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JAK Inhibitor CTP-543 Achieves Primary Endpoint in Phase 2 Trial in Alopecia Areata

An interim analysis of 4 and 8 mg BID

James Cassella, PhD; Colleen Hamilton; Jana von Hehn, PhD; Virginia Braman
Concert Pharmaceuticals, Lexington, MA 02421, USA

ClinicalTrials.gov Identifier: NCT03137381

Disclosures of Relationship with Industry

James V. Cassella, PhD

S034 - Late-breaking Research: Clinical Trials

DISCLOSURES

- Concert Pharmaceuticals: Employee; Salary and Stock Received

Alopecia Areata: A Serious Medical Disease

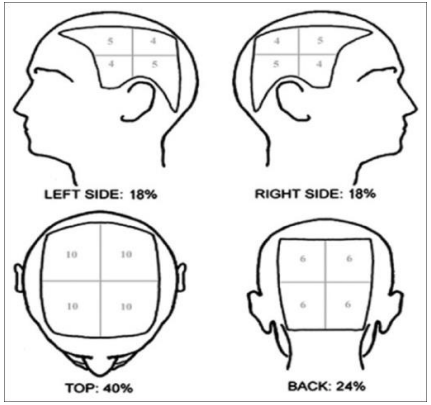
- A devastating and poorly treated autoimmune disease
- Up to 650,000 patients affected with alopecia areata (AA) in the U.S. at any given time*
- Chronic condition affecting women, men and children of all ages
- Disease profoundly impacts patients; associated with anxiety, depression and other autoimmune conditions
- No FDA-approved treatment options

*Fricke M. Clinical, Cosmetic and Investigational Dermatology, 2015.

CTP-543: Phase 2 Dose Ranging Trial Design

- Double-blind, randomized, placebo-controlled trial in adult patients with moderate-to-severe alopecia areata
- Entry criteria of at least 50% hair loss as measured by Severity of Alopecia Tool (SALT)
- Patients sequentially randomized to receive one of three doses of CTP-543 (4, 8 and 12 mg BID) or placebo for 24 weeks
- 4 mg BID and 8 mg BID Cohorts completed; 12 mg BID Cohort is currently ongoing
  - Interim analysis: 4 mg BID and 8 mg BID Cohorts
- Primary Endpoint: 50% relative reduction in SALT between Week 24 and baseline

SALT Scoring



Interim Analysis

Key Demographics and Baseline Alopecia Areata Characteristics

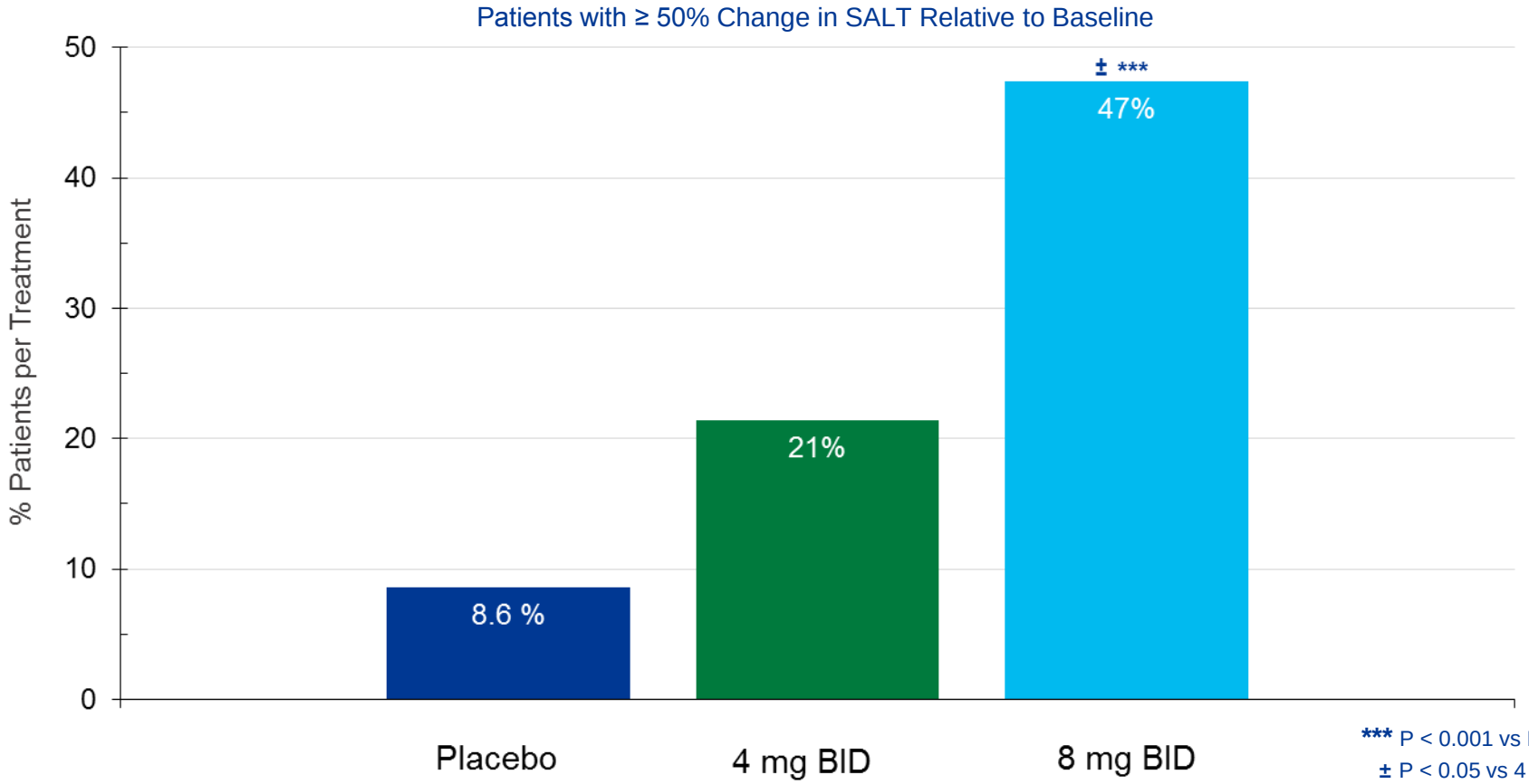
	Placebo	CTP-543 4 mg BID	CTP-543 8 mg BID
Efficacy Population (N)	35	28	38
Age: Yrs	37	36	37
Females, N (%)	24 (67%)	22 (73%)	26 (68%)
Episode Duration: Yrs	3.6	6	3.8
SALT score, Mean (SD)	85.0 (19.4)	88.8 (16.2)	89.1 (16.4)
AA Patchy, N(%)	19 (52.8%)	16 (53.3%)	16 (42.1%)
AA Totalis, N(%)	5 (13.9%)	2 (6.7%)	6 (15.8%)
AA Universalis, N(%)	12 (33.3%)	12 (40.0%)	14 (36.8%)
AA Ophiasis, N(%)	0 (0%)	0 (0%)	2 (5.3%)

Interim Analysis

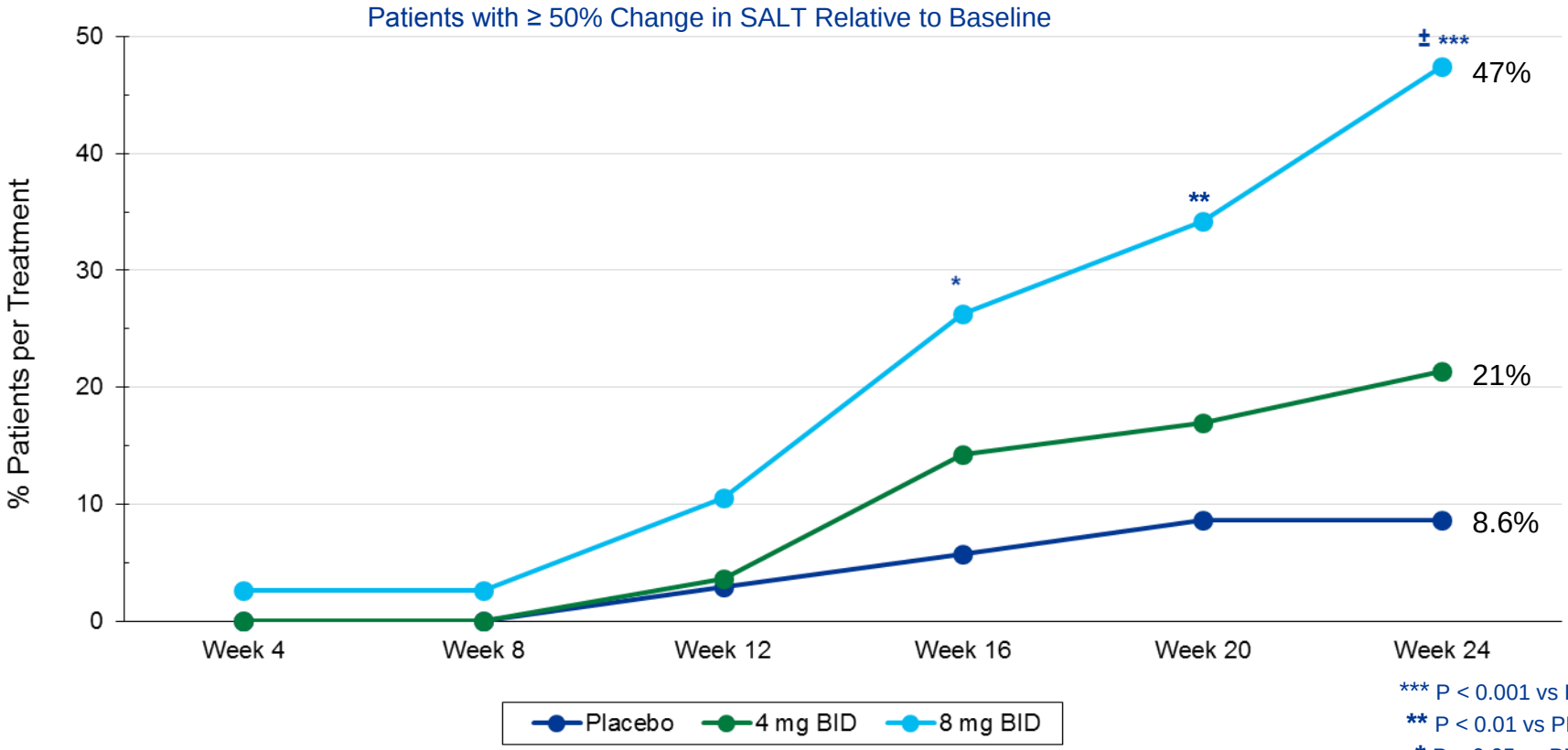
Most Common Treatment Emergent Adverse Events by Patient

Preferred Term	Placebo	CTP-543 4 mg BID	CTP-543 8 mg BID
Headache	4 (11.1%)	5 (17.2%)	10 (26.3%)
Nausea	4 (11.1%)	4 (13.8%)	4 (10.5%)
Acne	2 (5.6%)	4 (13.8%)	4 (10.5%)
Cough	0	4 (13.8%)	1 (2.6%)
Diarrhoea	3 (8.3%)	3 (10.3%)	1 (2.6%)
Nasopharyngitis	1 (2.8%)	3 (10.3%)	3 (7.9%)
Folliculitis	0	3 (10.3%)	2 (5.3%)
Blood creatine phosphokinase increased	1 (2.8%)	3 (10.3%)	2 (5.3%)
Oropharyngeal pain	0	3 (10.3%)	1 (2.6%)
Upper respiratory tract infection	6 (16.7%)	2 (6.9%)	2 (5.3%)
Discontinuations Due to AE	3 (37.5%)	0 (0%)	2 (25%)

Primary Analysis: Responders at Week 24



Responders by Visit



*** P < 0.001 vs PBO
** P < 0.01 vs PBO
* P < 0.05 vs PBO
± P < 0.05 vs 4 mg

Interim Analysis

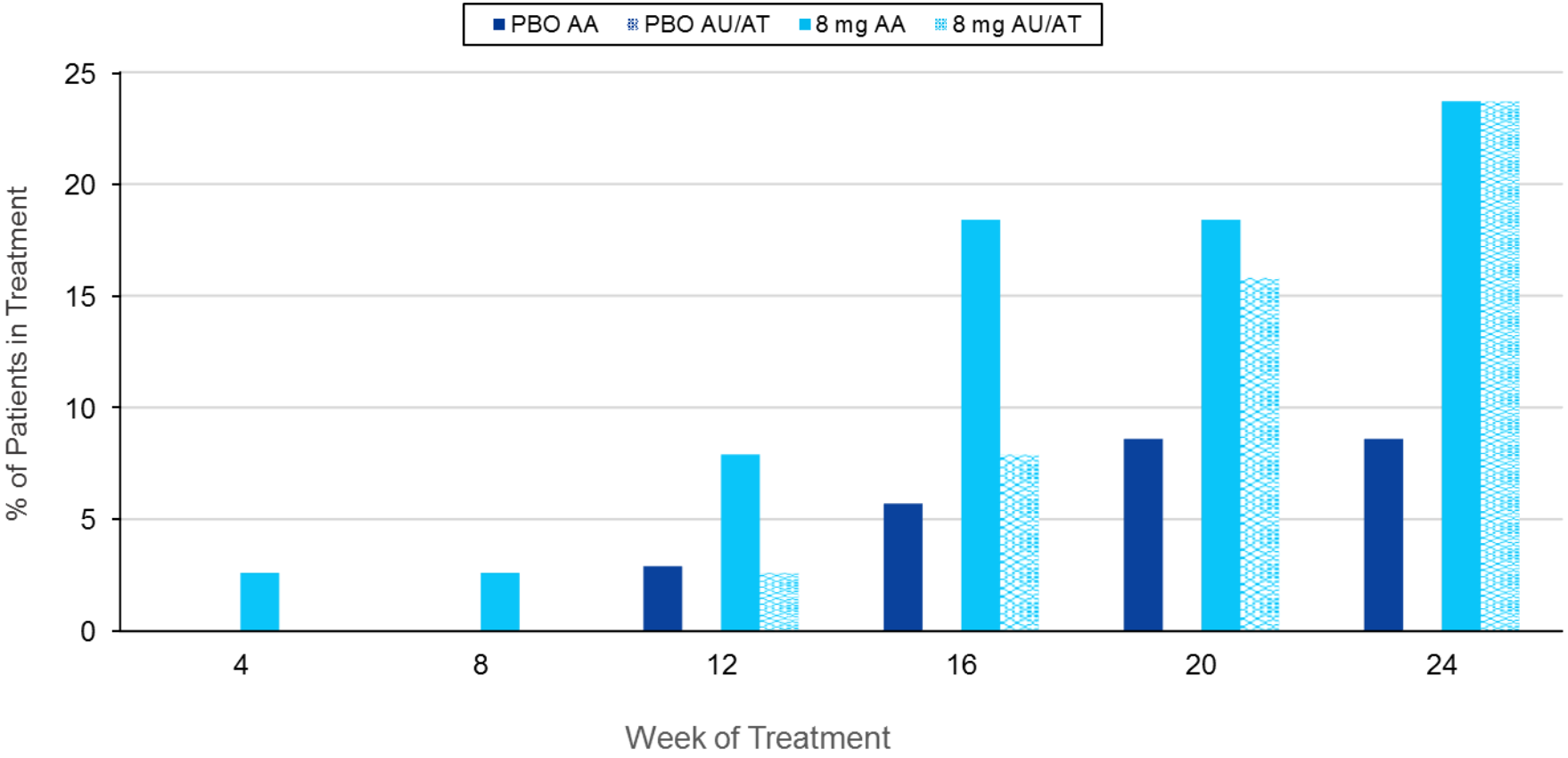
Responders by Alopecia Areata Subtype at Week 24

	Placebo	CTP-543 4 mg BID	CTP-543 8 mg BID
Responders by Subtype per Treatment, N%			
Alopecia patchy	3 (8.6%)	3 (10.7%)	8 (21.1%)
Alopecia ophiasis	0	0	1 (2.6%)
Alopecia totalis	0	0	2 (5.3%)
Alopecia universalis	0	3 (10.7%)	7 (18.4%)

Responder: ≥ 50% relative reduction in SALT between Week 24 and Baseline

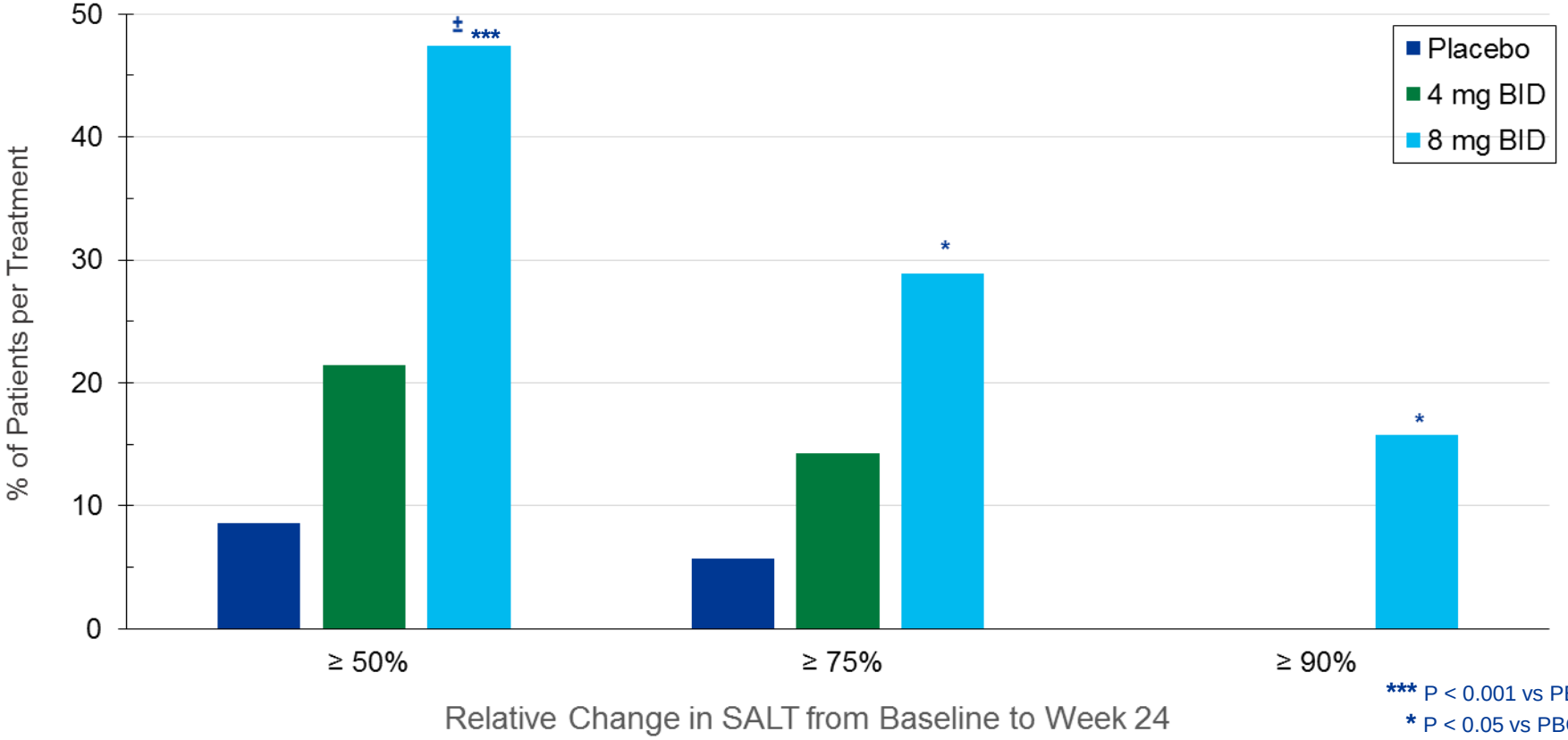
Interim Analysis

Responders by Alopecia Areata Subtype by Visit



Interim Analysis

CTP-543 Phase 2: Patient SALT Improvement



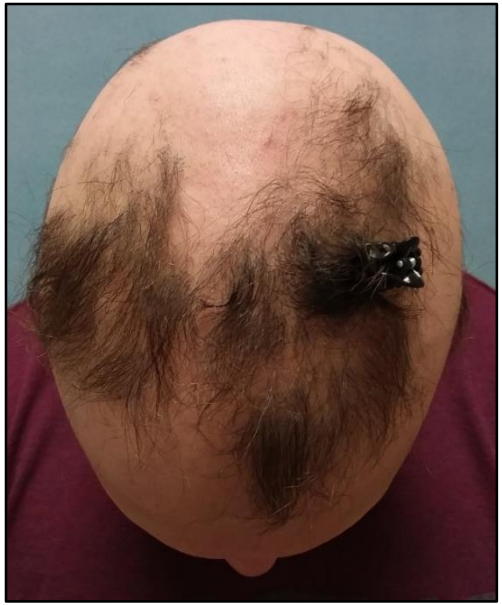
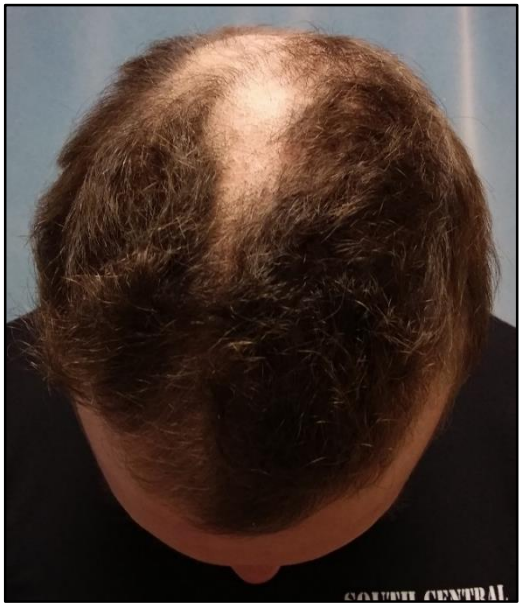
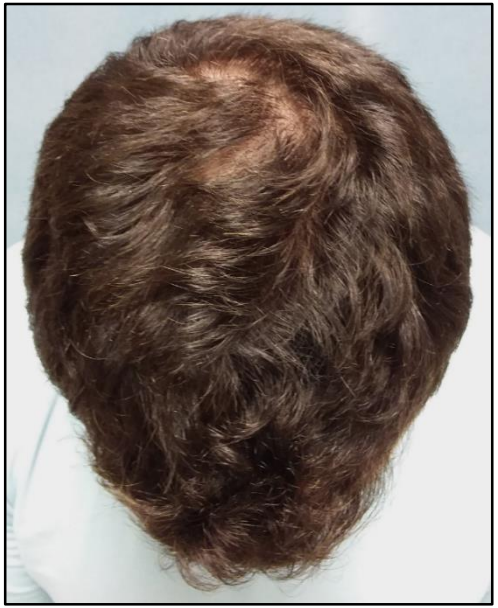
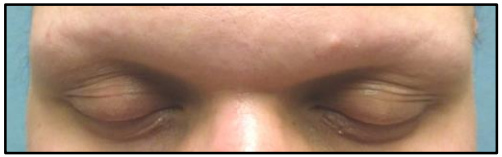
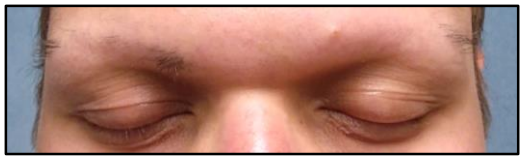
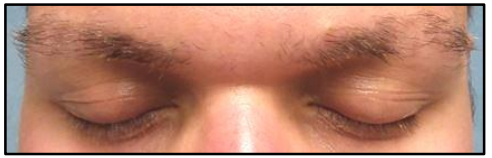
*** P < 0.001 vs PBO

* P < 0.05 vs PBO

± P < 0.05 vs 4 mg

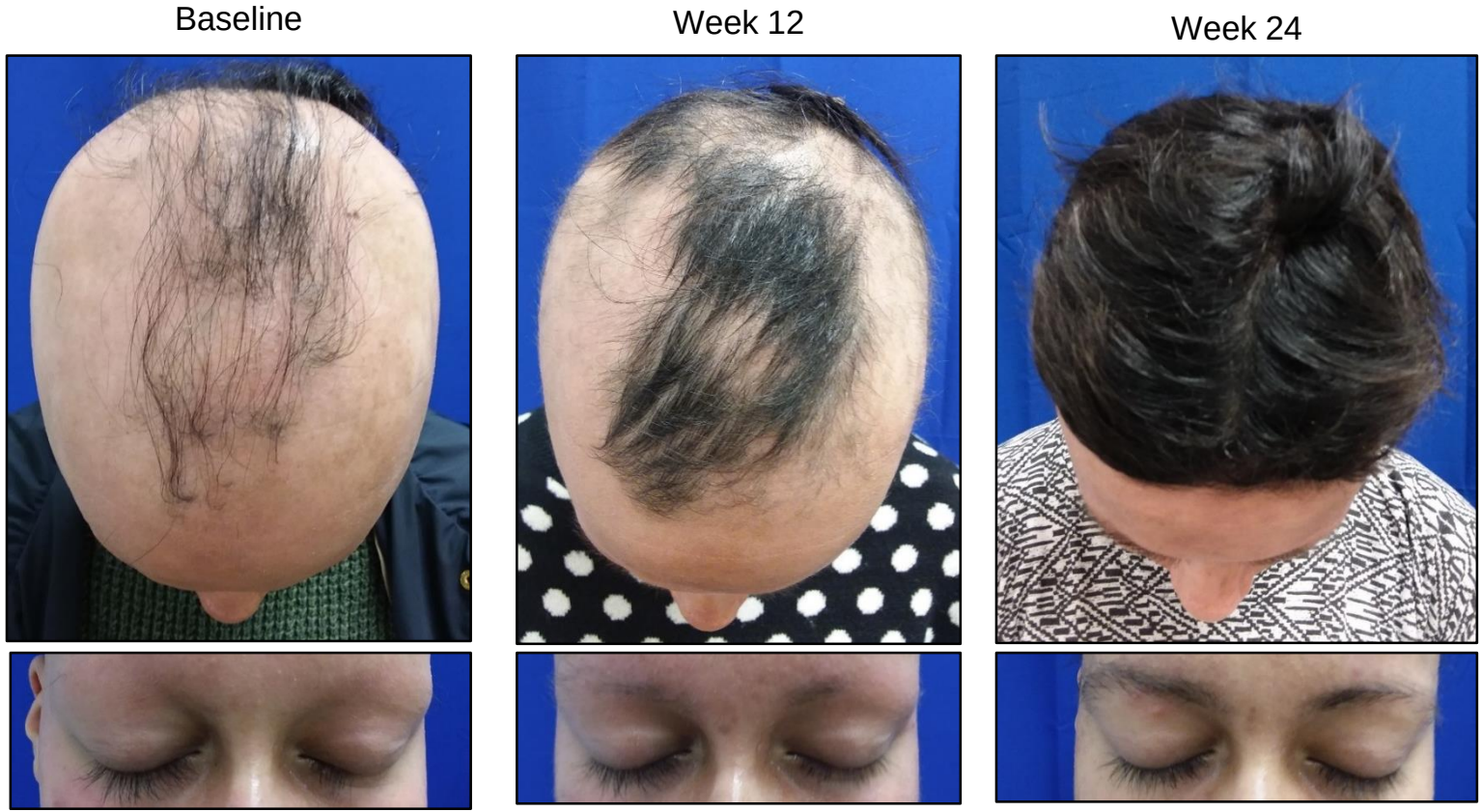
Interim Analysis

Response Over Treatment Period

Baseline	Week 12	Week 24
		
		

Interim Analysis

Response Over Treatment Period



Conclusions

Phase 2 interim analysis showed:

- 4 mg BID and 8 mg BID CTP-543 generally well-tolerated
- Primary endpoint met with 8 mg BID cohort
  - 8 mg BID dose group was significantly different from Placebo and 4 mg BID dose groups
  - 8 mg BID determined to be the minimally effective dose
  - Response did not appear to have plateaued at Week 24
    - Significant changes in SALT score were observed starting at 12 weeks
- Similar overall scalp regrowth response rate between patchy AA and AU/AT

Thank You

- To the Alopecia Areata Patients volunteering for participation in clinical studies
- To the Investigators and clinical study teams:

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