Ascendis Pharma A/S

March 2019

Cautionary Note On Forward-Looking Statements

This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation, such as statements regarding our future results of operations and financial position, including our business strategy, prospective products, availability of funding, clinical trial results, product approvals and regulatory pathways, collaborations, timing and likelihood of success, plans and objectives of management for future operations, and future results of current and anticipated products, 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, including, without limitation, our most recent Annual Report on Form 20-F filed with the SEC on March 28, 2018, particularly in the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations". 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.

Any forward-looking statement made by us in this presentation speaks only as of the date of this presentation and represents our estimates and assumptions only as of the date of this presentation. Except as required by law, we assume no obligation to update these statements publicly, whether as a result of new information, future events or otherwise after the date of this presentation.

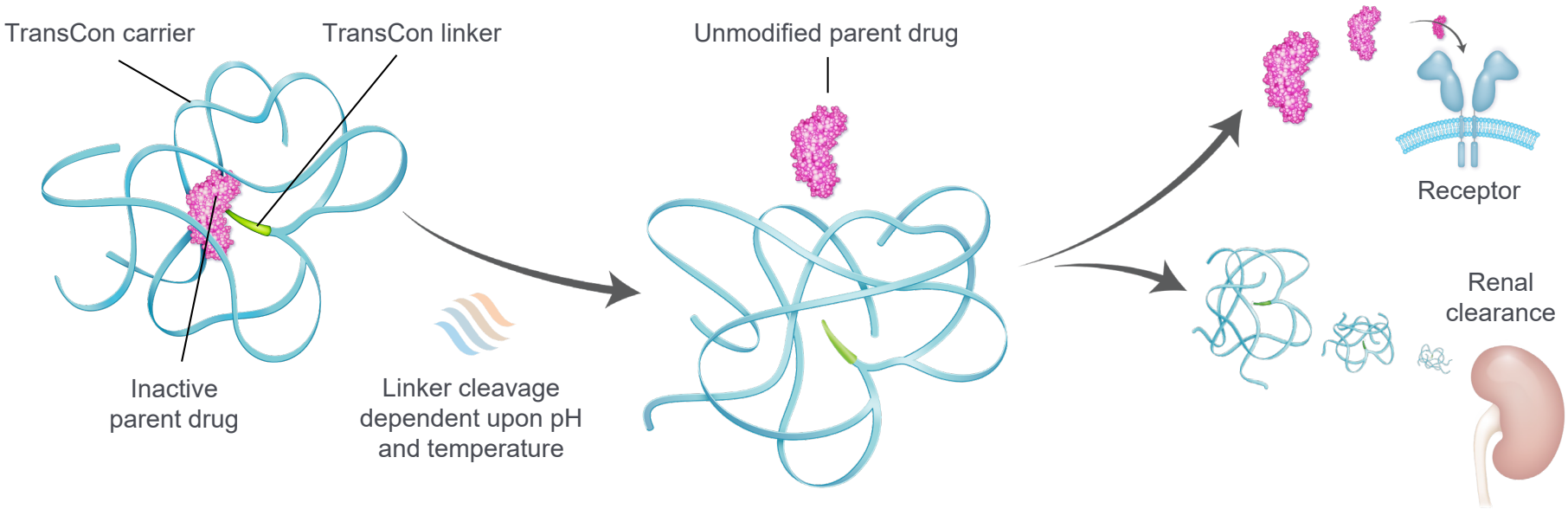
This presentation concerns product candidates that are or have been under clinical investigation and which have not yet been approved for marketing by the U.S. Food and Drug Administration, European Medicines Agency or other foreign regulatory authorities. These product candidates are currently limited by U.S. Federal law to investigational use, and no representations are made as to their safety or effectiveness for the purposes for which they are being investigated.

Company Overview

- Create best-in-class products addressing unmet medical needs by applying TransCon™ technologies to parent drugs with clinical proof-of-concept
  - Unique algorithm for product innovation has resulted in clinical validation of 3 out of 3 product candidates within the endocrinology rare disease area
- Endocrinology rare disease internal pipeline and expected near-term milestones
  - TransCon hGH for pediatric GH deficiency: Phase 3 demonstrated superiority of TransCon hGH
  - TransCon PTH for hypoparathyroidism: Phase 2 top-line data Q4 2019
  - TransCon CNP for achondroplasia: Phase 2 initiation Q3 2019
- Build leading positions for each endocrinology rare disease product with commercial focus on the U.S. and select European markets
  - Partnership with VISEN Pharmaceuticals for commercialization of endocrinology rare disease products in China
- Established high-value collaborations with Roche/Genentech in ophthalmology and Sanofi in diabetes
- As of December 31, 2018, cash and cash equivalents of ~€278 million[1]

[1] Based on exchange rates as of December 31, 2018. This amount is preliminary, has not been audited and is subject to change upon completion of the Company's audited financial statements for the year ended December 31, 2018. Additional information and disclosures would be required for a more complete understanding of the Company's financial position and results of operations as of December 31, 2018. Does not include net proceeds of approximately US$540 million from a follow-on offering completed in March 2019 of the ADSs.

TransCon Technology: Sustained Systemic Delivery

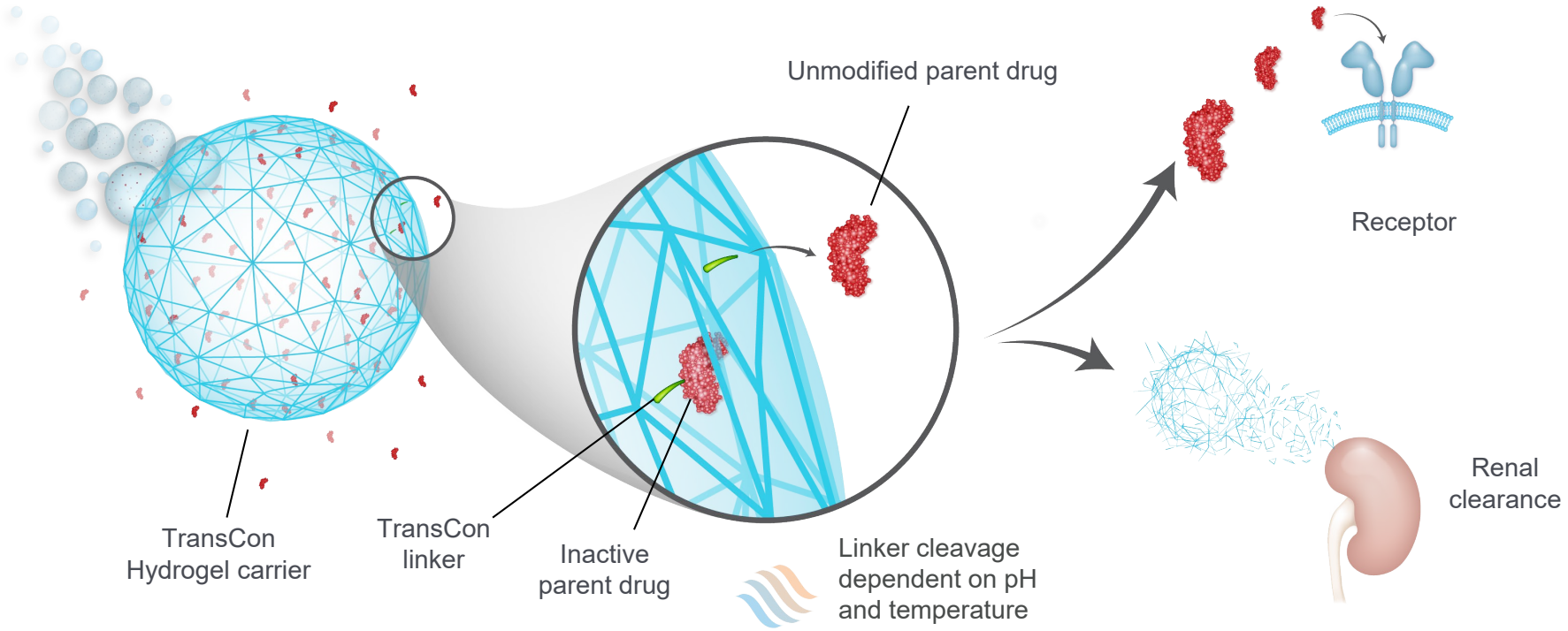


Parent drug is transiently bound to a TransCon linker-soluble carrier moiety, which inactivates and shields parent drug from clearance

Following injection, the linker is designed to autohydrolyze at specific rates to predictably release unmodified parent drug

Designed to distribute parent drug like the endogenous compound; linker-carrier is cleared renally

TransCon Technology: Sustained Localized Delivery



Parent drug is transiently bound to TransCon linker-hydrogel carrier, which inactivates, shields parent drug and prevents clearance

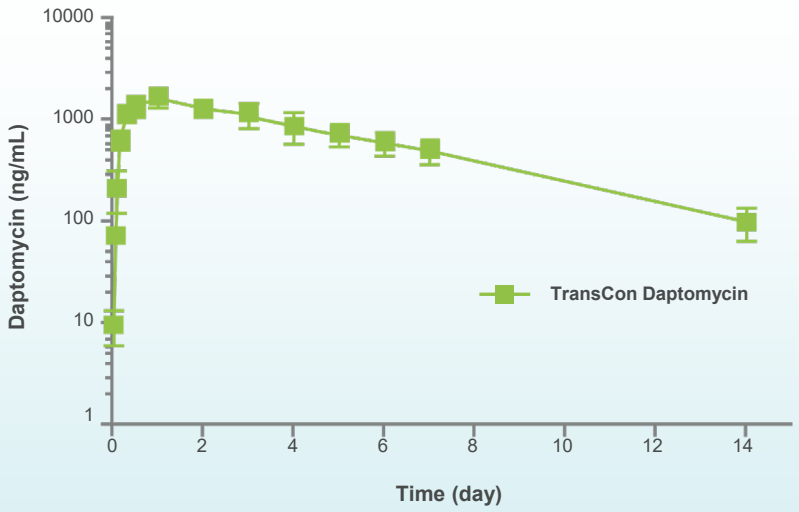
Following injection, the linker is designed to autohydrolyze at specific rates to predictably release unmodified parent drug

Designed to provide sustained high local drug levels with low systemic exposure; hydrogel degrades into small polymers that are renally cleared following drug release

Sustained Localized Delivery: Validated Across Multiple Drugs and Administration Sites

Small Molecules

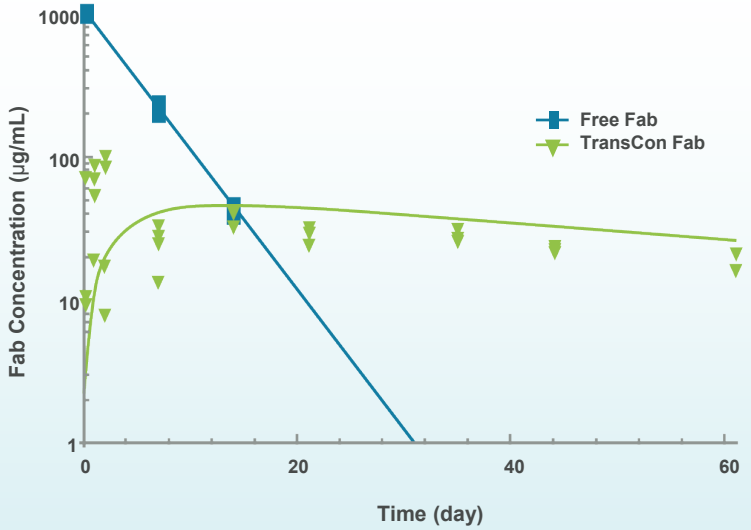
Intra-articular administration



Plasma PK of Daptomycin following intra-articular administration in rabbits. TransCon Daptomycin half-life was ~3 days.

Antibody Fragments (Fab)

Intra-vitreous administration[1]

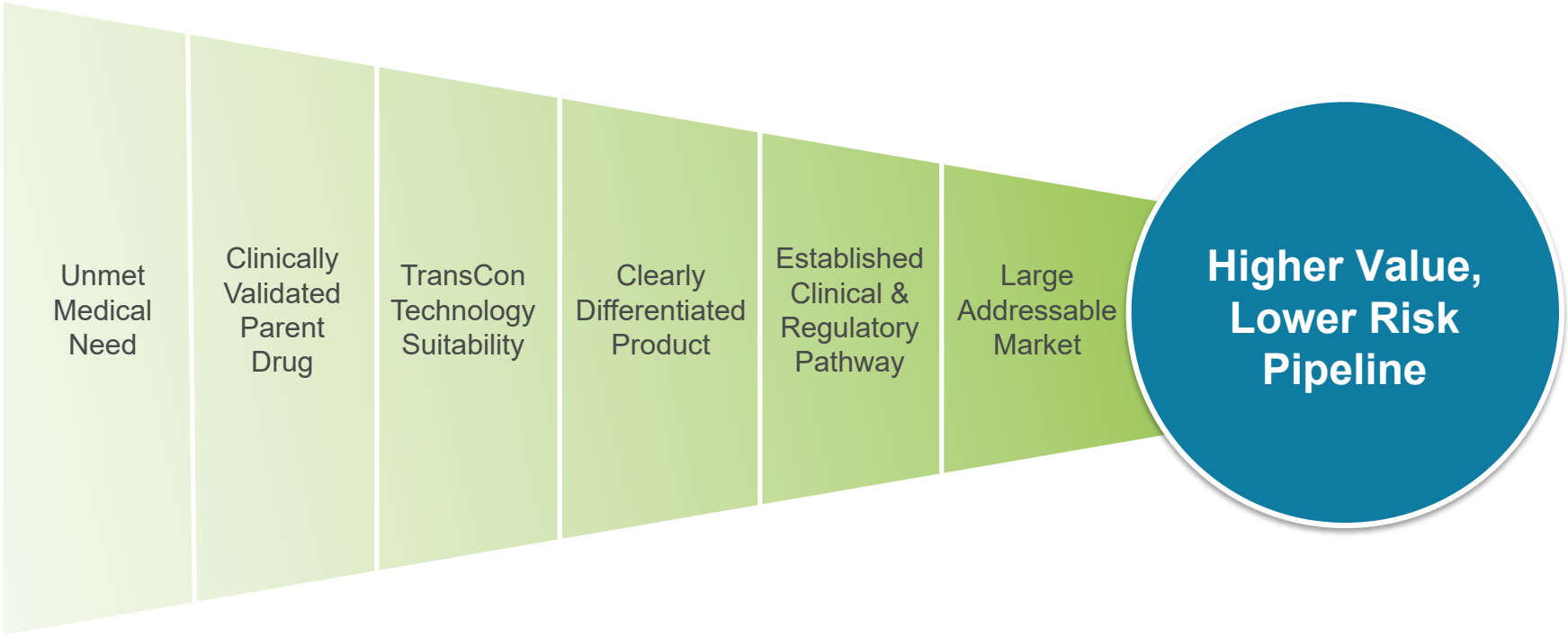


Vitreal PK of Fab following intra-vitreous administration in rabbits. TransCon Fab half-life was ~53 days compared to free Fab half-life of ~3.2 days.

- Excellent local tolerability of TransCon hydrogel carrier
- Sustained high local concentration with low systemic exposure

[1] Patent WO2018175788

Ascendis Algorithm for Product Innovation



Our unique algorithm for product innovation has resulted in clinical validation of 3 out of 3 product candidates in Endocrinology Rare Disease

Building a Leading Company in Rare Diseases

Internal Endocrinology Pipeline

PRODUCT CANDIDATE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	POTENTIAL WW MARKET[1]	COMMERCIAL RIGHTS[2]
TransCon hGH	Pediatric Growth Hormone Deficiency				> $3 billion[3]	ascendis pharma
	Adult Growth Hormone Deficiency					
TransCon PTH	Hypoparathyroidism				> $2 billion[4]	ascendis pharma
TransCon CNP	Achondroplasia				> $1 billion	ascendis pharma

Strategic Collaborations

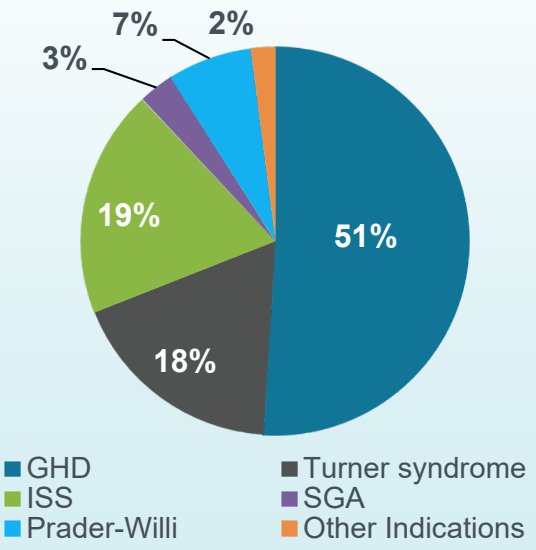
PRODUCT CANDIDATE	PRIMARY INDICATION	DEVELOPMENT STAGE	POTENTIAL WW MARKET[1]	WW COMMERCIAL RIGHTS
TransCon Anti-VEGF	Ophthalmology	Not disclosed	>$7 billion	Genentech
TransCon Peptides	Diabetes	Not disclosed	>$1 billion	SANOFI

[1] Based on market data and Company estimates
[2] Excludes rights granted to VISEN Pharmaceuticals in Greater China
[3] Includes all indications
[4] Based on treatment of ~25% of the U.S. patient population of ~80,000 patients

TransCon Growth Hormone: **Once-Weekly Replacement Therapy**

The Growth Hormone Market[1]

- ~$3.5 billion in worldwide rhGH[2] sales and growing (2.4% CAGR)[3]
- Fragmented market with same undifferentiated hGH molecule competing on differentiated formulations, devices, services and access strategies
- Pediatric indications comprise ~90% of the market
- Indications for growth hormone treatment include:
  - Growth Hormone Deficiency (GHD) ~50% of market
  - Turner syndrome
  - Idiopathic short stature (ISS)
  - Prader-Willi syndrome
  - Small for gestational age (SGA)



Well-established market primed for disruption by a long-acting growth hormone product

[1] Based on company research
[2] Recombinant human growth hormone; subsequently referred to in this presentation as hGH
[3] Compounded Annual Rate of Growth based on 2010-2014 data

Clinical Implications of Untreated GHD

BODY COMPOSITION

Increased fat mass, decreased muscle mass, and decreased bone density have been shown to occur soon after treatment discontinuation.[2,3,4]

CARDIOVASCULAR DISEASE

Early discontinuation of GH treatment may induce impairment of patients' lipid profiles and cardiac structure and performance, leading to increased risk for CV disease.[6,7]

ULTIMATE HEIGHT ACHIEVEMENT

Adults who had child-onset GHD may not achieve full height potential if treatment is stopped prior to adulthood.[1]

MENTAL HEALTH

A high incidence of psychiatric disorders, usually accompanied by poor life quality, is associated with adults who were GHD as children.[5]

FRACTURES

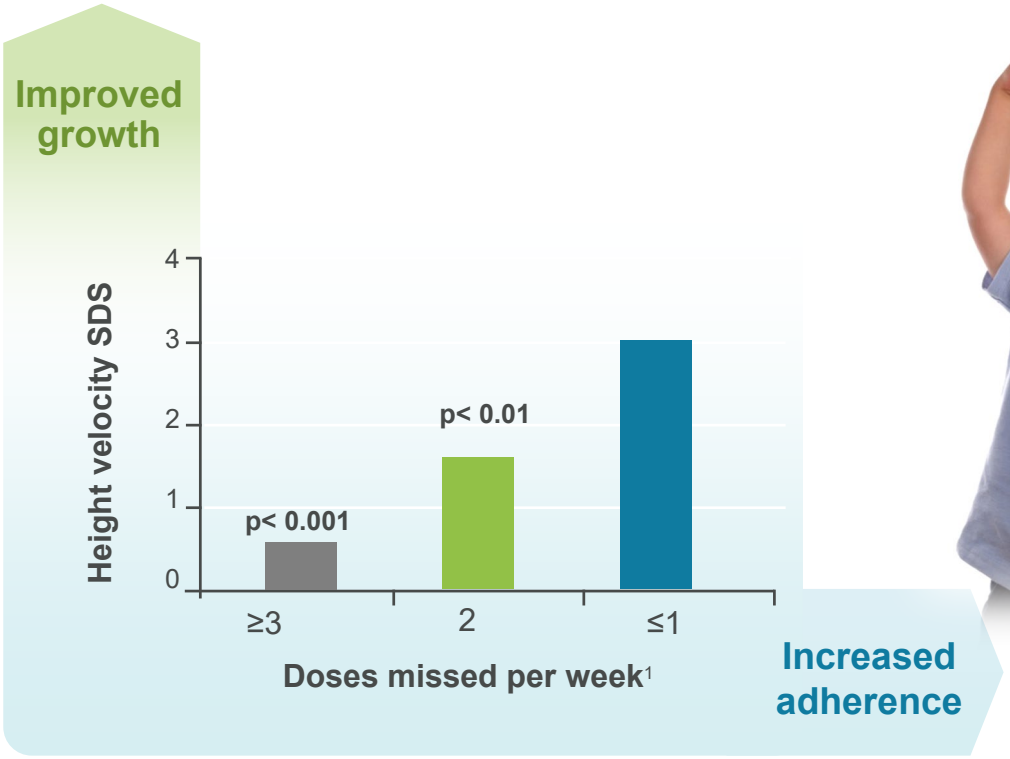
Adult peak bone mass is considerably lower, and rates of fractures are significantly higher among adults with GHD who were not treated as children.[8]

Daily hGH addresses all the symptoms of the disease; long-acting growth hormone products must fully mimic daily hGH to adequately address the totality of the disease

Sources: 1. de Boer, H. et al. 1997; 2. Rutherford, O. M. et al. 1991. 3. Colle, M., J. Auzerie.1993. 4. Johannsson, Gudmundur, et al. 1999.
5. Stabler, Brian et al. 1996. 6. Leong, Gary M., Gudmundur Johannsson. 2003. 7. Colao, Annamaria et al. 2002. 8. Bex, M, and R Bouillon. 2003

Daily Growth Hormone: The Problem

Poor adherence with daily hGH therapy is associated with reduced height velocity and impaired quality of life[1]



Reduced frequency of administration is associated with better adherence[2]

In the 1st year, two of three patients miss >1 injection on average per week[1]

[1] PLoS ONE 2011, 6(1): e16223
[2] Clinical Therapeutics 2008, 30(2): 307-316

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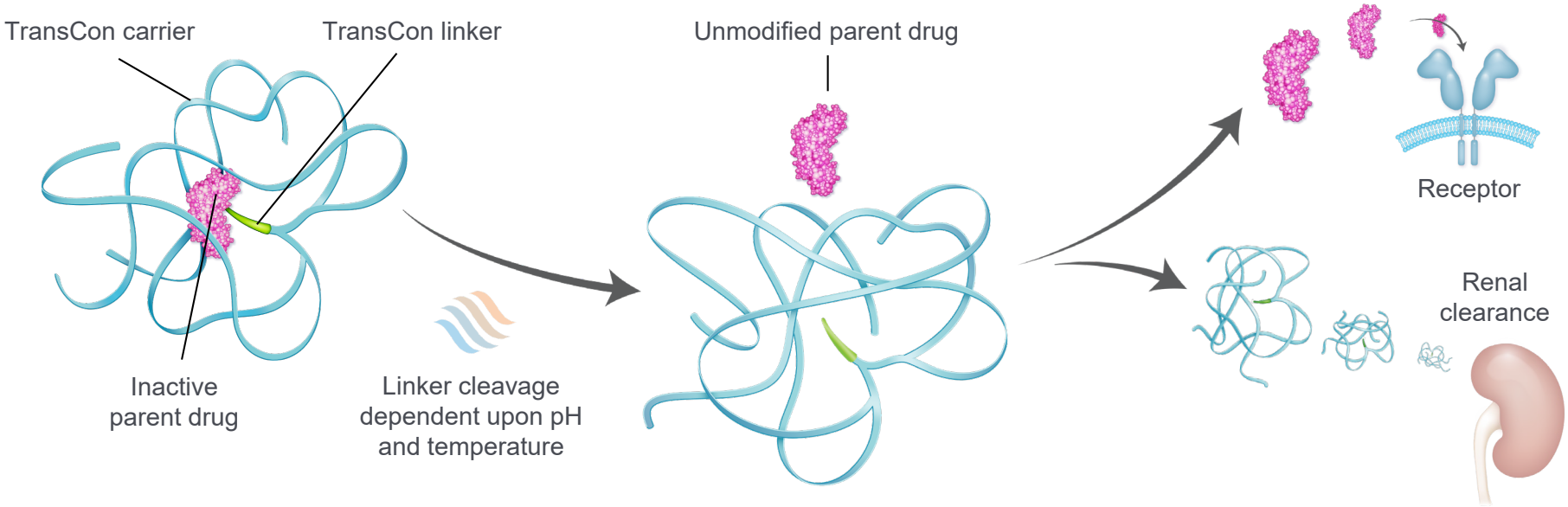
TransCon hGH Design



Once-weekly prodrug releases unmodified hGH designed to mimic daily hGH:

- ✓ Tissue distribution
- ✓ Physiological levels
- ✓ Therapeutic effects: efficacy, safety and tolerability

TransCon Growth Hormone Target Product Profile

- Efficacy
- Safety (including immunogenicity)
- Tolerability

Comparable to Daily Growth Hormone

- Weekly subcutaneous administration
- Small injection volume (31G needle)
- Room temperature storage
- Device
  - Easy to use
  - Automatic data capture
  - Empty-all design

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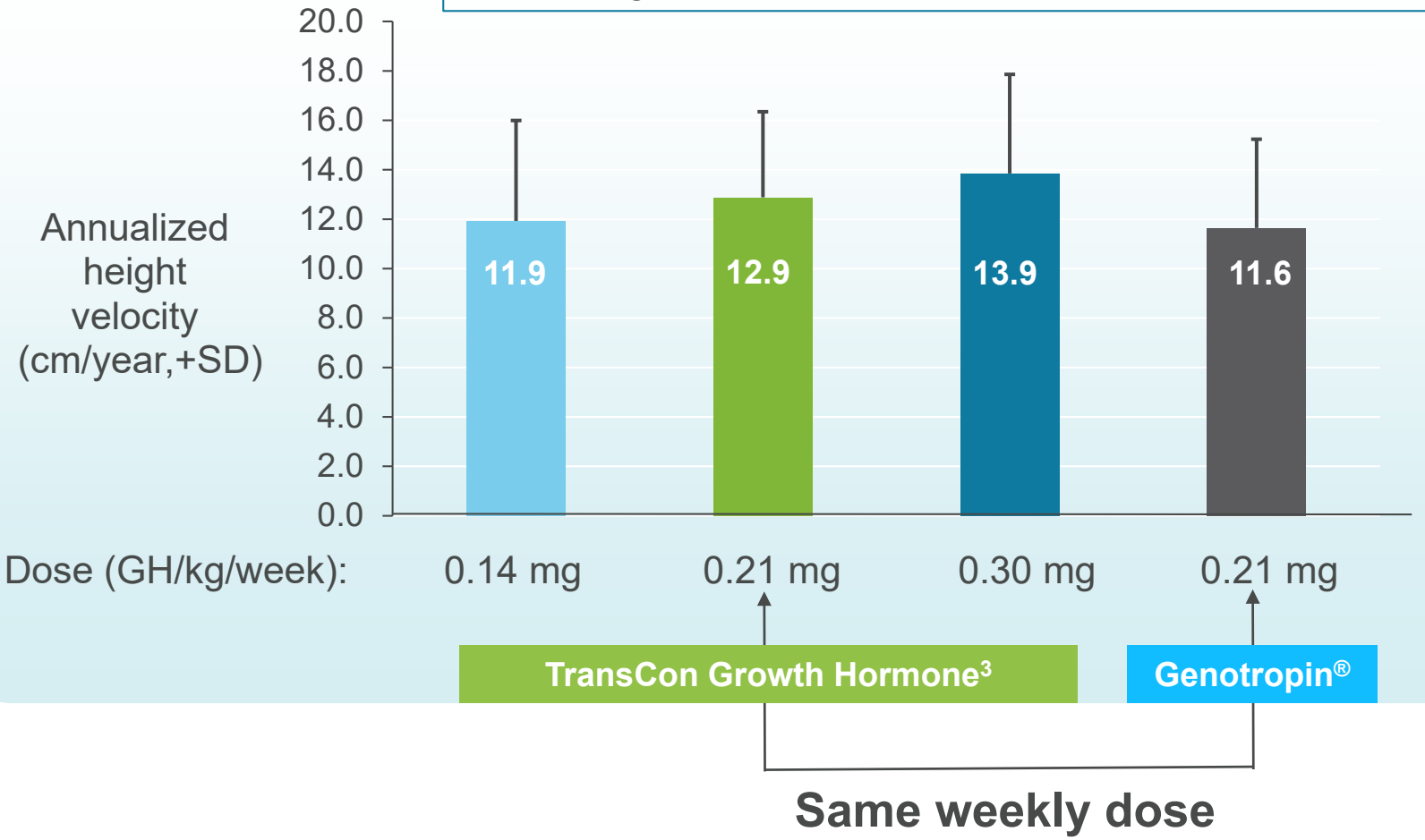
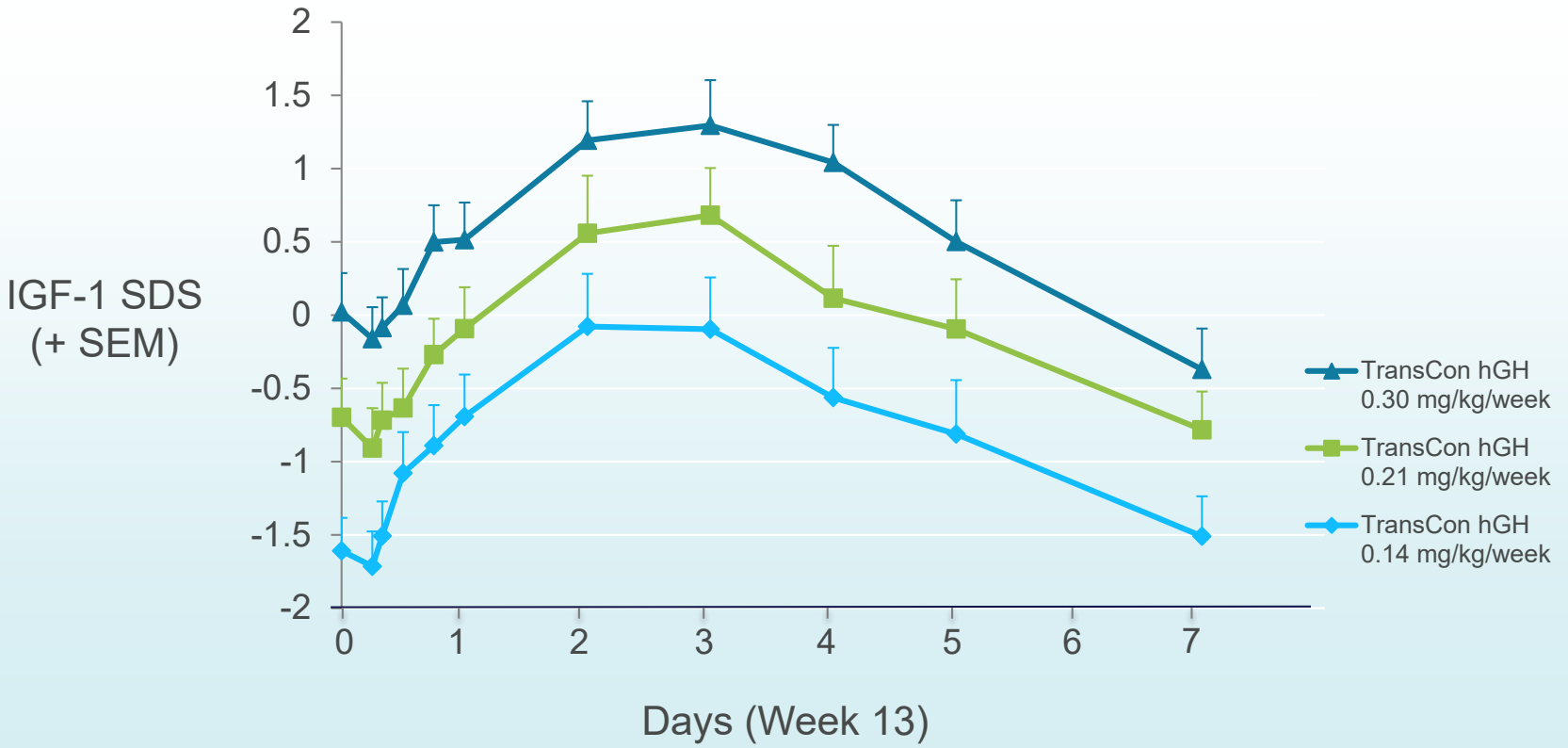
Growth Comparable to a Daily hGH in Phase 2[1,2]

- 26-week treatment period
- Thorough PK/PD assessments at weeks 1 and 13



[1] Intergroup differences not statistically significant
[2] J Clin Endocrinol Metab 2017, 102(5): 1673–1682
[3] Conducted with a previous lower strength version of TransCon Growth Hormone

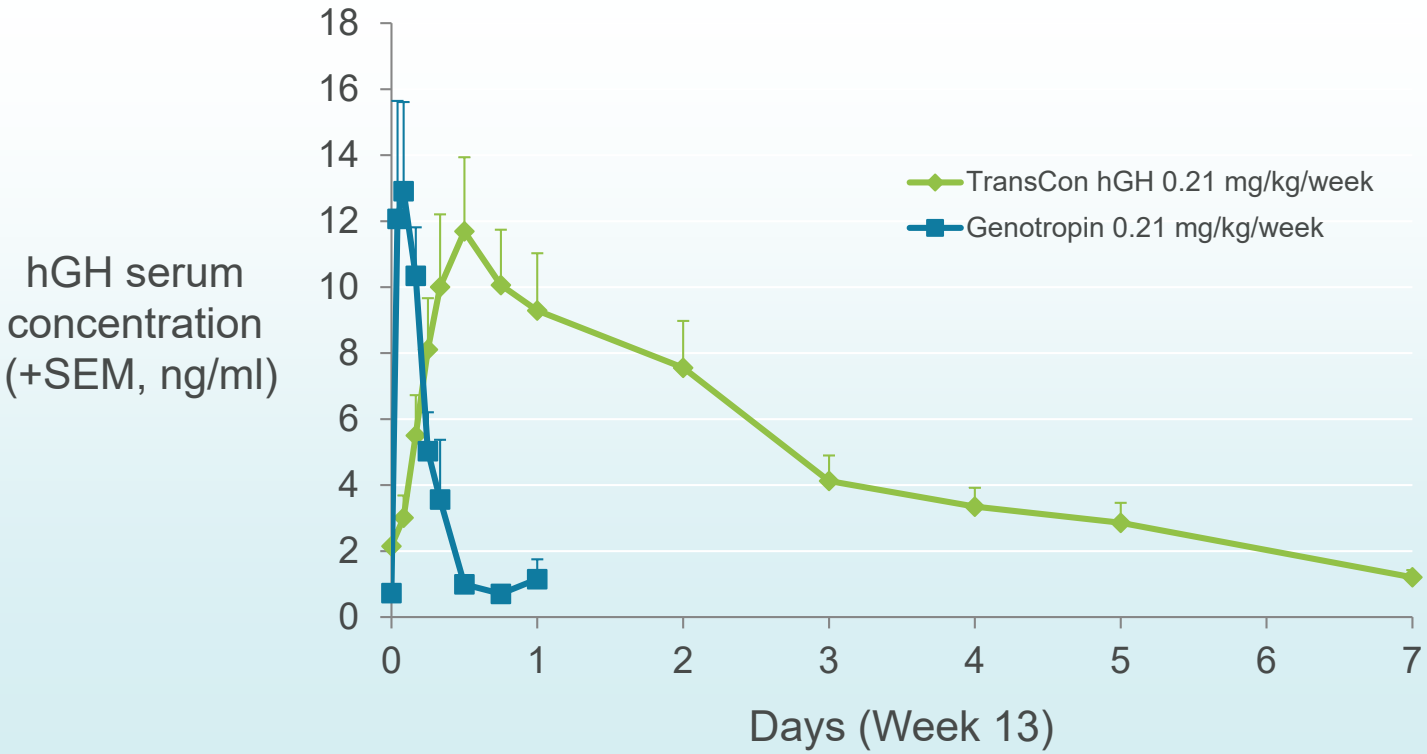
Dose Proportional IGF-1 Response in Phase 2[1]



Transient values greater than +2.0 observed in a small number of patients primarily at the highest dose level

[1] J Clin Endocrinol Metab 2017, 102(5): 1673–1682

Comparable hGH Levels in Phase 2[1]



Maximum hGH concentration comparable between equivalent weekly doses of TransCon hGH and a daily hGH

[1] J Clin Endocrinol Metab 2017, 102(5): 1673–1682

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Comparable Safety to a Daily hGH in Phase 2[1]

No serious adverse events related to study drug

- Adverse events consistent with daily hGH therapy observed and not different between cohorts

Immunogenic profile comparable to a daily hGH

- No occurrence of neutralizing antibodies
- Low incidence of low-titer non-neutralizing antibodies

Injection site tolerability comparable to a daily hGH

- >1100 TransCon hGH injections administered
- No reports of lipoatrophy or nodule formation

[1] J Clin Endocrinol Metab 2017, 102(5): 1673–1682

TransCon hGH Phase 3 Program

- Top-line data reported Q1 2019

- Switch trial (n=146)
- Fully enrolled
- Data expected Q2 2019

Extension trial (n~300)

Regulatory Filings

- FDA and EMA support size and scope of program for pediatric GHD filing
- Database lock for filing package expected Q3 2019

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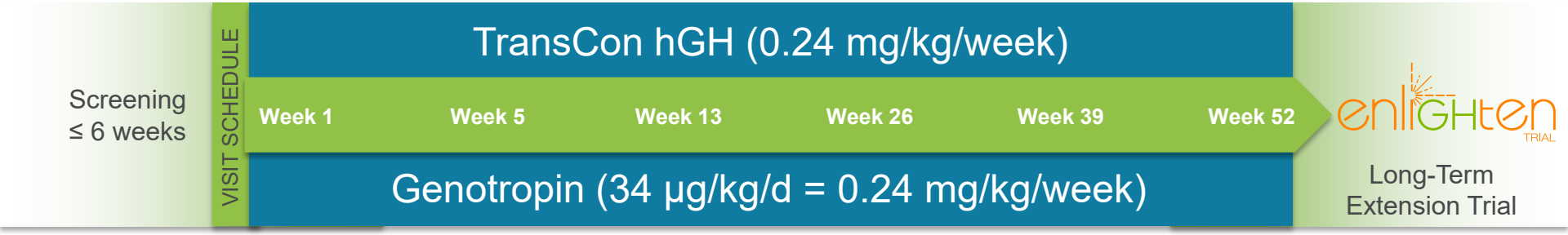
Phase 3 heiGHt Trial

161 treatment-naïve children with GHD dosed (2:1 randomization)



Key Inclusion Criteria

- Prepubertal children with GHD
- Height SDS ≤ -2.0
- IGF-1 SDS ≤-1.0
- 2 GH stimulation tests (GH ≤10 ng/mL)
- Bone age ≥ 6 months behind chronological

Key Endpoints

- Annualized height velocity at 52 weeks (primary endpoint)
- Annualized HV at earlier time points
- Change in HT SDS over 52 weeks
- Change in serum IGF-1/IGFBP-3 levels
- Change in IGF-1 SDS and IGFBP-3 SDS
- Normalization of IGF-1 SDS

Demographics Comparable Between Arms

	TransCon hGH (n=105) Mean	Genotropin (n=56) Mean	Total (n=161) Mean
Age (years)	8.51	8.48	8.50
Bone Age (years)	5.84	5.98	5.88
Height SDS	-2.89	-3.00	-2.93
BMI SDS	-0.32	-0.14	-0.25
Delta Mid-Parental Height SDS	-2.32	-2.55	-2.40
IGF-1 SDS	-2.08	-1.96	-2.04
Peak GH (ng/mL)	5.89	5.48	5.75
White (%, in race)	95	93	94
Male (%)	82	82	82

- Of the 161 subjects, two subjects, one from each arm, withdrew from the trial prior to the final visit

Top-line Analysis: Annualized Height Velocity

Primary Endpoint: Annualized Height Velocity at Week 52[1] (ITT Population)

	TransCon hGH (n=105)	Genotropin (n=56)	Estimate of Treatment Difference	P-value
AHV at Week 52 (cm/year)	11.2	10.3	0.86	0.0088
Standard Error	0.23	0.30	0.33	
95% Confidence Interval	10.7 – 11.6	9.7 – 10.9	0.22 – 1.50	

- TransCon hGH was non-inferior and, additionally, superior to Genotropin®
- Treatment difference reached statistical significance from and including Week 26 onward

[1] Based on least square mean calculation using ANCOVA (analysis of covariance).

Top-line Summary of Adverse Events

Summary of Adverse Events: Safety Population

	TransCon hGH (n=105) n (%)	Genotropin (n=56) n (%)	Total (n=161) n (%)
Treatment-emergent Adverse Events (TEAE)	81 (77.1%)	39 (69.6%)	120 (74.5%)
TEAEs Related to Study Drug	12 (11.4%)	10 (17.9%)	22 (13.7%)
Serious Adverse Events (AEs)	1 (1.0%)	1 (1.8%)	2 (1.2%)
Serious AEs Related to Study Drug	0	0	0
TEAEs Leading to Any Action on Study Drug	2 (1.9%)	1 (1.8%)	3 (1.9%)
TEAEs Leading to Discontinuation of Study Drug	0	0	0

- Adverse events for TransCon hGH consistent with the type and frequency observed with Genotropin
- No serious adverse events related to study drug in either arm
- No treatment-emergent adverse events leading to discontinuation of study drug in either arm

Preliminary Efficacy and Safety Analyses

- Efficacy
  - The incidence of poor responders (AHV < 8.0 cm/year) was 4% and 11% in the TransCon hGH and daily hGH arms, respectively
  - Height standard deviation score (SDS) at 52 weeks increased over baseline by 1.05 for TransCon hGH and by 0.94 for the daily hGH, and the treatment difference in height SDS increased at each visit over 52 weeks
  - Body Mass Index (BMI) SDS was stable over 52 weeks and was -0.03 for TransCon hGH and -0.40 for the daily hGH at week 52
- Safety and tolerability
  - No neutralizing antibodies detected, and low level (<10%) of low-titer non-neutralizing antibodies was similar between the two arms
  - Mean hemoglobin A1c values were generally stable over the course of the trial and remained within the normal range for both arms
  - Two subjects in each treatment arm experienced mild injection site reactions that were considered adverse events

Preliminary Analyses of IGF-1

- Observed peak and trough insulin-like growth factor-1 (IGF-1) SDS values were 1.3 and -0.5 over 52 weeks, respectively, for TransCon hGH compared to an approximate average IGF-1 SDS of 0.0 for the daily hGH at week 52
- In a pre-defined subset of 11 subjects, IGF-1 levels were assessed during week 13 and results were similar to those reported in the TransCon hGH pediatric phase 2 trial
- Consecutive IGF-1 SDS values >2.0 were uncommon (<10% of subjects) and IGF-1 SDS >3.0 were rare (<3% of subjects)
- Adverse events leading to dose reduction (IGF-1 levels or clinical symptoms) occurred twice in the TransCon hGH arm and once in the daily hGH arm

Auto-Injector Designed to Improve Adherence

Key Features

- Simple operation with few user steps
- Single low-volume (<0.60 mL) injection for patients ≤60kg
- Small needle, comparable to daily hGH (31G, 4mm)
- Room temperature storage
- No waste due to empty-all design
- Bluetooth® connectivity enabled for automatic data capture
- Device lifetime at least 4 years

Auto-injector introduction during extension study and for commercial launch

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Integrating with a Connected Healthcare Platform

Automated Data Capture of Dose and Injection Time

Data Analysis

TransCon hGH Auto-Injector

Improve Adherence Through Feedback & Intervention

Secure Cloud-Based Central Database

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TransCon hGH: Highlights

- Potential best-in and first-in-class long-acting hGH in pediatric GHD
- heIGHt TRIAL phase 3 top-line data demonstrated superiority of TransCon hGH
- fliGHt TRIAL top-line data expected Q2 2019
- Introduction of auto-injector into enlIGHten TRIAL Trial expected Q2 2019
- Clinical database lock for filing package expected Q3 2019
- Improving adherence through integrated automatic data capture and connected healthcare system
- Commercial-scale manufacturing and supply chain established
- Multiple patent filings provide potential protection, with auto-injector, into 2038

TransCon PTH:
PTH Replacement Therapy for Hypoparathyroidism

Hypoparathyroidism: Serious Unmet Medical Need

- Parathyroid hormone (PTH) regulates calcium/phosphate homeostasis
- Hypoparathyroidism (HP) is a rare disease characterized by deficient or absent PTH
  - Low serum calcium, increased serum phosphate and urine calcium levels
  - Most common cause (~75%) is inadvertent removal or damage to parathyroid glands during thyroid surgery
  - Approximately 80,000 patients in the U.S.
- HP results in diverse physical, cognitive, and emotional symptoms with high burden on the healthcare system despite current standard of care
  - 79% require hospitalizations or emergency department visits
  - 4-fold increased risk of renal disease (calcifications and renal insufficiency)

Sources: J Bone Miner Res 2013, 28: 2570-2576; J Clin Endocrinol Metab 2012, 97(12): 4507-4514; J Bone Miner Res 2013, 28: 2277-2285

Diverse Clinical Manifestations of HP

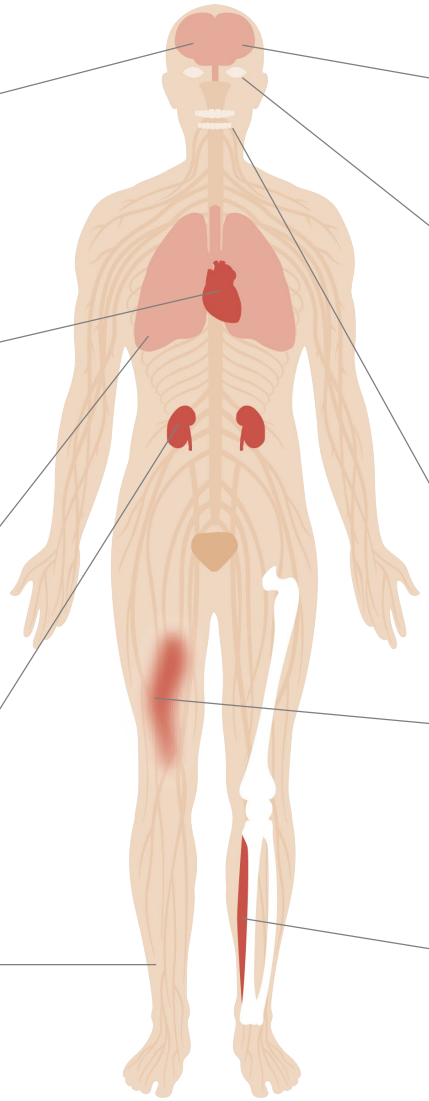
Central nervous system
- Seizures
- Calcifications
- Parkinsonism or dystonia

Cardiovascular system
- Cardiac arrhythmias
- Hypocalcemia-associated dilated cardiomyopathy

Respiratory system
- Laryngospasm

Renal system
- Nephrocalcinosis*
- Kidney stones*
- Chronic kidney disease*

Peripheral nervous system
- Paresthesia
- Muscle cramps
- Tetany



Neuropsychiatric system
- Anxiety and depression

Ophthalmological system
- Cataracts
- Papilloedema

Dental system
- Altered tooth morphology

Dermatological system
- Dry skin
- Onycholysis
- Coarse, thin hair
- Pustular psoriasis

Musculoskeletal system
- Myopathy
- Spondyloarthropathy

Adapted from Nature Reviews 2017, 3: 1-20

* These manifestations are mostly the result of treatment with calcium and activated vitamin D rather than of the disorder itself.

Burden of Illness Despite Treatment: The Patients' View

I am satisfied with the effectiveness of my medications to treat HP

40% strongly disagree

Despite taking my medications as prescribed, I still experience symptoms of HP

58% strongly agree

I am concerned with the long-term complications of my HP medications

75% strongly agree

High burden of illness, despite current standard of care

72% experienced >10 symptoms in preceding 12 months; mean of 13 ± 9 hours/day

85% report inability to perform household activities

20% experienced a disease-related change in employment status

Sources: Endocrine Practice 2014, 20: 671-679; results of Paradox survey of patients (n=374)

Treatment Contributes to Risk of Renal Disease

4.8-fold
increased risk of renal stones

5.0-fold
increased risk of renal insufficiency

4.3-fold
increased risk of renal disease

Vitamin D & calcium treatment associated with hypercalciuria

31% had renal calcifications (renal stones or nephrocalcinosis)

41% had chronic kidney disease stage 3 or worse (renal insufficiency)

Sources: J Bone Miner Res 2013, 28: 2277-2285; J Clin Endocrinol Metab 2012, 97(12): 4507-4514

HP Treatment Strategies Are Evolving

- Conventional treatment by calcium and vitamin D can lead to:[1,2]
  - Hypocalcemia, hypercalcemia and hypercalciuria (short-term)
  - Impaired renal function and extra skeletal calcifications (long-term)
- Once-daily Natpara® /Natpar® has been approved in the U.S. and Europe as an adjunct to calcium and vitamin D
  - Does not fully address all aspects of the disease, including no benefit on clinical episodes of hypocalcemia or hypercalcemia, or effect on 24-hour urine calcium excretion[3]

TransCon PTH is designed to address all aspects of the disease by restoring physiological levels of PTH throughout the day

[1] Front. Endocrinol. 2017, 7: 172
[2] Nature Reviews 2017, 3: 1-20
[3] Natpara/Natpar label

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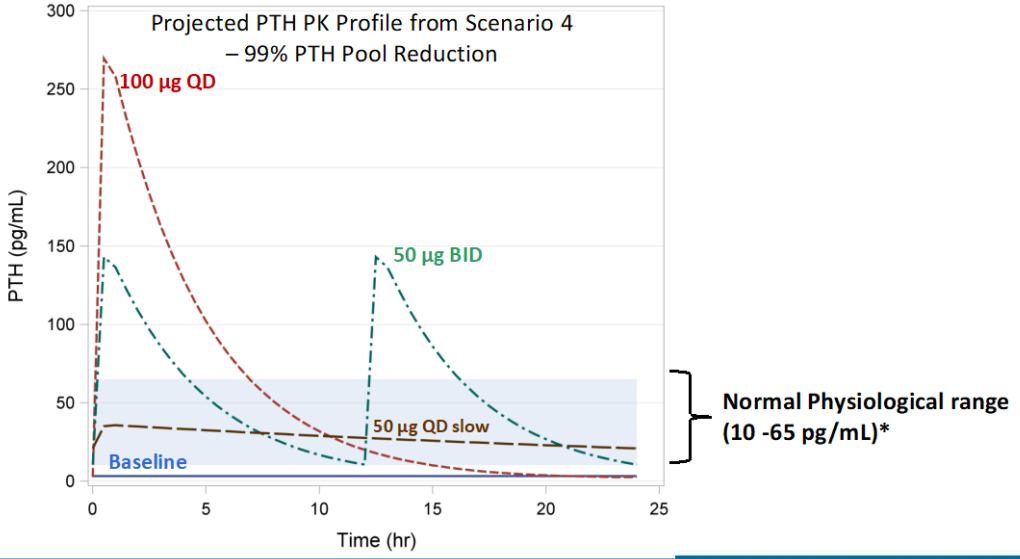
FDA Perspective on Optimal PTH PK Profile[1]

U.S. Food and Drug Administration
Protecting and Promoting Public Health
www.fda.gov

Altering Regimen (QD to BID) or Release Profile Bring PTH Levels Close to the Physiological Levels



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- Natpara QD provides dose-dependent increases in serum calcium for ~24 hours
- Natpara QD effect on urinary calcium excretion is short-lived (10-12 hours) as kidney reabsorption of calcium follows PK profile

[1] FDA presentation: Natpara Advisory Committee, September 12, 2014

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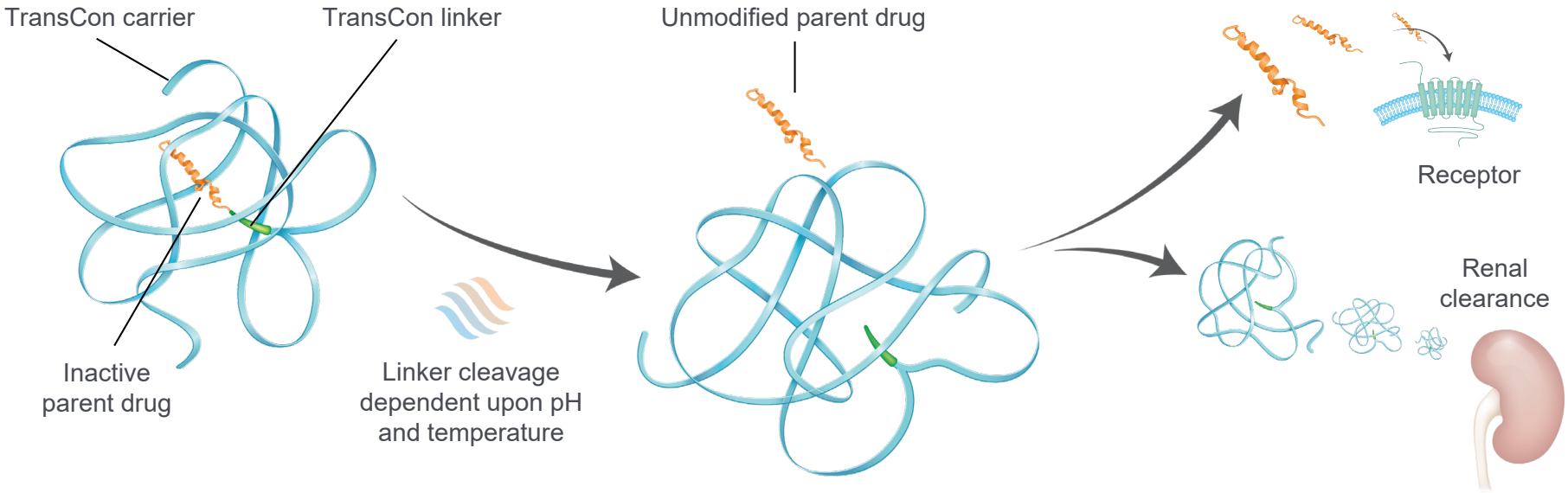
Continuous PTH Infusion Led to Improved Outcomes

Desired Treatment Outcomes in HP	Natpara Once-daily[1, 2]	PTH (1-34) Infusion[3-9]
Increase serum calcium	✓	✓
Reduce pill burden	✓	✓
Normalize urinary calcium excretion	X	✓
Reduce clinical hypercalcemia	X	✓
Reduce clinical hypocalcemia	X	✓
Normalize serum phosphate	✓ (high-normal range)	✓
Normalize bone turnover	X (cortical bone loss)	✓

NIH clinical trials demonstrated superiority of continuous infusion > twice-daily injections > once-daily injections

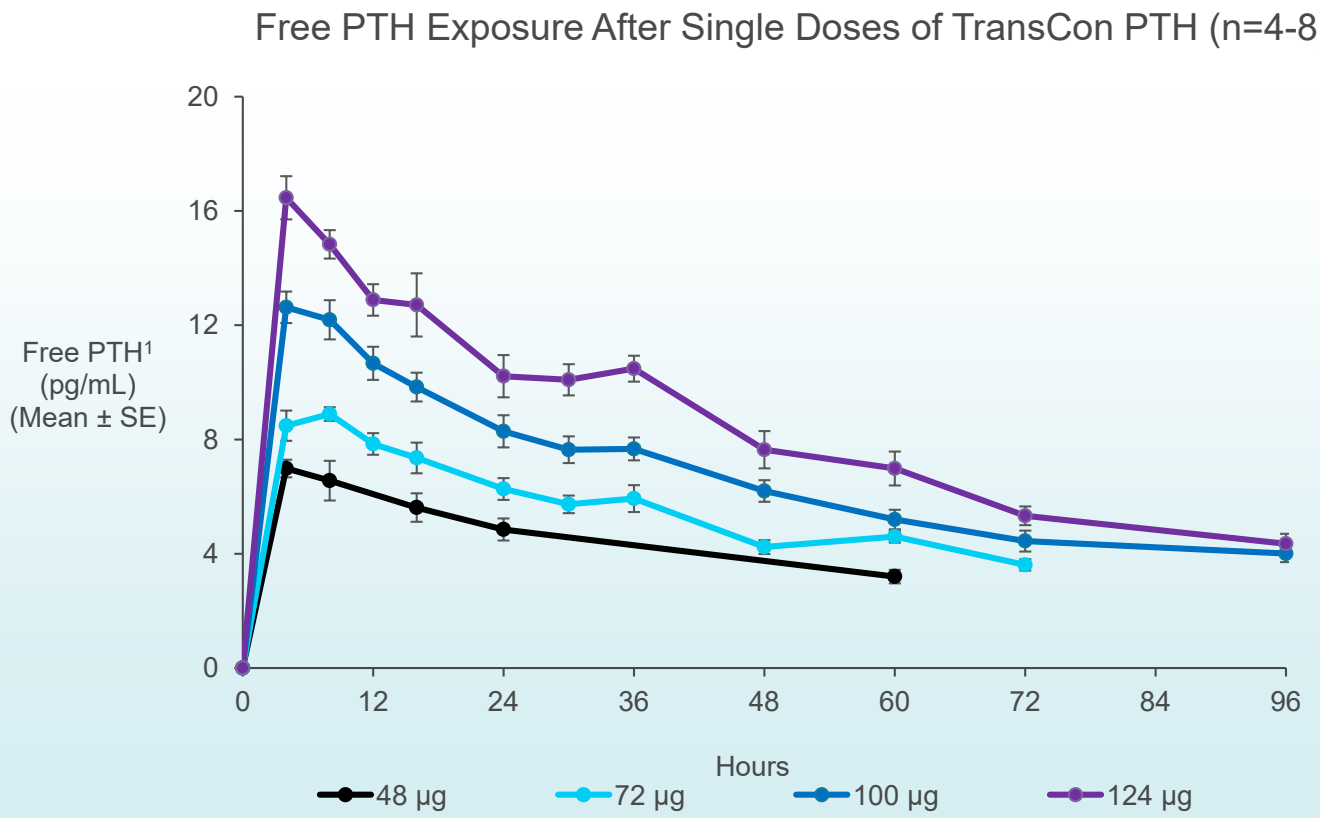
[1] Natpara Product Label
[2] J Clin Endocrinol Metab 2016, 101(7): 2742-2750
[3] JAMA 1996, 276(8): 631-636
[4] J Clin Endocrinol Metab 1998, 83(10): 3480-3486
[5] J Clin Endocrinol Metab 2003, 88(9): 4214-4220
[6] J Clin Endocrinol Metab 2008, 93(9): 3389-3395
[7] J Clin Endocrinol Metab 2011, 96(11): 3308-3312
[8] J Clin Endocrinol Metab 2012, 97(2): 391–399
[9] J Pediatr 2014, 165(3): 556-563

TransCon PTH Design



- TransCon PTH is a sustained-release prodrug designed to provide stable PTH levels in the physiological range for 24 hours/day
- TransCon PTH designed to normalize blood and urinary calcium levels, serum phosphate and bone turnover

Single Dose PK Data Support Infusion-Like Profile with Daily Administration

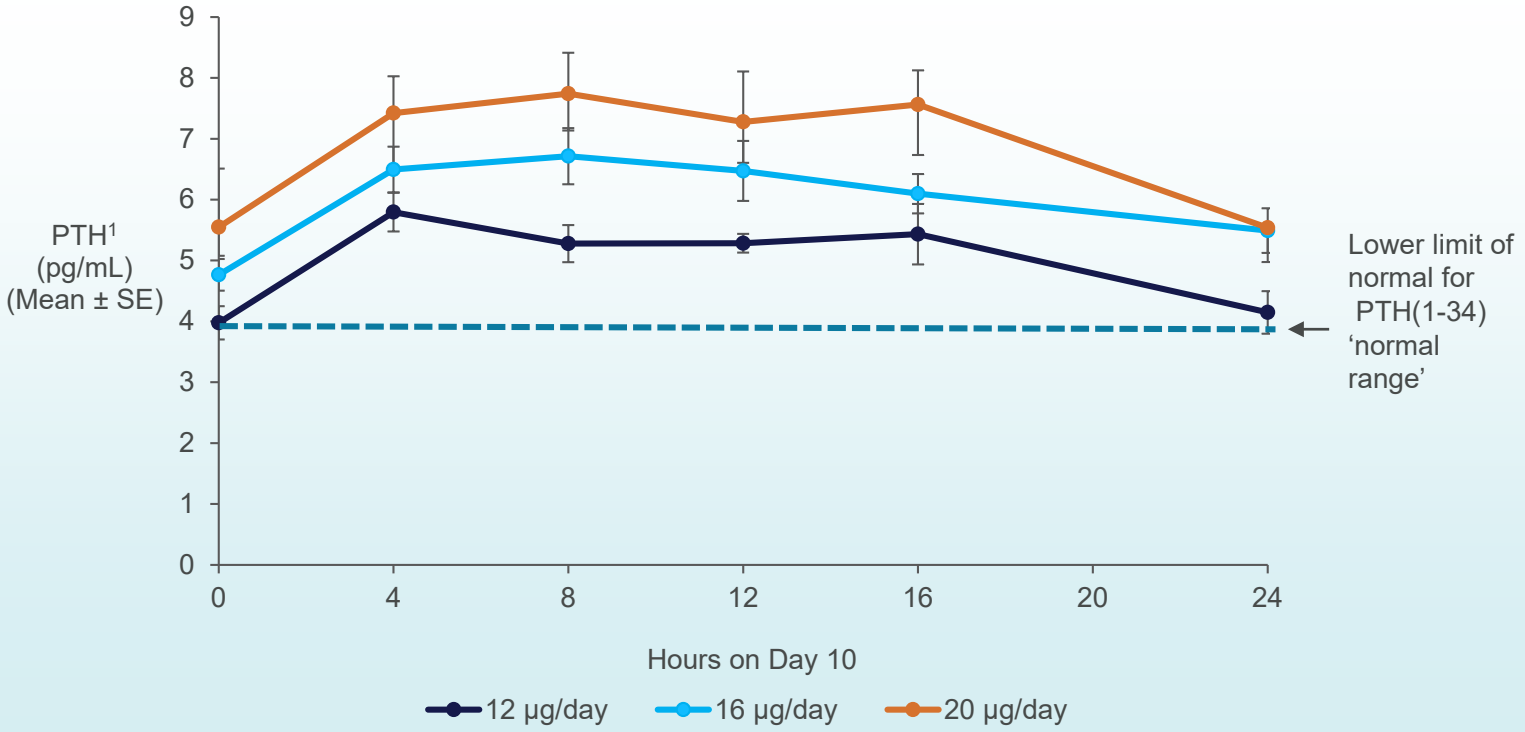


TransCon PTH phase 1 data reproduced PK profile from preclinical studies and showed $t_{1/2}$ of ~60 hours (versus Natpara $t_{1/2}$ ~3 hours)

[1] PTH measured as free PTH(1-34) and PTH(1-33)

PK Data Support Infusion-like Profile over 24 Hours

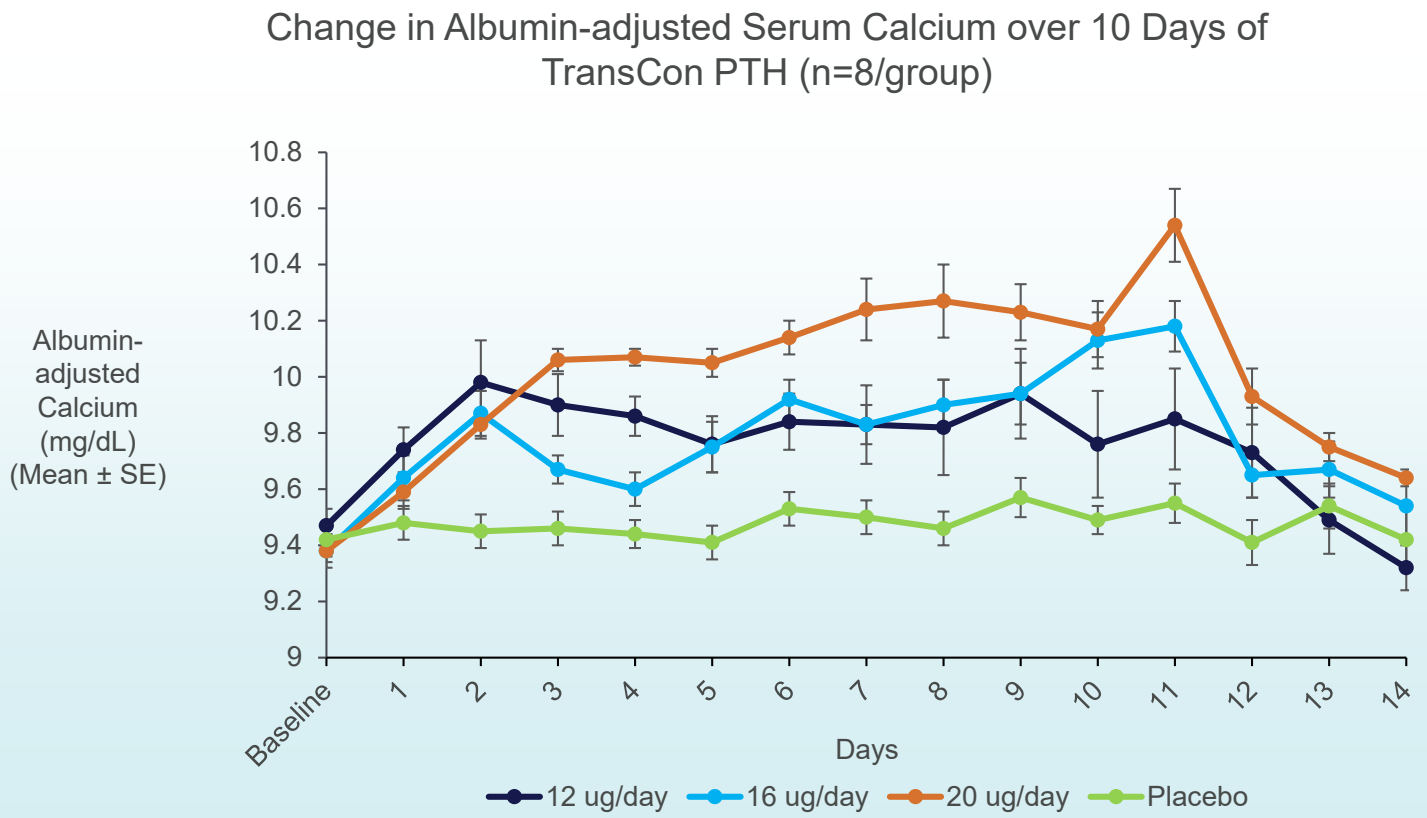
PTH Exposure After Multiple Doses of TransCon PTH (n=7-8)



TransCon PTH daily dosing for 10 days provided a flat infusion-like profile with low PTH peak-to-trough ratio at day 10

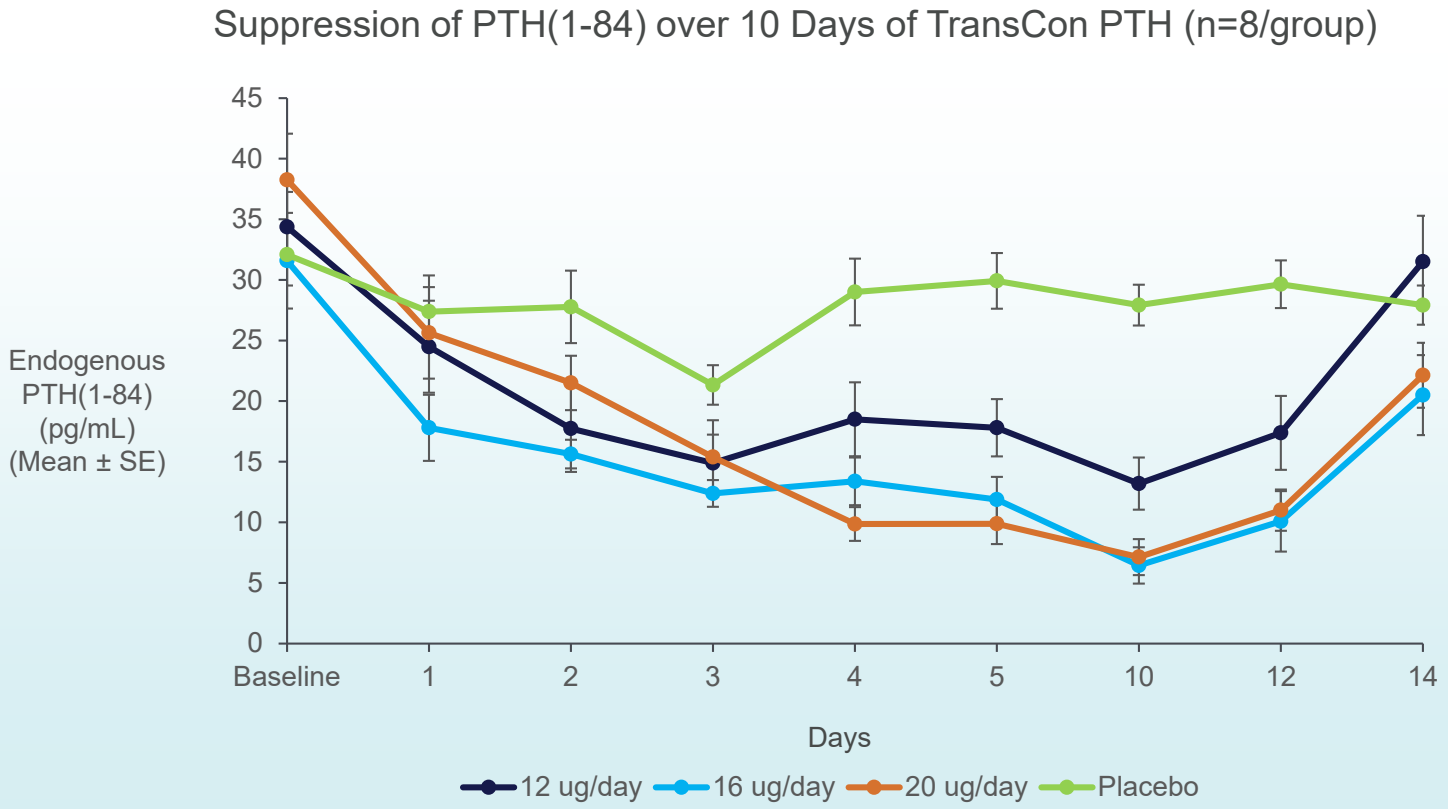
[1] PTH measured as free PTH(1-34) and PTH(1-33)

Multiple Doses Provided Dose-Dependent Increase of Serum Calcium



TransCon PTH daily dosing for 10 days provided dose-dependent increase of serum calcium

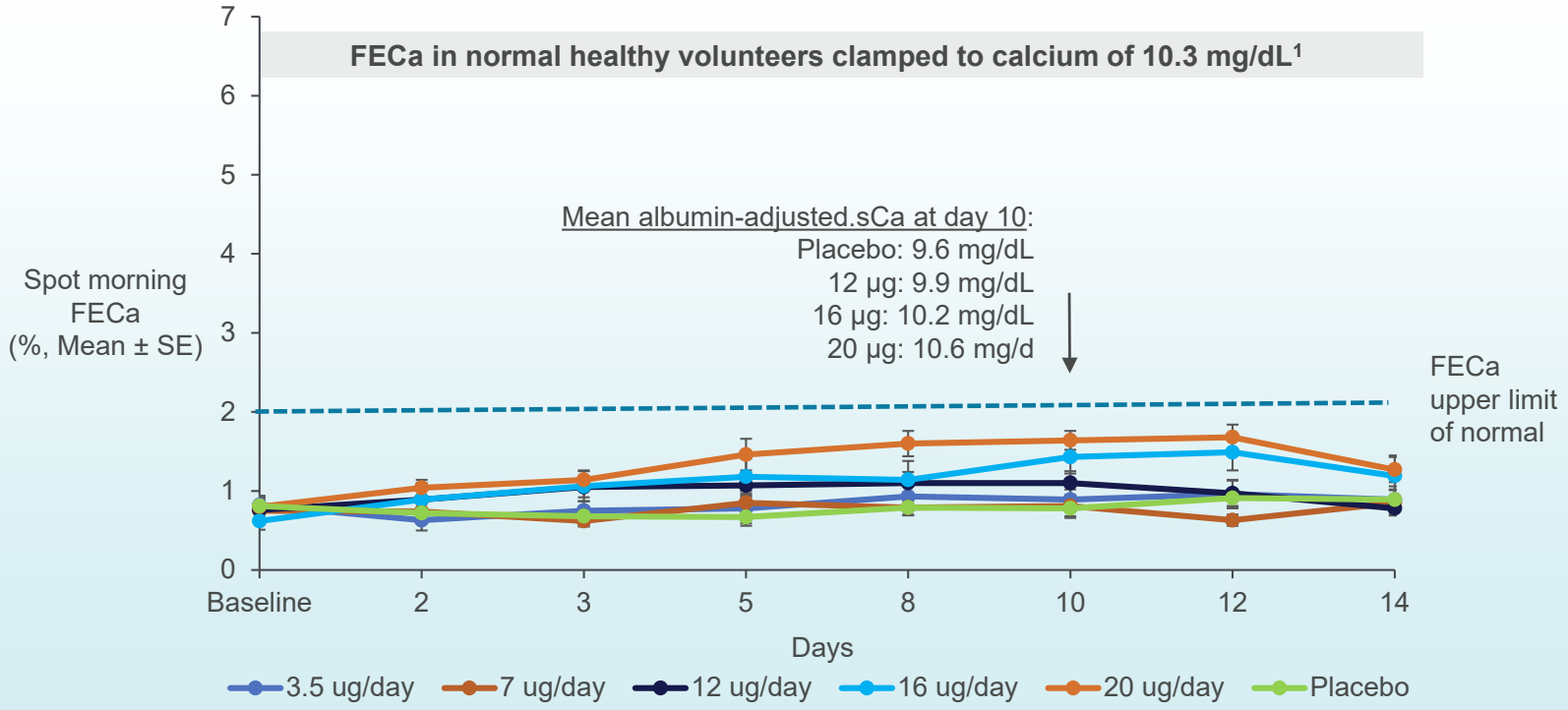
Multiple Doses Provided Dose-Dependent Suppression of Endogenous PTH(1-84)



TransCon PTH daily dosing for 10 days provided dose-dependent down regulation of endogenous PTH(1-84)

Control of Urinary Calcium with Multiple Doses

Spot FECa with daily doses of TransCon PTH (n=8/group) for 10 days



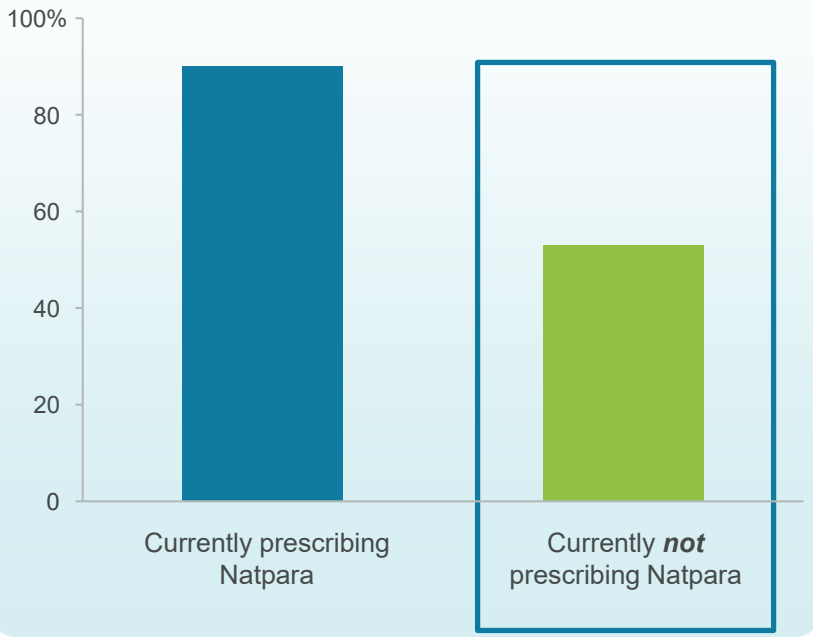
Despite serum Ca at 11 mg/dL, fractional calcium excretion remained normal and below 6.5% range reported for healthy volunteers clamped to serum Ca of 10.3 mg/dL[2], reflecting potent renal Ca reabsorption

[1] J Bone Miner Res 2011, 26(9): 2287–2297
[2] J Clin Endocrinol Metab 2001, 86(4): 1525-1531

Market Research Validates Unmet Medical Need for TransCon PTH

- Market research with 108 U.S. endocrinologists treating HP[1]
  - Variety of clinical settings, geographies, patient volumes, disease severity, and short-acting PTH use
- Endocrinologists' views on current short-acting PTH:
  - Reduces hypocalcemia, improves quality of life, reduces pill burden
  - Remaining unmet medical needs:
    - Providing true replacement therapy
    - Reducing hypercalciuria

Physicians Likely to Prescribe TransCon PTH[2] (N=108)



Confirms TransCon PTH target product profile as a true replacement therapy and reinforces significant unmet need

[1] Ascendis Pharma 2018 HP Survey; interviews conducted in Q2 2018; data on file
[2] Respondents who selected 5-7 on 1-7 scale considered "likely to prescribe" TransCon PTH

TransCon PTH: Highlights

- Phase 1 data support TransCon PTH as a true replacement therapy for HP
- Phase 2 trial initiated in adult HP subjects
  - Randomized placebo-controlled study for approximately four weeks with fixed TransCon PTH doses and titration regimen for complete withdrawal of SoC (i.e., active vitamin D and calcium supplements)
  - Validation of disease-specific patient-reported outcomes for use in phase 3 trial
  - Introduction of ready-to-use prefilled pen device in the phase 2 trial
  - Subjects from phase 2 trial expected to enter into a long-term extension trial
- Phase 2 top-line data expected Q4 2019
- Global regulatory discussions to incorporate Asian territories into phase 3 trial
- Multiple patent concepts provide potential protection into 2037

TransCon CNP:
Once-Weekly CNP for Achondroplasia

Achondroplasia – Not Only a Skeletal Disease

Autosomal dominant genetic disorder

- Most common form of human dwarfism
- Approximately 250,000 patients worldwide[1]
- 80% born to average-sized parents

Patients suffer numerous comorbidities

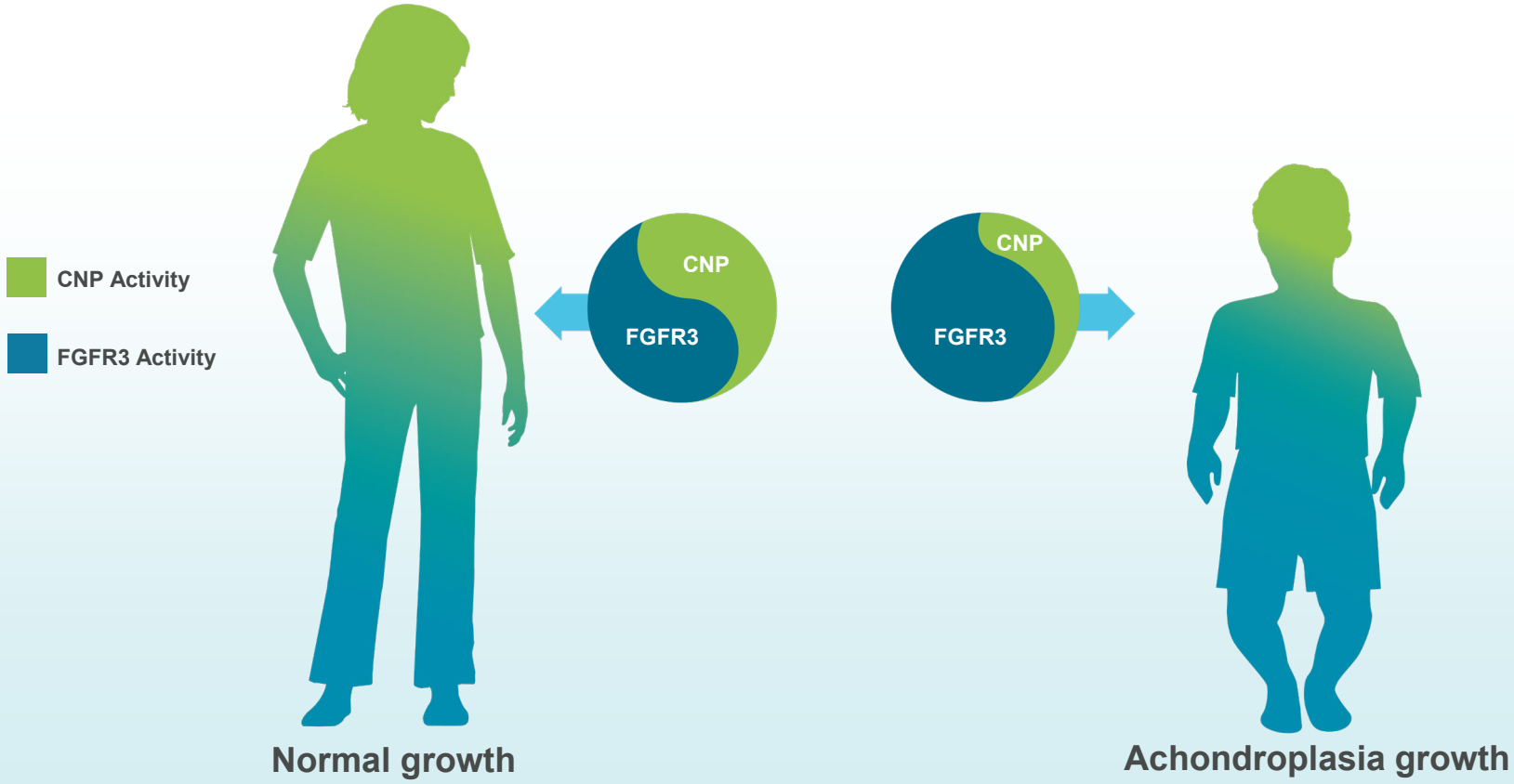
- Back/spine/cord compression
- Cardiovascular complications
- Dental complications
- Ear infections/sleep apnea
- Obesity
- Bowed legs

No FDA-approved therapy

- Only option to improve height is surgical limb lengthening

[1] Lancet 2007, 370: 162-172

Balanced Growth Depends on Balanced Pathways

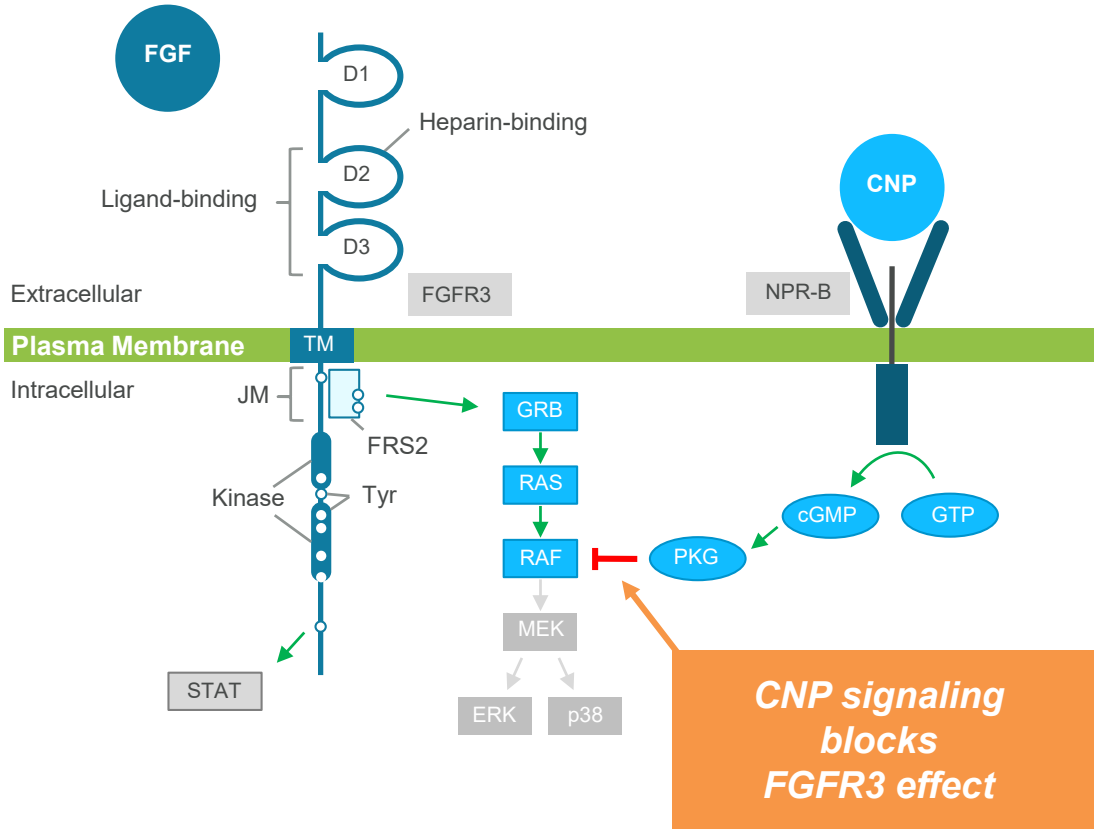


TransCon CNP is designed to provide continuous exposure to CNP to optimize efficacy with a well-tolerated and convenient once-weekly dose

Achondroplasia Signaling Defect is Well Understood

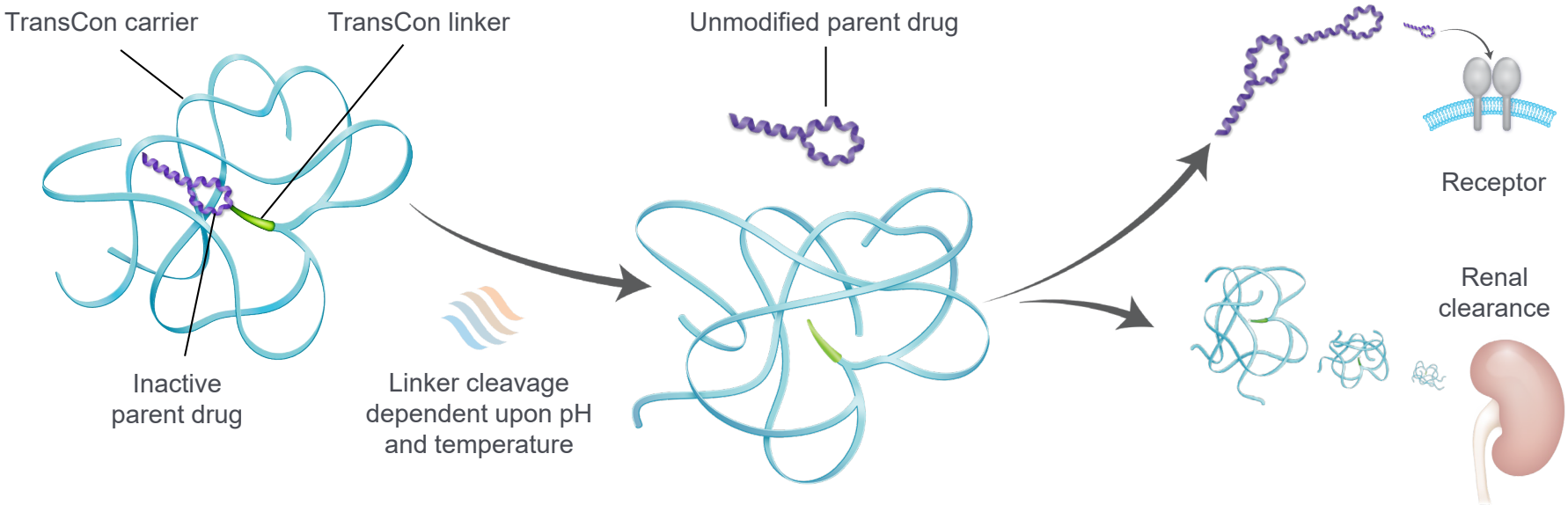
- **FGFR3 negatively regulates chondrocyte proliferation and differentiation and hence bone growth**
- **Achondroplasia results from a mutation in FGFR3 which leaves the receptor constitutively activated**
- **CNP inhibits the FGFR3 pathway and thereby promotes proliferation and differentiation of chondrocytes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

FGFR3 Signaling Pathway[1]



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

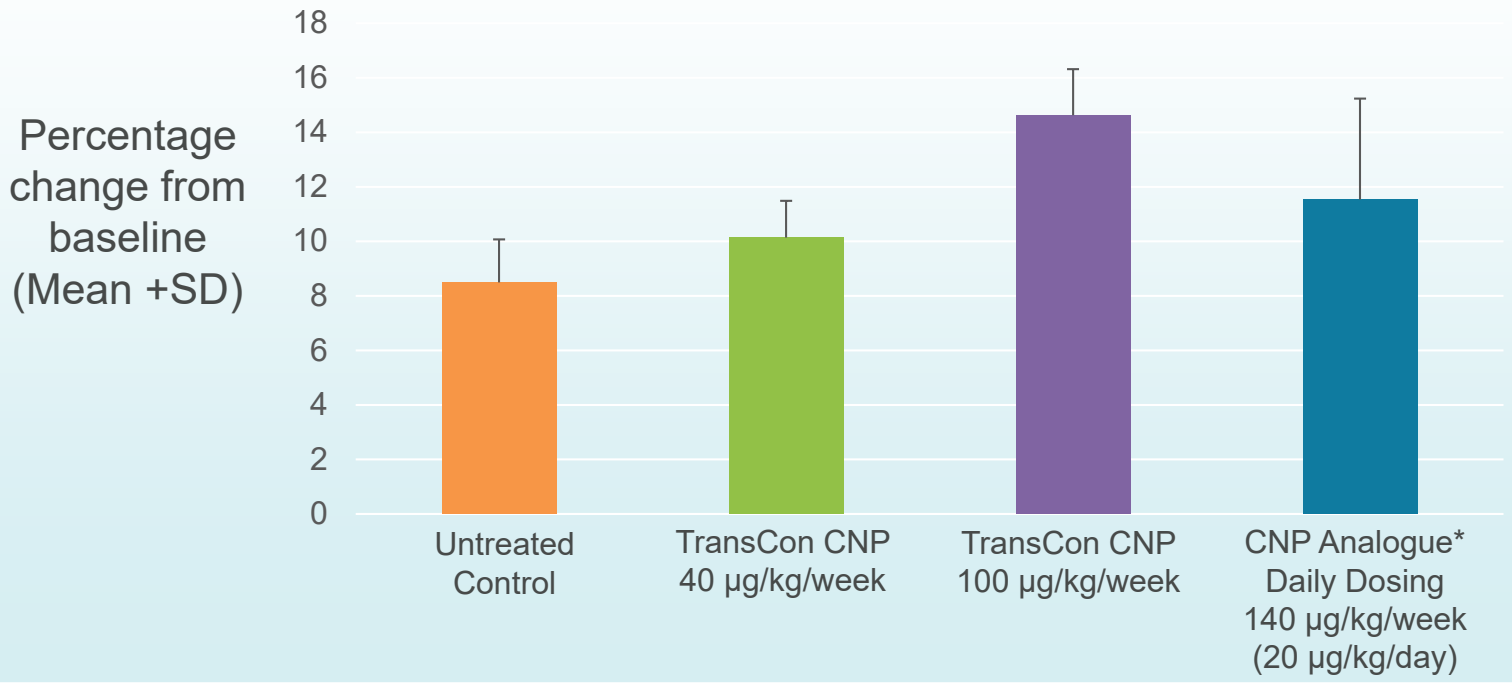
TransCon CNP Design



- 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

Juvenile Healthy Monkey Growth Study

Tibial growth at 6 months (n=4/group)[1]



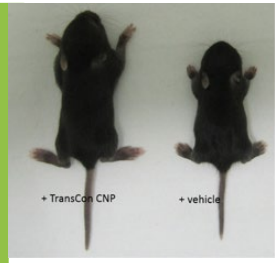
- Demonstrated dose-proportional tibial linear growth; ulnar growth consistent
- Compared to untreated control, growth increased >70% with highest TransCon CNP dose vs. 35% with CNP analogue* at a higher weekly dose

[1] Poster presented at ENDO 2017 and company data
* Refers to a synthesized molecule with a half-life of ~20 mins prepared by Ascendis Pharma

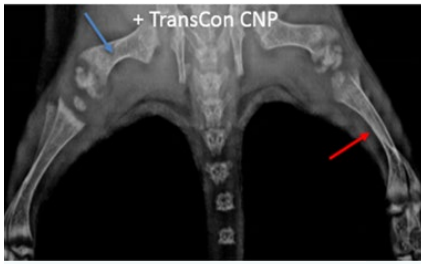
TransCon CNP in Achondroplasia Disease Model ($Fgfr3^{Y367C/+}$)[1]

Linear and Skeletal Growth in Achondroplasia Mice

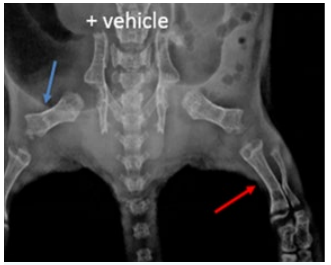
TransCon CNP reversed the phenotype, restoring growth



TransCon CNP/ Vehicle



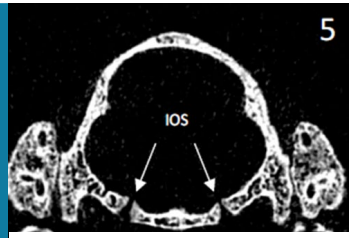
TransCon CNP

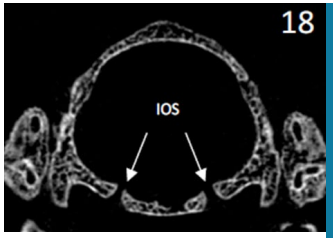


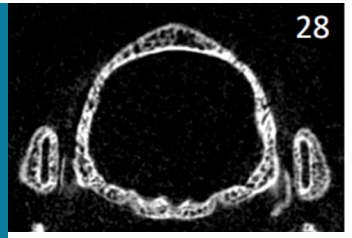
Vehicle

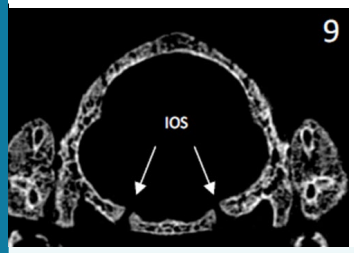
Preventing Premature Fusion of Synchondroses of Foramen Magnum

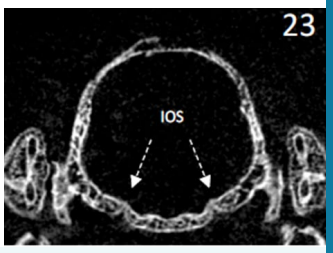
TransCon CNP may ameliorate most disabling achondroplasia traits, including stenosis of the foramen magnum

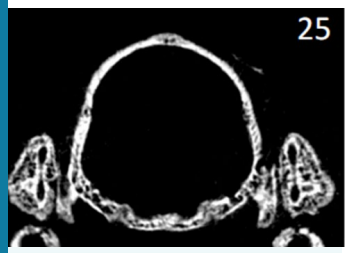












TransCon CNP

Vehicle

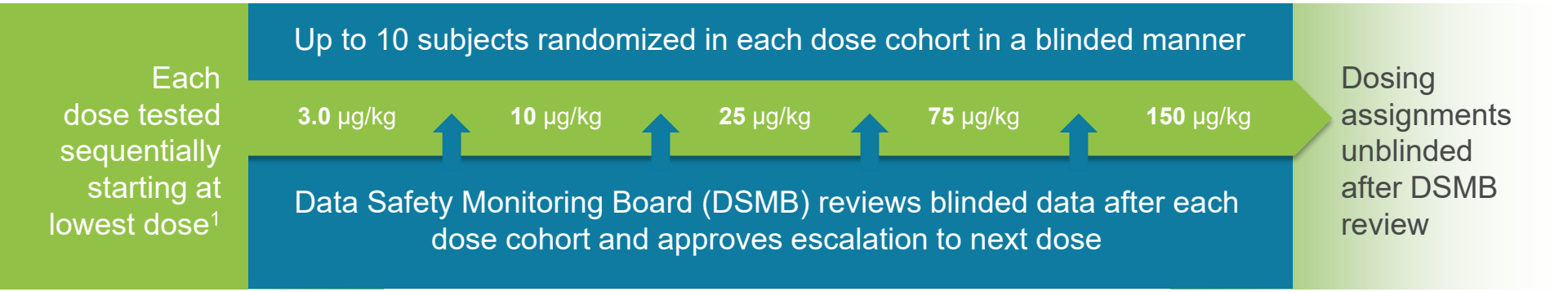
[1] Poster presented at ENDO 2017

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

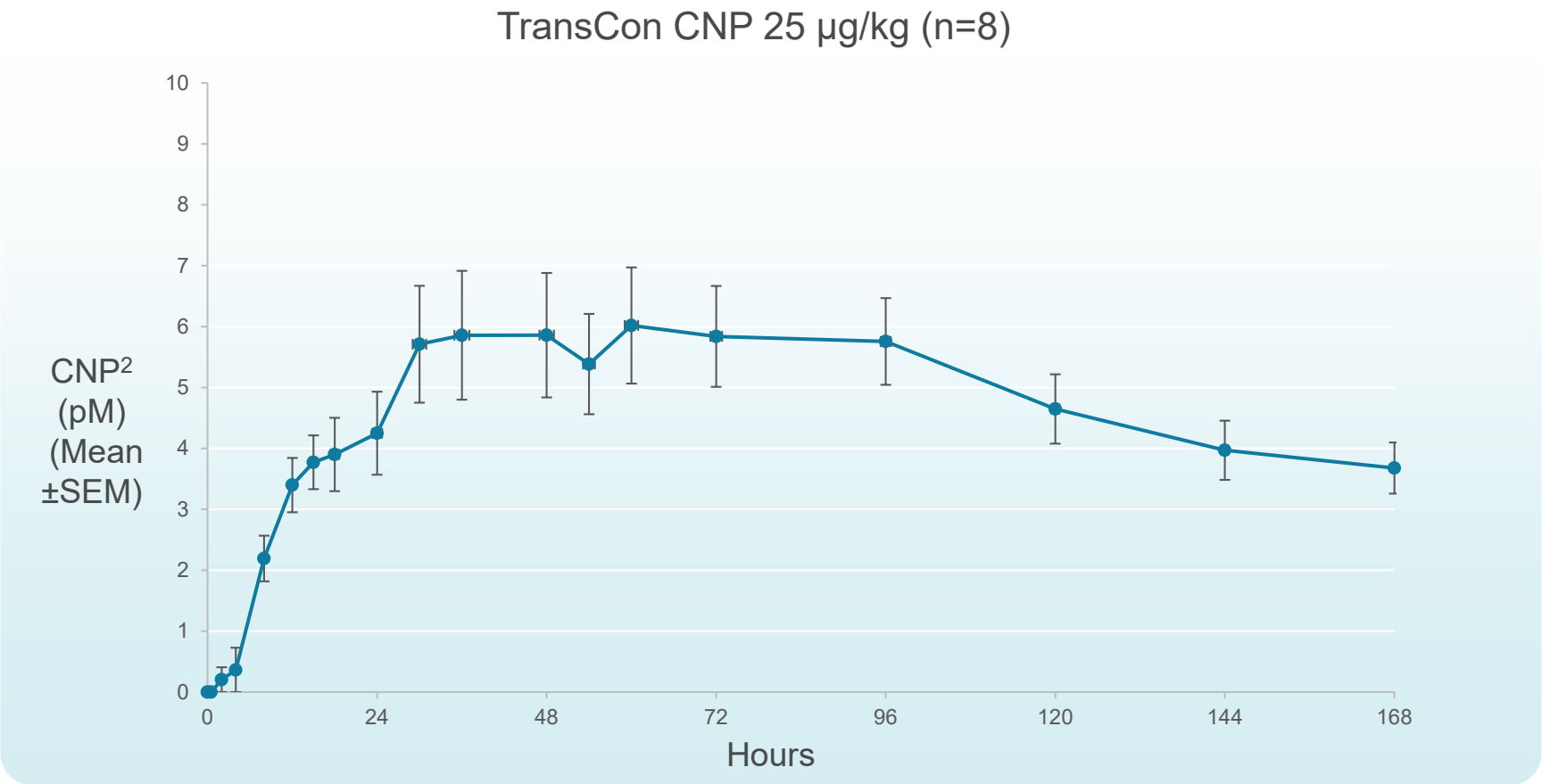
- Frequency of adverse events (AEs) reported after administration of TransCon CNP

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[1]

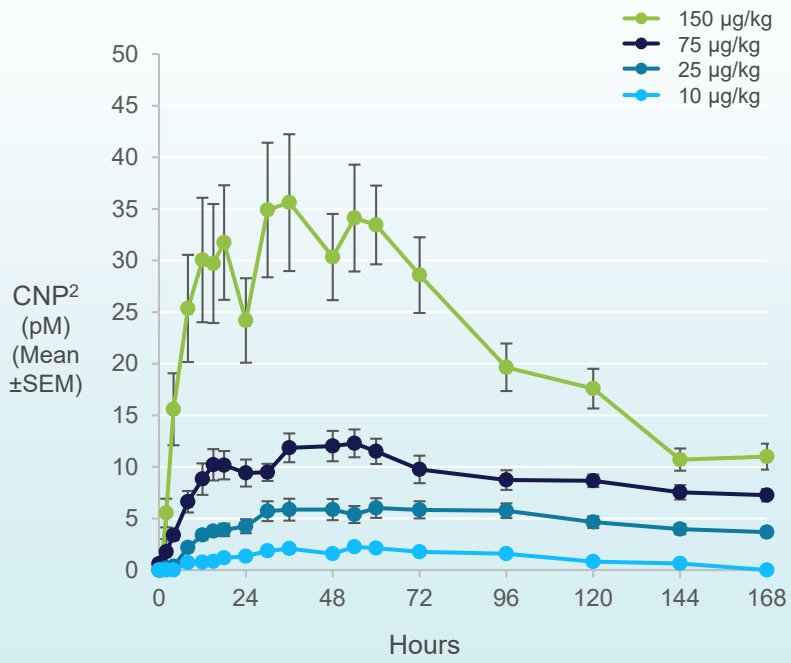


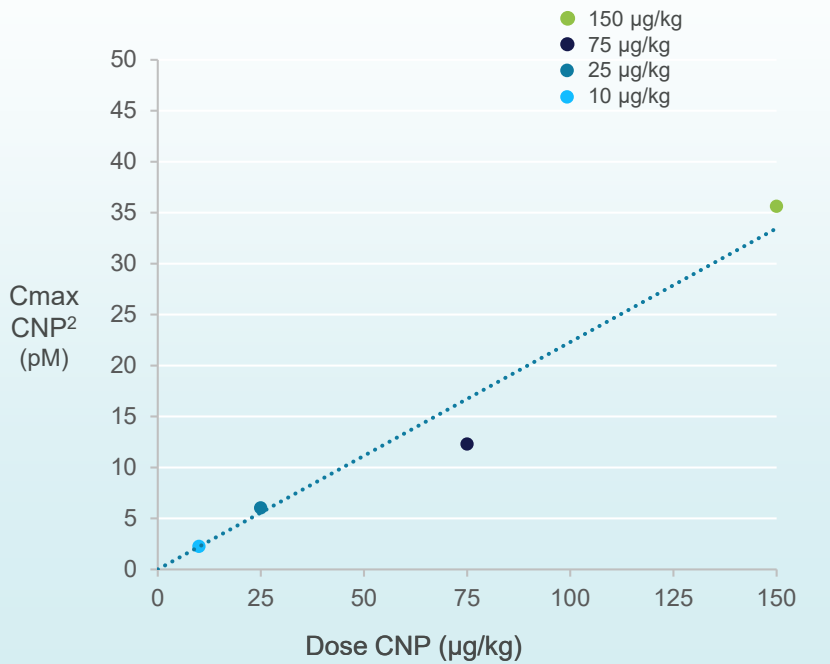
A single dose of TransCon CNP provided continuous CNP exposure over the entire week with low inter-subject variability

[1] Preliminary data
[2] CNP measured as CNP-38

Dose-related Increase in CNP Exposure[1]

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

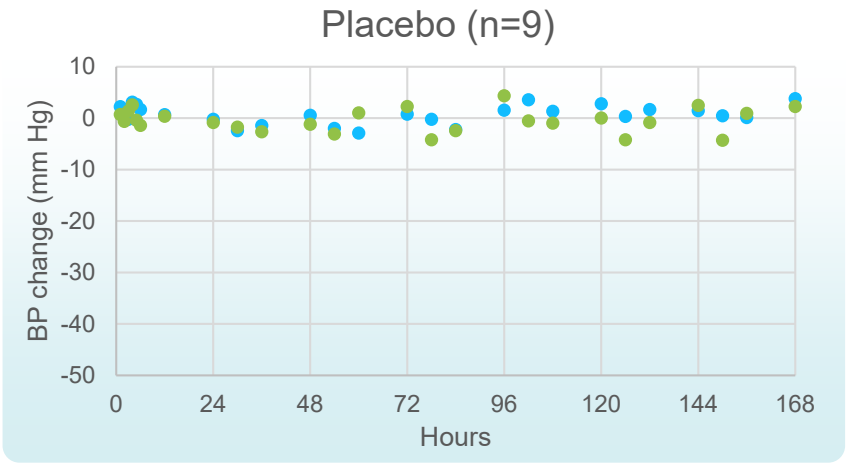


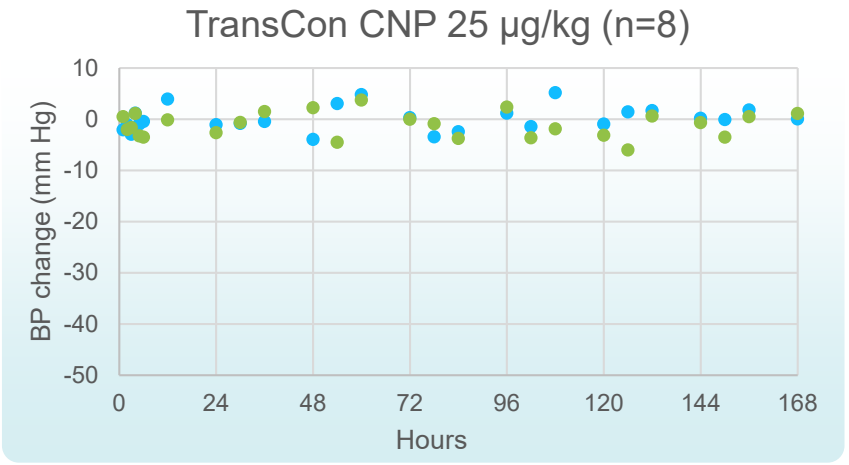


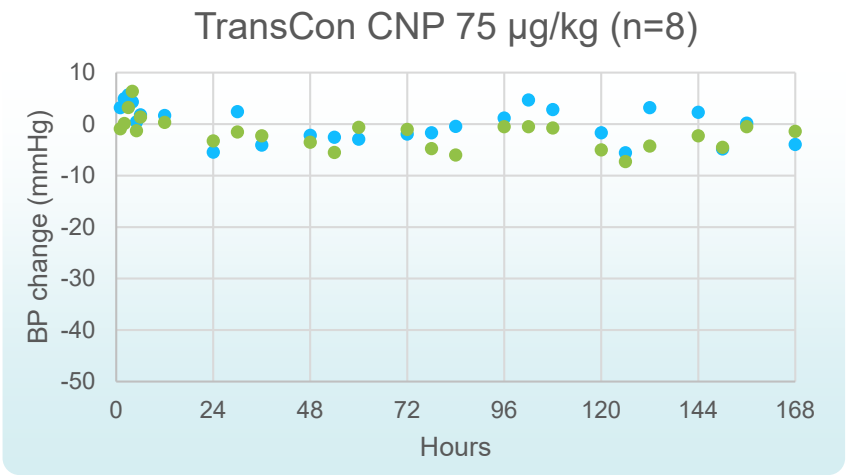
- Dose-related increase in CNP exposure suggests ability to titrate dosing
- Phase 1 showed effective CNP $t_{1/2}$ of ~ 90 hours (native CNP $t_{1/2}$ of 2-3 minutes)

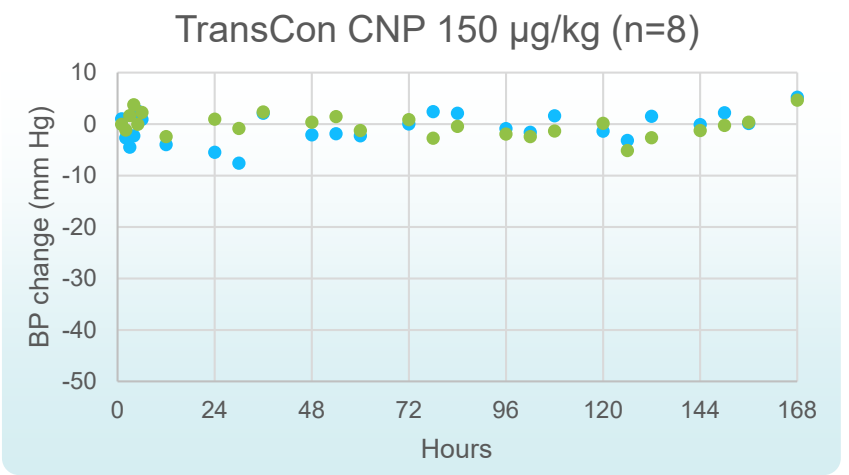
[1] Preliminary data
[2] CNP measured as CNP-38.

Mean Resting Blood Pressure Unchanged from Predose[1]



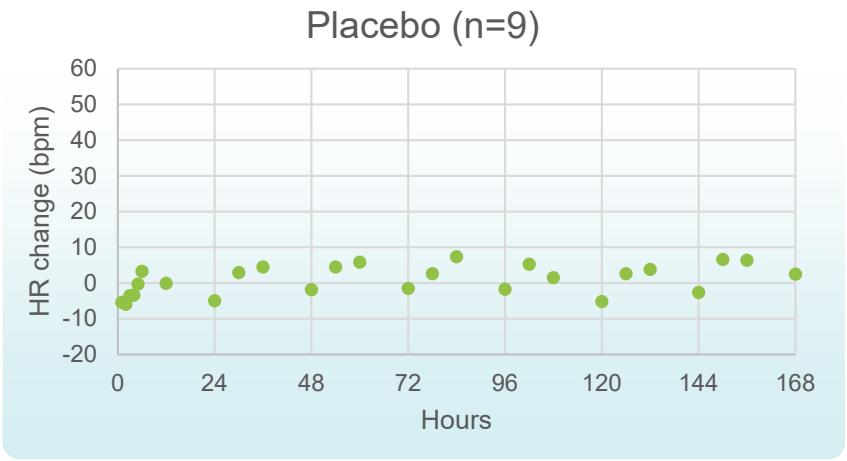


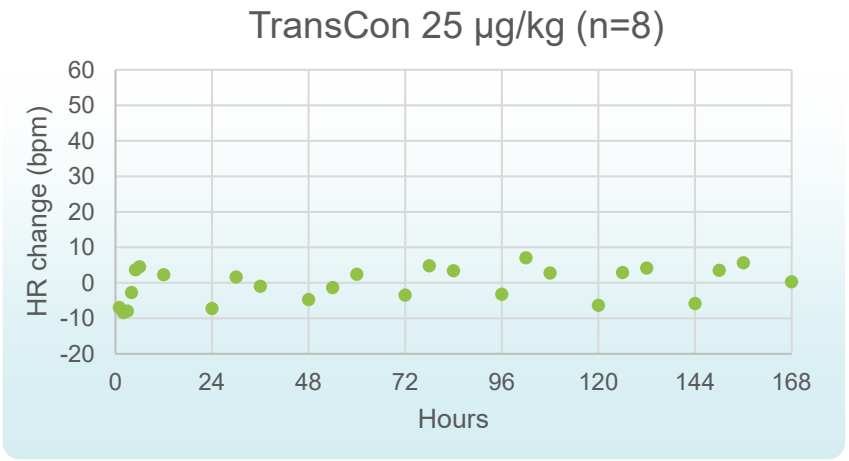


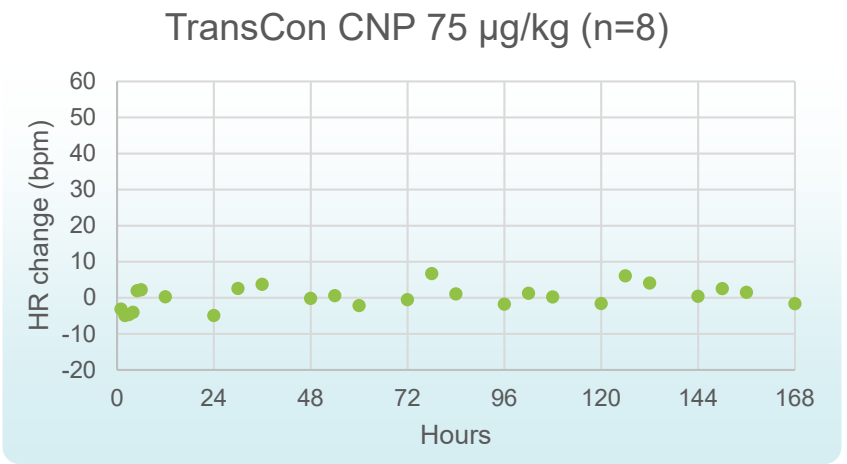


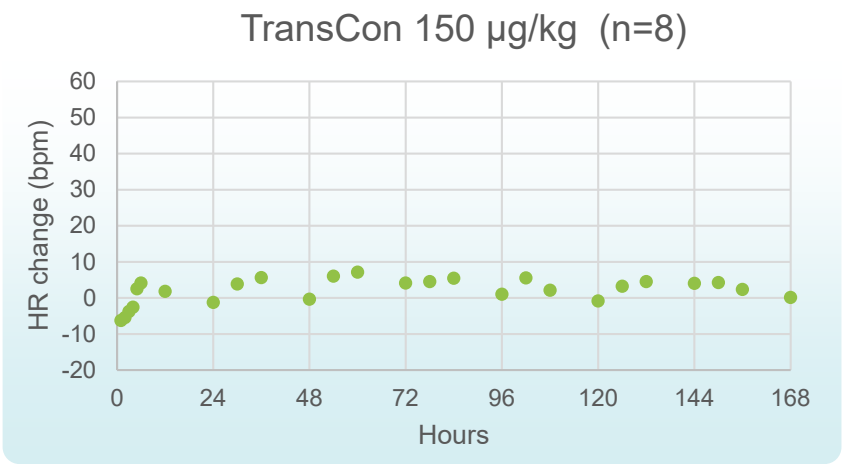
[1] Preliminary data. 3.0 and 10 µg/kg dose levels are not represented. Data from these cohorts are consistent with placebo.

Mean Resting Heart Rate Unchanged from Predose[1]









Change in heart rate

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

Well-tolerated Safety Profile[1]

- 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

[1] Preliminary data

TransCon CNP: Highlights

- 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
  - Continuous CNP exposure at target levels is important for balancing the CNP/FGFR3 pathways and normalizing growth
- Generally well tolerated across all cohorts
- Potential for a significant impact on patients' lives, not only affecting height but also addressing many comorbidities associated with achondroplasia
- Phase 2 initiation expected Q3 2019
- Multiple patent concepts provide potential protection into 2037

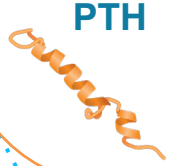
Vision 3x3:
Strategic Roadmap to 2025

Vision 20/20: Established the Foundation for a Leading Rare Disease Company

Clinical validation of 3 product candidates in endocrinology rare disease

TransCon hGH

TransCon PTH



TransCon CNP

- ✓ Advance our pipeline of three endocrinology rare disease product candidates towards approval
- ✓ Create a leading integrated commercial business primarily focused on the U.S. market with best-in-class products
- ✓ Identify a new rare disease therapeutic area with high-value product opportunities

ascendis pharma

Vision 3x3: Strategic Roadmap to 2025

Our Goal is to Achieve Sustainable Growth through Multiple Approaches

- Obtain regulatory approval for 3 Endocrinology Rare Disease products
  - TransCon hGH for pediatric growth hormone deficiency
  - TransCon PTH for adult hypoparathyroidism
  - TransCon CNP for achondroplasia
- Growth of Endocrinology Rare Disease pipeline through:
  - Label expansion programs with the goal of obtaining 9 indications in total
  - Global clinical reach direct or through partnerships
- Build an integrated commercial business for our Endocrinology Rare Disease franchise in North America and select European countries
  - Establish global commercial presence with partners outside our geographic areas
- Create 3 independent therapeutic areas each with a diversified pipeline built on TransCon technologies and our unique algorithm for product innovation
  - Established oncology as next independent therapeutic area

Extending Global Footprint: VISEN Pharmaceuticals

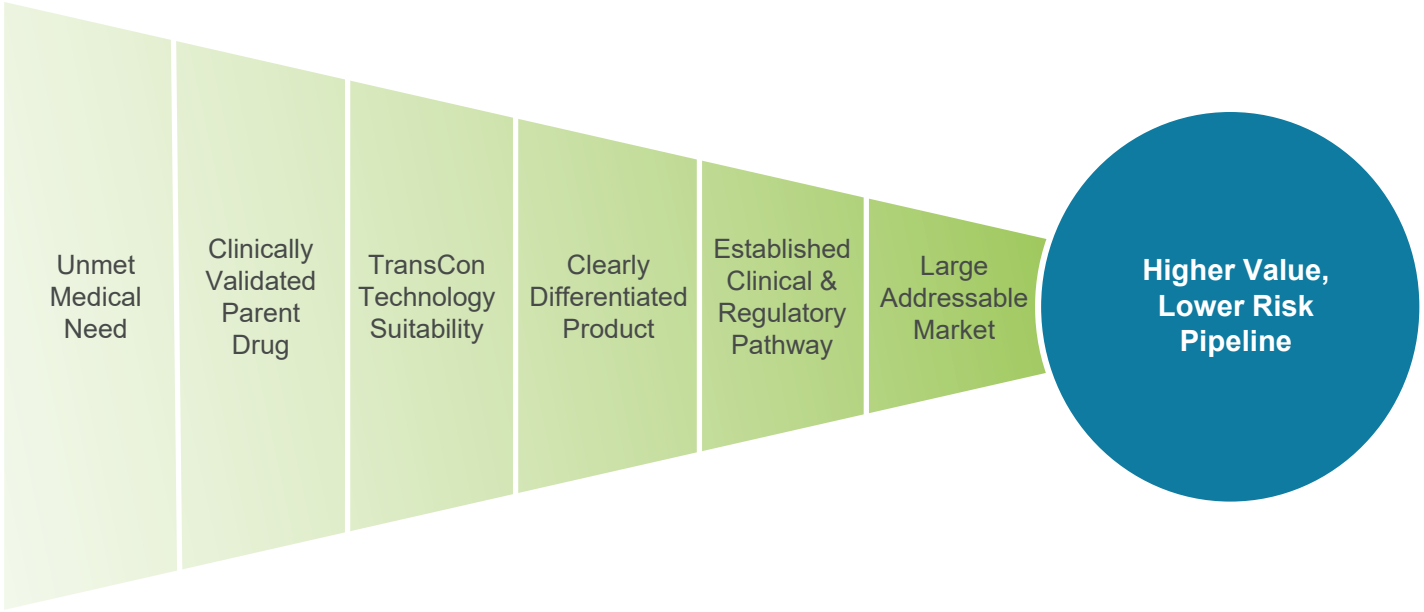
- Develop and commercialize TransCon hGH, TransCon PTH and TransCon CNP in Greater China
  - Right of negotiation on certain other endocrinology product candidates in Greater China[1]
  - Led by Pony Lu, an experienced Takeda executive in charge of Greater China
- Overview
  - Vivo Capital and Sofinnova Ventures invested $40 million
  - Visen responsible for all development, manufacturing and commercialization costs in Greater China; Ascendis will be reimbursed for clinical trial materials and technical support
  - Strengthens Ascendis global development strategy, increasing potential reach of clinical trials for rare diseases into China
  - Potentially significant upside through 50% equity ownership
  - Governance established through ownership, shareholder protections, board membership, joint development committee and separate licensing and supply agreements

Partnership with experienced investors builds presence in second largest pharmaceutical market in the world and expands rare disease clinical programs

[1] "Greater China" means People's Republic of China, including Hong Kong, Macau and Taiwan.

Why Oncology?

Checks all the boxes of the Ascendis product innovation algorithm

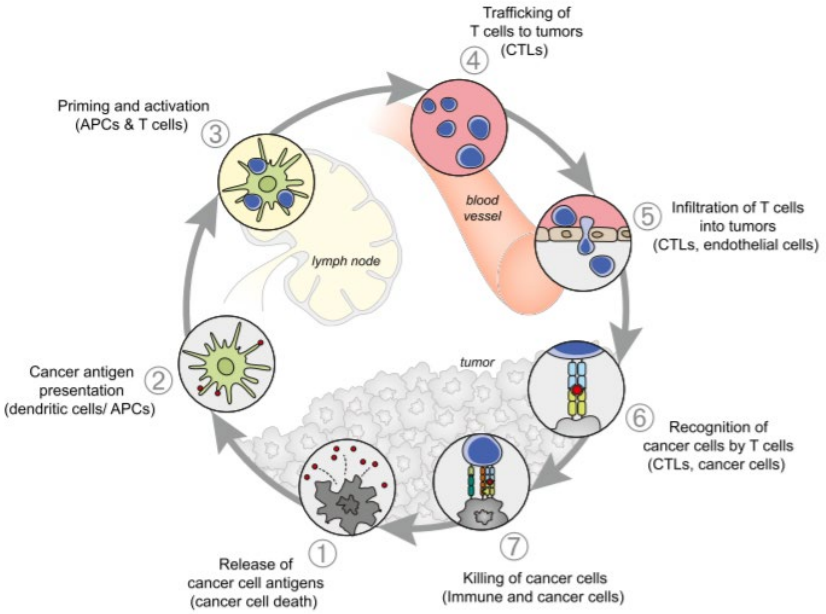


Both TransCon technologies well suited for Oncology:

- Sustained Systemic Delivery:
  Designed to provide predictable continuous exposure to increase efficacy and reduce toxicity
- Sustained Localized Delivery:
  Designed to maximize intratumoral (IT) exposure while minimizing systemic toxicity

Oncology Pipeline Strategy

- Impact all aspects of the Cancer Immunity Cycle
  - Stimulators of innate immunity
  - Stimulators of adaptive immunity
  - Modulators of tumor microenvironment

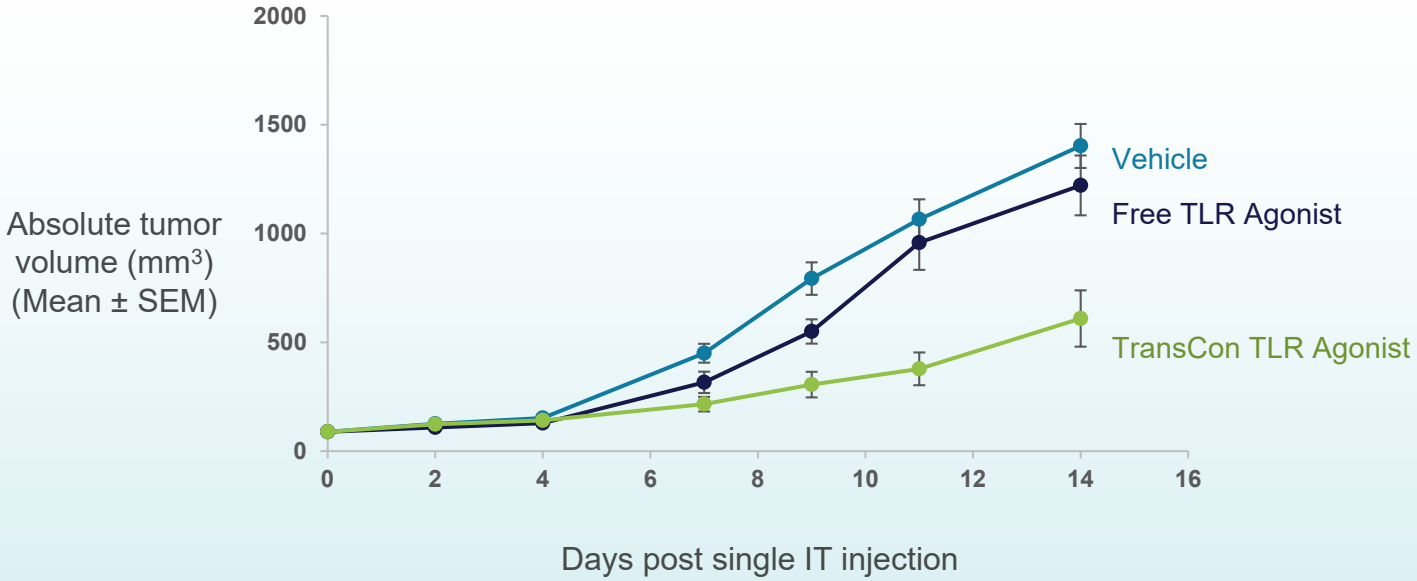


From Chen & Mellman, Immunity, 2013

Goal to create at least 3 best-in-class candidates from validated parent drugs – each addressing different aspects of the Cancer Immunity Cycle

In Vivo Sustained Localized Delivery Proof-of-Concept

- Single IT injection of TransCon TLR Agonist demonstrated superiority to equal IT dose of free TLR Agonist in a syngeneic mouse colon-derived tumor model
  - TransCon TLR Agonist was well tolerated and had no effect on body weights



- Sustained localized IT delivery *in vivo* demonstrated potential best-in-class product profile and foundation to expand to other targets

Selected 2019 Expected Milestones

Q1 2019

- ✓ TransCon hGH: heiGHt Trial top-line Phase 3 data
- ✓ TransCon PTH: Phase 2 trial initiation, including pen device

Q2 2019

- TransCon hGH: fliGHt Trial top-line Phase 3 data
- TransCon hGH: Introduction of auto-injector into enliGHten Trial
- Research Day: Unveiling oncology pipeline and near-term milestones

Q3 2019

- TransCon hGH: Long-term clinical database lock
- TransCon CNP: Phase 2 trial initiation

Q4 2019

- TransCon PTH: Phase 2 trial top-line data