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New Data from Santhera's Phase III Trial (DELOS) in Duchenne Muscular Dystrophy (DMD) in *Neuromuscular Disorders*

Raxone® Reduces Bronchopulmonary Complications in Patients with DMD

Liestal, Switzerland, June 1, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that additional data from the pivotal phase III trial (DELOS) were published online as an article in press in *Neuromuscular Disorders*, the official journal of the World Muscle Society (http://dx.doi.org/10.1016/j.nmd.2016.05.008). These data show that DMD patients treated with Raxone® (idebenone) have 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.

These results originate from additional analyses of the phase III DELOS trial which was performed in DMD patients not taking concomitant glucocorticoids and showed that Raxone® reduced the loss of respiratory function in a statistically significant and clinically relevant manner (Buyse et al., *The Lancet* 2015 385(9979):1748-57). The data now published in *Neuromuscular Disorders* demonstrated that Raxone also reduced the proportions of patients falling below clinically relevant thresholds for peak cough flow, relevant for proper airway clearance, and forced vital capacity, an indicator of respiratory failure. Researchers also found that there were fewer bronchopulmonary adverse events (BAEs), including upper and lower airway infections and related complications, in patients on Raxone therapy (6 of 31 patients with 7 BAEs) compared to patients in the placebo group (17 of 33 patients with 28 BAEs). For the patients receiving Raxone, there was a 67% reduction for the risk of experiencing at least one BAE during the 1-year study period ($p = 0.0187$), and a 72% reduction for the risk of experiencing one or more BAEs ($p = 0.0026$). Additionally, the overall BAE duration for the Raxone group (82 days) was markedly shorter than for the placebo group (222 days). This result was also supported by a smaller number of hospitalizations due to respiratory complications in the Raxone group compared to the placebo group. The need for systemic antibiotic use was also lower in the Raxone group (7 or 22.6% of patients with 8 episodes of antibiotic treatment) than in the placebo group (13 or 39.4% of patients with 17 episodes).

"The results published in *Neuromuscular Disorders* are clearly of clinical relevance," commented **Thomas Meier**, PhD, CEO of Santhera. "The data show that the efficacy of Raxone on the preservation of respiratory function, a major goal of DMD therapy, translates to a reduced risk of bronchopulmonary complications, which was associated with fewer hospitalizations caused by respiratory complications, and a reduced need for systemic antibiotics. The clinical relevance of these findings is supported by the outcome of the recent survey conducted by Parent Project Muscular Dystrophy (PPMD) and co-sponsored by Santhera, in which DMD patients and their caregivers placed a high value on treatments that could reduce pulmonary complications."

PPMD's Founding President & CEO, **Pat Furlong** was encouraged by the reported data: "PPMD congratulates Santhera on the latest update and publication. Pulmonary issues, and optimizing pulmonary care for people with Duchenne, has been a longtime priority for PPMD and is a significant part of our extensive care initiatives. Recently, with support from Santhera, we convened an incredible meeting of leading pulmonary experts to discuss the findings from our benefit-risk project and to map out next steps we can take to standardize pulmonary outcome measures collected in clinical trials and improve pulmonary care. We believe in the Santhera team and we are hopeful that Raxone will become one of the tools in the arsenal that will end Duchenne."

About 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, 10-18 years of age, to receive either Raxone tablets or matching placebo. Efficacy measurements included peak expiratory flow (PEF), forced vital capacity (FVC), forced expiratory volume (FEV1) and peak cough flow (PCF). The trial met its primary endpoint and demonstrated that Raxone can slow the loss of respiratory function in patients not taking concomitant glucocorticoids. 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).

About Parent Project Muscular Dystrophy (PPMD)

Parent Project Muscular Dystrophy (PPMD) is the largest most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne Muscular Dystrophy – their mission is to end Duchenne. PPMD invests deeply in treatments for this generation of people affected by Duchenne and in research that will benefit future generations. They advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. They demand optimal care, and strengthen, unite and educate the global Duchenne community. Everything PPMD does – and everything they have done since their founding in 1994 – helps people with Duchenne live longer, stronger lives. For more information, visit www.parentprojectmd.org.

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.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

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