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Santhera will hold a conference call today at 13:00 CEST, 12:00 BST, 07:00 EDT. Details are at the end of this statement.

Santhera to Discontinue Phase 3 SIDEROS Study and Development of Puldysa® in Duchenne Muscular Dystrophy (DMD) and Focus on Vamorolone

Pratteln, Switzerland, October 6, 2020 – Santhera Pharmaceuticals (SIX: SANN) announces the discontinuation of its Phase 3 SIDEROS study with Puldysa® (idebenone) in patients with Duchenne muscular dystrophy (DMD) who are in respiratory decline and receive concomitant glucocorticoid treatment. Data from an interim analysis conducted by the independent Data and Safety Monitoring Board (DSMB) concluded that the study was unlikely to meet its primary endpoint. As a consequence, Santhera will discontinue the study, withdraw the European marketing authorization application and end the global development program for Puldysa. The Company intends to initiate a restructuring plan for the business with a focus on retaining key functions for bringing DMD drug candidate vamorolone to patients and execute on its other pipeline programs.

Based on the now completed interim analysis which tested for efficacy, the DSMB has recommended the SIDEROS study be discontinued due to futility. The interim analysis was based on the primary endpoint of the study, the change of forced vital capacity % predicted (FVC%p) from baseline to 18 months of treatment. The outcome revealed that the probability of reaching the primary endpoint at the end of the study is too small to merit the continuation of the study. There were no safety concerns noted by the DSMB.

Santhera will stop the SIDEROS trial (including extension) and participants who are enrolled in the study will discontinue study medication and complete the study's follow-up evaluations. Furthermore, following up on the recommendation from the DSMB, Santhera will discuss the impact of ending the SIDEROS study on ongoing expanded access programs with the corresponding regulatory bodies.

"We would like to thank the patients and the families, as well as investigators and medical professionals, who participated in the SIDEROS study. Without their contributions we would not be able to advance DMD research," said **Dario Eklund, Chief Executive Officer of Santhera**. "While this is obviously not the outcome we expected, all our efforts in DMD will now be focused on progressing the promising drug candidate vamorolone which we recently licensed from ReveraGen to its next inflection point, the readout of 6-month topline data from the pivotal VISION-DMD study planned for the second quarter of 2021."

In connection with this decision, Santhera intends to start a restructuring process, aligning its operations to focus on progressing vamorolone for DMD, lonodelestat for cystic fibrosis and other lung diseases and its discovery-stage gene therapy approach for congenital muscular dystrophy.

Conference Call

Santhera will host a conference call today at 13:00 CEST / 12:00 BST / 07:00 EDT. Dario Eklund, CEO of Santhera, will discuss this update. Participants are invited to call one of the following numbers 10-15 minutes before the conference call starts (no dial-in code is required):

Europe: +41 58 310 50 00
UK: +44 207 107 06 13
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About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare neuromuscular and pulmonary diseases with high unmet medical need. Santhera has an exclusive license for all indications worldwide to vamorolone, a first-in-class anti-inflammatory drug candidate with novel mode of action, currently investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The clinical stage pipeline also includes lonodelestat (POL6014) to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases, as well as omigapil and an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed ex-North American rights to its first approved product, Raxone® (idebenone), for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Puldysa® and Raxone® are trademarks of Santhera Pharmaceuticals.

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