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Building quality cancer care together

Helsinn publishes PROVe MF-CTCL study providing new data for mechlorethamine gel use in a real-world setting

Lugano, Switzerland, March 18, 2021 – Helsinn, a Swiss pharmaceutical group focused on building quality cancer care and rare disease products, is pleased to note the publication of results from the PROVe study, providing two year real world data for mechlorethamine gel 0.016% topical use in combination with other therapies, for early stage MF-CTCL.

Mechlorethamine gel 0.016%, also known as chlormethine gel, is approved for use in multiple countries including the US and EU and is marketed under the trade names VALCHLOR® and LEDAGA®. The authorized use for each country varies based on the individual health authority requirements. For more details, please refer to the product information for the respective countries: VALCHLOR® USPI, and LEDAGA® SmPC.

The PROVe study is, to date, the largest open label, single arm, multicenter observational study assessing treatment patterns and efficacy, safety, and health-related quality of life (HRQOL) outcomes in early stage MF-CTCL patients treated with mechlorethamine gel and other therapies in a real-world setting in the US. 298 patients were monitored for up to two years during standard-of-care clinic visits.

Results from the PROVe study, published in the American Journal of Clinical Dermatology, provide additional data that mechlorethamine gel is commonly used in the US clinical practice with other therapies, the most commonly co-administered therapies were corticosteroids, phototherapy, and oral bexarotene. This information provides healthcare professionals flexibility in their approach to treating MF-CTCL with a variety of other skin directed therapies in addition to mechlorethamine gel. It was noted that the topical gel can be applied by the patient or caregiver in the privacy of their own home, offering patients convenient access to a useful therapeutic option. HRQOL data was also collected from patients, providing information based on three subscales: emotions, symptoms and functioning.

Link to publication: *The PROVe Study: US Real-World Experience with Chlormethine/Mechlorethamine Gel in Combination with Other Therapies for Patients with Mycosis*

Fungoides Cutaneous T-Cell Lymphoma - https://link.springer.com/article/10.1007/s40257-021-00591-x

This latest research builds on a growing body of evidence from Helsinn on the use of mechlorethamine gel, with publications recently featured in the journal of Clinical Lymphoma Myeloma and Leukemia and the Journal of Investigative Dermatology.

Ellen J. Kim, M.D. Lead Author, Principal Investigator and Professor of Dermatology at the Hospital of the University of Pennsylvania: "I'm pleased that we are able to publish this important study, the largest of its kind in MF-CTCL patients. It clearly demonstrates the effectiveness of this topical gel, particularly when administered over a sustained period."

William L. Bailey, Helsinn Therapeutics (U.S.) Vice President of Medical & Scientific Affairs, commented: "This valuable study builds on other recent research on the use of mechlorethamine treatment in MF-CTCL patients. These findings underline VALCHLOR®'s suitability for home use, making it an attractive treatment option for both patients and clinicians, as they seek to find treatment options outside the clinical setting."

About VALCHLOR® in the US

INDICATION

VALCHLOR® (mechlorethamine) gel 0.016% is indicated for the topical treatment of Stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma (MF-CTCL) in patients who have received prior skin-directed therapy.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

VALCHLOR® is contraindicated in patients with known severe hypersensitivity to mechlorethamine. Hypersensitivity reactions, including anaphylaxis, have occurred with topical formulations of mechlorethamine.

WARNINGS AND PRECAUTIONS

- Mucosal or eye injury: Exposure of mucous membranes to mechlorethamine such as the oral mucosa or nasal mucosa causes pain, redness, and ulceration, which may be severe. Exposure of the eyes causes pain, burns, inflammation, photophobia, and blurred vision. Blindness and severe irreversible anterior eye injury may occur. Should eye exposure or mucosal contact occur, immediately irrigate for at least 15 minutes with copious amounts of water, followed by immediate medical consultation

- Secondary exposure: Avoid direct skin contact with VALCHLOR® in individuals other than the patients due to risk of dermatitis, mucosal injury, and secondary cancers

- Dermatitis: Dermatitis may be moderately severe or severe. Monitor patients for redness, swelling, inflammation, itchiness, blisters, ulceration, and secondary skin infections. Stop treatment with VALCHLOR® or reduce dose frequency

- Embryo-fetal toxicity: May cause fetal harm. Women should avoid becoming pregnant while using VALCHLOR® due to the potential hazard to the fetus. For nursing mothers, do not breastfeed during treatment with VALCHLOR®

- Non-melanoma skin cancer: Monitor patients during and after treatment with VALCHLOR®

- Flammable gel: VALCHLOR® is an alcohol-based gel. Avoid fire, flame, and smoking until the gel has dried

ADVERSE REACTIONS

The most common adverse reactions (≥5%) were dermatitis (56%), pruritus (20%), bacterial skin infection (11%), skin ulceration or blistering (6%), and hyperpigmentation (5%). These reactions may be moderately severe or severe. Elderly patients aged 65 and older may be more susceptible. Depending on severity, treatment reduction, suspension, or discontinuation may be required.

USE IN SPECIFIC POPULATIONS

- Contraception: Females who are able to become pregnant, and males with female partners who are able to become pregnant, should use a barrier method of contraception to avoid direct exposure of reproductive organs to VALCHLOR®
- Infertility: The reproductive effects of VALCHLOR® have not been studied: however systemically administered mechlorethamine may impair fertility. The reversibility of the effect on fertility is unknown.

DOSING AND APPLICATION

VALCHLOR is for topical dermatologic use only. Apply a thin film of gel once daily to affected areas of the skin. VALCHLOR® is a cytotoxic drug and special handling and disposal procedures should be followed during use. Caregivers must wear disposable nitrile gloves when applying VALCHLOR®. Patients and caregivers must thoroughly wash hands after handling or applying VALCHLOR. ®

To report SUSPECTED ADVERSE REACTIONS, contact Helsinn Therapeutics (U.S.), Inc. at 1-855-482-5245 or FDA at 1-800-FDA-1088 or visit www.fda.gov/medwatch.

Please see the VALCHLOR full Prescribing Information and Medication Guide.

About the Helsinn Group

Helsinn is a privately-owned Swiss Pharma Company which, since 1976, has been improving the lives of patients, guided by core family values of respect, integrity and quality. The Group has an extensive portfolio of marketed innovative cancer and rare disease therapies, a robust drug development pipeline and ambitions to further accelerate its growth through in-licensing and acquisition to address unmet medical needs. Helsinn operates a unique integrated licensing business model, achieving success with long-standing partners in 190 countries, who share our values. The Group's pharmaceutical business, (Helsinn Healthcare) is headquartered in Lugano, Switzerland with operating subsidiaries in the U.S. (Helsinn Therapeutics US) and China (Helsinn Pharmaceuticals China) which market the Group's products directly in these countries. The Group has additional operating subsidiaries in Switzerland (Helsinn Advanced Synthesis, an active pharmaceutical ingredient manufacturer) and Ireland (Helsinn Birex Pharmaceuticals, a drug

product manufacturer). Helsinn Investment Fund was created to enhance the future of healthcare by providing funding and strategic support to innovative companies.

Helsinn Group plays an active and central role in promoting social transformation in favor of people and the environment. Corporate social responsibility is at the heart of everything we do which is reinforced in the company's strategic plan by a commitment to sustainable growth.

To learn more about Helsinn Group please visit www.helsinn.com

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