

**Summit Corporation plc**  
(‘Summit’ or ‘the Company’)

**SUMMIT RECEIVES REGULATORY APPROVAL TO START PHASE 1B CLINICAL TRIAL OF SMT C1100 IN DMD PATIENTS**

**Oxford, UK, 1 November 2013** – Summit (AIM: SUMM), a drug discovery and development company advancing therapies for Duchenne Muscular Dystrophy (‘DMD’) and *C. difficile* infection, announces that its Phase 1b Clinical Trial Application for SMT C1100 has received approval from the UK Medicines and Healthcare products Regulatory Agency (‘MHRA’) and the Ethics Review Committee. SMT C1100 is a small molecule utrophin modulator that has the potential to treat all patients with DMD, regardless of the underlying genetic fault.

“Securing regulatory approval for the first in patient Phase 1b clinical trial has achieved another important milestone in the development of utrophin modulator, SMT C1100, for DMD,” **commented Glyn Edwards, Chief Executive Officer**. “We believe that utrophin modulation is a novel disease-modifying approach for all boys with DMD and this Phase 1b trial forms an integral part of our wider clinical plans towards establishing SMT C1100 as a viable treatment for this devastating condition.”

The Phase 1b trial will be a dose-escalating, open-label study and will be conducted in a total of 12 paediatric patients with DMD, aged between 5 and 11 years. It will evaluate the safety and tolerability of SMT C1100, and will measure blood concentration levels of the drug as Summit aims to confirm the dose to be used in a subsequent patient proof of concept efficacy trial. The Phase 1b trial will be conducted at up to four NHS hospitals located in the UK and patient recruitment is expected to start shortly.

The Chief Investigator for the trial, **Professor Francesco Muntoni, Paediatric Neurologist at Great Ormond Street Hospital and Director of the Dubowitz Neuromuscular Centre** added, “Utrophin is a promising approach for the treatment of all DMD patients, regardless of their genetic mutation. It also has the potential to be complementary to other therapeutics approaches in clinical development and the start of the first patient trial of SMT C1100 is an important moment for the whole DMD community.”

Further details about the clinical trial will be made available via [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and [www.clinicaltrialsregister.eu](http://www.clinicaltrialsregister.eu).

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**Notes to Editors**

**About DMD, Utrophin Modulation and SMT C1100**

DMD is a progressive muscle wasting disease that affects around 50,000 boys in the developed world. It is caused by different genetic faults in the gene that encodes dystrophin, a protein that is essential for the healthy function of all muscles. There is currently no cure for DMD and life expectancy is around mid-twenty. Utrophin protein is the functional equivalent of dystrophin and studies have shown that maintaining utrophin production has the potential to slow down or even stop the progression of DMD, regardless of the underlying dystrophin mutation. It is also expected to be complementary to other therapeutic approaches in clinical trials. SMT C1100, an orally administered small molecule, is Summit’s most advanced utrophin modulator. Non-clinical studies showed SMT C1100 increases utrophin protein levels in skeletal and cardiac muscle resulting in a significant reduction in the dystrophic muscle pathology. In 2012, SMT C1100 successfully completed a Phase 1 trial demonstrating the drug was safe and achieved therapeutic blood levels in healthy volunteers.

**About Summit**

Summit is an Oxford, UK based drug discovery and development Company targeting high-value areas of unmet medical need including Duchenne Muscular Dystrophy and *C. difficile* infection. Summit is listed on the AIM market of the London Stock Exchange and trades under the ticker symbol SUMM. Further information is available at [www.summitplc.com](http://www.summitplc.com) and follow Summit on Twitter ([@summitplc](https://twitter.com/summitplc)).

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**Forward Looking Statements**

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