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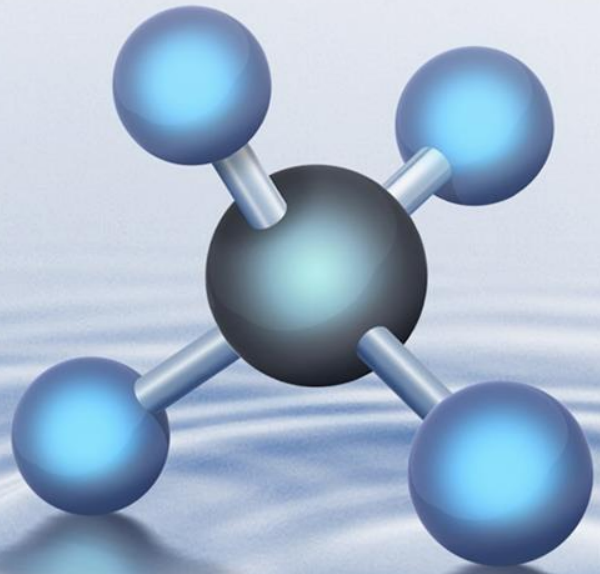
Product Innovation. Patient Impact.

June 2018



Forward-Looking Statements

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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.

DCE Platform® Drives Blockbuster Potential

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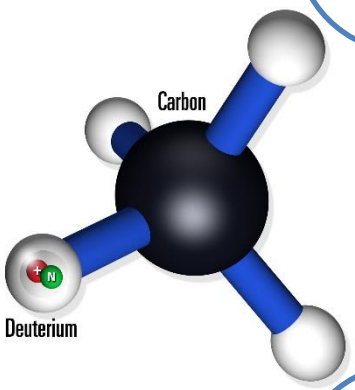

CTP-543
CTP-692

Novel, diverse pipeline with blockbuster potential

AVP-786
VX-561

Validating deals with significant non-dilutive upside

CTP-543
AVP-786

Key data readouts beginning Q4 2018

Robust Pipeline

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Product Candidate	Lead Indication(s)	Phase I	Phase 2	Phase 3	Market	Worldwide Rights
CTP-543 Deuterated ruxolitinib	Alopecia Areata					CoNCERT Pharmaceuticals Inc.
CTP-692 Deuterated D-serine	Schizophrenia					CoNCERT Pharmaceuticals Inc.
AVP-786 Deuterated dextromethorphan	Alzheimer's Agitation					AVANIR pharmaceuticals Otsuka
	Neurologic/Psychiatric Indications					
CTP-730 Deuterated apremilast	Inflammatory Diseases					Celgene
JZP-386 Deuterated sodium oxybate	Narcolepsy					Jazz Pharmaceuticals

Strong Validation of Platform: CTP-656 Asset Purchase

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- Vertex Pharmaceuticals acquired CTP-656 (D-ivacaftor) and other assets related to the treatment of cystic fibrosis
- Agreement closed in July 2017
  - $160 million upfront payment to Concert
  - $90 million in pre-commercial milestone potential
- Vertex is a pioneer in CF with a broad pipeline and the capability to efficiently advance CTP-656
  - Potential for once-daily combination products
  - VX-561 (formerly CTP-656) demonstrated strong efficacy in Phase 2
  - Potential inclusion in Phase 3 triple combination trial

CTP-543: Potential First Oral Treatment for Alopecia Areata

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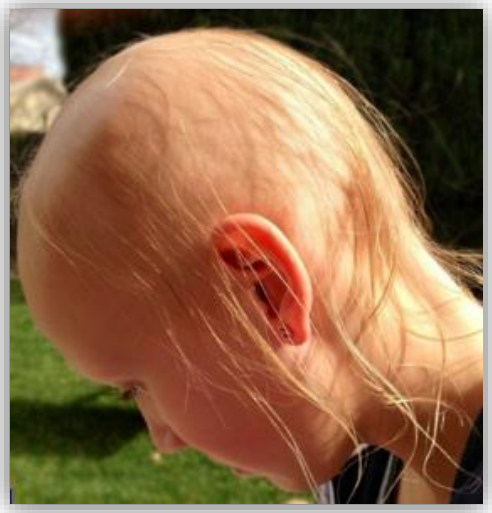
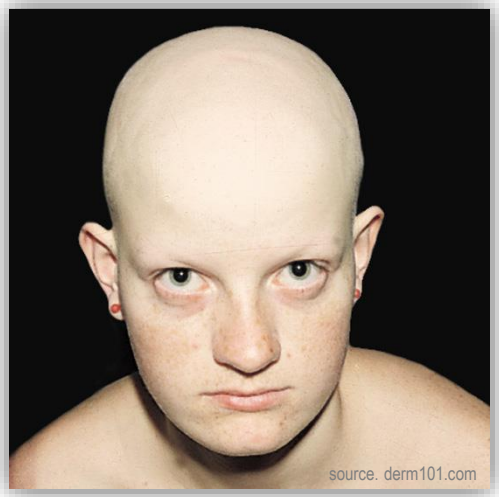
Opportunity to address important unmet medical need

- Initial target indication: moderate-to-severe alopecia areata
  - Common autoimmune disorder causing partial or widespread loss of hair on the scalp and/or body
- Clinical proof-of-concept demonstrated with ruxolitinib in alopecia areata
- CTP-543 is a deuterated ruxolitinib (JAK 1/2), possessing a potentially superior PK profile
- FDA granted Fast Track designation for CTP-543
- Phase 2a trial underway





Alopecia Areata: A Devastating and Poorly Treated 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

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

Clinical Evidence for Efficacy of Ruxolitinib in Alopecia Areata

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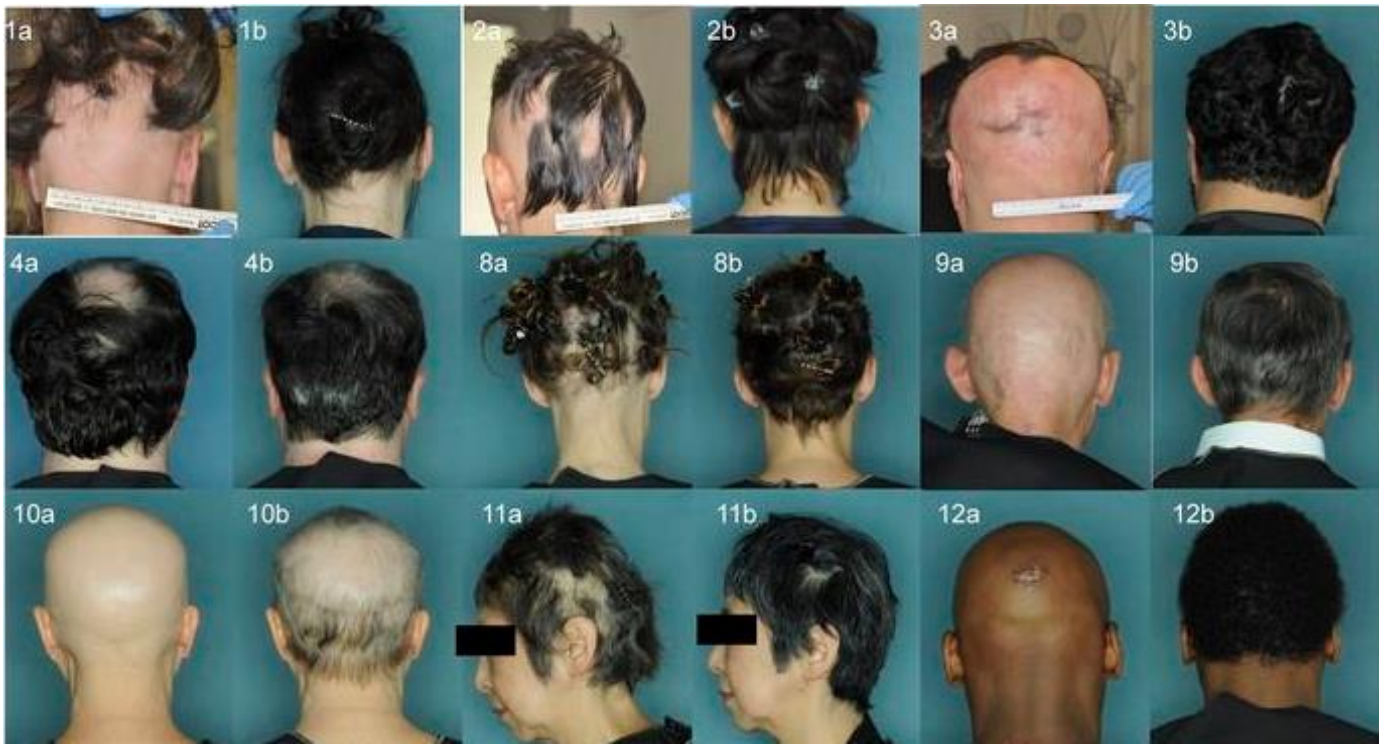
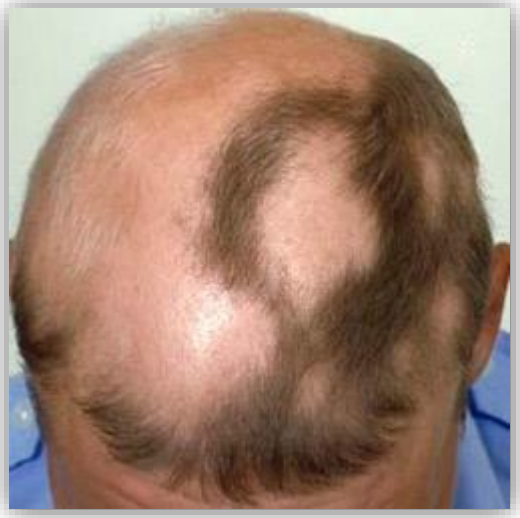
- Open label pilot study of 20 mg BID ruxolitinib in alopecia areata patients
- 9/12 patients (75%) achieved 50%+ regrowth by end of treatment (3-6 months)
  - Responders averaged 92% regrowth by end of treatment
  - <12% reported historical spontaneous remission
- Treatment was generally well-tolerated with no serious adverse events



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

- Phase 2a trial design
  - Double-blind, randomized, placebo-controlled
  - Approximately 90 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 two doses of CTP-543 (4 and 8 mg twice daily) or placebo for 24 weeks
  - Topline data expected Q4 2018



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 limited by renal safety concerns
- Deuterium improves safety profile and exposure in preclinical studies
- Phase 1 initiation expected by year-end 2018

Schizophrenia: Prevalent, Chronic, Severe Mental Disorder

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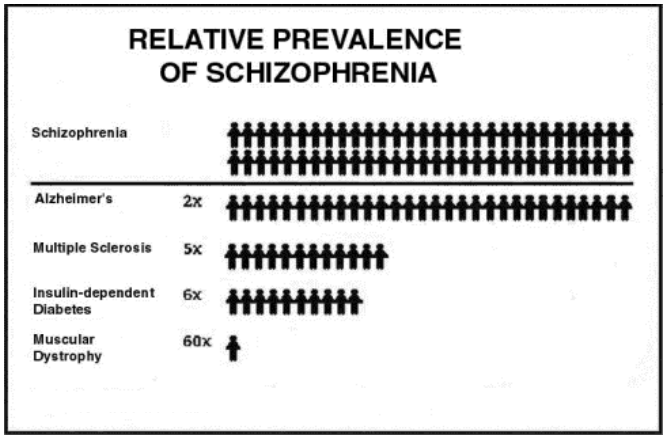
- Afflicts ~1% of the worldwide population
  - Chronic condition affecting both men and women equally
- Disease characterized by multiple symptoms including
  - Positive symptoms – hallucinations, delusional behaviors and thought disorder
  - Negative symptoms - social withdrawal, flattened affect and poverty of speech
  - Cognitive dysfunction - diminished capacity for attention, working memory, and executive function
- Positive symptoms can often be controlled with existing medications, but negative symptoms and cognitive dysfunction are generally poorly responsive to current drugs and lead to poor outcome for patients



Source: www.schizophrenia.com

NMDA Function: Distinct Mechanism to Address Difficult to Treat Symptoms of Schizophrenia

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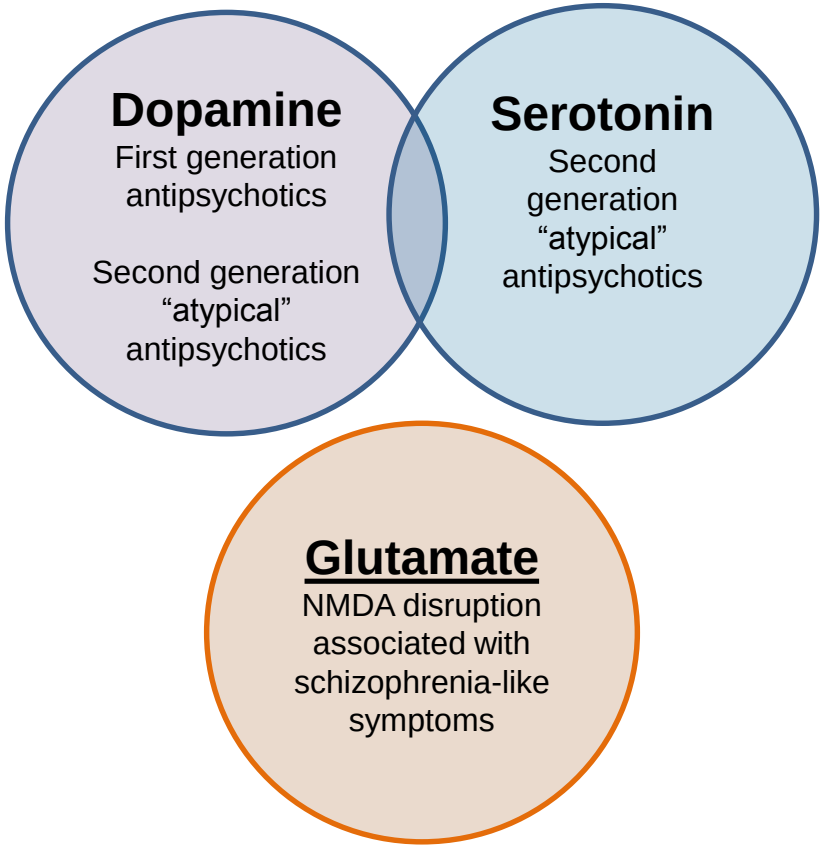

- D-serine is the most important human NMDA synaptic co-agonist
- In schizophrenia poor formation or increased D-serine clearance may result in NMDA hypofunction
- Preclinical models demonstrate oral dosing of D-serine increases its concentrations in relevant areas of the brain

D-Serine Provides Dose-Dependent Benefits in Schizophrenia

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- NMDA receptors are important to affect, memory, and cognition
  - NMDA hypofunction believed to be associated with schizophrenia
  - Pro-psychotic effects of PCP and ketamine support mechanism

- D-serine levels in plasma and CSF are decreased in patients with schizophrenia[1]
- In multiple studies, ~2 g (30 mg/kg) or greater per day of D-serine demonstrated efficacy, particularly on negative symptoms[2]
  - Higher doses of D-serine (≥ 60 mg/kg) appear more effective, producing improvements in positive and negative symptoms and cognition[3]
  - The clinical development of D-serine has been limited as a result of renal safety concerns

[1]Cho S-E et al, Neurosci Lett 2016, 634: 42; Bendikov I, Schizophrenia Res 2007, 90: 41
[2]Singh SP and Singh V, CNS Drugs 2011, 25: 859
[3]Kantrowitz JT et al, Schizophrenia Res 2010, 121: 125; Kantrowitz JT et al, Lancet Psychiatry 2015, 2: 403

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

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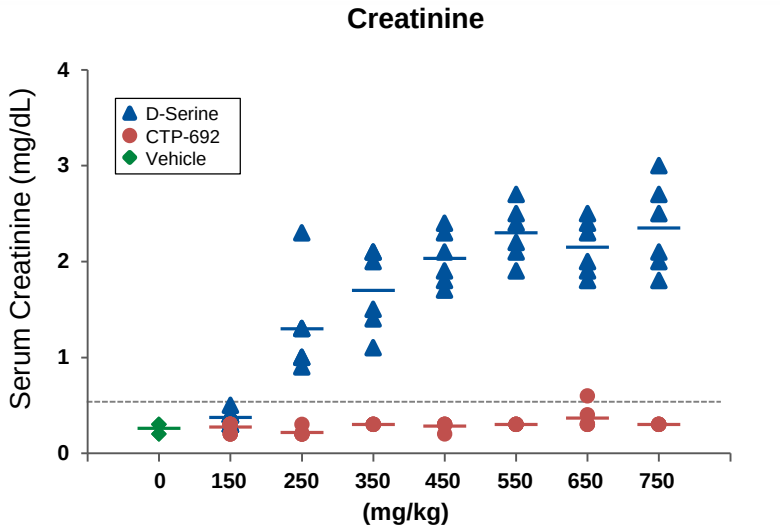
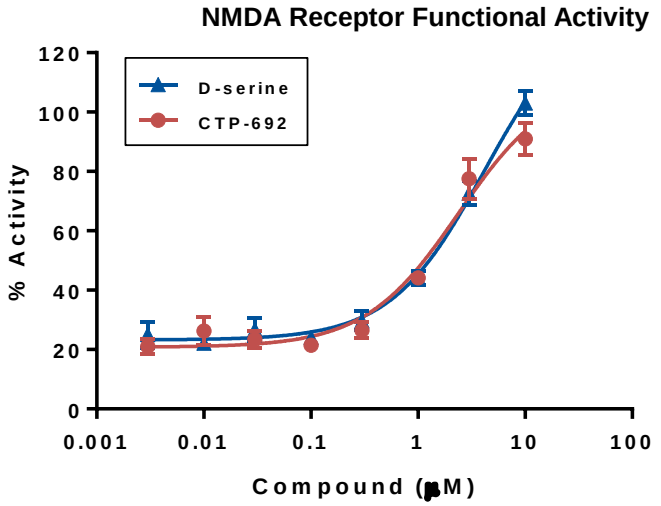
- D-serine is well-known to cause nephrotoxicity in preclinical testing
- CTP-692 appears to markedly decrease preclinical renal toxicity
- Deuterium modification increases D-serine oral exposure (C_{max}, AUC, $T_{1/2}$)
- CTP-692 may achieve therapeutically effective drug levels with significantly lower risk of renal impairment
- Phase 1 testing expected to begin by year-end 2018



*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

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- Estimated 5.3M Americans have Alzheimer's disease; approximately 50% of patients experience agitation
  - No currently approved therapies
- Phase 3 trials underway for blockbuster indication
  - Completion of two U.S. trials projected for 2019 and expected to support registration
- 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

First Quarter 2018 Financial Results

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Q1 2018	
Cash and Investments	$191.0 M
Revenue	$10.5 M
Operating Expenses	$14.3 M
Shares outstanding	23.4 M

- Cash is expected to fund the Company into 2021

Multiple Potential Value Creating Events

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Upcoming Development Milestones

- CTP-543
  - Complete Phase 2a alopecia areata trial (2H 2018)
  - Report Phase 2a alopecia areata topline data (Q4 2018)
- Pipeline Expansion
  - ✓ Announce new neuropsychiatry candidate (Q1 2018)
  - Initiate CTP-692 Phase 1 trial (2018)
- AVP-786
  - Avanir expected to complete first Phase 3 Alzheimer's agitation trial (4/2019)

DCE Platform® Drives Blockbuster Potential

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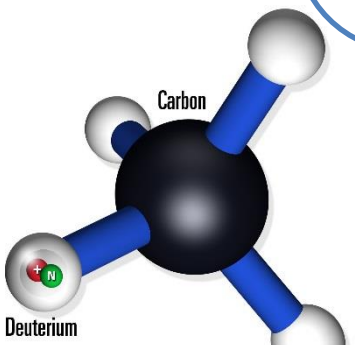

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