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Creating New Possibilities for Patients to Live Their Lives

August 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

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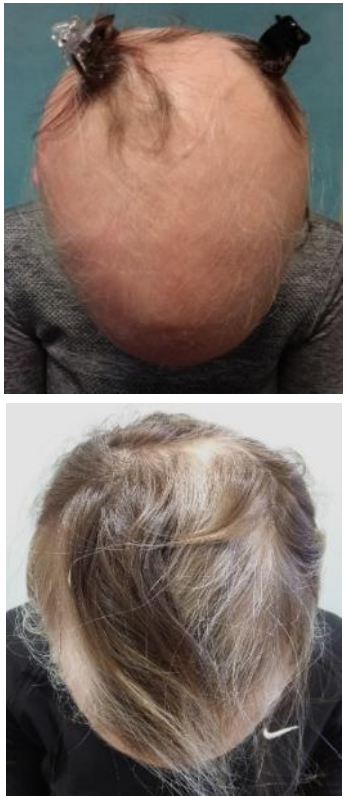
CTP-543: Late Stage Asset for Alopecia Areata

- 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 Breakthrough Therapy and Fast Track designations 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

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- Approximately 700,000 patients affected with alopecia areata in the U.S. at any given time*
- Estimated 40+% of patients reported to have ≥ 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

*Benigno M. Clinical, Cosmetic and Investigational Dermatology 2020

CTP-543: Phase 2 Dose Ranging Trial Complete

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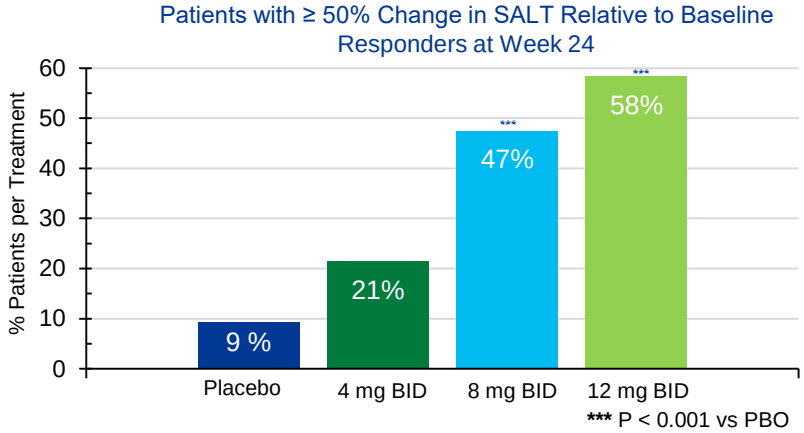
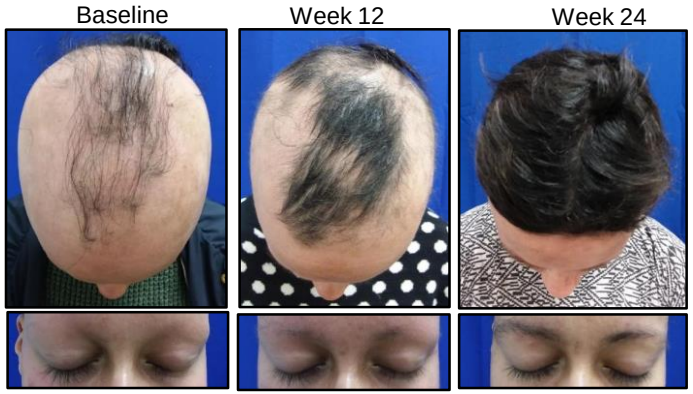
- Randomized 149 adult patients with moderate-to-severe alopecia areata in a double-blind, randomized, placebo-controlled trial
  - At least 50% hair loss as measured by Severity of Alopecia Tool (SALT)
  - Primary Endpoint: 50% relative reduction in SALT at Week 24 from baseline
  - Sequentially randomized 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: ≥ 50% Change in SALT Relative to Baseline

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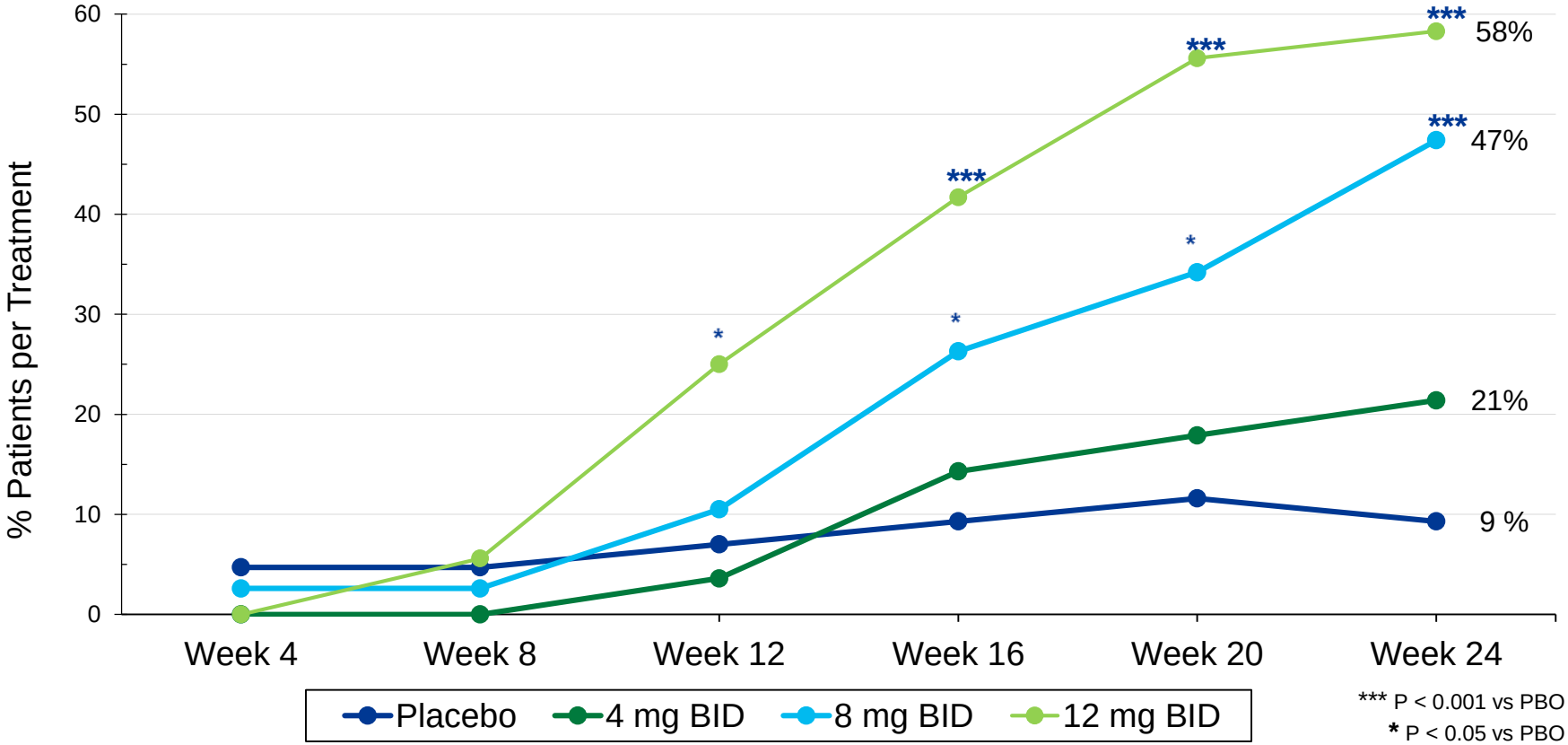

CTP-543 Phase 2: Patients Achieving SALT Score ≤ 20

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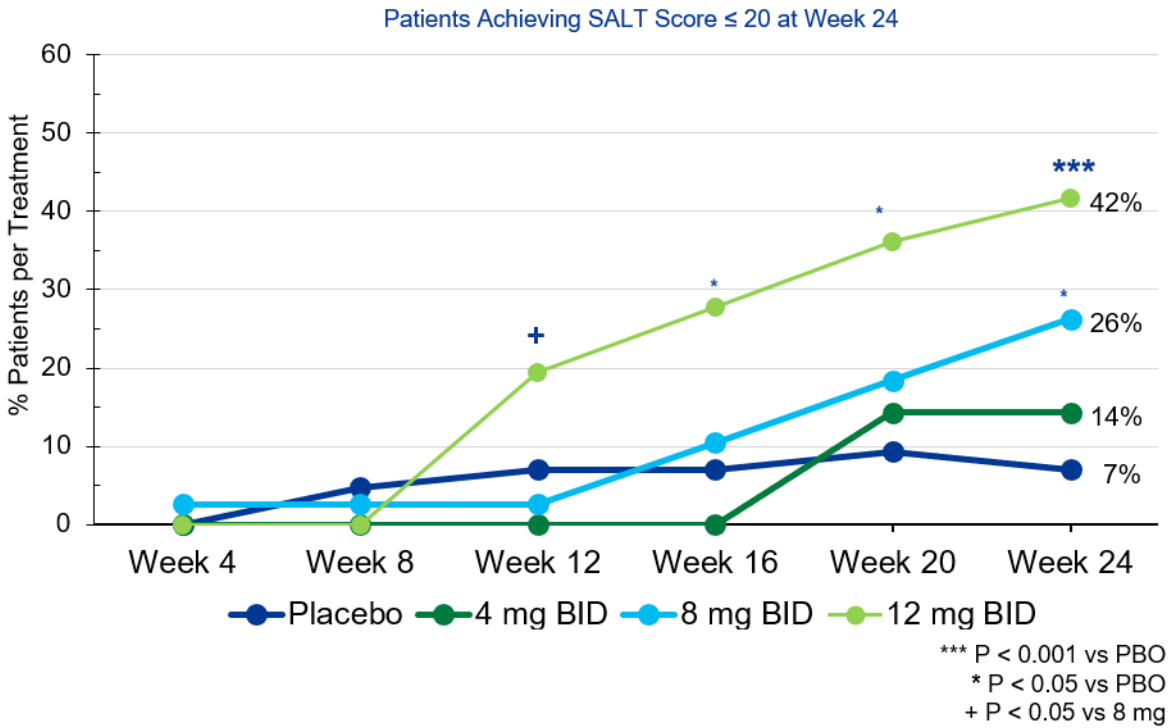
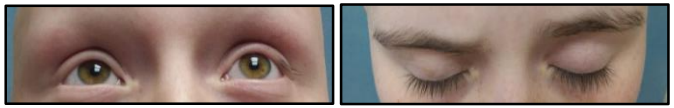
- CTP-543 8 mg BID and 12 mg BID significantly different from placebo on percent of patients achieving a SALT score ≤20 at 24 weeks
  - SALT 20 indicates 80 percent or greater scalp hair present
- SALT 20 is the primary efficacy endpoint to be utilized in Phase 3 studies



CTP-543 Response Over Treatment Period: 12 mg BID

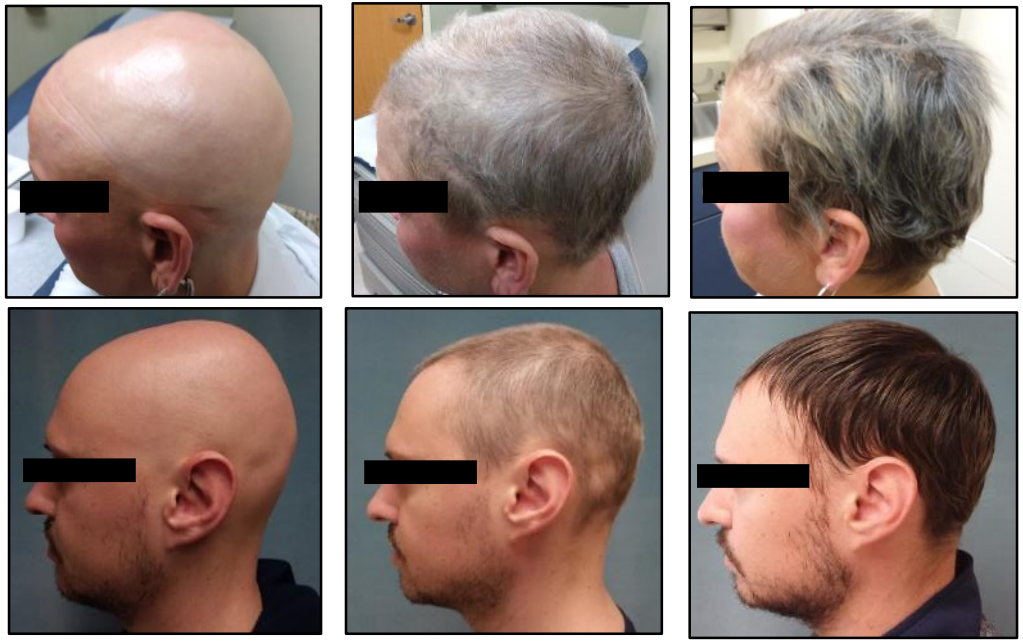
- 12 mg BID generally produced faster onset and greater magnitude of effect compared to 8 mg BID
- Eyebrow and eyelash involvement not formally assessed

Baseline | Week 24



  - Substantial regrowth observed
  - Further assessments planned in future trials

Baseline | Week 12 | Week 24



CTP-543 Phase 2 Dose Ranging Trial
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
Diarrhea	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 Phase 3 Trial Design Overview

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

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

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- 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

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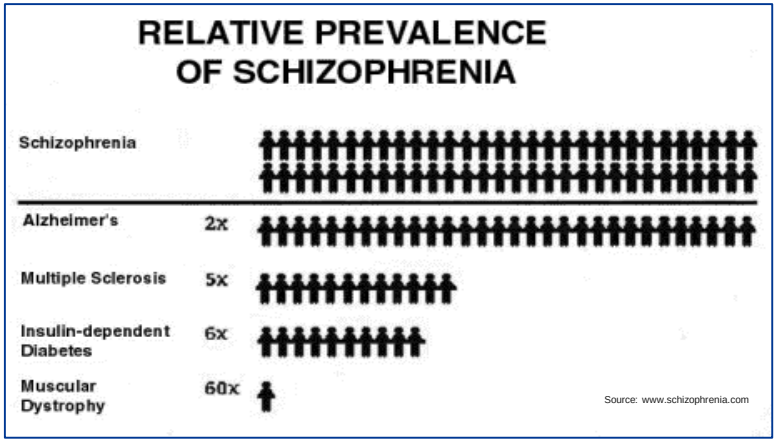
- Afflicts ~1% of the worldwide population
  - Chronic condition affecting both men and women equally
- Disease characterized by multiple symptoms including:
  - Positive symptoms – hallucinations, delusional behaviors and thought disorder
  - Negative symptoms – social withdrawal, flattened affect and 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

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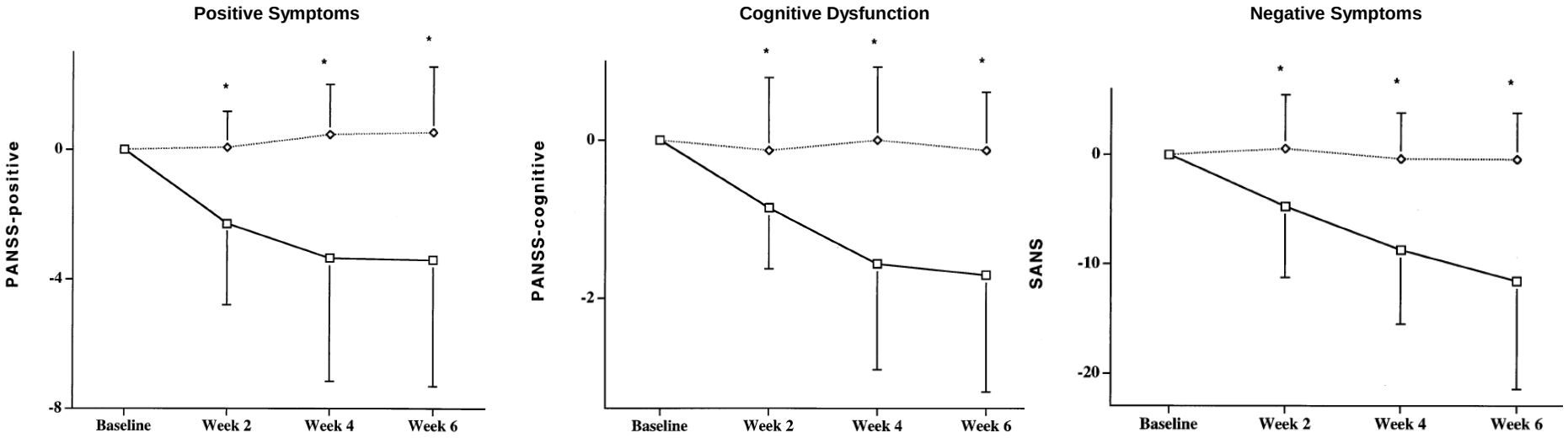

- First double-blind, placebo-controlled study as add-on to stable antipsychotic regimen
- D-serine administered 30 mg/kg per day for 6 weeks
- N = 31

Tsai et al., Biol Psychiatry, 44 (11): 1081-89, 1998

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

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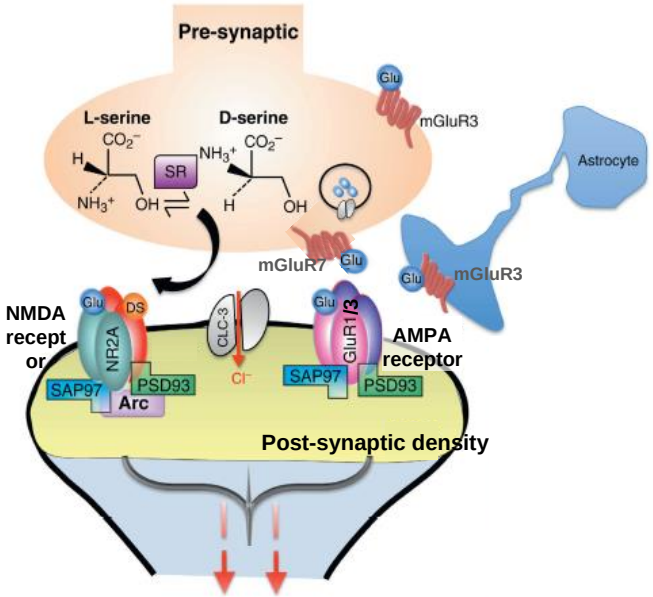
- Modern screening technology has enabled the discovery of a set of genes associated with schizophrenia
  - Genes coding for glutamatergic synaptic proteins are prominently represented
- Poor patient response to antipsychotics is associated with mutations that impair NMDA neurotransmission*



Signaling cascades regulate neuron function

Modified from Balu and Coyle, Curr Opin Pharmacol 2015

*Wang et al. JAMA Psychiatry 2018

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

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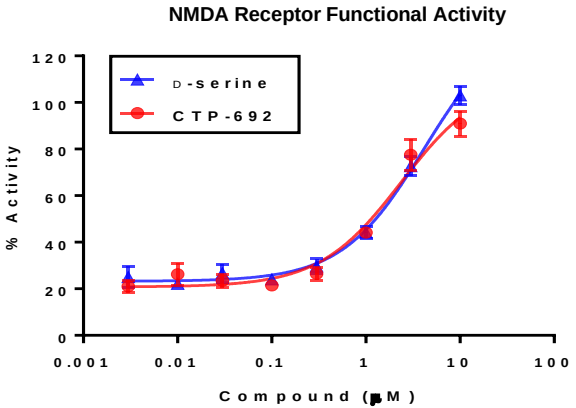
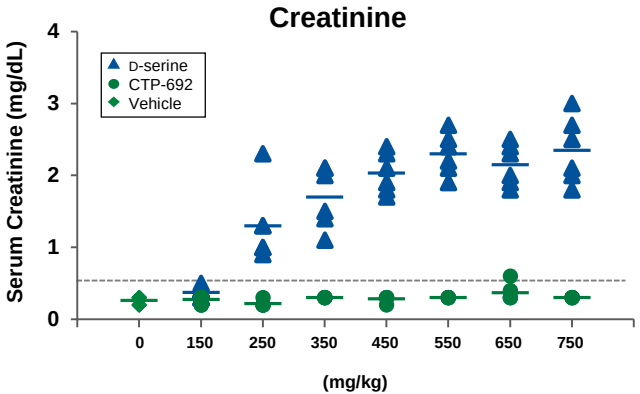
- Same pharmacology as D-serine with comparable binding and functional activity at NMDA receptor
- D-serine is well-known to cause nephrotoxicity in preclinical testing
- CTP-692 improved preclinical renal safety reflected by serum creatinine and blood urea nitrogen levels
- CTP-692 achieved higher brain concentration relative to plasma compared to D-serine





*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

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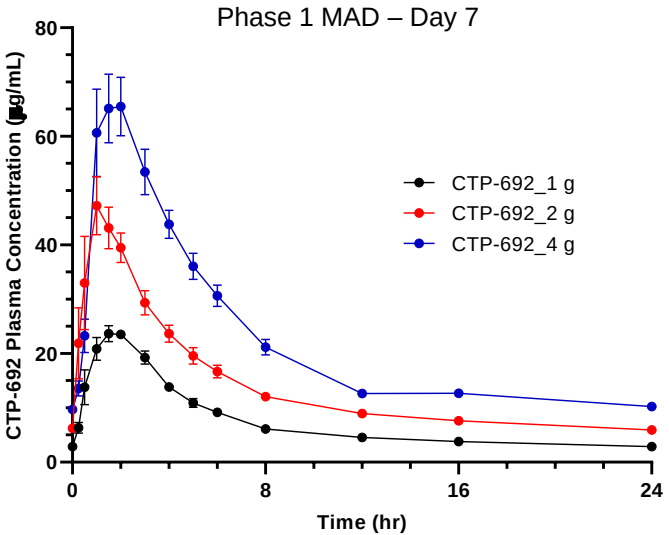
CTP-692 demonstrated a favorable safety, tolerability and PK profile with no SAEs reported

- 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



CTP-692: Phase 2 Trial

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- 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

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Strong Financial Position (Q2 2020)

- Cash: $144.7 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

VERTEX

Successful Out Licensing

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

AVANIR pharmaceuticals · Otsuka · Celgene · Cipla · Jazz Pharmaceuticals · Processa

Upcoming Development Milestones

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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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