



# Ascendis Pharma A/S

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Healthcare Conference  
January 8, 2024

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This presentation contains forward-looking statements. All statements other than statements of historical facts contained in this presentation, such as statements regarding our products and prospective product candidates; clinical trial results; the expected timing of future clinical trial results and feedback from regulatory authorities; the scope, progress, results and costs of developing our product candidates or any other future product candidates; timing and likelihood of success; plans and objectives of management for future operations and commercialization activities; and future results of current and anticipated products and product candidates are forward-looking statements. These forward-looking statements are based on our current expectations and beliefs, as well as assumptions concerning future events. These statements involve known and unknown risks, uncertainties and other factors that could cause our actual results to differ materially from the results discussed in the forward-looking statements. These risks, uncertainties and other factors are more fully described in our reports filed with or submitted to the Securities and Exchange Commission (SEC), including, without limitation, our most recent Annual Report on Form 20-F filed with the SEC on February 16, 2023 particularly in the sections titled “Risk Factors” and “Operating and Financial Review and Prospects.” In light of the significant uncertainties in our forward-looking statements, you should not place undue reliance on these statements or regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all.

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# Executive Summary

- Validated TransCon technology platform and algorithm for product innovation
  - Two approved products – SKYTROFA® (TransCon hGH) and YORVIPATH® (TransCon PTH)
  - Pivotal data and submission of NDA for TransCon CNP expected in Q4 2024
  - Oncology portfolio Phase 2 data expected in Q4 2024
  - Creation of Ophthalmology NewCo, financed by institutional investors, expected in Q1 2024
- SKYTROFA achieved U.S. market value leadership in 2023
  - Expected full year 2023 SKYTROFA revenue of ~€179 million<sup>1</sup>
  - Full year 2024 SKYTROFA revenue expected to be €320 to €340 million<sup>2</sup>
- YORVIPATH® first launch in Germany January 2024
  - Rolling out to Europe Direct and International Markets starting in 2024
  - In the U.S., PDUFA goal date of May 14, 2024, and launch expected the following quarter, if approved
- Business model built on fast, successful drug development and commercial therapeutic synergies
- Expect to be operating cash flow breakeven on a quarterly basis by the end of 2024

<sup>1</sup> Unaudited preliminary estimate.

<sup>2</sup> Based on average 2023 exchange rates.

# 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**

Ascendis Pharma's 2020 - 2025 strategic roadmap

# Endocrinology Rare Disease Pipeline & Ascendis Direct Commercialization

# Endocrinology Rare Disease Products Approved in Major Markets

## TransCon hGH

- Pediatric Growth Hormone Deficiency



- **United States<sup>1</sup>**  
Approved in the U.S. as SKYTROFA (lonapegsomatropin-tcgd)



- **European Union<sup>2</sup> and Selected Other Countries<sup>3</sup>**  
Approved in the EU as SKYTROFA (lonapegsomatropin)

## TransCon PTH

- Adult Chronic Hypoparathyroidism



- **European Union<sup>4</sup> and Selected Other Countries<sup>5</sup>**  
Approved in the EU as YORVIPATH (palopegteriparatide)  
First country launch planned in Germany in January 2024

<sup>1</sup> SKYTROFA® [package insert]. Palo Alto, CA: Ascendis Pharma Inc. October 2022.

<sup>2</sup> SKYTROFA® SMPC. Hellerup, Denmark: Ascendis Pharma Inc. January 2022.

<sup>3</sup> SKYTROFA® is also approved in Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland).

<sup>4</sup> YORVIPATH® SMPC. Hellerup, Denmark: Ascendis Pharma Inc. November 2023.

<sup>5</sup> YORVIPATH® is also approved in Norway and Iceland.

# TransCon hGH: Expanding Beyond Pediatric GHD

- Pediatric GHD accounts for approximately 50% of the hGH market by patients
- Growth hormone plays an essential role in the health of children and adults
  - Maintains normal body composition and cardiometabolic health
  - Promotes normal growth in children
- Daily hGH is approved for multiple indications in adult and pediatric patients
  - Adult GHD accounts for approximately 10% of the daily hGH market
  - However, fewer than 10% of adults with suspected GHD<sup>1</sup> are treated with hGH
- TransCon hGH Phase 3 foresiGHt Trial in adult GHD
  - Demonstrated superiority to placebo on primary and secondary efficacy endpoints
  - Demonstrated comparable results to daily hGH on primary and secondary endpoints<sup>2</sup>
  - Expected sBLA submission in Q2 2024

<sup>1</sup> A. Hoffman et al (2023). SAT611 Prevalence Of Comorbidities Among Treated And Untreated Adults With Suspected Growth Hormone Deficiency. *Journal of the Endocrine Society*. 7. 10.1210/endo/bvad114.1344.

<sup>2</sup> Exploratory post-hoc analysis for patients with IGF-1 SDS levels  $\leq 1.75$  at Week 38.

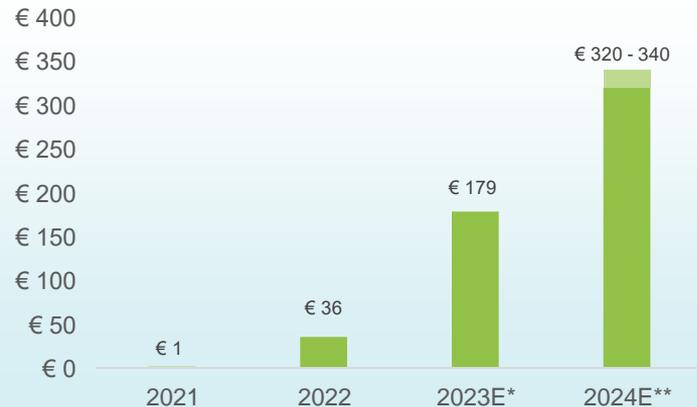
# SKYTROFA Revenue Development and Expectations

- SKYTROFA was launched in U.S. in Q4 2021, Germany in Q3 2023
  - SKYTROFA Q4 2023 revenue of ~€64 million\*
  - Estimated SKYTROFA U.S. pediatric GHD market penetration exiting 2023 at ~16%
  - Expect full year 2024 SKYTROFA revenue of €320 - €340 million\*\*

Quarterly SKYTROFA Revenue (million)



Annual SKYTROFA Revenue (million)



\* Unaudited preliminary estimate.

\*\* Reflects 2024 SKYTROFA revenue expectations announced on Jan. 7, 2024, based on average 2023 exchange rates.

# TransCon hGH Program Summary and Outlook

- Attained U.S. market value leadership in 2023
- Completed enliGHten Trial in pediatric GHD<sup>1</sup>
  - Majority of patients met or exceeded average parental height SDS
  - Demonstrated the long-term safety in patients treated up to 6 years
- Planned sBLA submission to FDA for adult GHD Q2 2024
- Topline results from Phase 2 trial in Turner syndrome expected Q4 2024
- Expanding reach for SKYTROFA through Ascendis commercial organization building on therapeutic synergies
- Full year 2024 SKYTROFA revenue expected to be €320 to €340 million<sup>2</sup>

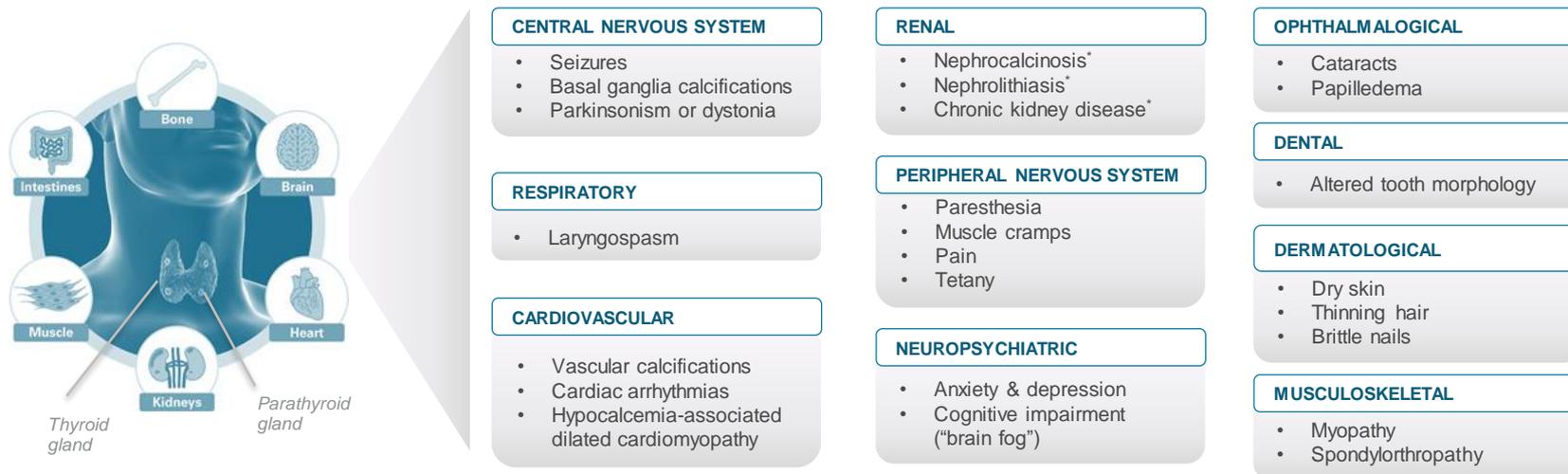
Committed to making TransCon hGH the global leading product in value  
in a growing growth hormone market

<sup>1</sup> Vlachopapadopoulou et al. Poster LB17, presented at ESPE 2023.

<sup>2</sup> Based on average 2023 exchange rates.

# Hypoparathyroidism: One Disease, Multiple Consequences<sup>1-4</sup>

Absence or deficiency of parathyroid hormone (PTH) is linked to multi-organ manifestations<sup>5,6</sup>



\* These manifestations are mostly the result of treatment with calcium supplements and activated vitamin D rather than of the disease itself.<sup>5</sup>

<sup>1</sup> Underbjerg L, Sikjaer T, Mosekilde L, et al. Cardiovascular and renal complications to postsurgical hypoparathyroidism: a Danish nationwide controlled historic follow-up study. *J Bone Miner Res.* 2013;28(11):2277-2285. doi:10.1002/jbmr.1979.

<sup>2</sup> Underbjerg L, Sikjaer T, Mosekilde L, et al. The epidemiology of nonsurgical hypoparathyroidism in Denmark: a nationwide case finding study. *J Bone Miner Res.* 2015;30(9):1738-1744. doi:10.1002/jbmr.2501.

<sup>3</sup> Shoback DM, Bilezikian JP, Costa AG, et al. Presentation of hypoparathyroidism: etiologies and clinical features. *J Clin Endocrinol Metab.* 2016;101(6):2300-2312. doi:10.1210/clinem.2015-3909.

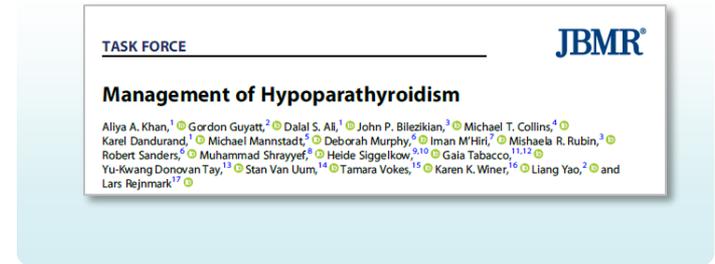
<sup>4</sup> Underbjerg L, Sikjaer T, Mosekilde L, et al. The epidemiology of nonsurgical hypoparathyroidism in Denmark: a nationwide case finding study. *J Bone Miner Res.* 2015;30(9):1738-1744. doi:10.1002/jbmr.2501.

<sup>5</sup> Mannstadt M, Bilezikian JP, Thakker RV, et al. Hypoparathyroidism. *Nat Rev Dis Primers.* 2017;3:17055. doi:10.1038/nrdp.2017.55.

<sup>6</sup> Shoback DM, Bilezikian JP, Costa AG, et al. Presentation of hypoparathyroidism: etiologies and clinical features. *J Clin Endocrinol Metab.* 2016;101(6):2300-2312. doi:10.1210/clinem.2015-3909.

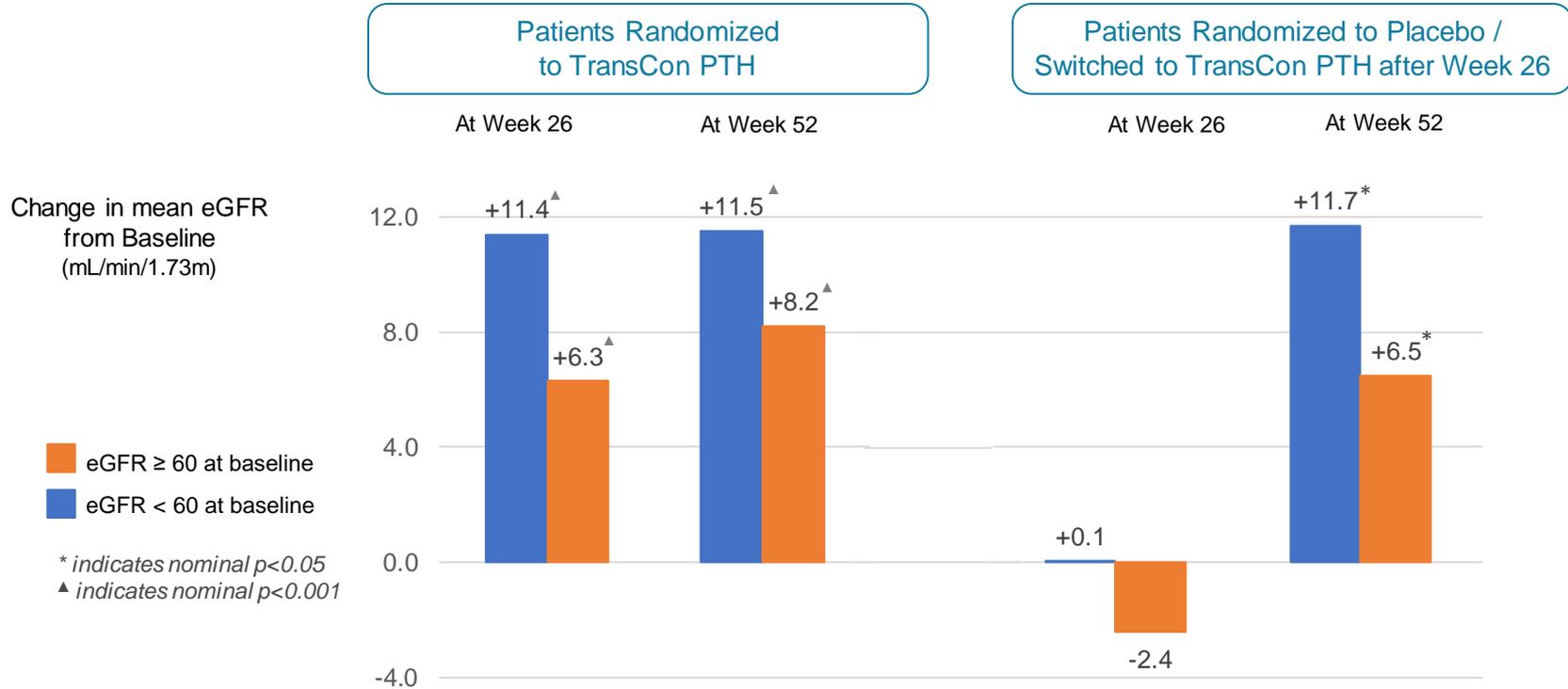
# Latest Clinical Practice Guideline

- Consider PTH replacement therapy in patients not adequately controlled on conventional therapy
- Inadequate control is considered to be any one of the following:
  - Symptomatic hypocalcemia
  - Hyperphosphatemia
  - Renal insufficiency
  - Hypercalciuria
  - Poor quality of life
- In addition, individuals with poor compliance, malabsorption, or who are intolerant of large doses of calcium and active vitamin D may also benefit from PTH replacement therapy



<sup>1</sup>: Aliya A Khan et al, The Second International Workshop on the Evaluation and Management of Hypoparathyroidism, Journal of Bone and Mineral Research, 10, 1002/jbmr.4671.

# Significantly Improved Renal Function with TransCon PTH Treatment in Phase 3 PaTHway Trial *Post Hoc* Analysis



eGFR (an assessment of kidney filtering capacity) was calculated by the trial's central lab using the Modification of Diet in Renal Disease Study Group (MDRD) equation (Levey, Ann Intern Med 2006). Data on file.

# Ascendis Commercial Organization - TransCon PTH Launch

- Europe Direct<sup>1</sup> – Germany
  - ~70,000 adults<sup>2</sup> with chronic hypoparathyroidism in Germany
  - Launch of YORVIPATH planned in January 2024, initial list price<sup>3</sup> of €105,000 per patient per year
- Europe Direct – other selected countries
  - Over 100,000 additional adults with chronic hypoparathyroidism
  - Next major market expected end of 2024, expected availability across Europe by end of 2025
  - Providing commercial product through early access routes, such as ‘named patient,’ until commercial reimbursement established
- U.S.
  - Over 80,000<sup>4</sup> adults with chronic hypoparathyroidism in the U.S.
  - PDUFA date May 14, 2024; expect to launch in Q3 2024, if approved
  - Experienced commercial team in place for rapid, efficient launch

<sup>1</sup> DACH, France & BeNeLux, Iberia, Italy, Nordics, UK & Ireland.

<sup>2</sup> German SHI claims data from the Institute for Applied Health Research Berlin (Institut für angewandte Gesundheitsforschung Berlin - InGef) research database.

<sup>3</sup> Herstellerabgabepreis.

<sup>4</sup> U.S. prevalence literature review and epi meta-analysis (Powers, Clarke, Milliman project, ipm.ai claims project; HCUPnet, Healthcare Cost and Utilization Project. Agency for Healthcare Research and Quality, Rockville, MD. for surgical cohort projection).

# TransCon PTH Program Summary and Outlook

- Completed two successful Phase 3 trials: PaTHway and PaTHway Japan
- *Post hoc* analysis showed significantly improved renal function in TransCon PTH-treated patients
- Approved in European Union and other territories as YORVIPATH
  - German launch of YORVIPATH planned in January 2024
  - Rolling out to Europe Direct and International Markets starting in 2024
- In the U.S., PDUFA date of May 14, 2024
  - U.S. launch of YORVIPATH expected in Q3 2024, if approved
- Launching TransCon PTH through established, proven endocrinology rare disease infrastructure

# Changing the Treatment Paradigm of Achondroplasia

Current Consensus

Updated View

Primary Manifestations

Growth disorder

Growth disorder

Muscle disorder

Secondary Manifestations

Height

ACH-AE

HRQoL

Muscle Strength/  
Stamina

Height

ACH-AE

HRQoL

Muscle Strength/  
Stamina

← direct consequence

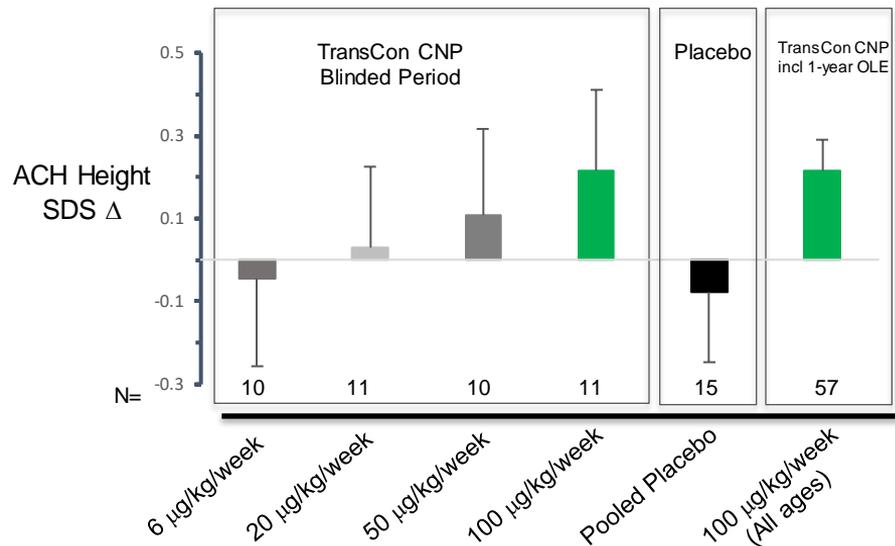
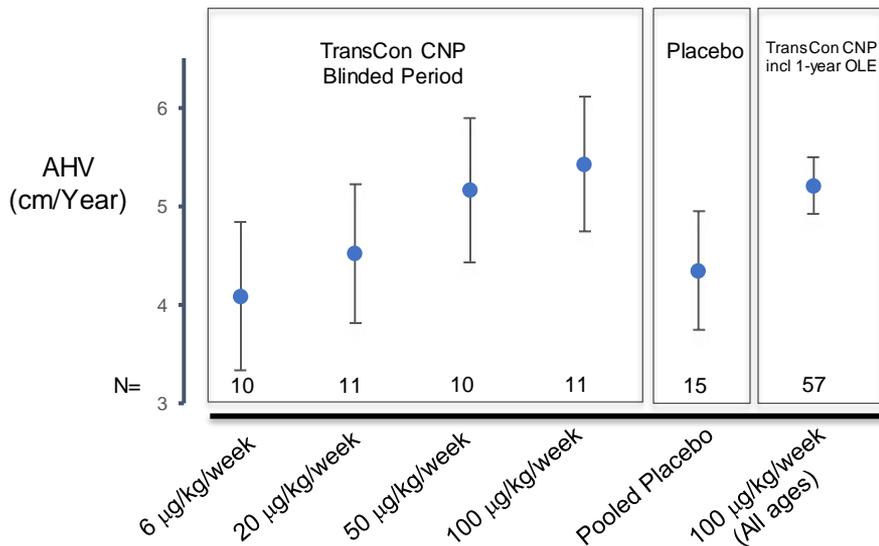
← indirect/secondary consequence

Address all aspects of achondroplasia for patients of all ages

ACH-AE: Increased incidence of Achondroplasia-related Adverse Events.

HRQoL: Reduced Health-Related Quality of Life; Height; Reduced height. Muscle Strength/Stamina; Reduced muscular functionality, including reduced strength and stamina

# Treatment Effect of TransCon CNP 100 µg/kg/week, Age 2-10 years



Growth across full trial population (n=57) on TransCon CNP 100 µg/kg/week for 52 weeks consistent with results from randomized period

ANCOVA model. Data on file.

AHV: Annualized height velocity; ACH: achondroplasia; SDS: standard deviation score; OLE: open-label extension.

TransCon CNP is an investigational product candidate.

For investor communication only. Not for use in product promotion. Not for further distribution.

# Health-Related Quality-of-Life Measures (HRQoL)

## SF-10™ Health Survey for Children

A 10-item non-disease specific survey of a child's functional health and well-being.<sup>1</sup>

Physical measures include assessments of:

- **Limitations** in activities, schoolwork
- **Bodily pain** or discomfort
- **Overall health** relative to other children
- **Physical coordination** or clumsiness

Lower scores indicate greater impairment.

Validated in children ages 5 yrs and older.

## Achondroplasia Child Experience Measure

A condition-specific clinical outcome measure that assesses the impact of achondroplasia on a child's health-related quality of life.

Includes assessments of:<sup>2</sup>

- **Daily Living** – Reach objects or high places. Toilet themselves. Bath, shower, wash. Perform fine motor skill tasks.
- **Emotional Well-Being** – Feeling different, frustrated, etc.
- **Physical Functioning** – Sit for long periods of time. Climb stairs. Be physically active. Walk long distances. Run.
- **School** – Difficulty participating. Limited or modified gym class participation. Missed school.

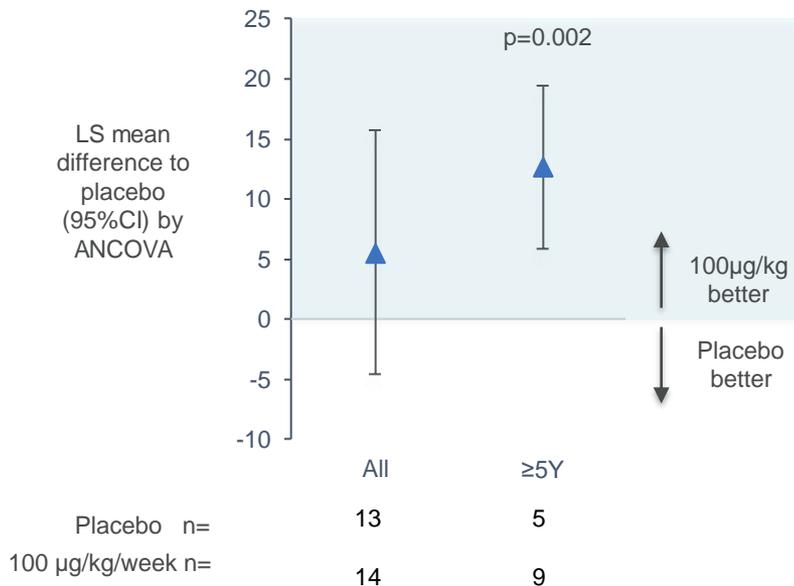
The measure is scored on a 0-100 scale, with higher scores indicating greater impairment.

<sup>1</sup> SF-10 Health Survey for Children™ | QualityMetric Accessed 28 December 2023.

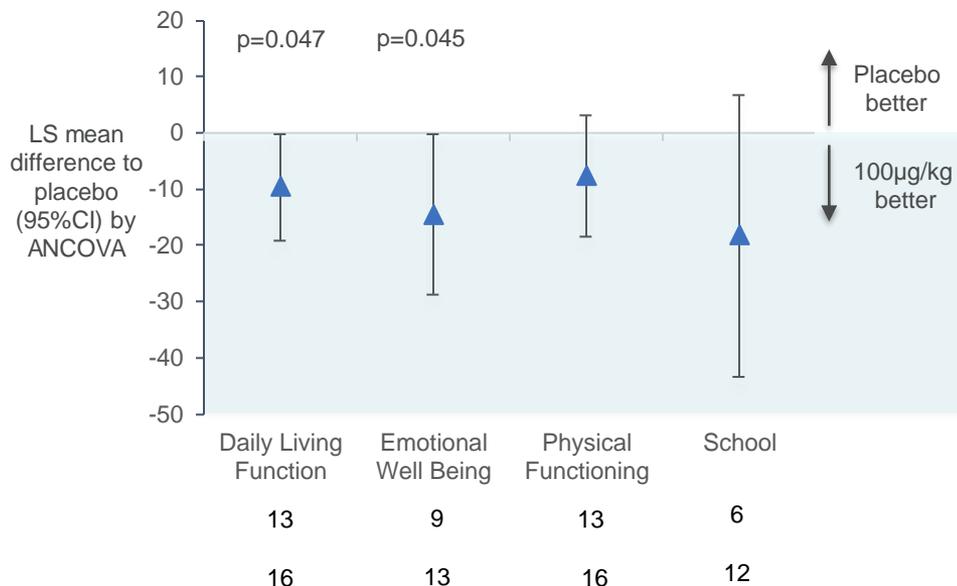
<sup>2</sup> ACEM-Impact | Achondroplasia Child Experience Measure - Impact described in ePROVIDE (mapi-trust.org) Accessed 28 December 2023.

# Consistent Positive Treatment Effect Across HRQoL Domains

## SF-10 Physical Summary



## Achondroplasia Child Experience Measure



Patients dosed with TransCon CNP demonstrated HRQoL improvements in multiple domains compared to placebo

All participants in blinded or OLE period of ACcomplish who were initiated directly on 100µg/kg/week treatment (total n=19) compared to all participants from blinded period receiving placebo (n=15). All domains are missing some data points. Nominal p-values. Data on file.

TransCon CNP is an investigational product candidate.

# TransCon hGH / CNP Combination Trial - COACH Trial

- Phase 2 open-label, single-arm trial (age 2-11 years), Clinical Trial Application (CTA) submitted
  - Primary objectives: evaluate treatment effect on linear growth and safety
  - Secondary objectives: evaluate treatment effect on quality of life, radiological endpoints, physical functioning, and body composition
- Clinical design
  - Cohort A: treatment naïve (n=12)
  - Cohort B: prior treatment with TransCon CNP (100 µg/kg/week) for at least 1 year (n=6)
  - Treatment with TransCon CNP (100 µg/kg/week) and TransCon hGH (0.30 mg/kg/week)
- Interim analysis at 26 Weeks, full analysis at 52 Weeks
  - Week 26 topline AHV data expected Q4 2024

# TransCon CNP Program Summary and Outlook

- Pediatric patients:
  - Topline data from pivotal ApproaCH Trial expected Q4 2024
  - COACH Trial (hGH/CNP combination) Week 26 topline data expected Q4 2024
  - Plan to submit NDA to FDA in Q4 2024 for the treatment of achondroplasia
- Infant patients (age 0-2 years) CTA submitted:
  - 52-week randomized, double-blind, placebo-controlled trial
  - Primary endpoints: incidence of TEAEs and change in length/height Z-score
- Adult patients:
  - IND or similar for trial in adults with achondroplasia planned for Q4 2024

TransCon CNP treatment goal to improve linear growth, quality of life, radiological endpoints, physical functioning, and body composition

## Global Commercial Presence Through Partners with Local Expertise and Infrastructure

# Growing Commercial Presence – Rest of the World

- China
  - VISEN has exclusive license agreement for TransCon hGH, TransCon PTH, and TransCon CNP
  - VISEN's TransCon hGH Phase 3 and TransCon CNP Phase 2 trials completed
- Japan
  - Teijin has exclusive license agreement for TransCon hGH, TransCon PTH, and TransCon CNP
  - Phase 3 PaTHway Japan Trial completed
- International Markets for Endocrinology Rare Disease programs
  - Expanding global reach through exclusive distribution agreements with geographic market leaders
  - Three regional agreements established as of January 2024:
    - Specialised Therapeutics Asia Pte Ltd. (Australia, New Zealand, Singapore, Malaysia, Brunei, Thailand and Vietnam)
    - Er-Kim (Central & Eastern Europe and Turkey)
    - Vector Pharma FZCO (Saudi Arabia, United Arab Emirates, Kuwait, Oman, Qatar, and Bahrain)

# Financial Update & 2024 Selected Milestones

## Preliminary unaudited 2023 results

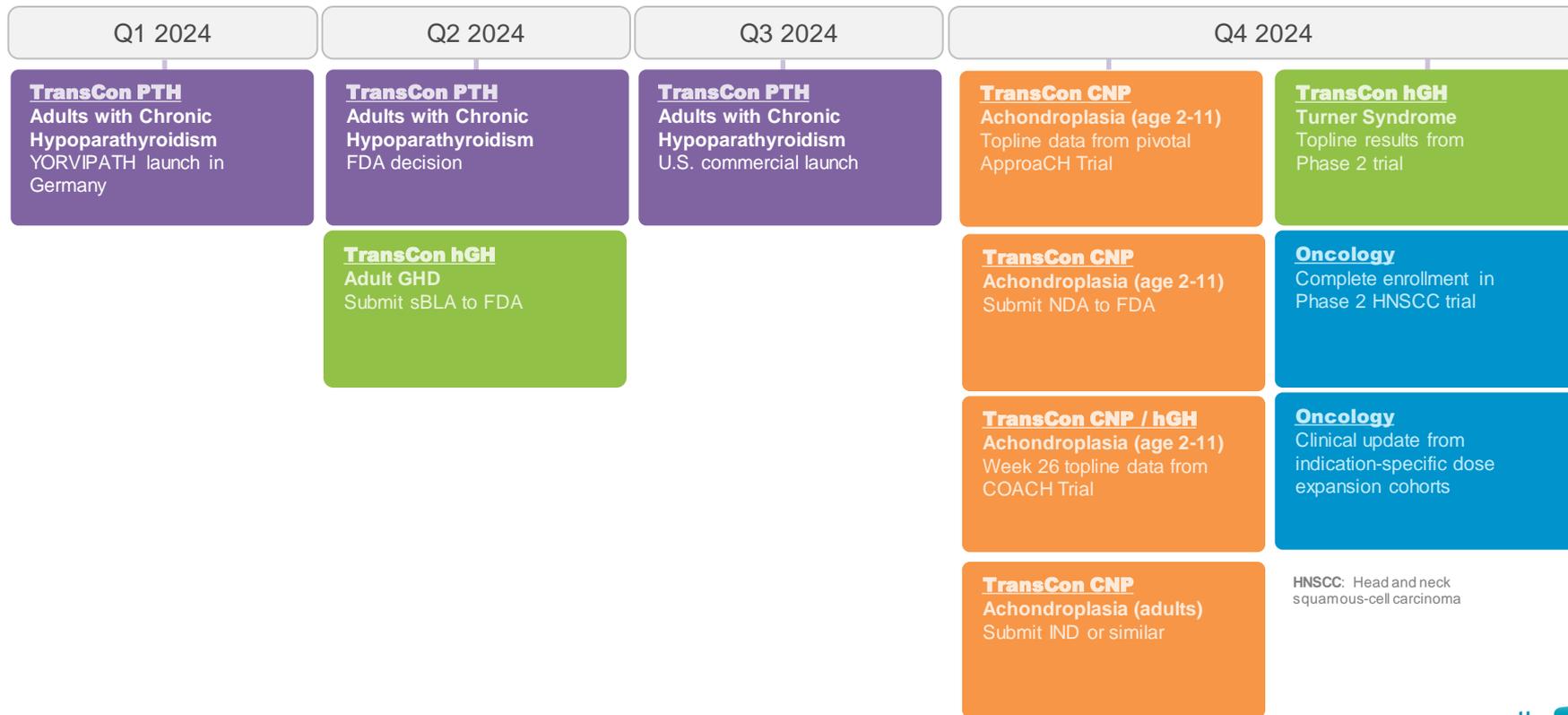
- SKYTROFA Q4 revenue of ~€64 million; full year 2023 SKYTROFA revenue of ~€179 million
- Full year 2023 total revenue of ~€267 million
  - Includes \$70 million Teijin upfront payment as non-product revenue in Q4
- December 31, 2023 cash, cash equivalents, and marketable securities of ~€400 million

## Full Year 2024 outlook based on current plans

- Revenue
  - Expect SKYTROFA revenue of €320 to €340 million<sup>1</sup>
  - Expect to provide YORVIPATH revenue update during 2024
- Operating expenses (SG&A and R&D) expected to be ~€600 million
- Operating cash flow: expect to be breakeven on quarterly basis by end of 2024

<sup>1</sup> Based on average 2023 exchange rates.

# Selected Milestones Expected in 2024



HNSCC: Head and neck squamous-cell carcinoma

## Introducing Vision 2030

# Vision 2030

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

### • Be the Leading Endocrinology Rare Disease Company

- Achieve blockbuster status (>\$1B) for TransCon PTH, TransCon hGH, and TransCon CNP through worldwide commercialization
- Be the leader in Growth Disorders and Hypoparathyroidism, pursuing clinical conditions, innovative LCM and complementary patient offerings
- Expand pipeline with Endocrinology Rare Disease blockbuster product opportunities

### • Create Value in Additional Therapeutic Areas through Innovative Business Models

- Obtain accelerated approval in oncology with registrational trials ongoing
- Pursue TransCon product opportunities in >\$5B indications
- Maximize value creation of these product opportunities through collaboration with therapeutic area market leaders

### • Differentiate with Ascendis Fundamentals

- Outperform industry drug development benchmarks with Ascendis' product innovation algorithm
- Remain independent as a profitable biopharma through lean and flexible ways of working
- Let our values Patients, Science, Passion drive our decisions to success



**PATIENTS  
SCIENCE  
PASSION**

**Thank you!**