Summit Therapeutics plc

('Summit', 'the Company' or 'the Group')

SUMMIT TO EXTEND ONGOING PhaseOut DMD CLINICAL TRIAL OF EZUTROMID IN PATIENTS WITH DMD

- **Decision follows interim safety review by PhaseOut DMD's independent Data Monitoring Committee**

Oxford, UK, 27 March 2017 – 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 will proceed with the planned extension phase of PhaseOut DMD, a Phase 2 clinical trial evaluating the utrophin modulator ezutromid, subject to regulatory approval. This follows an interim review of the safety and tolerability data from the ongoing trial by an independent Data Monitoring Committee ('DMC') and its support of Summit's plans to extend the clinical trial.

Summit has now applied for regulatory approval to extend PhaseOut DMD from the UK Medicines and Healthcare products Regulatory Agency and Ethics Committee, and has submitted the necessary regulatory updates to the US Food and Drug Administration. These submissions are intended to facilitate the transition of patients participating in PhaseOut DMD onto an open-label extension phase at the end of the initial 48-weeks of dosing with ezutromid without a cessation in dosing. The extension phase will be used to gather long term safety and efficacy data and is expected to last until ezutromid either receives marketing approval in the relevant country or its development is discontinued.

"The proposed extension phase will allow us to gather important long term safety and efficacy data of ezutromid in patients with DMD that we believe will comprise part of a data package necessary for future applications for regulatory approval of ezutromid," **Ralf Rosskamp, MD, Chief Medical Officer of Summit commented.** *"While the PhaseOut DMD trial continues with the aim of establishing proof of concept for ezutromid, we are pleased to see that the DMC supports the trial's extension based on their review of the safety and tolerability data to date, which includes data from patients dosed over longer periods of time than have previously been tested."*

In addition to the extension phase of the trial, the regulatory submissions also include the addition of a safety arm to allow for the enrolment of patients who have previously taken part in Phase 1 clinical trials of ezutromid, but who did not meet the inclusion criteria for PhaseOut DMD. Although regulatory approval for the safety arm is being sought, the timing for inclusion of these patients into the trial is to be determined.

About PhaseOut DMD

PhaseOut DMD aims to provide proof of concept for ezutromid and utrophin modulation by measuring muscle fat infiltration, as well as by measuring utrophin protein and muscle fibre regeneration in muscle biopsies. The primary endpoint of the open-label trial is the change from baseline in magnetic resonance imaging parameters related to fat infiltration and inflammation of the leg muscles. Exploratory endpoints include the six-minute walk distance, the North Star Ambulatory Assessment and patient reported outcomes. PhaseOut DMD is a 48-week open-label trial expected to enrol up to 40 boys ranging in age from their fifth to their tenth birthdays at sites in the UK and the US. Each patient will receive two biopsies, one at baseline and the second either at 24 or 48 weeks. Further information is available at: https://clinicaltrials.gov/ct2/show/NCT02858362 and www.utrophintrials.com.

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, ezutromid, is an orally administered, small molecule. DMD is an orphan disease, and the US Food and Drug Administration ('FDA') and the European Medicines Agency have granted orphan drug status to ezutromid. Orphan drugs receive a number of benefits including additional regulatory support and a period of market exclusivity following approval. In addition, ezutromid has been granted Fast Track designation and Rare Pediatric Disease designation by the FDA.

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).

For more information, please contact:

Summit
Glyn Edwards **/** Richard Pye (UK office) — Tel: +44 (0)1235 443 951
Erik Ostrowski / Michelle Avery (US office) — +1 617 225 4455

Cairn Financial Advisers LLP
(Nominated Adviser)
Liam Murray / Tony Rawlinson — Tel: +44 (0)20 7213 0880

N+1 Singer
(Broker)
Aubrey Powell / Lauren Kettle — Tel: +44 (0)20 7496 3000

MacDougall Biomedical Communications
(US media contact)
Chris Erdman / Karen Sharma — Tel: +1 781 235 3060
cerdman@macbiocom.com
ksharma@macbiocom.com

Consilium Strategic Communications
(Financial public relations, UK)
Mary-Jane Elliott / Sue Stuart /
Jessica Hodgson / Lindsey Neville — Tel: +44 (0)20 3709 5700
summit@consilium-comms.com

Forward-looking Statements
Any statements in this press release about Summit's future expectations, plans and prospects, including but not limited to, statements about the clinical and preclinical development of Summit's product candidates, the therapeutic potential of Summit's product candidates, and the timing of initiation, completion and availability of data from clinical trials, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation of future clinical trials, availability and timing of data from on-going and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials or preclinical studies will be indicative of the

results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for Summit's foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of filings that Summit makes with the Securities and Exchange Commission including Summit's Annual Report on Form 20-F for the fiscal year ended January 31, 2016. Accordingly readers should not place undue reliance on forward looking statements or information. In addition, any forward looking statements included in this press release represent Summit's views only as of the date of this release and should not be relied upon as representing Summit's views as of any subsequent date. Summit specifically disclaims any obligation to update any forward-looking statements included in this press release.

This announcement contains inside information for the purposes of Article 7 of EU Regulation 596/2014 (MAR).

-END-