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Santhera's Marketing Authorization Application for Raxone® in Duchenne Muscular Dystrophy (DMD) Validated by the European Medicines Agency

Liestal, Switzerland, June 21, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that the European Medicines Agency (EMA) has validated its Marketing Authorization Application (MAA) for Raxone® for Duchenne muscular dystrophy (DMD) in patients with respiratory function decline who are not taking concomitant glucocorticoids. Validation confirms that the submission, which was filed as Type II variation of the Company's existing marketing authorization for Raxone, is complete and that the review process by the CHMP (Committee for Medicinal Products for Human Use) has begun. Santhera expects an opinion from the CHMP in the first quarter of 2017.

The regulatory dossier was submitted as a Type II Variation of the existing marketing authorization and is based on data from Santhera's phase II (DELPHI) program and the successful pivotal phase III (DELOS) study which enrolled patients not taking concomitant glucocorticoids. These data, which demonstrated a statistically significant and clinically relevant benefit of Raxone treatment in preserving respiratory function compared to placebo, have been substantiated by a natural history study showing that the benefit observed in the Raxone-treated group would not have been expected from the natural course of the disease. The MAA also includes recently published data that demonstrated that patients receiving Raxone in the DELOS trial had a reduced risk of bronchopulmonary complications including fewer hospitalizations caused by such complications and a reduced need for systemic antibiotic treatment compared to patients receiving placebo. The clinical relevance of these findings is supported by the outcome of a patient-centered benefit-risk survey which showed that DMD patients and their caregivers placed a high value on treatments that could reduce pulmonary complications.

"Validation of our submission by the EMA acknowledges the completeness of our dossier which combines data from our clinical program and supporting natural history data together establishing the clinical meaningfulness of Raxone treatment in DMD," commented **Thomas Meier**, PhD and CEO of Santhera. "Preservation of respiratory function is a major goal of DMD therapy and the initiation of the CHMP review of our MAA is an important milestone for patients with DMD who currently have no alternative treatment."

Raxone was approved in September 2015 for the treatment of visual impairment in adolescent and adult patients with Leber's hereditary optic neuropathy (LHON) in all EU member states, Norway, Iceland and Liechtenstein.

About Raxone® (Idebenone), Duchenne Muscular Dystrophy and DELOS

Duchenne muscular dystrophy (DMD) is one of the most common and devastating types of muscle degeneration and results in rapidly progressive muscle weakness. DMD is characterized by a loss of the protein dystrophin, leading to cell damage, impaired calcium homeostasis, elevated oxidative stress and reduced energy production in muscle cells. This results in progressive muscle weakness and wasting and early morbidity and mortality due to respiratory failure.

Idebenone is a synthetic short-chain benzoquinone and a cofactor for the enzyme NAD(P)H:quinone oxidoreductase (NQO1) capable of stimulating mitochondrial electron transport, reducing and scavenging reactive oxygen species (ROS) and supplementing cellular energy levels.

DELOS was a phase III, double-blind, placebo-controlled trial which randomized 64 patients, not taking concomitant glucocorticoids, to receive either Raxone tablets or matching placebo. The trial met its primary endpoint and demonstrated that Raxone can slow the loss of respiratory function and reduces bronchopulmonary complications. The positive outcome of the phase III DELOS study was first published in *The Lancet* (Buyse et al., The Lancet 2015 385(9979):1748-57) and in *Neuromuscular Disorders* (article in press http://dx.doi.org/10.1016/j.nmd.2016.05.008).

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative pharmaceutical products for the treatment of orphan mitochondrial and neuromuscular diseases. Santhera's lead product Raxone® is authorized in the European Union for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), the second indication for Raxone®, Santhera has filed a Marketing Authorization Application (MAA) in the European Union. Santhera develops Raxone® in a third indication, primary progressive multiple sclerosis (PPMS), and omigapil for congenital muscular dystrophy (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

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