



Ascendis Pharma A/S

40th Annual J.P. Morgan Healthcare Conference
January 10, 2022

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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, expectations regarding the potential benefits of SKYTROFA, prospective products, availability of funding, clinical trial results, product approvals and regulatory pathways, collaborations, licensing or other arrangements, the scope, support progress, results and costs of developing our product candidates or any other future product candidates, the potential market size and size of the potential patient populations for SKYTROFA and our product candidates, timing and likelihood of success, plans and objectives of management for future operations, the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates, future results of current and anticipated products, and the future operations of VISEN Pharmaceuticals, 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 preliminary prospectus supplement related to the proposed public offering and our most recent Annual Report on Form 20-F filed with the SEC on March 10, 2021 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.

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SKYTROFA (lonapegsomatropin-tcgd) has been approved by the U.S. Food and Drug Administration for the treatment of pediatric growth hormone deficiency. SKYTROFA is and has been under clinical investigation and has not yet been approved for marketing by the European Medicines Agency or other foreign regulatory authorities. In addition, this presentation concerns other product candidates that are 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.

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Ascendis Pharma, a Global Company

- **Founded** in 2007 in Copenhagen, Denmark
- **Publicly listed** on NASDAQ since 2015 (ASND)
- **TransCon™**: Innovative technology platform utilized in all product candidates
- **Our mission**: Develop best-in-class therapeutics addressing unmet medical needs
- **Our values**: Patients, Science and Passion
- **Financials**: As of September 30, 2021, cash, cash equivalents and marketable securities of €930 million

An expanding global footprint



Committed to making a meaningful difference in patients' lives

*VISEN Pharmaceuticals was established in 2018 to develop and commercialize endocrinology rare disease therapies in Greater China.

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Vision 3x3: Building a Leading Global BioPharma Company

Our Goal Is to Achieve Sustainable Growth through Multiple Approaches

- Obtain regulatory approval for three independent Endocrinology Rare Disease products
 - TransCon hGH for pediatric growth hormone deficiency
 - TransCon PTH for adult hypoparathyroidism
 - TransCon CNP for achondroplasia
- Grow 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
- Advance a high-value oncology pipeline with one IND or similar filing each year
- Create a third independent therapeutic area with a diversified pipeline

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Five Independent Product Candidates in Clinical Development

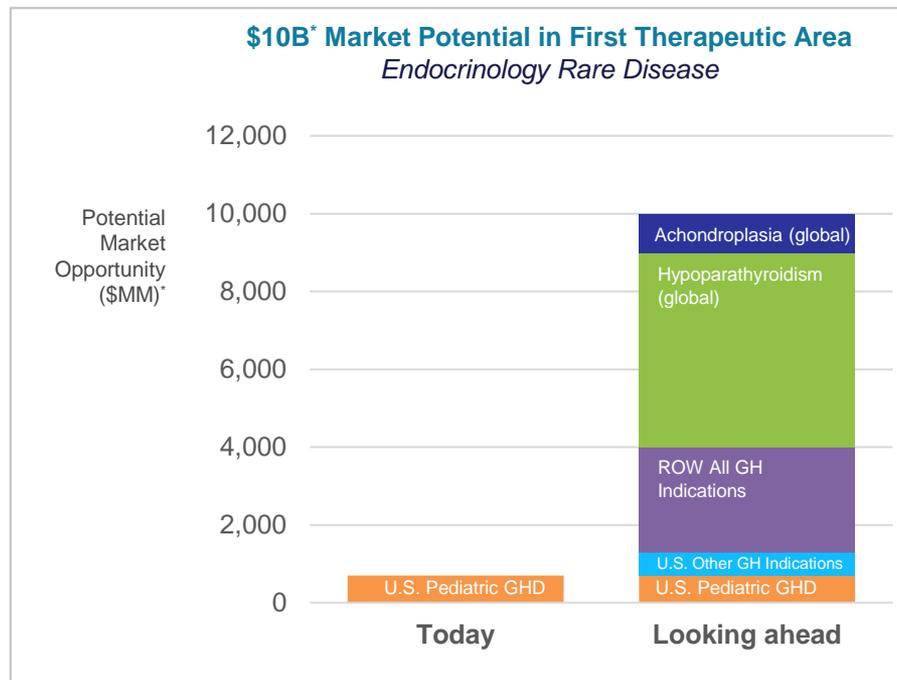
- Endocrinology Rare Disease
 - TransCon hGH:
 - SKYTROFA® (lonapegsomatropin-tcgd) approved in the US by FDA in August 2021 for pediatric growth hormone deficiency (GHD)
 - TransCon hGH in Europe: positive CHMP opinion for pediatric GHD received November 12, 2021
 - Pediatric GHD: Phase 3 trials in China¹ and Japan ongoing
 - Adult GHD: Global Phase 3 foresiGHt Trial ongoing
 - TransCon PTH: Adult hypoparathyroidism (HP) Phase 3 PaTHway Trial in North America and Europe ongoing
 - TransCon CNP: Achondroplasia Phase 2 trials: ACcomplisH Trial and ACcomplisH China Trial¹ ongoing
- Oncology
 - TransCon TLR7/8 Agonist: Phase 1/2 transcendIT-101 Trial
 - TransCon IL-2 β/γ : Phase 1/2 IL- β elie γ e Trial

Potential to create best-in-class products addressing unmet medical needs by applying TransCon technologies to parent drugs with clinical proof-of-concept or clinically validated pathways

Building a Viable, Sustainable, Long-Term Business Model

- Unmet medical needs; focused on large rare disease – potential multi billion \$ product opportunities
- Aim to achieve global market leadership with highly differentiated products developed using validated, proprietary technology platform
- Diversified multiple product candidates in each therapeutic area to leverage synergies
- Sustainability and long-term growth from additional therapeutic areas
- The right capabilities to execute

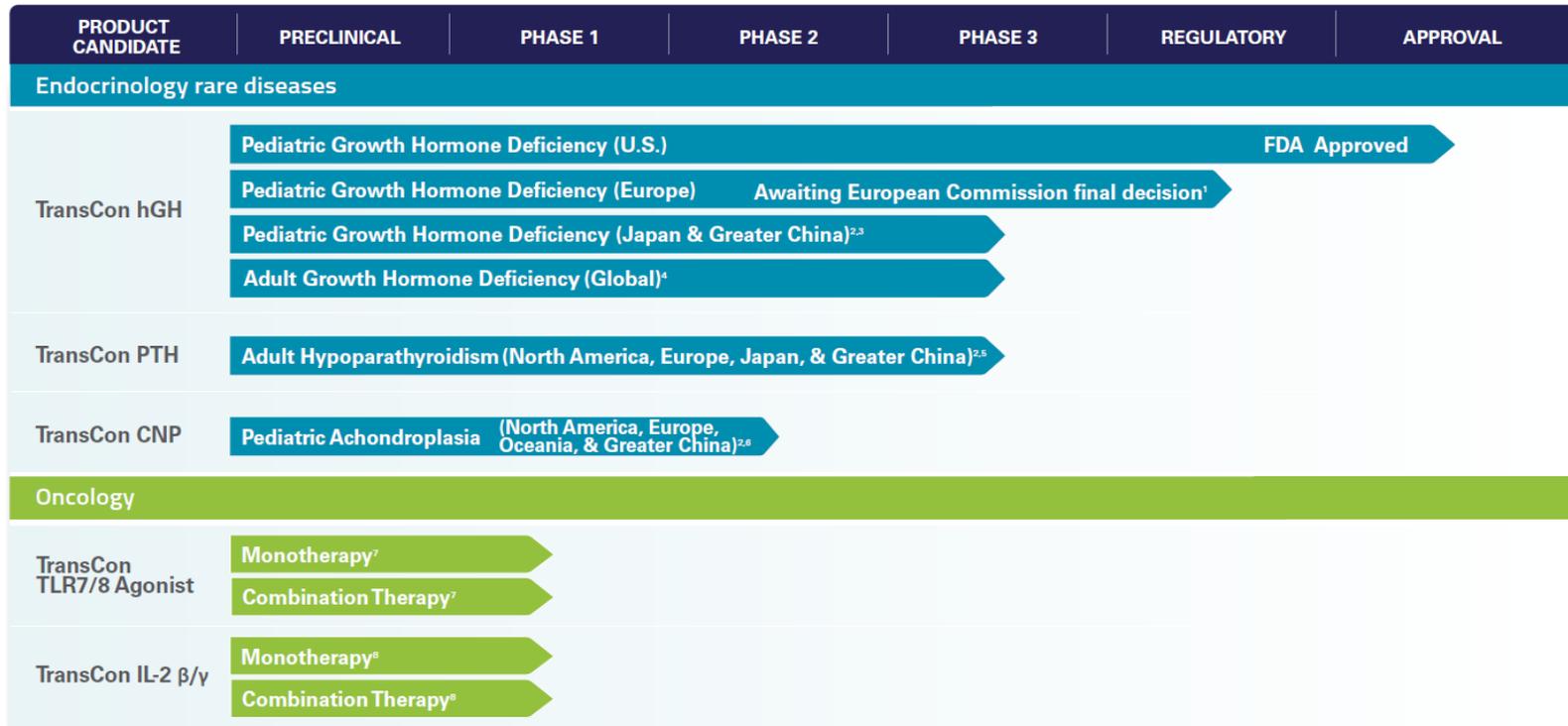
Strong anticipated profitability



* Ascendis Pharma internal estimates. Estimates are subject to significant business, economic, regulatory and competitive uncertainties and contingencies, including the outcome of clinical trials and regulatory review.

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Diverse Pipeline of Independent Product Candidates



¹Received positive CHMP opinion on November 12, 2021. Final EC decision expected within 67 days, or by end of January 2022.

²In development in Greater China through strategic investment in VISEN Pharmaceuticals.

³Japanese rGHt Trial.

⁴Global foresiGHt Trial.

⁵North American and European PaTHway Trial, Japanese PaTHway Japan Trial.

⁶North America, Europe, and Oceania ACcomplish Trial.

⁷transcendIT-101 Trial.

⁸L-Believe Trial.

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Endocrinology Rare Disease

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TransCon hGH

Once-Weekly Replacement Therapy

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SKYTROFA® (lonapegsomatropin-tcgd) FDA-Approved in the U.S.



First FDA-approved once-weekly product for pediatric growth hormone deficiency (GHD)

First FDA-approved product utilizing TransCon™ technology

In-use room temperature storage for 6 months

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SKYTROFA (lonapegsomatropin-tcgd): Selected Highlights of U.S. Prescribing Information

INDICATIONS AND USAGE SKYTROFA is a human growth hormone indicated for the treatment of pediatric patients 1 year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone (GH)

DOSAGE AND ADMINISTRATION SKYTROFA should be administered subcutaneously into the abdomen, buttock, or thigh with regular rotation of the injection sites.
The recommended dose is 0.24 mg/kg body weight once-weekly. See Full Prescribing Information for instructions on preparation and administration of drug.

CONTRAINDICATIONS

- **Acute critical illness** after open heart surgery, abdominal surgery or multiple accidental trauma, or those with acute respiratory failure due to the risk of increased mortality with use of pharmacologic doses of somatropin
- **Hypersensitivity** to somatropin or any of the excipients in SKYTROFA
- **Children with closed epiphyses**
- **Active malignancy**
- Active proliferative or severe non-proliferative **diabetic retinopathy**
- **Prader-Willi syndrome** who are severely obese, have a history of upper airway obstruction or sleep apnea or have severe respiratory impairment due to the risk of sudden death

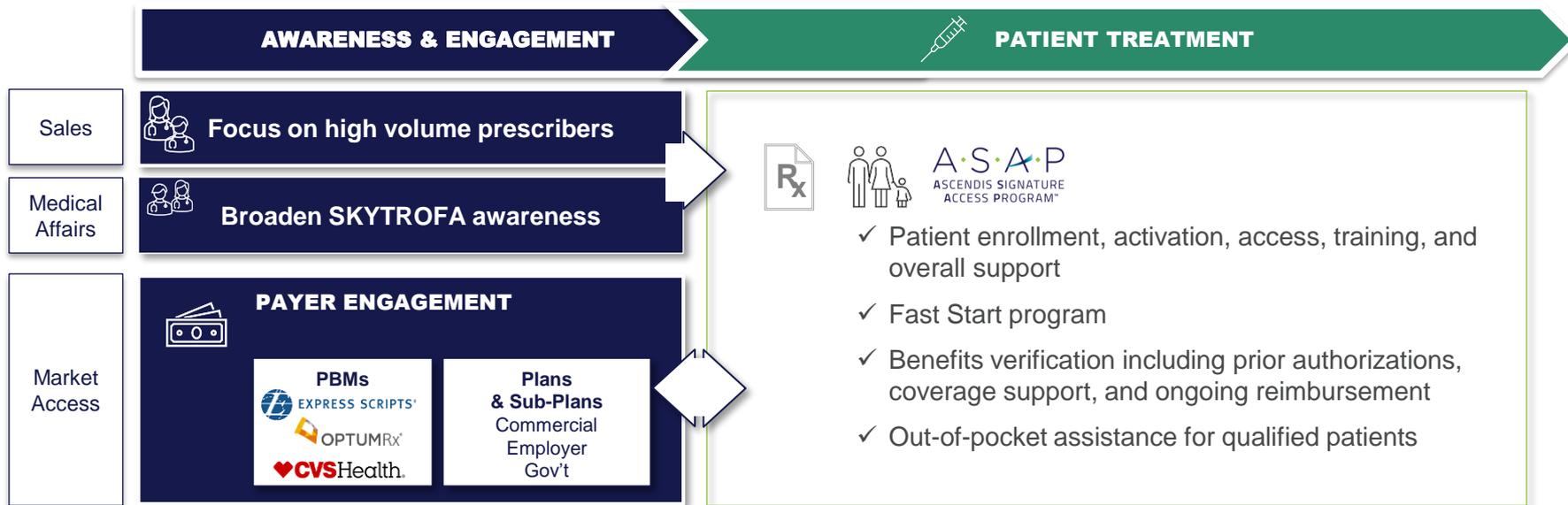
ADVERSE REACTIONS Most common adverse reactions ($\geq 5\%$) in a clinical trial were viral infection (15%), pyrexia (15%), cough (11%), nausea and vomiting (11%), hemorrhage (7%), diarrhea (6%), abdominal pain (6%), and arthralgia and arthritis (6%).

Reference: SKYTROFA® (lonapegsomatropin-tcgd) prescribing information, Ascendis Pharma
To report SUSPECTED ADVERSE REACTIONS, contact Ascendis Pharma, Inc., at 1-844-442-7236 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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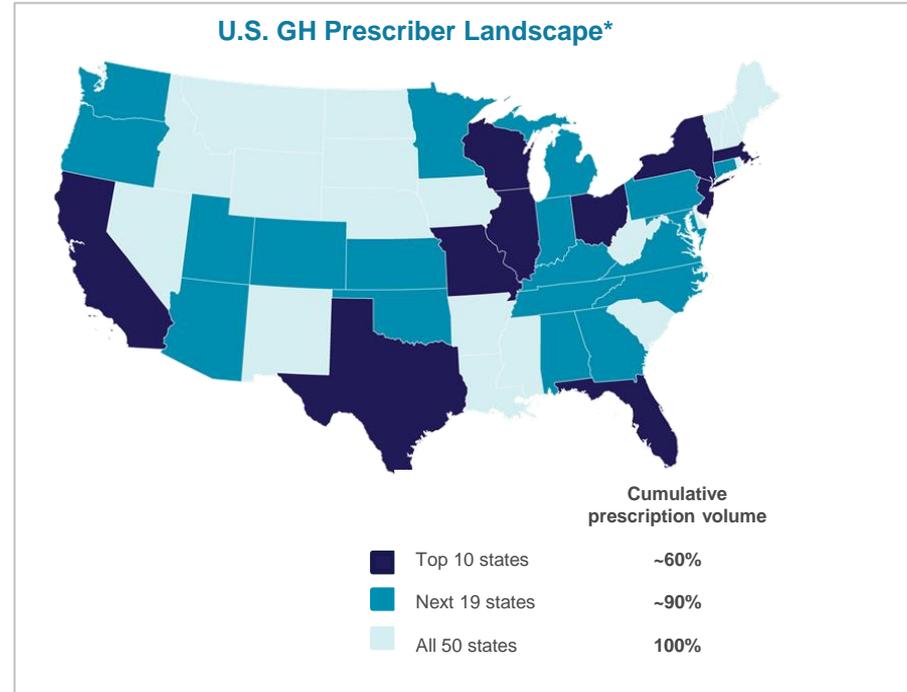
U.S. Commercial Focus: Building the Leading Brand



In-house teams with deep endocrine experience maximizing SKYTROFA awareness, patient treatment, and payer coverage

Positioned to Build Market Leadership

- Size of established U.S. commercial organization on par with current market leader
- U.S. sales team covers ~1,400 out of 7,000 prescribers who represent ~80% of the prescriptions
- Strong foundation established to support current and future product launches



Snapshot of First Two Months of Launch*



369 Prescriptions



139 Targeted prescribers



42% Repeat prescribers



Initial Payer Coverage

for pediatric GHD indication
in the U.S.



Plan Formulary Exceptions available
while formulary reviews take place

Physician enthusiasm for SKYTROFA reflected in increase in prescriptions,
submission of formulary exceptions, and repeat prescribers

* As of 12/31/21. Data on file. These numbers have not been verified by any third party and represent the Company's estimates as of the date indicated. The Company assumes no obligation to update them.

**MMIT Data as of 01/07/22

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Planned Expansion of Global Reach and Indications for TransCon hGH

- FDA Approved¹ for pediatric GHD in the U.S. under brand name SKYTROFA[®] (lonapegsomatropin-tcgd)
- Planned geographic expansion for pediatric GHD:

Europe	<ul style="list-style-type: none">• Received positive CHMP opinion• MAA approval expected by end of January 2022	
Japan	<ul style="list-style-type: none">• Pediatric GHD Phase 3 riGHt Trial• Enrollment ongoing	
Greater China²	<ul style="list-style-type: none">• Pediatric GHD Phase 3 trial in China• Target recruitment reached in Q1 2021	

- Planned label expansion

Adult GHD	<ul style="list-style-type: none">• Global foresiGHt Phase 3 trial• Enrollment ongoing	
Turner Syndrome	<ul style="list-style-type: none">• Protocol submission to FDA planned for Q2 2022	

¹SKYTROFA is a human growth hormone indicated for the treatment of pediatric patients 1 year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone (GH)

²Conducted by VISEN Pharmaceuticals.

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TransCon hGH: Potential to Transform the Global hGH Market

- In a Phase 3 clinical trial, once-weekly TransCon hGH demonstrated higher annualized height velocity at 52 weeks with comparable safety and immunogenicity to daily somatropin
- Additional studies planned and/or underway to potentially expand geographic reach and label indication beyond pediatric GHD in the U.S.
- TransCon hGH has the potential to increase the size of the estimated \$4B global hGH market through increased adherence, persistence, and penetration

Upcoming milestones:

- EC MAA approval for pediatric growth hormone deficiency expected by end of January 2022
- Planned protocol submission to FDA for Turner Syndrome in Q2 2022
- Complete enrollment in Phase 3 foresiGHt Trial in adult GHD expected in Q2 2022

Ascendis aspires to become the global leader in the hGH market

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TransCon PTH

PTH Replacement Therapy for Hypoparathyroidism

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Chronic Hypoparathyroidism: Significant Patient Population

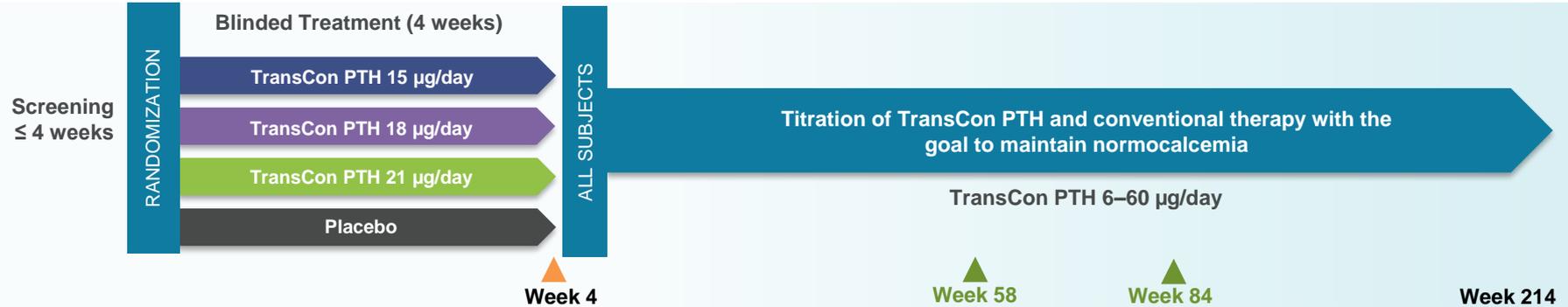
Estimated Prevalence: ~400k in these 5 regions



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TransCon PTH PaTH Forward (Phase 2) Trial Design

Adults with hypoparathyroidism who required conventional therapy (active vitamin D + calcium) at baseline



Primary Composite Endpoint (4 weeks)

Proportion of subjects with:

- Normal serum calcium; **and**
- Independence from active vitamin D; **and**
- Requiring ≤ 1,000 mg/day calcium supplements; **and**
- Normal FECa (or at least 50% decrease from baseline)

Open Label Extension Endpoints

- Intake of active vitamin D and calcium supplements
- Serum calcium and phosphate
- 24-hour urine calcium
- Adverse events
- Bone mineral density (Week 58 only)
- Patient-reported outcomes (Week 58 only)

- 58 subjects completed 84 weeks of follow-up*
- Continued treatment with TransCon PTH demonstrated that:
 - Mean serum calcium remained stable and in the normal range
 - 93% of subjects were free from active vitamin D and were taking ≤ 600 mg/day of calcium supplements
- TransCon PTH was well-tolerated at all doses administered through week 84 in PaTH Forward
 - No treatment-related serious or severe adverse events occurred, and no treatment-emergent adverse events (TEAEs) led to discontinuation of study drug

Week 84 data continue to support TransCon PTH as a potential hormone replacement therapy for adults with hypoparathyroidism

*As of January 5th, 2022, 57 subjects continue in the open-label extension; One subject withdrew after week 97, not related to study drug.

**Not taking active vitamin D and taking ≤ 600 mg/day of calcium supplements.
Data on file, Ascendis Pharma; PaTH Forward week 84 top-line data; Q4 2021.

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- TransCon PTH was well-tolerated at all doses administered
- No drug-related serious TEAEs were reported
- No TEAEs leading to discontinuation of study drug
- TEAEs with TransCon PTH reflect known PTH pharmacology
- Injections were well-tolerated using pen injector planned for commercial presentation

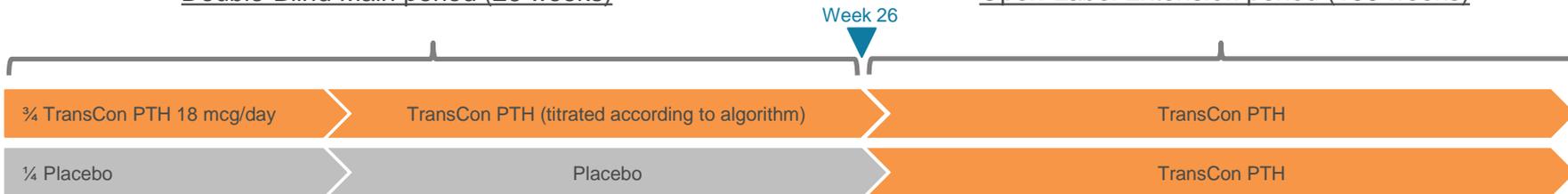
No subjects had TEAEs related to hyper- or hypocalcemia leading to ER/urgent care visit and/or hospitalization

TransCon PTH PaTHway (Phase 3) Trial Design

Double-blind, placebo-controlled trial with an open-label extension period
82¹ adults with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo)

Double-Blind Main period (26 weeks)

Open-Label Extension period (156 weeks)



Primary Objective

Confirm treatment effect of TransCon PTH in adults with hypoparathyroidism

Key Eligibility Criteria

- Adults with chronic hypoparathyroidism (*i.e.* for at least 26 weeks)
- Age \geq 18 years
- Reliant on calcitriol \geq 0.50 mcg per day or alfacalcidol \geq 1.0 mcg per day, **and** therapeutic elemental calcium \geq 800 mg/day
- Serum calcium in normal (or just below normal) range: 7.8–0.6 mg/dL (1.96–2.64 mmol/L)
- No PTH or PTHrP therapy within 4 weeks prior to Screening

Countries

- Europe (Germany, Denmark, Norway, Italy, Hungary)
- North America (United States, Canada)

Primary Composite Endpoint at Week 26

Proportion of subjects with:

- Serum calcium in the normal range (8.3–10.6 mg/dL) **and**
- Independence from active vitamin D **and**
- Independence from calcium supplements²

Selected Other Endpoints at Week 26

- 24-hour urine calcium
- Serum phosphate levels
- Domains from Hypoparathyroidism Patient Experience Scale measures
- Domains from 36-Item Short Form Survey (SF-36) measure

¹ Sample size selected to ensure evaluable data for 68.

² If needed to meet recommended dietary intake of calcium, it is permitted to take calcium supplements \leq 600 mg/day as a nutritional supplement.

PaTHway Phase 3 Trial: Baseline Characteristics

Characteristics	Total Randomized (N = 82)
Age, mean years	49 years
Female sex, %	78%
Geographic region, %	
North America	62%
Europe	38%
Postmenopausal, %	28%
Duration of hypoparathyroidism, mean years	12 years
Post-surgical etiology of hypoparathyroidism, %	85%

Baseline characteristics of Phase 3 trial are similar to those of the Phase 2 trial

Potential New Treatment Paradigm for Hypoparathyroidism

- TransCon PTH has the potential to be the first hormone replacement therapy for hypoparathyroidism addressing major unmet medical need for a large rare disease patient population

Upcoming milestones:

- Phase 3 PaTHway Trial (North America + EU) top-line results expected in Q1 2022
- Planned NDA submission to FDA in Q3 2022
- PaTHway Japan Trial top-line results expected in Q3 2022
- Planned MAA submission to EMA in Q4 2022
- Planned IND or equivalent submission for pediatric hypoparathyroidism in Q4 2022

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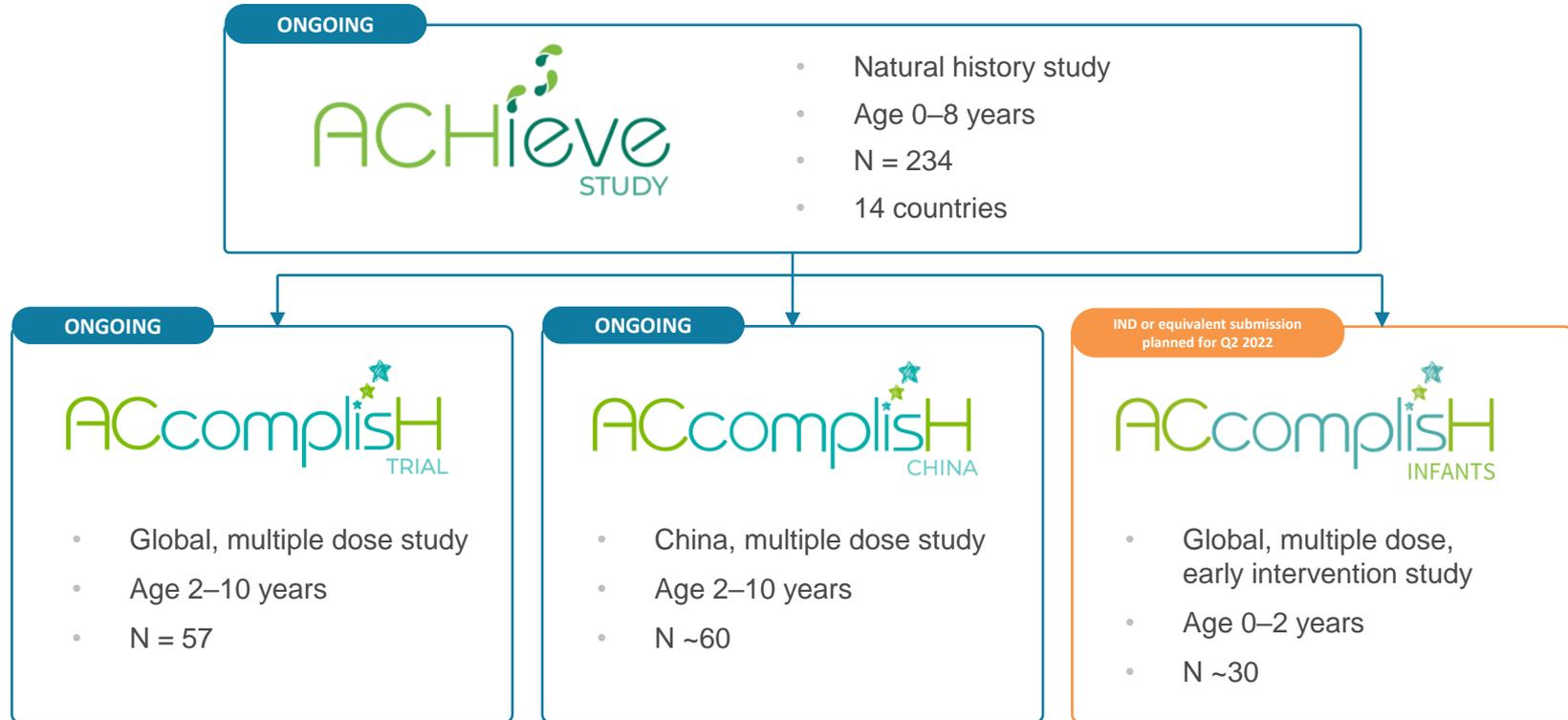


TransCon CNP

Potential New Once-Weekly Growth Treatment Option

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TransCon CNP: Clinical Development Program

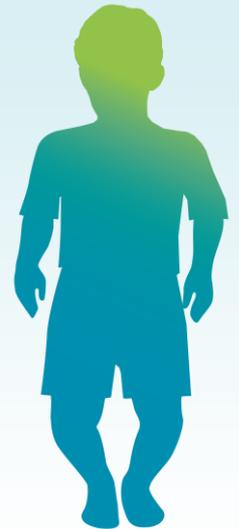


TransCon CNP Clinical Program Is Progressing

- ACcomplishH enrollment completed (N = 57)
- Interim blinded data informed dose selection of 50 and 100 $\mu\text{g}/\text{kg}/\text{week}$ for ACcomplishH China
- Preliminary PK and safety data on TransCon CNP suggest continuous CNP is well-tolerated across all doses administered
- ACcomplishH Infant in children 0–2 years of age; evaluate early intervention in ACH to prevent growth disorder progression; safety, PK

Upcoming milestones:

- Planned IND or equivalent submission for ACcomplishH Infants Trial in Q2 2022
- Phase 2 ACcomplishH Trial top-line data expected in Q4 2022





Oncology

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Initial Interim Data Indicate TransCon TLR7/8 Agonist Was Well-Tolerated as Monotherapy or in Combination with Pembrolizumab*

- Initial data* from ongoing TransCon TLR7/8 Agonist first-in-human trial (transcendIT-101) indicated:
 - Observed low systemic exposure and mean plasma half-life of ~7 days based on early PK data
 - Target engagement in tumor at least 7 days post-dose
 - Early signs of activity in three out of three efficacy-evaluable patients including those previously treated with checkpoint inhibitors; abscopal effect observed in one patient on monotherapy
- No dose-limiting toxicities observed to date
- Limited safety-evaluable population (n = 8) so far demonstrated no systemic side effects related to TransCon TLR7/8 Agonist
 - Consistent with low systemic exposure of resiquimod
- TransCon TLR7/8 Agonist-related AEs observed as monotherapy or in combination with pembrolizumab
 - Transient, mild injection site-related reactions (Grade 1/2)
 - No Grade 3 or higher related AEs; No related Serious AEs observed

*Datacut date: 16 Nov 2021

Designing a Portfolio to Transform Cancer Immunotherapy Treatment

- TransCon TLR7/8 Agonist has the potential for:
 - Sustained immune activation
 - Systemic anti-tumor response with dosing interval of at least three weeks
- Create a pipeline using TransCon technologies that may enable a new treatment paradigm building on well-known biology:
 - TransCon IL-2 β/γ first-in-human trial (IL- β elieve; NCT05081609) is now open for enrollment

Upcoming milestones:

- TransCon IL-2 β/γ first patient dosed in combo-therapy and dose escalation expected in Q1 2022
- TransCon TLR7/8 Agonist top-line monotherapy & combo-therapy dose escalation data expected in Q3 2022
- TransCon IL-2 β/γ monotherapy top-line results expected in Q4 2022
- Planned IND or similar submission for Phase 2 cohort expansion for combination TransCon TLR7/8 Agonist and TransCon IL-2 β/γ therapy in Q4 2022

Selected Milestones Expected in 2022

Q1 2022	Q2 2022	Q3 2022	Q4 2022
<p>TransCon hGH <i>Pediatric GHD</i> EC MAA Approval</p>	<p>TransCon hGH <i>Turner Syndrome</i> Submit protocol to FDA</p>	<p>TransCon PTH <i>Adult PTH</i> Submit NDA to FDA</p>	<p>Announce 3rd Therapeutic Area</p>
<p>TransCon PTH <i>Adult PTH</i> Phase 3 top-line results PaTHway Trial</p>	<p>TransCon hGH <i>Adult GHD</i> Complete enrollment foresiGHt Trial</p>	<p>TransCon TLR7/8 Agonist <i>Cancer Immunotherapy</i> Top-line monotherapy & combo-therapy dose escalation data transcendIT-101 Trial</p>	<p>TransCon PTH <i>Adult PTH</i> Submit MAA to EMA</p>
<p>TransCon IL-2 β/γ <i>Cancer Immunotherapy</i> First patient dosed in combo- therapy and dose escalation IL-βelieγe Trial</p>	<p>TransCon CNP <i>Achondroplasia</i> Submit IND or equivalent ACcomplish Infants Trial</p>		<p>TransCon CNP <i>Pediatric Achondroplasia</i> Phase 2 top-line results ACcomplish Trial</p>
			<p>TransCon IL-2 β/γ <i>Cancer Immunotherapy</i> Monotherapy top-line results IL-βelieγe Trial</p>
			<p>Combo Cancer Immunotherapy Submit IND or similar for Phase 2 cohort expansion TransCon TLR7/8 Agonist and TransCon IL-2 β/γ</p>

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Thank you

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