Summit Therapeutics plc

("Summit" or "the Company")

SUMMIT THERAPEUTICS PRESENTS ADDITIONAL DATA FROM PHASE 1B MODIFIED DIET CLINICAL TRIAL OF SMT C1100 IN DMD PATIENTS AT WMS

- **10 of 12 Patients Achieved Plasma Concentrations Expected to Increase Utrophin Levels by Approximately 30% or Greater**
- **Phase 2 Proof of Concept Trial Design Outlined, Expected to Initiate Q4 2015**
- **Preclinical Presentations Support Utrophin Modulation as Potential Treatment for all Patients with DMD**

Oxford, UK, 30 September 2015 – Summit Therapeutics plc (NASDAQ: SMMT, AIM: SUMM), the drug discovery and development company advancing therapies for Duchenne muscular dystrophy ('DMD') and *Clostridium difficile* infection, will today present detailed pharmacokinetic data from the Phase 1b modified diet clinical trial of SMT C1100 for the treatment of DMD, as well as preclinical data supporting both SMT C1100 and the broader utrophin modulator pipeline, at the 20th International Congress of the World Muscle Society ('WMS'). SMT C1100 is a utrophin modulator that the Company believes has potential to treat all patients with DMD.

"We believe utrophin modulation has significant potential as a universal treatment for DMD. For example, in preclinical studies, continual production of utrophin has resulted in disease-modifying effects in DMD models.Those preclinical findings, together with the detailed pharmacokinetic data from this recent trial, further our belief that SMT C1100 could have clinical benefit across all patients with DMD," **said Ralf Rosskamp, MD, Chief Medical Officer of Summit.** *"We are thrilled to have the opportunity to provide hope for the patients and families living with this devastating disease, and look forward to initiating a Phase 2 trial, which aims to provide proof of concept for SMT C1100 through measurements of muscle health and function, in the fourth quarter of this year."*

As previously reported, the Phase 1b modified diet trial met its primary objective. In this trial, increases in plasma absorption of SMT C1100 were observed in DMD patients who received SMT C1100 while following specific dietary guidance providing for a balanced diet of fats, proteins and carbohydrates. In the detailed pharmacokinetic data to be presented at WMS, ten of 12 patients achieved plasma exposure levels above 30 ng/ml for a mean of 14 hours in a 22-hour period, and six of those patients achieved plasma exposure levels above 67 ng/ml for a mean of 8.2 hours in the 22-hour period. These plasma levels correlate to an *in vitro* increase in utrophin levels of approximately 30% and 50%, respectively, in DMD myoblast cells and human myotubes. Since sustaining utrophin production is likely to be key in providing therapeutic benefit, the remaining two patients may also achieve some clinical benefit as they had plasma levels that exceeded 20 ng/ml. Summit's upcoming Phase 2 proof of concept trial will evaluate the potential benefit of SMT C1100 with longer-term dosing. Based on the detailed analysis of the Phase 1b modified diet trial, drug plasma exposure will not be used as an entry criterion for the Phase 2 proof of concept trial.

Summit plans to initiate its Phase 2 proof of concept trial of SMT C1100 in patients with DMD in the fourth quarter of this year. The 48-week open-label study is expected to enroll up to 40 patients between the ages of five and 10 years old at sites in Europe and in the US. Planned endpoints are expected to include changes in the fat content of muscle and in muscle inflammation as measured by magnetic resonance imaging ('MRI') of the leg muscles, measurements of utrophin protein and muscle fibre regeneration from muscle biopsies, and functional measures such as the six minute walk test and the North Star Ambulatory Assessment. The Company expects to report data from this trial periodically over the course of the study. The design and initiation of this study remain subject to regulatory approval. In addition, Summit continues to evaluate the timing and design for a larger, placebo-controlled trial of SMT C1100.

Additional Highlights from the WMS Presentations

SMT C1100 Clinical Development – Phase 1b Modified Diet Clinical Trial

- Seven of the 12 patients had higher plasma exposure of SMT C1100 after Day 14 compared to Day 1, which was not observed in previous studies of this drug.
- Six of the seven patients who had also participated in a previous Phase 1b study had increased plasma exposures of SMT C1100 in the modified diet trial ranging from 50% to nearly 400% with the modified diet compared to the previous trial; the seventh patient had plasma concentrations that exceeded 130 ng/ml for the entire final day of dosing in both Phase 1b trials.
- SMT C1100 was well-tolerated in all patients with no severe or serious adverse events and no discontinuations due to an adverse event.
- Patient compliance was 100%.

Biomarker Development Programme

- Presentation on biomarker development to automate the quantification of muscle fibre regeneration in muscle biopsies from patients with DMD and Becker muscular dystrophy ('BMD').
- Results show the ability to detect reduced rates of fibre regeneration in an automated fashion, potentially removing the uncertainty of manual grading of the muscle fibres. This was shown through an excellent correlation in fibres predicted to have lower fibre regeneration in BMD patients compared to dystrophin deficient DMD biopsies.
- Aim is to provide an automated technique for use in future utrophin modulator clinical trials.

Second Generation Utrophin Modulator Pipeline Development

- Preclinical data from the *in vivo mdx* mouse disease model showing that second generation utrophin modulators increase utrophin protein levels along the entire length of a muscle fibre and that this results in significantly improved muscle stability and reduction in levels of muscle fibre regeneration, necrosis and fibrosis.
- Second generation modulators show increased utrophin expression in the diaphragm and protection against muscle fibre damage and fibrosis in the *mdx* mouse disease model. This is a significant observation due to the disease pathology in the diaphragm of the *mdx* model closely resembling that of DMD patients.
- Data set supports the potential of utrophin modulation as a mechanism to treat DMD that is independent of the underlying genetic fault.

Copies of the posters are now available from the 'Programmes' section of Summit's website, www.summitplc.com.

About the Phase 1b Modified Diet Trial

The Phase 1b randomised, placebo controlled clinical trial was designed to evaluate the blood plasma levels of SMT C1100 in paediatric patients with DMD. Patients and their caregivers were provided with specific dietary guidance on recommended balanced proportions of fats, proteins and carbohydrates. The trial enrolled a total of 12 patients aged between 5 and 13 years, divided equally into three dose cohorts, at trial sites in the UK. Patients were randomised to three groups over 14-day treatment periods during which each patient received two different doses of SMT C1100 and a placebo control. There was a washout period of at least 14 days in between each of the treatment periods.

About Utrophin Modulation in DMD

DMD is a progressive muscle wasting disease that affects around 50,000 boys 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. Utrophin modulation has the potential to slow down or even stop the progression of DMD, regardless of the underlying dystrophin mutation. It is also expected that utrophin modulation could potentially be complementary to other therapeutic approaches for DMD.

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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