Summit Therapeutics plc

('Summit', or 'the Company')

Summit Presents New 24-Week Analyses from PhaseOut DMD at the 2018 American Academy of Neurology Annual Meeting

- **Data show correlation between decrease in muscle damage and reduction in muscle inflammation in patients treated with ezutromid**

Oxford, UK, and Cambridge, MA, US, 20 April 2018 – Summit Therapeutics plc (NASDAQ:SMMT, AIM:SUMM) announces the presentation of new 24-week interim analyses from PhaseOut DMD, a Phase 2 open-label, multi-centre clinical trial of the utrophin modulator ezutromid in Duchenne muscular dystrophy ('DMD'), at the 70th American Academy of Neurology Annual Meeting ('AAN'). These new analyses showed a high correlation between reductions in developmental myosin, a biomarker of muscle damage which was measured by muscle biopsy, and reductions in muscle inflammation, which was measured by magnetic resonance, in patients after 24 weeks of ezutromid treatment. These findings underpin existing evidence that by modulating utrophin protein production, ezutromid is reducing the severity of DMD.

"The correlation observed between decreases in developmental myosin, a biomarker of muscle damage, and decreases in muscle fibre inflammation, is highly encouraging, and we believe further supports that ezutromid is breaking the DMD disease cycle of muscle damage and repair," **commented Dr David Roblin, Chief Medical Officer and President of R&D of Summit**. *"We look forward to reporting the full results of this trial, expected in the third quarter of 2018."*

The presentation was selected for the Emerging Science dual oral and poster presentation at AAN and authored by Professor Francesco Muntoni on behalf of the PhaseOut DMD Study Group, Gary Layton, Indranil Bhattacharya, Crystal Faelan, Anne C Heatherington, David Roblin, Jon Tinsley, and Professor Kay E Davies. A copy of the late-breaking presentation is available on Summit's website, www.summitplc.com.

About PhaseOut DMD

PhaseOut DMD is an open-label, multi-centre trial that has enrolled 40 patients in the US and UK, aged from their fifth to their tenth birthdays. PhaseOut DMD is 48 weeks in length after which patients have the option of enrolling into an extension phase and continuing to be dosed with ezutromid. The primary endpoint is the change from baseline in magnetic resonance parameters related to the leg muscles. Biopsy measures evaluating utrophin and muscle damage are included as secondary endpoints, with patients having two biopsies: one at baseline and their second after either 24 weeks or 48 weeks of ezutromid treatment. Exploratory endpoints include the six-minute walk distance, the North Star Ambulatory Assessment and patient reported outcomes. Top-line 48-week results are expected to be reported in the third quarter of 2018.

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 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) Erik Ostrowski / Michelle Avery (US office)	**Tel:** 44 (0)1235 443 951 +1 617 225 4455
Cairn Financial Advisers LLP (Nominated Adviser) Liam Murray / Tony Rawlinson	**Tel:** +44 (0)20 7213 0880
N+1 Singer (Joint Broker) Aubrey Powell / Jen Boorer	**Tel:** +44 (0)20 7496 3000
Panmure Gordon (Joint Broker) Freddy Crossley, Corporate Finance Tom Salvesen, Corporate Broking	**Tel:** +44 (0)20 7886 2500
MacDougall Biomedical Communications (US) Karen Sharma	**Tel:** +1 781 235 3060 ksharma@macbiocom.com
Consilium Strategic Communications (UK) Mary-Jane Elliott / Jessica Hodgson / Philippa Gardner	**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, the timing of initiation, completion and availability of data from clinical trials, the potential submission of applications for regulatory approvals, the sufficiency of Summit's cash resources, 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, 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, 2018. 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.

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