Summit Therapeutics plc

("Summit" or "the Company")

SUMMIT THERAPEUTICS PRESENTS PRECLINICAL DMD DATA AT THE 21ST INTERNATIONAL CONGRESS OF THE WORLD MUSCLE SOCIETY

- **Summit reports on development of new, automated biomarker tools to evaluate disease status of muscle biopsies**

Oxford, UK, 6 October 2016 – Summit Therapeutics plc (AIM: SUMM, NASDAQ: SMMT), the drug discovery and development company advancing therapies for Duchenne muscular dystrophy ('DMD') and *Clostridium difficile* infection, announces the presentation of preclinical data at the 21st International Congress of the World Muscle Society, which is taking place in Granada, Spain from 4-8 October 2016. The data include further findings from Summit's programme to develop tools to measure biomarkers of muscle health for use in patient clinical trials evaluating utrophin modulator therapies.

"Our approach to DMD aims to maintain production of utrophin, a protein typically found in young and repairing muscle fibres, so that it can replace the missing dystrophin protein in mature muscle fibres," **commented Dr Ralf Rosskamp, Chief Medical Officer of Summit**. *"The development of new, fully automated biomarker tools capable of evaluating the disease status of muscle biopsies will play an important role in clinical trials, including our ongoing PhaseOut DMD trial. In this trial, we seek to establish the potential of utrophin modulation, including our most advanced candidate ezutromid, as an effective treatment for all patients with this disease."*

The findings reported at WMS highlight progress in the development of fully automated quantification techniques for muscle biopsies capable of measuring structural proteins such as utrophin and biomarkers of muscle regeneration. The techniques described are immunohistochemical based assays and digital tissue image analysis tools that can robustly measure utrophin, beta-dystroglycan (a member of the dystrophin protein complex) and developmental myosin (a biomarker of muscle fibre maturity) in individual muscle fibres across a whole biopsy section.

Patients with DMD have higher levels of immature, regenerating muscle fibres because the absence of dystrophin leads to a continual cycle of muscle fibre damage and repair. By seeking to replace missing dystrophin with utrophin, utrophin modulators such as ezutromid aim to maintain the integrity of muscle fibres and allow them to mature. The automated approaches described at WMS make it feasible to look at associations between fibre maturity and integrity and utrophin expression in individual fibres. This provides a basis to distinguish new and beneficial expression of utrophin caused by a utrophin modulating drug, from the expression of utrophin seen generally during fibre regeneration. This is an important step in understanding and characterising the activity of these drugs.

This biomarker research is being conducted in partnership with Flagship Biosciences using their computational Tissue Analysis (cTA™) approach to accelerate precise muscle fibre biomarker quantification. This work builds on a recently published manual quantification approach that was developed by Summit and researchers at the Institute of Child Health, London with financial support from the charity Joining Jack. Further validation work to optimise the Flagship Biosciences' cTA™ approach is ongoing; it is expected the tools will be used in Summit's Phase 2 proof of concept trial of ezutromid, PhaseOut DMD.

Additional presentations at WMS 2016 reported preclinical data highlighting the potential of utrophin modulation as a disease-modifying treatment for all patients with DMD, regardless of the underlying fault in the dystrophin gene. This research was conducted at the University of Oxford under the UtroDMD alliance.

Copies of the presentations given at WMS 2016 are available from the 'Programmes' section of Summit's website, www.summitplc.com.

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.

Further information is available at: www.utrophintrials.com and https://clinicaltrials.gov/ct2/show/NCT02858362.

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).

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