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Building quality cancer care together

Helsinn announces publication of latest mechlorethamine gel research, highlighting its efficacy and safety

Lugano, Switzerland, February 18, 2021 – Helsinn, a Swiss pharmaceutical group focused on building quality cancer care and rare disease products, is pleased to note two recent journal publications on the use of mechlorethamine gel 0.016% as a topical treatment of early stage mycosis fungoides-type cutaneous T-cell lymphoma ("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 design of the registrational trial and the individual health authority requirements. For more details, please refer to the product information for the respective countries: VALCHLOR® USPI, and LEDAGA® SmPC.

The two publications are based on analyses of the registration study data (NCT168064). The pivotal study assessed the non-inferiority of mechlorethamine gel vs mechlorethamine ointment in early stage MF-CTCL in 260 patients undergoing 12 months of treatment. The first publication: "Evaluating the Treatment Patterns of Chlormethine/Mechlorethamine Gel in Patients with Stage I-IIA Mycosis Fungoides: By-time Reanalysis of a Randomized Controlled Phase 2 Study" is now available in the journal of Clinical Lymphoma Myeloma and Leukemia. The By-time post-hoc analyses of clinical response provides complementary data to the traditional ORR analysis from the pivotal study and enables visualization of the changing response rates over time during the twelve-month treatment period.

Link to publication: https://www.clinical-lymphoma-myeloma-leukemia.com/article/S2152-2650(20)30664-9/fulltext

The second publication: "Lack of Systemic Absorption of Topical Mechlorethamine Gel in Patients with Mycosis Fungoides Cutaneous T-Cell Lymphoma", is now published as a letter to editors in the Journal of Investigative Dermatology. This pre-specified analysis looks at whether mechlorethamine is absorbed into the bloodstream after mechlorethamine gel 0.016% application – with systemic absorption linked to the occurrence of adverse events. Bioanalytic testing results

indicate lack of systemic absorption of mechlorethamine in plasma samples from the 201 and 202 pivotal study.

Link to publication: https://www.jidonline.org/article/S0022-202X(20)32402-7/fulltext

Larisa J. Geskin, MD, Associate Professor of Dermatology at Columbia University Medical Center. Lead Author for the 201 Study By-Time Reanalysis Manuscript, and Co-Author for the Lack of Systemic Absorption Research Letter commented: "The 201 study has proven an important resource for data analysis. These two recent publications provide a deeper understanding of the effect of mechlorethamine treatment in MF-CTCL patients and the treatment life-cycle."

William L. Bailey, Helsinn Therapeutics (U.S.) Vice President of Medical & Scientific Affairs, commented: "We are pleased that research into the use of mechlorethamine is increasing, improving our understanding of current treatment practices and helping us aid clinicians to offer patients the highest quality care. Mechlorethamine is a valuable treatment option, a topical gel that can be used at home, a particularly important characteristic as hospitals find new ways to deliver treatment remotely."

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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V-VALC-US-0298