Summit Therapeutics plc

('Summit', or 'the Company')

SUMMIT JOINS cTAP IN COLLABORATIVE EFFORT TO ENHANCE THE DEVELOPMENT OF UTROPHIN MODULATORS AND OTHER TREATMENTS FOR DUCHENNE MUSCULAR DYSTROPHY

Oxford, UK, 25 September 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 it has joined the Collaborative Trajectory Analysis Project ('cTAP') to support cTAP's mission of accelerating the development of drugs to treat DMD through a coalition of Duchenne clinical experts, patient advocates and biopharmaceutical companies.

"In the Duchenne field, there is a strong community amongst the families, patient organisations, caregivers and industry that comes together to improve the lives of patients living with this disease, where time is of the essence," **said Dr David Roblin, President of R&D of Summit.** *"cTAP is leveraging the natural history and clinical data collected within this community to create more predictive models of disease progression with the aim of enabling companies to interpret data and improve the design of clinical trials in DMD. We believe this could potentially benefit the development of our utrophin modulators for the treatment of all patients with DMD. In addition, we have the opportunity to contribute our own data to aid others as we all seek to improve the lives of DMD patients and their families."*

Debra Miller, founder and CEO of CureDuchenne and initial funder of cTAP commented: *"cTAP was started with the single mission of helping biopharmaceutical companies to bring treatments to our children living with DMD as quickly as we possibly can. This mission is only made possible through a collaboration of clinicians, patient advocates and the biopharma industry, and we welcome Summit in this effort."*

Professor Eugenio Mercuri, Neurology and Pediatrics, Università Cattolica del Sacro Cuore, Rome, Italy, added: *"cTAP's initiative has brought together a wealth of data from clinicians and biopharmaceutical companies. This resource could assist Summit in developing its potentially universal treatments for patients with DMD, and in turn, Summit's data could contribute to the wider cTAP collaboration."*

Summit's lead utrophin modulator candidate for the treatment of DMD, ezutromid, is currently in a Phase 2 clinical trial called PhaseOut DMD. The trial aims to establish proof of concept of ezutromid through a range of muscle structure, muscle health and functional endpoints. The trial uses quantitative magnetic resonance to measure fat fraction in leg muscles as its primary endpoint. This technique is emerging as a tool to measure muscle health since the fat fraction increases as the disease progresses over time. In addition, the Company plans to measure utrophin and a biomarker of muscle regeneration (developmental myosin) from muscle biopsies provided at baseline and again at either 24 or 48 weeks of treatment. These measurements could provide early evidence of ezutromid's activity. Finally, PhaseOut DMD assesses functional measures, such as the six minute walk distance and North Star Ambulatory Assessment, as exploratory endpoints. Summit expects to report 24-week data in the first quarter of 2018, and data from the full 48-week trial are expected 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 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 cTAP
Driven by a shared mission to overcome the challenges of developing drugs for diseases characterized by heterogeneous progression, cTAP brings advanced data science to a dynamic alliance of all stakeholders in the ecosystem - a first in Duchenne. The Collaborative Trajectory Analysis Project, or cTAP, is enabling clinical experts to solve the most critical problems in drug development for Duchenne muscular dystrophy. The first community-wide coalition in Duchenne, cTAP has forged an alliance between clinical experts, drug companies developing therapies, patient advocacy organizations and collaborating registries and clinical centers across Europe and the US. cTAP brings advanced data science to the fight against Duchenne through a partnership with outcomes research experts at Analysis Group Inc. (http://www.analysisgroup.com/). cTAP is curating and growing what is already the largest natural history database of patient data in Duchenne. This rich resource enables cTAP to develop solutions with the urgency necessary to enhance clinical trial design and analysis, near-term. http://ctap-duchenne.org

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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