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Swissmedic Accepts for Review Santhera's Marketing Authorization Application for Raxone® for the Treatment of Duchenne Muscular Dystrophy

Liestal, Switzerland, November 8, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that the Swiss Agency for Therapeutic Products (Swissmedic) has accepted for review Santhera's Marketing Authorization Application (MAA) for Raxone® (idebenone) for the treatment of Duchenne muscular dystrophy (DMD) in patients with respiratory function decline not taking concomitant glucocorticoids. Raxone was granted Orphan Drug Designation for DMD in Switzerland in 2012.

Santhera submitted a MAA to Swissmedic for Raxone in DMD in October 2016. Swissmedic has now confirmed that the dossier is sufficiently complete to permit substantive review. The standard assessment time is typically 15-18 months. The MAA for Raxone is already under review for the same indication by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA).

"This submission reflects our continued efforts to make Raxone available to patients and physicians beyond the European Union," said **Thomas Meier**, PhD, CEO of Santhera. "We look forward to Swissmedic's review of our application and to the approval of Raxone in Switzerland for DMD patients with respiratory function loss."

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. This indication would include patients who were previously treated with glucocorticoids or in whom glucocorticoid treatment is not desired, not tolerated or is contraindicated.

As part of the Swiss MAA, Santhera submitted data from its phase II (DELPHI) program and the successful pivotal phase III (DELOS) study, the results of which have been further substantiated by a comparative natural history study. Data from all studies demonstrate that Raxone slows the rate of respiratory function decline compared to untreated patients to a degree that is of major clinical relevance for patients with DMD. Raxone (900 mg/day) was safe and well tolerated with adverse event rates comparable to placebo.

In parallel to the already ongoing MAA reviews in Europe, Santhera continues to prepare for further discussions with the United States' Food and Drug Administration on the most appropriate regulatory pathway to approval in the US.

About Raxone® (Idebenone) in Duchenne Muscular Dystrophy

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 (900 mg/day) 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 statistically significant and clinically relevant outcomes of the phase III DELOS study were published: Buyse et al., *The Lancet* 2015, 385:1748-1757; McDonald et al. *Neuromuscular Disorders* 2016, 26: 473-480 and Buyse et al., *Pediatric Pulmonology* 2016: http://dx.doi.org/10.1002/ppul.23547.

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, Norway, Iceland and Liechtenstein 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. In collaboration with the US 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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