Actelion is granted marketing authorization by the European Commission for Ledaga (chlormethine gel) for the treatment of MF-CTCL

ALLSCHWIL/BASEL, SWITZERLAND – 07 March 2017 – Actelion Ltd (SIX: ATLN) announced today that the European Commission has granted marketing authorization for the use of Ledaga® (chlormethine gel) 160 micrograms/g for the treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF-CTCL).

MF-CTCL is a rare, potentially life-threatening immune system cancer that is chronic and usually progresses slowly. The course of disease in individual patients is unpredictable. In about 34% of cases, a progression of the disease is observed, and in the most advanced stages, MF-CTCL cells can metastasize to other body tissues, including the liver, spleen and lungs.

Ledaga is indicated for the topical treatment of mycosis fungoides-type cutaneous T-cell lymphoma (MF type CTCL) in adult patients.

The market authorization for Ledaga is based on the results of the pivotal 201 study, the largest randomized controlled study ever conducted in early stage MF-CTCL, involving 260 patients. In this study, within the efficacy evaluable (EE) population, 77% of patients who were treated for at least 6 months with chlormethine gel achieved a clinical response in the Composite Assessment of Index Lesion Severity (CAILS) score, while 59% of those treated with the compounded control had a clinical response. A response was defined as at least a 50% improvement in the baseline CAILS score. Complete response was achieved in 19% of patients treated with chlormethine gel in the EE population versus 15% of patients treated with the compounded control. Reductions in mean CAILS scores were seen as early as four weeks into the study, with further reductions observed with continuing therapy.

In the 201 study, the most frequent adverse reactions reported with chlormethine gel were skin related: dermatitis (54.7%; e.g., skin irritation, erythema, rash, urticaria, skin-burning sensation, pain of the skin), pruritus (20.3%), skin infections (11.7%), skin ulceration and blistering (6.3%), and skin hyperpigmentation (5.5%). No evidence of systemic absorption of chlormethine was observed with the treatment.
Actelion is working diligently to launch Ledaga in the EU as rapidly as possible. Actelion has agreed to a list of post-approval measures with the CHMP (Committee for Medical Products for Human Use, the scientific committee of the European Medicines Agency). Subject to fulfilling the agreed commitments and achieving market access in various countries, a potential first European launch of Ledaga is not expected before January 2018.

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Notes to Editor:

ABOUT LEDAGA (CHLORMETHINE)
Ledaga (chlormethine) is an alkylating drug indicated for the treatment of mycosis fungoides-type cutaneous T-Cell lymphoma (MF-CTCL) formulated as a topical, once-daily, colorless gel.

Chlormethine gel, under the brand name Valchlor® (mechlorethamine) is commercially available in the US (since 2013) and in Israel through special import authorization procedure (since 2016). In France, patients benefit from the drug under a temporary authorization for use ("ATU") program initiated during the second half of 2014.

ABOUT MF-CTCL
Mycosis fungoides-type cutaneous T-Cell lymphoma (MF-CTCL) is a rare, but serious and life-threatening, immune system cancer that appears in the skin. MF-CTCL is the most common form of cutaneous T-cell lymphoma.

MF-CTCL typically appears in patients over 50 years of age (median age is 54), and is more common in men. It presents first as dry skin and a red rash, with or without itching. As a result, MF-CTCL is often mistaken for eczema or psoriasis, delaying diagnosis. MF-CTCL goes on to form scaly plaques on the skin, which can cover small or large areas of the skin. Large bumps or tumor nodules may also develop, and lymph nodes may be involved.

While MF-CTCL is a chronic and usually slowly progressing disease, the course of disease in individual patients is unpredictable, with some patient progressing into advanced stages. In about 34% of cases, a progression of the disease is observed, and in the most advanced stages, MF-CTCL cells can metastasize to other body tissues, including the liver, spleen and lungs.

Current research suggests that patients who are diagnosed in early stages of MF-CTCL have a normal life expectancy, however, the average time to diagnosis ranges from two to seven years. An important therapeutic objective in treating MF-CTCL is prevention of disease progression. Failure to maintain MF-CTCL in its early stages results in a drastically reduced median survival.

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ABOUT STUDY 201

Study 201 was a multicenter, randomized, observer-blinded, active-controlled, 12-month study of Stage I and IIA MF-type CTCL patients, conducted in 13 centers in the US to evaluate the efficacy and safety of chlormethine gel compared with chlormethine HCl 0.02% compounded in Aquaphor® ointment. In total, 260 patients were randomized 1:1 to topical treatment with chlormethine gel or chlormethine HCl 0.02% compounded in Aquaphor® ointment once daily for up to 12 months.

In the study, within the efficacy evaluable (EE) population, 77% of patients treated with chlormethine gel had a clinical response at 12 months, in the Composite Assessment of Index Lesion Severity (CAILS*) score, while 59% of those treated with the compounded control achieved a confirmed response. (*A response was defined as at least a 50% improvement in the baseline CAILS score).

Complete response was achieved in 19% of patients treated with chlormethine gel versus 15% of patients treated with the compounded control in the EE population. Reductions in mean lesion severity (CAILS) were seen as early as four weeks, with further reductions observed with continuing therapy.

The most frequent adverse reactions reported with chlormethine gel were skin related: dermatitis (54.7%; e.g., skin irritation, erythema, rash, urticaria, skin-burning sensation, pain of the skin), pruritus (20.3%), skin infections (11.7%), skin ulceration and blistering (6.3%), and skin hyperpigmentation (5.5%). No clinical evidence of systemic absorption of chlormethine was observed with the treatment.

Actelion Ltd.

Actelion Ltd. is a leading biopharmaceutical company focused on the discovery, development and commercialization of innovative drugs for diseases with significant unmet medical needs.

Actelion is a leader in the field of pulmonary arterial hypertension (PAH). Our portfolio of PAH treatments covers the spectrum of disease, from WHO Functional Class (FC) II through to FC IV, with oral, inhaled and intravenous medications. Although not available in all countries, Actelion has treatments approved by health authorities for a number of specialist diseases including Type 1 Gaucher disease, Niemann-Pick type C disease, Digital Ulcers in patients suffering from systemic sclerosis, and mycosis fungoides type cutaneous T-cell lymphoma.

Founded in late 1997, with now over 2,600 dedicated professionals covering all key markets around the world including Europe, the US, Japan, China, Russia and Mexico, Actelion has its corporate headquarters in Allschwil / Basel, Switzerland.

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®). All trademarks are legally protected by their respective owners.
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The above information contains certain “forward-looking statements”, relating to the company’s business, which can be identified by the use of forward-looking terminology such as “estimates”, “believes”, “expects”, “may”, “are expected to”, “will”, “will continue”, “should”, “would be”, “seeks”, “pending” or “anticipates” or similar expressions, or by discussions of strategy, plans or intentions. Such statements include descriptions of the company’s investment and research and development programs and anticipated expenditures in connection therewith, descriptions of new products expected to be introduced by the company and anticipated customer demand for such products and products in the company’s existing portfolio. Such statements reflect the current views of the company with respect to future events and are subject to certain risks, uncertainties and assumptions. Many factors could cause the actual results, performance or achievements of the company to be materially different from any future results, performances or achievements that may be expressed or implied by such forward-looking statements. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected.