Media Release

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Actelion obtains an option to in-license vamorolone from ReveraGen

Vamorolone, a novel compound for the treatment of Duchenne Muscular Dystrophy, holds potential to better preserve muscle function and prolong ambulation for the patient, without some of the side effects associated with glucocorticoid therapy.

ALLSCHWIL, SWITZERLAND, and ROCKVILLE (MD), USA – 03 November 2016 – Actelion Ltd (SIX: ATLN) and ReveraGen BioPharma, Inc., a privately held company engaged in the discovery and development of proprietary therapeutic products for neuromuscular and inflammatory diseases, announced today that they have entered into an agreement. By this agreement, Actelion has obtained an exclusive option to in-license ReveraGen's lead compound vamorolone for the treatment of Duchenne Muscular Dystrophy at two different stages in its development.

Vamorolone is a novel compound that has the potential to preserve muscle function and prolong ambulation, without some of the known side effects associated with corticosteroids currently in use. This is important especially for very young patients with Duchenne, where glucocorticoid therapy is not appropriate due to these side effects, which include growth stunting and immune suppression.

Jean-Paul Clozel, MD and Chief Executive Officer of Actelion, commented: "We have been very impressed by the pioneering work performed by Dr. Hoffman and his team at ReveraGen, and by the encouragement the development of vamorolone receives from the patient community. With our proven scientific, regulatory and marketing expertise in the area of orphan drugs, Actelion is ideally positioned to support the development of this new therapeutic approach, to benefit the boys affected by Duchenne Muscular Dystrophy and help those who care for them."

Eric Hoffman, Chief Executive Officer of ReveraGen, commented: "Duchenne has been the focus of our efforts for many years, and we now have the chance to slow the progression of this devastating disease. The project has already greatly benefited from broad philanthropic support, and we are delighted with the option agreement, which will bring Actelion's scientific and commercial competencies to the table, enabling vamorolone to rapidly reach patients with Duchenne and their families."

ReveraGen is pursuing parallel clinical development for vamorolone in both the US and Europe. Phase I clinical trials were completed in late 2015, funded through venture philanthropy contracts by organizations including the Muscular Dystrophy Association (USA), Joining Jack (UK), Duchenne Research Fund (UK) and Duchenne Children's Trust (UK).

A Phase IIa program is currently underway and investigates the safety and efficacy of vamorolone in 4-7 year old steroid-naïve boys with Duchenne (patients that have not taken prednisone or deflazacort). The DMD clinical program is being developed and run by a collaboration between the CINRG group and Newcastle University (Kate Bushby and Michela Guglieri). A Phase IIb program is in the planning stage.

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NOTES TO EDITOR:

ABOUT THE AGREEMENT

Actelion signed a license, collaborative development and commercialization agreement with ReveraGen BioPharma, Inc., a corporation organized under the laws of Delaware, US, to research and co-develop vamorolone, a non-hormonal steroid modulator primarily for the treatment of Duchenne Muscular Dystrophy ("DMD"). To date Actelion has paid a total of USD 10 million to ReveraGen which has been expensed as R&D costs. Under the terms of the agreement Actelion will also support R&D activities up to a maximum amount of USD 1 million p.a. for the next three years. In addition, Actelion acquired an option to obtain the exclusive worldwide license rights on vamorolone at any time but not later than following the receipt of the Phase IIb study results. If the option is exercised, ReveraGen will be entitled to receive up to USD 165 million in development and regulatory milestones for the DMD indication and up to USD 190 million for three further indications depending on achievement of certain development, regulatory approval and commercialization milestones. Furthermore, Actelion will pay increasing tiered single- to double-digit royalties on the net sales of vamorolone.

ABOUT VAMOROLONE

Vamorolone is the first-in-human steroid-like compound that shows signs of effectively separating a number of sub-properties seen in corticosteroids. Animal models indicate that vamorolone retains sub-properties associated with efficacy (namely anti-inflammatory effects), and in addition shows new membrane stabilization and mineralocorticoid receptor antagonist sub-properties, all without the growth stunting and immune suppression side effects of corticosteroids.

Treatment with vamorolone in the earlier stages of DMD holds potential to better preserve muscle function than is seen currently, without some of the side effects that have limited the use of corticosteroids in very young children in particular.

Vamorolone has received Orphan Drug Designation in both the US and Europe. ReveraGen is pursuing parallel clinical development for vamorolone in both the US and Europe. Phase I clinical trials have been completed in late 2015. A Phase IIa program is currently underway and investigates the safety and efficacy of

vamorolone in 4-7 year old steroid-naïve DMD boys (patients that have not taken prednisone or deflazacort). The DMD clinical program is being developed and run by a collaboration between the CINRG group and Newcastle University (Kate Bushby and Michela Guglieri). A Phase IIb program is in the planning stage.

ABOUT DUCHENNE MUSCULAR DYSTROPHY (DMD)

Duchenne Muscular Dystrophy is a devastating and aggressive genetic disorder where progressive muscle wasting leads to increasing weakness, disability and early death. It is caused by a mutation of the gene which codes for dystrophin, a protein that is essential to maintain normal muscle function. If dystrophin is absent, the stability of the muscle cell membranes is reduced – the muscle cells are easily damaged and die.

Duchenne is an X-linked disease, such as hemophilia and red-green color blindness. As the dystrophin gene is located on the X chromosome - of which females have two, but males only one - females have a “backup” if one of the genes is flawed, and will produce enough dystrophin to preserve muscle function. Females can however be carriers, giving the X chromosome with the mutated dystrophin gene to their sons, who will likely develop Duchenne or Becker Muscular Dystrophy, a milder form of muscular dystrophy related to Duchenne, depending on the mutation.

It is estimated that approximately 1 in every 3,500 live male births is affected, with about 20,000 new cases each year worldwide. Onset typically occurs in early childhood, with early motor developmental milestones being delayed. By age four or five, mobility and movement problems like falling or trouble climbing stairs become increasingly apparent. As muscles continue to weaken, the boys begin to require increased physical support and have to use a wheelchair around the age of 7-13 years. With the disease progressing, arms, chest and neck muscles can also be affected and the heart muscle and diaphragm may weaken. By young adulthood, the condition causes premature death, mostly due to respiratory or cardiac failure resulting from extreme muscle weakness.

REVERAGEN BIOPHARMA, INC.

ReveraGen BioPharma, Inc. is a private pharmaceutical company, founded in 2008 and headquartered in Rockville (MD), USA. ReveraGen’s single asset, vamorolone, is a proprietary compound for the treatment of Duchenne Muscular Dystrophy. Vamorolone development has progressed through venture philanthropy, partnering with government programs such as the US National Institutes of Health, European Community, and international stakeholder foundations, including The Foundation to Eradicate Duchenne, Parent Project Muscular Dystrophy, Save Our Sons, and others. In total, approximately USD 20 million has been provided by government and stakeholders to date.

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 also 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 2,500 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.

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