

Summit Therapeutics plc

('Summit', or 'the Company')

SUMMIT HIGHLIGHTS DMD BIOMARKER VALIDATION DATA AND PHASE 2 CLINICAL TRIAL BASELINE CHARACTERISTICS IN PRESENTATIONS AT 22ND WMS CONGRESS

Oxford, UK, 4 October 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, announces that a series of presentations will be given by Summit and its collaborators at the 22nd International Congress of the World Muscle Society, which is taking place in Saint-Malo, France, from 3-7 October 2017.

The presentations include validation data of muscle biopsy biomarkers designed to assess utrophin modulator activity in clinical trials that have been developed in collaboration with Flagship Biosciences ('Flagship'), a leader in quantitative tissue-based biomarkers. In addition, baseline characteristics of patients enrolled into Summit's ongoing Phase 2 clinical trial, called PhaseOut DMD, will be presented.

"Developing new biomarkers capable of more reliably analysing muscle biopsies in clinical trials has been a major priority for Summit, and I am delighted with the progress made in our collaboration with Flagship. These automated techniques are capable of analysing thousands of muscle fibres in whole muscle biopsy sections, and we believe that their use could help us establish proof of mechanism for our utrophin modulator, ezutromid, in our ongoing PhaseOut DMD clinical trial," commented Dr David Roblin, Chief Operating and Medical Officer of Summit. "Summit is looking forward to an important period in our utrophin modulation programme: we expect to report the first 24-week biopsy, MRI and functional data from PhaseOut DMD in Q1 2018, with top-line data from the complete 48-week clinical trial expected in Q3 2018."

Dr David Young, CEO of Flagship Biosciences, added: *"We proudly support Summit's critical work towards a therapy potentially applicable to all patients with DMD with our advanced tissue analysis solutions. The progress we have made in developing these tools could advance the understanding of how patients are responding to treatment with ezutromid in this challenging rare disease."*

Posters P392, P393 and P469: Validation Data and Handling of Muscle Biopsy Biomarkers

Continuous utrophin expression by utrophin modulator therapies represents a potential disease modifying treatment for all patients with DMD, irrespective of their underlying genetic mutation. A series of presentations to be given at the WMS Congress details the development and validation of three biomarkers that allow for fibre identification, measurement of utrophin protein and measurement of muscle fibre regeneration.

The presentations detail an automated immunohistochemical analysis process that accurately and precisely quantifies utrophin protein and developmental myosin, a biomarker of muscle fibre immaturity, in whole section muscle biopsies each comprising several thousand individual fibres. High levels of consistency were observed between images from consecutive biopsy sections, and biopsy sections separated by 110µm or 150µm. High levels of consistency were also observed when the analysis of samples was performed within the same batch or performed over three days.

Use of these biomarkers requires taking biopsy samples of muscle from patients via a surgical procedure. Knowing how burdensome and precious these biopsies are for patients, Summit has developed a robust standardised practise to handle, process and ship muscle biopsy samples. This protocol builds on the processes developed by Sarepta Therapeutics Inc.

These muscle biopsy biomarker techniques and the biopsy handling protocol are being used in PhaseOut DMD.

Poster P403: PhaseOut DMD - Baseline Characteristics and MRI Measures

PhaseOut DMD is seeking to establish mechanistic proof of concept for the small molecule utrophin modulator ezutromid. The clinical trial design, endpoints and inclusion/exclusion criteria are presented. A total of 40 boys have been enrolled aged between their 5th and 10th birthdays and are being dosed with ezutromid twice daily for a total of 48 weeks. There is an optional extension phase at the end of this 48-week dosing period. The baseline characteristics of the enrolled patients, including age and body weight, are detailed, along with the baseline pharmacodynamics measures related to the magnetic resonance spectroscopy endpoint assessing muscle health by fat fraction percentage.

A further series of posters is being presented by the research group of Professor Kay Davies as part of Summit's strategic alliance with the University of Oxford. This includes reporting known serum biomarkers and emerging micro RNA biomarkers that are elevated in the dystrophin deficient *mdx* mouse disease model but found at close to normal levels in this disease model where utrophin is expressed continually (Posters P235 and P239). The continued development of future generation utrophin modulators is also presented (Poster P308).

A total of seven posters are being presented by Summit and its collaborators at WMS 2017 and these are now available for download from Summit's website. Details of the poster presentations are as follows:

Date & Time: 17:00-18:30 CET, Thursday, 5 October 2017

Session: 4

Abstract Number: P.392

Title: Analytical validation (based on CLIA & CLSI standards) of utrophin-laminin $\alpha 2$ and MHCd-laminin $\alpha 2$ duplex immunohistochemical assays using Computational Tissue Analysis (cTA™) for evaluation of Duchenne muscular dystrophy therapeutics

Authors: C. Faelan; J. Tinsley; A. Milici; S. Moore; J. Patterson-Kane

Date & Time: 17:00-18:30 CET, Thursday, 5 October 2017

Poster Session: 4

Abstract Number: P.393

Title: Computational alignment of duplex immunohistochemically-stained muscle sections in support of therapies for Duchenne muscular dystrophy

Authors: L. Cerkovnik; J. Patterson-Kane; K. Ryall; A. Milici; J. Tinsley; S. Moore; C. Faelan

Date & Time: 17:00-18:30 CET, Thursday, 5 October 2017

Session: 4

Abstract Number: P.403

Title: PhaseOut DMD: A Phase 2, proof of concept, clinical study of utrophin modulation with ezutromid

Authors: F. Muntoni; K. Maresh; K. Davies; S. Harriman; G. Layton; R. Roskamp; A. Russell; B. Tejura; J. Tinsley

Date & Time: 17:00-18:30 CET, Thursday, 5 October 2017

Session: 4

Abstract Number: P.469

Title: Collection of high quality muscle biopsies for use in DMD clinical trial analysis; process development and implementation

Authors: J. Tinsley; D. Frank; J. Dworzak; C. Faelan; J. Patterson-Kane; H. Wolff; F. Muntoni; PhaseOut DMD Study Group

Date & Time: 15:30-17:00 CET, Thursday, 5 October 2017

Session: 3

Abstract Number: P.235

Title: Circulating miRs biomarkers for therapeutic monitoring in utrophin based DMD therapy

Authors: N. Ramadan; S. Guiraud; B. Edwards; S. Squire; S. Hemming; K. Davies

Date & Time: 15:30-17:00 CET, Thursday, 5 October 2017

Session: 3

Abstract Number: P.239

Title: Identification of serum protein biomarkers for utrophin based DMD therapy

Authors: S. Guiraud; B. Edwards; S. Squire; A. Babbs; N. Shah; A. Berg; H. Chen; K. Davies

Date & Time: 15:30-17:00 CET, Thursday, 5 October 2017

Session: 3

Abstract Number: P.308

Title: Discovery of small molecule utrophin modulators for the therapy of Duchenne muscular dystrophy

Authors: G. Wynne; A. Vuorinen; E. Emer; D. Conole; M. Chatzopoulou; S. Davies; A. Russell; S. Guiraud; S. Squire; A. Berg; B. Edwards; S. Hemming; T. Kennedy; L. Moir; K. Davies; S. Harriman; J. Tinsley; F. Wilson

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 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 drug. 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 PhaseOut DMD Clinical Trial

PhaseOut DMD is a 48-week open-label Phase 2 clinical trial that has enrolled 40 patients at sites in the UK and the US. The trial aims to establish proof of concept of ezutromid and is evaluating a range of muscle structure, muscle health and functional endpoints. As part of the trial, each patient has two muscle biopsies taken, a baseline biopsy on enrolment and a second either at 24 or 48 weeks. In the first quarter of 2018, Summit expects to report 24-week biopsy analysis from approximately 20 patients, as well as 24-week MRI and functional data from all 40 patients enrolled in the trial. Top-line data from the complete 48-week trial are expected in the third quarter of 2018.

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).

For more information, please contact:

Summit

Glyn Edwards / Richard Pye (UK office)
Erik Ostrowski / Michelle Avery (US office)

Tel: +44 (0)1235 443 951
+1 617 225 4455

Cairn Financial Advisers LLP

(Nominated Adviser)
Liam Murray / Tony Rawlinson

Tel: +44 (0)20 7213 0880

N+1 Singer

(Broker)
Aubrey Powell / Lauren Kettle

Tel: +44 (0)20 7496 3000



MacDougall Biomedical Communications

(US media contact)
Karen Sharma

Tel: +1 781 235 3060
ksharma@macbiocom.com

Consilium Strategic Communications

(Financial public relations, UK)
Mary-Jane Elliott / Sue Stuart /
Jessica Hodgson / Philippa Gardner

Tel: +44 (0)20 3709 5700
summit@consilium-comms.com

Forward-looking Statements

Any statements in this press release about Summit's future expectations, plans and prospects, including but not limited to, statements about the clinical and preclinical development of Summit's product candidates, Summit's license and collaboration agreement with Sarepta and the expected receipt of any milestone payments under the agreement, the therapeutic potential of Summit's product candidates, the potential benefit and utility of using biomarkers to analyse muscle biopsies, and the timing of initiation, completion and availability of data from clinical trials, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation of future clinical trials, availability and timing of data from on-going and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials or preclinical studies will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for Summit's foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of filings that Summit makes with the Securities and Exchange Commission including Summit's Annual Report on Form 20-F for the fiscal year ended January 31, 2017. Accordingly, readers should not place undue reliance on forward-looking statements or information. In addition, any forward-looking statements included in this press release represent Summit's views only as of the date of this release and should not be relied upon as representing Summit's views as of any subsequent date. Summit specifically disclaims any obligation to update any forward-looking statements included in this press release.

-END-