Overview
Results Day Center
Actelion today
Financial information
News archive
Annual Report
Stock Information
Events
First-time investor
Stay informed
Contact us

ACTELION CLOSES ACQUISITION OF PRIVATELY-HELD CEPTARIS THERAPEUTICS ADDING VALCHLOR TO ACTELION'S PRODUCT PORTFOLIO

- **Valchlor(TM) is the only FDA-approved topical formulation of mechlorethamine**
- **US launch planned for Q4 2013**
- **US patient support and assistance programs will be available for Valchlor**

ALLSCHWIL / BASEL, SWITZERLAND - 19 September 2013 - Actelion (SIX: ATLN) announced today that it has concluded the acquisition of Ceptaris Therapeutics, Inc. following US Food and Drug Administration (FDA) approval for Valchlor (mechlorethamine) gel 0.016% and the satisfaction of additional closing conditions. Ceptaris was a privately held, specialty pharmaceutical company established to develop the proprietary gel formulation of mechlorethamine for the treatment of early stage mycosis fungoides, a type of Cutaneous T-Cell Lymphoma (CTCL).

Valchlor (mechlorethamine) gel 0.016% is indicated 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 gel is applied topically once-a-day and dries on the skin. The availability of Valchlor allows US physicians to treat mycosis fungoides type CTCL with an FDA-approved formulation of topical mechlorethamine. In addition to consistent, controlled manufacturing processes, Valchlor will be provided with labeling that includes data and instructions for correct use, to help achieve the best possible clinical results.

Jean-Paul Clozel, M.D. and CEO at Actelion, commented: "Prior to the approval of Valchlor, there were no approved topical mechlorethamine products available for prescription. As a result, patients were dependent on formulations prepared locally by compounding pharmacies in a non-standardized environment. Information about pharmacy-compounded preparations is not required to be submitted to, or reviewed by, the FDA prior to their use by patients. Therefore, such preparations do not undergo the same rigorous FDA review as Valchlor has. This new standardized product represents a clinically relevant improvement for patients suffering from mycosis fungoides."

COMMERCIALIZATION OF VALCHLOR

Valchlor will be distributed in the US by Accredo Specialty Pharmacy with target availability in the fourth 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 insurance verification and financial assistance for eligible patients, as well as disease and product information.

Otto Schwarz, Chief Operating Officer at Actelion, commented: "Actelion is ready to leverage our expertise in orphan and ultra-orphan indications to appropriately commercialize Valchlor, a meaningfully differentiated medicine, to specialists in the field of dermatology and oncology. We intend to make Valchlor available in the US during the fourth quarter of this year, using a dedicated sales force and will evaluate the opportunity outside the US, before filing for registration in other regions."

ABOUT VALCHLOR (MECHLORETHAMINE) GEL

Valchlor (mechlorethamine) gel 0.016% is indicated 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 gel is applied topically once-a-day and dries on the skin. Mechlorethamine is a chemotherapeutic agent previously approved for intravenous treatment of mycosis fungoides, the most common type of Cutaneous T-Cell Lymphoma (CTCL). Topical mechlorethamine preparations are currently recommended for the treatment of early stage CTCL by the National Comprehensive Cancer Network (NCCN). Valchlor is the first and only FDA-approved topical formulation of mechlorethamine.

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.

###

NOTES TO THE EDITOR

CONTACT

Investor Relations
Actelion Pharmaceuticals Ltd

Gewerbestrasse 16
CH-4123 Allschwil
Switzerland

Tel.: +41 61 565 62 62
Fax: +41 61 565 65 07

Contact us online

RELATED LINKS

Actelion press releases

DOWNLOADS

Press Release PDF

ABOUT MYCOSIS FUNGOIDES AND CUTANEOUS T-CELL LYMPHOMA

Mycosis fungoides is the most common type of Cutaneous T-Cell Lymphoma (CTCL), 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 a mutation of T-cells. The malignant T-cells in the body initially migrate to the skin, causing various 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.

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 CTCL. Patients had received at least one prior skin-directed therapy. Qualifying prior therapies included topical corticosteroids, phototherapy, bexarotene gel (retinoid selectively activating the retinoid X receptors), and topical nitrogen mustard (mechlorethamine). Patients were not required to be refractory to or intolerant of prior therapies. In the thirteen cancer 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. CAILS responses were seen as early as 1 month, with further responses observed through 11 months of treatment.

The most common side effects associated with Valchlor in the pivotal trial were local skin reactions, including dermatitis (56%), pruritus (20%), bacterial skin infections (11%), skin ulceration or blistering (6%) and skin hyperpigmentation (5%). The most common of these skin reactions, dermatitis, ranged from mild to severe. No systemic absorption of mechlorethamine was detected with Valchlor treatment.

ABOUT THE ACQUISITION

On July 30, 2013, Actelion US Holding Company, a subsidiary of Actelion Ltd, and Ceptaris Therapeutics, Inc. entered into an agreement whereby Actelion would acquire Ceptaris. Under the terms of the agreement, the merger was contingent upon certain closing conditions, including the US FDA approval of Ceptaris' product, Valchlor.

Actelion paid to Ceptaris USD 25 million upon signing, followed by a further payment of USD 225 million upon closing of the transaction. Ceptaris' shareholders are also eligible to receive additional payments based on net sales of Valchlor and/or the achievement of certain commercial milestones.

References

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.

ABOUT ACTELION LTD

Actelion Ltd is a biopharmaceutical company with its corporate headquarters in Allschwil/Basel, Switzerland. Actelion's first drug Tracleer® (bosentan), an orally available dual endothelin receptor antagonist, has been approved as a therapy for pulmonary arterial hypertension. Actelion markets Tracleer through its own subsidiaries in key markets worldwide, including the United States (based in South San Francisco), the European Union, Japan, Canada, Australia and Switzerland. Actelion, founded in late 1997, is a leading player in innovative science related to the endothelium - the single layer of cells separating every blood vessel from the blood stream. Actelion's over 2,300 employees focus on the discovery, development and marketing of innovative drugs for significant unmet medical needs. Actelion shares are traded on the SIX Swiss Exchange (ticker symbol: ATLN) as part of the Swiss blue-chip index SMI (Swiss Market Index SMI®).

For further information please contact:

Roland Haefeli
Senior Vice President, Head of Investor Relations & Public Affairs
Actelion Pharmaceuticals Ltd, Gewerbestrasse 16, CH-4123 Allschwil
+41 61 565 62 62
+1 650 624 69 36
www.actelion.com