
29TH CONGRESS
EADVIRTUAL
29th – 31st October 2020
NEW FRONTIERS
IN DERMATOLOGY AND VENEREOLOGY

European Academy of Dermatology and Venereology Virtual Congress
Late Breaking News Session October 29, 2020

Initial Results from a Long-Term, Open-Label Extension Study with CTP-543, an Oral Janus Kinase Inhibitor, in Patients with Moderate to Severe Alopecia Areata

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Concert Pharmaceuticals, Inc.

ClinicalTrials.gov Identifier: NCT03898479

Disclosures of Relationship with Industry

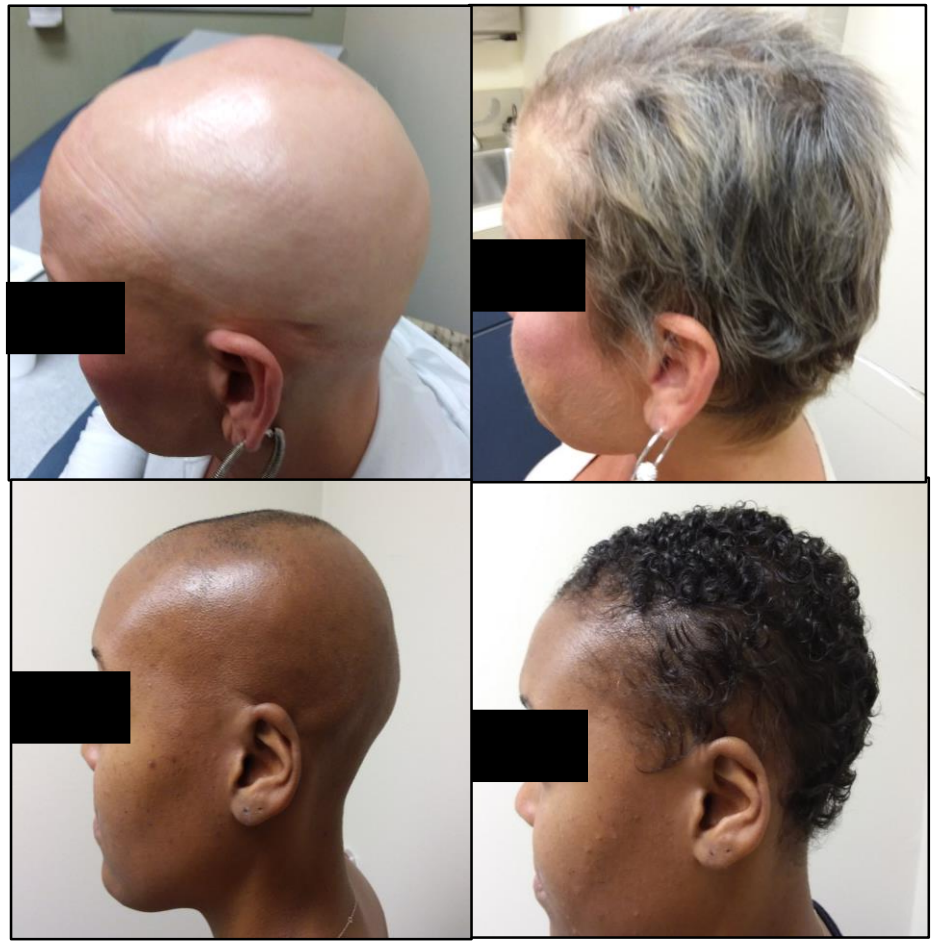
James V. Cassella, PhD

DISCLOSURES

- Concert Pharmaceuticals: Employee; Salary and Stock Received

Alopecia Areata Background

- A devastating and poorly treated autoimmune disease
- Alopecia Areata occurs worldwide
  - Prevalence of approximately 0.2% of the population with a lifetime risk of 1.7% - 2%*
- Chronic condition affecting women, men and children of all ages
- Disease profoundly impacts patients; associated with anxiety, depression and other autoimmune conditions
- Few treatment options
  - In the US, no FDA-approved treatment options



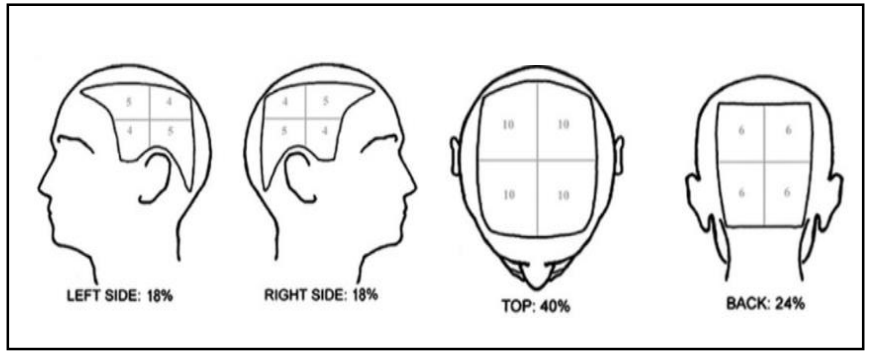
CTP-543 12 mg dose: Baseline and Week 24

*Safavi et al., 1995; Benigno et al., 2020

Study CP543.5001: Long-Term, Open-Label Extension (OLE) Study

- Objective: Evaluate long-term safety and effects of CTP-543 on treating hair loss in adult patients with moderate to severe alopecia areata.
  - Study is ongoing; Current trial duration is 108 weeks
  - Patients previously completing 24-weeks of treatment in a qualifying CTP-543 clinical trial are eligible
    - Three qualifying Phase 2 studies to date
    - First patient enrolled in April 2019
- Treatment: Patients receive 8 mg BID or 12 mg BID
  - Dose selection at the discretion of the investigator, based on efficacy and tolerability from the previous study
  - Dose adjustments allowed during study
- Assessments monthly for first 3 months, bimonthly thereafter
  - Safety is evaluated by clinical laboratory measurements, AEs and physical exams.
  - Hair assessments performed using the Severity of Alopecia Tool (SALT)

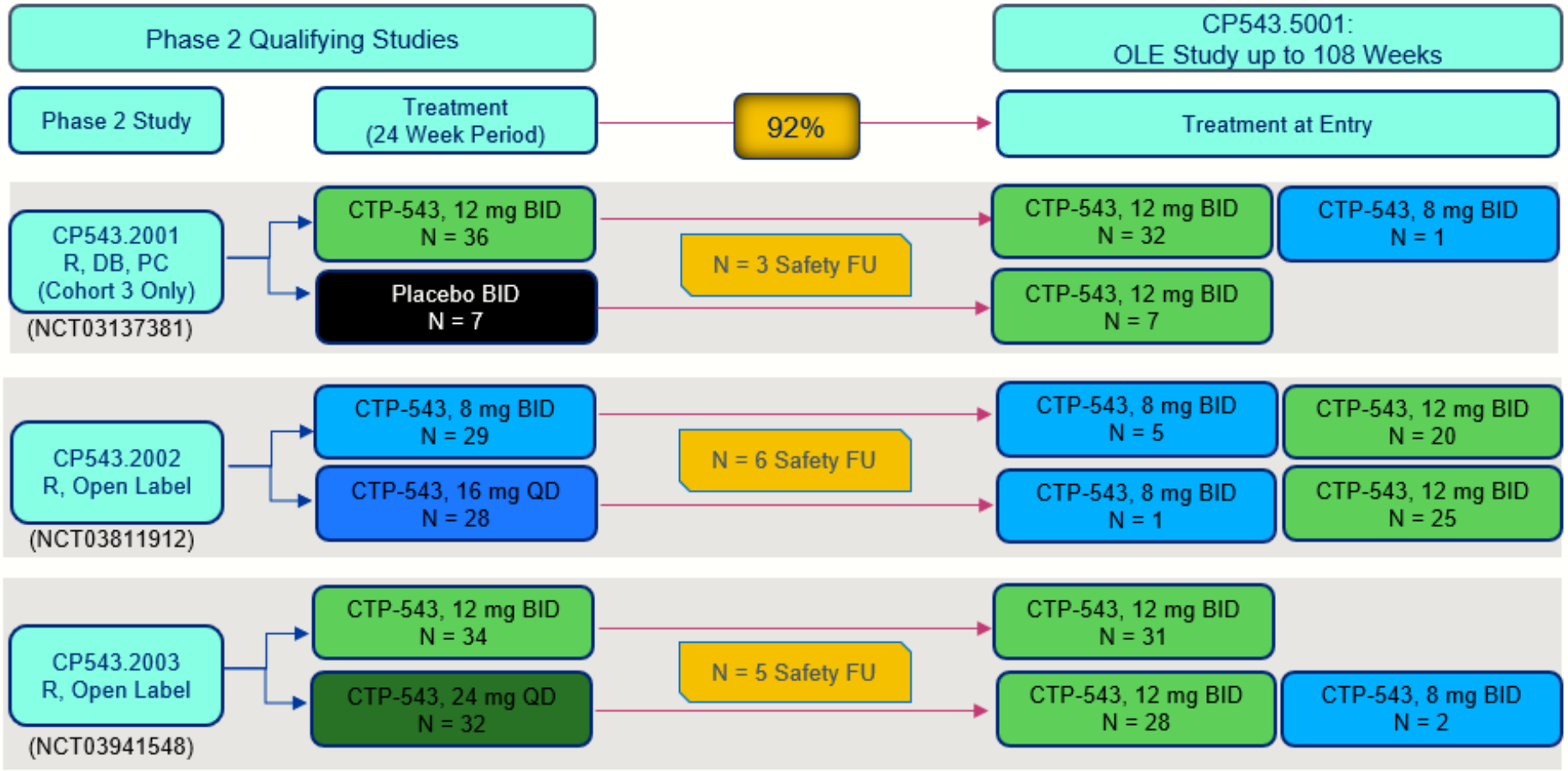
SALT Scoring



Phase 2 Qualifying Studies: Patient Demographics and AA Baseline

Study	CP543.2001 (Aug 2017 – Jul 2019)		CP543.2002 (Mar 2019 – Nov 2019)		CP543.2003 (Jun 2019 – Mar 2020)	
Phase 2 Demographics	**Placebo**	**CTP-543 12 mg BID**	**CTP-543 8 mg BID**	**CTP-543 16 mg QD**	**CTP-543 12 mg BID**	**CTP-543 24 mg QD**
Efficacy Population, N	43	36	29	28	34	32
Age: Mean (SD)	38.0 (14)	36.0 (12)	40.4 (13)	39.8 (14)	38.8 (12)	40.4 (14)
Males, N (%)	15 (34%)	9 (24%)	9 (31%)	10 (36%)	13 (38%)	9 (28%)
Females, N (%)	29 (66%)	28 (76%)	20 (69%)	18 (64%)	21 (62%)	23 (72%)
White, N (%)	33 (75%)	30 (81%)	27 (93%)	23 (82%)	25 (74%)	23 (72%)
Black or African American, N (%)	7 (16%)	3 (8%)	0	3 (11%)	4 (12%)	4 (13%)
Phase 2 AA Baseline						
AA Episode Duration: Mean	4.1 yr	3.5 yr	3.6 yr	3.8 yr	4.1 yr	3.1 yr
Baseline SALT Score, Mean (SD)	86.8 (18.4)	87.3 (18.7)	87.0 (17.2)	90.4 (17.5)	85.7 (19.5)	87.7 (17.3)

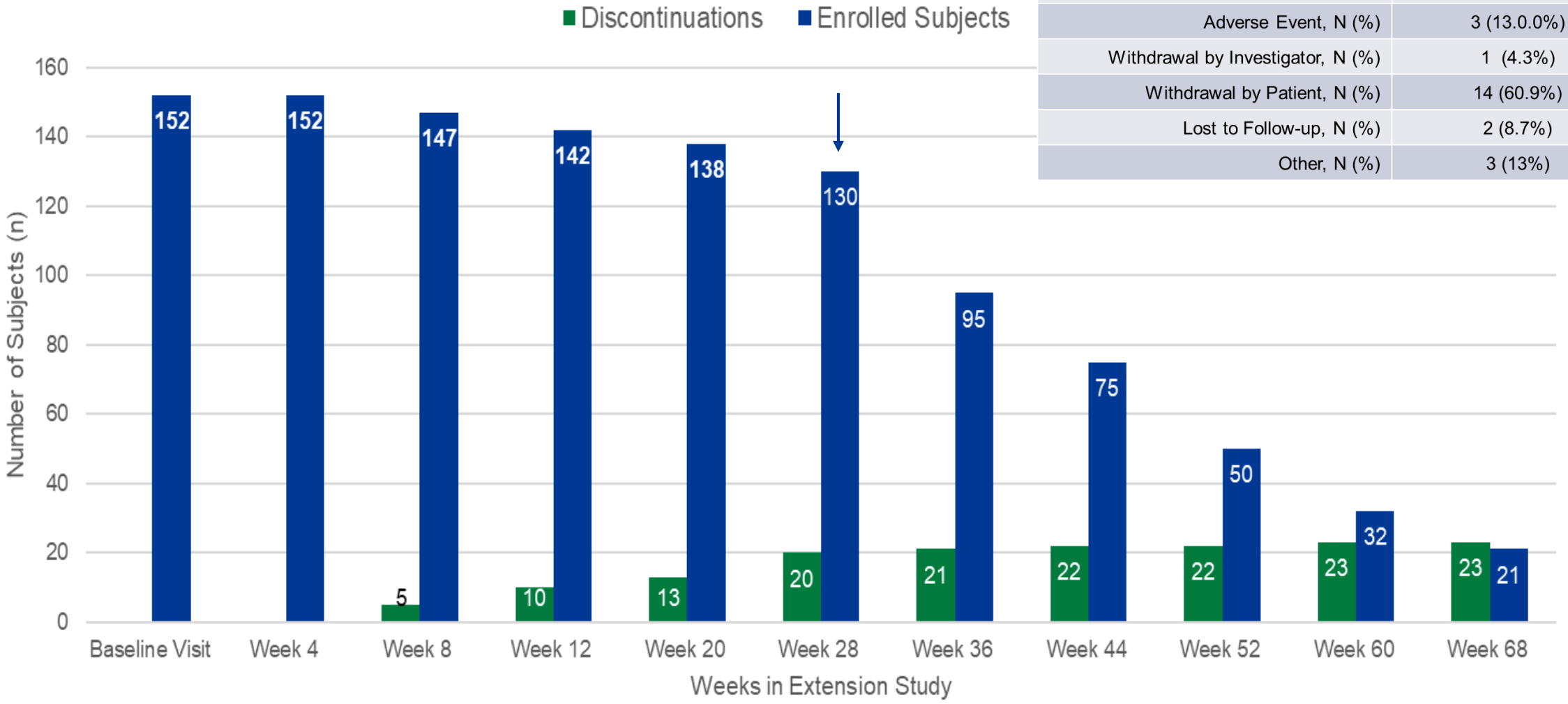
Study CP543.5001: Patient Entry and Disposition



R: Randomized; DB: Double Blind; PC: Placebo Controlled
Safety FU: Safety Follow Up then discontinue study

CP543.5001 Study Status: 130 Patients on CTP-543 > 1 year

Study Discontinuations	
Patients Enrolled, N	152
Total Patients Discontinued, N (%)	**23 (15.1%)**
Adverse Event, N (%)	3 (13.0.0%)
Withdrawal by Investigator, N (%)	1 (4.3%)
Withdrawal by Patient, N (%)	14 (60.9%)
Lost to Follow-up, N (%)	2 (8.7%)
Other, N (%)	3 (13%)



Most Common Adverse Events: Consistent with Phase 2 Studies

 # Patients with AEs

Study CP543.5001*

Preferred Term	CTP-543 N (%)
Nasopharyngitis	30 (19.7%)
Acne	27 (17.8%)
Headache	12 (7.9%)
Blood CPK (increase)	10 (6.6%)
Weight increased	8 (5.3%)
SAEs	2 Possibly Related 3 Not/Unlikely Related

*Common AEs ≥ 5%
-Total Study N = 152
-Most patients on 12 mg BID
-Severity of AEs (through 1 September 2020):

- 76% mild
- 21% moderate
- 2% severe

Qualifying Phase 2 Studies+

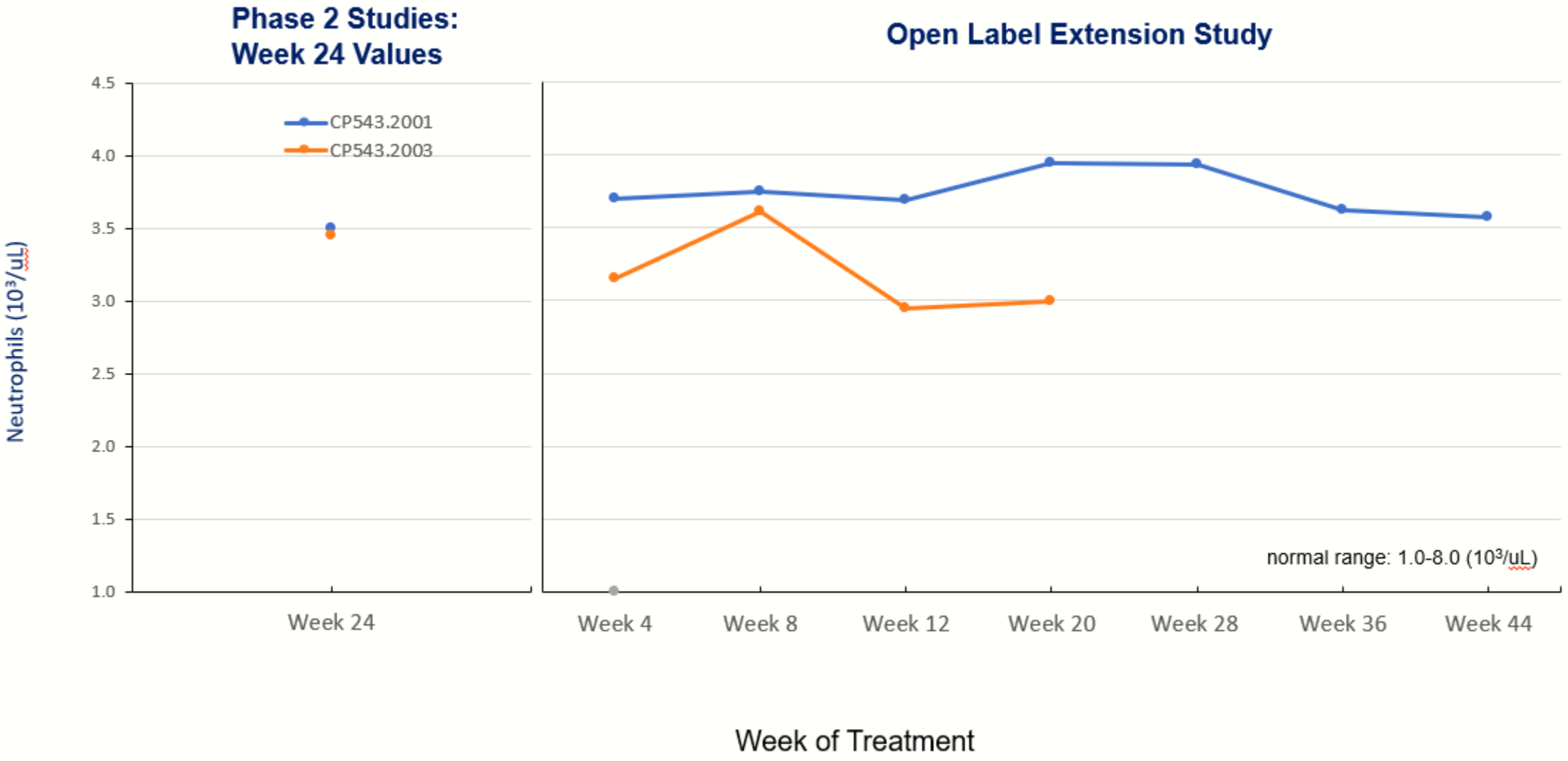
Preferred Term	CP543.2001 N (%)	CP543.2003 N (%)
Nasopharyngitis	9 (25.0%)	4 (11.8%)
Acne	6 (16.7%)	4 (11.8%)
Headache	7 (19.4%)	3 (8.8%)
Blood CPK (increase)	1 (2.8%)	11 (32.4%)
Weight increased	NA	NA
URI	7 (19.4%)	4 (11.8%)
Lipase increased	NA	4 (11.8%)
SAEs	1 Possibly Related	1 Possibly Related

+ Matching Preferred Term to CP543.5001 or AE ≥ 10% for 12 mg BID dose group from each study

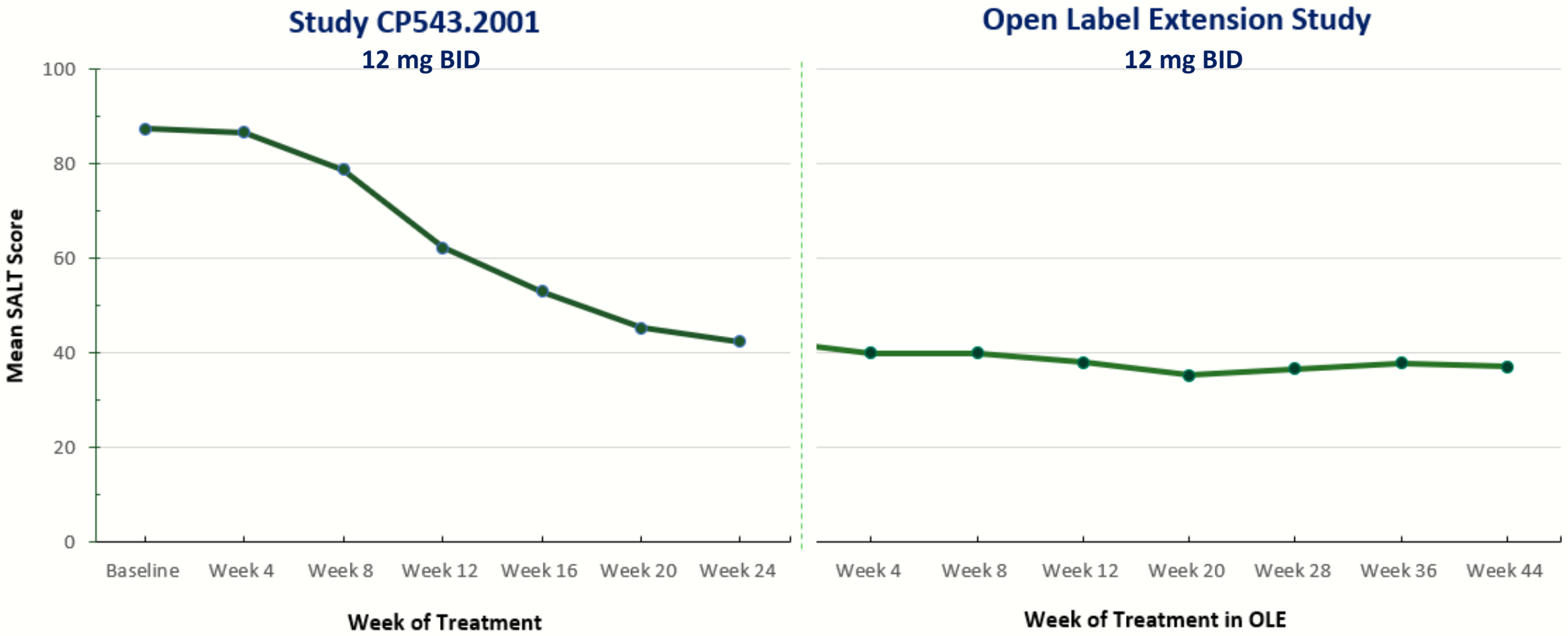
Mean Hemoglobin Values Over Time: 12 mg BID dose

Mean Platelet Values Over Time: 12 mg BID dose

Mean Neutrophil Values Over Time: 12 mg BID dose



CTP-543 Maintains Hair Regrowth Beyond Initial 24-Week Treatment



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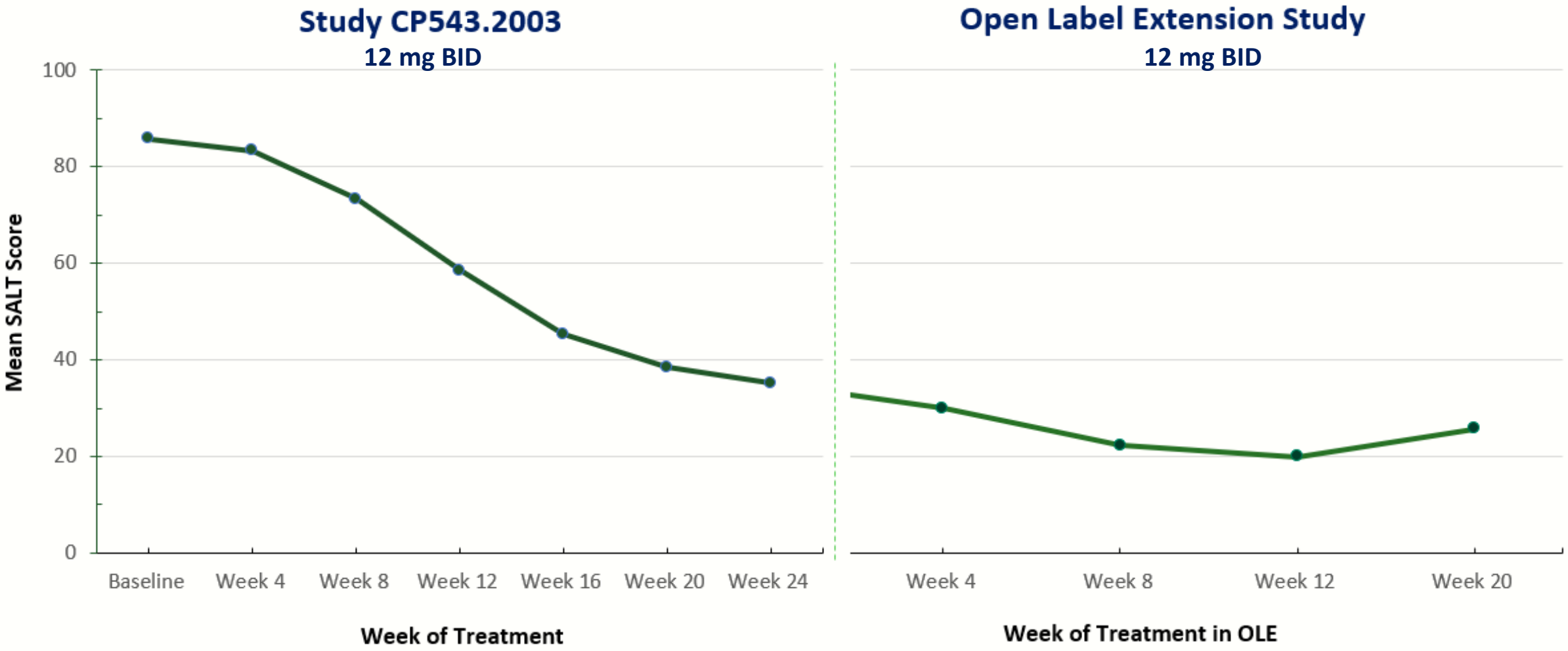
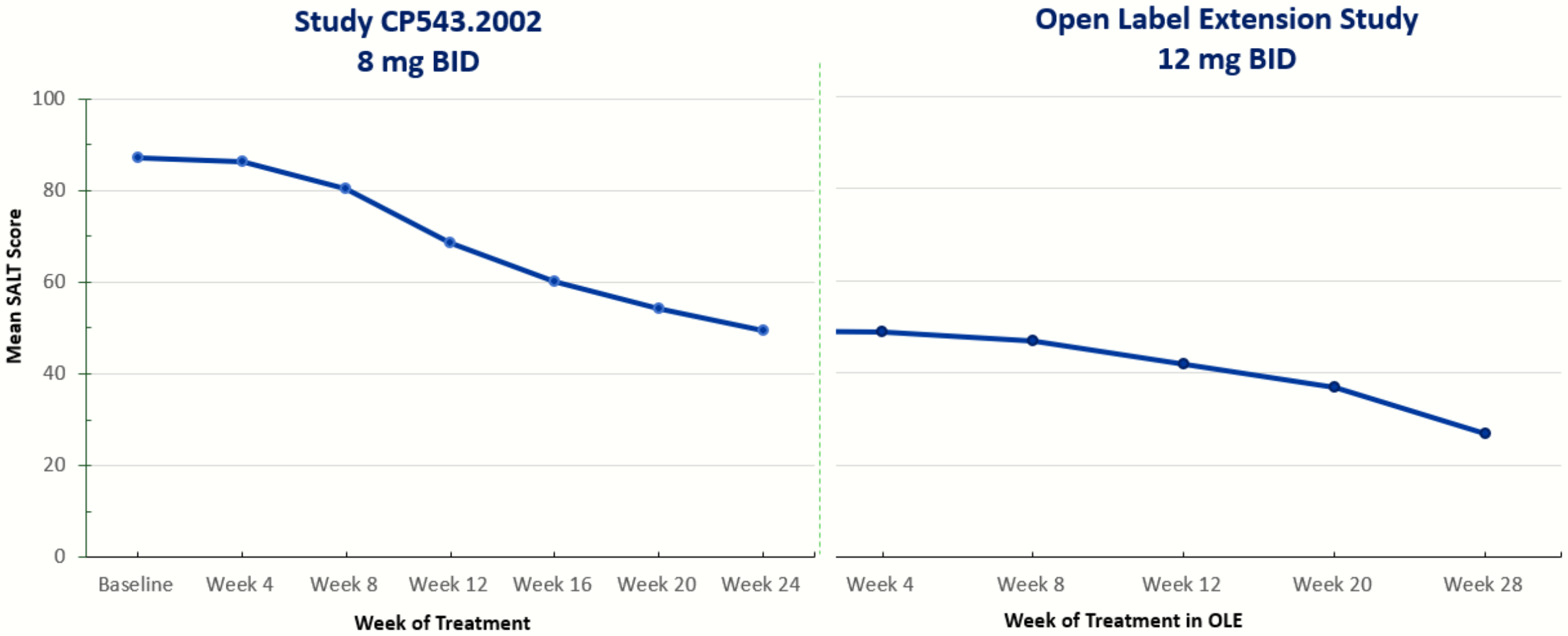


CTP-543 Continued Hair Regrowth: 8 mg BID to 12 mg BID



Summary and Conclusion

- Hair regrowth assessed by SALT was sustained or improved in the vast majority of patients relative to Phase 2 results
  - Majority of data based on 12 mg BID dosing
  - Dose escalation from 8 BID to 12 BID improves response
  - Only 2 patients (approximately 1%) had clear loss of response in OLE
- Treatment with CTP-543 in the OLE study continues to be generally well tolerated
  - Over 130 patients have been dosed cumulatively for > 1 year
  - Adverse Events are consistent with those observed in the previous 24 week studies
  - Clinical labs for hematology parameters (platelets, neutrophils, hemoglobin) appear stable across the OLE relative to end of treatment in the Phase 2 studies
- Patients participating in Phase 3 trials are eligible to enroll in long term extension study

A Special THANK YOU

- The Alopecia Areata Patients who volunteer to participate in clinical studies
- The CTP-543 clinical study teams and Investigators:

Wilma Bergfeld	Natasha Mesinkovska	Maryanne Senna
Suzanne Bruce	Paradi Mirmirani	Wei Jing Loo
Maria Colavincenzo	Janet Roberts	Sameh Hanna
Emma Guttman	Julian MacKay Wiggan	Ted Lain
Timothy Jochen	Seth Forman	Maryam Shayesteh Alam
Thomas Munyon	Theodore Alkousakis	Sheetal Sapra
Steven Kempers	Dusan Sajic	Charles Lynde
Brett King	Scott Guenthner	Catherine Maari
Justin Ko	Amy McMichael	Arash Mostaghimi

- All Health Care Professionals for your service, especially during the COVID-19 pandemic

Thank You