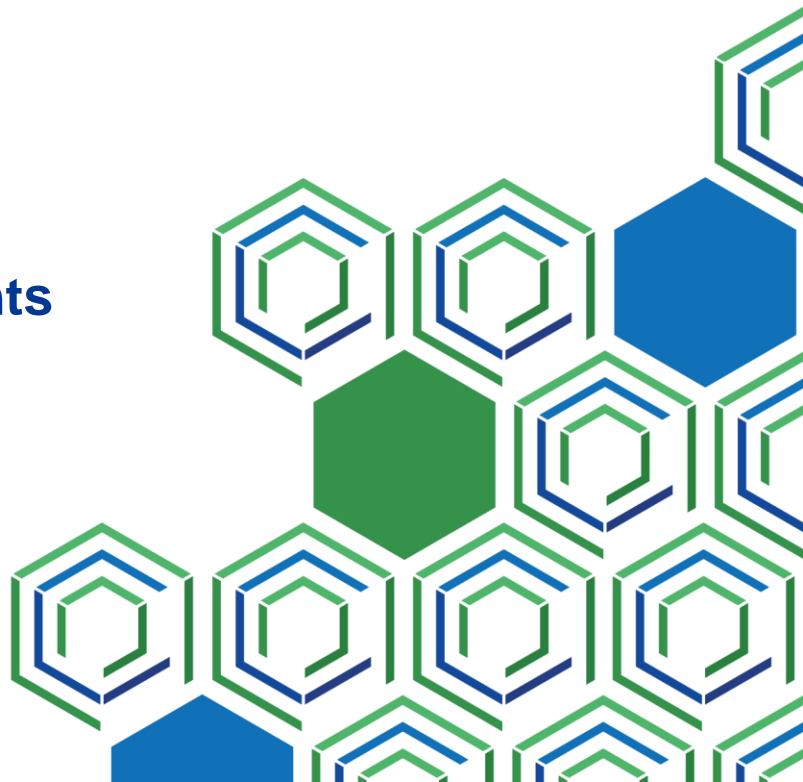


# CoNCERT

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

March 2022



## 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 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. 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

## 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
- Two fully-enrolled Phase 3 trials with >1,200 total patients
- CTP-543 NDA filing projected 1H 2023



Creating new  
possibilities for  
**PATIENTS TO LIVE  
THEIR LIVES**

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

		Phase 1	Phase 2	Phase 3	Anticipated Milestones
<b>CTP-543</b> Deuruxolitinib  	Alopecia Aretata	  			Phase 3 topline data expected in 2022  NDA submission planned 1H 2023
<b>AVP-786*</b> Deudextromethorphan hydrobromide and quinidine sulfate  	Alzheimer's Agitation  Schizophrenia Negative Symptoms		  		Next trial completion expected in 2H 2022

\*Potential economics to Concert: \$37M in regulatory milestones | \$125M in sales milestones | Royalties

# Alopecia Areata: A Devastating Autoimmune Disease

## Common Disorder



- Up to approximately 1.5 million patients affected with alopecia areata in the U.S. at any given time<sup>1</sup>
- Estimated 40+% of patients reported to have  $\geq 50\%$  loss of scalp hair<sup>1</sup>
- 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<sup>2</sup>
  - Major impact on self esteem and self confidence
- Associated with anxiety, depression and other autoimmune conditions

## No FDA-approved Treatment Options



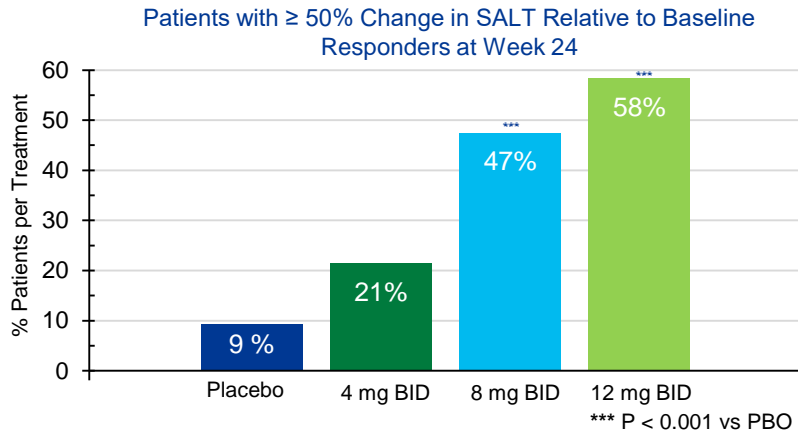
- Strong patient advocacy
- FDA PFDDI meeting held September 2017
- CTP-543 potentially among the first drugs approved for AA
  - Breakthrough Therapy designation granted by FDA

<sup>1</sup> Est. 700,000-1,600,000 patients: Benigno M. *Clinical, Cosmetic and Investigational Dermatology* 2020

<sup>2</sup> Mesinkovska N. *Journal of Investigative Dermatology* 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
  - 8 mg responders average 78% SALT improvement
  - 12 mg responders average 86% SALT improvement



CTP-543 Response Over Treatment Period: 8 mg BID

# Patients and Clinicians Both Reported Great Improvement With CTP-543

- 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

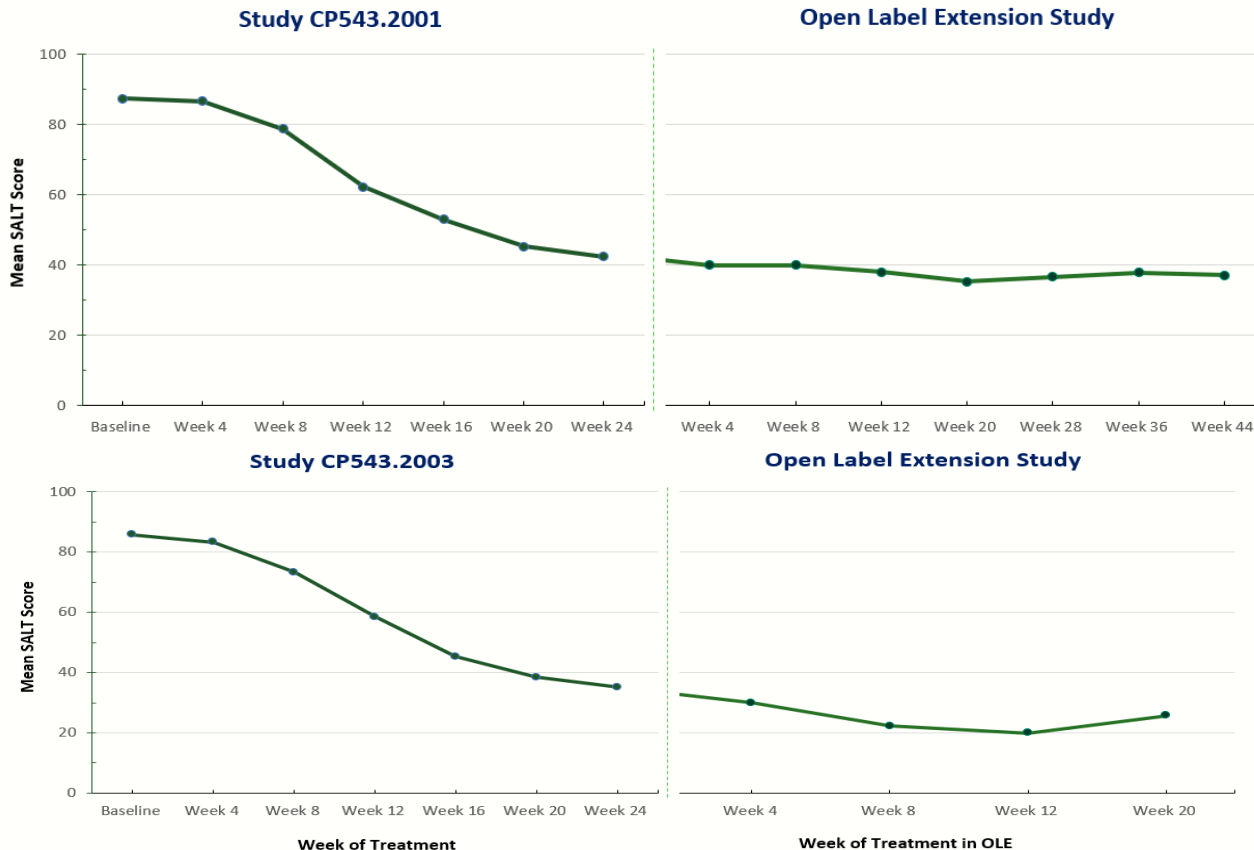
- CTP-543 treatment was generally well tolerated
  - Large majority of eligible patients rolled into open label, long-term extension study
- 5 AE-related discontinuations in the trial: 3 in placebo group and 2 in 8 mg dose group
- Most common ( $\geq 10\%$ ) side effects in the 8 mg or 12 mg CTP-543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



# CTP-543 (12 mg BID) Open Label, Long-Term Extension Study: Maintains/Improves Hair Regrowth Beyond 24 Weeks



# CTP-543 Phase 3 THRIVE-AA Program to Support Planned NDA Submission in First Half of 2023

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



Two double-blind, randomized, placebo-controlled trials in patients with moderate to severe alopecia areata

Combined enrollment: ~1,200 patients age 18-65 years with  $\geq 50\%$  hair loss

8 mg BID or 12 mg BID or placebo for 24 weeks

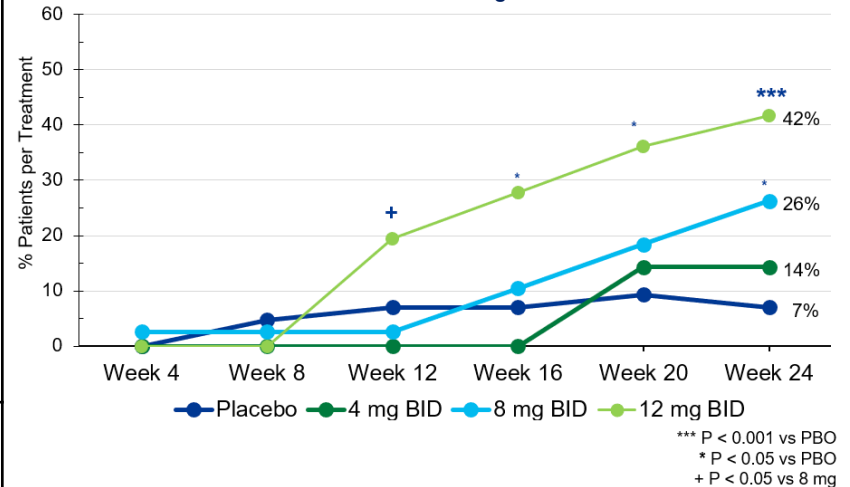
Sites: United States, Canada and Europe

## Endpoint

Primary endpoint is SALT score  $\leq 20$

Secondary endpoints include patient and clinician impression scores, PRO measures and regrowth of eyebrows and eyelashes

CTP-543 Phase 2: Patients Achieving SALT Score  $\leq 20$  at Week 24



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

# Enhancing Value: Capital Efficiency and Strategic Agreements <sup>Co</sup>NCERT

## Financial Position (Q4 2021)

- Cash: \$141.6M
- Potential to receive additional \$103.1M under financing arrangement\*

## Strong Validation of Platform

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



## Successful Out-Licensing

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



\*Announced an agreement with BVF Partners L.P. and RA Capital Management to raise gross proceeds of \$65 million on November 4, 2021; Concert has the potential to receive an additional \$103.1 million upon the full exercise of the warrants issued in connection with the November 2021 financing.

## Creating new possibilities for patients to live their lives



### CTP-543: Potential Best-in-Class for Alopecia Areata

- THRIVE-AA1 Phase 3 trial fully enrolled; Topline results expected in Q2 2022
- THRIVE-AA2 Phase 3 trial fully enrolled; Topline results expected in Q3 2022
- Expect positive results from THRIVE-AA program to support NDA filing in 1H 2023

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NASDAQ: CNCE

[www.concertpharma.com](http://www.concertpharma.com)

@ConcertPharma

**For additional information contact:**

Justine Koenigsberg

[ir@concertpharma.com](mailto:ir@concertpharma.com)

