Castle Creek Pharmaceuticals Announces Late-Breaking Results at EADV of Phase 2 Topical Diacerein 1% Data in Patients with Epidermolysis Bullosa Simplex (EBS)

Topical diacerein 1% meets primary efficacy endpoint in study of patients with EBS

VIENNA -- October 1, 2016 -- Castle Creek Pharmaceuticals (CCP) today announced the presentation of final results from a Phase 2 study evaluating the efficacy and safety of topical diacerein 1%, an inhibitor of interleukin-1β (IL-1β), compared with placebo in patients with Epidermolysis Bullosa Simplex (EBS).

The data were presented at the 25th Annual Congress of the European Academy of Dermatology and Venereology (EADV) in Vienna, Austria[1] by Professor Johann Bauer, M.D., MBA HCM; Head of the University Clinic for Dermatology of the SALK/Paracelsus Medical University and the principal investigator in the trial.

"We are very pleased to see positive results from this important Phase 2 trial, which is the first of its type for EBS patients, who are an underserved population," said Dr. Greg Licholai, President and Chief Scientific Officer at CCP. "EBS is a debilitating disease, and this represents a significant step forward in developing a treatment for patients suffering from this condition, which has a very high unmet medical need. Based on these data and additional findings from our ongoing studies, CCP plans to initiate a clinical registration trial of topical diacerein 1% by the end of the year."

Data from the completed multicenter, randomized, double-blind, placebo-controlled Phase 2 trial were presented from 17 patients with EBS that were treated for a four-week period followed by a three-month follow-up phase in two subsequent years, with a crossover after the first year. The patient age range was 4-12 years with approximately equal numbers of males and females.

- 60% of patients in the topical diacerein 1% treatment group achieved the primary end point (at least a 40% reduction in the number of blisters) at four weeks. Only 15% of placebo patients showed a similar benefit.
- Continued benefit of topical diacerein 1% was also demonstrated after cessation of treatment through the follow up period of 12 weeks.
- Topical diacerein 1% was well tolerated by patients with no treatment-related adverse events reported.

Dr. Bauer stated, "We are happy that this study confirms our initial experimental results and Phase 1 data. The superior efficacy of diacerein coupled with an excellent tolerability profile may indicate a potential new and safe approach for treating patients with EBS."

About the Phase 2 Trial of Topical Diacerein 1% for the Treatment of Patients with Epidermolysis Bullosa Simplex

This was a Phase 2 randomized, placebo-controlled, double-blind multicenter clinical trial. The primary objective was reduction of blister numbers by 40% in the treated skin area (3% of body surface) compared with placebo after 4 weeks**.** The study involved a crossover design over a two year period, and included an intervention phase for four weeks followed by a three-month follow up. The study population was EBS-generalized/severe with K14 or K5 gene mutations, and patients aged 4-19 years. Therapy was a once-daily, self-application for four weeks covering 3% total body surface area (BSA).

About Epidermolysis Bullosa Simplex

Epidermolysis Bullosa Simplex (EBS) is a rare, inherited skin disease for which there is no cure[2]**.** EBS is characterized by mild to severe blistering on the skin, beginning in infancy and continuing into adulthood. Currently, only supportive care is used in the management of EBS that may include wound care, use of bandages and pain management. The etiology of EBS is a defect in basal keratinocytes due to genetic mutations, which produce an abnormal and fragile form of the structural cellular element keratin.

About Diacerein

Diacerein is the prodrug of rhein, a small molecule that blocks the activity of Interleukin-1β (IL-1β) by inhibiting plasma membrane-bound IL-1 converting enzyme[3]. IL-1β is a pro-inflammatory cytokine that has been demonstrated to be upregulated in EBS[3]. Diacerein has been studied previously in patients with EBS in a Phase 1 study[4].

About Castle Creek Pharmaceuticals

Castle Creek Pharmaceuticals (CCP) is a high-growth biopharmaceutical company that has rapidly assembled a robust and diversified late-stage pipeline of products backed by strong science and focused on meeting patient needs in the treatment of rare and debilitating dermatologic and head and neck conditions.

Footnotes

1. Wally, W., Hovnanian, A., Ly, J., Bukova, H., Brunner, V., Lettner, T., Ablinger, M., Feldner, T., Hofbauer, P., Wolkersdorfer, M., Lagner, F., Hitzl, W., Bauer, J., Diacerein for the treatment of epidermolysis bullosa – a phase II randomized, placebo-controlled, double-blind multicenter clinical trial, presented as a late-breaking abstract at the European Academy of Dermatology and Venereology 2016. October 1, 2016.
2. Garg A, Levin, N.A and Bernhard, J.D. 2008. Approach to dermatological diagnosis. In Fitzpatrick's dermatology in general medicine. 7th edition. K. Wolf, et al., editors. McGraw-Hill. New York, New York, USA. 23–39.
3. Wally V et.al. J Invest Dermatol 133:1901-1903-2013.
4. Wally, V., et al., Topical diacerein for epidermolysis bullosa: a randomized controlled pilot study. Orphanet Journal of Rare Diseases, 2013. 8: p. 69.

For more information, visit: www.castlecreekpharma.com.

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