# Ascendis Pharma A/S

TransCon™ PTH – 58-Week Open-Label Extension (OLE) Update
May 10, 2021

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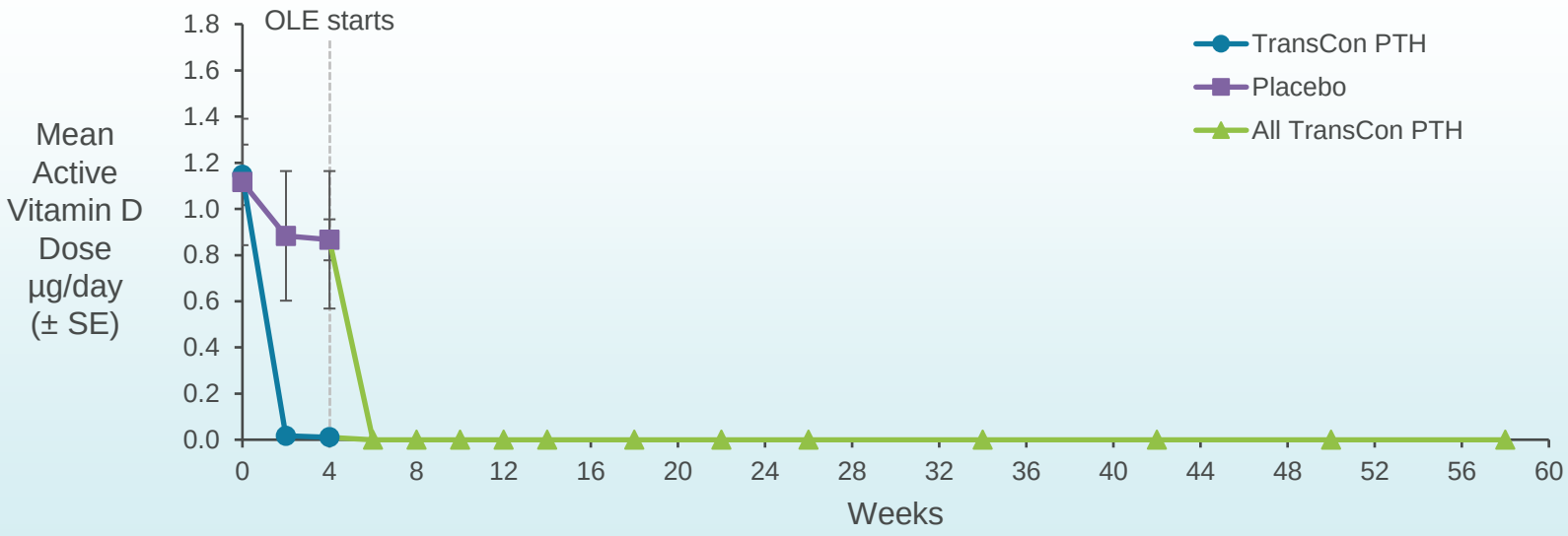
# Preliminary PaTH Forward Open-Label Extension (OLE) Data at 58 Weeks

- 58 subjects continue in the open-label extension beyond 58 weeks*
- Continued treatment with TransCon PTH demonstrated that:
  - 91% of subjects were off standard of care therapy**
  - Urinary calcium maintained in the normal range
  - Bone markers trended towards the mid-normal levels
  - Quality of life benefits measured by SF-36 continued within normal range
- TransCon PTH was well-tolerated at all doses administered
  - No treatment-related serious or severe adverse events occurred, and no treatment-emergent adverse events (TEAEs) led to discontinuation of study drug
  - No change to the safety profile in the OLE portion of the study

**Preliminary data support TransCon PTH as a potential hormone replacement therapy for adult HP**

Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
*As of May 7, 2021.
**Not taking active vitamin D and taking ≤600 mg/day of calcium supplements.

# PaTH Forward OLE Mean Active Vitamin D Dose

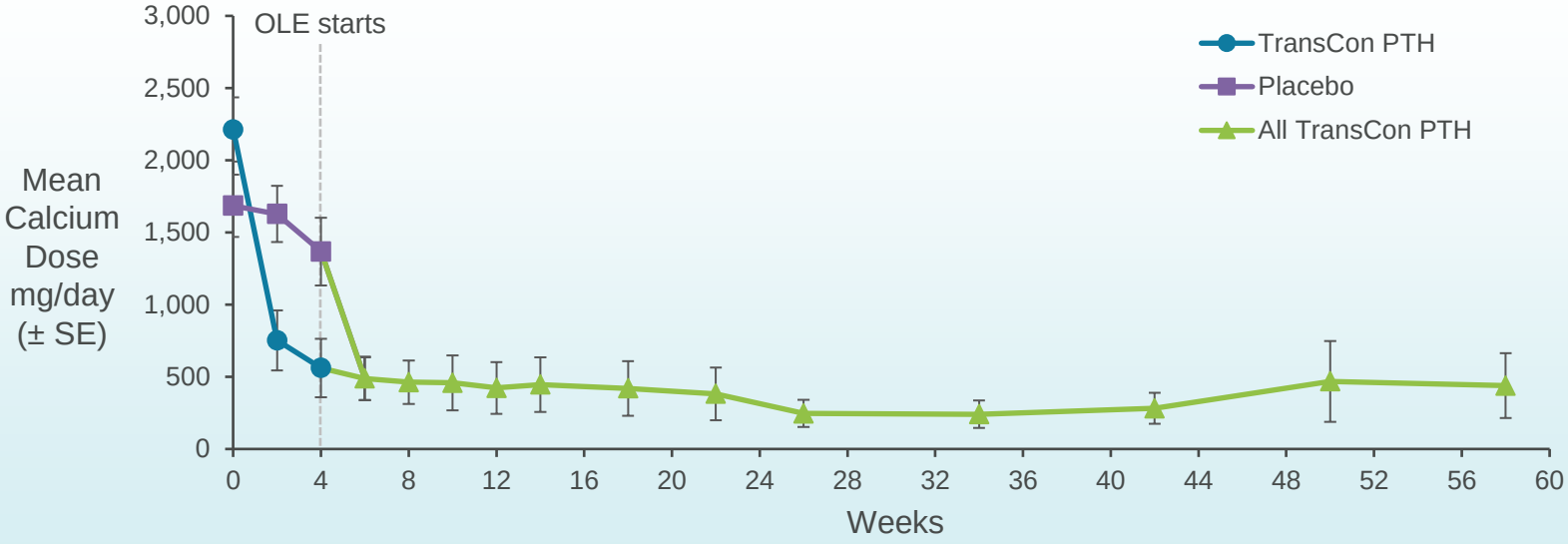



TransCon PTH enabled discontinuation of active vitamin D within two weeks of treatment initiation



Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.

# PaTH Forward OLE Mean Calcium Supplement Dose



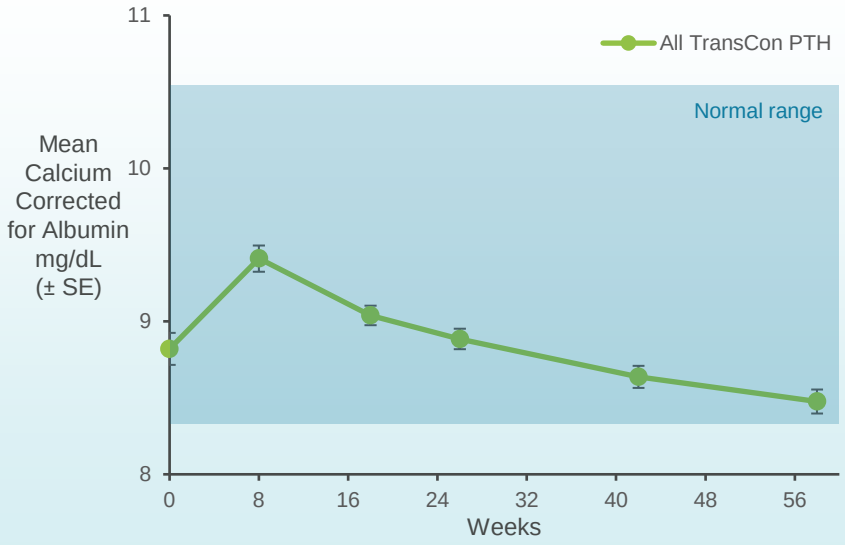

TransCon PTH enabled rapid and continuous calcium supplement reduction;
40 of 58 subjects were taking 0 mg, and 53 of 58 subjects were taking 0 to 600 mg at week 58



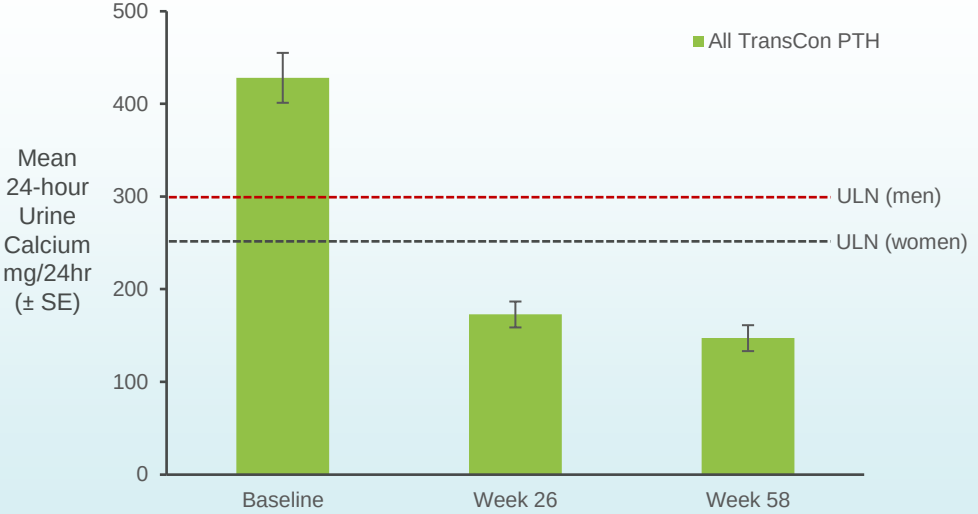
Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.

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

## Mean Serum Calcium




## Mean 24-hour Urine Calcium




Mean 24-hour urine calcium normalized while maintaining normal mean serum calcium



Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
ULN = Upper limit of normal.

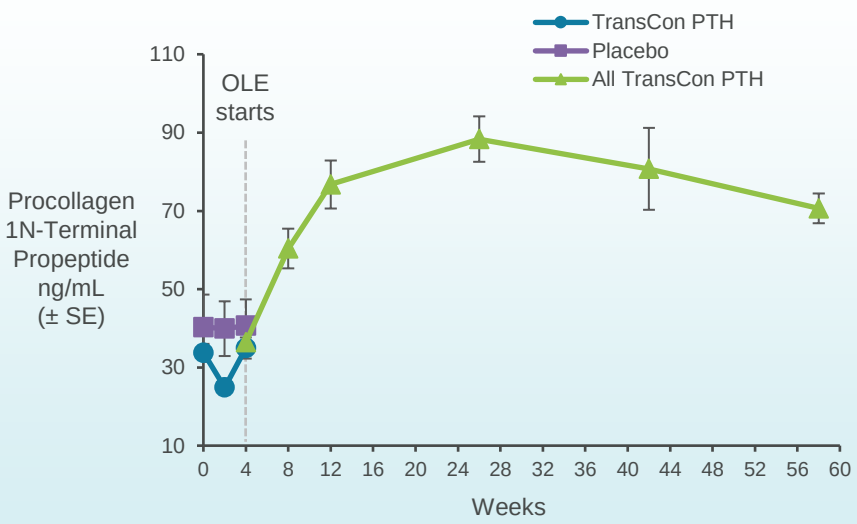
# PaTH Forward OLE SF-36® Health Survey Domain Mean Scores (SD) - Continued Normalization of Quality of Life

| | All TransCon PTH (N = 59) | | |
|---|---|---|---|
| **SF-36 Domain** | **Baseline** | **Week 26** | **Week 58** |
| Physical Function | 46 (10) | 51 (6) | 51 (6) |
| Role Physical | 42 (10) | 50 (7) | 50 (8) |
| Bodily Pain | 45 (10) | 50 (9) | 50 (8) |
| General Health | 43 (10) | 51 (8) | 51 (9) |
| Vitality | 43 (11) | 53 (8) | 51 (10) |
| Social Functioning | 43 (10) | 52 (6) | 52 (7) |
| Role Emotional | 43 (13) | 50 (7) | 49 (8) |
| Mental Health | 46 (9) | 52 (7) | 50 (9) |
| **Physical Component Summary** | **44 (11)** | **50 (8)** | **51 (8)** |
| **Mental Component Summary** | **44 (11)** | **52 (8)** | **50 (9)** |

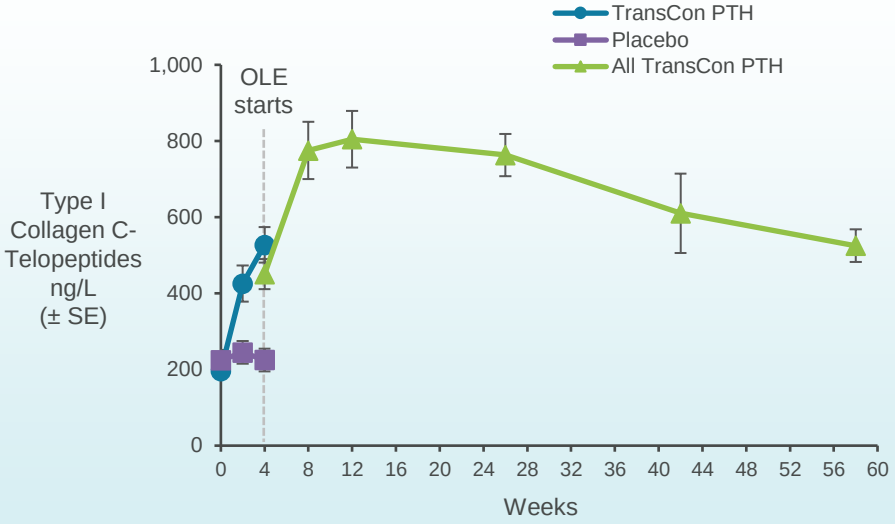
Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
Green: Means above lower limit of population norm (47)

# PaTH Forward OLE - P1NP and CTx Bone Markers

## Mean P1NP




## Mean CTx




TransCon PTH treatment initially increased the levels of anabolic and catabolic bone markers which were trending to mid-normal levels at 58 weeks



Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.

# Preliminary PaTH Forward OLE Safety Summary at 58 Weeks

- 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 change to the safety profile in the OLE portion of the study

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

Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
*As of May 7, 2021.
TEAE = Treatment emergent adverse event

# PaTH Forward OLE Overall TEAE Summary

| | Up to Week 26 | Up to Week 58 |
|---|---|---|
| | All TransCon PTH (N = 59) | All TransCon PTH (N = 59) |
| **Subjects With – n (%)** | | |
| Treatment-Emergent Adverse Events (TEAE) | 37 (63) | 44 (75) |
| Serious TEAE | 2 (3) | 3 (5) |
| Severity | | |
| Severe TEAE | 1 (2) | 2 (3) |
| Moderate TEAE | 9 (15) | 10 (17) |
| Mild TEAE | 27 (46) | 32 (54) |
| Related TEAE* | 14 (24) | 16 (27) |
| Serious Related TEAE | 0 | 0 |
| TEAE Related to Hyper- or Hypocalcemia Leading to ER/Urgent Care Visit and/or Hospitalization | 0 | 0 |
| TEAE Leading to Discontinuation of Study Drug | 0 | 0 |
| TEAE Leading to Discontinuation of Trial | 0 | 0 |
| TEAE Leading to Death | 0 | 0 |

Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
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.

# TransCon PTH: A Potential PTH Replacement Therapy

- Preliminary phase 2 PaTH Forward OLE results at 58 weeks demonstrated:*
  - Durable benefits of TransCon PTH
  - TransCon PTH was well-tolerated at all doses
- Subjects appeared to be establishing physiologic calcium metabolism*
  - Bone markers continued to trend towards the mid-normal levels
  - Urine calcium normal
- 58 subjects continue in open-label extension beyond 58 weeks**
  - Further optimization of TransCon PTH dosing being implemented
  - 84-week topline OLE update anticipated in Q4 2021
- Expect to file CTN for Japanese adult HP phase 3 study in Q2 2021
- North American and European phase 3 PaTHway Trial topline results expected Q4 2021



*Preliminary PaTH Forward OLE week 58 data from live database snapshot. Data on file.
**As of May 7, 2021.



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