



Sarepta Therapeutics and Summit Enter Into Exclusive License and Collaboration Agreement for European Rights to Summit's Utrophin Modulator Pipeline for the Treatment of Duchenne Muscular Dystrophy

- Sarepta and Summit collaborate to advance the development of novel therapies for patients with Duchenne muscular dystrophy
- Summit receives \$40 million upfront, with potential future ezutromid-related milestone payments totalling up to \$522 million plus royalties
- Sarepta and Summit to share research and development costs
- Sarepta also receives option for Latin American rights

**Cambridge, MA, and Oxford, UK, 4 October 2016 –** Sarepta Therapeutics (NASDAQ: SRPT) and Summit Therapeutics plc (NASDAQ: SMMT, AIM: SUMM) today announced that they have entered into an exclusive license and collaboration agreement granting Sarepta rights in Europe, as well as in Turkey and the Commonwealth of Independent States ('the licensed territory'), to Summit's utrophin modulator pipeline, including its lead clinical candidate, ezutromid, for the treatment of Duchenne muscular dystrophy ('DMD'). As part of the agreement, Sarepta also obtains an option to license Latin American rights to Summit's utrophin modulator pipeline.

Utrophin modulation is a potential disease-modifying treatment for all patients with the fatal muscle wasting disease DMD, regardless of their underlying dystrophin gene mutation. Ezutromid is currently in a Phase 2 proof of concept trial called PhaseOut DMD.

"This partnership with Summit Therapeutics furthers our commitment to invest in innovative approaches to treating Duchenne and supports our common goal of improving the lives of patients with DMD," said Edward Kaye, M.D., Sarepta's Chief Executive Officer. "Summit's utrophin modulation technology represents a potentially promising approach to treat DMD, which may complement our current approach of exon skipping therapy."

"Sarepta Therapeutics has paved the way in the development of disease-modifying therapies for DMD with the first FDA-approved drug in this disease area, making them a strong strategic partner to support our utrophin modulator pipeline," commented Glyn Edwards, Chief Executive Officer of Summit. "This agreement provides us with access to Sarepta's development, regulatory and commercialisation expertise for the continued advancement of our promising utrophin modulator pipeline. We look forward to this partnership and working together to bring great advances to patients and families living with DMD."

Under the terms of the agreement, Summit will receive an upfront fee of \$40 million. In addition, Summit will be eligible for future ezutromid related development, regulatory and sales milestone payments totalling up to \$522 million, including a \$22 million milestone upon the first dosing of the last patient in Summit's PhaseOut DMD trial, and escalating royalties ranging from a low to high teens percentage of net sales in the licensed territory. Summit will also be eligible to receive development and regulatory milestones related to its next-generation utrophin modulators. Sarepta and Summit will share specified utrophin modulator-related research and development costs at a 45%/55% split, respectively, beginning in 2018. If Sarepta elects to exercise its option for Latin American rights, Summit would be entitled to additional fees, milestones and royalties.

Sarepta and Summit will host an update call for the Duchenne community on Monday, October 10 at 12:00 EDT. Details of the call can be accessed by visiting <u>http://www.parentprojectmd.org/communitycall</u>.

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

## About Utrophin Modulation in DMD

DMD is a progressive muscle wasting disease that 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. Summit believes that utrophin modulation has the potential to treat all patients with 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 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 programmes 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).

## **About Sarepta**

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. For more information, please visit us at www.sarepta.com.

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# Sarepta Forward-looking Statements

This press release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the terms of the license and collaboration agreement Sarepta has entered into with Summit (Oxford) LTD, including the rights, obligations and benefits of each party under the agreement such as Sarepta's payments associated with those rights to Summit; the potential of ezutromid and utrophin modulation as a disease-modifying treatment for all patients with DMD regardless of their dystrophin gene mutation; the potential benefits to the parties and the DMD community resulting from the agreement; the pattership between the parties furthering their common goal of improving the lives of patients with DMD; the potential of utrophin modulation technology to complement Sarepta's current approach of exon skipping therapy; Summit's plans to access Sarepta's expertise for the continued advancement of their promising utrophin modulator pipeline and working together to bring great advances to patients and families living with DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the license and collaboration and agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership between Sarepta and Summit may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreement, including any inability by Sarepta to fulfill its financial commitments to Summit; and even if the agreement results in commercialized products the parties may not achieve any significant revenues from the sale of such products.

Any of the foregoing risks could adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2015 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

## **Summit 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 potential benefits and future operation of the collaboration with Sarepta Therapeutics, including any potential future payments thereunder, clinical and preclinical development of Summit's product candidates, the therapeutic potential of Summit's product candidates, 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 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 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, 2016. 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.

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