# Summit Therapeutics plc

("Summit" or the "Company")

## SUMMIT COMPLETES INITIAL 24 WEEKS OF DOSING OF EZUTROMID IN PATIENTS WITH DMD IN PhaseOut DMD CLINICAL TRIAL

- **Summit Continues to Expect 24-week Data Q1 2018, which Could Provide Potential Proof of Mechanism for Ezutromid**

**Oxford, UK, 20 November 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 the completion of the initial 24-weeks of dosing of ezutromid in patients with DMD in the Company's Phase 2 clinical trial called PhaseOut DMD. With the achievement of this milestone, Summit continues to expect to report results from this initial 24-week dosing period in the first quarter of 2018.

The 24-week results are expected to include data from muscle biopsies, MRI and functional tests, as well as safety data.

*"With the completion of the 24-week dosing period, we remain on-track to conduct the planned biopsy, MRI and functional analyses and deliver the results for the initial dosing period during the first quarter of 2018,"* **commented Dr David Roblin, Chief Operating Officer and Medical Officer of Summit**. *"In the interim data readout, we aim to show a positive change following ezutromid treatment in muscle structure and health through the evaluation of muscle biopsies. This could potentially provide the first evidence of proof of mechanism for utrophin modulators in patients and give hope to those living with DMD."*

PhaseOut DMD is a 48-week open-label Phase 2 clinical trial that has enrolled 40 patients at sites in the UK and the US. The trial aims to establish proof of concept of ezutromid and is evaluating a range of muscle structure, muscle health and functional endpoints. As part of the trial, each patient has two muscle biopsies taken; a baseline biopsy on enrolment and a second either after 24 weeks or 48 weeks of dosing. Top-line data from the complete 48-week trial are expected in the third quarter of 2018. Following the 48 weeks of treatment, patients have the option to continue onto an extension phase. 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.

**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 had 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 drug. 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 programmes 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).

**Contacts**


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| **Summit**<br>Glyn Edwards **/** Richard Pye (UK office)<br>Erik Ostrowski / Michelle Avery (US office) | **Tel:** | 44 (0)1235 443 951<br>+1 617 225 4455 |
| **Cairn Financial Advisers LLP** (Nominated Adviser)<br>Liam Murray / Tony Rawlinson | **Tel:** | +44 (0)20 7213 0880 |
| **N+1 Singer** (Joint Broker)<br>Aubrey Powell / Jen Boorer | **Tel:** | +44 (0)20 7496 3000 |
| **Pamure Gordon** (Joint Broker)<br>Freddy Crossley / Duncan Monteith, Corporate Finance<br>Tom Salvesen, Corporate Broking | **Tel:** | +44 (0)20 7886 2500 |
| **MacDougall Biomedical Communications** (US)<br>Karen Sharma | **Tel:** | +1 781 235 3060<br>ksharma@macbiocom.com |
| **Consilium Strategic Communications** (UK)<br>Mary-Jane Elliott / Sue Stuart /<br>Jessica Hodgson / Philippa Gardner | **Tel:** | +44 (0)20 3709 5700<br>summit@consilium-comms.com |


**Summit Forward-looking Statements**

Any statements in this press release about the Company's future expectations, plans and prospects, including but not limited to, statements about the clinical and preclinical development of the Company's product candidates, the therapeutic potential of the Company's product candidates, the potential commercialisation of the Company's product candidates, the sufficiency of the Company's cash resources, the timing of initiation, completion and availability of data from clinical trials, the potential submission of applications for marketing approvals 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 ongoing 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, laws and regulations affecting government contracts, availability of funding sufficient for the Company's foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of filings that the Company makes with the Securities and Exchange Commission, including the Company's Annual Report on Form 20-F for the fiscal year ended 31 January 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 the Company's views only as of the date of this release and should not be relied upon as representing the Company's views as of any subsequent date. The Company 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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