Media Release

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Actelion provides an update on the Phase III IMPACT program with cadazolid in CDAD

ALLSCHWIL, SWITZERLAND – 08 June 2017 – Actelion Ltd (SIX: ATLN) today provided an update on the Phase III program IMPACT to investigate the efficacy and safety of Actelion's novel anti-infective cadazolid versus vancomycin in the treatment of *Clostridium difficile*-associated diarrhea (CDAD). In the pivotal program, IMPACT 1 met its primary endpoint, while the second study IMPACT 2 did not meet the primary endpoint. Cadazolid demonstrated an acceptable tolerability and safety profile in the IMPACT program.

IMPACT 1 and 2 compared the efficacy and safety of cadazolid (250 mg administered orally twice daily for 10 days) versus vancomycin (125 mg administered orally four times daily for 10 days). A total of 1263 patients worldwide participated in the IMPACT program, which assessed as primary endpoint whether the clinical response after administration of cadazolid is non-inferior to vancomycin in patients with CDAD.

The company will now work diligently to complete the analyses of the full study results and detailed results will be made available through scientific disclosure at upcoming congresses and in peer-reviewed publications.

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NOTES TO EDITOR:

ABOUT THE IMPACT PROGRAM

The IMPACT program (International Multi-center Program Assessing Cadazolid Treatment in patients suffering from *Clostridium difficile*-associated diarrhea) comprised two identical Phase III studies that were designed as multi-center, randomized, double-blind studies to compare the efficacy and safety of cadazolid versus vancomycin in patients with *Clostridium difficile*-associated diarrhea (CDAD). Primary endpoint of the studies was the clinical cure rate at end of treatment (+ 2 days). The clinical cure rate was defined as the resolution of diarrhea (≤3 unformed bowel movements per days for at least 2 consecutive days) and no further need for CDAD therapy on study treatment and maintained for 2 days after the end of study treatment.

ABOUT CADAZOLID

Cadazolid, a novel quinoxolidinone antibiotic, is a strong inhibitor of *Clostridium difficile* protein synthesis, leading to suppression of toxin production and spore formation. In preclinical studies, cadazolid showed potent

in vitro activity against *C. difficile* isolates and a low propensity for resistance development. In a human gut model of CDAD, cadazolid had a very limited impact on the normal gut microflora.

Cadazolid was previously investigated in a randomized, double-blind, active reference group Phase II study, with 84 patients randomized 1:1:1:1 to 250, 500, or 1,000 mg cadazolid or oral 125 mg vancomycin. The results provided proof of concept for the efficacy and safety of cadazolid for the treatment of CDAD and supported the progression to the Phase III program with a cadazolid dose of 250 mg BID.

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 2,500 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 currently traded on the SIX Swiss Exchange (ticker symbol: ATLN). All trademarks are legally protected.

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