# **Castle Creek Pharmaceuticals**

Dermatology Summit Entrepreneurial Showcase 2018

Michael Derby, CEO January 7, 2018

#### CASTLE CREEK PHARMA

### Castle Creek Pharma Driving innovation in dermatology

We are a high-growth biotech company focused on identifying, developing and commercializing innovative drugs that address high unmet medical needs in dermatology



- Rare genetic orphan dermatology with potential first ever drug approvals
- Broad dermatology with differentiated products



- Ongoing Phase 3 for high potential rare dermatologic condition (epidermolysis bullosa simplex) with no approved treatments and significant unmet need
- Potentially disease modifying therapy with \$1bn+ peak sales potential

# Our Dermatology Development Pipeline High potential candidates targeting multiple indications



	2018	2019	2020	2021	2022	Est Peak Sales
Orphan Dermatology						
CCP-020 (topical diacerein)						
Epidermolysis bullosa simplex (EBS)	Phase 3	🔵 NDA Filing				\$1bn +
CCP-060						
Rare genetic dermatologic condition	Preclinical		Phase 3			\$300mm +
CCP-070						
Rare genetic dermatologic condition	Preclinical	Phase 1/2	2	Phase 3		\$100mm
Total Orphan Derm						\$1.4bn +
Broad Dermatology						
CCP-050						
Atopic dermatitis (AD)	Phase 2	Pha	se 3			\$500mm +
CCP-043						
Severe acne	Preclinical	Phase 1/2	P	hase 3		\$500mm +
ССР-070						
Vitiligo	Preclinical	Phase	e 1/2	Phase	3	\$1bn +
Total Broad Derm						\$2bn +
Total Derm						\$3.4bn +



# **Our Leadership Team** Deep expertise in dermatology and rare disease



McKinsey & Co., Proteostasis, Amicus, Domain Associates, Medtronic

Chief Development Officer of Viamet, Senior Executive of Topica, Merck & Co., Schering Plough, Novartis, Colgate Palmolive, University of Michigan

President of LEO Pharma, Senior Executive of Sanofi Genzyme, Abbott Laboratories

LEO Pharma, Alpharma



Amir Tavakkol, PhD EVP and Chief Development Officer

John Koconis EVP and Chief Commercial Officer

**Regina Donohue** Vice President, Human Resources

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A Strong Investor Base Industry leaders with proven records of success







CAPITAL GROUP

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#### **❖**July 2015

 Initial investment from Paragon Biosciences and Valor

#### September 2016

• Series A Financing of \$48 million from Fidelity Investments

### Understanding Epidermolysis Bullosa The worst disease you've never heard of



Epidermolysis bullosa (EB) is a rare genetic connective tissue disorder that affects approximately 1 in 20,000-50,000 births. There are no FDA-approved therapies for any type of EB.



- EB manifests as fragile skin causing local to widespread blistering
- Estimated 20,000 patients (many children) in the U.S. and >500,000 worldwide
- Estimated 70%-90% of EB patients have EB Simplex (EBS)



- Current treatment focuses on symptoms, such as itching, pain and wound care
- Our investigational medicine, CCP-020, is a potentially disease modifying therapy to reduce blister formation in EBS patients

# Understanding Epidermolysis Bullosa Simplex Aberrant IL-1 $\beta$ signaling in disease pathogenesis





• IL-1 $\beta$  activates the inflamma some assembly

- Upregulation of pro-inflammatory cytokines
- Generation of reactive oxygen species
- Aggregation of mutant keratin protein
- ✤ Diacerein blocks aberrant IL-1 β signaling
  - Prevents inflammatory cascade and protects keratinocytes

- Mutations in keratin 5 or 14 in the basal keratinocytes of EBS patients disrupt cellular integrity during physical stress.
- IL-1β is over-expressed in EBS patients, leading to the hypothesis that IL-1β may mediate the EBS phenotype.





### Evaluating topical diacerein in EBS An overview of the Phase 2 study design

Title	Phase 2 Study: Topical diacerein for the treatment of EBS		
Primary Objective	Reduction in blister number by 40% in treated skin area (3% body surface area) vs. placebo at 4 weeks		
Secondary Objective	Time to return to initial blister number (+/- 10%) during follow up		
Study Design	Placebo controlled, randomized, double blinded Part 1: intervention Part 2: follow up Cross-over design		
Study Population	Generalized-severe EBS Mutations in K5 or K14 Age 4-19		
Patient Number	17		
Therapy	Once daily self application		
Time Schedule	Intervention phase: 4 weeks Follow up phase: 12 weeks		

Evaluating topical diacerein in EBS Phase 2 study results



Reduction of blister numbers by 40% on treated skin areas compared to placebo after 4 weeks



Highly significant difference in responders (defined as >40% reduction in blister number) between treatment and placebo groups at 4 weeks, with continued improvement persisting out to 4 months.



### CCP-020 (diacerein 1% ointment) in EBS Phase 3 study (DELIVERS trial) design

Title	Phase 3 Study: CCP-020 for the treatment of EBS		
Primary Objective	Blister surface area improvement of at least 40% vs. placebo at 16 weeks		
Secondary Objectives	Investigator Global Assessment (IGA) Patient reported outcomes (pain, itch) Adverse events		
Study Design	Placebo controlled, randomized, double blinded, multi-center global trial Intervention and follow up Parallel group design		
Study Population	Genetically confirmed diagnosis of EBS Level 3/4 on IGA scale of disease severity Age 4+		
Patient Number	80		
Therapy	Once daily self application		
Time Schedule	Intervention phase: 8 weeks Follow up phase: 8 weeks		





- Completion of DELIVERS trial, targeted Q4 2018
- Advancement of each of our additional pipeline products into mid/late stage clinical development
- Completion of sale or partnership of non-strategic assets
- Potential private financing round to support advancement of pipeline and other corporate objectives

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