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Ceptaris Receives FDA Approval for VALCHLOR (TM) (mechlorethamine) Gel for the Treatment of Stage IA and IB Mycosis Fungoides-Type Cutaneous T-Cell Lymphoma in Patients Who Have Received Prior Skin-Directed Therapy

By

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First and only FDA-approved topical formulation of mechlorethamine (nitrogen mustard)

MALVERN, Pa., Aug 26, 2013 (BUSINESS WIRE) -- --Patient support and assistance programs to be established for VALCHLOR

Ceptaris Therapeutics, Inc., a privately held, specialty pharmaceutical company, announced today that the U.S. Food and Drug Administration (FDA) has granted marketing approval for the orphan drug VALCHLOR(TM) (mechlorethamine) gel for the topical treatment of stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma (CTCL) in patients who have received prior skin-directed therapy. VALCHLOR is the first and only FDA-approved topical formulation of mechlorethamine, commonly known as nitrogen mustard. VALCHLOR is a gel that is applied topically once a day and dries on the skin.

"This is good news for patients and the treatment community," said Youn H. Kim, M.D., Joanne and Peter Haas Jr. Professor for Cutaneous Lymphoma Research, Professor of Dermatology, and Director, Multidisciplinary Cutaneous Lymphoma Clinic, Stanford University School of Medicine. "We now have the confidence of a FDA-approved product backed by evidence from a well-controlled clinical trial that demonstrated clinically meaningful responses in the majority of patients with stage IA and IB mycosis fungoides-type cutaneous T-cell lymphoma (CTCL) who have received prior skin-directed therapy."

Mechlorethamine is a chemotherapeutic agent previously approved for intravenous treatment of mycosis fungoides, the most common type of CTCL. Topical mechlorethamine preparations are currently recommended for the treatment of early stage CTCL by the National Comprehensive Cancer Network (NCCN). Prior to the approval of VALCHLOR, there were no FDA-approved topical mechlorethamine products; only non-standardized, pharmacy-compounded petroleum ointment or aqueous-based topical preparations were available. Information about pharmacy-compounded preparations is not required to be submitted to or reviewed by FDA prior to their use by patients. Therefore, such preparations do not undergo the same rigorous FDA review as FDA-approved products.

The availability of VALCHLOR will allow physicians to treat patients with stage IA and IB mycosis fungoides-type CTCL who have received prior skin-directed therapy with a FDA-approved version of topical mechlorethamine. In addition to consistent, controlled manufacturing processes, VALCHLOR will be provided with labeling, which includes data and instructions for proper use to help achieve the best possible clinical results.

"The use of topical mechlorethamine has been documented over several decades, but this is the first time that a product has gone through the rigorous FDA approval process," said Stuart R. Lessin, M.D., former Director of Dermatology at the Fox Chase Cancer Center, President of the Board of Directors of the Cutaneous Lymphoma Foundation and lead investigator in the VALCHLOR pivotal trial. "Not only is VALCHLOR manufactured under FDA's good manufacturing practices, but it will also be accompanied by patient support and assistance programs which are not currently available with compounded mechlorethamine."

Results of the VALCHLOR Pivotal Trial

The approval of VALCHLOR was based on a randomized, observer-blinded, non-inferiority pivotal trial comparing VALCHLOR to a pharmacy-compounded mechlorethamine preparation in patients with stage IA-IIA MF-type CTCL. Patients had received at least one prior skin-directed therapy. Qualifying prior therapies included topical corticosteroids, phototherapy, Targretin(R) gel, and topical nitrogen mustard (mechlorethamine). Patients were not required to be refractory to or intolerant of prior therapies. In the thirteen center study, 260 patients (the vast majority of whom were IA and IB) were enrolled (1:1 randomization), making it the largest randomized study ever conducted in mycosis fungoides-type CTCL. Results of the study were published earlier this year in JAMA Dermatology.(1)

In the study, 60% of patients treated with VALCHLOR had a confirmed response at 12 months, defined as reduction of at least 50% in the Composite Assessment of Index Lesion Severity (CAILS) score, while 48% of those treated with the compounded control achieved a confirmed response. Complete responses constituted a minority of the CAILS overall response. CAILS responses were seen as early as 1 month, with further responses observed through 11 months of treatment. No systemic absorption of mechlorethamine was detected with VALCHLOR treatment.

Important Safety Information for VALCHLOR

VALCHLOR is for topical dermatologic use only. VALCHLOR is a cytotoxic drug. Avoid direct skin contact with VALCHLOR in individuals other than the patients due to risk of dermatitis, mucosal injury and secondary cancers. The use of VALCHLOR is contraindicated in patients with a history of severe or systemic hypersensitivity to mechlorethamine or inactive ingredients. Contact with mucous membranes, especially those of the eyes, must be avoided. Exposure of the eyes to mechlorethamine may cause pain, burns, inflammation, photophobia, blurred vision and in some cases severe and long-lasting injury to the eye. Patients should be monitored for non-melanoma

skin cancers during and after treatment with VALCHLOR. The most common adverse reaction to VALCHLOR is dermatitis, which in some cases may be severe and require dosing changes or discontinuation. Elderly patients may be more susceptible to dermatitis. Women should avoid becoming pregnant or nursing while using VALCHLOR due to potential hazard to the fetus. VALCHLOR is an alcohol-based gel. Avoid fire, flame and smoking until the gel has dried.

Commercialization of VALCHLOR

VALCHLOR will be distributed by Accredo Specialty Pharmacy with a target availability in the 4th quarter of 2013. Physicians will be able to prescribe VALCHLOR by visiting www.valchlor.com. Patient support and assistance programs will be available to patients. The program services offered will include reimbursement and financial support for eligible patients, as well as disease and product information.

On July 30, 2013, Ceptaris signed an agreement to merge with Actelion US Holding Company, a subsidiary of Actelion Ltd. (six:ATLN). The approval of VALCHLOR is a condition of closing the merger. Both companies anticipate that Actelion's expertise in rare diseases will facilitate the delivery of VALCHLOR to patients.

"The FDA approval of VALCHLOR is an important milestone which was achieved through the dedicated efforts of our team, our clinical investigators, and the patients who participated in our clinical studies," said Stephen Tullman, President and Chief Executive Officer, Ceptaris Therapeutics, Inc. "With this approval, we look forward to working with Actelion to close the merger and make VALCHLOR available to patients."

About Mycosis Fungoides and Cutaneous T-Cell Lymphoma

Mycosis fungoides is the most common type of cutaneous T-cell lymphoma, a rare form of non-Hodgkin's lymphoma. The cause of mycosis fungoides remains unknown and there is no known cure. Unlike most non-Hodgkin's lymphomas, mycosis fungoides is caused by malignant T-cells. The malignant T-cells in the body initially migrate to the skin, causing lesions to appear. These lesions typically begin as what appears to be a rash and may progress to form plaques and disfiguring tumors. Early stage cases may be confused with other skin conditions until a definitive diagnosis is made based upon skin biopsy. Most cases of mycosis fungoides are early stage and are diagnosed in patients over the age of 50.

About Ceptaris Therapeutics

Ceptaris Therapeutics, Inc. is a privately held, specialty pharmaceutical company that has developed a proprietary gel formulation of mechlorethamine hydrochloride for the treatment of stage IA and IB mycosis fungoides, a type of CTCL. VALCHLOR (mechlorethamine) gel is the first and only FDA-approved topical mechlorethamine product available to treat the signs and symptoms of this rare cancer. Please visit <http://www.ceptaris.com> for more information.

This release includes forward-looking statements concerning the Company including expectations regarding regulatory filings. The statements are based on assumptions about many important factors, including the following, which could cause actual results to differ materially from those in the forward-looking statements: satisfaction of regulatory and other requirements; actions of regulatory bodies and other governmental authorities; changes in laws and regulations; product quality or patient safety issues. The information contained in this press release was

accurate at the time of issuance and Ceptaris assumes no responsibility for updating the information to reflect subsequent developments.

(1) Lessin SR, Duvic M, Guitart J, Pandya AG, Strober BE, Olsen EA, et al. Topical chemotherapy in cutaneous T-cell lymphoma: Positive results of a randomized, controlled, multicenter trial testing the efficacy and safety of a novel mechlorethamine, 0.02%, gel in mycosis fungoides. JAMA Dermatol. 2013;149(1):25-32.

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