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Creating New Possibilities for Patients to Live Their Lives

February 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 progress of clinical development of CTP-543, the timing of availability of clinical trial data, the timing of initiation and design of future clinical trials 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

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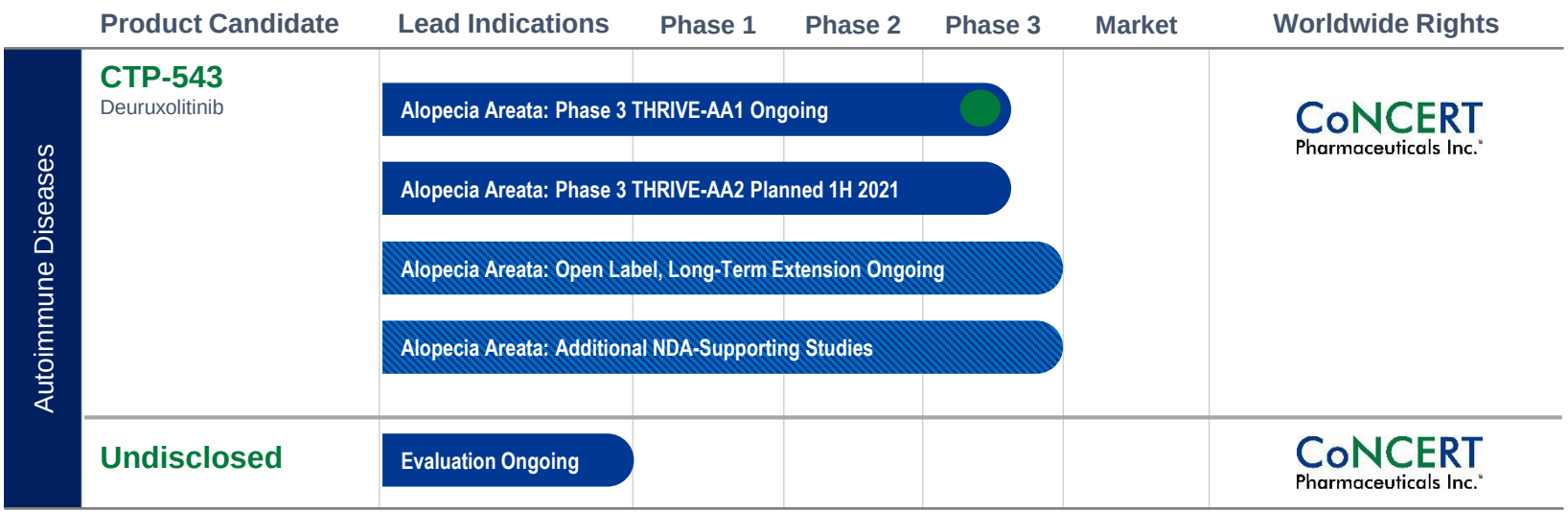
Late-Stage Development Organization with Potential Best-in-Class Clinical Asset

Opportunity to address important unmet need in autoimmune disorders

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

Evaluation of additional pipeline candidates ongoing

Late-Stage Clinical Asset Advancing In Phase 3 Evaluation



- Robust clinical program evaluating CTP-543 as an oral medicine for the treatment of alopecia areata
- Positive results from THRIVE-AA Phase 3 program to support NDA submission in early 2023
- Potential blockbuster opportunity

Alopecia Areata: A Devasting Autoimmune Disease

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Common Disorder

- Approximately 700,000 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

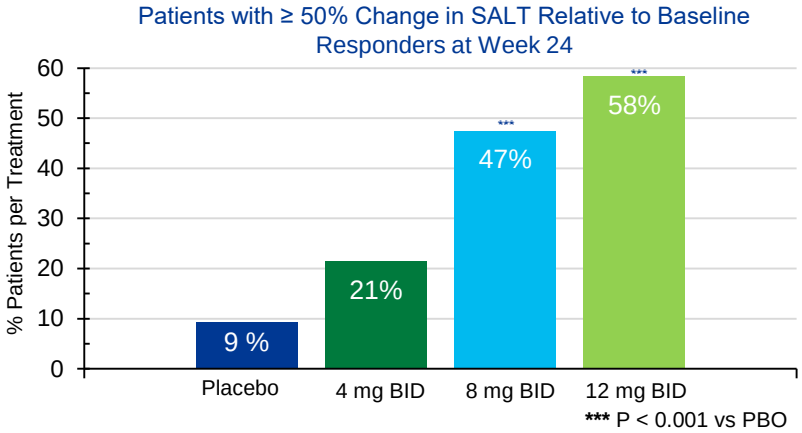
No FDA-approved Treatment Options

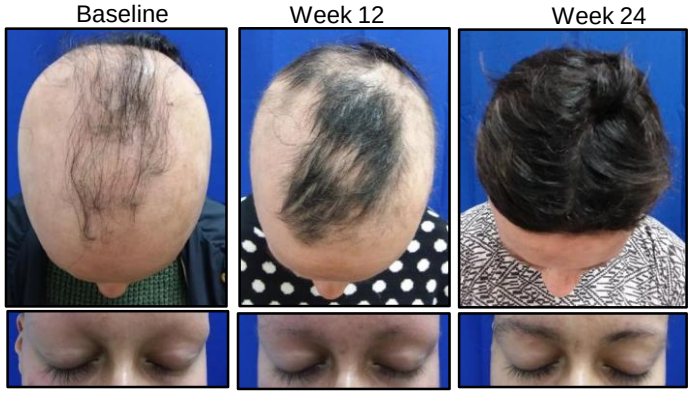
- Strong patient advocacy
- FDA PFDDI meeting held September 2017
- CTP-543 potentially among the first drugs approved for AA
  - Breakthrough Therapy designation granted by FDA

[1] 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
  - 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





CTP-543 Response Over Treatment Period: 8 mg BID

CTP-543 Response Over Treatment Period: 12 mg BID

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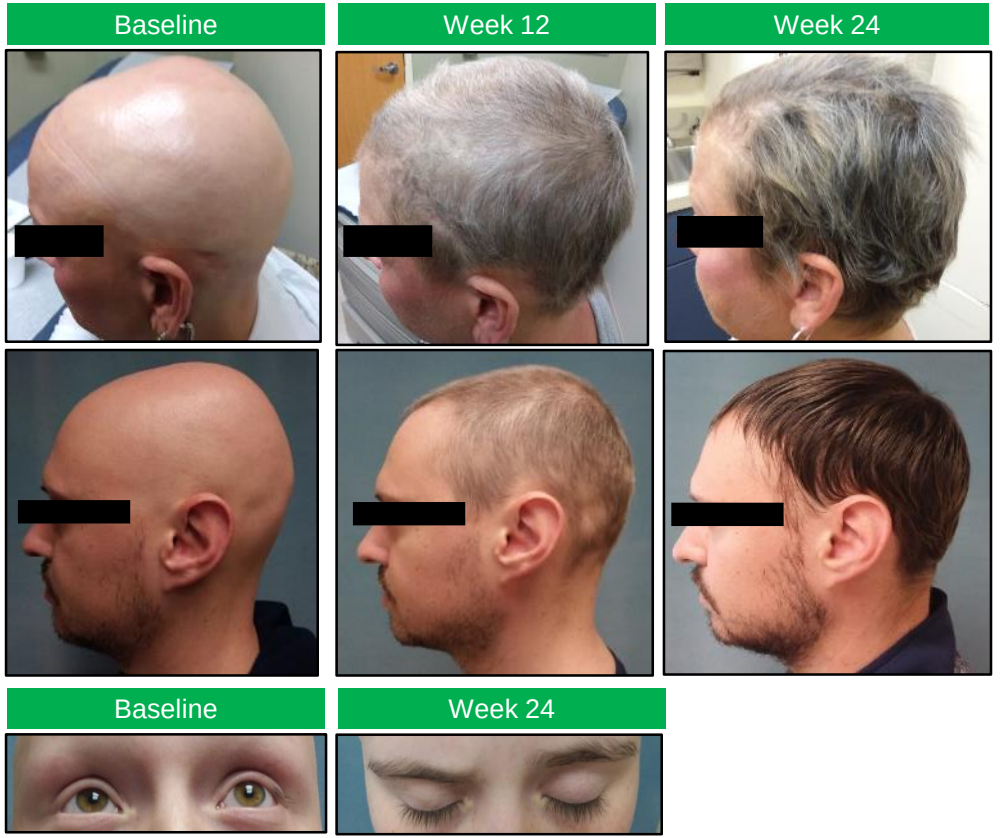
- Good correlation between patient- and clinician-rated impression of improvement
- Patient Global Impression of Improvement scale (PGI-I): 78% (12 mg BID) and 58% (8 mg BID) of patients rated themselves “much improved” or “very much improved” at Week 24
- Clinician Global Impression of Improvement scale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

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

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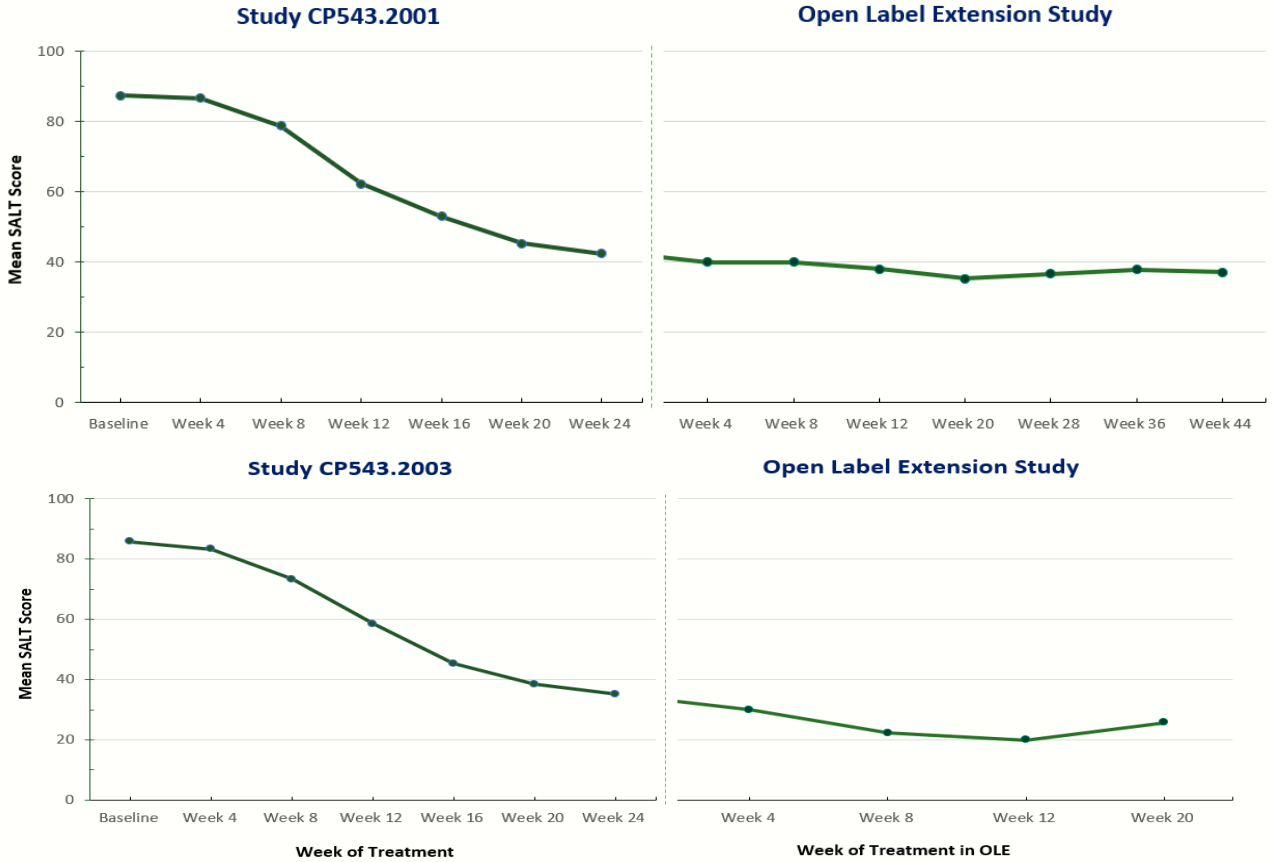

Results presented at Late Breaking News Session: European Academy of Dermatology and Venereology Virtual Congress, October 2020

CTP-543 Phase 3 THRIVE-AA1 Enrolling Patients

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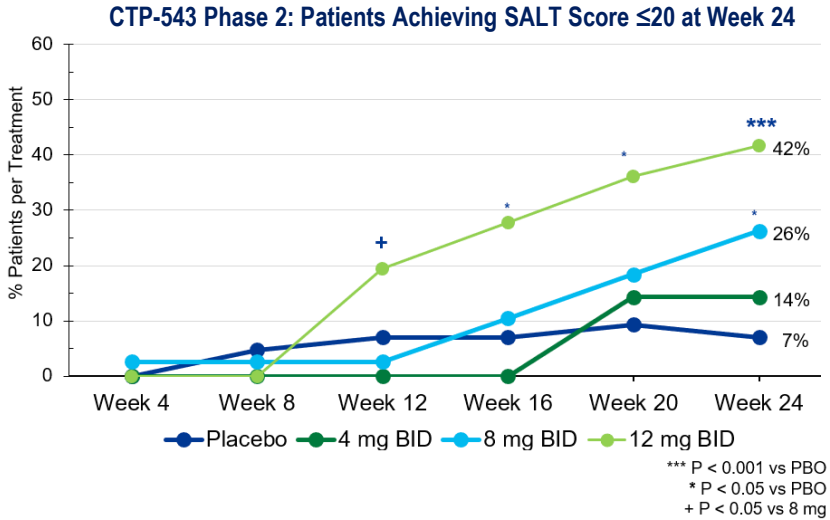
THRIVE-AA1 design is consistent with Phase 2 trial to support registration
Potential best-in-class oral treatment for alopecia areata based on Phase 2 results

Design	**THRIVE-AA1** Phase 3 study of CTP-543 in adults with moderate to severe alopecia areata Double-blind, randomized, placebo-controlled trial in patients with moderate to severe alopecia areata Approximately 700 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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Strong Financial Position (Q4 2020)

- Cash: $130.0 M
- Shares outstanding: 31.9 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 · Cipla · Jazz Pharmaceuticals · Processa

Planned CTP-543 Clinical and Regulatory Advancements

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CTP-543 for Alopecia Areata

- THRIVE-AA1 Phase 3 enrollment underway; Data expected in 2022
- Next anticipated milestone: Initiate THRIVE-AA2 Phase 3 study in 1H 2021
- Expect positive results from THRIVE-AA program to support NDA filing in early 2023

*Milestones pending impact of COVID-19 on development activities

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