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Santhera Files Marketing Authorization Application in the European Union for Raxone® for the Treatment of Duchenne Muscular Dystrophy

Liestal, Switzerland, May 31, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that it has submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for Raxone® (idebenone) for the treatment of Duchenne muscular dystrophy (DMD) in patients with respiratory function decline and not taking concomitant glucocorticoids. The new indication was submitted as Type II variation of the company's existing marketing authorization for Raxone granted last year. Raxone has Orphan Drug Designation for DMD in the EU.

The regulatory dossier now submitted as Type II Variation of the existing marketing authorization summarizes data from Santhera's phase II (DELPHI) program and the successful pivotal phase III (DELOS) study which demonstrated a statistically significant and clinically relevant benefit of Raxone treatment in slowing the rate of respiratory function loss compared to placebo in patients with DMD not taking concomitant glucocorticoids. This benefit also translated to fewer bronchopulmonary events (e.g. airway infections), shorter event duration and less antibiotic treatment in the Raxone-treated patients. The data have been substantiated by a natural history study showing that the significant decline of respiratory function observed in the placebo group of DELOS reflected the natural course of the disease whilst the outcome for Raxone-treated patients did not. The MAA also includes data from a patient-centered benefit-risk survey which highlighted the importance of treating pulmonary disease in patients with DMD. The findings showing that Raxone slows the rate of respiratory function decline, decreases the proportion of patients crossing clinically relevant functional thresholds and increases the time until such thresholds are reached, represent an important treatment effect and are of major clinical relevance for patients with DMD. Raxone (900 mg/day) was safe and well tolerated with adverse event rates comparable to placebo.

The intended indication for Raxone is for patients with DMD in whom respiratory function has started to decline and who are currently not taking concomitant glucocorticoids. The indication would include patients who previously were treated with glucocorticoids or in whom glucocorticoid treatment is not desired, not tolerated or is contraindicated. Patients with respiratory function decline currently not using glucocorticoids account for approximately 40% of DMD patients above the age of 10 years. There is currently no treatment available for this group of DMD patients.

"Raxone provides clinically relevant benefit across multiple pulmonary function parameters as shown in our DELOS trial, the first and to date only double-blind, placebo-controlled phase III trial in DMD with a positive outcome", said **Thomas Meier**, PhD, CEO of Santhera. "We are now working diligently with European regulators to provide rapid access to Raxone for patients with respiratory function loss, who currently have no effective treatment options".

In the USA, Santhera has submitted comprehensive briefing material to the FDA in preparation for discussion on the filing of a New Drug Application (NDA) for idebenone under Subpart H for the same indication as in the MAA. Feedback from the FDA is currently expected by the end of July.

Santhera holds global commercialization rights to the DMD program which has been granted Orphan Drug Designation in the EU and the US and Fast Track Designation in the US. Patent protection extends until March 2026 (EU, Japan) and December 2027 (USA).

About Raxone® (Idebenone) as Treatment of Duchenne Muscular Dystrophy (DMD)

Duchenne muscular dystrophy (DMD) is one of the most common and devastating types of muscle degeneration and results in rapidly progressive muscle weakness. It is a genetic, degenerative disease that is inherited in an X-linked recessive mode with an incidence of up to 1 in 3,500 live born males worldwide. 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. 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 published in *The Lancet* (Buyse et al., The Lancet 2015 385(9979):1748-57).

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). Santhera develops Raxone® in two additional indications, Duchenne muscular dystrophy (DMD) and primary progressive multiple sclerosis (PPMS), and omigapil for congenital muscular dystrophies (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

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