Summit Therapeutics plc

('Summit' or 'the Company')

SUMMIT THERAPEUTICS RECOGNISES 10TH ANNUAL RARE DISEASE DAY

Oxford, UK, 28 February 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, recognises the tenth annual Rare Disease Day taking place today, 28 February 2017. The Rare Disease Day 2017 theme, 'with research, possibilities are limitless,' emphasises the importance of scientific research in helping to understand, diagnose and treat rare diseases that affect millions of people and their families worldwide.

Summit seeks to remain at the forefront of utrophin modulation research through its strategic alliance with the University of Oxford, under the guidance of Professor Kay Davies. The collaboration is focussed on developing future-generation utrophin modulators for the potential treatment of all patients with the progressive muscle wasting disorder, DMD. To date, the research team has identified two series of novel utrophin modulators, one of which has a mechanism of action potentially distinct from ezutromid, the Company's lead utrophin modulator that is in a Phase 2 clinical trial in DMD patients.

"In our quest to bring a potentially disease-modifying treatment to all patients with DMD, we have collaborated with the preeminent expert in utrophin modulation biology, Professor Kay Davies, and her research team at the University of Oxford," **said Glyn Edwards, Chief Executive Officer of Summit.** "*We applaud EURORDIS, the organisation representing rare disease patients in Europe, for bringing an annual spotlight to the plight of millions of people affected by rare diseases and in this year, recognising the immense impact that research is having and will continue to have for those living with rare diseases."*

In the European Union a rare disease is defined as one that affects fewer than 5 in 10,000 of the general population, while in the United States, it is defined as a disease that affects fewer than 200,000 people. There are between 6,000 and 8,000 known rare diseases with around five new rare diseases described in the literature each week. Rare diseases are often chronic and life threatening and include rare conditions, such as childhood cancers, and some other well-known conditions including cystic fibrosis and DMD.

Rare Disease Day takes place on the last day of February each year, and its objective is to raise awareness among the general public and decision-makers about rare diseases and their impact on patients' lives. Rare Disease Day was launched in Europe in 2008 by EURORDIS. It is now observed in more than 80 nations, and is sponsored in the US by the National Organization for Rare Disorders (NORD). For more information, please visit www.rarediseaseday.org.

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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