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CTP-543 Phase 2 Results in Patients with Moderate-to-Severe Alopecia Areata

September 3, 2019

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth, are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make as a result of various important factors, including the factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission and in other filings that we make with the Securities and Exchange Commission. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we assume no obligation to update any forward-looking statements except as required by applicable law.

Alopecia Areata: A Serious Medical Disease

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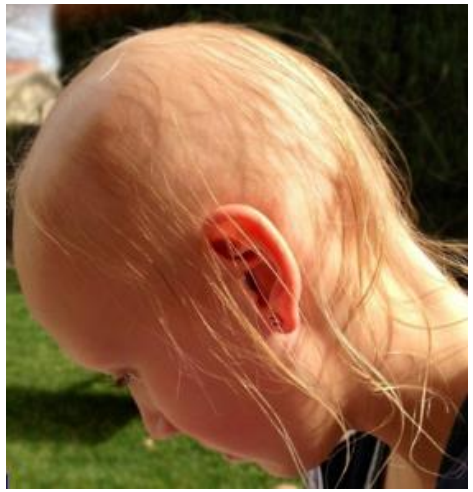
- A devastating and poorly treated autoimmune disease
- Up to 650,000 patients affected with alopecia areata in the U.S. at any given time*
- Chronic condition affecting women, men and children of all ages
- Disease profoundly impacts patients; associated with anxiety, depression and other autoimmune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



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

CTP-543: Phase 2 Dose-Ranging Trial

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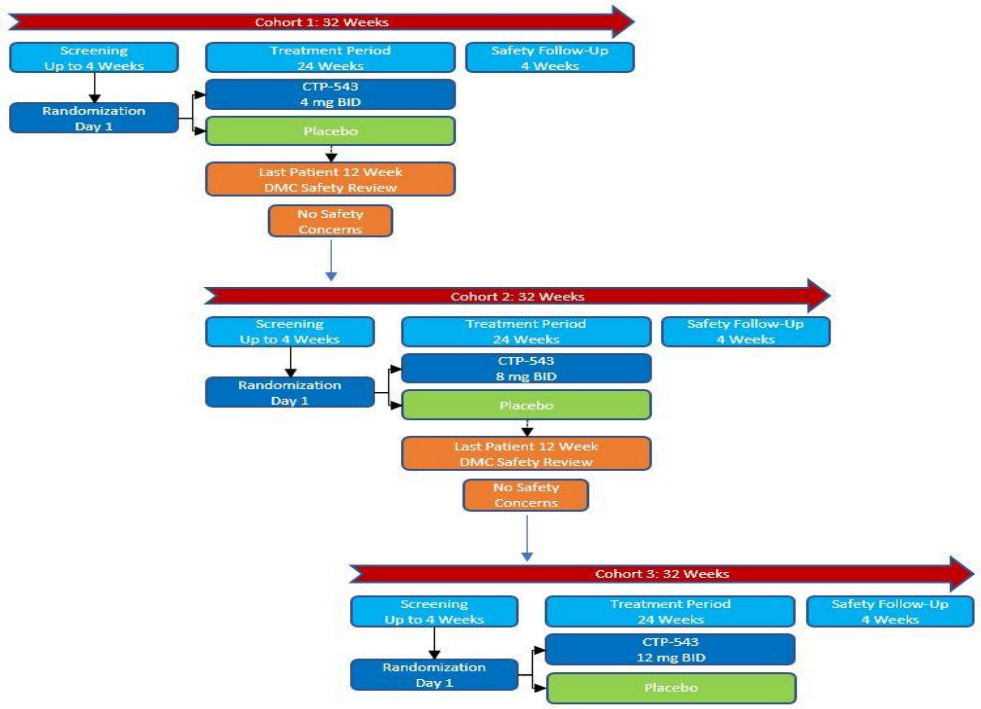
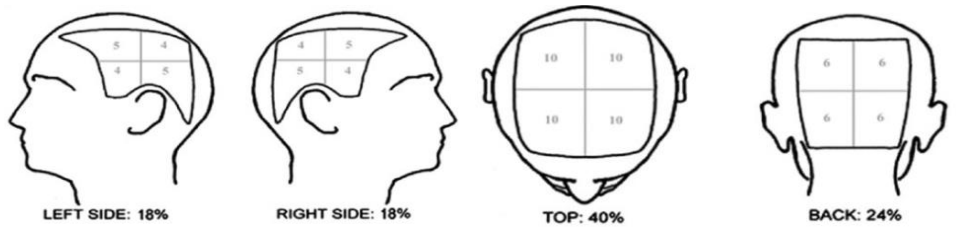
- Double-blind, randomized, placebo-controlled trial in adult patients with moderate-to-severe alopecia areata
- Entry criteria of at least 50% hair loss as measured by Severity of Alopecia Tool (SALT)
- Patients sequentially randomized to receive one of three doses of CTP-543 (4, 8 and 12 mg) or placebo twice daily for 24 weeks
- Primary endpoint: Percent of patients achieving a 50% relative reduction in SALT at Week 24 from baseline
- Additional clinical endpoints include:
  - Percent of patients achieving 75% and 90% relative change in SALT at Week 24 from baseline
  - Patient Global Impression of Improvement

Trial Design



Cohort 3: Eligible to enroll in open label extension study

SALT Scoring



Demographics

	Placebo	CTP-543 4 mg	CTP-543 8 mg	CTP-543 12 mg
Randomized Population	44	30	38	37
Efficacy Population	43	28	38	36
Age: Mean (SD)	38 (14%)	36 (11%)	37(14%)	36 (12%)
Males, n (%)	15 (34%)	8 (27%)	12 (32%)	9 (24%)
Females, n (%)	29 (66%)	22 (73%)	26 (68%)	28 (76%)
Race: n (%)				
White	33 (75%)	25 (83%)	26 (68%)	30 (81%)
Black or African American	7 (16%)	2 (7%)	7 (18%)	3 (8%)
Asian	2 (4.5%)	2 (7%)	2 (5%)	4 (11%)
Other	2 (4.5%)	1 (3%)	3 (8%)	0 (0%)

Baseline Alopecia Areata Characteristics

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	Placebo	CTP-543 4 mg	CTP-543 8 mg	CTP-543 12 mg
Episode Duration: Yr, Mean	4.1	6	3.8	3.5
SALT score, Mean (SD)	86.8 (18.4)	88.8 (16.2)	89.1 (16.4)	87.3 (18.7)
AA Patchy, n (%)	21 (47.7%)	16 (53.3%)	16 (42.1%)	16 (43.2%)
AA Totalis, n (%)	6 (13.6%)	2 (6.7%)	6 (15.8%)	8 (21.6%)
AA Universalis, n (%)	17 (38.6%)	12 (40.0%)	14 (36.8%)	10 (27.0)
AA Ophiasis, n (%)	0 (0%)	0 (0%)	2 (5.3%)	3 (8.1%)

Primary Analysis: Responders at Week 24

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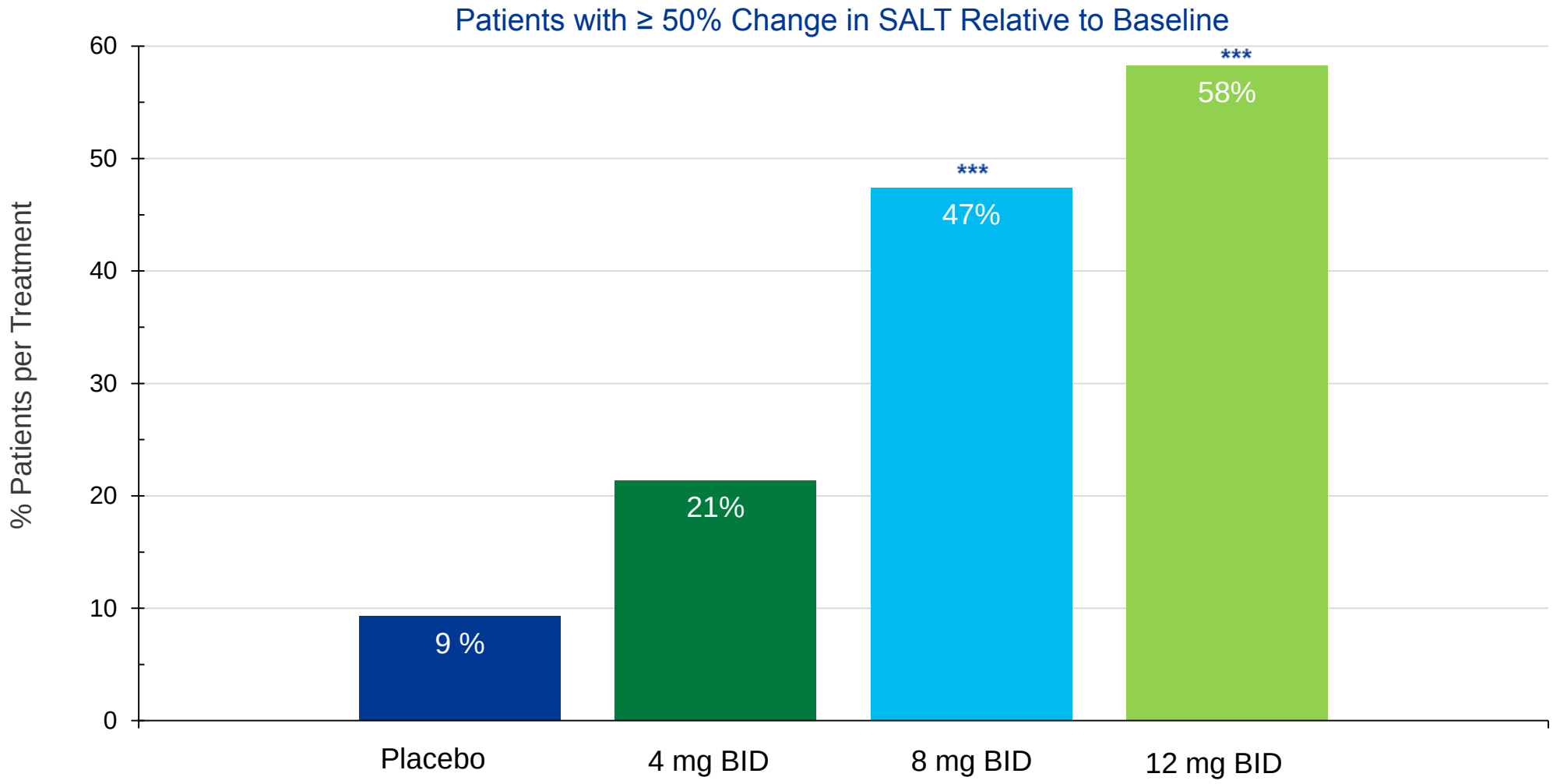

- 12 mg BID responders average 86% SALT improvement
- 8 mg BID responders average 78% SALT improvement

*** P < 0.001 vs PBO

Responders: ≥ 50% Change in SALT Relative to Baseline

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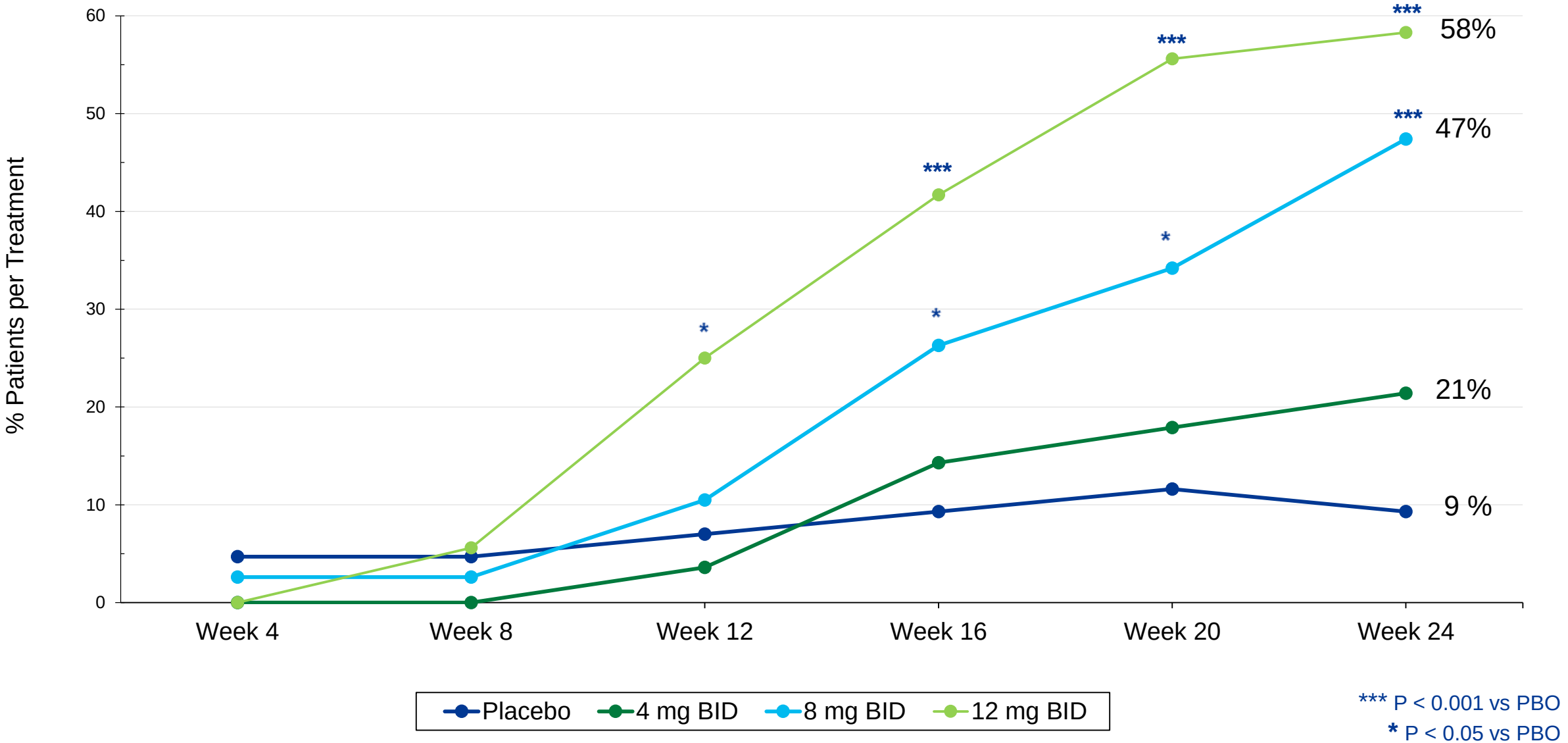

*** P < 0.001 vs PBO

* P < 0.05 vs PBO

Responders: ≥ 75% Change in SALT Relative to Baseline

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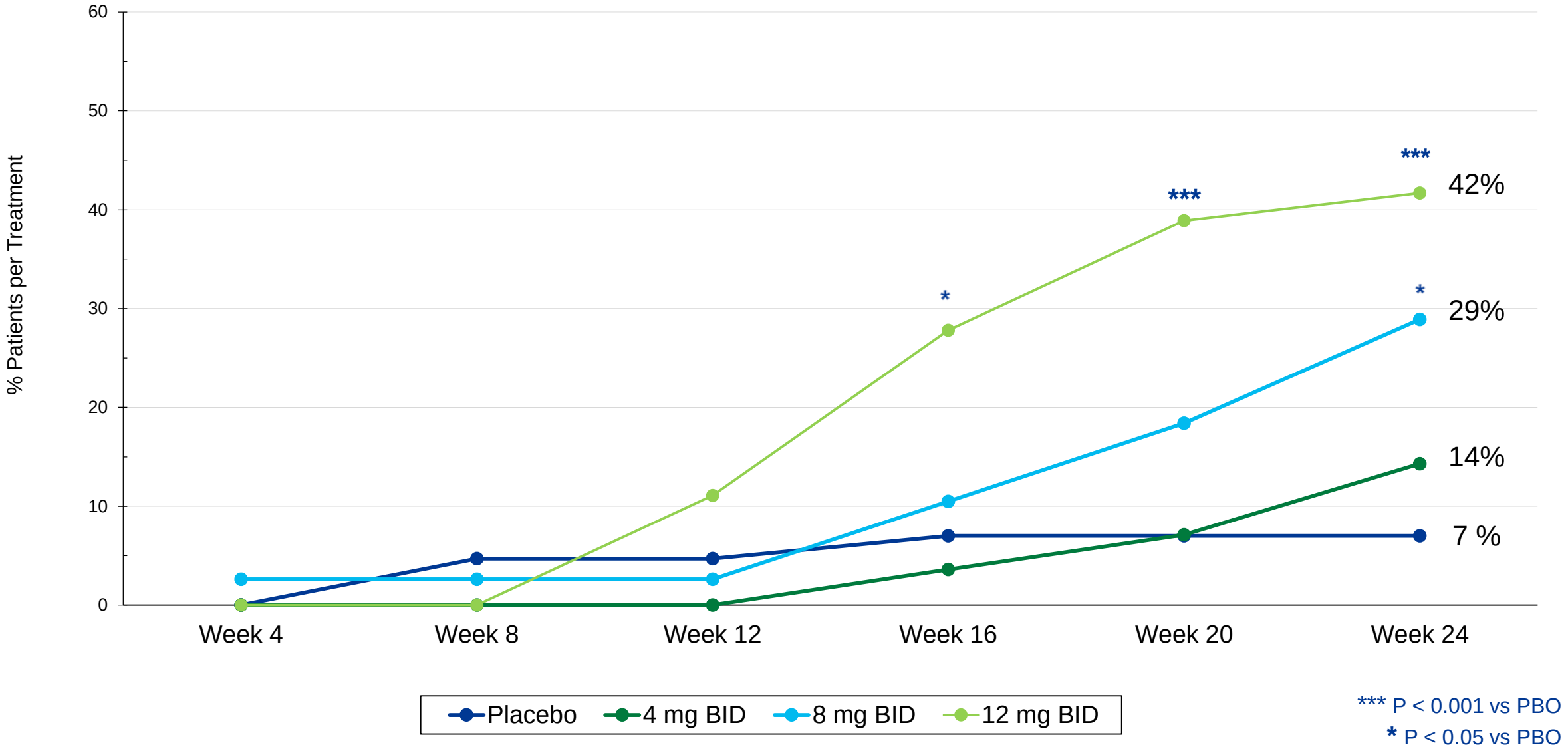

*** P < 0.001 vs PBO

* P < 0.05 vs PBO

Responders: ≥ 90% Change in SALT Relative to Baseline

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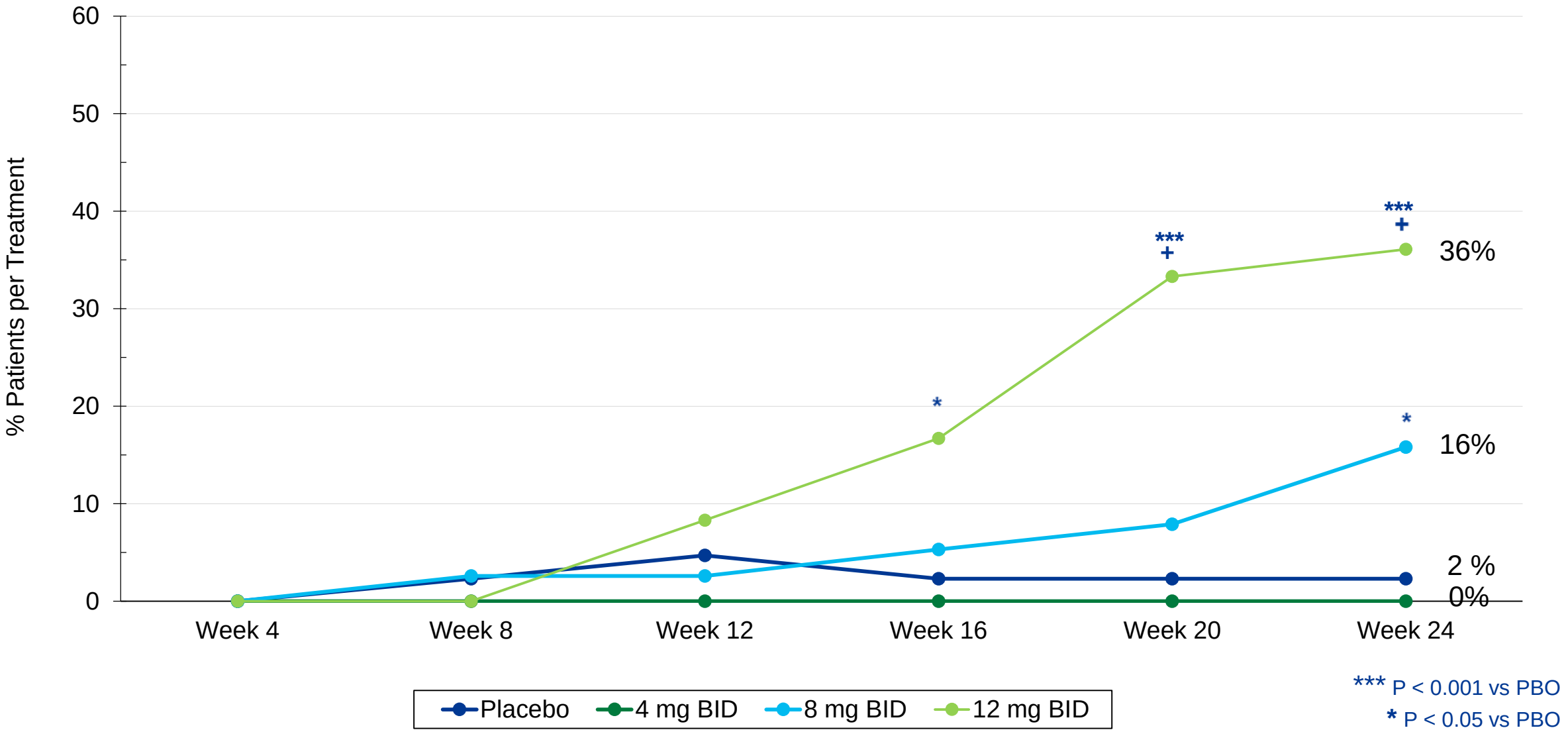
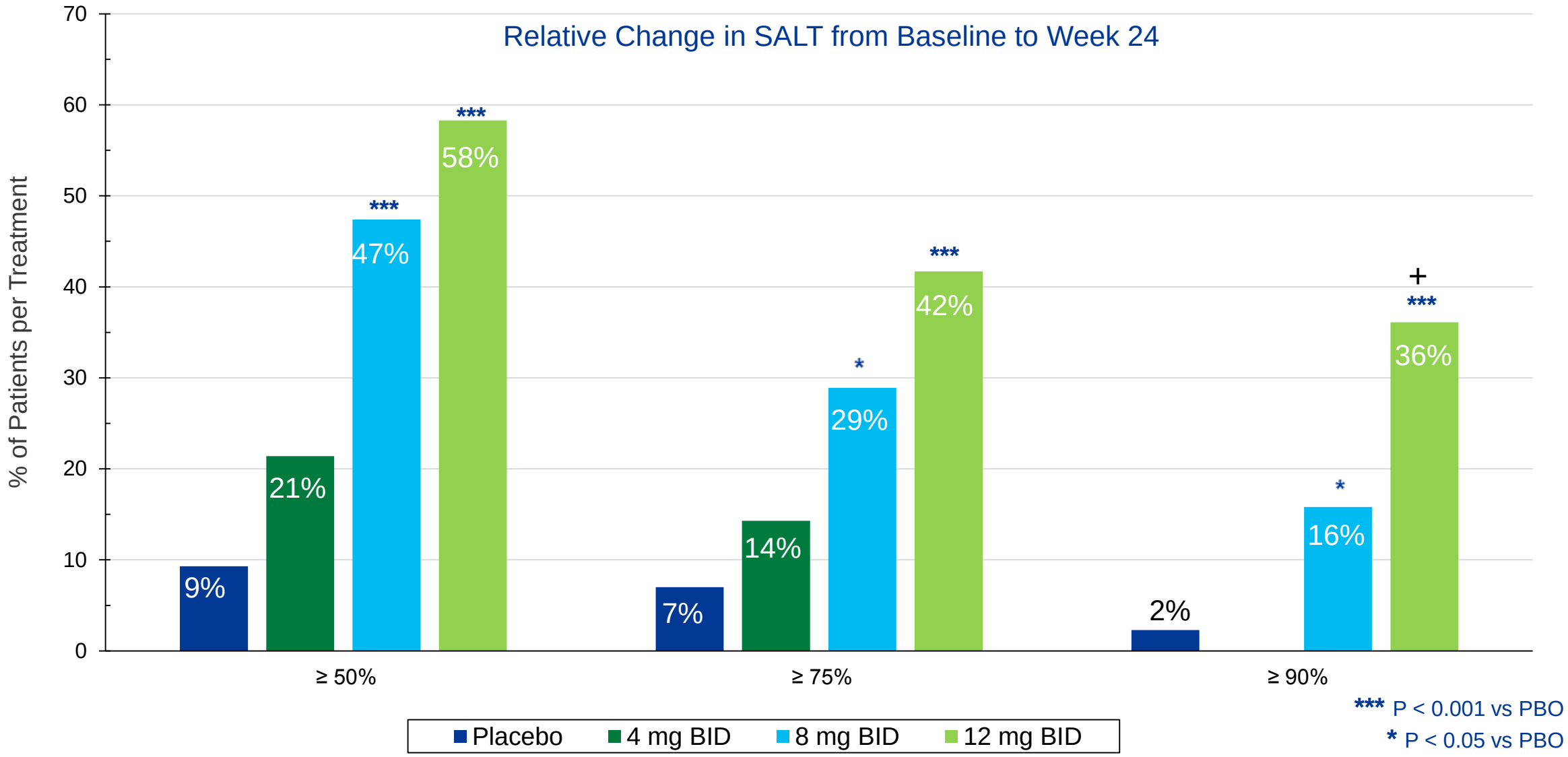


*** P < 0.001 vs PBO

* P < 0.05 vs PBO

+ P < 0.05 vs 8 mg

Patient SALT Improvement Thresholds



*** P < 0.001 vs PBO
* P < 0.05 vs PBO
+ P < 0.05 vs 8 mg

Relative Change in SALT

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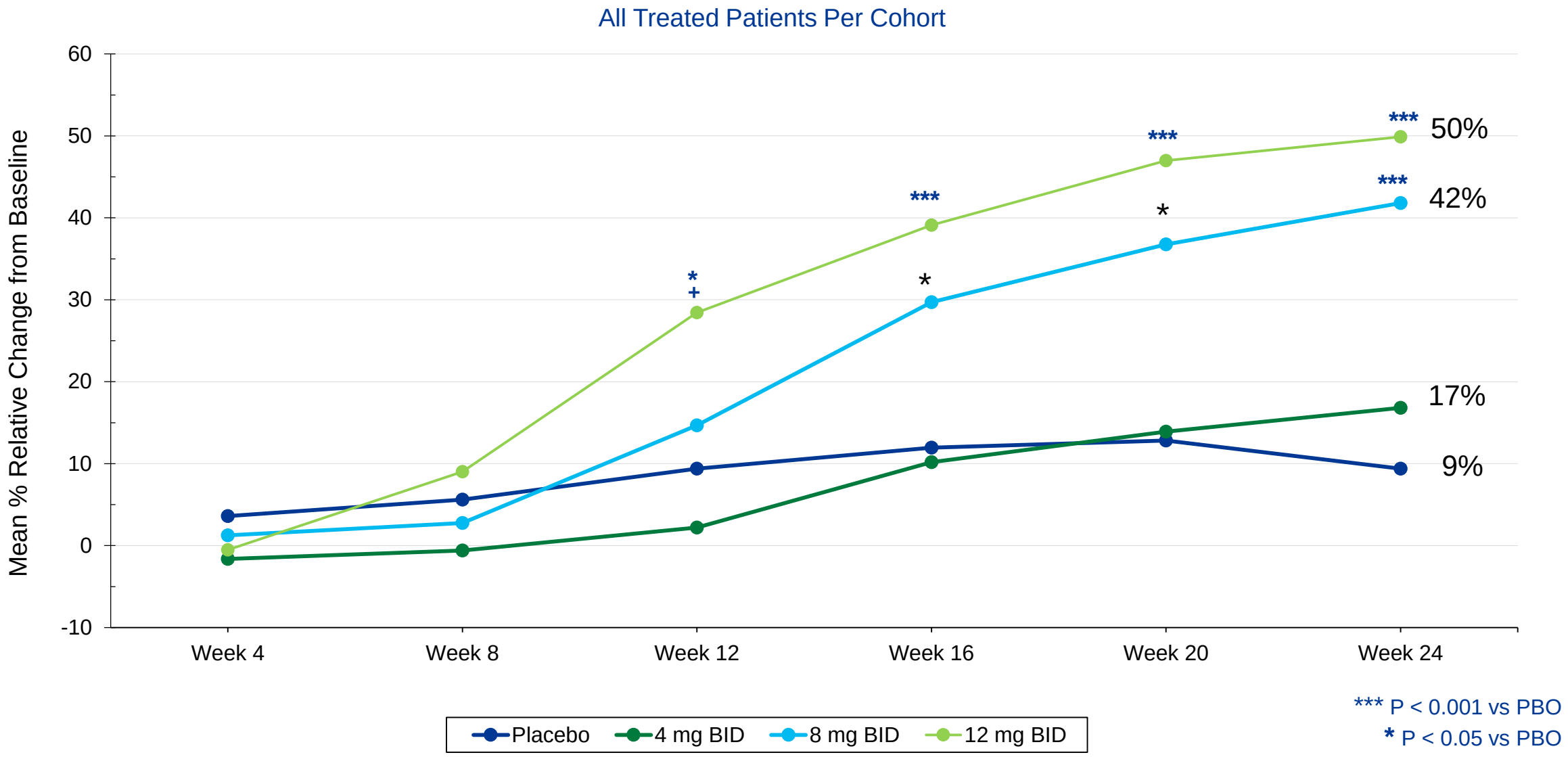

Patient Global Impression of Improvement: Responders

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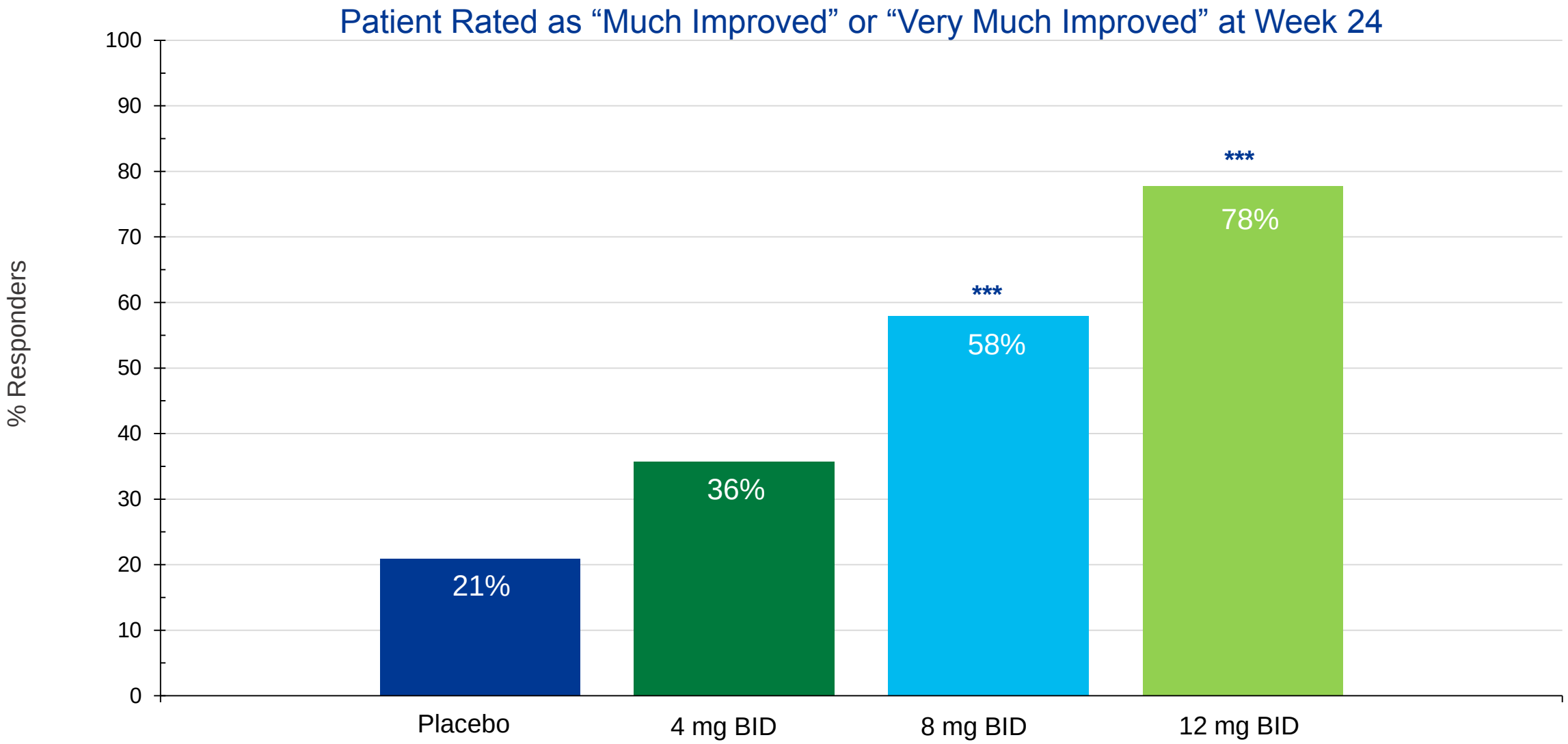

*** P < 0.001 vs PBO

Common (≥ 10%) Treatment Emergent Adverse Events (# Patients)

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Preferred Term	Placebo	CTP-543 4 mg	CTP-543 8 mg	CTP-543 12 mg
Headache	4 (9.1%)	5 (17.2%)	10 (26.3%)	7 (19.4%)
Nasopharyngitis	1 (2.3%)	3 (10.3%)	3 (7.9%)	9 (25.0%)
URI	7 (15.9%)	2 (6.9%)	2 (5.3%)	7 (19.4%)
Acne	2 (4.5%)	4 (13.8%)	4 (10.5%)	6 (16.7%)
Nausea	4 (9.1%)	4 (13.8%)	4 (10.5%)	1 (2.8%)
Cough	0	4 (13.8%)	1 (2.6%)	2 (5.6%)
LDL increase	0	0	4 (10.5%)	0
Diarrhoea	3 (6.8%)	3 (10.3%)	1 (2.6%)	0
Folliculitis	0	3 (10.3%)	2 (5.3%)	1 (2.8%)
Blood CPK (increase)	1 (2.3%)	3 (10.3%)	2 (5.3%)	1 (2.8%)
Oropharyngeal pain	1 (2.3%)	3 (10.3%)	1 (2.6%)	0

One SAE was reported for facial cellulitis in the 12 mg cohort; following a brief interruption, treatment was continued and this patient completed the trial.

CTP-543 Response Over Treatment Period: 12 mg BID

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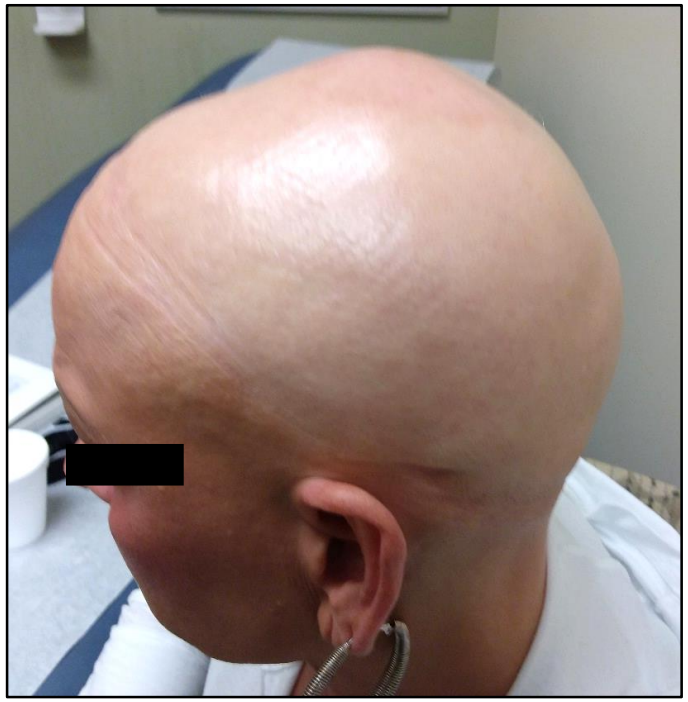
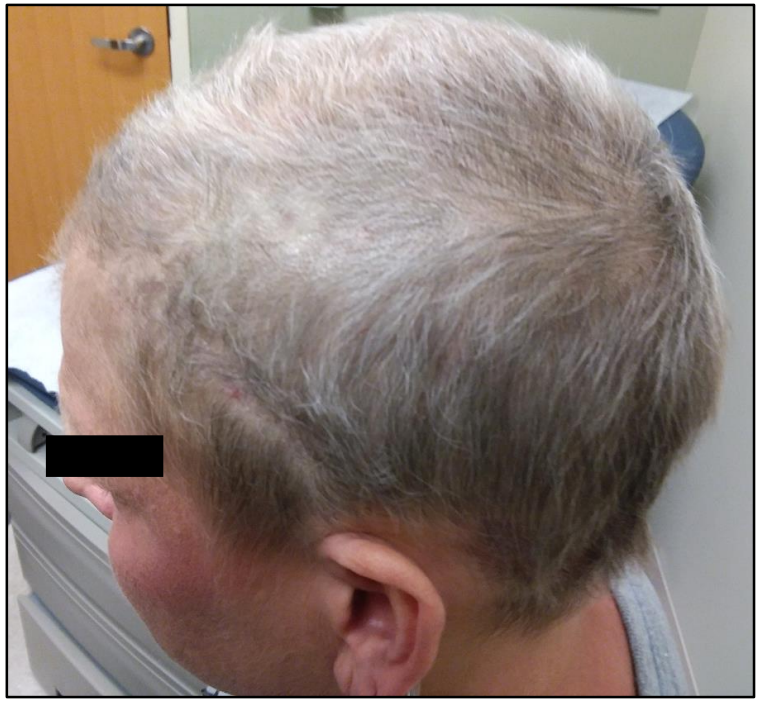
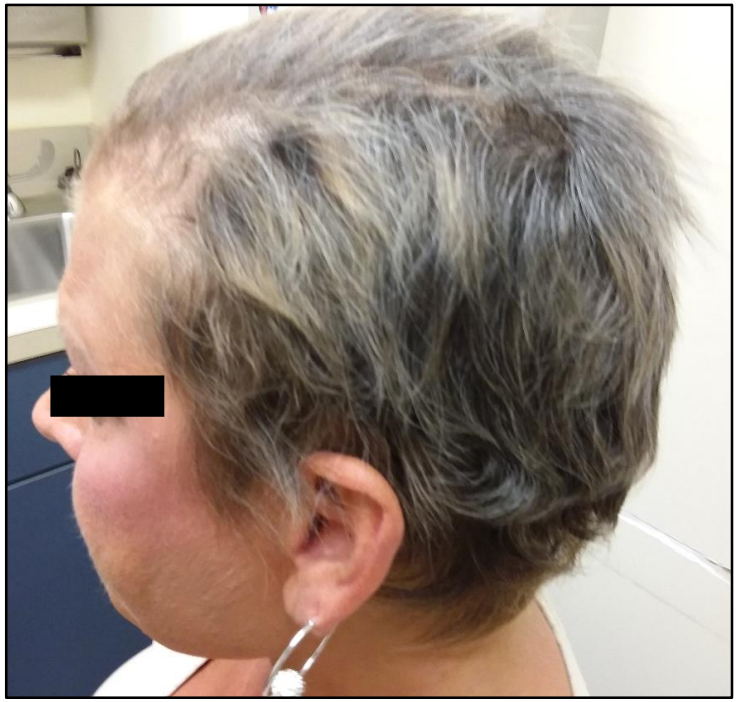
Baseline



Week 12



Week 24



CTP-543 Response Over Treatment Period: 12 mg BID

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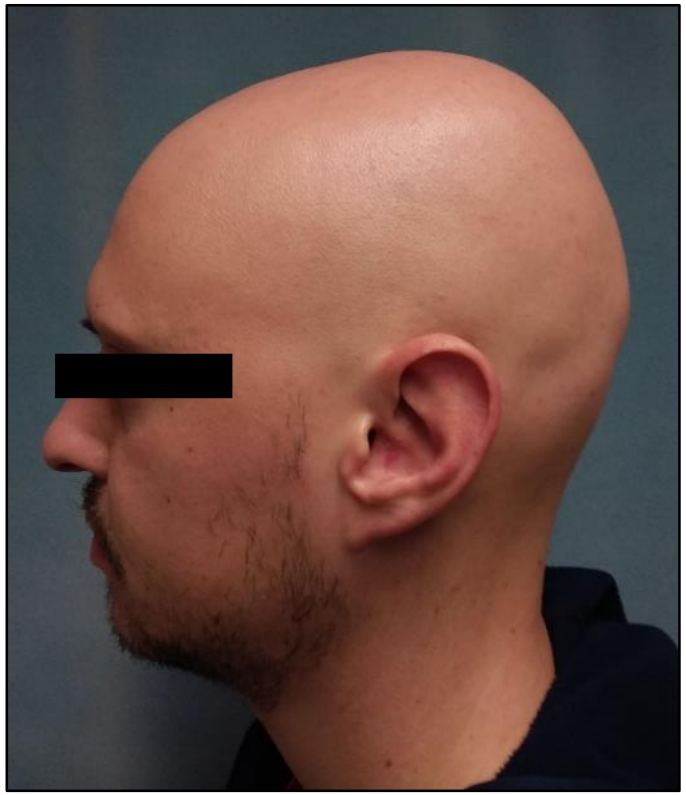
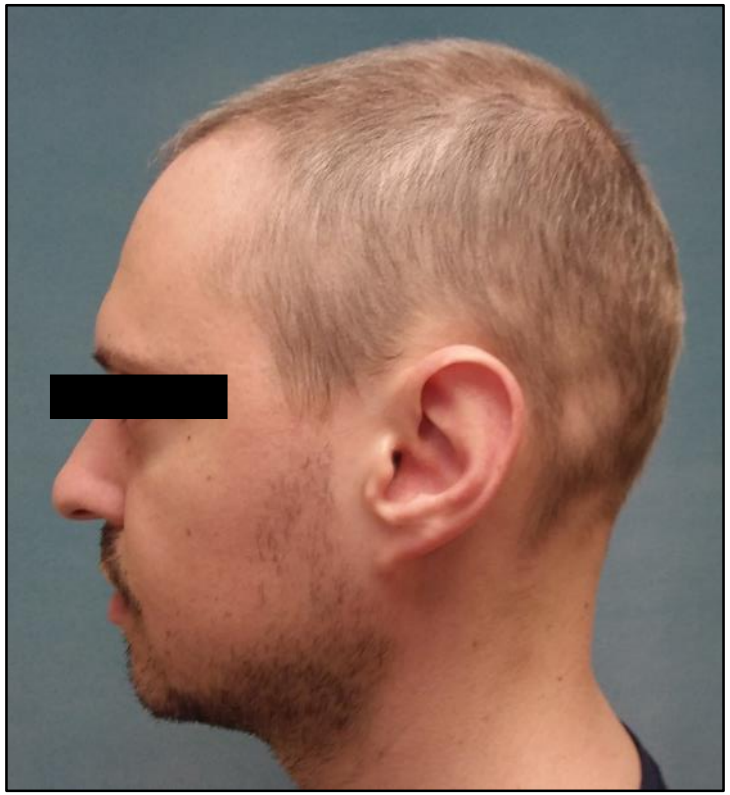
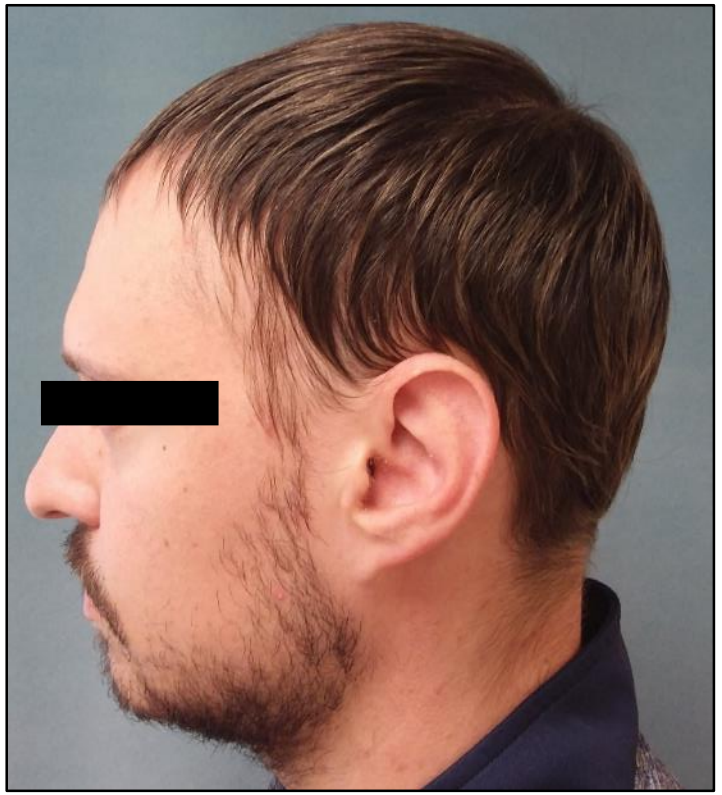
Baseline



Week 12



Week 24



CTP-543 Response Over Treatment Period: 8 mg BID

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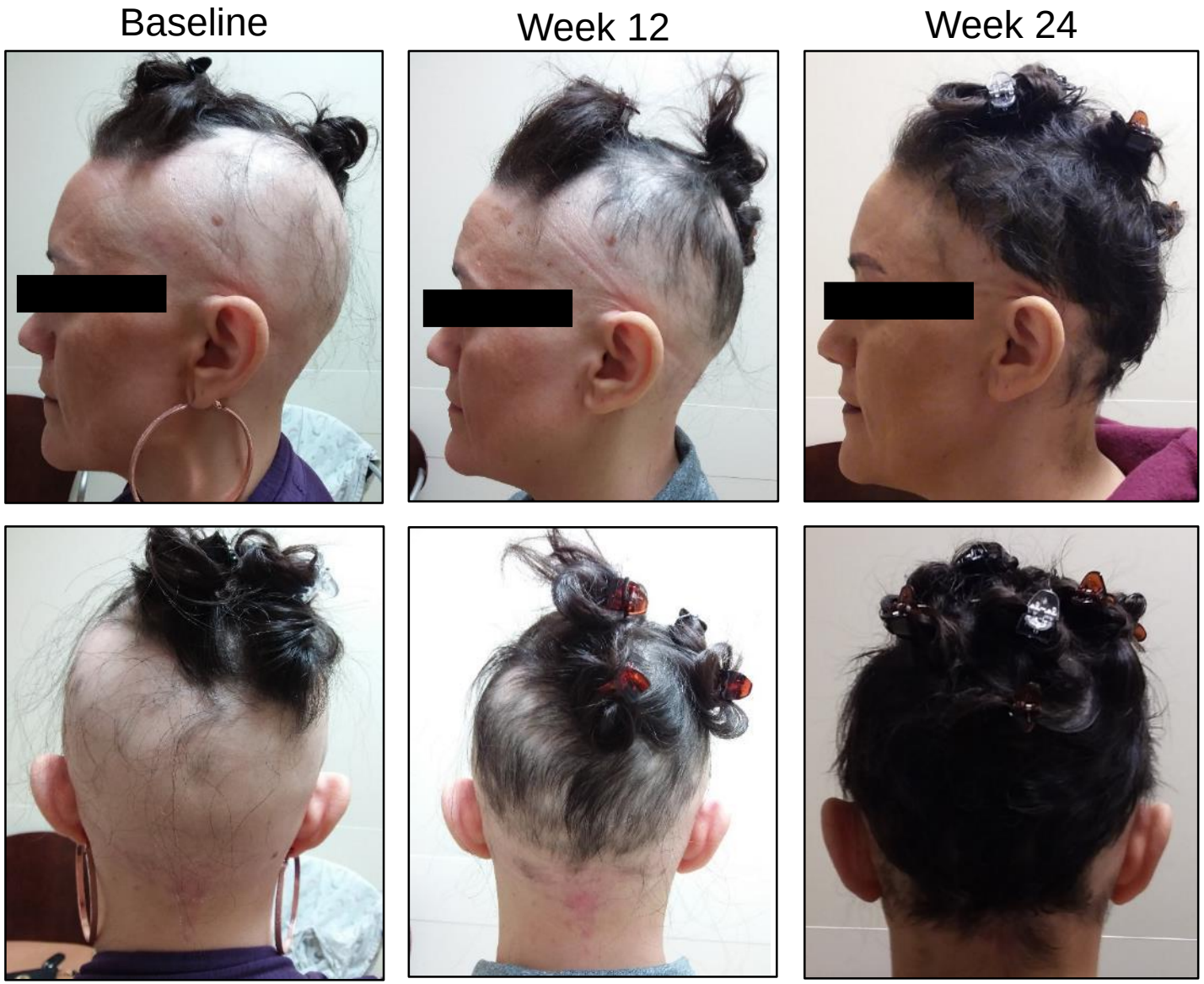
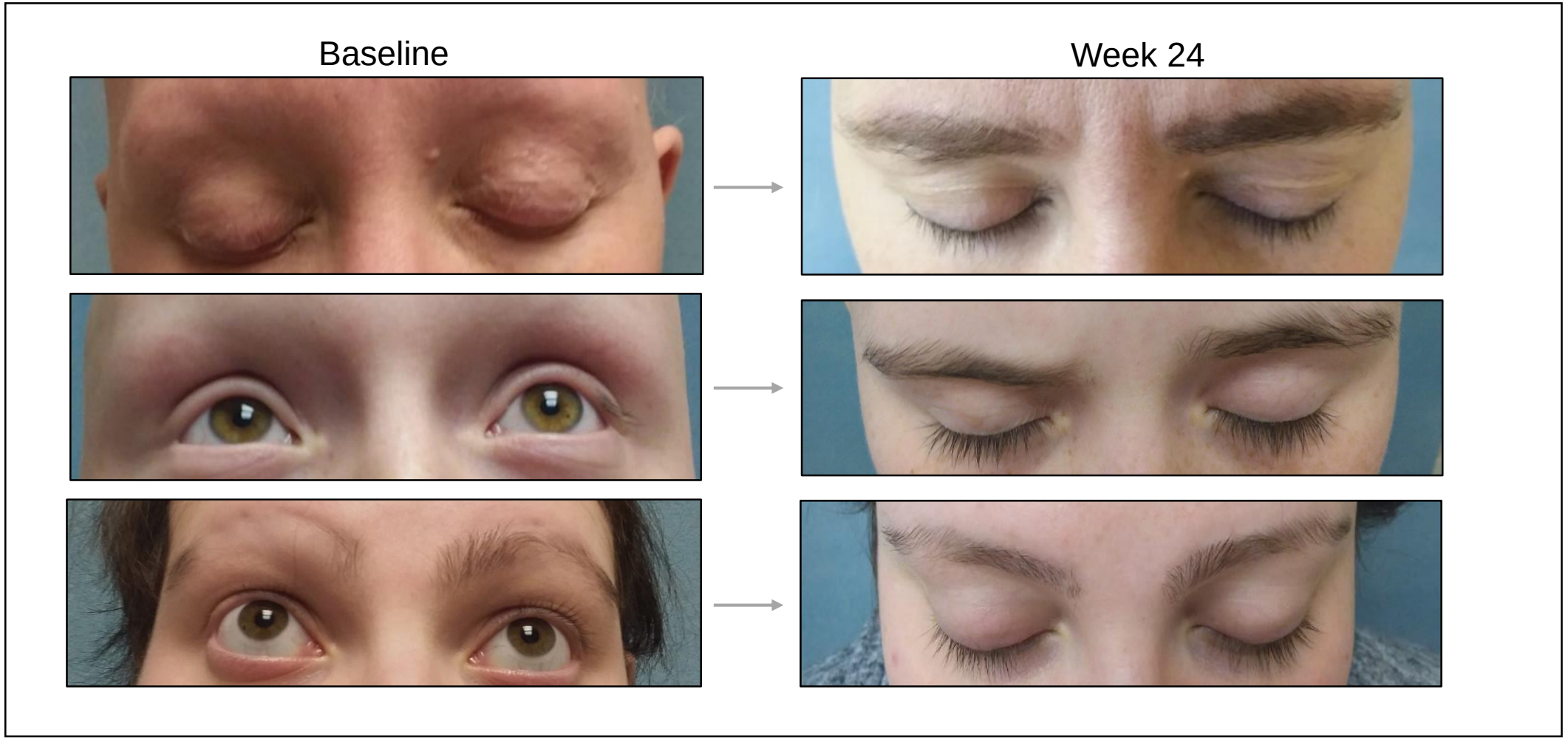


CTP-543 Eyebrow/Eyelash Response Over Treatment Period: 12 mg BID



Conclusion

- The primary efficacy endpoint was met for 8 mg BID and 12 mg BID
  - 58% of patients treated with 12 mg BID and 47% of patients treated with 8 mg BID of CTP-543 achieved a ≥50% reduction in their overall SALT score compared to 9% placebo (p's <0.001)
- Dose-related improvements for 8 mg BID and 12 mg BID across all efficacy assessments
  - 8 mg BID and 12 mg BID significantly different from placebo on all SALT measures and Global Impression of Improvement
  - 12 mg BID numerically superior and generally produced faster onset and greater effect compared to 8 mg BID
- CTP-543 treatment generally well-tolerated
  - Majority of patients from 12 mg BID cohort rolled into long-term open label extension study
- Results support advancement of CTP-543 into pivotal testing
  - Company expects end of Phase 2 meeting with FDA in Q1 2020

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September is Alopecia Areata Awareness Month

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