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EMA Validates Santhera's Marketing Authorization Application for Puldysa® in Duchenne Muscular Dystrophy

Pratteln, Switzerland, June 25, 2019 – Santhera Pharmaceuticals (SIX: SANN) announces that the European Medicines Agency (EMA) has validated its marketing authorization application (MAA) for Puldysa® (idebenone) in the treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) who are not using glucocorticoids. Validation confirms that the submission, which was filed as conditional marketing authorization (CMA), is complete and that the review process by the EMA's Committee for Medicinal Products for Human Use (CHMP) has begun. Santhera expects an opinion by the CHMP around mid 2020.

"The initiation of the CHMP review of our application is an important milestone for patients with DMD who currently have no alternative treatment for the preservation of respiratory function. We are looking forward to working closely with the rapporteurs and CHMP during the review process to make Puldysa available to patients as soon as possible," said **Kristina Sjöblom Nygren, MD, Chief Medical Officer and Head of Development at Santhera**.

The MAA review follows the centralized procedure. If approved by the EMA, Puldysa will receive marketing authorization in all member states of the European Union, as well as in Norway, Liechtenstein and Iceland.

The EMA has granted orphan drug designation for idebenone in DMD.

About Duchenne Muscular Dystrophy

DMD is one of the most common and devastating types of progressive muscle weakness and degeneration starting at an early age and leading to early morbidity and mortality due to respiratory failure. It 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. 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. With age, progressive respiratory muscle weakness affecting thoracic accessory muscles and the diaphragm causes respiratory disease, impaired clearance of airway secretions, recurrent pulmonary infections due to ineffective cough, and eventually respiratory failure. There is currently no treatment approved for slowing loss of respiratory function in patients with DMD.

About Idebenone in Duchenne Muscular Dystrophy

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) expressed as percent of predicted, which demonstrated that idebenone can slow the loss of respiratory function.

Supportive data for idebenone were shown in the Phase II double-blind, placebo-controlled DELPHI study and its 2-year open-label extension study (DELPHI-E).

SYROS was a prospectively planned, retrospective collection of long-term respiratory function data from 18 patients who completed the DELOS study and subsequently received idebenone (900 mg/day) under Expanded Access Programs (EAPs). The SYROS study showed that the previously observed beneficial effect of idebenone in reducing the rate of respiratory function decline was maintained for up to six years during treatment.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein, Israel and Serbia for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.

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