Summit Therapeutics plc

("Summit" or "the Company")

SUMMIT THERAPEUTICS RECEIVES REGULATORY APPROVAL TO INITIATE PhaseOut DMD, A PHASE 2 CLINICAL TRIAL OF SMT C1100 IN PATIENTS WITH DMD

Oxford, UK, 21 January 2016 - Summit Therapeutics plc (NASDAQ: SMMT, AIM: SUMM), the drug discovery and development company advancing therapies for Duchenne muscular dystrophy ('DMD') and *Clostridium difficile* infection, announces that it has received approval from the UK Medicines and Healthcare products Regulatory Agency and the Research Ethics Committee to initiate PhaseOut DMD, a Phase 2 proof of concept clinical trial of SMT C1100 in patients with DMD. SMT C1100 is an orally administered, small molecule utrophin modulator that the Company believes has the potential to treat all boys and young men with DMD, regardless of their underlying dystrophin gene mutation. Utrophin is functionally and structurally similar to dystrophin, a protein which is essential for the healthy function of muscles.

"Our lead utrophin modulator, SMT C1100, has demonstrated disease-modifying potential in preclinical studies to date, and with the initiation of PhaseOut DMD, we are at a stage in SMT C1100's clinical development where we are evaluating the possibility of this benefit in patients," **said Ralf Rosskamp, MD, Chief Medical Officer of Summit.** *"We look forward to opening clinical trial sites in the UK and enrolling the first patients soon."*

PhaseOut DMD aims to provide proof of concept for SMT C1100 and utrophin modulation through measurements of muscle fat infiltration, as well as measuring utrophin protein and muscle fibre regeneration in muscle biopsies. The 48-week open-label trial is expected to enrol up to 40 boys ranging in age from their fifth to their tenth birthdays at sites in Europe and the US (subject to the US Food and Drug Administration, or FDA, regulatory approval). The primary endpoint of the trial is the change from baseline in magnetic resonance imaging parameters related to fat infiltration and inflammation of the leg muscles. Functional endpoints, including the six-minute walk test, North Star Ambulatory Assessment and patient reported outcomes, are also being explored. Summit expects to report data from the first group of patients enrolled in the trial periodically from the second half of 2016 onwards with the first set of 24-week muscle biopsy data expected to be available before the end of 2016.

The Company expects to submit an investigational new drug application to the FDA to allow PhaseOut DMD to also enrol patients in the US. In addition, the Company is exploring means by which to enrol patients who have participated in previous clinical trials of SMT C1100, but who may not meet the inclusion and exclusion criteria for PhaseOut DMD. Summit expects that further information about PhaseOut DMD will shortly be available on www.clinicaltrials.gov.

About Utrophin Modulation in DMD

DMD is a progressive muscle wasting disease that affects around 50,000 boys and young men in the developed world. The disease 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 into the late twenties. Utrophin protein is functionally and structurally similar to dystrophin. In preclinical studies, the continued expression of utrophin has a meaningful, positive effect on muscle performance. Summit believes that utrophin modulation has the potential to slow down or even stop the progression of DMD, regardless of the underlying dystrophin gene mutation. Summit also believes that utrophin modulation could potentially be complementary to other therapeutic approaches for DMD. The Company's lead utrophin modulator is an orally administered, small molecule called SMT C1100. DMD is an orphan disease, and the US Food and Drug Administration and the European Medicines Agency have granted orphan drug status to SMT C1100. Orphan drugs receive a number of benefits including additional regulatory support and a period of market exclusivity following approval.

About Summit Therapeutics

Summit is a biopharmaceutical company focused on the discovery, development and commercialisation of novel medicines for indications for which there are no existing or only inadequate therapies. Summit is conducting clinical programs focused on the genetic disease Duchenne muscular dystrophy and the infectious disease *C. difficile* infection. Further information is available at www.summitplc.com and Summit can be followed on Twitter (@summitplc).

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