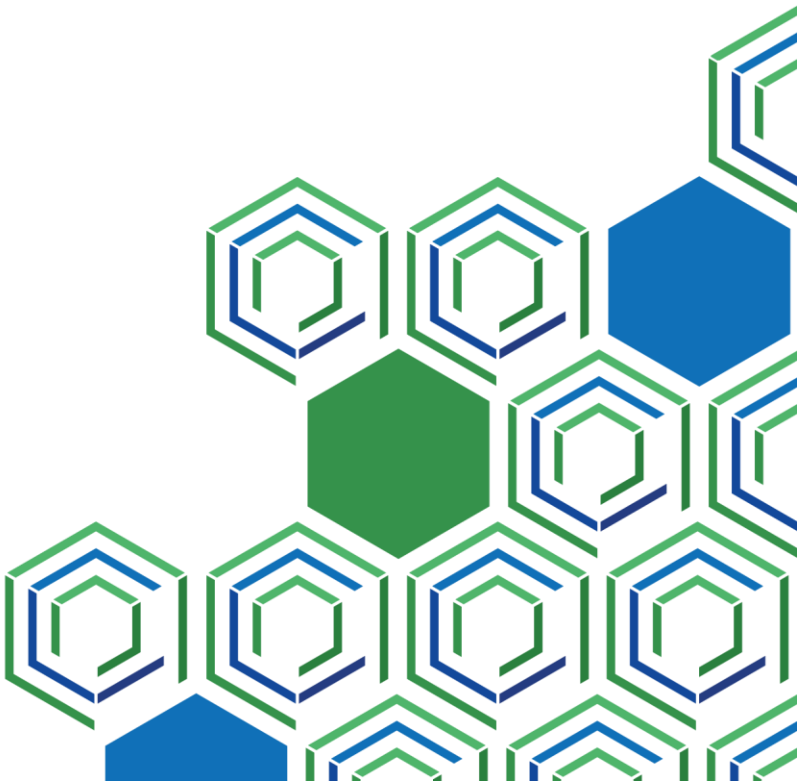
CoNCERT

Product Innovation. **Patient Impact.**

February 2019



Forward-Looking Statements

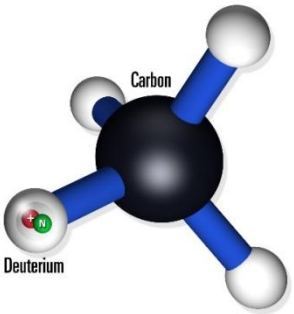
CoNCERT

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 Annual Report on Form 10-K 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.

DCE Platform® Drives Blockbuster Potential

CoNCERT



CTP-543
CTP-692

Novel, diverse pipeline with blockbuster potential in autoimmune and CNS disorders

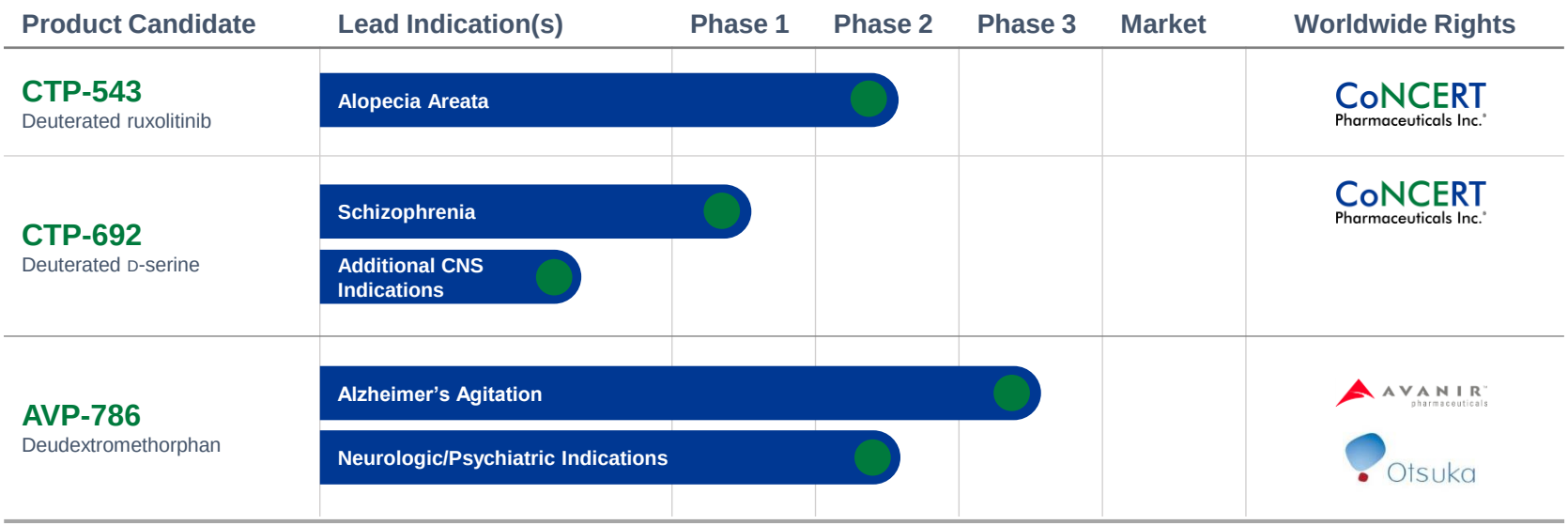
AVP-786
VX-561

Validating deals with potential significant non-dilutive upside

CTP-543
CTP-692
AVP-786

Key data readouts throughout 2019 beginning Q1

Robust Pipeline

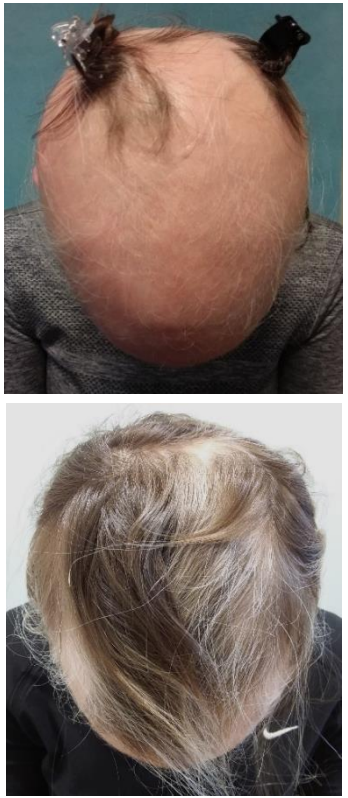


CTP-543: Potential First FDA-Approved Oral Treatment for Alopecia Areata

CoNCERT

Opportunity to address important unmet medical need

- Initial target indication: moderate-to-severe alopecia areata
  - Common autoimmune disorder causing partial or widespread loss of hair on the scalp and/or body
- CTP-543 is a deuterated ruxolitinib analog, possessing a differentiated, potentially superior PK profile
- FDA granted Fast Track designation for CTP-543
- Phase 2 positive topline results reported in interim analysis; primary endpoint achieved with 8 mg twice-daily dose



CTP-543 Phase 2 Trial: Baseline vs. Week 24

Alopecia Areata: A Devastating-Autoimmune Disease

CoNCERT

- 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
- FDA PFDDI meeting held September 2017
  - Strong patient advocacy

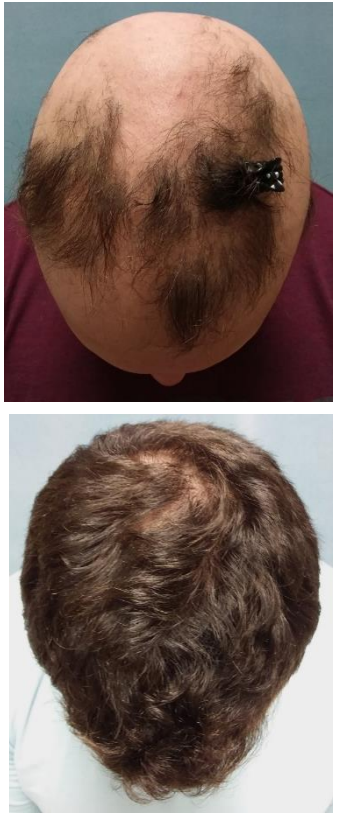
Non-Trial Participants

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

CTP-543: Phase 2 Dose Ranging Trial

CoNCERT

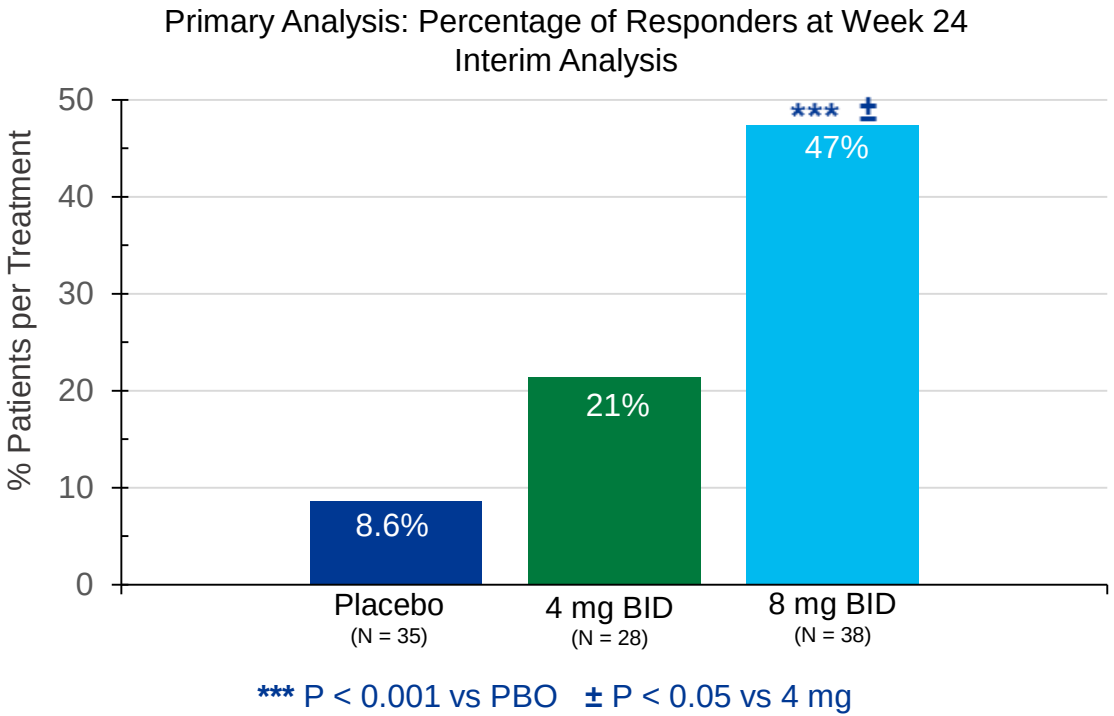
- Phase 2 trial design
  - Double-blind, randomized, placebo-controlled
  - Approximately 145 adults with moderate-to-severe alopecia areata
    - At least 50% hair loss as measured by Severity of Alopecia Tool (SALT)
    - Primary Endpoint: 50% relative reduction in SALT between week 24 and baseline
  - Sequentially randomized to receive one of three doses of CTP-543 (4, 8,12 mg BID) or placebo for 24 weeks
    - 12 mg BID cohort fully enrolled, readout expected 3Q 2019
- Interim results presented at Late-Breaker session at American Academy of Dermatology 2019 annual meeting



CTP-543 Phase 2 Trial: Baseline vs. Week 24

CTP-543 Phase 2: Interim Results Support Advancement

CoNCERT

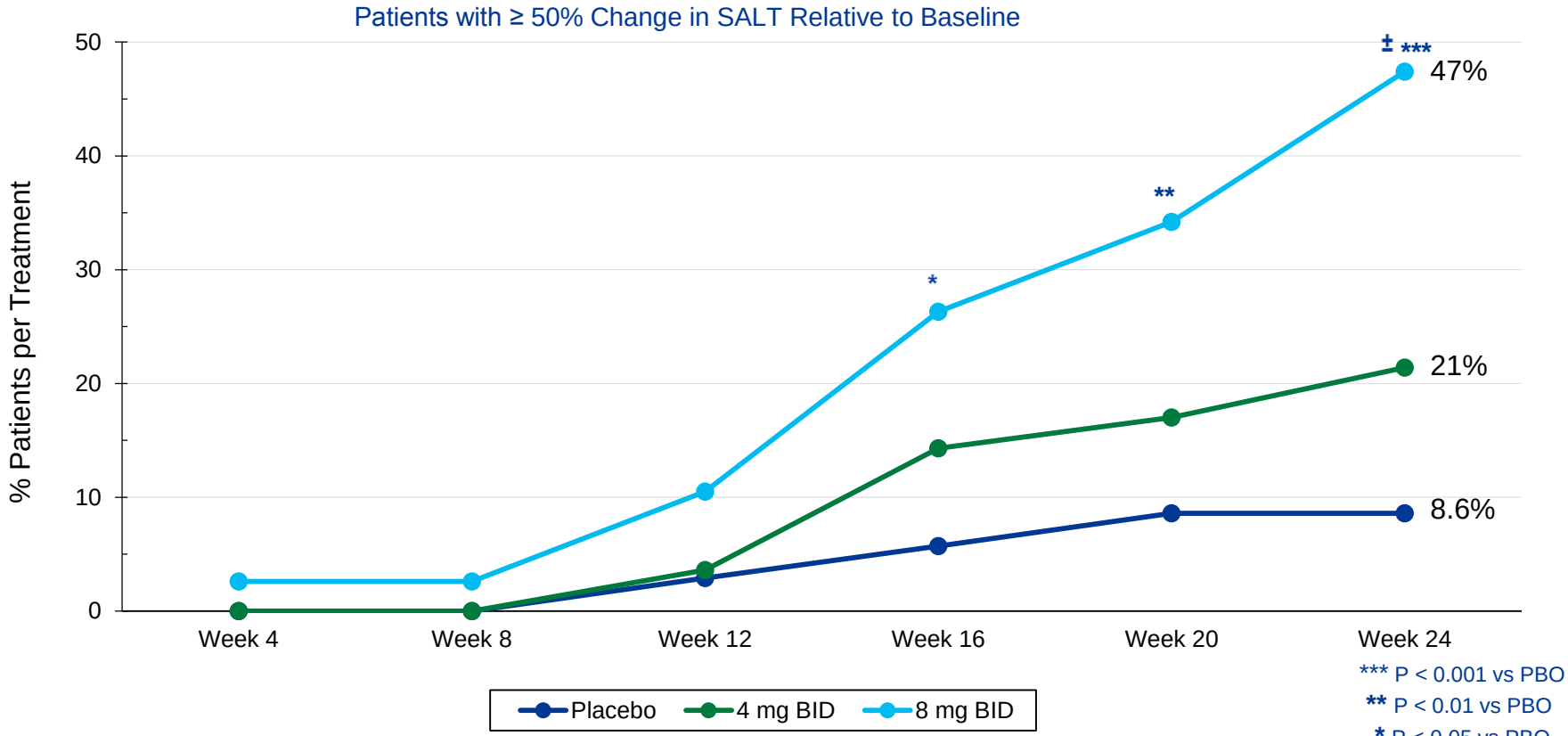


- The primary efficacy endpoint was met in the 8 mg BID cohort
  - 47% of patients achieved a ≥ 50% reduction in their overall SALT score compared to 8.6% placebo ($p < 0.001$)
  - 4 mg BID of CTP-543 was not significantly effective (21% vs. 8.6%, $p > 0.05$)
  - 8 mg BID dose group was significantly different than the 4 mg BID dose group ($p < 0.05$)
- Treatment generally well tolerated with no serious adverse events
  - Only 3 Grade 3/Grade 4 hematology events; distributed equally across placebo, 4 mg and 8 mg groups

Interim Analysis. Responder defined as > 50% Change in SALT Relative to Baseline

CTP-543 Phase 2: Responders by Visit

CoNCERT



*** P < 0.001 vs PBO

** P < 0.01 vs PBO

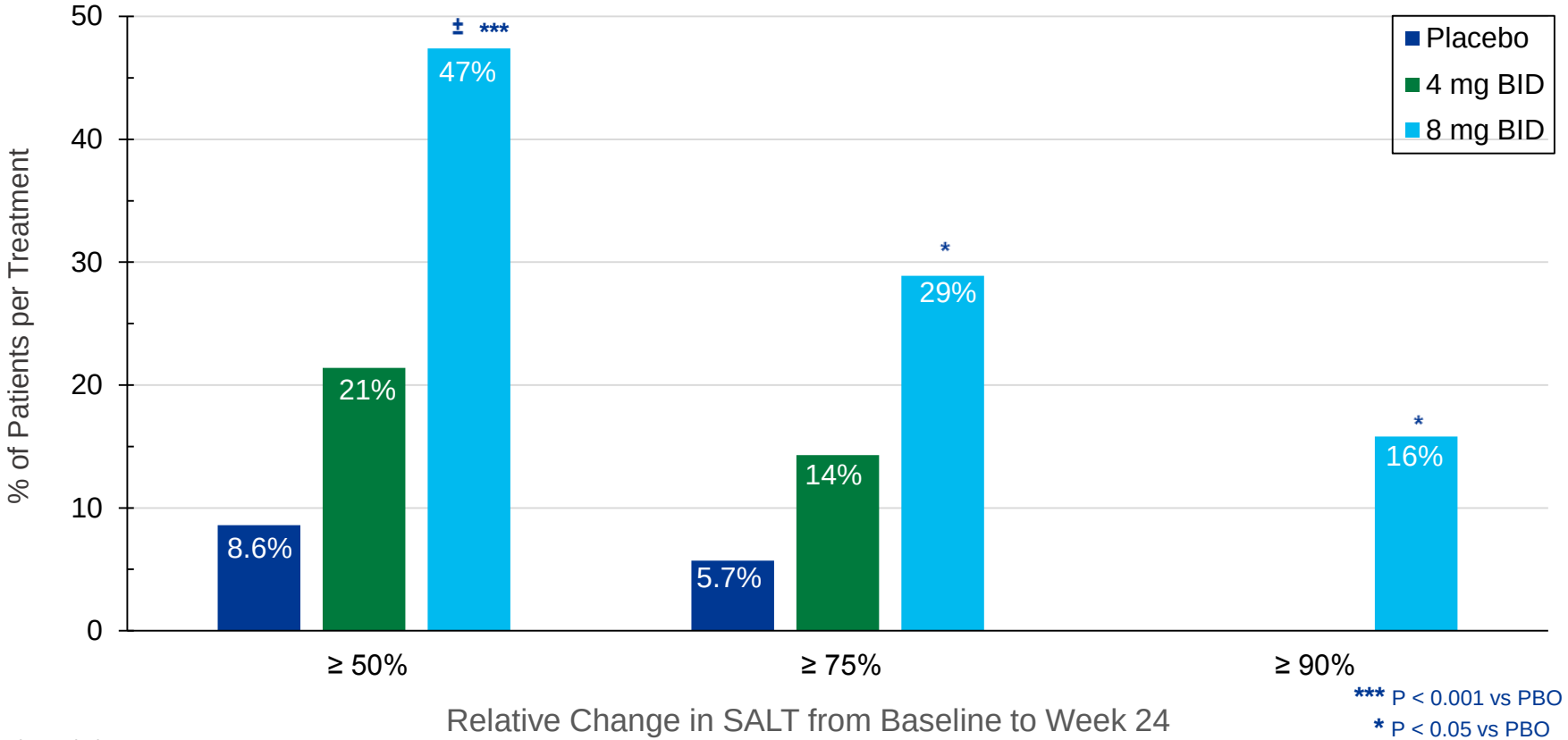
* P < 0.05 vs PBO

± P < 0.05 vs 4 mg

Interim Analysis.

CTP-543 Phase 2: Patient SALT Improvement

CoNCERT



*** P < 0.001 vs PBO

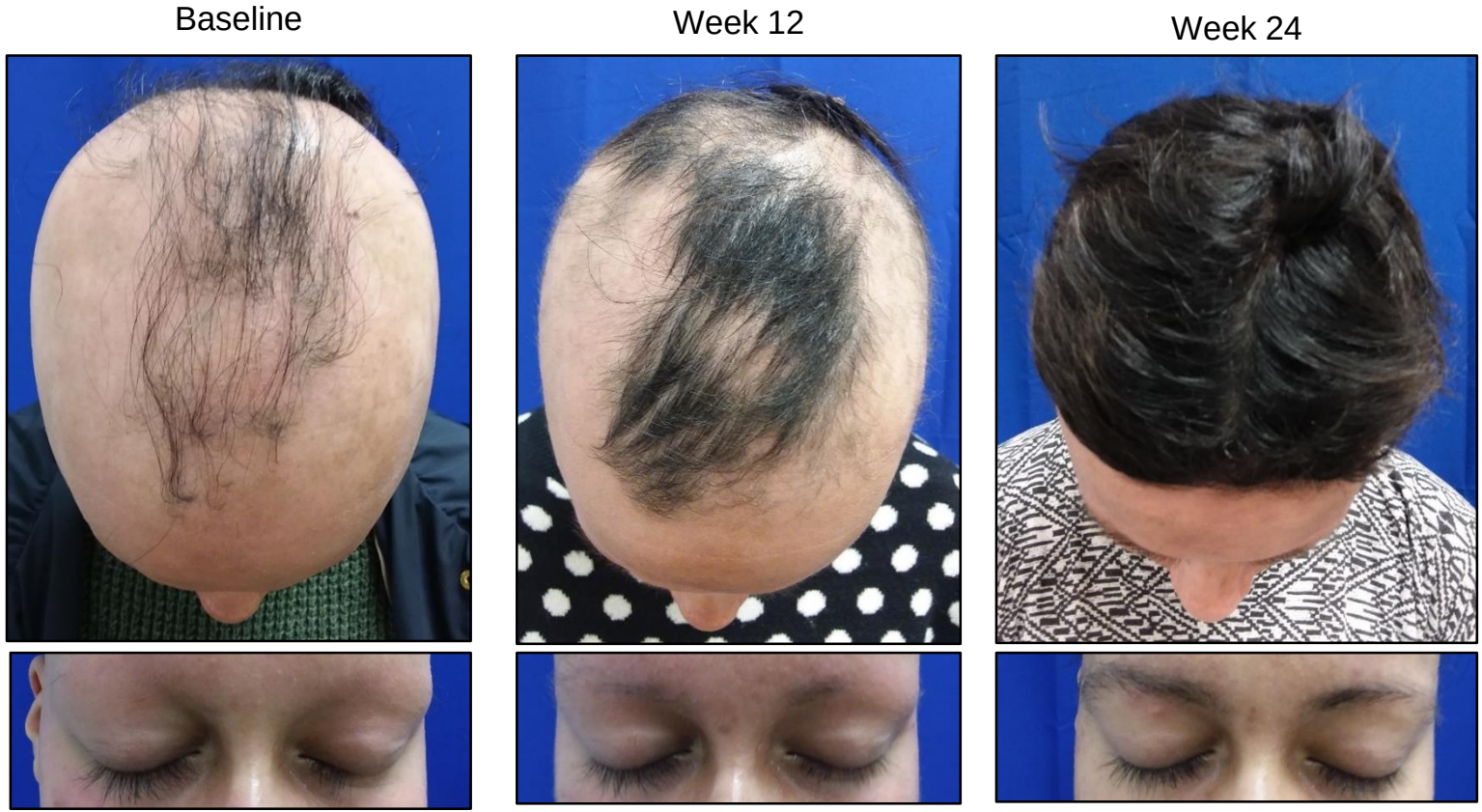
* P < 0.05 vs PBO

± P < 0.05 vs 4 mg

Interim Analysis.

CTP-543 Phase 2: Response Over Treatment Period

CoNCERT

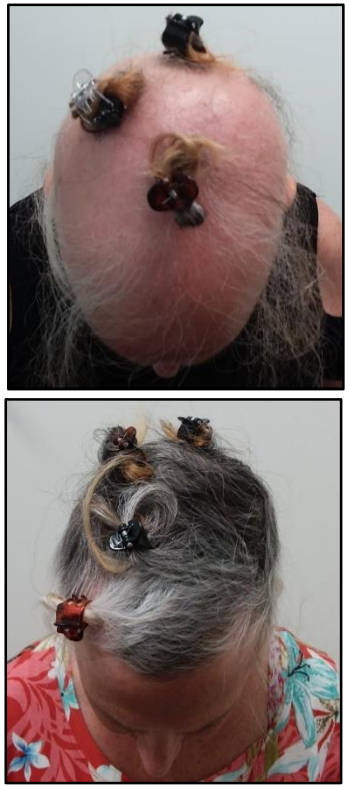


Interim Analysis

CTP-543 Next Steps

CoNCERT

- Met primary endpoint for 8 mg BID cohort in Phase 2 trial with statistical significance ($p<0.001$)
  - 8 mg BID determined to be the minimally effective dose
  - Response did not appear to have plateaued at Week 24
- Dosing in 12 mg BID cohort underway
  - Expect to complete the trial Q3 2019
- Expect to conduct open-label dosing regimen trial and initiate open-label extension study in 2019
- Intend to discuss data and registration strategy with FDA in end of Phase 2 meeting



CTP-543 Phase 2 Trial: Baseline vs. Week 24

CTP-692: Potential First-in-Class Adjunctive Treatment in Schizophrenia

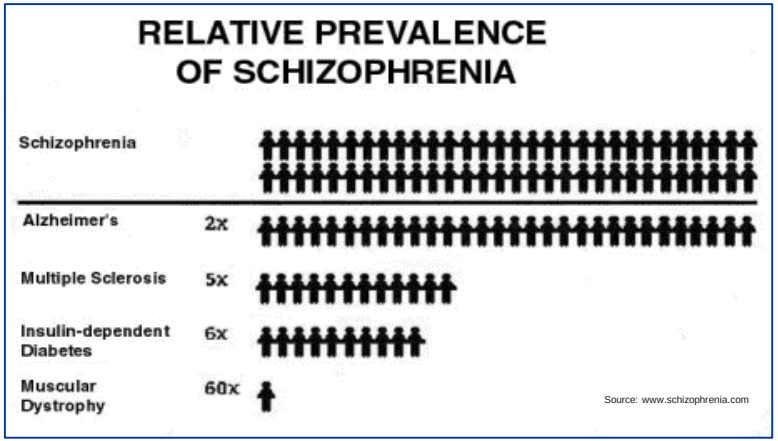
CoNCERT

- CTP-692: deuterated D-serine (NMDA receptor co-agonist)
  - Distinct mechanism added to existing standard of care
- Patients with schizophrenia have low levels of D-serine
- Academic studies with D-serine show benefit on negative and cognitive symptoms of schizophrenia as well as effects on positive symptoms
- Use of D-serine may be limited by renal safety concerns
- Deuterium improves safety profile and exposure in preclinical studies
- Phase 1 program underway

Schizophrenia: Prevalent, Chronic, Severe Mental Disorder

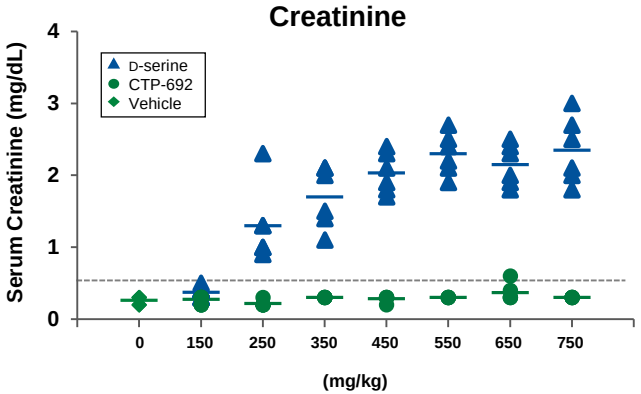
CoNCERT

- Afflicts ~1% of the worldwide population
  - Chronic condition affecting both men and women equally
- Disease characterized by multiple symptoms including:
  - Positive symptoms – hallucinations, delusional behaviors and thought disorder
  - Negative symptoms – social withdrawal, flattened affect and poverty of speech
  - Cognitive dysfunction – diminished capacity for attention, working memory, and executive function
- Unmet need exists to treat symptoms of schizophrenia

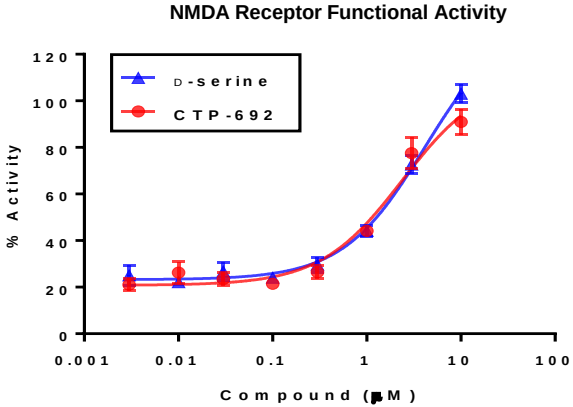


CTP-692: Designed to Overcome Limitations of D-Serine

- D-serine is well-known to cause nephrotoxicity in preclinical testing
- CTP-692 improved preclinical renal safety reflected by serum creatinine and blood urea nitrogen levels
- Deuterium modification increases D-serine oral exposure (C_{max}, AUC, $T_{1/2}$)
- CTP-692 may achieve therapeutically effective drug levels with potential for renal safety advantage
- Phase 1 testing underway



*N=6 for D-serine and CTP-692 except at 150mg/kg where N=12 for both
Dashed line indicates Upper Range of Normal



AVP-786: Potential First-in-Class Treatment for Agitation in Alzheimer's Disease

CoNCERT

- Estimated 5.7M Americans have Alzheimer's disease[1]; approximately 50% of patients experience agitation[2]
  - No currently approved therapies
- Phase 3 trials underway for blockbuster indication
  - Completion of two North American trials projected for 2019 and expected to be part of NDA submission
- Otsuka (Avanir) responsible for development and commercialization
  - $170M upfront/milestone potential; $8M achieved to date
  - $5M milestone on acceptance of NDA
  - Mid-single to low-double digit royalties

[1] https://www.alz.org/media/Documents/facts-and-figures-2018-r.pdf
[2] Lyketsos CG et al. American Journal of Psychiatry 2000;157:708–714

Enhancing Value: Capital Efficiency and Strategic Agreements

CoNCERT

Strong Financial Position (Q418)

- Cash: $153.3 million
- Shares outstanding: 23.4 M

Late Stage Collaboration

- First key AVP-786 readout Q2 2019
- Significant milestone and royalty potential

AVANIR pharmaceuticals · Otsuka

Strong Validation of Platform

- VX-561 (CTP-656) asset sale
- $90M in pre-commercial milestones

VERTEX

Successful Out Licensing

- Out-license of non-core development provides additive value
- Downstream financial potential

Cipla · Celgene · Jazz Pharmaceuticals · Processa

Multiple Potential Value Creating Events

CoNCERT

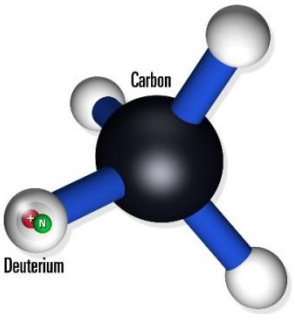
Upcoming Development Milestones

- **CTP-543 for Alopecia Areata**
  - Report Phase 2a (12 mg) data (Q3 2019)
  - Initiate Phase 2 QD vs BID trial (1H 2019)
  - Report Phase 2 QD vs BID results (2H 2019)
  - Develop registration strategy (2019)
- **CTP-692 for Schizophrenia**
  - Report Phase 1 data (1H 2019)
  - Initiate Phase 2 trial (Q4 2019)
- **AVP-786 for Alzheimer's Agitation**
  - Avanir expected to complete Phase 3 trials (Q2 2019 and Q4 2019)
- **VX-561 for Cystic Fibrosis***
  - Vertex to initiate Phase 2 monotherapy trial (1H 2019)

* VX-561 for cystic fibrosis: $90M in pre-commercial milestones remaining

DCE Platform® Drives Blockbuster Potential

CoNCERT



CTP-543
CTP-692

Novel, diverse pipeline with blockbuster potential in autoimmune and CNS disorders

AVP-786
VX-561

Validating deals with potential significant non-dilutive upside

CTP-543
CTP-692
AVP-786

Key data readouts throughout 2019 beginning Q1

CoNCERT

NASDAQ: CNCE
www.concertpharma.com
@ConcertPharma

For additional information contact:
Justine Koenigsberg
ir@concertpharma.com