TransCon™ PTH

Top-Line Phase 2 Data from PaTH Forward

April 19, 2020

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PaTH Forward Top-Line Data from 4-Week Fixed Dose Period

- PaTH Forward top-line data support TransCon PTH as a potential replacement therapy for adult HP
  - TransCon PTH eliminated standard of care (i.e. off active vitamin D and ≤ 500 mg per day of calcium supplements) in 100% of subjects in the 21 µg/day arm and in 82% of subjects across all dosage arms
  - Both the 21 µg/day arm and the combined TransCon PTH dosage arms showed a statistically significant response for the primary endpoint compared to placebo at 4 weeks
  - TransCon PTH increased mean serum calcium
  - TransCon PTH reduced mean urinary calcium excretion
  - TransCon PTH reduced mean serum phosphate and calcium-phosphate product
- All doses of TransCon PTH were well-tolerated
  - No serious or severe adverse events at any point
  - No treatment-emergent adverse events (TEAEs) led to discontinuation of study drug
  - Overall incidence of TEAEs comparable between TransCon PTH and placebo
- No drop-outs in blinded period

TransCon PTH Phase 2 Trial Design

PaTHforward TRIAL

~40 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 supplements

Key Secondary Composite Endpoint (4 weeks)

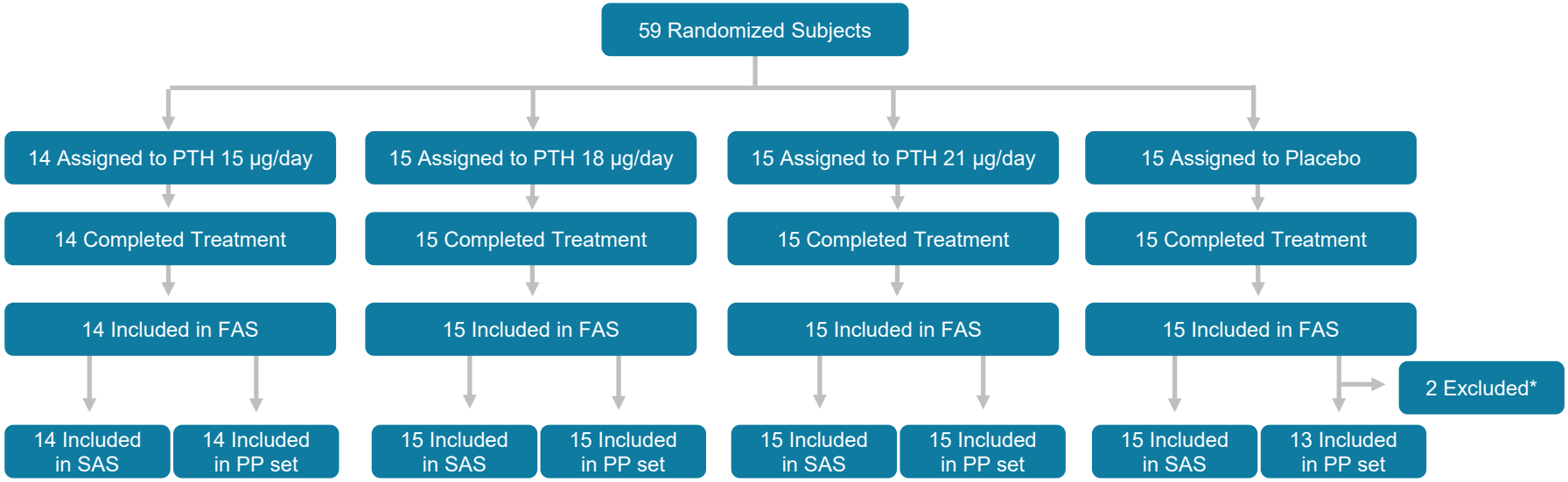
- Primary composite ***and*** taking ≤500 mg/day calcium supplements

Additional Endpoints ≥4 weeks

- PRO* measures (HPES: a disease-specific PRO for HP)
- 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

PaTH Forward Trial Profile



- Full Analysis Set (FAS): All randomized subjects who received at least 1 dose of randomized treatment
- Per Protocol (PP): Subjects from FAS who met inclusion/exclusion criteria and completed full double-blind trial period
- Safety Analysis Set (SAS): All randomized subjects who received at least 1 dose of randomized treatment

* Two subjects were excluded because they received < 0.25 µg BID of calcitriol (active vitamin D)

Demographics and Baseline Characteristics – PP

	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Age (years) (n)	14	15	15	44	13
Mean (SD)	47 (13)	47 (11)	54 (11)	49 (12)	50 (13)
Age Group (years) – n (%)					
< 30	1 (7.1)	1 (6.7)	0	2 (4.5)	1 (7.7)
≥ 30 - < 65	11 (79)	14 (93)	13 (87)	38 (86)	11 (85)
≥ 65	2 (14)	0	2 (13)	4 (9.1)	1 (7.7)
Sex at Birth n (%)					
Female	12 (86)	12 (80)	12 (80)	36 (82)	10 (77)
Body Mass Index (kg/m^2) (n)	14	15	15	44	13
Mean (SD)	27 (5.7)	29 (3.1)	26 (4.6)	27 (4.6)	28 (3.8)
Menopausal Status – n (%)	12	12	12	36	10
Postmenopausal	4 (33)	4 (33)	5 (42)	13 (36)	3 (30)

Demographics and Baseline Characteristics – PP

	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Race – n (%)					
American Indian or Alaska Native	0	0	0	0	0
Asian	0	0	2 (13)	2 (4.5)	0
Black or African American	0	0	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0	0	0
White	14 (100)	12 (80)	13 (87)	39 (89)	13 (100)
Unknown	0	0	0	0	0
Other	0	3 (20)	0	3 (6.8)	0
Geographic Region – n (%)					
North America	7 (50)	12 (80)	10 (67)	29 (66)	7 (54)
Europe	7 (50)	3 (20)	5 (33)	15 (34)	6 (46)

HP Disease Characteristics and History – PP

	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Cause of Hypoparathyroidism (HP)					
Acquired from neck surgery	10 (71)	12 (80)	12 (80)	34 (77)	11 (85)
Autoimmune disease	1 (7.1)	0	0	1 (2.3)	0
Idiopathic disease	3 (21)	3 (20)	3 (20)	9 (20)	2 (15)
Duration of HP (Years) (n)	14	15	15	44	13
Mean	12	9.3	12	11	13
Min, Max	1, 39	2, 29	3, 25	1, 39	3, 30
Renal Insufficiency History	1 (7.1)	3 (20)	1 (6.7)	5 (11)	0
Kidney Stones History	2 (14)	1 (6.7)	1 (6.7)	4 (9.1)	4 (31)
Ectopic Calcifications History	0	0	1 (6.7)	1 (2.3)	0
Vascular Calcifications History	0	0	0	0	0
Brain Calcification History	0	0	0	0	0
Cataract History	0	0	0	0	0
Seizure History	1 (7.1)	0	0	1 (2.3)	1 (7.7)

Baseline HP Supplements – PP

HP Supplements at Baseline collected by eDiary/ Total Daily Dose (TDD)	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Calcium /TDD (mg) (n)	14	14	15	43	13
Mean	1643	2395	2334	2129	1636
Min, Max	500, 4000	900, 8000	500, 4500	500, 8000	800, 3200
Calcium Category, n (%)					
≤ 2000 mg TDD	11 (79)	9 (60)	6 (40)	26 (59)	9 (69)
> 2000 mg TDD	3 (21)	5 (33)	9 (60)	17 (39)	4 (31)
Calcitriol (Active Vitamin D) /TDD (µg) (n)	10	11	13	34	8
Mean	1.025	0.750	0.750	0.831	0.719
Min, Max	0.50, 3.00	0.50, 1.25	0.50, 2.00	0.50, 3.00	0.50, 1.00
Alfacalcidol (Active Vitamin D) /TDD (µg) (n)	4	3	2	9	4
Mean	2.75	2.00	2.00	2.33	2.50
Min, Max	2.0, 4.0	1.0, 3.0	1.0, 3.0	1.0, 4.0	1.0, 4.0

2 subjects did not have eDiary information confirmed by prescription information

Baseline of Spot FECa & Albumin-Adjusted sCa – PP

Lab Summary at Baseline	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Albumin-Adjusted sCa (mg/dL) (n)	14	15	15	44	13
Mean (SD)	8.6 (0.49)	9.1 (1.3)	8.7 (0.62)	8.8 (0.91)	8.9 (0.39)
Spot AM FECa (%) (n)	14	15	15	44	13
Mean (SD)	2.5 (1.4)	3.3 (1.5)	2.4(1.2)	2.8 (1.4)	2.3 (0.76)
Spot AM FECa normal (≤ 2%) at baseline	7 (50%)	4 (27%)	8 (53%)	19 (43%)	5 (39%)

Treatment-Emergent Adverse Event Summary – SAS

	PTH 15 µg/day (N=14) n (%)	PTH 18 µg/day (N=15) n (%)	PTH 21 µg/day (N=15) n (%)	Total PTH (N=44) n (%)	Placebo (N=15) n (%)
TEAEs	6 (43)	3 (20)	8 (53)	17 (39)	5 (33)
Serious TEAE	0	0	0	0	0
Severity*					
Severe TEAE	0	0	0	0	0
Moderate TEAE	1 (7.1)	1 (6.7)	1 (6.7)	3 (6.8)	3 (20)
Mild TEAE	5 (36)	2 (13)	7 (47)	14 (32)	2 (13)
Related TEAE	3 (21)	1 (6.7)	5 (33)	9 (20)	1 (6.7)
Serious Related TEAE	0	0	0	0	0
TEAE Related to Hyper- or Hypocalcaemia Leading to ER/Urgent Care Visit and/or Hospitalization	0	0	0	0	0
TEAE Leading to Discontinuation of Study Drug	0	0	0	0	0
TEAE Leading to Discontinuation of Trial	0	0	0	0	0
TEAE Leading to Death	0	0	0	0	0

* Subjects are counted only in the highest severity category

Treatment-Emergent Adverse Events of Interest - SAS

Preferred Term	PTH 15 µg/day (N=14) n (%)	PTH 18 µg/day (N=15) n (%)	PTH 21 µg/day (N=15) n (%)	Total PTH (N=44) n (%)	Placebo (N=15) n (%)
TEAEs	6 (43)	3 (20)	8 (53)	17 (39)	5 (33)
Headache	3 (21)	1 (6.7)	2 (13)	6 (14)	0
Nausea	2 (14)	1 (6.7)	1 (6.7)	4 (9.1)	1 (6.7)
Fatigue	0	1 (6.7)	1 (6.7)	2 (4.5)	0
Injection site haemorrhage	1 (7.1)	0	1 (6.7)	2 (4.5)	0
Injection site pain	1 (7.1)	0	1 (6.7)	2 (4.5)	0
Thirst	0	1 (6.7)	1 (6.7)	2 (4.5)	0
Urinary tract infection	1 (7.1)	0	1 (6.7)	2 (4.5)	0
Hypertension	1 (7.1)	1 (6.7)	0	2 (4.5)	0
Hypercalcaemia	0	0	2 (13)	2 (4.5)	0
Hypocalcaemia	0	0	0	0	1 (6.7)

Includes ≥ 2 subjects in the PTH group or hyper- or hypocalcaemia

PaTH Forward 4-Week Fixed Dose Safety Summary

- All doses of TransCon PTH were well-tolerated
- No drop-outs during 4-week blinded period
- No serious or severe TEAEs were reported
- No TEAEs leading to discontinuation of study drug
- Overall incidence of TEAEs comparable between TransCon PTH and placebo
- TEAEs in TransCon arms reflect known PTH pharmacology
- Injections were well-tolerated using pen injector planned for commercial presentation

Titration algorithm to eliminate standard of care demonstrated no hypocalcaemic AEs

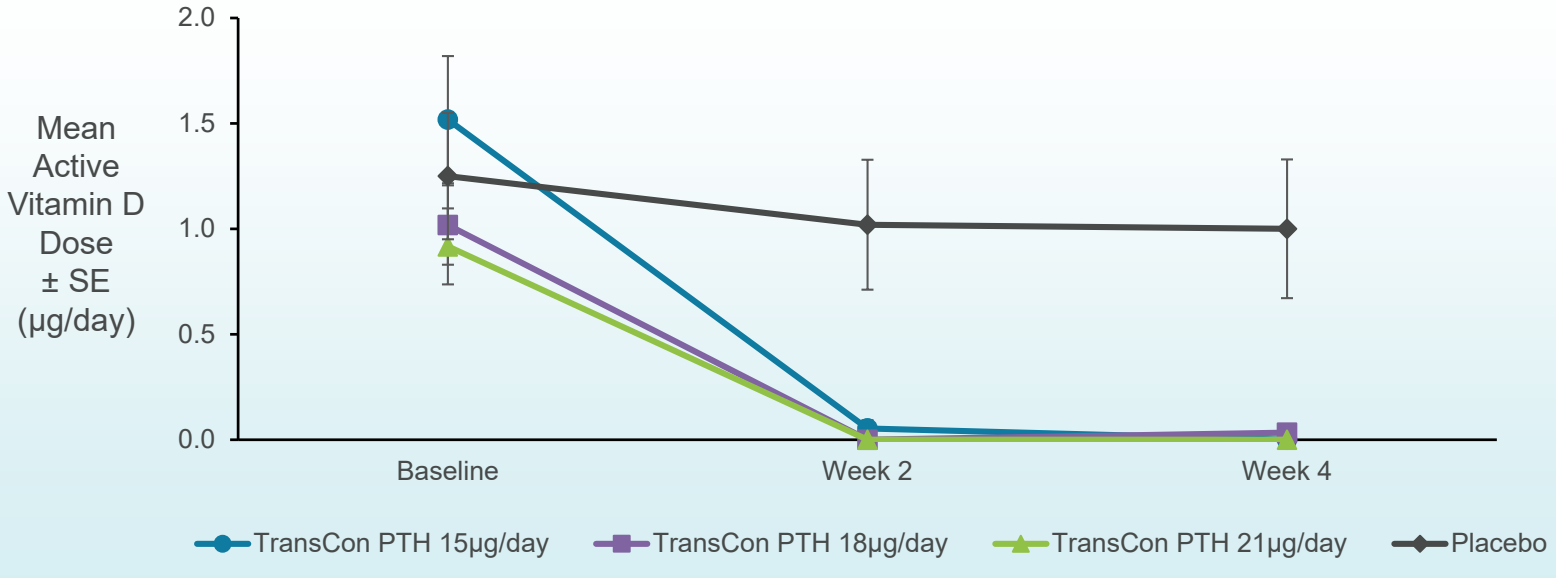
Elimination of Standard of Care – PP

Number of Subjects Meeting Each Component	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Not taking active vitamin D supplements	14 (100%)	14 (93%)	15 (100%)	43 (98%)	4 (31%)
Taking ≤1000 mg/day of calcium supplements	13 (93%)	13 (87%)	15 (100%)	41 (93%)	6 (46%)
Taking ≤500 mg/day of calcium supplements	12 (86%)	9 (60%)	15 (100%)	36 (82%)	2 (15%)
Taking 0 mg/day of calcium supplements	7 (50%)	7 (47%)	8 (53%)	22 (50%)	0
Not taking active vitamin D and 0 mg/day of calcium supplements	7 (50%)	7 (47%)	8 (53%)	22 (50%)	0
Not taking active vitamin D and ≤500 mg/day of calcium supplements	12 (86%)	9 (60%)	15 (100%)	36 (82%)	2 (15%)

100% of subjects in the 21 µg/day arm and 82% of all subjects across all TransCon PTH dosage arms were able to eliminate standard of care*

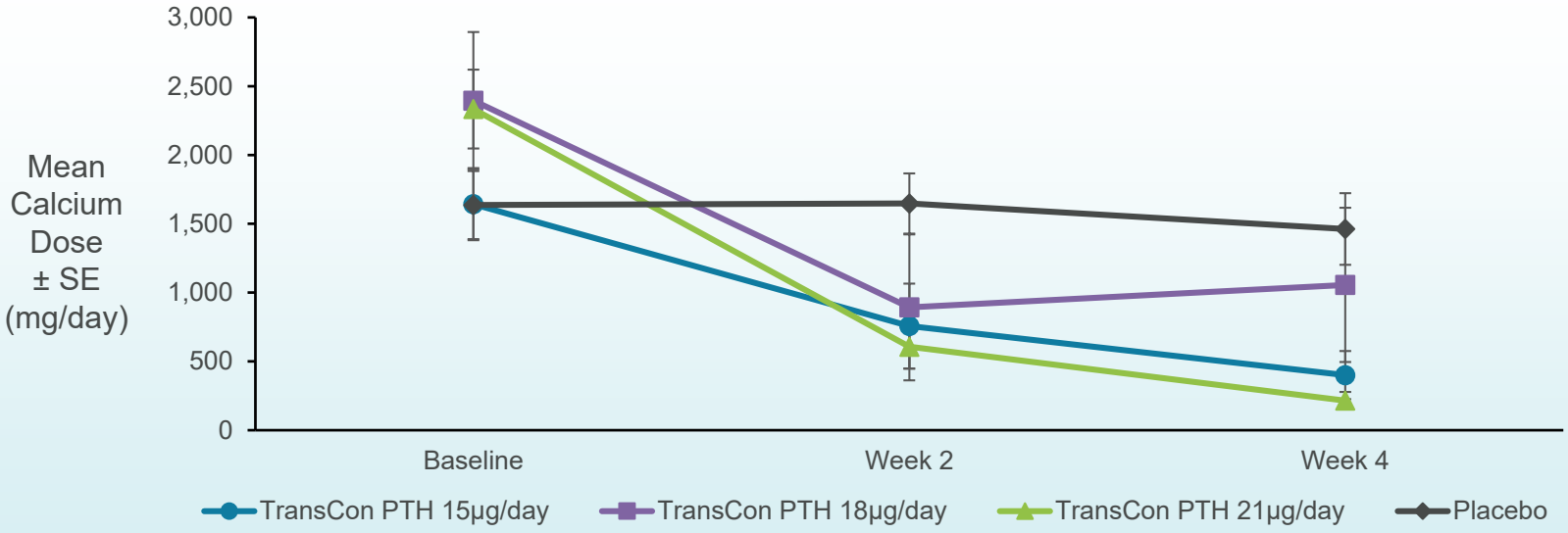
* Not taking active vitamin D and ≤500 mg/day of calcium supplements

Mean Active Vitamin D Dose by Visit – PP



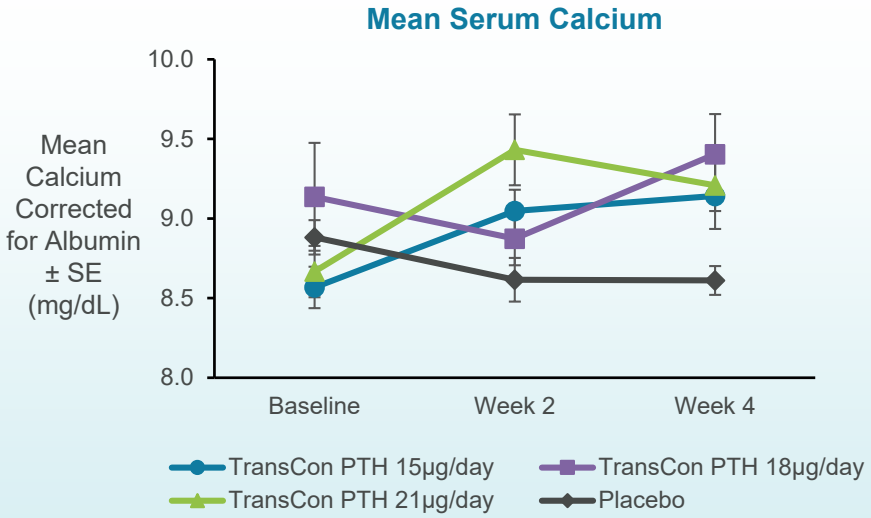
TransCon PTH enabled discontinuation of active vitamin D at week 2

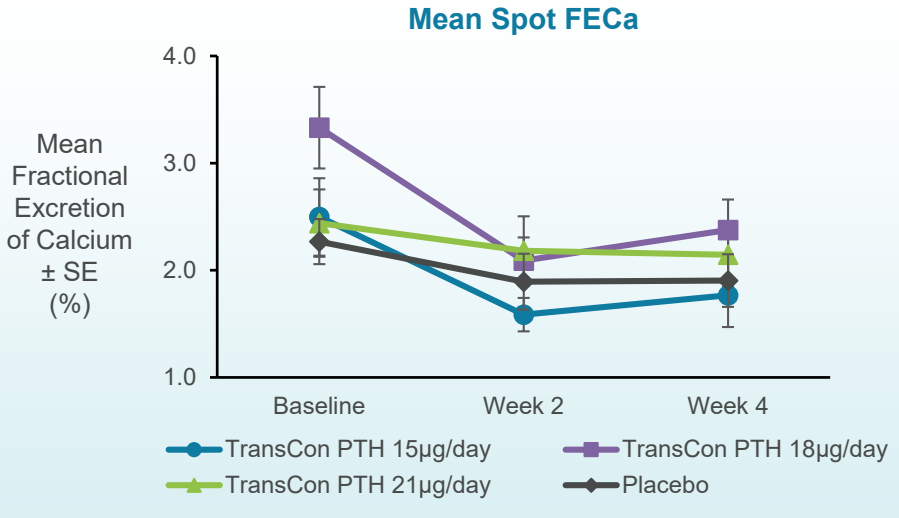
Mean Calcium Supplement Dose by Visit – PP



TransCon PTH enabled continuous calcium supplement reduction over 4-week study period

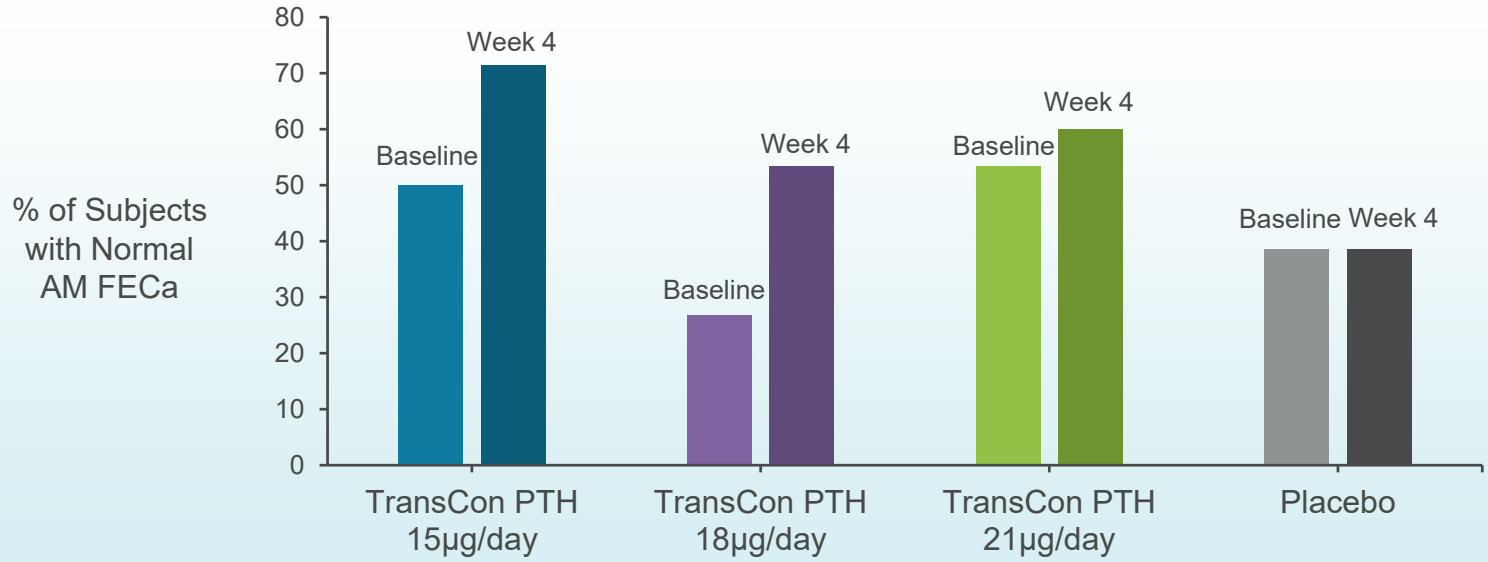
Mean Serum Calcium and Spot FECa by Visit – PP





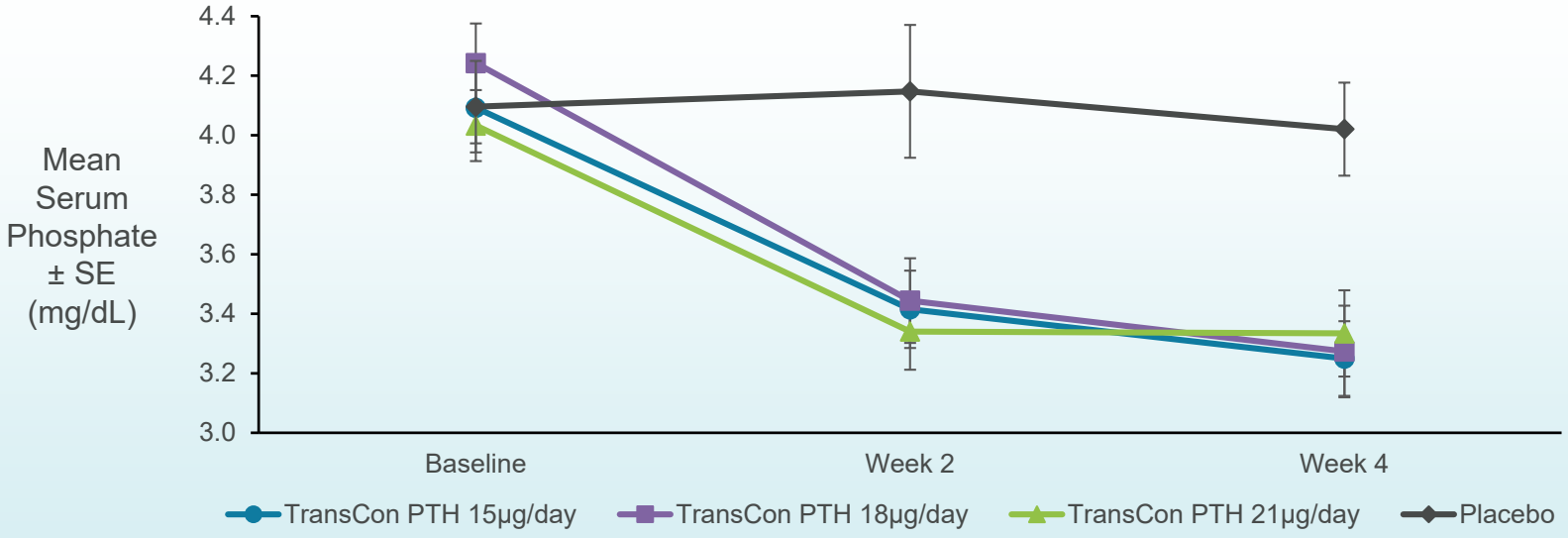
TransCon PTH subjects exhibited reduced FECa, despite increased serum calcium
For placebo subjects, FECa followed serum calcium levels

TransCon PTH Increased Number of FECa Responders – PP



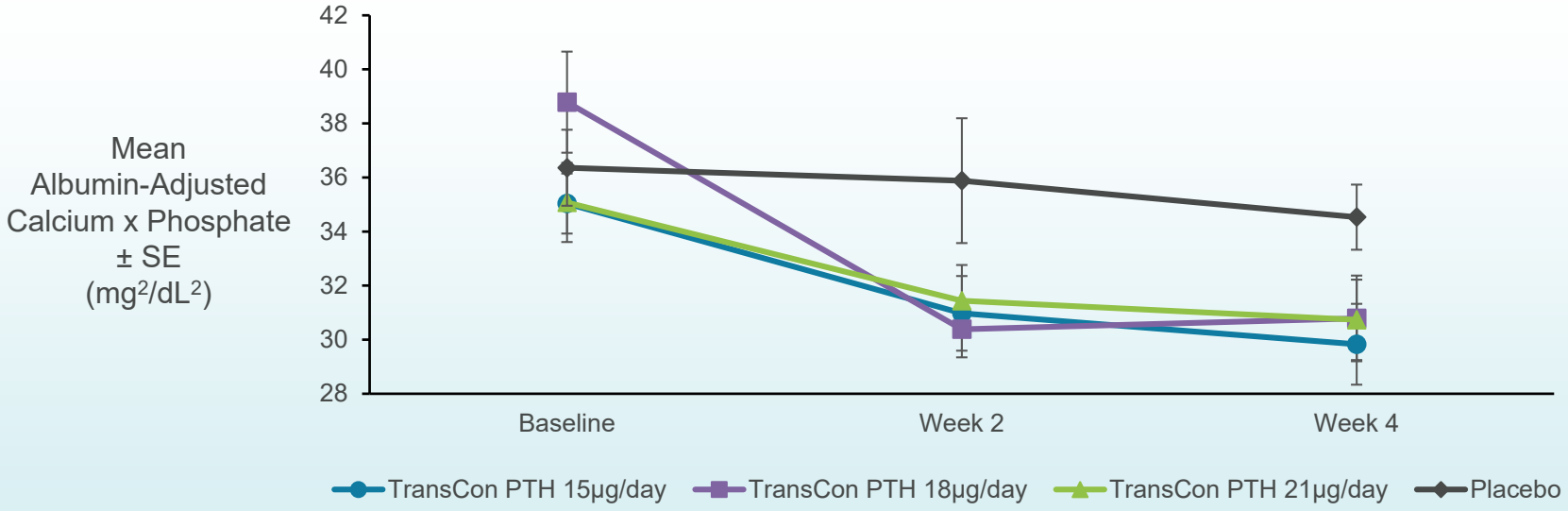
By week 4 of treatment, TransCon PTH had normalized an additional 8 subjects compared to none on placebo

Mean Serum Phosphate by Visit – PP



TransCon PTH subjects demonstrated consistent, sustained reductions in serum phosphate

Mean Calcium-Phosphate Product by Visit – PP



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

Primary Composite Endpoint at Week 4 – PP

	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Number of Subjects Meeting Primary Composite Endpoint at Week 4 with Fixed Dosing	7	6	9	22	2
Proportion (95% CI)	50 (23, 77)	40 (16, 68)	60 (32, 84)	50 (35, 65)	15 (1.9, 45)
P-value	0.10	0.22	0.02	0.03	
Number of Subjects Meeting Each Component:					
Serum calcium within the normal range, n (%)	12 (86%)	12 (80%)	14 (93%)	38 (86%)	12 (92%)
Below lower limit of normal (<8.3 mg/dL)	2	1	0	3	1
Above upper limit of normal (>10.6 mg/dL)	0	2	1	3	0
Spot AM FECa within normal range (≤2%) or a reduction by at least 50% from baseline, n (%)	10 (71%)	8 (53%)	9 (60%)	27 (61%)	5 (38%)
Not taking active vitamin D supplements, n (%)	14 (100%)	14 (93%)	15 (100%)	43 (98%)	4 (31%)
Taking ≤1000 mg/day of calcium supplements, n (%)	13 (93%)	13 (87%)	15 (100%)	41 (93%)	6 (46%)

The 21 µg/day arm and the combined TransCon PTH dosage arms showed a statistically significant response compared to placebo at week 4

Key Secondary Composite Endpoint at Week 4 – PP

PaTHforward TRIAL

	PTH 15 µg/day (N=14)	PTH 18 µg/day (N=15)	PTH 21 µg/day (N=15)	Total PTH Subjects (N=44)	Placebo (N=13)
Number of Subjects Meeting Key Secondary Composite Endpoint at Week 4 with Fixed Dosing	7	4	9	20	2
Proportion (95% CI)	50 (23, 77)	27 (7.8, 55)	60 (32, 84)	45 (30, 61)	15 (1.9, 45)
P-value	0.10	0.65	0.02	0.06	
Number of Subjects Meeting Each Component:					
Serum calcium within the normal range, n (%)	12 (86%)	12 (80%)	14 (93%)	38 (86%)	12 (92%)
Below lower limit of normal (<8.3 mg/dL)	2	1	0	3	1
Above upper limit of normal (>10.6 mg/dL)	0	2	1	3	0
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The 21 µg/day arm showed a statistically significant response compared to placebo at week 4

Key Takeaways from the PaTH Forward Trial – Primary Endpoint

- PaTH Forward Trial met the primary endpoint

- Overall statistical significance achieved notwithstanding:
  - Short study duration of 4 weeks
  - Fixed dose not individualized for each subject's optimal dose
  - Subjects continued to titrate off calcium supplements
  - Small study population

Open-label Extension Trial

- Subjects from fixed-dose PaTH Forward Trial rolled over to the open-label extension which enabled individually optimized TransCon PTH dosing to evaluate long-term safety and efficacy
- 58 out of 59 randomized subjects currently receiving TransCon PTH in the open-label extension
  - Both placebo responders continue in the open-label extension
  - One subject (randomized to placebo) withdrew for reasons unrelated to safety or efficacy of the study drug
- Long-term data from open-label extension evaluates a composite endpoint. Evaluating proportion of subjects with:
  - Normal serum calcium; ***and***
  - Off active vitamin D; ***and***
  - Taking ≤500 mg/day calcium; ***and***
  - Normal 24-hour urine calcium excretion (or at least 50% decrease from baseline)

Planned Next Steps

- Engage with global regulatory authorities on next steps for development of TransCon PTH
- Report PaTH Forward open-label extension six-month data in Q3 2020
- Submit proposed PRO instrument for FDA review in Q3 2020
- Submit regulatory filings to initiate a global phase 3 trial in North America, Europe and Asia in Q4 2020:
  - Ethnobridging study showed comparable PK profile between Japanese and non-Japanese populations, enabling inclusion of Japan in global phase 3 program

PaTH Forward Top-Line Data from 4-Week Fixed Dose Period

- PaTH Forward top-line data support TransCon PTH as a potential replacement therapy for adult HP
  - TransCon PTH eliminated standard of care (i.e. off active vitamin D and ≤ 500 mg per day of calcium supplements) in 100% of subjects in the 21 µg/day arm and in 82% of subjects across all dosage arms
  - Both the 21 µg/day arm and the combined TransCon PTH dosage arms showed a statistically significant response for the primary endpoint compared to placebo at 4 weeks
  - TransCon PTH increased mean serum calcium
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  - TransCon PTH reduced mean serum phosphate and calcium-phosphate product
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  - Overall incidence of TEAEs comparable between TransCon PTH and placebo
- No drop-outs in blinded period