kg cohort was deemed not clinically relevant based on emerging pharmacokinetic data from previous cohorts and therefore not dosed.

Sustained CNP Exposure Over One Week

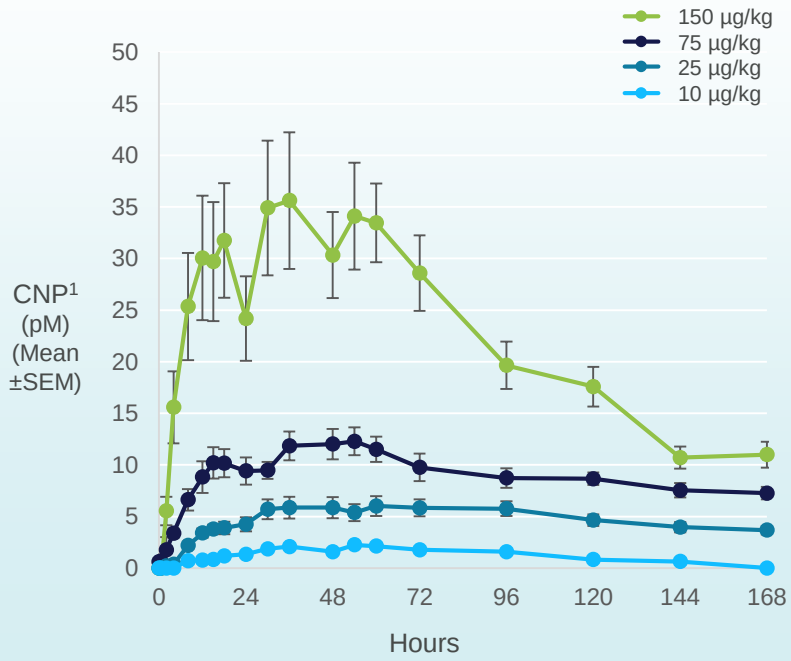


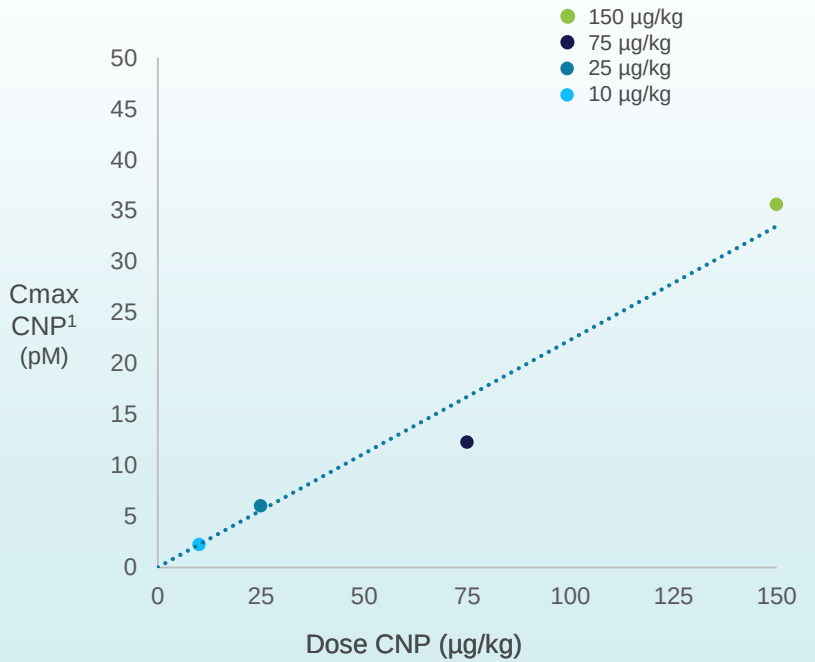
A single dose of TransCon CNP provided continuous CNP exposure with low inter-subject variability over the entire week

[1] CNP measured as CNP-38.

Dose-related Increase in CNP Exposure

TransCon CNP 10, 25, 75 and 150 µg/kg
(n=5-8/group)

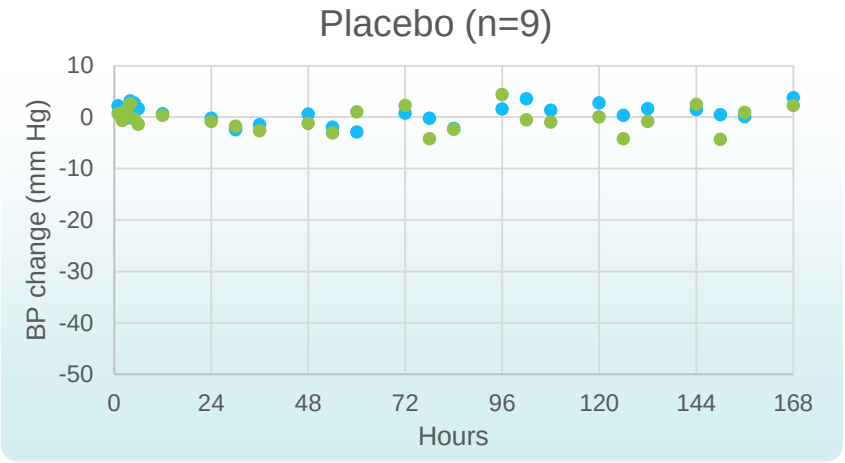


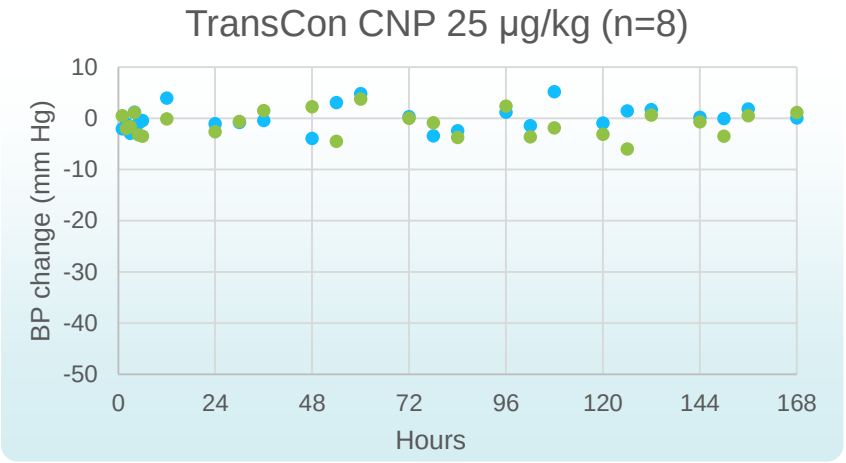


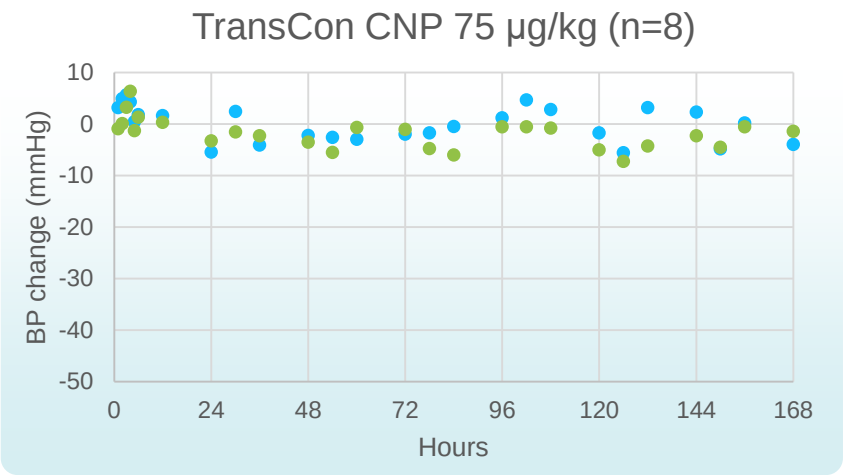
- Dose-related increase in CNP exposure suggests ability to titrate dosing
- Phase 1 showed effective CNP $t_{1/2}$ of ~ 90 hours (native CNP $t_{1/2}$ of 2-3 minutes)

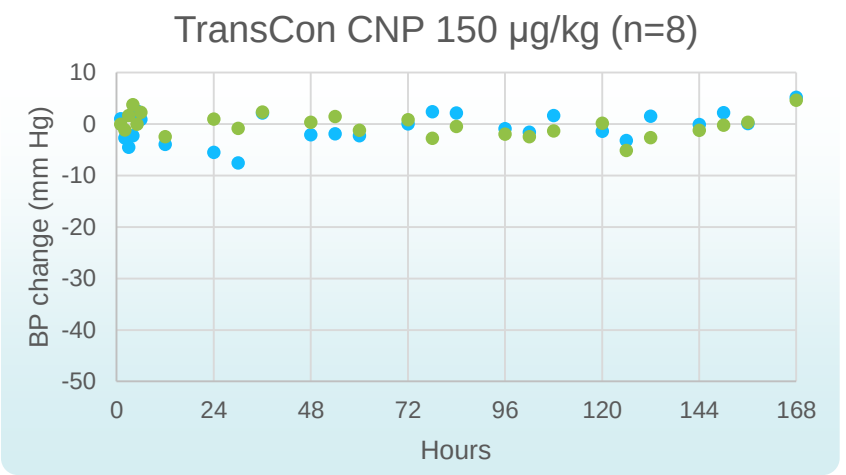
[1] CNP measured as CNP-38.

Mean Resting Blood Pressure Unchanged from Predose[1]



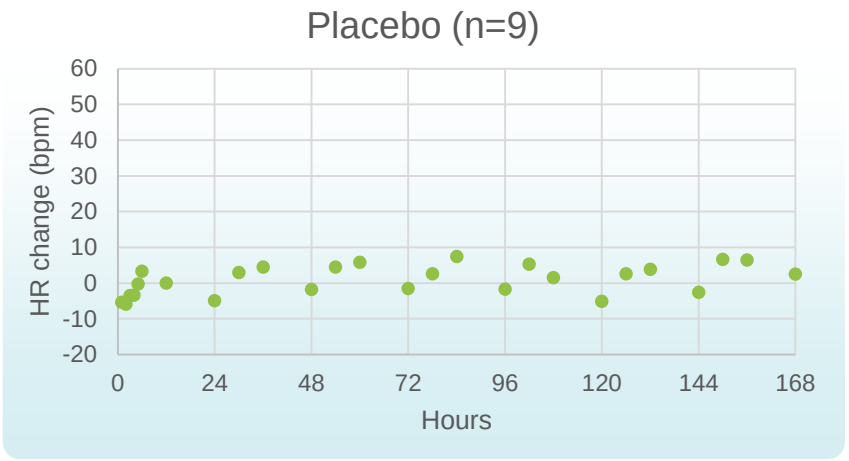


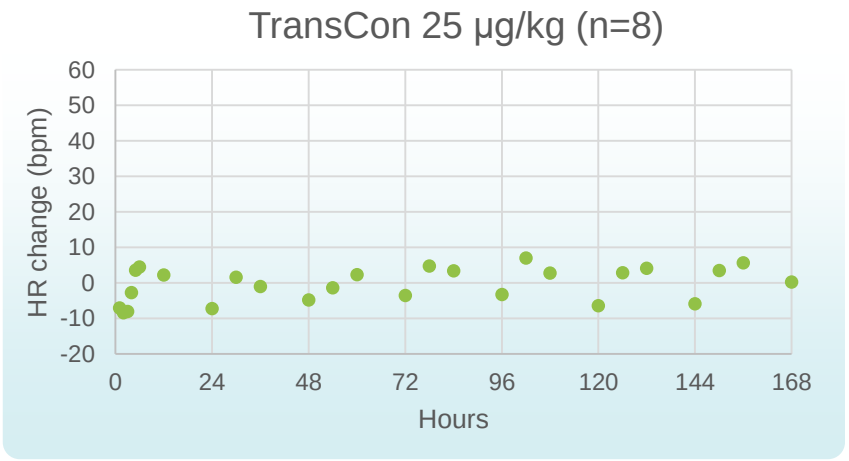


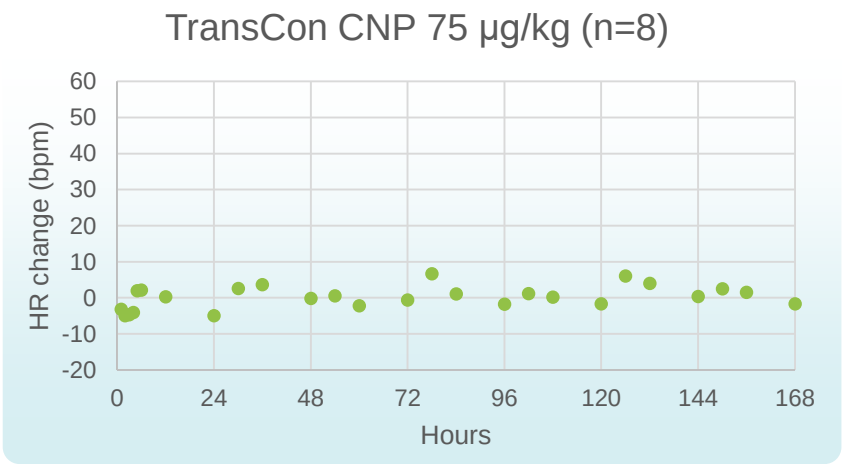


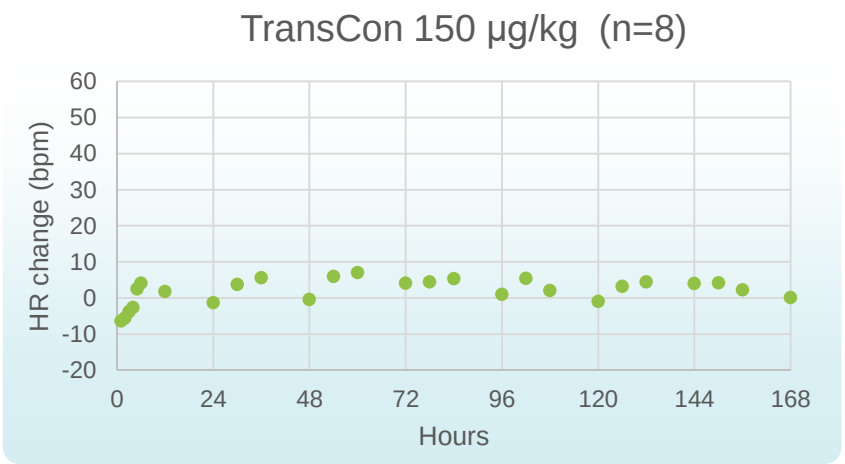
[1] 3.0 and 10 µg/kg dose levels are not represented. Data from these cohorts are consistent with placebo.

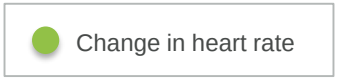
Mean Resting Heart Rate Unchanged from Predose[1]











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Well-tolerated Safety Profile

- No serious AEs were reported in the trial
- TransCon CNP was generally well tolerated at doses up to 150 µg/kg
- Mean resting blood pressure and heart rate were unchanged from predose at all time points, in all cohorts
- Mean orthostatic changes in vital signs appear unrelated to TransCon CNP exposure; consistent between placebo and TransCon CNP cohorts
- Injections were well tolerated in all dose cohorts; no reported injection AEs

Achieved Target Product Profile in Phase 1

- TransCon CNP phase 1 data reproduced PK profile and cardiovascular safety from preclinical studies
- Provided continuous CNP exposure over seven days with a single subcutaneous administration, supporting once-weekly dosing
- Delivered continuous CNP exposure at target levels which is important for balancing the CNP/FGFR3 pathways and normalizing growth
- Generally well tolerated across all cohorts
  - Unchanged mean resting blood pressure and heart rate compared to predose
  - Well-tolerated injections
- Potential for a significant impact on patients' lives, not only affecting height but also addressing many comorbidities associated with achondroplasia