

CoNCERT

**Creating New Possibilities for Patients
to Live Their Lives**

June 2020



Forward-Looking Statements

Any statements in this presentation about our future expectations, plans and prospects, including, among others, statements about our expectations on the progress of clinical development of CTP-543 and CTP-692 and the timing of availability of clinical trial data, and any other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation and timing of future clinical trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements, expectations with respect to the protection of our intellectual property afforded by our patents and other 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. In addition, any forward-looking statements included in this presentation represent our views only as of the date of this presentation and should not be relied upon as representing our views as of any subsequent date. We specifically disclaim any obligation to update any forward-looking statements included in this presentation.

Clinical Pipeline

Product Candidate	Lead Indications	Phase 1	Phase 2	Phase 3	Market	Worldwide Rights
CTP-543 Deuterated ruxolitinib	Alopecia Areata: Phase 3 Expected to Begin Q4 2020					
	Alopecia Areata: Dose Ranging Completed		●			
	Alopecia Areata: Dose Regimen Completed (8 BID vs 16 QD)		●			
	Alopecia Areata: Dose Regimen Completed (12 BID vs 24 QD)		●			
	Alopecia Areata: Long Term Extension			➔		
CTP-692 Deuterated D-serine	Schizophrenia: Dose Ranging Ongoing		●			
	Phase 1 Crossover Completed		●			
	Phase 1 SAD/MAD Completed		●			
	Additional CNS Indications		●			

CTP-543: Late Stage Asset for Alopecia Areata

- Initial target indication: moderate-to-severe alopecia areata
 - Common autoimmune disorder causing partial or widespread loss of hair on the scalp and/or body
 - Opportunity to address important unmet medical need
- CTP-543 is a deuterated ruxolitinib analog, possessing a differentiated, potentially superior PK profile
- FDA granted Fast Track designation for CTP-543
- IP estate provides protection into 2037
- Plan to initiate Phase 3 program in Q4 2020
 - Phase 2 positive topline results reported: primary endpoint achieved with 8 mg and 12 mg twice-daily doses



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

Alopecia Areata: A Devastating Autoimmune Disease

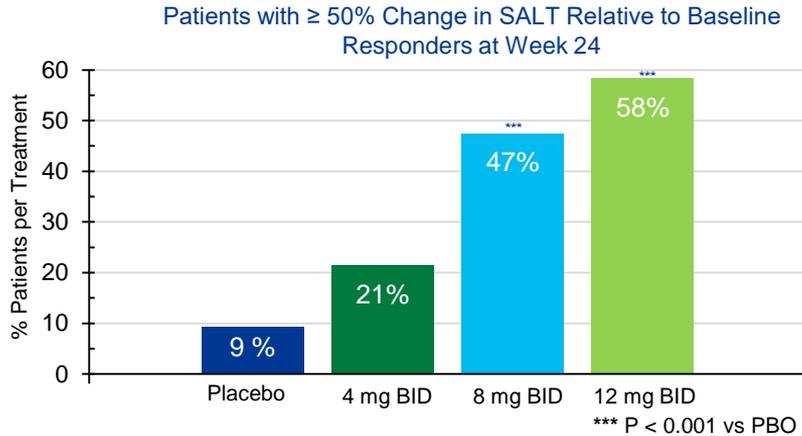
- Approximately 700,000 patients affected with alopecia areata in the U.S. at any given time*
- Estimated 40+% of patients reported to have $\geq 50\%$ loss of scalp hair*
- Chronic condition affecting women, men and children of all ages
- Disease profoundly impacts patients
 - Associated with anxiety, depression and other autoimmune conditions
- No FDA-approved treatment options
- FDA PFDDI meeting held September 2017
 - Strong patient advocacy



Non-Trial Participants

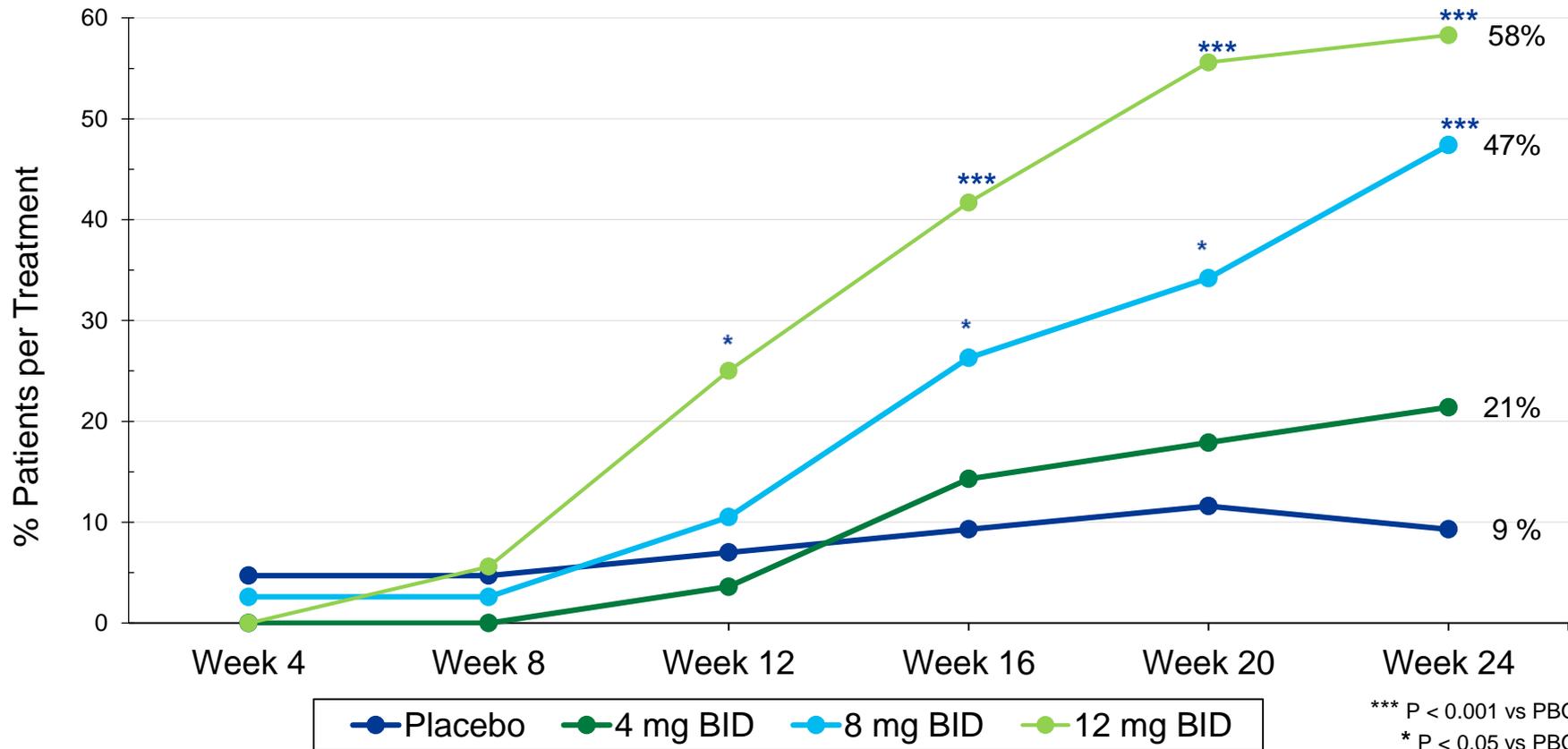
CTP-543: Phase 2 Dose Ranging Trial Complete

- Randomized 149 adult patients with moderate-to-severe alopecia areata in a double-blind, randomized, placebo-controlled trial
 - At least 50% hair loss as measured by Severity of Alopecia Tool (SALT)
 - Primary Endpoint: 50% relative reduction in SALT at Week 24 from baseline
 - Sequentially randomized to receive one of three doses of CTP-543 (4, 8,12 mg BID) or placebo for 24 weeks
- Primary endpoint met with statistical significance for 8 mg and 12 mg doses at Week 24
 - 12 mg responders average 86% SALT improvement
 - 8 mg responders average 78% SALT improvement
- Patient Global Impression of Improvement scale
 - 78% (12 mg BID) and 58% (8 mg BID) of patients rated “much improved” or “very much improved” at Week 24



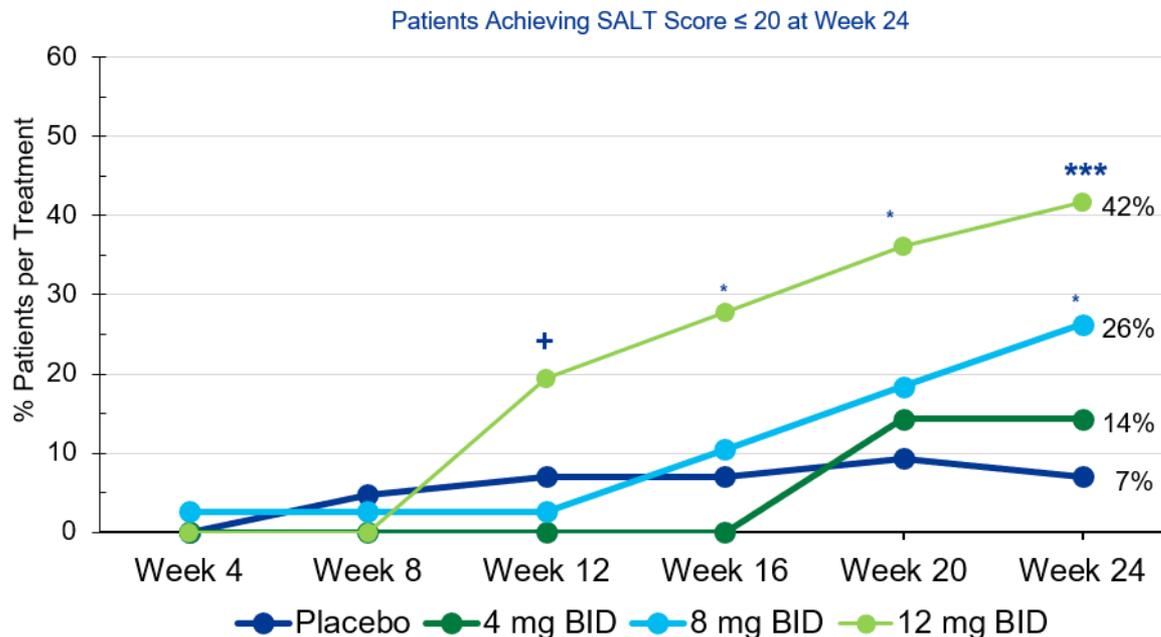
Response Over Treatment Period: 8 mg BID

Responders: $\geq 50\%$ Change in SALT Relative to Baseline



CTP-543 Phase 2: Patients Achieving SALT Score ≤ 20

- CTP-543 8 mg BID and 12 mg BID significantly different from placebo on percent of patients achieving a SALT score ≤ 20 at 24 weeks
 - SALT 20 indicates 80 percent or greater scalp hair present
- SALT 20 is the primary efficacy endpoint to be utilized in Phase 3 studies



*** P < 0.001 vs PBO

* P < 0.05 vs PBO

+ P < 0.05 vs 8 mg

CTP-543 Response Over Treatment Period: 12 mg BID

- 12 mg BID generally produced faster onset and greater magnitude of effect compared to 8 mg BID
- Eyebrow and eyelash involvement not formally assessed

Baseline

Week 24



- Substantial regrowth observed
- Further assessments planned in future trials

Baseline



Week 12



Week 24



Dose Ranging Phase 2 Trial

- Treatment was generally well tolerated with few AE-related drop outs
- Most common ($\geq 10\%$) side effects in either the 8 mg or 12 mg BID dose groups were headache, nasopharyngitis, upper respiratory tract infection, acne, nausea and LDL increase
- 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
- No thromboembolic events were reported during the trial

CTP-543 Phase 3 Trial Design Overview

Phase 3 design is consistent with Phase 2 trial to support registration
Discussed key aspects of planned Phase 3 trials with FDA at End-of-Phase 2 meeting
Potential best-in-class oral treatment for alopecia areata based on Phase 2 results

Design	<p>Randomized, double-blind, placebo-controlled trial in patients with moderate-to-severe alopecia areata</p> <p>Approximately 700 patients age 18-65 years with $\geq 50\%$ hair loss</p> <p>3:3:1 randomization to CTP-543 (8 mg BID or 12 mg BID) or placebo for 24 weeks</p> <p>Opportunity for completers to roll over into open label, long-term extension study</p> <p>Sites: U.S., Canada and Europe</p>
Endpoint	<p>Primary endpoint is SALT score ≤ 20</p> <p>Secondary endpoints include patient and clinician impression scores, patient reported outcome measures and regrowth of eyebrows and eyelashes</p>
Status	<p>First Phase 3 expected to initiate in Q4 2020</p>

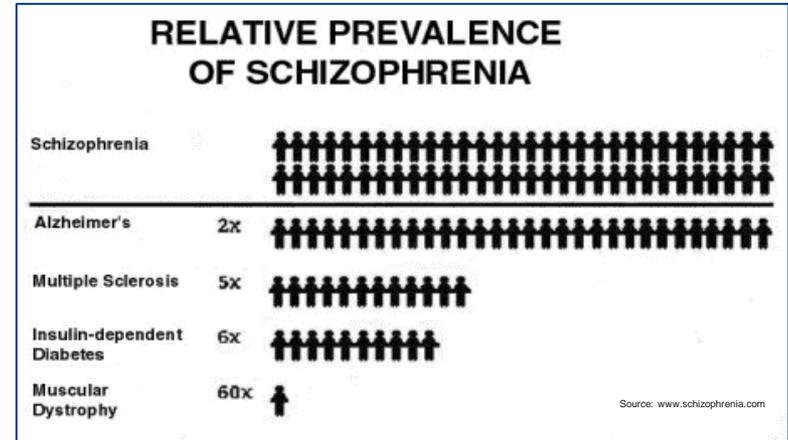
CTP-692: Potential First-in-Class Adjunctive Treatment in Schizophrenia

- 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 2 trial underway

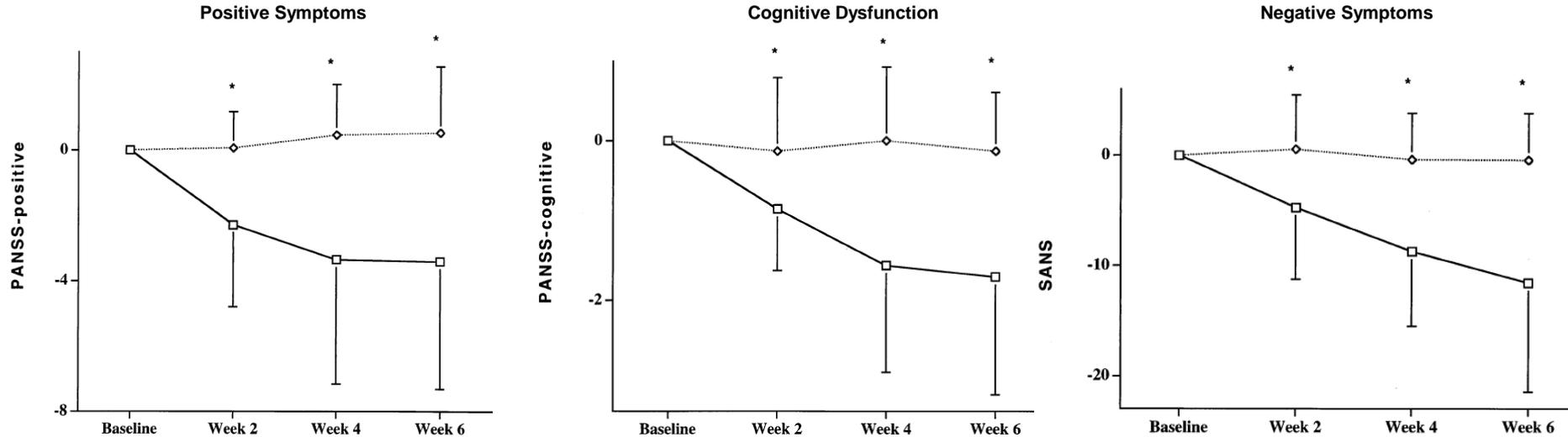


Schizophrenia: Prevalent, Chronic, Severe Mental Disorder

- 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



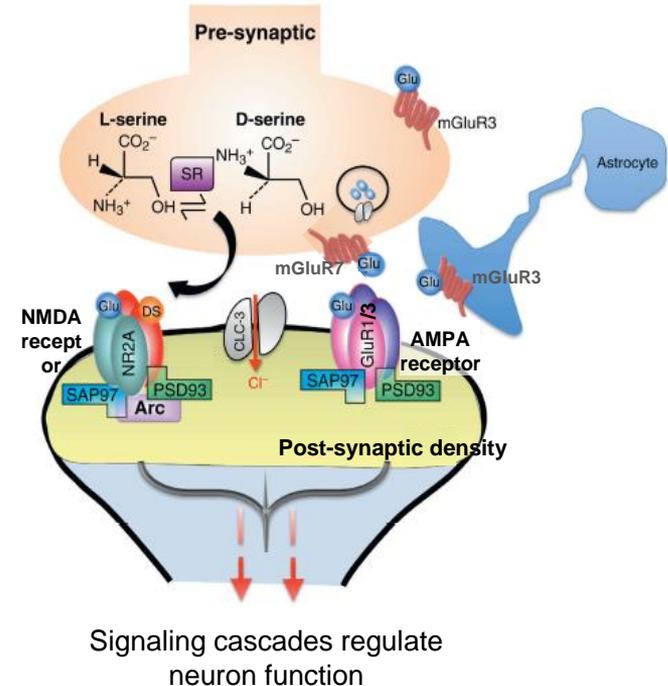
Published Literature: D-Serine Improves Multiple Symptom Domains of Schizophrenia CoNCERT



- First double-blind, placebo-controlled study as add-on to stable antipsychotic regimen
- D-serine administered 30 mg/kg per day for 6 weeks
- N = 31

Genetic Studies Support CTP-692 Mechanism For Adjunctive Treatment of Schizophrenia CoNCERT

- Modern screening technology has enabled the discovery of a set of genes associated with schizophrenia
 - Genes coding for glutamatergic synaptic proteins are prominently represented
- Poor patient response to antipsychotics is associated with mutations that impair NMDA neurotransmission*

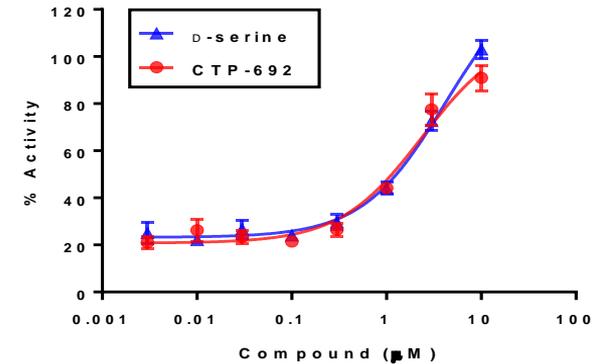


Modified from Balu and Coyle, Curr Opin Pharmacol 2015

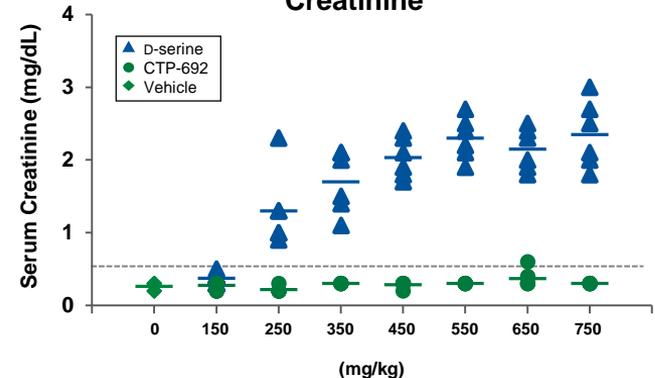
CTP-692: Designed to Leverage D-Serine Benefits and Overcome its Limitations

- Same pharmacology as D-serine with comparable binding and functional activity at NMDA receptor
- 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
- CTP-692 achieved higher brain concentration relative to plasma compared to D-serine

NMDA Receptor Functional Activity



Creatinine

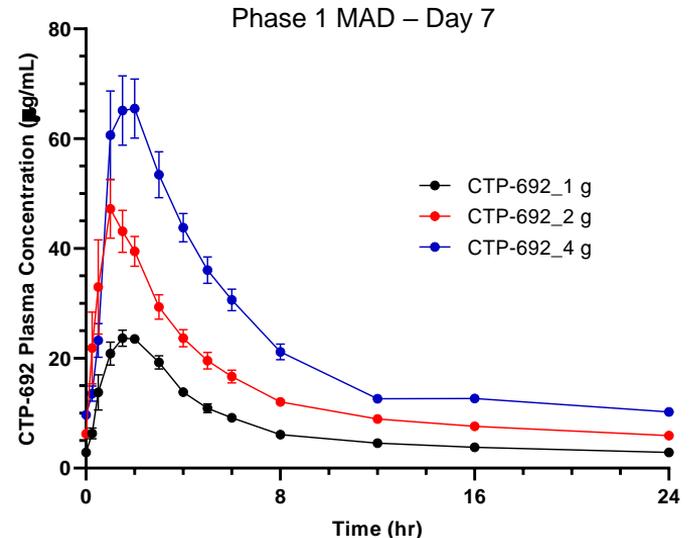


*N=6 for D-serine and CTP-692 except at 150mg/kg where N=12 for both
Dashed line indicates Upper Range of Normal

CTP-692: Phase 1 Topline Results

CTP-692 demonstrated a favorable safety, tolerability and PK profile with no SAEs reported

- Safety assessments in the SAD/MAD trials showed CTP-692 was well tolerated over the dose ranges tested; include doses expected to be evaluated in Phase 2
- Key blood and urine markers of kidney function did not indicate any signs of renal impairment
 - Data are consistent with CTP-692 preclinical findings indicating an improved renal safety profile compared to non-deuterated D-serine
- CTP-692 has a well behaved PK profile
 - D-serine is reported to be substantially variable
- Phase 1 data presented at American Society of Clinical Psychopharmacology 2020 Annual Meeting



- Phase 2 evaluation underway
 - Indication: Adjunctive treatment of schizophrenia
 - Primary endpoint: Change in total Positive and Negative Syndrome Scale (PANSS) score Week 12 from baseline
 - Inclusion: Stable on antipsychotic medication
 - Dosing: 1, 2 and 4 grams of CTP-692 once-daily vs. placebo
 - Number of patients: ~300 randomized
- Expect single Phase 2 to support Phase 3 development



Enhancing Value: Capital Efficiency and Strategic Agreements ^{CoNCERT}

Strong Financial Position (Q1 2020)

- Cash: \$159.6 M
- Shares outstanding: 29.7 M

Strong Validation of Platform

- VX-561 (CTP-656) asset sale; \$160 M upfront
- Up to \$90 M in pre-commercial milestones



Successful Out Licensing

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



Creating new possibilities for patients to live their lives



CTP-543 for Alopecia Areata

- ✓ Conducted End-of-Phase 2 FDA meeting Q1 2020 to support advancement into pivotal testing
- Next milestone: Expect to initiate Phase 3 clinical program in Q4 2020

CTP-692 for Schizophrenia*

- Expect single Phase 2 to support Phase 3 development
- Next milestone: Complete enrollment in ongoing Phase 2 trial

*Pending impact of COVID-19 on development activities

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