



Media Release

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US Food and Drug Administration grants priority review for Actelion's miglustat in Niemann-Pick type C disease

ALLSCHWIL/BASEL, SWITZERLAND – 19 November 2009 – Actelion Ltd (SIX: ATLN) announced today that a supplemental new drug application (sNDA) for an extension of indication for Zavesca® (miglustat) for the treatment of progressive neurological manifestations in adult and pediatric patients with Niemann-Pick type C disease (NP-C) has been accepted by the U.S. Food and Drug Administration (FDA).

In the US, Zavesca® is currently indicated for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease where enzyme replacement therapy is unsuitable or is not a therapeutic option.

The sNDA, based on results from the clinical trial OGT 918-007, and two multicenter retrospective cohort studies in patients with NP-C, has been granted a priority review. A priority review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. It also means that the FDA will aim to complete the review within 6 months.

Actelion has been informed by the FDA that this sNDA will be reviewed by the Endocrine and Metabolic Drug Advisory Committee (EMDAC) on 12th January 2010. The FDA often seeks the advice of an Advisory Committee when evaluating potential treatments for diseases, for instance when there is no approved therapy available.

Jean-Paul Clozel, M.D. and Chief Executive Officer commented: "Actelion is working closely with the FDA to provide information as needed to support the review process and make miglustat available in the United States to patients suffering from this fatal neurodegenerative genetic disorder affecting both children and adults. Miglustat could become the first treatment for NP-C in the USA, which would represent a major therapeutic breakthrough for patients and their treating physicians".

In 2008, FDA granted orphan drug status to miglustat for NP-C in the United States.

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Notes to the editor**About Zavesca® (miglustat)**

Zavesca® (100 mg miglustat capsule) is indicated for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease. Zavesca® may only be used in the treatment of type 1 Gaucher patients for whom enzyme replacement therapy is unsuitable or is not a therapeutic option. It is approved in the European Union, the United States, Canada, Switzerland, Brazil, Australia, Turkey, Israel, South Korea, New Zealand and Russia.

In the European Union, South Korea, Brazil and Russia, Zavesca® is also indicated for the treatment of progressive neurological manifestations in adult patients and pediatric patients with Niemann-Pick type C disease.

About Niemann-Pick type C disease

NP-C is a very rare, fatal, neurodegenerative, genetic condition, primarily affecting children and teenagers but which can strike at any age. The symptoms are caused by the storage of some glycosphingolipids within certain cells in the body, including the brain. It is invariably progressive and most patients die within five to ten years of diagnosis; for the majority the disease is fatal during childhood. Neurological deterioration is the key feature of the disease, and can manifest itself as clumsy body movements, balance problems, slow and slurred speech, difficulty in swallowing, problems with eye movements and seizures. Intellectual decline is also common. In the final stages of the disease the child or young adult is frequently bedridden, has little muscle control and is intellectually impaired. Diagnosis of the disease can be difficult and lengthy due to its rarity and heterogeneity.

Zavesca® safety information

Gastrointestinal events, mainly diarrhea, have been observed in more than 80% of patients treated with Zavesca®, either at the onset of treatment or intermittently during treatment. The majority of cases are mild and are expected to resolve after the first weeks on therapy. In clinical practice, diarrhea has been observed to respond to diet modification (reduction of lactose and other carbohydrate intake), to taking Zavesca® away from meals, and/or to antidiarrheal medicinal products such as loperamide. In some patients, temporary dose reduction may be necessary. Patients with chronic diarrhea or other persistent gastrointestinal events that do not respond to these interventions should be investigated according to clinical practice. Zavesca® has not been evaluated in patients with a history of significant gastrointestinal disease, including inflammatory bowel disease.

Cases of peripheral neuropathy have been reported in patients with type 1 Gaucher disease treated with Zavesca®. Peripheral neuropathy seems to be more common in patients with type 1 Gaucher disease compared to the general population. All patients should undergo baseline and repeat neurological

evaluation. Patients who develop symptoms such as numbness and tingling should have a careful re-assessment of risk benefit.

Zavesca® may cause fetal harm if administered to a pregnant woman and is contraindicated in women who are or who may become pregnant; patients should be informed of the potential hazard to the fetus. There is a risk of impaired fertility in men. Men should maintain reliable contraceptive methods and not plan to conceive while taking Zavesca® and for three months thereafter.

Actelion Ltd

Actelion Ltd is a biopharmaceutical company with its corporate headquarters in Allschwil/Basel, Switzerland. Actelion's first drug Tracleer®, 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 2'200 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®).

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