Ascendis Pharma A/S

January 2020

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 April 3, 2019 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
- Endocrinology rare disease internal pipeline and expected 2020 milestones
  - TransCon hGH for pediatric GH deficiency: BLA and MAA filings expected Q2 and Q4
  - TransCon PTH for hypoparathyroidism: Phase 2 top-line data end March[1]; long-term data Q3
  - TransCon CNP for achondroplasia: Phase 2 ACcomplisH dose escalation and initiate second trial in China[2] Q4
- Build leading positions for each endocrinology rare disease product with commercial focus on maximizing global reach
  - Partnership with VISEN Pharmaceuticals for endocrinology rare disease products in China
- Oncology pipeline in development with highly differentiated product candidates
  - First IND filing or equivalent expected in 2020
- As of September 30, 2019, cash and cash equivalents of ~€659 million

[1] Results timing +/- two weeks; [2] Through VISEN Pharmaceuticals

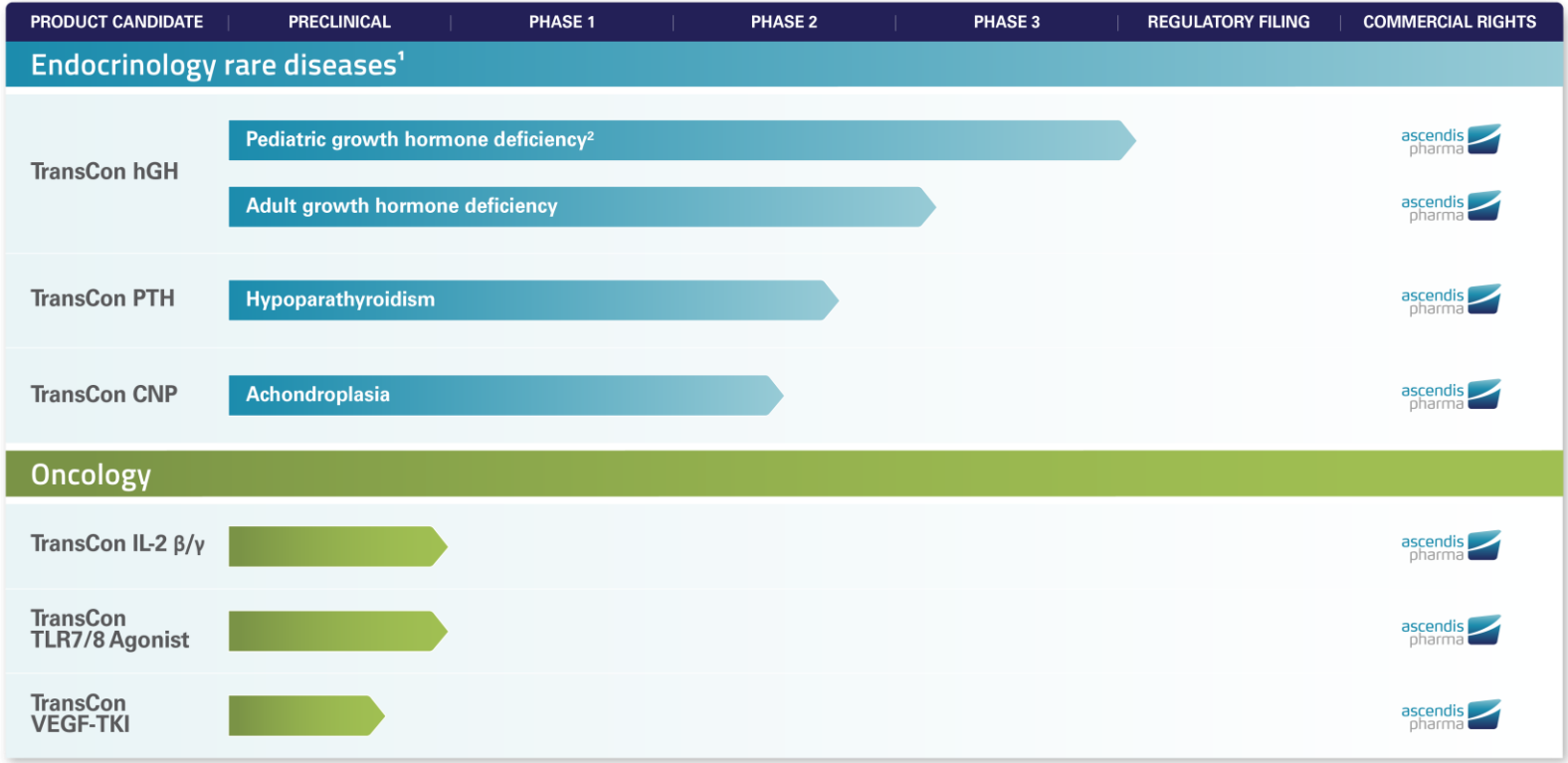
Vision 3x3: Building a Leading BioPharma Company

Our Goal is to Achieve Sustainable Growth through Multiple Approaches

- Obtain regulatory approval for three independent Endocrinology Rare Disease products
  - TransCon Growth Hormone for pediatric growth hormone deficiency
  - TransCon PTH for adult hypoparathyroidism
  - TransCon CNP for achondroplasia
- Create further growth of Endocrinology Rare Disease pipeline through
  - Global clinical reach
  - Pursuing 9 total indications, label optimization, and life cycle management
  - New endocrinology products
- Establish global commercial presence for our Endocrinology Rare Disease area
  - Build integrated commercial organization in North America and select European countries
  - Establish global commercial presence through partners with local expertise and infrastructure
- In Oncology, create a high value pipeline with one IND or equivalent filing each year
- Creation of a third independent therapeutic area with a diversified pipeline

ascendis pharma

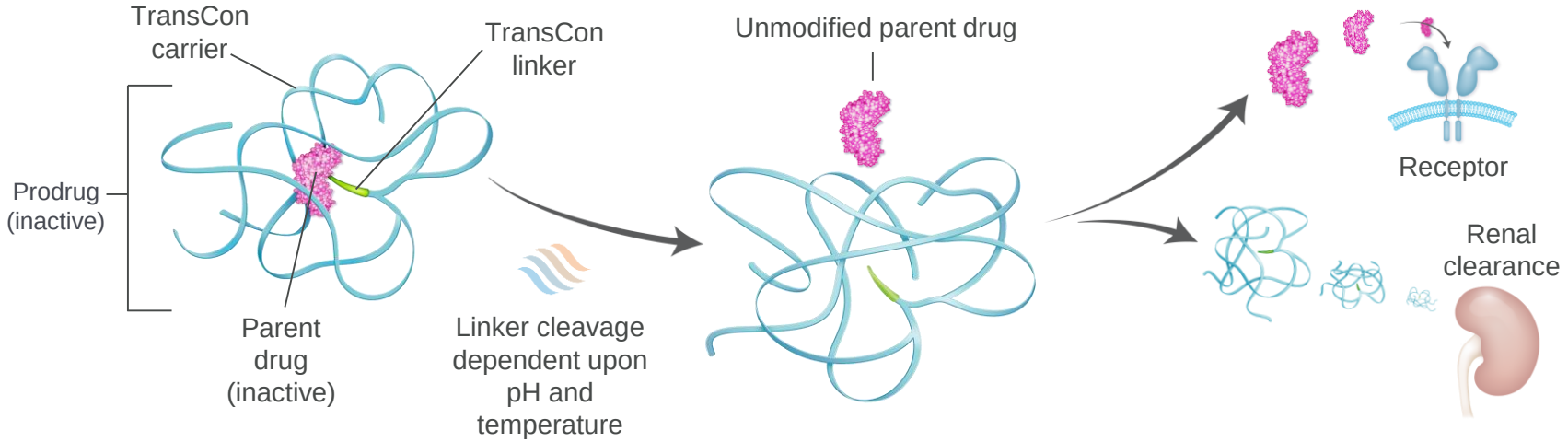
Diverse Pipeline of Independent Product Candidates



[1] Excludes rights granted to VISEN Pharmaceuticals in Greater China
[2] In phase 3 development for pediatric growth hormone deficiency in Greater China through strategic investment in VISEN Pharmaceuticals

All product candidates are investigational. For investor communication only. Not for use in promotion or product commercialisation.

TransCon Technology: Sustained Systemic Delivery

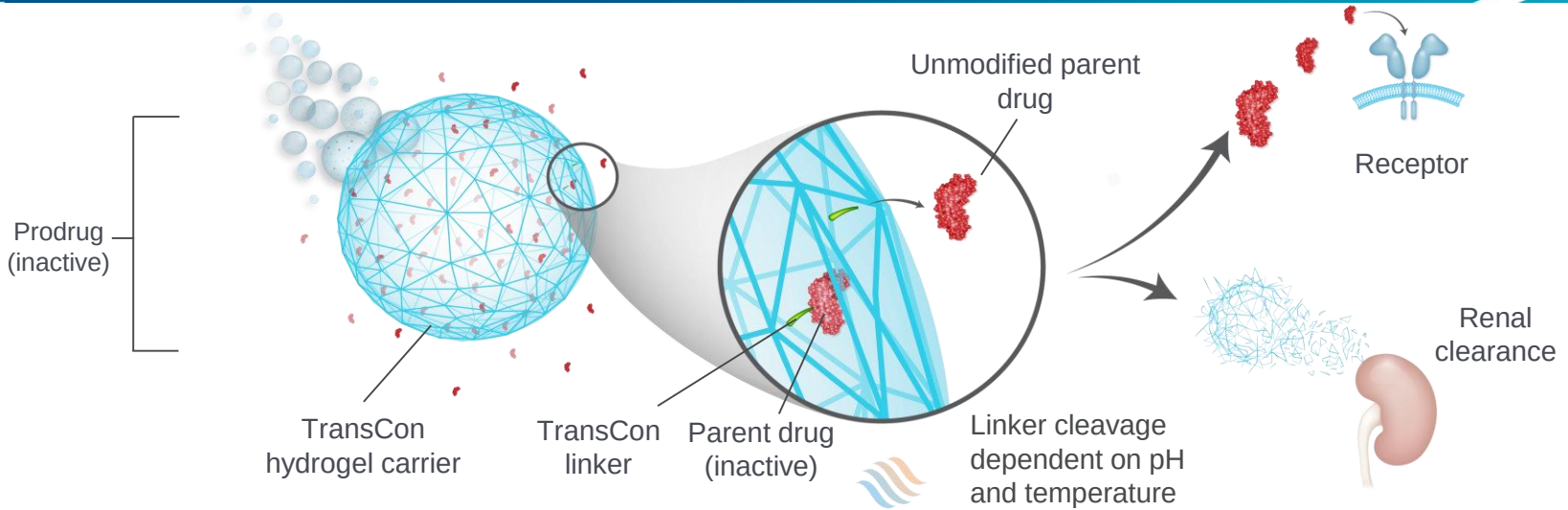


Parent drug is transiently bound to a TransCon linker-soluble carrier moiety, which inactivates and shields parent drug from clearance

Following injection, the linker is designed to autocleave at a specific rate to predictably release unmodified parent drug

Designed to distribute released drug like the parent molecule; linker-carrier is cleared renally

TransCon Technology: Sustained Localized Delivery



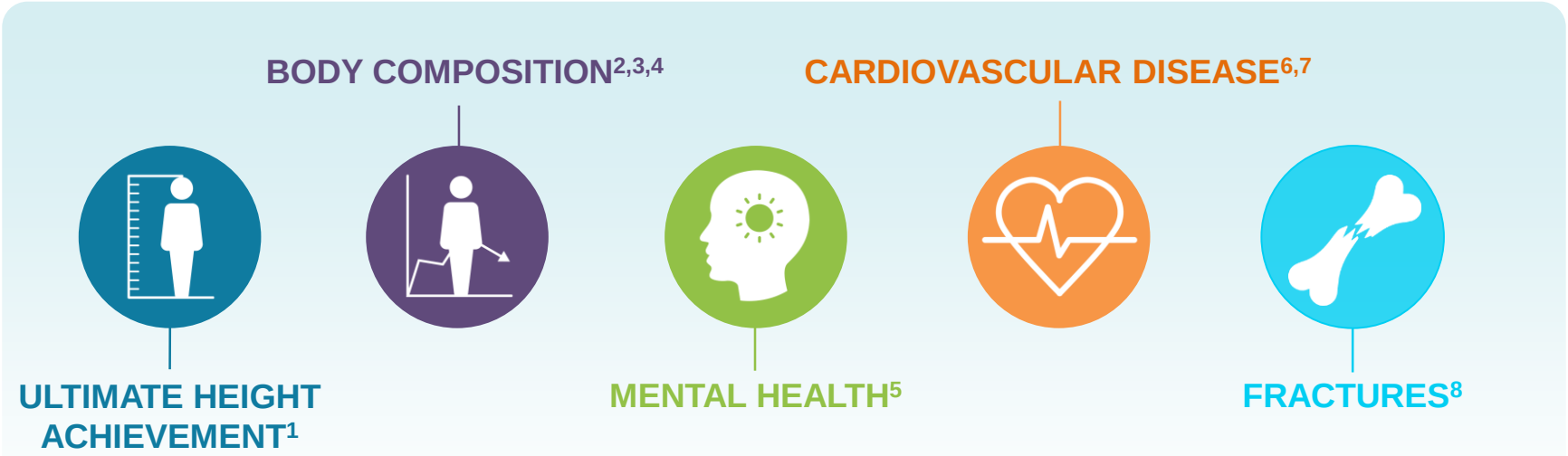
Parent drug is transiently bound to TransCon linker-hydrogel carrier, which inactivates, shields parent drug and prevents clearance

Following injection, the linker is designed to autocleave at a specific rate 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

TransCon Growth Hormone: Once-Weekly Replacement Therapy

Growth Hormone Supports Overall Endocrine Health



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

ascendis pharma

TransCon hGH Phase 3 Program in Pediatric GHD

N=161

- Treatment-naïve subjects

N=146

- Subjects previously treated (n=143) and treatment-naïve (<3 years, n=3)

Extension trial (N=296)

Expected Regulatory filings (BLA Q2 2020, MAA Q4 2020)

ascendis pharma

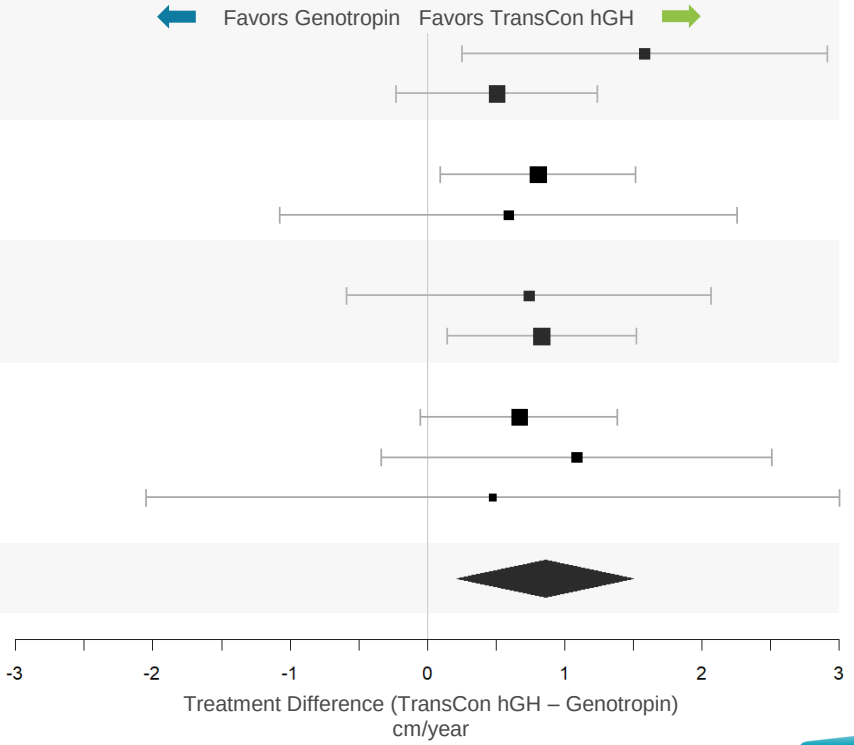
TransCon hGH Met Primary Objective of Non-inferiority and Demonstrated Superiority in AHV at Week 52

	TransCon hGH 0.24 mg/kg/week (n=105)	Genotropin® 0.24 mg/kg/week (n=56)	Estimate of Treatment Difference	P-value
LS Mean 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 (cm/year)	10.71 – 11.62	9.73 – 10.89	0.22 – 1.50	

ANCOVA model was applied after missing data were imputed by multiple imputation method.

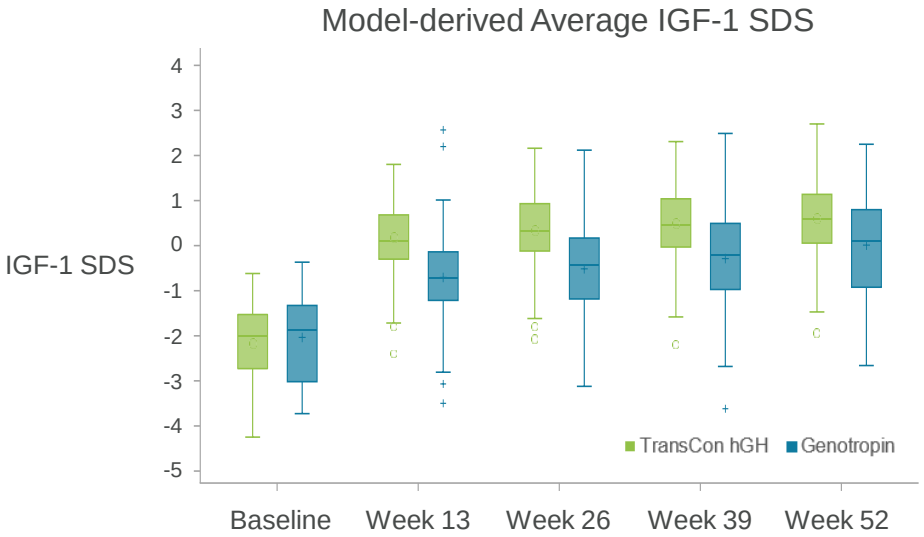
AHV Consistently Favors TransCon hGH Across All Subgroups at Week 52

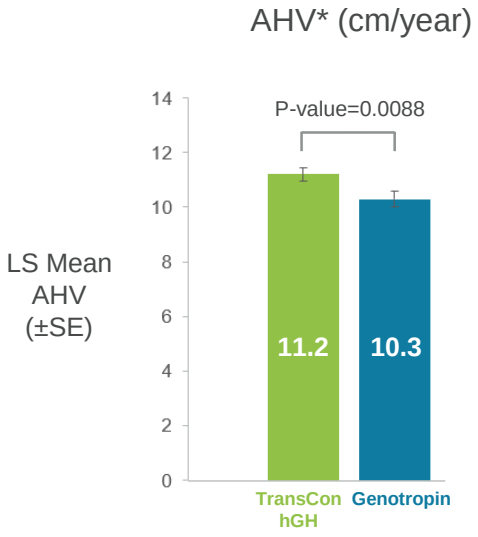
	TransCon hGH (n=105)	Genotropin (n=56)
Age		
< 6 years	24%	25%
≥ 6 years	76%	75%
Sex		
Male	82%	82%
Female	18%	18%
Baseline GH-Stimulation		
≤ 5ng/mL	35%	38%
> 5 ng/mL	65%	63%
Etiology and Extent of GHD		
Isolated Idiopathic	65%	66%
Isolated Organic	18%	16%
Multiple Pituitary Hormone Deficiency	17%	18%
Overall		



ANCOVA Model

AHV Paralleled the Difference in Average IGF-1





TransCon hGH preserved the balance between direct and indirect effects of daily hGH

*ANCOVA model

Key Learnings from TransCon hGH Clinical Trials

- TransCon hGH demonstrated an adverse event and immunogenicity profile comparable to that of a daily hGH
- TransCon hGH demonstrated superior height velocity[1] to a daily hGH through a PK profile of released hGH that may be more efficiently utilized by target tissues
- TransCon hGH data showed predictable linear response to dose titrations
- TransCon hGH data suggest the same mode of action as daily hGH and preservation of the biological balance between direct hGH and IGF-1 effects in target tissues

[1] Based on results from phase 3 heiGHt Trial at 52 week endpoint

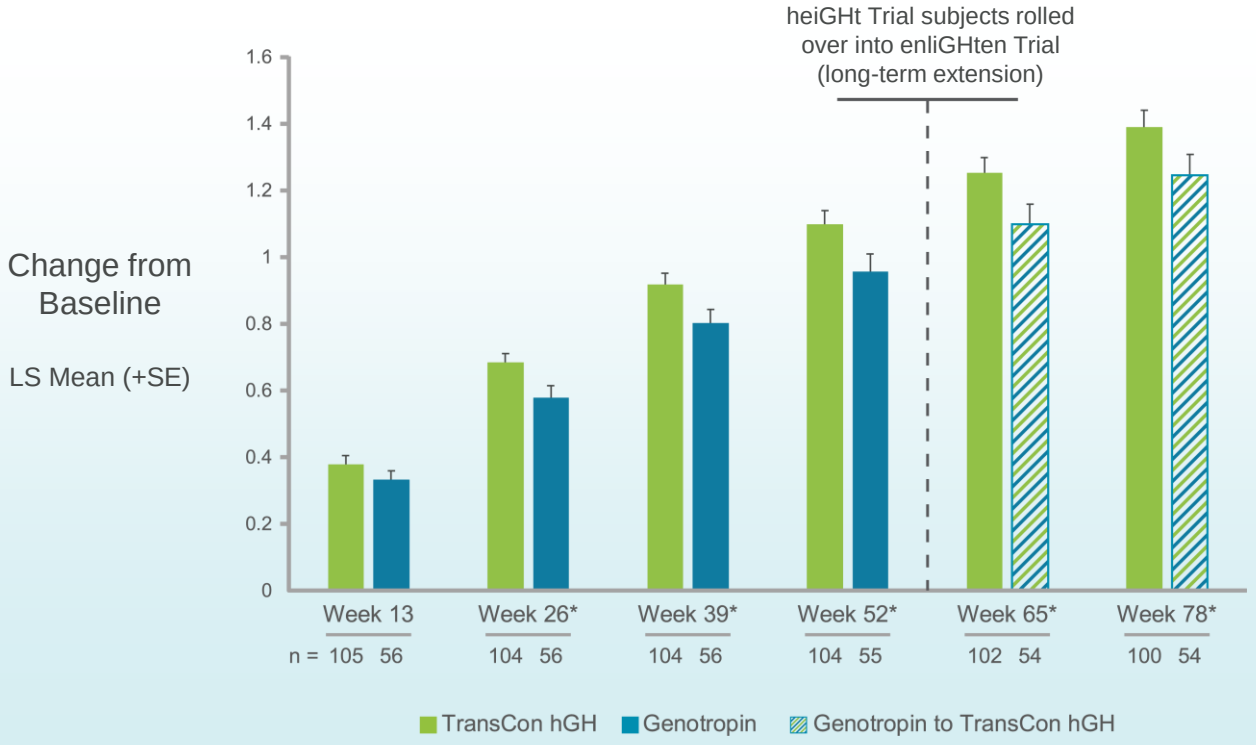
Adverse Event Profile of TransCon hGH in the Phase 3 Program[1]

	heiGHt Trial		fliGHt Trial	enliGHten Trial[2]
	TransCon hGH 0.24 (n=105) n (%)	Genotropin 0.24 (n=56) n (%)	TransCon hGH 0.24 (N=146) n (%)	TransCon hGH 0.24 (N=296) n (%)
Treatment-emergent Adverse Events (TEAEs)	81 (77)	39 (70)	83 (57)	161 (54)
TEAEs Related to Study Drug	12 (11)	10 (18)	6 (4.1)	10 (3.4)
Serious Adverse Events (SAEs)	1 (1.0)	1 (1.8)	1 (0.7)[3]	5 (1.7)[4]
SAEs Related to Study Drug	0	0	0	0
TEAEs Leading to Any Action on Study Drug	2 (1.9)	1 (1.8)	2 (1.4)	5 (1.7)
TEAEs Leading to Discontinuation of Study Drug	0	0	0	0

TransCon hGH had an adverse event profile comparable to daily hGH which was consistent across phase 3 trials

[1] All doses expressed in mg/kg/week
[2] Based on data reported up to September 2019
[3] One subject reported two SAEs; both considered unrelated to study drug
[4] Two subjects reported two SAEs; all considered unrelated to study drug

TransCon hGH Sustained Improvement in Height SDS



heiGHt subjects treated for 1.5 years with TransCon hGH demonstrated:

- Superior growth after 52 weeks compared to Genotropin[1]
- Superior growth continued in the enliGHten extension trial

[1] Based on results from phase 3 heiGHt Trial at 52 week endpoint
*Treatment difference resulted in a nominal p-value <0.05
ANCOVA model

Auto-Injector Designed to Improve Adherence

Key Features to Enhance Patient Experience

- Room temperature storage
- Small needle, comparable to daily hGH (31G, 4mm)
- Single low-volume (<0.60mL) injection for patients ≤60kg
- Simple operation
- No waste due to empty-all design
- Device lifespan at least 4 years
- Easy to titrate
- Bluetooth® connectivity enabled for automatic data capture
- Development of integrated connectivity platform underway

>160 subjects are using Auto-Injector and dual-chamber cartridges (DCCs) in extension trial

On-track towards Filing TransCon hGH BLA Q2 and MAA Q4

- Completed both the fliGHt and heiGHt Trials, including rollover into enliGHten, and completed two-year follow-up for 46 subjects on TransCon hGH
- Completed manufacturing of PPQ batches and development of the auto-injector
- Proprietary Auto-Injector and DCCs introduced in phase 3 enliGHten Trial; met objective of collecting required usability data to support auto-injector as part of initial BLA submission
- Two pre-BLA meetings held with FDA related to Chemistry, Manufacturing and Controls (CMC), and for clinical/non-clinical packages
- In Europe, received orphan designation for TransCon hGH and Conformité Européenne (CE) mark for Auto-Injector

TransCon hGH: Raising the Bar

- Phase 3 heiGHt Trial demonstrated superior height velocity of TransCon hGH in pediatric GHD, with comparable safety and tolerability as compared to a daily hGH
- BLA filing expected Q2 2020 and MAA filing expected Q4 2020
- Create further growth:
  - China: Pediatric GHD phase 3 initiated
  - Global: Adult GHD phase 3 expected to be initiated Q1 2020
  - Japan: Pediatric phase 3 expected to be initiated Q4 2020
- Easy-to-use Auto-Injector part of initial BLA/MAA filings
- Commercial manufacturing ongoing
- Commercial leadership team, infrastructure and launch plan in place
- Multiple independent patent filings to provide additional potential protection into 2039

TransCon PTH:
PTH Replacement Therapy for Hypoparathyroidism

Chronic Hypoparathyroidism: Significant Patient Population

Estimated Prevalence: ~200k in these 4 regions

USA

~70k-112k

- 2013, Powers et. al., Prevalence and Incidence of Hypoparathyroidism in the United States Using a Large Claims Database, JBMR
- 2011, Clarke et. al., Co-morbid Medical Conditions Associated with Prevalent Hypoparathyroidism: A Population-Based Study

Europe

~86k-223k

- 2013, Underbjerg et. al., Cardiovascular and Renal Complications to Postsurgical Hypoparathyroidism: A Danish Nationwide Controlled Historic Follow-up Study
- 2015, The Epidemiology of Nonsurgical Hypoparathyroidism in Denmark: A Nationwide Case Finding Study
- 2016, Astor et. al., Epidemiology and Health-Related Quality of Life in Hypoparathyroidism in Norway

Japan

~25k-32k

- 2016, Suzuki et. al., Factors Associated with Neck Hematoma After Thyroidectomy
- 2018, Interview conducted with Japanese HP expert

South Korea

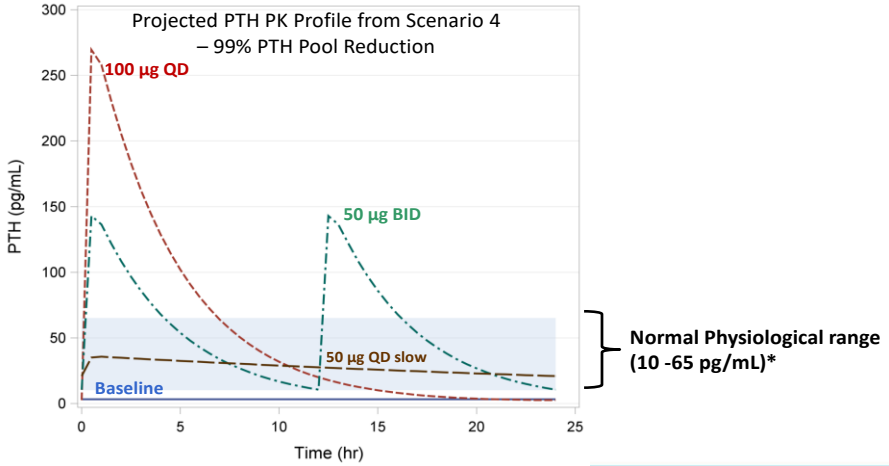
~12k-13k

- S. Korean ICD-10 codes
- 2018, Interview conducted with S. Korean HP expert

Constant Normal Level of PTH is Optimal - FDA Perspective[1,2]

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



20

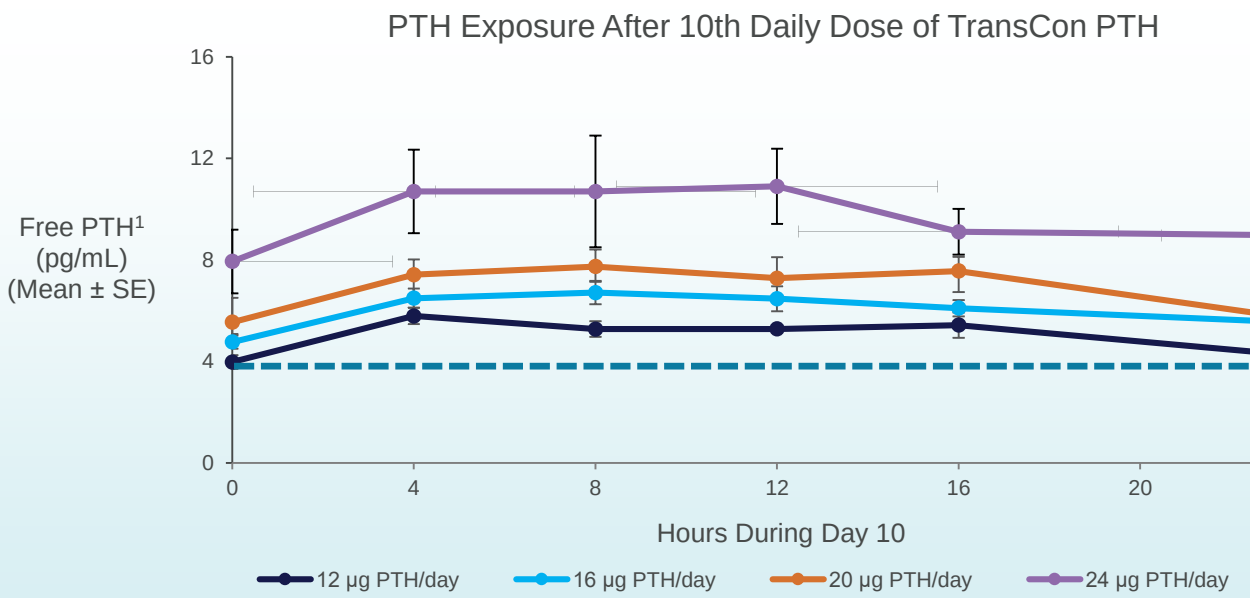
Continuous infusion of PTH demonstrated[3,4]:

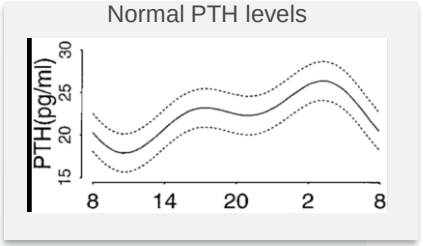
- Normalization of serum calcium and phosphate
- Complete removal of current standard of care (vitamin D and calcium supplements)
- Normalization of urinary calcium

[1,2] FDA presentation: Natpara Advisory Committee, September 12, 2014; Clin Pharmacol Ther. 2019 105(3):710
[3,4] J Clin Endo Metab 2012 97(2);391–399; J Pediatr 2014 165(3);556-563

ascendis pharma

Phase 1: PK Data Support Infusion-like Profile over 24 Hours





TransCon PTH daily dosing provided a flat infusion-like profile of released PTH at day 10

[1] PTH measured as Free PTH(1-34) and Free PTH(1-33)
Analyses from TransCon PTH Phase 1 trial; data not shown for doses <12 µg/day, as levels of Free PTH are BLQ.
Poster presented at ECTS 2019

TransCon PTH Phase 2 Trial Design

PaTHforward TRIAL

~40 adult subjects with HP currently receiving standard of care (active vitamin D + calcium)

Primary Composite Endpoint (4 weeks)

Proportion of subjects with:

- Normal serum calcium; ***and***
- Normal FeCa (or at least 50% decrease from baseline); ***and***
- Off active vitamin D; ***and***
- Taking ≤1,000 mg/day calcium

Key Secondary Endpoints (4 weeks)

- Primary composite ***and*** taking ≤500 mg/day calcium

Additional Endpoints ≥4 weeks

- PRO* measures (HPES: a disease-specific PRO for HP)
- Nephrolithiasis, nephrocalcinosis, vascular calcification, ER/urgent care visits and hospitalizations
- BMD and TBS by DXA, bone turnover markers, 24-hour urine calcium excretion (in extension only)

* PRO = patient-reported outcome

Expanded Phase 2 Trial and Open-label Extension Trial

- Implemented addendum to protocol to expand and expedite enrollment in the U.S. for subjects affected by the NATPARA® recall
- Subjects from fixed-dose PaTH Forward Trial roll over to the open-label extension with individually optimized TransCon PTH dosing to evaluate long-term safety and efficacy
- Long-term data from open-label extension evaluates a composite endpoint. Evaluating proportion of subjects with:
  - Normal serum calcium; ***and***
  - Off active vitamin D; ***and***
  - Taking ≤500 mg/day calcium; ***and***
  - Normal 24-hour urine calcium excretion (or at least 50% decrease from baseline)

PaTH Forward Update

- Sites in Canada, Denmark, Germany, Italy, Norway and U.S.
  - Addendum implemented in the U.S.
- Screening completed with expected enrollment of ~55 subjects
- To date, no dropouts in the double-blind portion of PaTH Forward
- Preliminary data on first 8 subjects completing 4 weeks follow-up in open-label extension[1]
  - All subjects are completely off current standard of care
  - 8 of 8 subjects no longer require active vitamin D
  - 7 of 8 subjects no longer require calcium supplements (one subject taking < 500 mg calcium)
- Top-line phase 2 data expected by end of March 2020[2]
- Six-month data from open-label extension expected Q3 2020

[1] Original treatment assignment remains blinded
[2] Results timing +/- two weeks

Simple Pen Injector in Phase 2

Key Features

- Simple operation
- Three multi-use pens with three different strengths (6, 9, 12 µg; 15, 18, 21 µg; 24, 27, 30 µg)
- Ready-to-use liquid formulation, room temp stability for 14 days
- Low injection volume (≤0.1 mL)
- Small (31G), short (5 mm) safety pen needle

Pen injector planned for commercial launch being used in phase 2

PaTHforward TRIAL

ascendis pharma

TransCon PTH: Developing a “True” Replacement Therapy

- Phase 1 data support infusion-like profile of TransCon PTH
- Carcinogenicity study waiver granted in the U.S. and EU
- TransCon PTH Phase 2 trial expanded to allow easier and faster enrollment of subjects previously treated with NATPARA®
- Manufacturing of PPQ batches including pen injector progressing as planned
- Maximizing enrollment to demonstrate substantial evidence of effectiveness

TransCon CNP: The New Frontier of Growth Biology

TransCon CNP: Pursuing New Frontier of Growth Biology

- C-type natriuretic peptide (CNP) pathway has demonstrated clinical proof of concept
  - Short half-life of native CNP (2-3 minutes) limits therapeutic use
- TransCon CNP provides continuous CNP exposure 24 hours a day, seven days a week to balance constantly activated FGFR3 pathway, aiming to restore normal growth
- In phase 1, TransCon CNP demonstrated
  - Effective CNP $t_{1/2}$ of approximately 120 hours
  - No serious AEs, no impact on resting blood pressure or heart rate, no downregulation of endogenous CNP production; no anti-CNP antibodies
- ACHieve natural history study and ACcomplisH phase 2 trial (ages 2 – 10 years) initiated, with escalation of sequential dose cohorts in ACcomplisH throughout 2020
- Expansion of clinical program in China through VISEN Pharmaceuticals
  - ACHieve initiated; ACcomplisH China expected to be initiated Q4 2020
- Potential for significant impact on patients' lives, including height and comorbidities

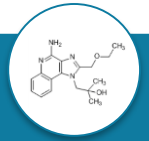
Oncology

Potential to Impact Efficacy, Safety and Practicality of Both Systemic and Intratumoral Cancer Treatments

- Applying TransCon technologies to clinically validated mechanisms to develop differentiated and potentially best-in-class products
  - Large number of validated oncology targets with known limitations
  - Applicable for diverse drug classes and mechanisms of action
  - Enable both systemic and intratumoral (IT) approaches

Advancing a diversified high-value pipeline

TransCon IL-2 β/γ



TransCon TLR 7/8 Agonist

TransCon VEGF-TKI

Design of TransCon IL-2 β/γ: 1) Designed for Desired Receptor Binding

Generation of IL-2 Variant

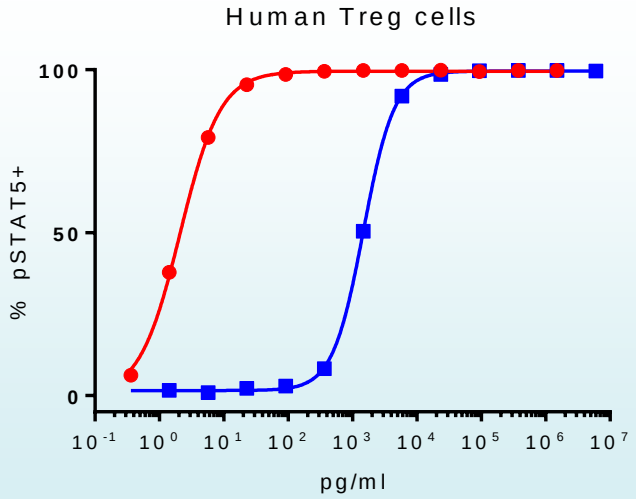
Introduction of cysteine at α–binding site of IL-2 (aldesleukin)

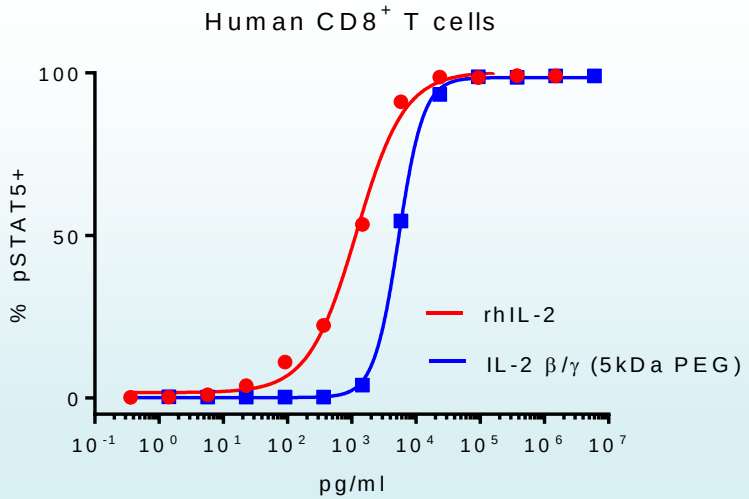
Blocking α–binding

Site-selective permanent PEG conjugation (5kDa) of introduced cysteine

Optimized IL-2 β/γ receptor selectivity and potency by permanent site-selective PEG conjugation at IL-2Rα–binding site

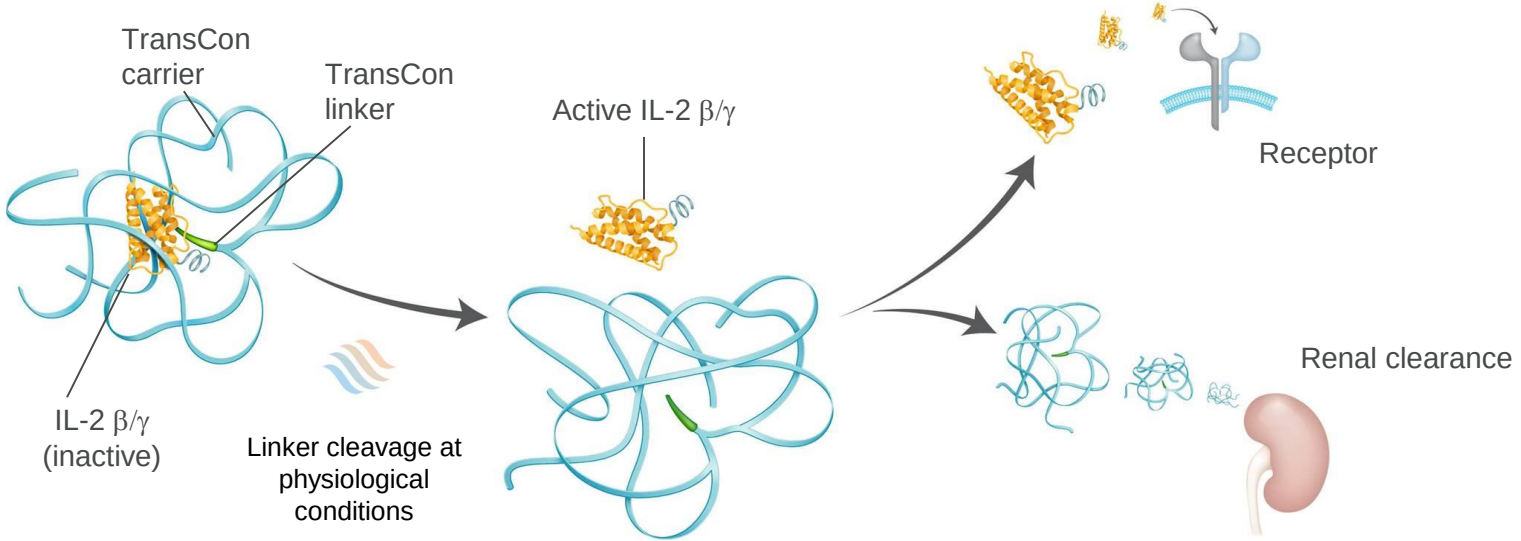
IL-2 β/γ – Desired Receptor Selectivity Demonstrated





~770-fold reduced potency on primary human Treg cells compared to rhIL-2
while only ~4-fold loss in potency on CD8+ T cells and NK cells

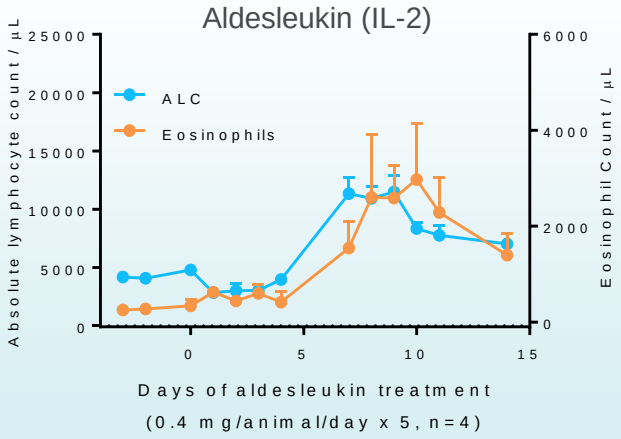
Design of TransCon IL-2 β/γ:
2) TransCon Technology to Optimize Exposure

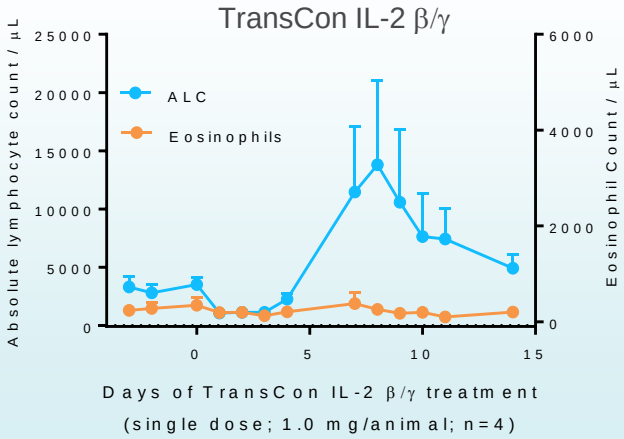


Sustained, long-lasting exposure utilizing the TransCon hGH linker and carrier, expected to support every 3 week dosing

TransCon IL-2 β/γ – Prolonged Activity and Receptor Selectivity Demonstrated in Cynomolgus Monkeys

A single 1 mg dose/animal (~0.1 mg/kg) TransCon IL-2 β/γ resulted in >3-fold enhancement of Lymphocyte Counts, Minimal Effect on Eosinophils Compared to Aldesleukin in Cynomolgus Monkeys





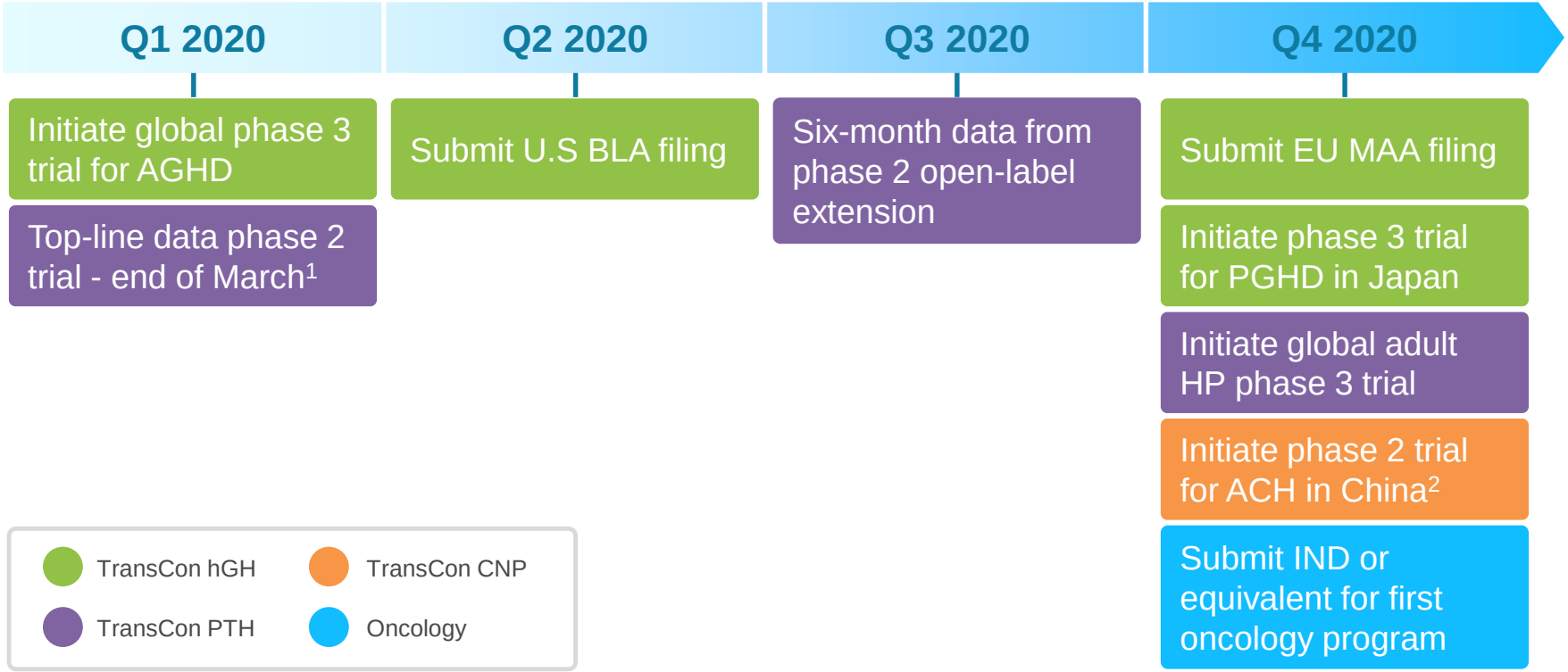
- Single dose provided >3-fold and prolonged enhancement of lymphocyte counts supporting Q3W dosing
- Well tolerated in monkeys with low risk of vascular leak syndrome; minimal effect on eosinophils[1]
- Monotherapy and combination anti-tumor activity observed in mice

[1] Rand et al., JCI, 1991; Van Haelst Pisani C et al., Blood, 1991

Oncology Summary

- Best-in-class potential using systemic and intratumoral TransCon technologies
  - Preclinical anti-tumor proof-of-concept demonstrated with small molecules, cytokines and antibodies
  - TransCon intratumoral technologies acceptance into the FDA´s Emerging Technology Program
- Differentiated product candidates with potential in multiple indications
  - TransCon IL-2 β/γ
  - TransCon TLR7/8 Agonist
  - TransCon VEGF-TKI
- Potent anti-tumor effects of TransCon oncology candidates demonstrated in preclinical studies
- First IND or equivalent expected to be filed Q4 2020
- Over 20 patents and applications in support of TransCon oncology candidates

Selected 2020 Expected Milestones



[1] Results timing +/- two weeks
[2] Conducted through strategic investment in VISEN Pharmaceuticals