



**Summit Corporation plc**  
(*'Summit' or 'the Company'*)

**Summit Announces Initiation of Phase 1 Trial for the Treatment of Duchenne Muscular Dystrophy**

**Oxford, UK, May 25<sup>th</sup>, 2012**, Summit (AIM: SUMM), a UK drug discovery company, today announced that it has dosed the first cohort of patients in a Phase 1 study of SMT C1100 for the treatment of Duchenne Muscular Dystrophy (DMD), a fatal, rare genetic disease characterized by rapidly worsening muscle weakness. SMT C1100, an oral small molecule compound, is a potential disease-modifying drug that works to increase, or upregulate, the amount of a naturally occurring protein called utrophin.

"There is currently no known cure for DMD, and the only treatments available mask the symptoms of the disease," said Glyn Edwards, Chief Executive Officer of Summit. "SMT C1100 has the potential to modify the underlying disease, and the initiation of this Phase 1 trial represents a great step forward in bringing our breakthrough science to patients suffering from DMD. We expect to report top-line data from the full trial before the end of this year."

SMT C1100 has been extensively evaluated in non-clinical efficacy and safety studies and has demonstrated its ability to restore and maintain the function of muscles. This Phase 1 dose-escalating clinical trial in healthy volunteers will evaluate if the Company's aqueous formulation of SMT C1100 can provide the consistent levels of drug in blood that non-clinical efficacy studies predicted would be required to confer therapeutic benefit in DMD patients, while also assessing its safety and tolerability. A successful outcome from this trial is expected to lead to a Phase 2 trial of SMT C1100 in DMD patients.

The Phase 1 trial is being supported by \$1.5 million from a group of US-based DMD organisations: the Muscular Dystrophy Association, Charley's Fund, Cure Duchenne, the Foundation to Eradicate Duchenne, Nash Avery Foundation and Parent Project Muscular Dystrophy.

SMT C1100 is designed to upregulate and maintain the production of utrophin. Utrophin is a protein that is highly expressed in regenerating muscle, but decreases as the muscle fibre matures and is eventually replaced by dystrophin, a protein that maintains the integrity and healthy function of muscles. Patients with DMD are unable to make dystrophin, resulting in muscle fibre degeneration. However, if utrophin is continually expressed in the mature muscle fibre, it can replace the function of dystrophin and thereby overcome the deficit in patients with DMD. This approach is expected to be a universal treatment for all DMD patients regardless of whether the disease was caused by an inherited or spontaneous genetic mutation. Summit has demonstrated in non-clinical efficacy studies that SMT C1100 is capable of increasing utrophin to restore and maintain the healthy function of muscles.

**About Summit**

Summit is an Oxford, UK based drug discovery Company with an innovative Seglin™ technology platform for the discovery of new medicines and a portfolio of drug programme assets. Summit's programme portfolio consists of a number of drug programmes targeting high-value areas of unmet medical need including Duchenne Muscular Dystrophy and *C. difficile* infection. Summit is listed on the AIM market of the London Stock Exchange and trades under the ticker symbol SUMM. Further information is available at [www.summitplc.com](http://www.summitplc.com).

**Forward Looking Statements**

*This document contains "forward-looking statements" within the meaning of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as "anticipates", "intends", "plans", "seeks", "believes", "estimates", "expects" and similar references*

*to future periods, or by the inclusion of forecasts or projections. Forward-looking statements are based on the Company's current expectations and assumptions regarding our business, the economy and other future conditions. Because forward-looking statements relate to the future, by their nature, they are subject to inherent uncertainties, risks and changes in circumstances that are difficult to predict. The Company's actual results may differ materially from those contemplated by the forward-looking statements. The Company cautions you therefore that you should not rely on any of these forward-looking statements as statements of historical fact or as guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements and regional, national, global political, economic, business, competitive, market and regulatory conditions.*

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## Notes to Editors

### About DMD

Duchenne muscular dystrophy is a fatal genetic neuromuscular disorder that affects 1 in 3,500 boys with an estimated patient population of 50,000 in the developed world. The disease is caused by defects in the gene required to make dystrophin, a protein, which maintains the integrity and healthy function of muscles. One in three new cases is due to a spontaneous mutation where there is no familial history of the disease. The progressive muscle wasting begins in early childhood and typically leads to death in the twenties due to cardiac and respiratory failure. Currently there is no cure for the disease.

### About Utrophin Upregulation

Utrophin is a naturally occurring protein that has a similar function to dystrophin. Utrophin is produced during foetal muscle development but is switched off in mature muscle fibres. If its production could be switched back on, utrophin could act as a substitute for the missing dystrophin to maintain the healthy function of muscles. One method of turning utrophin production back on is through pharmacological means. Utrophin upregulation will be beneficial to all DMD patients regardless of their specific genetic mutation and is also expected to be complimentary to other therapeutic approaches in development.

### About SMT C1100

Discovered and developed by scientists at Summit, SMT C1100 has demonstrated its potential as a disease-modifying drug in non-clinical efficacy studies. SMT C1100 disengages normal utrophin control such that utrophin RNA and protein is made continually in muscle. It has received orphan drug designation in the US and Europe.

### About MDA Venture Philanthropy (MVP)

MVP is the Muscular Dystrophy Association's drug development program, which operates within MDA's translational research program. MVP is exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular diseases. For more information, visit [mda.org](http://mda.org) and follow MDA on Facebook ([facebook.com/MDANational](https://facebook.com/MDANational)) and Twitter (@MDAnews).

### About Charley's Fund, Cure Duchenne, Foundation to Eradicate Duchenne, and Nash Avery Foundation

Charley's Fund ([www.charleystfund.org](http://www.charleystfund.org)), Cure Duchenne ([www.cureduchenne.org](http://www.cureduchenne.org)), Foundation to Eradicate Duchenne ([www.duchennemd.org](http://www.duchennemd.org)), and Nash Avery Foundation ([www.nashaveryfoundation.org](http://www.nashaveryfoundation.org)) are independent organisations devoted to developing treatments for Duchenne muscular dystrophy. These groups, founded by parents of children with Duchenne, support the most promising research.

### About Parent Project Muscular Dystrophy

Parent Project Muscular Dystrophy (PPMD) is a national not-for-profit organization founded in 1994 by parents of children with Duchenne and Becker muscular dystrophy. Our mission is to end Duchenne. We accelerate research, raise our voices in Washington, demand optimal care for all young men, and educate the global community. PPMD is headquartered in Middletown, Ohio with offices in Fort Lee, New Jersey. For more information, visit [www.ParentProjectMD.org](http://www.ParentProjectMD.org).