



Media Release

12 January 2010

Zavesca® (miglustat) receives positive vote from the FDA's Endocrinologic and Metabolic Drugs Advisory Committee for the treatment of Niemann-Pick type C disease

ALLSCHWIL/BASEL, SWITZERLAND – 12 January 2010 – Actelion Ltd (SIX: ATLN) announced today that United States (US) Food and Drug Administration's (FDA) Endocrinologic and Metabolic Drugs Advisory Committee voted (10 yes to 3 no) in its final vote in question that the benefit/risk profile of Zavesca® (miglustat) supports its approval for the treatment of progressive neurological manifestations in adult patients and pediatric patients with Niemann-Pick type C (NP-C) disease. NP-C disease is a very rare, relentlessly progressive and eventually fatal neurodegenerative genetic disorder for which no specific treatment is currently approved in the US.

The decision was based on results from the clinical trial OGT 918-007 and two multicenter NP-C disease cohort studies as well as other clinical trials in related lysosomal storage disorders for the safety and tolerability evaluation.

Jean-Paul Clozel, M.D. and Chief Executive Officer of Actelion commented: "We are pleased that the Advisory Committee today recognized the importance of this therapeutic advance and voted to recommend approval of the supplemental New Drug Application (sNDA) of Zavesca® for the treatment of NP-C disease. We will continue to work closely with the FDA to facilitate the completion of the priority review of the sNDA."

Zavesca® is the only specific treatment available for patients with NP-C disease. It received approval in the European Union (EU) and other countries in 2009. Zavesca® is also indicated in the US, the EU and other countries for the oral treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is unsuitable or is not a therapeutic option. The use of Zavesca® is supported by over 10 years of clinical trials and post-marketing experience across indications.

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. The FDA is not bound by the committee's guidance, but takes its recommendation into consideration. The decision of the FDA for the sNDA is expected in March 2010.

About Niemann-Pick type C disease

NP-C disease is a very rare, fatal, neurodegenerative, genetic condition, primarily affecting children and teenagers although the clinical manifestations can become apparent at any age. The symptoms are caused by the storage of some lipids, such as glycosphingolipids and cholesterol, within certain tissues 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 may take years due to the rarity and heterogeneity of this condition.

For more information:

<http://podcasts.mayoclinic.org/2008/07/11/niemann-pick-disease-type-c/>

###

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 for this indication 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.

Zavesca® safety information

In clinical studies, the most common adverse events due to Zavesca® included weight loss, diarrhea, and tremor. Other common adverse reactions were flatulence, abdominal pain, headache, and influenza-like symptoms. The most common serious adverse reaction was peripheral neuropathy. Patients should undergo neurological examination at the start of treatment and every 6 months thereafter; Zavesca® should be reassessed in patients who develop symptoms of peripheral neuropathy. Zavesca® should not be used in pregnant woman. Men should maintain reliable contraceptive methods while taking Zavesca® and for 3 months after discontinuing treatment.

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 1900 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

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

<http://www.actelion.com>