

Summit Therapeutics plc (‘Summit’, the ‘Company’ or the ‘Group’)

SUMMIT OUTLINES PHASE 3 PROGRAMME FOR NOVEL CDI ANTIBIOTIC RIDINILAZOLE FOLLOWING FDA AND EMA REGULATORY MEETINGS

Oxford, UK, 1 February 2017 – Summit Therapeutics plc (AIM: SUMM, NASDAQ: SMMT), the drug discovery and development company advancing therapies for Duchenne muscular dystrophy and *C. difficile* infection (‘CDI’), today outlines its Phase 3 programme for its novel antibiotic, ridinilazole, following recent regulatory meetings with the US Food and Drug Administration (‘FDA’) and European Medicines Agency (‘EMA’). With input from the FDA and EMA, Summit intends to design the Phase 3 clinical programme to evaluate superiority of ridinilazole over standard of care in the treatment of CDI. A positive Phase 3 result on superiority has the potential to support the commercial launch of ridinilazole as a differentiated therapy that can both treat initial CDI and reduce disease recurrence.

Mr Glyn Edwards, Chief Executive Officer of Summit commented: *“The constructive end of Phase 2 meetings with the US and European regulators have enabled us to design a Phase 3 programme that focuses on evaluating ridinilazole’s superiority over standard of care. This is something we believe would help differentiate our novel class antibiotic from currently marketed CDI treatments and those in late-stage development. Superiority in the combined measure of treatment of initial infection and importantly, reduction in recurrence, could position ridinilazole for front-line treatment of CDI.”*

Summit discussed its Phase 3 development programme with the FDA at an End of Phase 2 meeting and through a scientific advice process with EMA. With input from both agencies, the Phase 3 programme is expected to include two trials evaluating ridinilazole as compared to the standard of care, vancomycin, each of which would enrol approximately 700 patients with CDI with the primary endpoint being superiority in sustained clinical response (‘SCR’). Other planned endpoints will include health economic outcome measures. The Phase 3 trial designs are consistent with the successful proof of concept Phase 2 trial, CoDIFy, in which ridinilazole achieved statistical superiority over vancomycin in SCR. SCR is a combined endpoint that measures cure at the end of treatment and a lack of recurrence in the 30 days after treatment. FDA also confirmed that ridinilazole would be eligible for Priority Review based on its QIDP designation.

Mr Edwards continued: *“As we continue to evaluate our options to maximise the value of ridinilazole, our stronger financial position following the DMD programme partnership with Sarepta Therapeutics, Inc. means Summit has more time to fully explore all options. These include potentially entering into a collaboration with a third party or securing meaningful non-dilutive funding from government and charitable organisations. In parallel, activities to prepare ridinilazole for Phase 3 trials continue with these anticipated to start in the first half of 2018.”*

About *C. difficile* Infection

C. difficile infection is a serious healthcare threat in hospitals, long-term care homes and increasingly the wider community with over one million estimated cases of CDI each year in the United States and Europe. It is caused by an infection of the colon by the bacterium *C. difficile*, which produces toxins that cause inflammation and severe diarrhoea, and in the most serious cases can be fatal. Patients typically develop CDI following the use of broad-spectrum antibiotics that can cause widespread damage to the natural gastrointestinal (gut) flora and allow overgrowth of *C. difficile* bacteria. Existing CDI treatments are predominantly broad spectrum antibiotics, and these cause further damage to the gut flora and are associated with high rates of recurrent disease. Recurrent disease is the key clinical issue as repeat episodes are typically more severe and associated with an increase in mortality rates and healthcare costs. The economic impact of CDI is significant with one study estimating annual acute care costs at \$4.8 billion in the US.

About Ridinilazole

Ridinilazole is an orally administered small molecule antibiotic that Summit is developing specifically for the treatment of CDI. In preclinical efficacy studies, ridinilazole exhibited a narrow spectrum of activity and had a potent bactericidal effect against all clinical isolates of *C. difficile* tested. In a Phase 2 proof of concept trial in CDI patients, ridinilazole showed statistical superiority in sustained clinical response

('SCR') rates compared to the standard of care, vancomycin. In this trial, SCR was defined as clinical cure at end of treatment and no recurrence of CDI within 30 days of the end of therapy. Ridinilazole has received Qualified Infectious Disease Product ('QIDP') designation and has been granted Fast Track designation by the US Food and Drug Administration. The QIDP incentives are provided through the US GAIN Act and include an extension of marketing exclusivity for an additional five years upon FDA approval.

About Summit Therapeutics

Summit is a biopharmaceutical company focused on the discovery, development and commercialization 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](https://twitter.com/summitplc)).

For more information, please contact:

Summit Therapeutics

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)
Chris Erdman / Karen Sharma

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

Consilium Strategic Communications

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

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

Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including statements about development and potential commercialisation of our product candidates, the therapeutic potential of our product candidates, the timing of initiation, completion and availability of data from clinical trials, the potential benefits and future operation of the collaboration with Sarepta Therapeutics Inc., including any potential future payments thereunder, any other potential third-party collaborations and expectations regarding the sufficiency of our cash balance to fund operating expenses and capital expenditures, 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 ongoing 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 will be indicative of the results of later clinical trials, expectations for regulatory approvals, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements and other factors discussed in the "Risk Factors" section of filings that we make with the Securities and Exchange Commission, including our Annual Report on Form 20-F for the fiscal year ended 31 January 2016. In addition, any forward-looking statements included in this press release represent our views only as of the date of this release



and should not be relied upon as representing our views as of any subsequent date. We specifically disclaim any obligation to update any forward-looking statements included in this press release.

This announcement contains inside information for the purposes of Article 7 of EU Regulation 596/2014 (MAR).

- END -