



TransCon™ PTH

Clinical Development Update

September 29, 2020

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TransCon PTH Overview

- Preliminary PaTH Forward OLE data at month 6 support TransCon PTH as a potential hormone replacement therapy for adult HP
 - Eliminated standard of care¹ with a well-tolerated once daily injection
 - Normalized mean SF-36 quality-of-life scores on all summary and subdomains
 - Normalized mean 24-hour urine calcium, a key marker of long-term safety
- IND amendment filed for phase 3 PaTHway Trial of TransCon PTH compared to placebo; European filings in preparation
 - Based on feedback from U.S. and European regulatory authorities
 - Randomized, double-blind, placebo-controlled trial of 76 adults with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo) with an open-label extension period
 - Well-powered to achieve primary composite endpoint of proportion of subjects with: (1) serum calcium in the normal range, and (2) independence from active vitamin D, and (3) calcium supplement dose ≤ 600 mg/day
- Conclusions
 - TransCon PTH was well-tolerated during 6 months of treatment with no treatment-related serious or severe TEAEs
 - TransCon PTH demonstrated ability to eliminate standard of care, reduce 24-hour urine calcium and maintain serum calcium well into the normal range at 6 months
 - 86% of PaTH Forward OLE subjects had (1) normal serum calcium, and (2) were not taking active vitamin D, and (3) were taking ≤ 600 mg of calcium per day at 6 months

Preliminary PaTH Forward OLE 6-month data.

¹Not taking active vitamin D and taking ≤ 500 mg/day of calcium supplements

Preliminary PaTH Forward Open-Label Extension (OLE) Data at Month 6

- TransCon PTH eliminated standard of care¹ in 91% of subjects
- TransCon PTH demonstrated significant reduction in urinary calcium
 - 86% of subjects had 24-hour urine calcium in the normal range or 50% reduction from baseline
 - Mean 24-hour urine calcium fell by 57% from 415 mg at baseline in all groups to 178 mg after 6 months
- TransCon PTH demonstrated normalization of quality of life as measured by mean SF-36 scores
- TransCon PTH demonstrated a strong response on composite endpoints
 - 71% of subjects responded to composite of (1) normal serum calcium, (2) off vitamin D, (3) taking ≤ 500 mg calcium, and (4) 24-hour urine calcium in the normal range or 50% reduction from baseline
 - 86% of subjects (1) had normal serum calcium, (2) were off vitamin D, and (3) were taking ≤ 600 mg calcium
- All doses of TransCon PTH were well-tolerated
 - No treatment-related serious or severe adverse events occurred, and no treatment-emergent adverse events (TEAEs) led to discontinuation of study drug
 - No new safety signals identified in the open label extension portion of the study
- 58 subjects continue in open-label extension beyond 6 months

Preliminary PaTH Forward OLE data at 6 months support TransCon PTH as a potential hormone replacement therapy for adult HP

Preliminary PaTH Forward OLE 6-month data.

¹Not taking active vitamin D and taking ≤ 500 mg/day of calcium supplements

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TransCon PTH Phase 2 Trial Design



59 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 (including HPES and SF-36)
- 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. HPES = Hypoparathyroidism Patient Experience Scale. BMD = Bone Mineral Density. TBS = Trabecular Bone Score. DXA = Dual-Energy X-Ray Absorptiometry. FECa = Fractional Excretion of Calcium.

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PaTH Forward Demographics and Baseline Characteristics

	Total (N=59)
Age (years) (n)	59
Mean (SD)	50 (12)
Age Group (years) – n (%)	
<30	3 (5)
≥30 – <65	51 (86)
≥65	5 (9)
Sex at Birth n (%)	
Female	48 (81)
Body Mass Index (kg/m²) (n)	59
Mean (SD)	28 (4)
Menopausal Status – n (%)	48
Postmenopausal	17 (35)

PaTH Forward Demographics and Baseline Characteristics

	Total (N=59)
Race – n (%)	
American Indian or Alaska Native	0
Asian	2 (3)
Black or African American	0
Native Hawaiian or Other Pacific Islander	0
White	54 (92)
Unknown	0
Other	3 (5)
Geographic Region – n (%)	
North America	38 (64)
Europe	21 (36)

PaTH Forward HP Disease Characteristics and History

	Total (N=59)
Cause of Hypoparathyroidism (HP)	
Acquired from neck surgery	47
Autoimmune disease	1
Idiopathic disease	11
Duration of HP (Years)	
Mean	12
Min, Max	1, 39
Renal Insufficiency History	5
Kidney Stones History	8
Ectopic Calcifications History	1
Vascular Calcifications History	0
Brain Calcification History	0
Cataract History	0
Seizure History	2

PaTH Forward Baseline HP Supplements

HP Supplements at Baseline collected by eDiary/Total Daily Dose (TDD)	Total (N=59)
Calcium /TDD (mg) (n)	59
Mean	2079
Min, Max	500, 8000
Calcium Category, n (%)	
≤2000 mg TDD	37 (63)
>2000 mg TDD	22 (37)
Calcitriol (Active Vitamin D) /TDD (µg) (n)	45
Mean	0.794
Min, Max	0.25, 3.00
Alfacalcidol (Active Vitamin D) /TDD (µg) (n)	13
Mean	2.38
Min, Max	1.0, 4.0

PaTH Forward Baseline of Spot FECa & Albumin-Adjusted sCa

Lab Summary at Baseline	Total (N=59)
Albumin-Adjusted sCa (mg/dL) (n)	59
Mean (SD)	8.8 (0.80)
Spot AM FECa (%) (n)	59
Mean (SD)	2.6 (1.3)
Spot AM FECa normal ($\leq 2\%$) baseline, n (%)	25 (42)

PaTH Forward OLE Overall TEAE Summary

	TransCon PTH/ TransCon PTH (N=44)	Placebo/ TransCon PTH (N=15)	All TransCon PTH (N=59)
Subjects With – n (%)			
Treatment-Emergent Adverse Events (TEAE)	27 (61)	10 (67)	37 (63)
Serious TEAE	0	2 (13)	2 (3.4)
Severity			
Severe TEAE	0	1 (6.7)	1 (1.7)
Moderate TEAE	6 (14)	3 (20)	9 (15)
Mild TEAE	21 (48)	6 (40)	27 (46)
Related TEAE	10 (23)	4 (27)	14 (24)
Serious Related TEAE	0	0	0
TEAE Related to Hyper- or Hypocalcemia Leading to ER/Urgent Care Visit and/or Hospitalization	0	0	0
TEAE Leading to Discontinuation of Study Drug	0	0	0
TEAE Leading to Discontinuation of Trial	0	0	0
TEAE Leading to Death	0	0	0

Preliminary PaTH Forward OLE 6-month 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.

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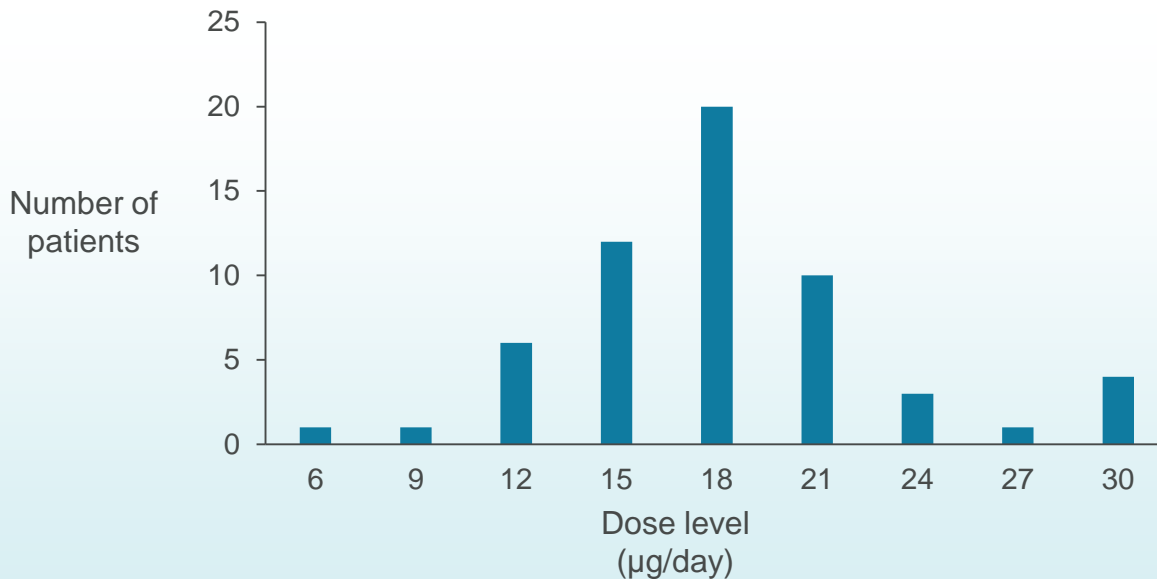
Preliminary PaTH Forward OLE Safety Summary

- TransCon PTH was well-tolerated
- 58 subjects continue in open label extension beyond 6 months
- 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 new safety signals identified in the open label extension portion of the study

No subjects had PTH treatment-emergent adverse events related to Hyper- or Hypocalcemia leading to ER/urgent care visit and/or hospitalization

PaTH Forward OLE Distribution of Doses at Month 6

At month 6 in PaTH Forward



At month 6 of PaTH Forward, subjects used all available doses of TransCon PTH, with a mean of 18 µg/day

PaTH Forward OLE Overall Compliance with Dosing

	TransCon PTH (n=44)	Placebo/TransCon (n=15)	All TransCon PTH (n=59)
Compliance (%)			
Mean	99.7	99.8	99.7
SD, SE	10.6, 0.16	0.39, 0.10	0.94, 0.12
Median	100.0	100.0	100.0
Min, Max	93,100	99,100	93,100
Compliance with dosing			
≤80%	0	0	0
>80% to ≤90%	0	0	0
>90%	100%	100%	100%

Overall compliance with once-daily TransCon PTH was almost 100%

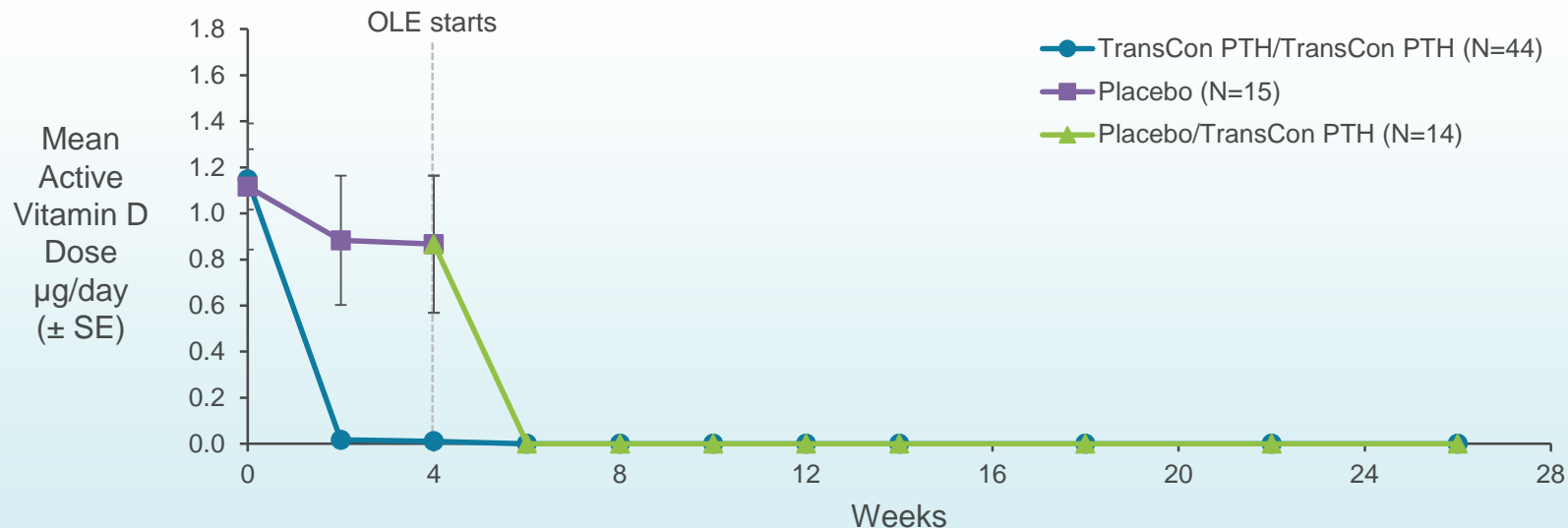
PaTH Forward OLE Elimination of Standard of Care at Month 6

Number of Subjects Meeting Each Component	TransCon PTH/TransCon PTH (N=44)	Placebo/TransCon PTH (n=15)	All TransCon PTH (N=59)
The number of subjects reached 6 month visit	44	14	58
Active vitamin D = 0 mcg/day	44 (100%)	14 (100%)	58 (100%)
Calcium ≤1000 mg/day	41 (93%)	14 (100%)	55 (95%)
Calcium ≤500 mg/day	40 (91%)	13 (93%)	53 (91%)
Calcium = 0 mg/day	33 (75%)	11 (79%)	44 (76%)
Active vitamin D = 0 and Calcium = 0 mg/day	33 (75%)	11 (79%)	44 (76%)
Active vitamin D = 0 and Calcium ≤500 mg/day	40 (91%)	13 (93%)	53 (91%)

The percentages are calculated based on the number of subjects with 6-month visit.

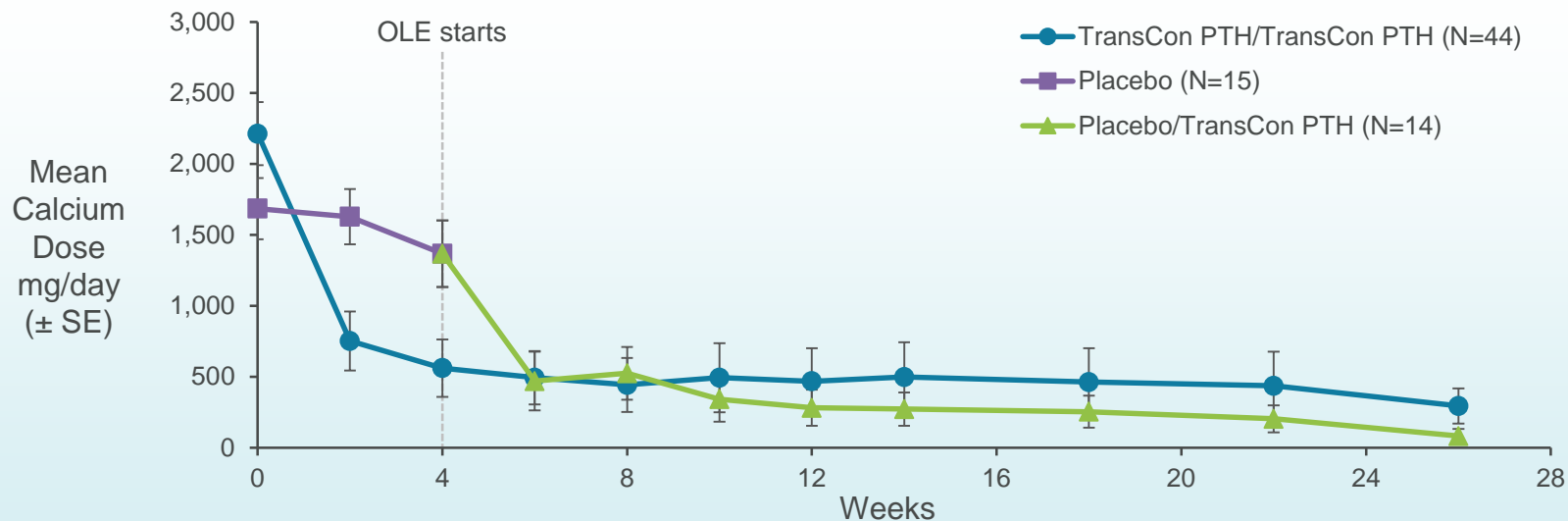
9 out of 10 subjects were able to eliminate standard of care*
8 of 10 subjects were able to eliminate all supplements

PaTH Forward OLE Mean Active Vitamin D Dose



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

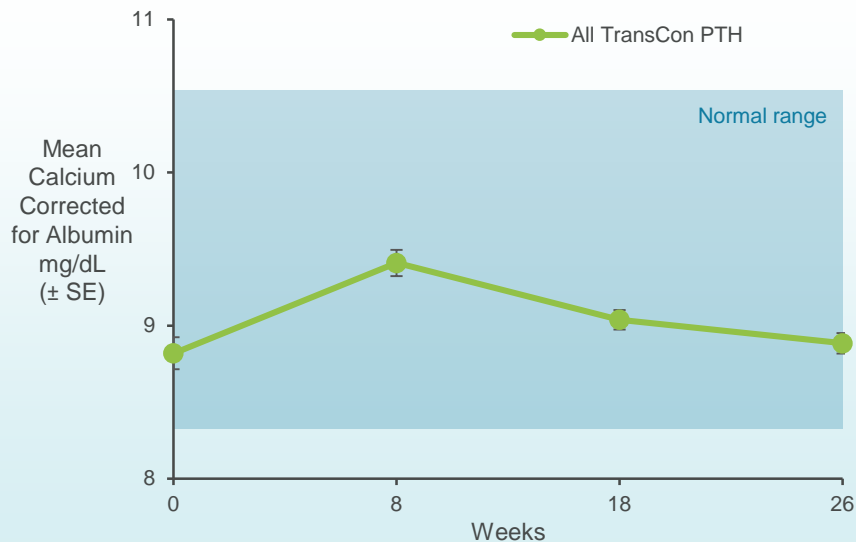
PaTH Forward OLE Mean Calcium Supplement Dose



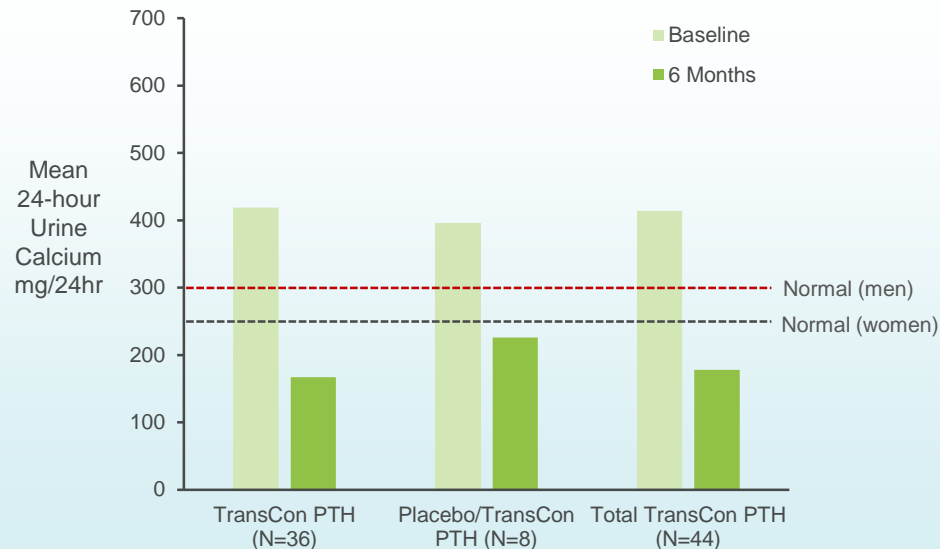
TransCon PTH enabled rapid and continuous calcium supplement reduction over 6-month study period

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

PaTH Forward OLE Mean 24-Hour Urine Calcium at Month 6

Parameter (unit) Visit Statistics	TransCon PTH/ TransCon PTH (N=44)	Placebo/ TransCon PTH (N=15)	All TransCon PTH (N=59)
24-Hour Urine Calcium (mg/24 hr)			
Baseline – n	39	11	50
Mean (SD)	425 (200)	440 (162)	428 (191)
6 Month – n	36	8	44
Baseline			
Mean (SD)	419 (198)	395 (148)	415 (189)
Observed			
Mean (SD)	167 (98)	226 (107)	178 (101)
Change from Baseline			
Mean (SD)	-252 (200)	-169 (194)	-237 (199)

At each post-baseline visit, only data from subjects with both baseline and the corresponding visit values available are used to compute the statistical summaries

Mean 24-hour urine calcium decreased 57% while maintaining normal mean serum calcium

PaTH Forward OLE Mean Spot AM FECa at Month 6

Parameter (unit) Visit Statistics	TransCon PTH/ TransCon PTH (N=44)	Placebo/ TransCon PTH (N=15)	All TransCon PTH (N=59)
Spot AM FECa (%)			
Baseline – n	44	15	59
Mean (SD)	2.8 (1.4)	2.2 (0.8)	2.6 (1.3)
6 Month – n	33	9	42
Baseline			
Mean (SD)	2.9 (1.4)	2.5 (0.7)	2.8 (1.3)
Observed			
Mean (SD)	1.5 (1.0)	1.5 (0.6)	1.5 (1.0)
Change from Baseline			
Mean (SD)	-1.4 (1.5)	-0.9 (0.9)	-1.3 (1.4)

Mean spot AM FECa decreased while maintaining normal mean serum calcium

Preliminary PaTH Forward OLE 6-month data.

At each post-baseline visit, only data from subjects with both baseline and the corresponding visit values available are used to compute the statistical summaries.

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PaTH Forward OLE 10 Subjects without Complete Composite Endpoint at Month 6

- One subject (randomized to placebo) withdrew for reasons unrelated to safety or efficacy of the study drug
- Four subjects excluded due to 24-hour urine sampling out of window (day 169–210):
 - All four subjects were off active vitamin D and ≤ 500 mg of calcium supplements
 - Three of four had normal 24-hour urine calcium and normal serum calcium
- Three 24-hour urine samples not prepared properly; redo samples were outside of window and were not received by data cutoff
- Two 24-hour urine samples were not received by laboratory

Proportion of Subjects Achieving Composite Endpoint at Month 6 in PaTH Forward OLE

	TransCon PTH/ TransCon PTH (N=44)	Placebo / TransCon PTH ¹ (N=15)	All TransCon PTH (N=59)
Number of Subjects Who Have Data on All Criteria at Month 6	39	10	49
Number of Subjects Meeting The Primary Endpoint Criteria at Month 6	29	6	35
Proportion (95% CI) ²	74.4 (57.9, 87.0)	60.0 (26.2, 87.8)	71.4 (56.7, 83.4)
Number of Subjects Meeting Each Component:			
Albumin-adjusted sCa within the normal range	36	9	45
24-Hour urine calcium within normal range or ≥50% decrease from baseline	34	8	42
Not taking active vitamin D supplements	39	10	49
Taking ≤500 mg/day of calcium supplements	35	9	44

35 subjects met all criteria for response, and 14 subjects demonstrated partial response

Preliminary PaTH Forward OLE 6-month data.

¹Subjects in this group switched from placebo at 4 weeks to TransCon PTH for the open-label portion of the trial.

²Percentage is based on the number of subjects who have data on all criteria at Month 6. The normal range for albumin-adjusted sCa is 8.3-10.6 mg/dL (2.07-2.64 mmol/L). The normal range for 24-hour urine calcium is defined as ≤250 mg/day for female, ≤300 mg/day for male.

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PaTH Forward OLE Partial Responder Analysis at Month 6

	Albumin-adjusted sCa	24-Hour Urine Calcium	Calcium Dose	Active Vitamin D Dose	TransCon PTH Dose
Partial Responder	mg/dL	mg	mg/day	µg/day	µg/day
Subject 1	9.5	256	0	0	18
Subject 2	9.3	196	600	0	15
Subject 3	9.3	307	0	0	21
Subject 4	8.4	441	3000	0	30
Subject 5	8.3	63	4000	0	30
Subject 6	8.2	35	70	0	18
Subject 7	9.4	269	0	0	18
Subject 8	9.32	288	0	0	15
Subject 9	8.92	422	500	0	18
Subject 10	8.48	79	2400	0	18
Subject 11	8.02	71	800	0	21
Subject 12	7.96	67	0	0	18
Subject 13	9.28	449	0	0	18
Subject 14	8.2	63	250	0	21

All 14 partial responders met two or more response criteria¹ and 12 met three

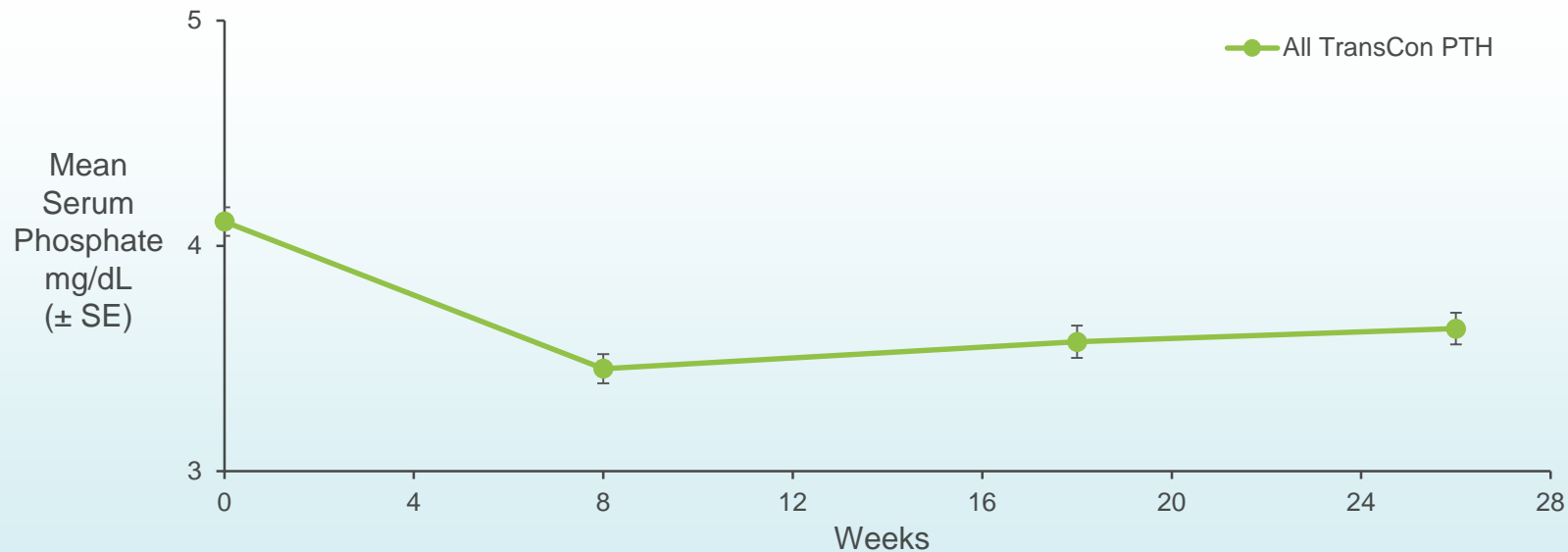
Preliminary PaTH Forward OLE 6-month data.

Green = meet response criteria. Yellow = did not meet response criteria.

¹ Response criteria are normal serum calcium, off vitamin D, taking ≤500 mg calcium, and 24-hour urine calcium in the normal range or 50% reduction from baseline.

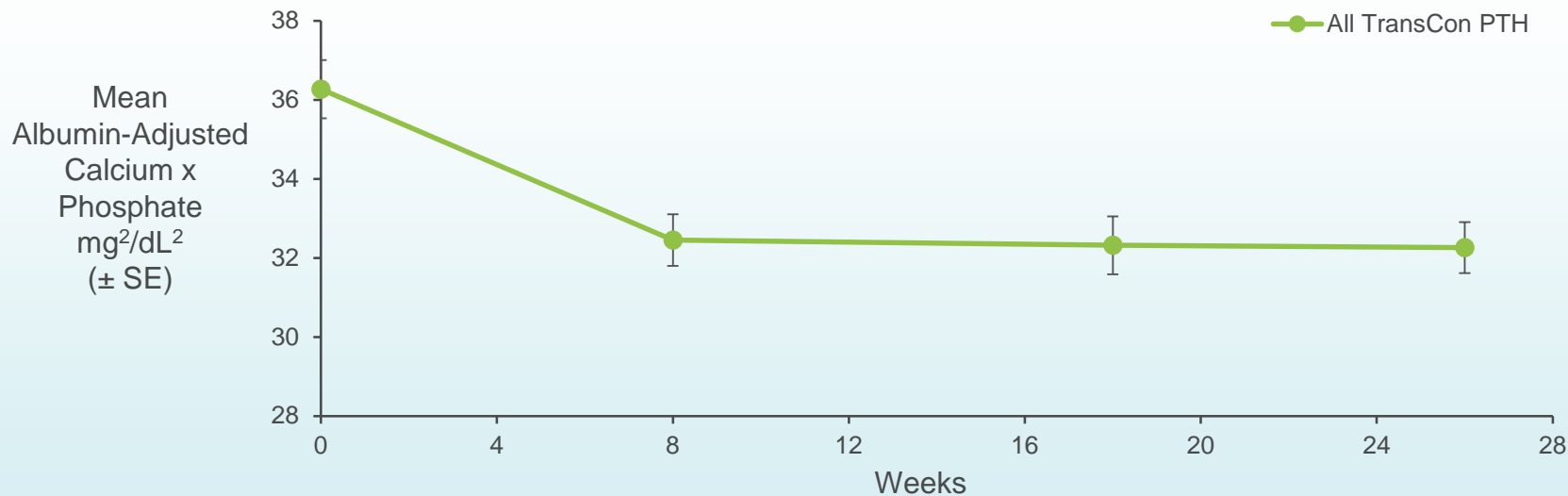
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PaTH Forward OLE Mean Serum Phosphate



TransCon PTH subjects demonstrated consistent, sustained reductions in serum phosphate

PaTH Forward OLE Mean Serum Calcium-Serum Phosphate Product



TransCon PTH demonstrated consistent, sustained reductions in calcium-phosphate product

PaTH Forward OLE Change in SF-36[®] Health Survey Domain Mean Scores (SD)

SF-36 domain*	Placebo (n=15)		Placebo Switch to TransCon PTH (n=15)	TransCon PTH (n=44)			All TransCon PTH (n=59)	
	Baseline	Week 4	6 Months	Baseline	Week 4	6 Months	Baseline	6 Months
PF	45 (11)	46 (14)	51 (7)	46 (9)	51 (6)	52 (5)	46 (10)	51 (6)
RP	42 (10)	42 (14)	49 (11)	42 (10)	49 (8)	51 (6)	42 (10)	50 (7)
BP	43 (11)	40 (16)	46 (10)	46 (10)	49 (8)	51 (9)	45 (10)	50 (9)
GH	44 (10)	47 (11)	50 (7)	43 (10)	47 (8)	51 (9)	43 (10)	51 (8)
VT	44 (12)	43 (12)	52 (10)	42 (11)	49 (9)	53 (8)	43 (11)	53 (8)
SF	44 (11)	41 (15)	53 (5)	42 (10)	50 (8)	52 (6)	43 (10)	52 (6)
RE	45 (12)	39 (16)	51 (7)	42 (13)	49 (10)	50 (8)	43 (13)	50 (7)
MH	47 (9)	47 (11)	55 (5)	46 (9)	51 (8)	51 (8)	46 (9)	52 (7)
PCS	43 (12)	44 (14)	48 (8)	45 (10)	49 (7)	51 (7)	44 (11)	50 (8)
MCS	46 (10)	43 (12)	54 (6)	43 (11)	50 (9)	51 (8)	44 (11)	52 (8)

Preliminary PaTH Forward OLE 6-month data.

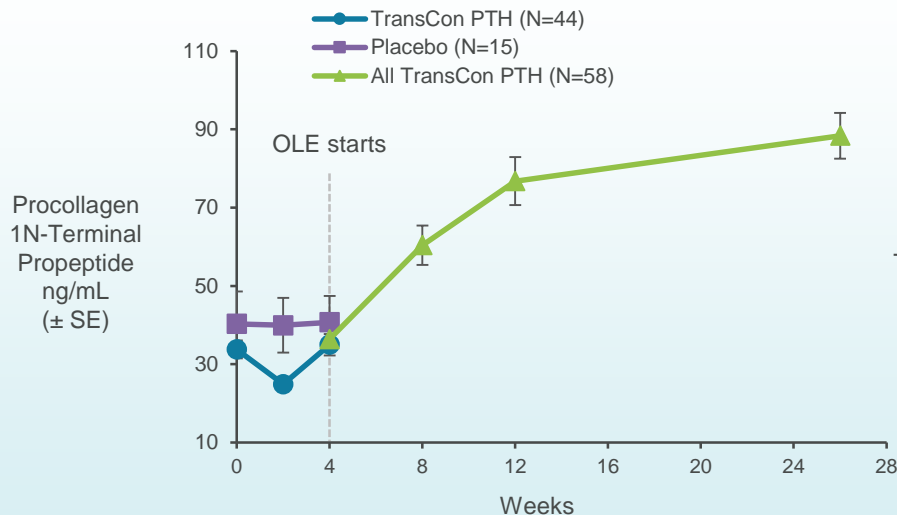
Green: patients within normal range. (SD). *PF (physical functioning), RP (role physical), BP (bodily pain), SF (social functioning), MH (mental health), RE (role emotional), VT (vitality), GH (general health), PCS (physical component summary), MCS (mental component summary).

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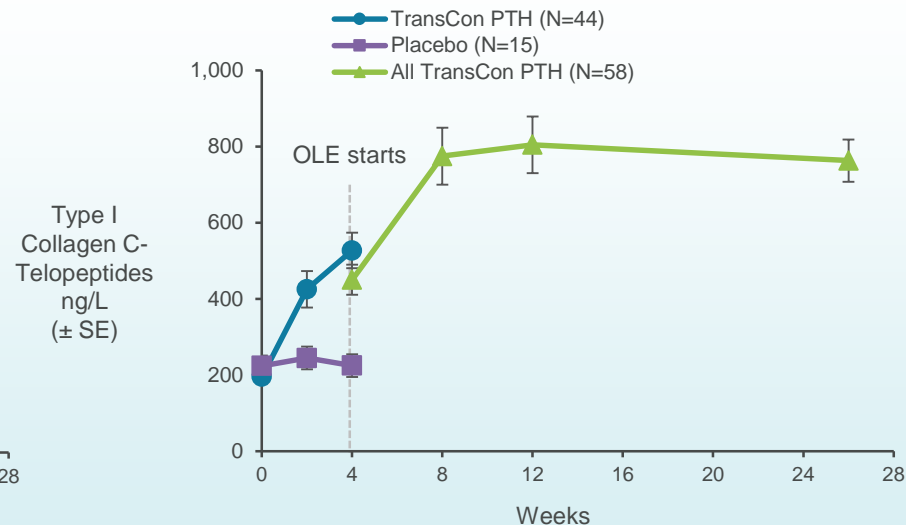


PaTH Forward OLE Mean P1NP and CTx

Mean P1NP



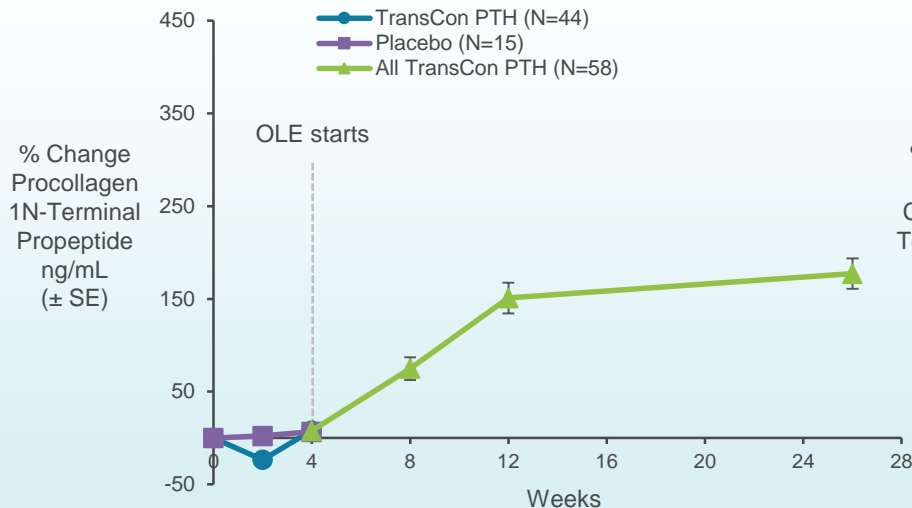
Mean CTx



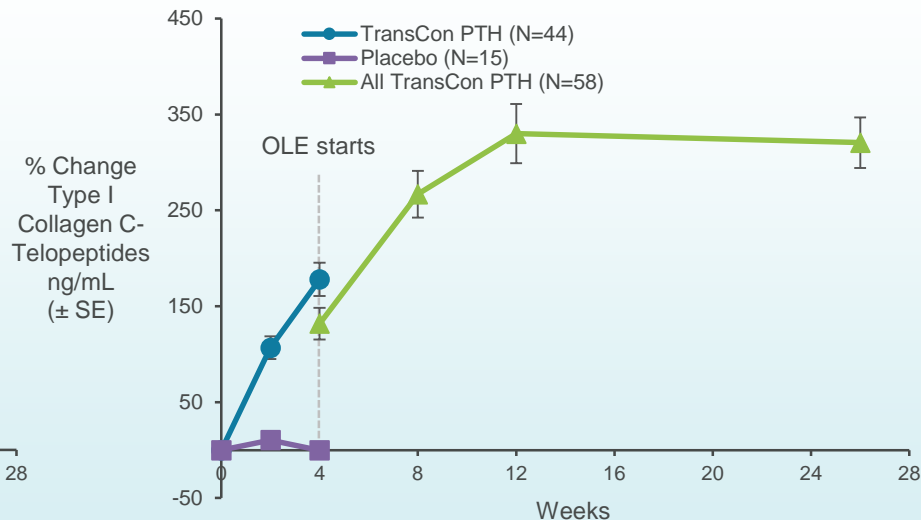
PaTH Forward patient population had expected low level of bone turnover at baseline and demonstrated at 6 months increased levels of anabolic and catabolic turnover

PaTH Forward OLE % Change in Mean P1NP and CTx

% Change in Mean P1NP



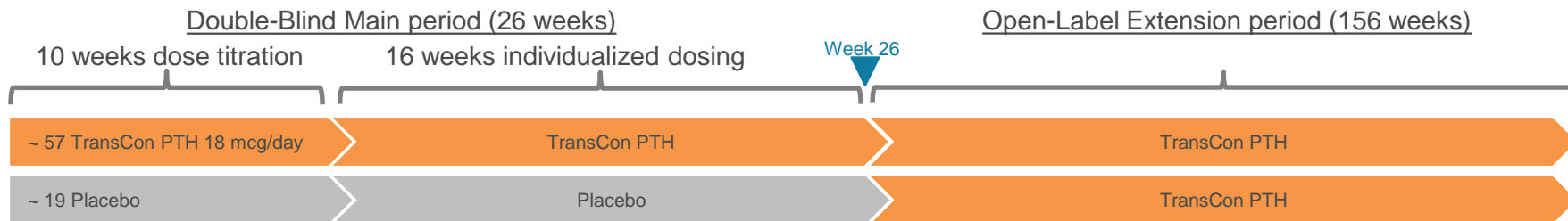
% Change in Mean CTx



At 6-month point, observed lower level of increase for anabolic compared to catabolic bone turnover

PaTHway Phase 3 Trial Design

Double-blind, placebo-controlled trial with an open-label extension period 76¹ adults with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo)



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 ≥ 18 years
- Reliant on calcitriol ≥ 0.50 mcg per day or alfacalcidol ≥ 1.0 mcg per day, **and** therapeutic elemental calcium ≥ 800 mg/day
- Serum calcium in normal (or just below normal) range: 7.8 – 10.6 mg/dL (1.96 – 2.64 mmol/L)
- No PTH or PTHrP therapy within 4 weeks prior to Screening

Countries Planned

- Europe (Germany, United Kingdom, Denmark, Norway, France, 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 Secondary Endpoints at Week 26

- Proportion of subjects meeting the primary endpoint above **and** with normal 24-hour urine calcium excretion (or $\geq 50\%$ reduction from baseline)
- Serum phosphate levels
- Hypoparathyroidism Patient Experience Scale measures
- 36-Item Short Form Survey (SF-36) measure

¹ Enrollment increased to 76 subjects to ensure evaluable data for 68.

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

Summary of TransCon PTH Update

- Preliminary phase 2 PaTH Forward OLE results at month 6*:
 - 86% of subjects had (1) normal serum calcium, (2) off active vitamin D and (3) taking ≤ 600 mg/day of calcium
 - 71% of subjects achieved composite endpoint of (1) normal serum calcium, (2) off vitamin D, (3) taking ≤ 500 mg calcium, and (4) 24-hour urine calcium in the normal range or 50% reduction from baseline
 - Mean scores for all summary and subdomains of SF-36 were normal for all TransCon PTH subjects at 6 months in PaTH Forward OLE
- IND amendment filed for phase 3 PaTHway Trial of TransCon PTH compared to placebo based on feedback from U.S. and European regulatory authorities
 - Randomized, double-blind, placebo-controlled trial of 76 adults (to ensure 68 evaluable subjects) with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo) with an open-label extension period
 - Primary composite endpoint of proportion of subjects with: (1) normal serum calcium, (2) off active vitamin D, and (3) taking ≤ 600 mg/day of calcium
- Regulatory filing to initiate European sites in phase 3 planned for later this year