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Santhera Receives Negative Opinion from the CHMP for its Marketing Authorization Application for Raxone® in DMD and Intends to Appeal this Opinion

Liestal, Switzerland, September 15, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a negative opinion for its Type II extension application for Raxone® (idebenone) in Duchenne muscular dystrophy (DMD). Santhera plans to appeal the opinion and will seek a re-examination by the CHMP.

Santhera gave its oral explanation to the CHMP on Wednesday this week to further support the clinical relevance of the existing data in the proposed indication. The CHMP expressed uncertainties whether the phase III DELOS trial provides sufficient evidence of efficacy to allow a Type II variation of Santhera's existing marketing authorization for Raxone.

"We are surprised and disappointed by the opinion of the CHMP. Data from the phase III DELOS trial demonstrated statistically significant and clinically relevant evidence that Raxone slows the decline of respiratory function, and reduces the risk of bronchopulmonary complications and hospitalization in patients with DMD not using glucocorticoids," said **Thomas Meier**, PhD, CEO of Santhera. "These patients in the respiratory decline stage currently have no treatment options, and because we are confident that they could benefit from treatment with Raxone, we plan to appeal this opinion and seek re-examination."

The application was filed as a Type II Variation of the existing marketing authorization for Leber's hereditary optic neuropathy (LHON), and is based on data from Santhera's phase II (DELPHI) study and the successful pivotal phase III (DELOS) study, the latter in patients not taking concomitant glucocorticoids. The outcomes of the phase III DELOS study were published in several peer-reviewed journals: Buyse et al., The Lancet 2015, 385:1748-1757; McDonald et al., Neuromuscular Disorders 2016, 26:473-480, Buyse et al., Pediatric Pulmonology 2017, 52:580-515 and Mayer et al., Journal of Neuromuscular Diseases 2017, 4: 189-198.

The intended indication for Raxone is to slow the decline of respiratory function in patients with DMD who are currently not taking glucocorticoids. The indication would include patients who were previously treated with glucocorticoids or in whom glucocorticoid treatment is not tolerated or is considered inadvisable.

About Duchenne Muscular Dystrophy

DMD is one of the most common and devastating types of muscle degeneration and leads to progressive muscle weakness starting at an early age. DMD is a genetic, degenerative disease that occurs almost exclusively in males with an incidence of up to 1 in 3,500 live male births worldwide.

About Idebenone in Duchenne Muscular Dystrophy

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, muscle wasting, 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 52-week study which randomized 64 patients, not taking concomitant steroids, to receive either idebenone (900 mg/day) or matching placebo. The study met its primary endpoint, the change from baseline in Peak Expiratory Flow (PEF), which demonstrated that idebenone can slow the loss of respiratory function.

Idebenone was well tolerated in the DELOS study, with overall incidence of adverse events being similar to placebo.

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® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), Santhera has filed a Marketing Authorization Application in the European Union and Switzerland for DMD patients with respiratory function decline who are not taking glucocorticoids. In collaboration with the U.S. National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing 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® is a trademark of Santhera Pharmaceuticals.

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