Summit Therapeutics plc

('Summit', or 'the Company')

SUMMIT COMPLETES ENROLMENT OF PhaseOut DMD, A PHASE 2 CLINICAL TRIAL OF EZUTROMID IN PATIENTS WITH DMD

- **Summit to Receive $22 Million Milestone Payment**

Oxford, UK, 15 May 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, today announces that it has completed enrolment in PhaseOut DMD, a Phase 2 proof of concept clinical trial of the utrophin modulator, ezutromid, in patients with DMD. PhaseOut DMD aims to provide proof of concept for ezutromid through measures of a number of endpoints related to muscle structure, health and function. The Company believes the trial could provide valuable insight into utrophin modulation as a potential disease-modifying treatment for all patients with DMD, regardless of the underlying dystrophin mutation.

With the dosing of the last patient in the trial, the Company has triggered a $22 million milestone payment as part of the Company's licence and collaboration agreement with Sarepta Therapeutics, Inc.

"Completing enrolment in PhaseOut DMD is a major step in the development of ezutromid as we seek to understand if this utrophin modulator has positive effects on muscle structure leading to changes in muscle health and function in patients with DMD," **said Dr David Roblin, Chief Operating Officer and President of R&D of Summit.** *"Utrophin modulation has potential as a universal treatment option for patients with DMD, and we look forward to the 24-week data readout expected in the first quarter of 2018."*

PhaseOut DMD is a 48-week open-label trial that has enrolled 40 patients at sites in the UK and the US. As part of the trial, each patient undergoes two biopsies, a baseline biopsy on enrolment and a second either at 24 or 48 weeks. In the first quarter of 2018, Summit expects to report 24-week biopsy analysis from approximately 20 patients, as well as 24-week MRI and functional data from all 40 patients enrolled in the trial. Top-line data from the complete 48-week trial are expected in the third quarter of 2018.

Further information about PhaseOut DMD 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 the Licence and Collaboration Agreement with Sarepta Therapeutics, Inc.

In October 2016, Summit announced a licence and collaboration agreement with Sarepta. This granted Sarepta exclusive commercial rights to the Company's utrophin modulator pipeline, including ezutromid, in

Europe, Turkey and the Commonwealth of Independent States, with an option over specific countries in Central and South America. Summit retains commercialisation rights in all other countries, including the US and Japan. Under the agreement, Summit received an upfront payment and is eligible to receive development, regulatory and sales milestones related to its utrophin modulator pipeline, including ezutromid, as well as royalties on net sales in the licensed territories. For further details, please refer to earlier RNS announcements and/or Summit's filings with the Securities and Exchange Commission.

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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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, Summit's license and collaboration agreement with Sarepta and the expected receipt of any milestone payments under the agreement, 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, 2017. 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).

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