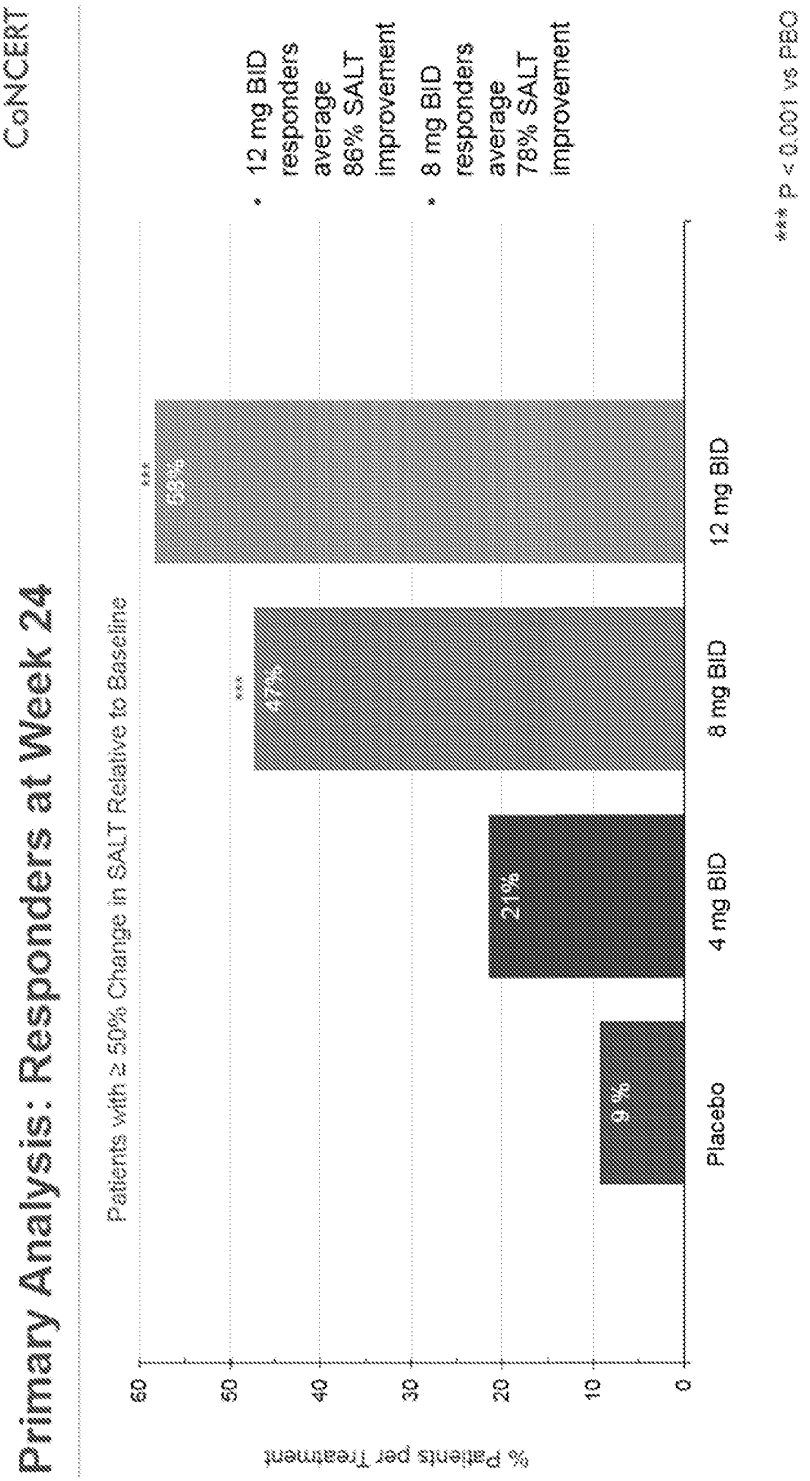

CoNCERT
Primary Analysis: Responders at Week 24
Patients with ≥ 50% Change in SALT Relative to Baseline
% Patients per Treatment
60
50
40
30
20
10
0
9%
21%
47%
***
53%
***
Placebo
4 mg BID
8 mg BID
12 mg BID
* 12 mg BID responders average 86% SALT improvement
* 8 mg BID responders average 78% SALT improvement
*** P < 0.001 vs PBO

FIG. 1

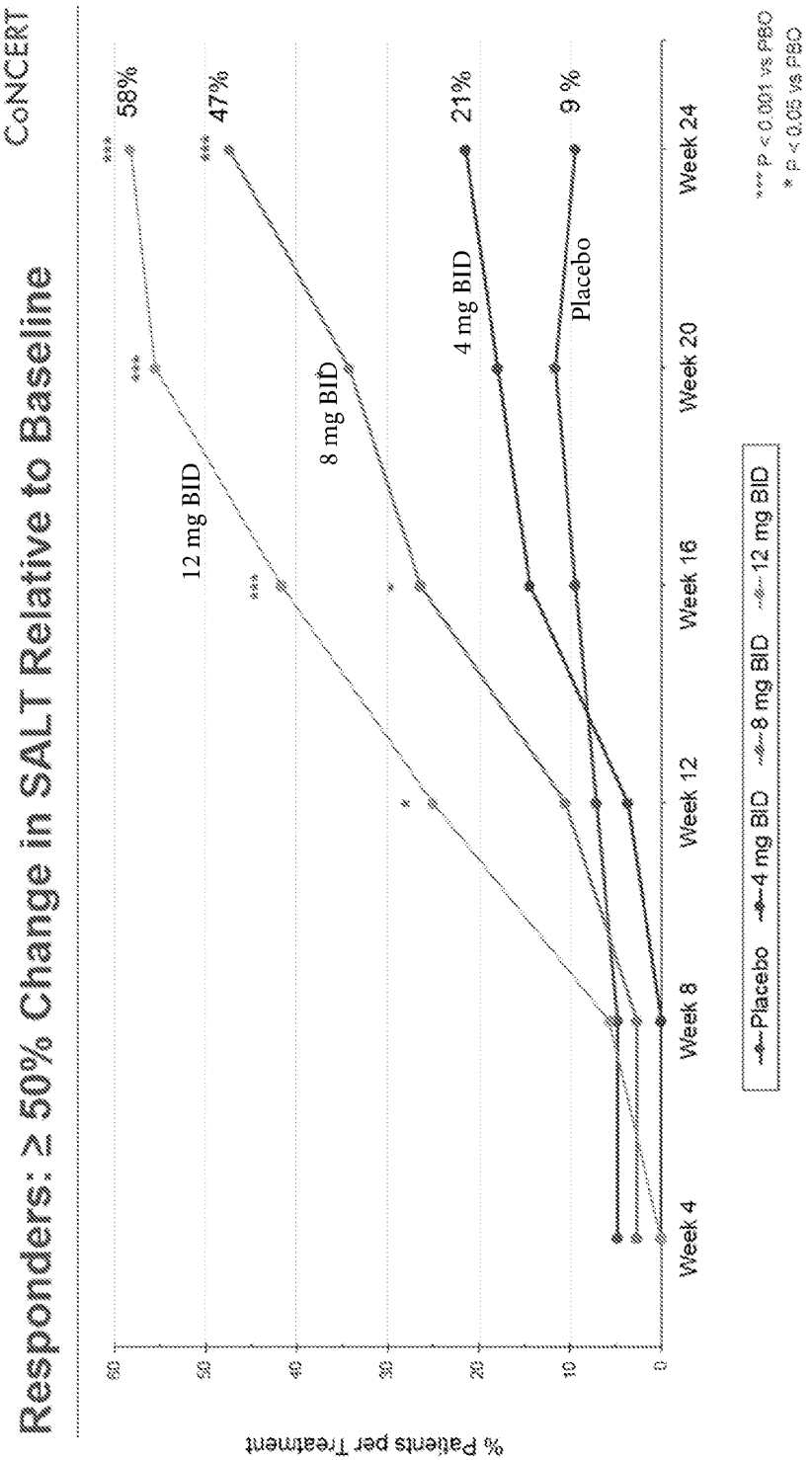

Responders: ≥ 50% Change in SALT Relative to Baseline
CONCERT
% Patients per Treatment
60
50
40
30
20
10
0
Week 4
Week 8
Week 12
Week 16
Week 20
Week 24
58%
47%
21%
9 %
12 mg BID
8 mg BID
4 mg BID
Placebo
*** P < 0.001 vs PBO
* p < 0.05 vs PBO
Placebo
4 mg BID
8 mg BID
12 mg BID

FIG. 2

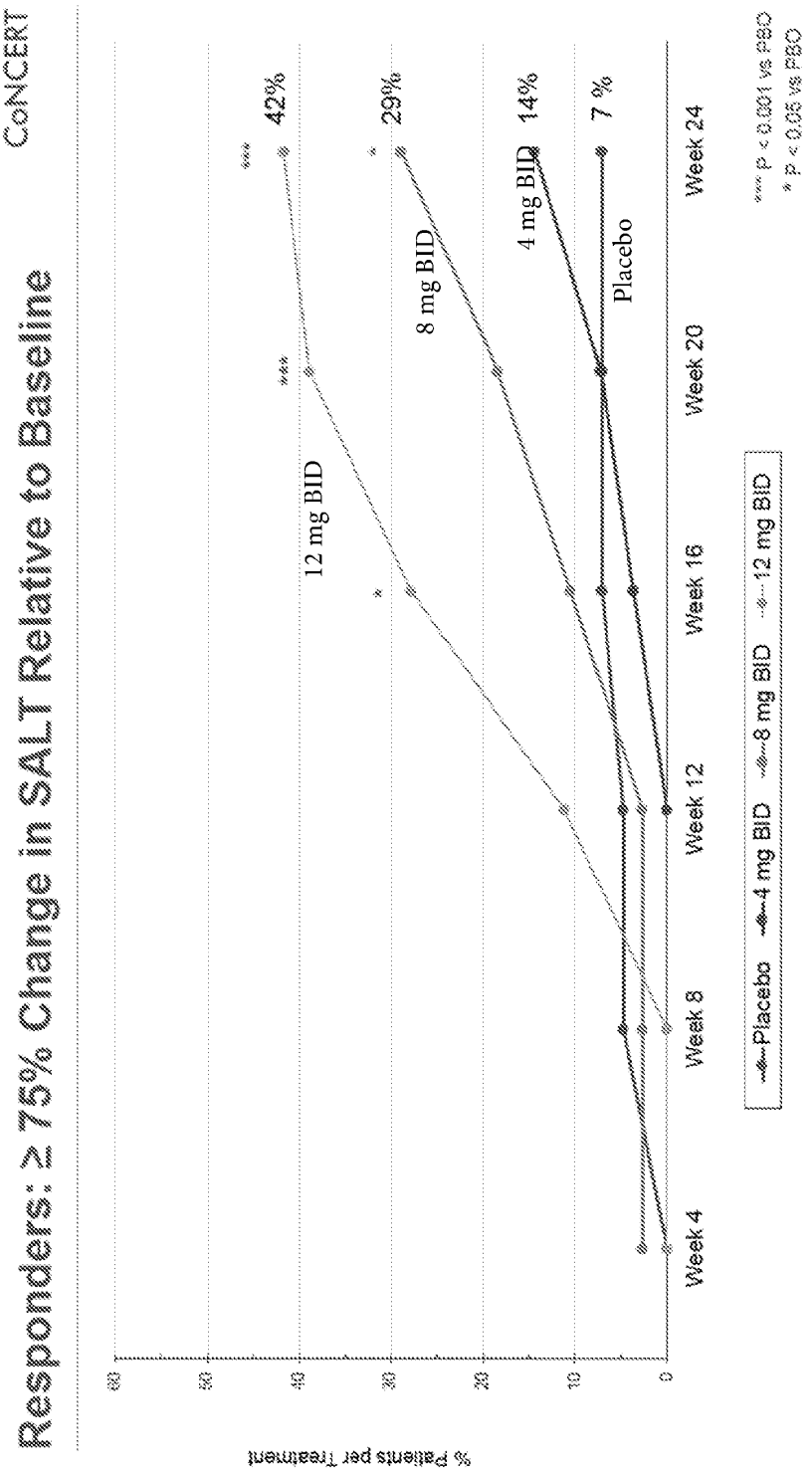

Responders: ≥ 75% Change in SALT Relative to Baseline
CONCERT
% Patients per Treatment
60
50
40
30
20
10
0
Week 4
Week 8
Week 12
Week 16
Week 20
Week 24
42%
29%
14%
7 %
12 mg BID
8 mg BID
4 mg BID
Placebo
***
*
Placebo
4 mg BID
8 mg BID
12 mg BID
*** p < 0.001 vs PBO
* p < 0.05 vs PBO

FIG. 3

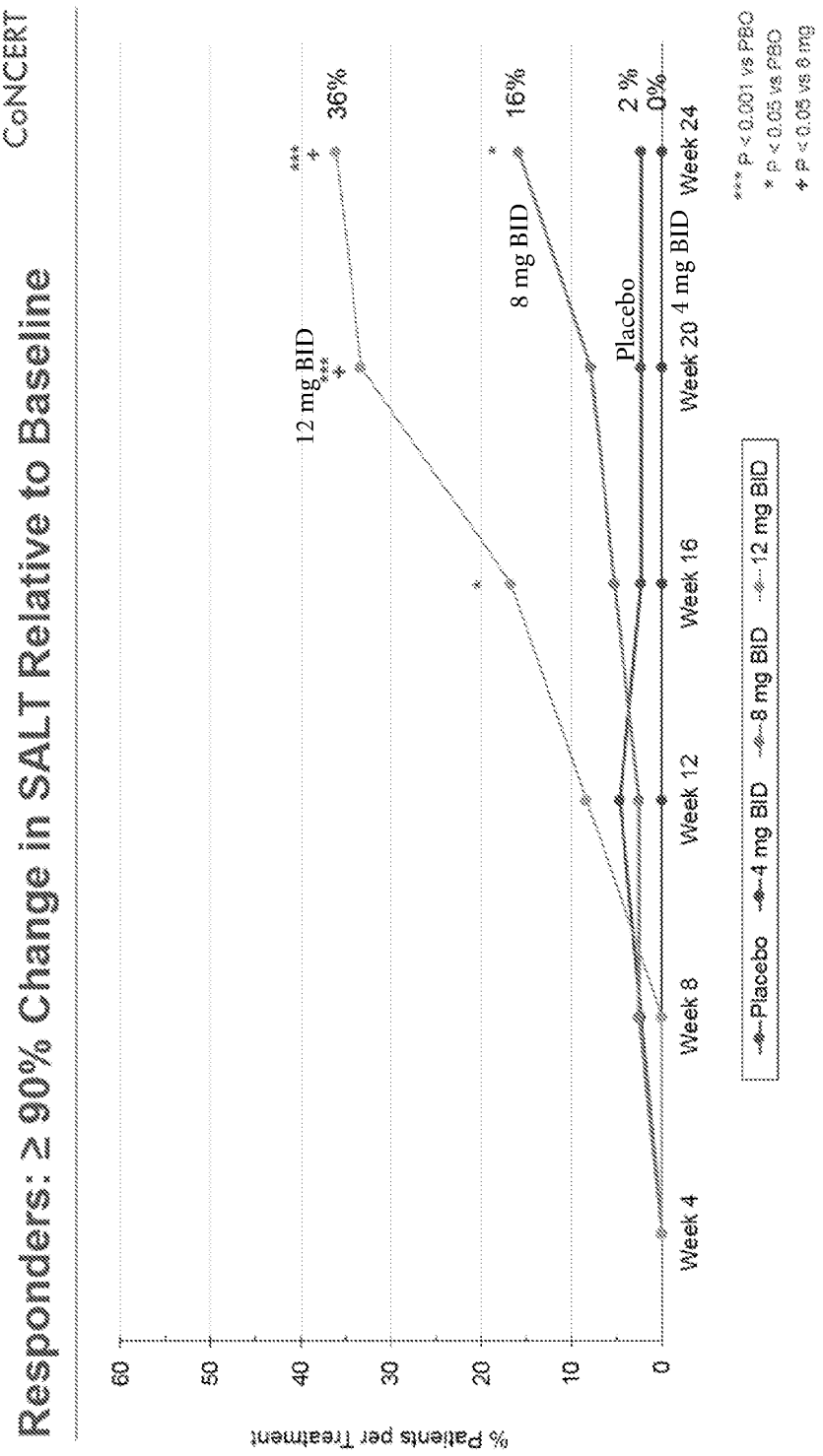

Responders: ≥ 90% Change in SALT Relative to Baseline
CONCERT
% Patients per Treatment
60
50
40
30
20
10
0
Week 4
Week 8
Week 12
Week 16
Week 20
Week 24
12 mg BID
36%
8 mg BID
16%
Placebo
2 %
4 mg BID
0%
*** p < 0.001 vs PBO
* p < 0.05 vs PBO
+ p < 0.05 vs 8 mg
Placebo
4 mg BID
8 mg BID
12 mg BID

FIG. 4

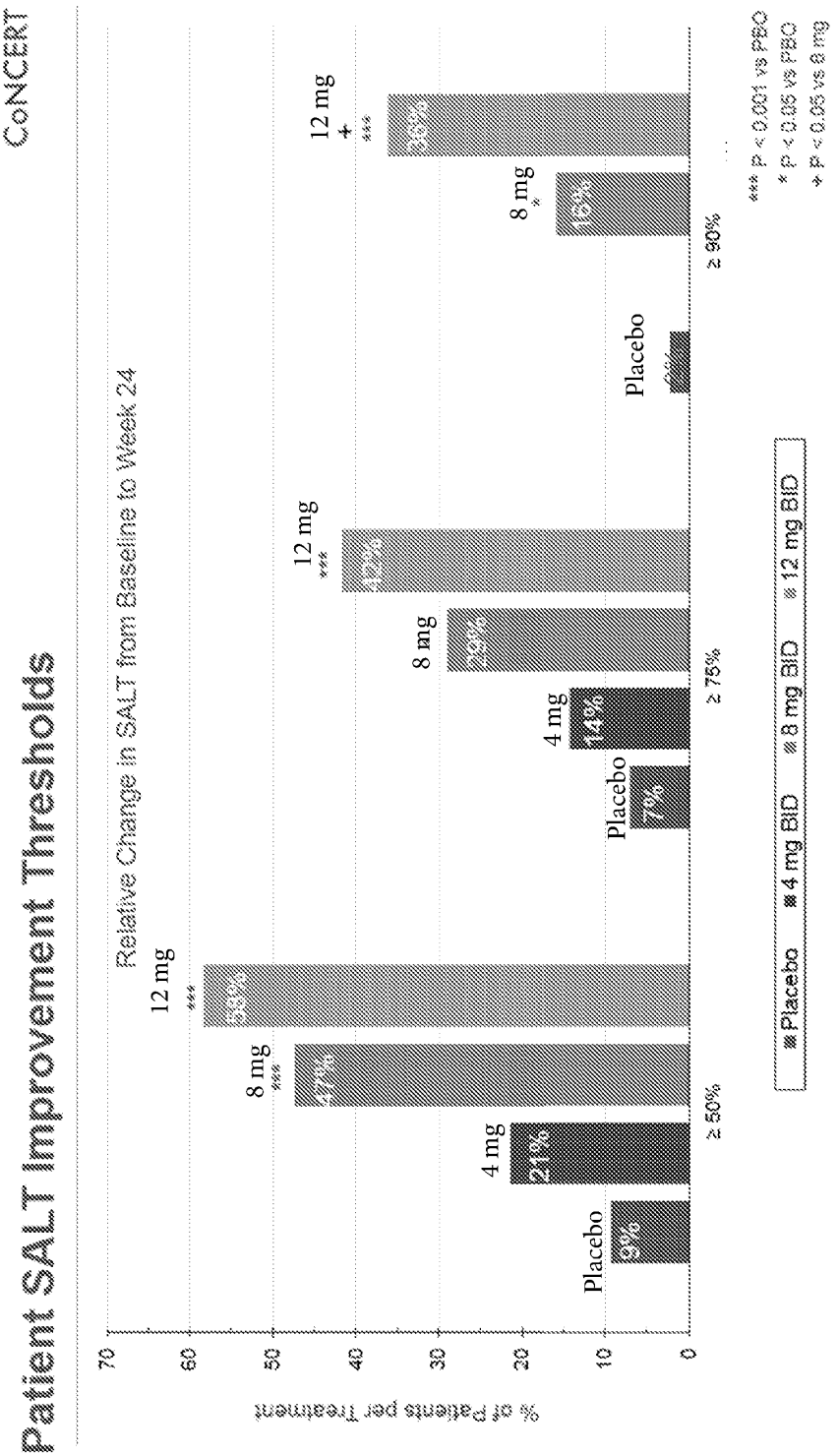

CoNCERT
Patient SALT Improvement Thresholds
Relative Change in SALT from Baseline to Week 24
% of Patients per Treatment
70
60
50
40
30
20
10
0
12 mg
***
53%
8 mg
***
47%
4 mg
21%
Placebo
9%
≥ 50%
12 mg
***
32%
8 mg
*
23%
4 mg
14%
Placebo
7%
≥ 75%
12 mg
+
***
26%
8 mg
*
16%
Placebo
≥ 90%
*** P < 0.001 vs PBO
* P < 0.05 vs PBO
+ P < 0.05 vs 8 mg
Placebo
4 mg BID
8 mg BID
12 mg BID

FIG. 5

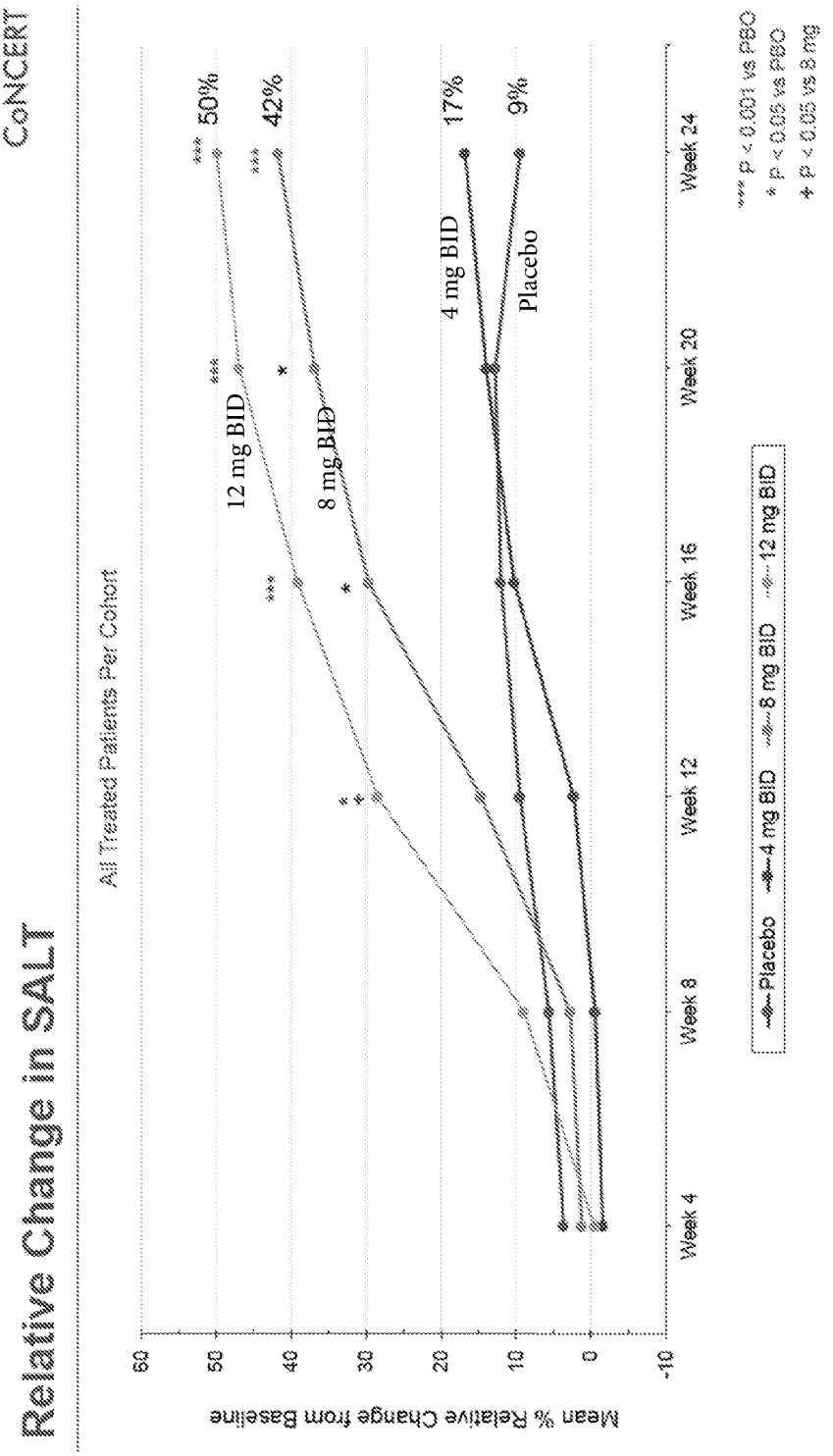

Relative Change in SALT
CONCERT
All Treated Patients Per Cohort
Mean % Relative Change from Baseline
50
40
30
20
10
0
-10
Week 4
Week 8
Week 12
Week 16
Week 20
Week 24
12 mg BID
8 mg BID
4 mg BID
Placebo
*** 50%
*** 42%
17%
9%
Placebo
4 mg BID
8 mg BID
12 mg BID
*** p < 0.001 vs PBO
* p < 0.05 vs PBO
+ p < 0.05 vs 8 mg

FIG. 6

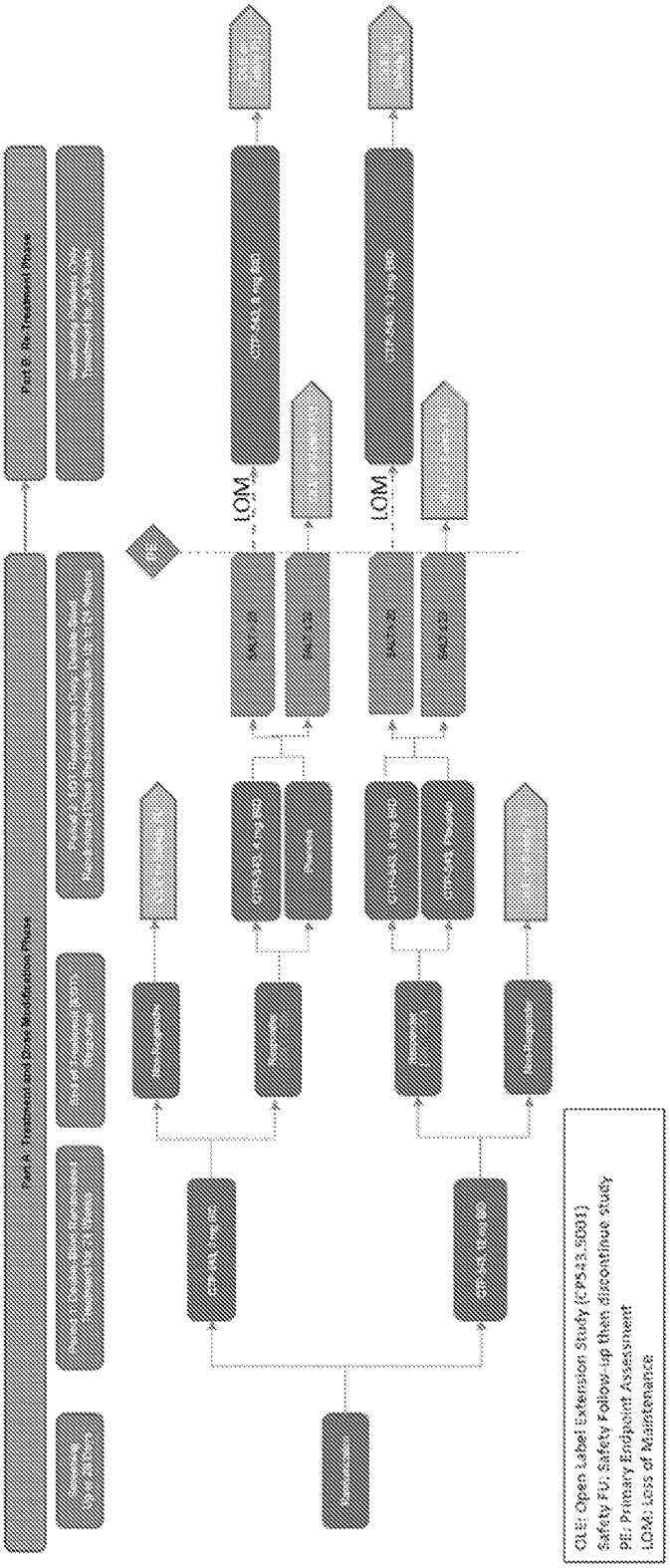

LOM
LOM
OLE: Open Label Extension Study (ECP543.5001)
Safety FU: Safety Follow-up Post discontinue Study
PE: Primary Endpoint Assessment
LOM: Loss of Maintenance

FIG. 7

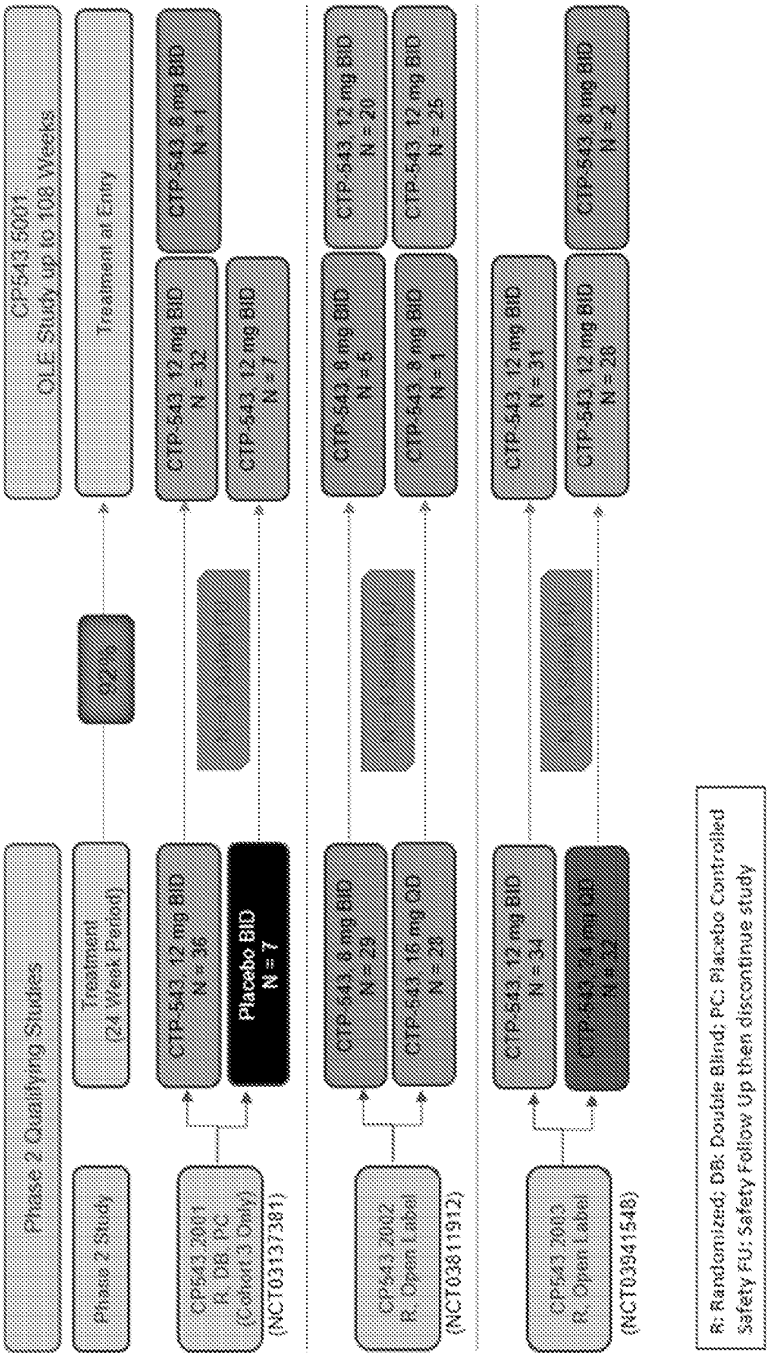

Phase 2 Qualifying Study
Phase 2 Study
Treatment (24 Week Period)
CTP-543 12 mg BID N = 36
Placebo BID N = 7
CPP543 2001 R, DB, PC (Cohort 3 Only) (NCT03137381)
CTP-543 12 mg BID N = 32
CTP-543 12 mg BID N = 7
CTP-543 8 mg BID N = 1
CPP543 5001 OLE Study up to 108 Weeks
Treatment at Entry
CTP-543 8 mg BID N = 29
CTP-543 16 mg QD N = 29
CPP543 2002 R, Open Label (NCT03811912)
CTP-543 8 mg BID N = 4
CTP-543 8 mg BID N = 1
CTP-543 12 mg BID N = 20
CTP-543 12 mg BID N = 25
CTP-543 12 mg BID N = 34
CTP-543 24 mg QD N = 32
CPP543 3003 R, Open Label (NCT03941548)
CTP-543 12 mg BID N = 31
CTP-543 12 mg BID N = 28
CTP-543 8 mg BID N = 2
R: Randomized; DB: Double Blind; PC: Placebo Controlled
Safety FU: Safety Follow Up then discontinue study

FIG. 8