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Creating New Possibilities for Patients to Live Their Lives

August 2021

Forward-Looking Statements

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Any statements in this presentation about our future expectations, plans and prospects, including, among others, statements about our expectations regarding the development of CTP-543, the timing of availability of clinical trial data and the timing of regulatory filings, 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, timing and design of future clinical trials, the availability and timing of data from ongoing and future clinical trials and the results of such 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.

Concert-at-a-Glance

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Late-Stage Development Organization with Potential Best-in-Class Clinical Asset

Opportunity to address important unmet need in autoimmune disorders

- CTP-543 selective JAK 1/2 inhibitor for alopecia areata
- CTP-543 NDA filing projected early 2023

Pipeline Snapshot:
Late-Stage Clinical Program and Strategic Value Creation

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Owner	Product Candidate	Phase 1	Phase 2	Phase 3	Anticipated Milestones
Wholly Owned By: CoNCERT Pharmaceuticals Inc.	**CTP-543** (Deuruxolitinib) Alopecia Areata	THRIVE-AA1 Ongoing THRIVE-AA2 Ongoing NDA Supporting Studies/OLE			**NDA submission planned for early 2023**
Partnered With: Otsuka / AVANIR pharmaceuticals	**AVP-786** (Deudextromethorphan hydrobromide and quinidine sulfate) Alzheimer's Agitation/Schizophrenia	Multiple Trials Ongoing *Otsuka/Avanir investing in multiple Phase 3 trials*			**Potential Economics to Concert:** • $37M in regulatory and launch milestones • $125M in sales milestones • Royalties (mid-single to low double digits)

Alopecia Areata: A Devasting Autoimmune Disease

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Common Disorder

- Approximately 1 million patients affected with alopecia areata in the U.S. at any given time[1]
- Estimated 40+% of patients reported to have ≥ 50% loss of scalp hair[1]
- Chronic condition affecting women, men and children of all ages

Significant Burden of Disease

- Disease profoundly impacts patients
- Patients suffer increased burdens, including significant psychosocial impact[2]
  - Major impact on self esteem and self confidence
- Associated with anxiety, depression and other autoimmune conditions

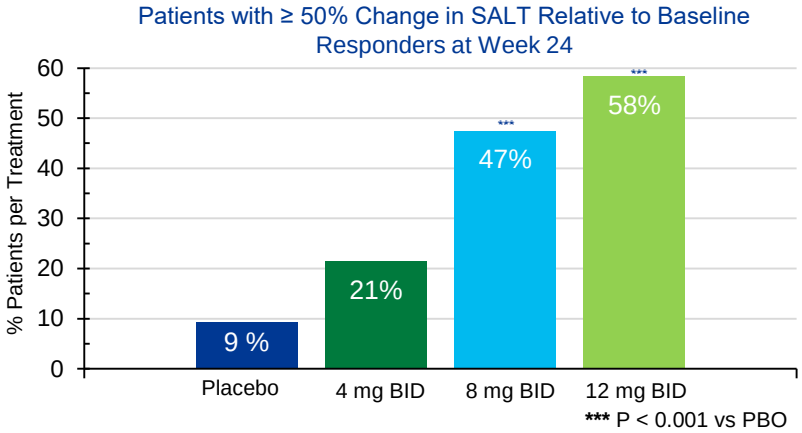
No FDA-approved Treatment Options

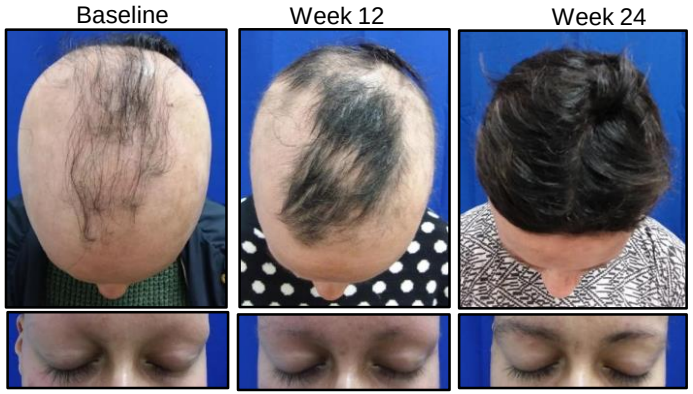
- Strong patient advocacy
- FDA PFDDI meeting held September 2017
- CTP-543 potentially among the first drugs approved for AA
  - Breakthrough Therapy designation granted by FDA

[1] Est. 700,000-1,600,000 patients: Benigno M. Clinical, Cosmetic and Investigational Dermatology 2020
[2] Mesinkovska N. Journal of Investigative Disease 2020

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





CTP-543 Response Over Treatment Period: 8 mg BID

CTP-543 Response Over Treatment Period: 12 mg BID

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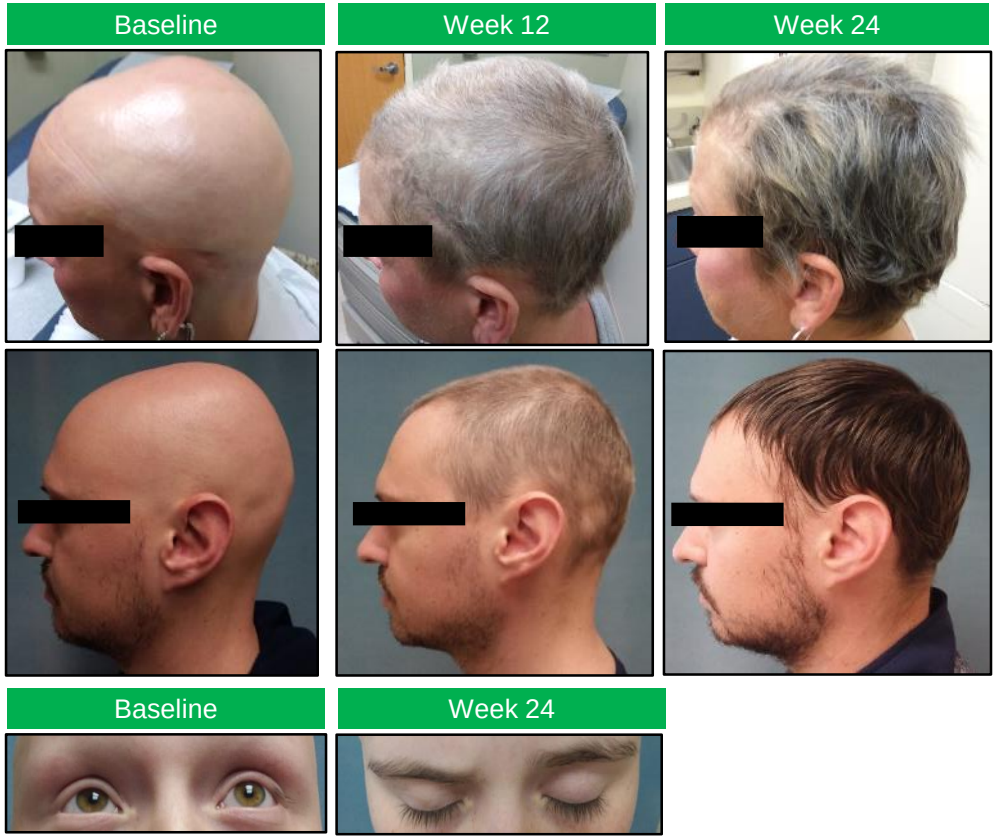
- Good correlation between patient- and clinician-rated impression of improvement
- Patient Global Impression of Improvement scale (PGI-I): 78% (12 mg BID) and 58% (8 mg BID) of patients rated themselves “much improved” or “very much improved” at Week 24
- Clinician Global Impression of Improvement scale (CGI-I): 75% (12 mg BID) and 61% (8 mg BID) of clinicians rated patients as “much improved” or “very much improved” at Week 24



Summary of CTP-543 Phase 2 Dose Ranging Safety Profile

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- CTP-543 treatment was generally well tolerated
  - Large majority of eligible patients rolled into open label, long-term extension study
- 5 AE-related discontinuations in the trial: 3 in placebo group and 2 in 8 mg dose group
- Most common (≥10%) side effects in the 8 mg or 12 mg CTP-543 dose groups were:

	Placebo	4 mg	8 mg	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%)
LDL increase	0	0	4 (10.5%)	0

- One serious adverse event was reported in the 12 mg dose group, possibly related to treatment (Facial Cellulitis)
  - After a brief dosing interruption, treatment was continued and patient completed trial

Study: CP543.2001; NCT03137381

CTP-543 (12 mg BID): Open Label, Long-Term Extension Study Maintains Hair Regrowth Beyond Initial 24-Week Treatment

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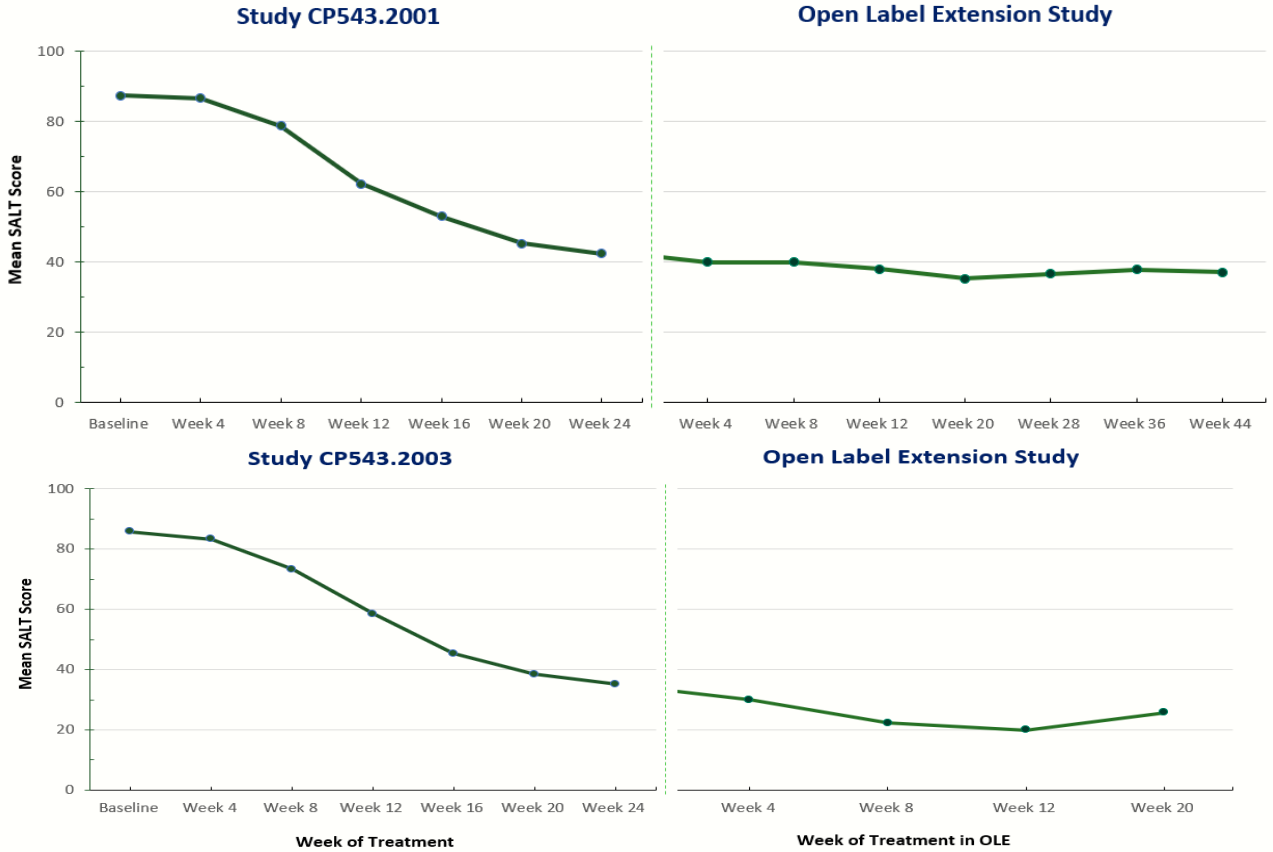

Results presented at Late Breaking News Session: European Academy of Dermatology and Venereology Virtual Congress, October 2020

CTP-543 Phase 3 THRIVE-AA Program Enrolling Patients

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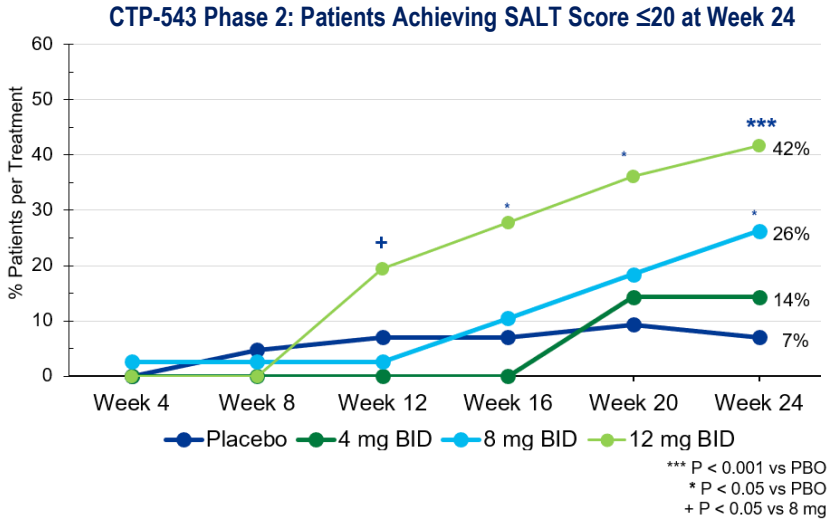
THRIVE-AA program design is consistent with Phase 2 trial to support registration
Potential best-in-class oral treatment for alopecia areata based on Phase 2 results

Design	THRIVE Two double-blind, randomized, placebo-controlled trials in patients with moderate to severe alopecia areata Combined enrollment: ~1,140 patients age 18-65 years with ≥ 50% hair loss 8 mg BID or 12 mg BID or placebo for 24 weeks Sites: United States, Canada and Europe
Endpoint	Primary endpoint is SALT score ≤20 Secondary endpoints include patient and clinician impression scores, PRO measures and regrowth of eyebrows and eyelashes



At Week 24, 8 mg BID and 12 mg BID significantly different from placebo on percent of patients achieving a clinically-meaningful SALT score ≤ 20

Enhancing Value: Capital Efficiency and Strategic Agreements

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Financial Position (Q2 2021)

- Cash: $122.4M
- Shares outstanding: 32.2M

Strong Validation of Platform

- VX-561 (CTP-656) asset sale; $160M upfront
- $32M realized from milestone monetization in May 2021

VERTEX

Successful Out-Licensing

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

Otsuka AVANIR pharmaceuticals Cipla Jazz Pharmaceuticals Processa

Planned CTP-543 Clinical and Regulatory Advancements

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CTP-543 for Alopecia Areata

- THRIVE-AA1 and THRIVE-AA2 Phase 3 enrollment underway
- Next anticipated milestone: THRIVE-AA1 results expected in first half of 2022
- Expect positive results from THRIVE-AA program to support NDA filing in early 2023

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NASDAQ: CNCE
www.concertpharma.com
@ConcertPharma

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