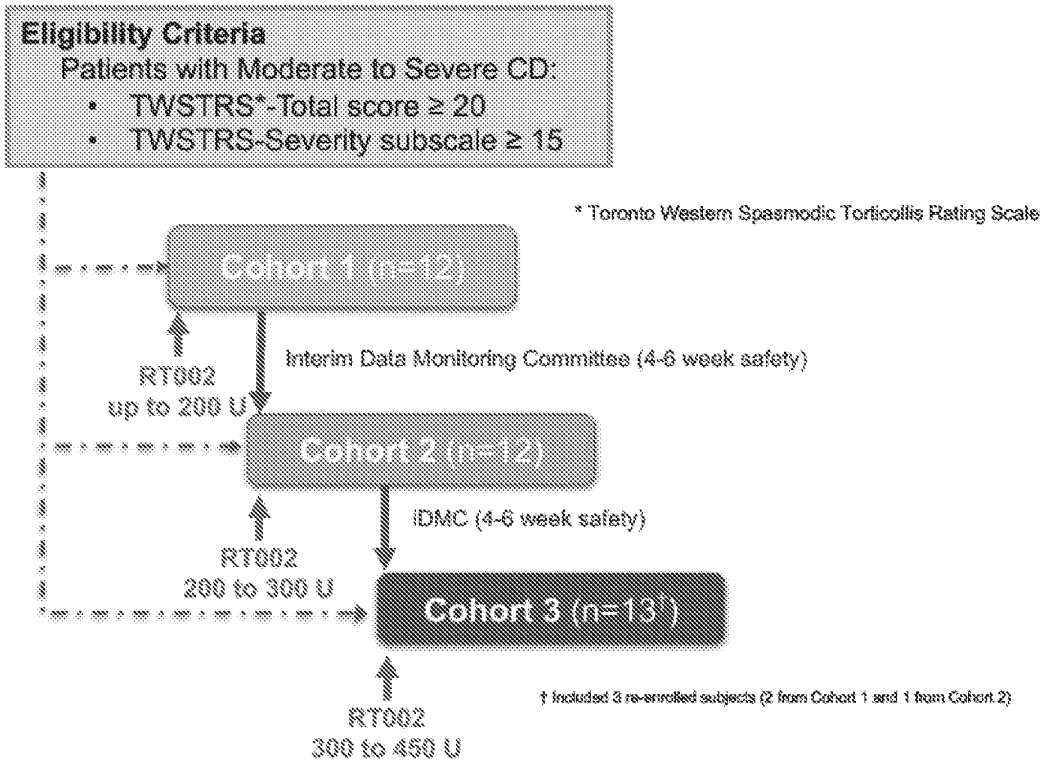

Eligibility Criteria
Patients with Moderate to Severe CD:
• TWSTRS*-Total score ≥ 20
• TWSTRS-Severity subscale ≥ 15
* Toronto Western Spasmodic Torticollis Rating Scale
Cohort 1 (n=12)
RT002
up to 200 U
Interim Data Monitoring Committee (4-6 week safety)
Cohort 2 (n=12)
RT002
200 to 300 U
IDMC (4-6 week safety)
Cohort 3 (n=13†)
RT002
300 to 450 U
† Included 3 re-enrolled subjects (2 from Cohort 1 and 1 from Cohort 2)

FIG. 1

	Cohort 1 (N=12)	Cohort 2 (N=12)	Cohort 3 (n=13)	All (n=37)
Mean age (range)	57 (46–74)	52 (32–70)	58 (30–69)	56 (30–74)
Females, n (%)	11 (92%)	8 (67%)	9 (69%)	28 (76%)
Mean RT002 dose, U, (range)	174 (100 to 200)	229 (200 to 300)	323 (300 to 450)	244 (100 to 450)
Mean CD Disease Duration, years, (range) Median	8.5 (0.4—21.7) 7.5	5.1 (0.02—24.1) 1.2	9.0 (0.6—23.3) 8.1	7.6 (0.02—24.1) 4.9
Mean Baseline TWSTRS Total Score	43.8	44.9	43.7	44.1
Prior Treatment with BotulinumtoxinA	5 (42%)	4 (33%)	8 (62%)*	17 (46%)

*Three subjects received RT002 in Cohort 1 or 2, and re-enrolled to Cohort 3.

FIG. 2

	Cohort 1 (N=12)	Cohort 2 (N=12)	Cohort 3 (n=13)	All (n=37)
Number of treatment-related AE's	8	8	6	22
Subjects with any treatment-related AE, n (%)*	6 (50%)	5 (42%)	2 (15%)	13 (35%)
Dysphagia	1 (8%)	2 (17%)	2 (15%)	5 (14%)**
Injection site erythema	2 (17%)	0	1 (8%)	3 (8%)
Injection site bruising	2 (17%)	0	0	2 (5%)
Muscular weakness	2 (17%)†	0	0	2 (5%)†
Injection site pain	0	1 (8%)	1 (8%)	2 (5%)
Muscle tightness	0	1 (8%)	1 (8%)	2 (5%)
Neck pain	1 (8%)‡	0	0	1 (3%)‡
Fatigue	0	1 (8%)	0	1 (3%)
Muscle spasms	0	1 (8%)	0	1 (3%)
Nausea	0	1 (8%)	0	1 (3%)
Trismus	0	1 (8%)	0	1 (3%)
Asthenia	0	0	1 (8%)	1 (3%)

* Multiple events in a subject counted only once; **All cases of dysphagia were mild in severity
† Muscle weakness included a mild (cervical paraspinal) and a moderate (cervical extensor) case
‡ A severe neck pain occurred at Day 10 and lasted for 2 days (200U)

FIG. 3

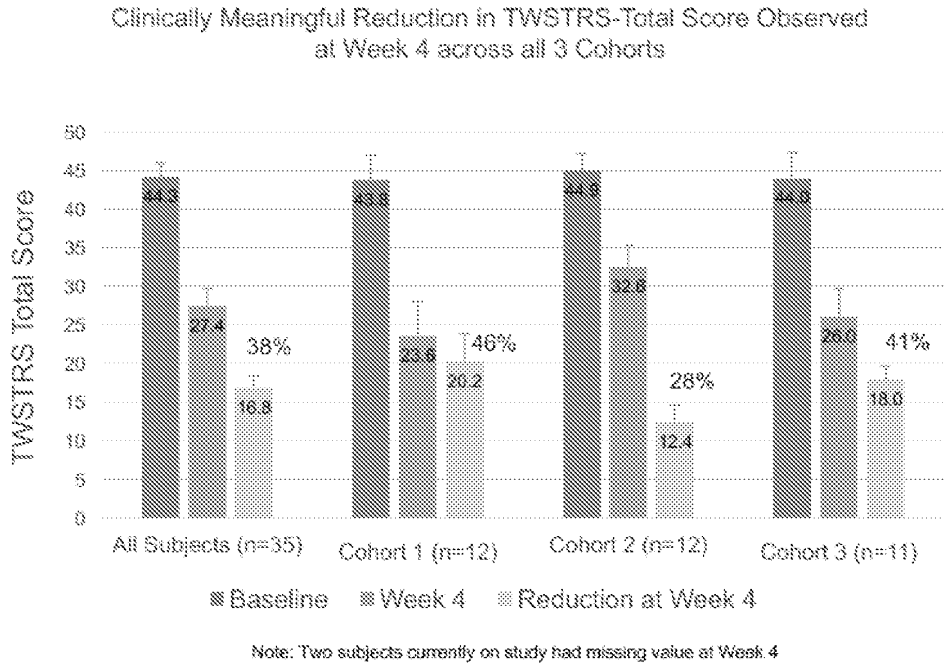

Clinically Meaningful Reduction in TWSTRS-Total Score Observed at Week 4 across all 3 Cohorts
TWSTRS Total Score
50
45
40
35
30
25
20
15
10
5
0
44.2
27.4
38%
16.8
43.8
23.6
46%
20.2
44.8
32.8
28%
12.4
44.0
26.0
41%
18.0
All Subjects (n=35)
Cohort 1 (n=12)
Cohort 2 (n=12)
Cohort 3 (n=11)
Baseline
Week 4
Reduction at Week 4
Note: Two subjects currently on study had missing value at Week 4

FIG. 4

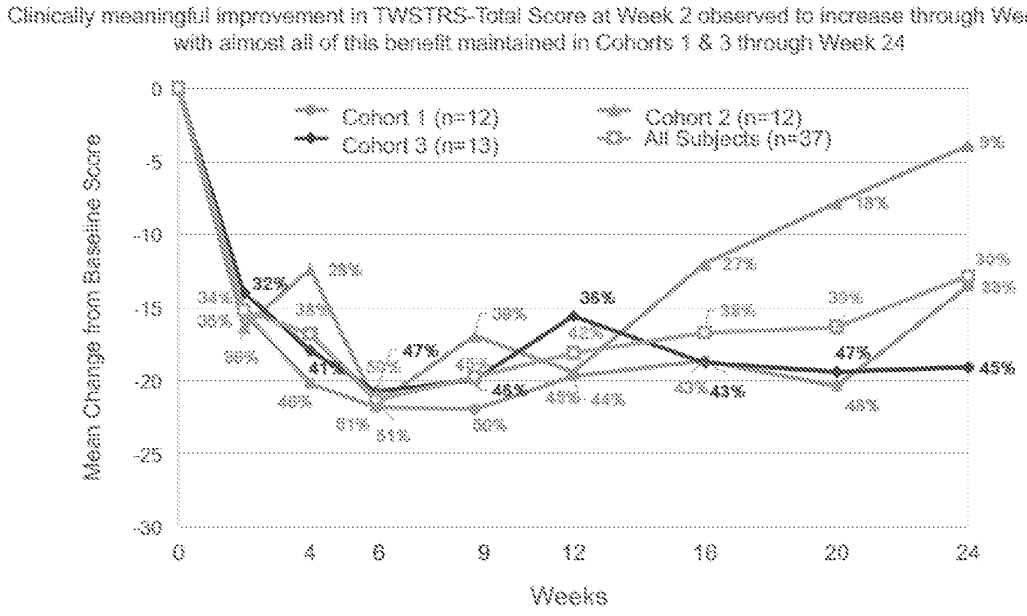

Clinically meaningful improvement in TWSTRS-Total Score at Week 2 observed to increase through Week 6, with almost all of this benefit maintained in Cohorts 1 & 3 through Week 24
Cohort 1 (n=12)
Cohort 2 (n=12)
Cohort 3 (n=13)
All Subjects (n=37)
Mean Change from Baseline Score
0
-5
-10
-15
-20
-25
-30
0
4
6
9
12
16
20
24
Weeks
32%
38%
47%
41%
38%
45%
43%
47%
45%

FIG. 5

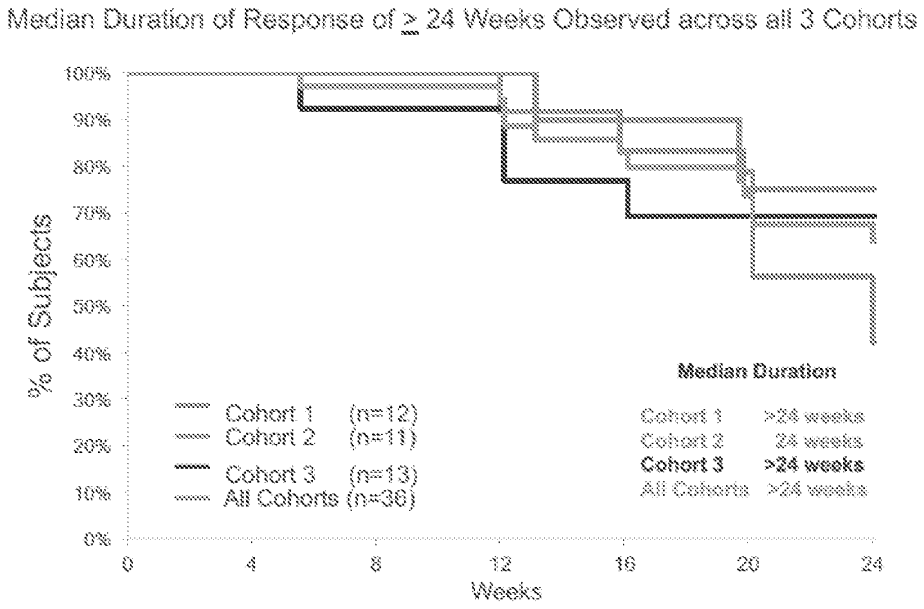

Median Duration of Response of ≥ 24 Weeks Observed across all 3 Cohorts
% of Subjects
100%
90%
80%
70%
60%
50%
40%
30%
20%
10%
0%
0
4
8
12
16
20
24
Weeks
Cohort 1 (n=12)
Cohort 2 (n=11)
Cohort 3 (n=13)
All Cohorts (n=36)
Median Duration
Cohort 1 >24 weeks
Cohort 2 24 weeks
Cohort 3 >24 weeks
All Cohorts >24 weeks

FIG. 6

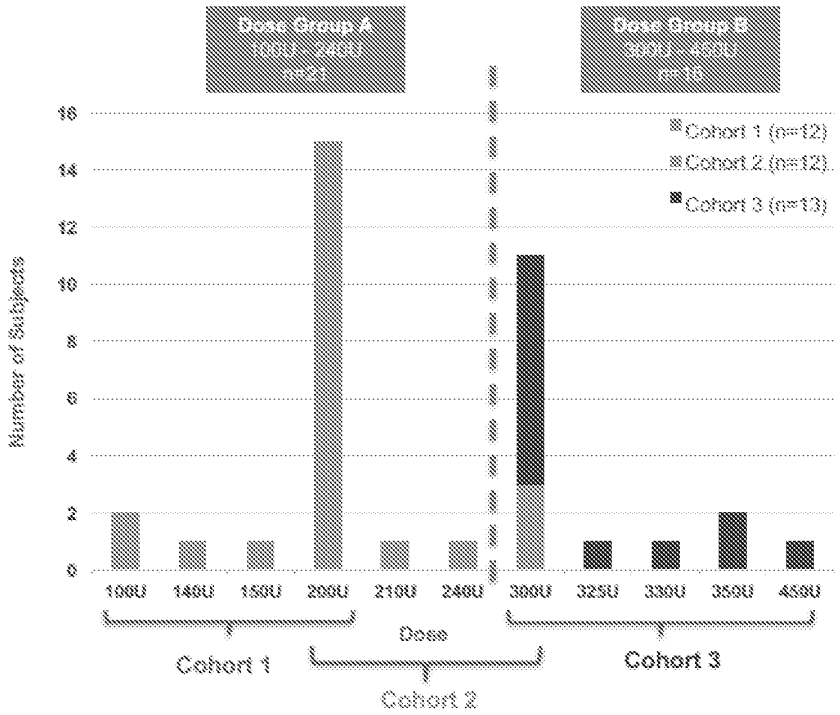

Dose Group A
100U - 240U
n=21
Dose Group B
300U - 450U
n=13
Cohort 1 (n=12)
Cohort 2 (n=12)
Cohort 3 (n=13)
Number of Subjects
0
2
4
6
8
10
12
14
16
100U
140U
150U
200U
210U
240U
300U
325U
330U
350U
450U
Dose
Cohort 1
Cohort 2
Cohort 3

FIG. 7

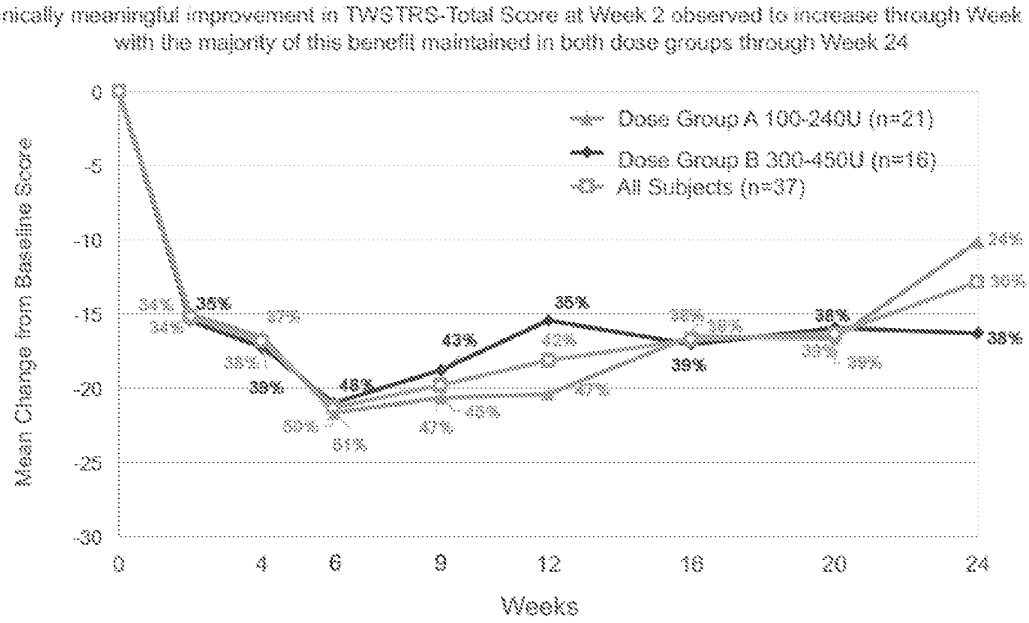

Clinically meaningful improvement in TWSTRS-Total Score at Week 2 observed to increase through Week 6, with the majority of this benefit maintained in both dose groups through Week 24
Dose Group A 100-240U (n=21)
Dose Group B 300-450U (n=16)
All Subjects (n=37)
Mean Change from Baseline Score
0
-5
-10
-15
-20
-25
-30
0
4
6
9
12
16
20
24
Weeks
34%
35%
39%
43%
35%
39%
38%
38%
26%
36%

FIG. 8