

Ascendis Pharma A/S

TransCon™ PTH
Week 84 Phase 2 PaTH Forward Trial
November 18, 2021

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 business strategy, prospective products, clinical trial results, the expected timing of top-line results, product approvals and regulatory pathways, collaborations, licensing or other arrangements, 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 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 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.

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, changed circumstances 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.

Week 84 Phase 2 PaTH Forward Trial Open-Label Extension (OLE) Data

- 58 out of 59 subjects are continuing in the open-label extension beyond 84 weeks*
- 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

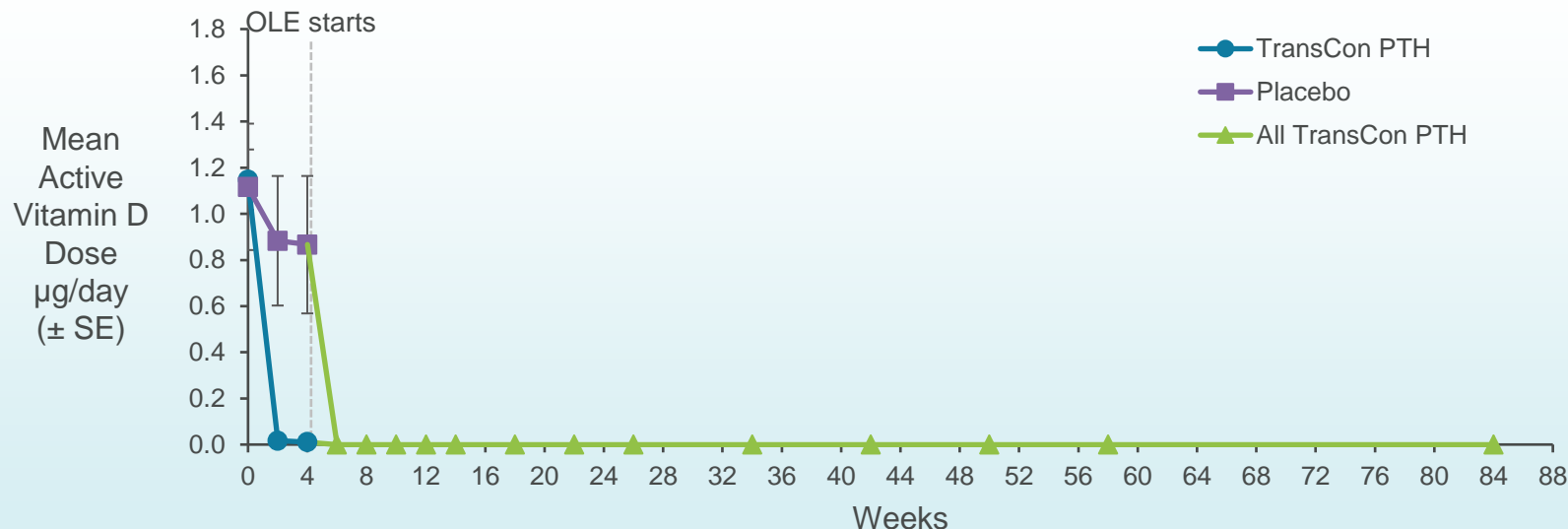
Top-line Week 84 data continue to support TransCon PTH as a potential hormone replacement therapy for adults with hypoparathyroidism

PaTH Forward week 84 top-line data.

*As of November 18, 2021.

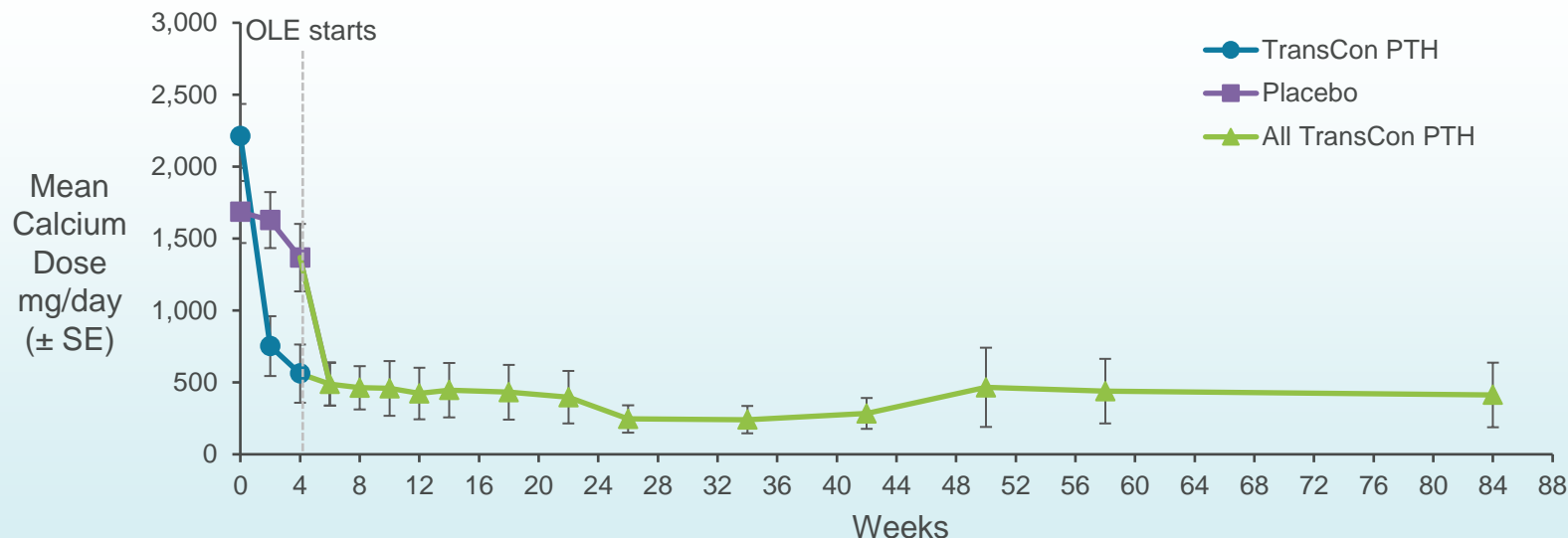
**Not taking active vitamin D and taking ≤ 600 mg/day of calcium supplements.

PaTH Forward Mean Active Vitamin D Dose



TransCon PTH enabled discontinuation of active vitamin D within two weeks of treatment initiation

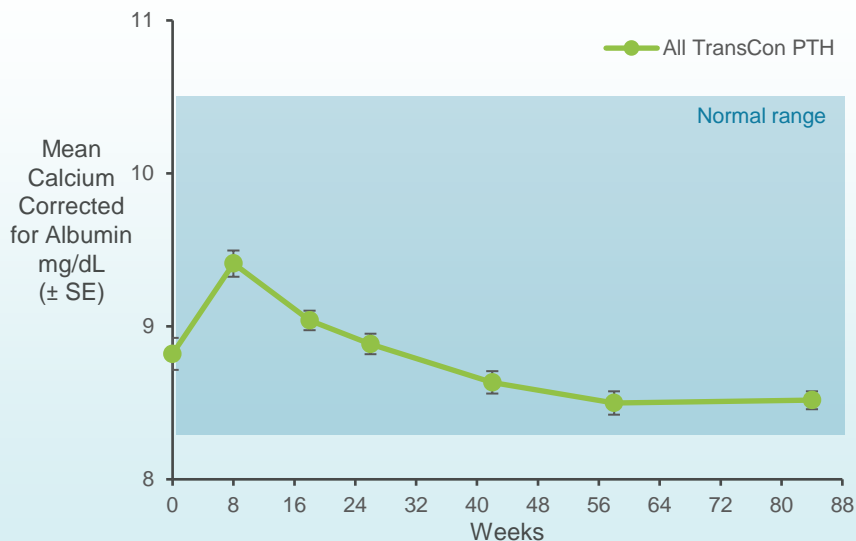
PaTH Forward Mean Calcium Supplement Dose



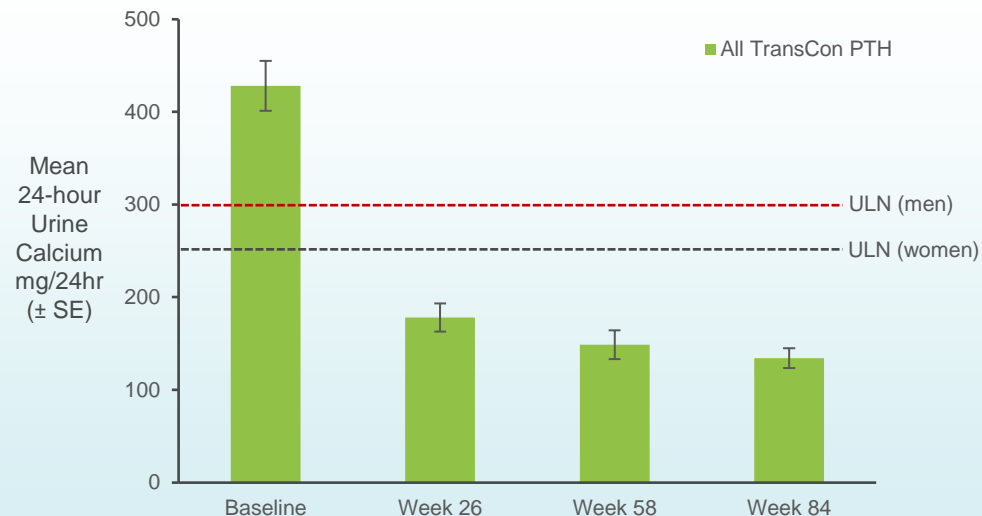
TransCon PTH enabled rapid and continuous calcium supplement reduction;
43 of 58 subjects were taking 0 mg, and 54 of 58 subjects were taking 0 to 600 mg at week 84

PaTH Forward Mean Serum Calcium and Mean 24-Hour Urine Calcium

Mean Serum Calcium



Mean 24-hour Urine Calcium



Mean serum calcium & mean 24-hour urine calcium remain in the normal range

Top-line 84 Week PaTH Forward Safety Summary

- TransCon PTH was well-tolerated at all doses administered
- 58 out of 59 randomized subjects currently receiving TransCon PTH in OLE*
- 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

PaTH Forward Overall TEAE Summary

	Week 84
	All TransCon PTH (N =59)
Subjects With – n (%)	
Treatment-Emergent Adverse Events (TEAE)	51 (86)
Serious TEAE	5 (8)
Severity	
Severe TEAE	3 (5)
Moderate TEAE	17 (29)
Mild TEAE	31 (53)
Related TEAE*	22 (37)
Related Serious TEAE	0
TEAE Related to Hyper- or Hypocalcemia Leading to ER/Urgent Care Visit and/or Hospitalization	0
TEAE Leading to Discontinuation of Study Drug	0
TEAE Leading to Discontinuation of Trial	0
TEAE Leading to Death	0

PaTH Forward week 84 top-line data. Percentages are calculated based on the number of subjects in the Safety Population. In the severity categories, subjects are displayed for the highest severity only. An AE is considered a TEAE if it occurred after the first dose of TransCon PTH. *Headache, hypocalcemia, nausea, dizziness, paresthesia, hypercalcemia and asthenia occurred in two or more subjects.

All product candidates other than SKYTROFA® are investigational.
Confidential, for investor communication only. Not for use in product promotion.



TransCon PTH: A Potential PTH Replacement Therapy

- Top-line 84-week Phase 2 PaTH Forward results demonstrated:
 - Durable efficacy of TransCon PTH
 - TransCon PTH was well-tolerated at all doses administered
- Continued stabilization of calcium metabolism in the absence of conventional treatment
 - Mean serum calcium remains stable and in the normal range
 - Mean urinary calcium excretion was maintained in the normal range
- 58 subjects continue in open-label extension beyond 84 weeks*
- Japanese Phase 3 PaTHway Japan in enrollment period
- North American and European Phase 3 PaTHway Trial top-line results expected Q1 2022

PaTH Forward week 84 top-line data.

*As of November 18, 2021.

Protocol Pre-Specified Key Follow-up Visits and Data Collected

Follow-up	Week 26	Week 58 (1 year)	Week 84	Week 110 (2 year)	Week 162 (3 year)	Week 214 (4 year)
Serum Calcium	✓	✓	✓	✓	✓	✓
Concomitant medication review	✓	✓	✓	✓	✓	✓
Quality of life measures	✓	✓		✓	✓	✓
24-hour Urine Collection	✓	✓	✓	✓	✓	✓
Bone turnover biomarkers	✓	✓		✓	✓	✓
Dual-energy x-ray absorptiometry (DXA)	✓	✓		✓	✓	✓

Extended follow-up planned to assess long-term safety of TransCon PTH