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Santhera's Idebenone (Raxone) Receives Orphan Drug Designation for Duchenne Muscular Dystrophy in Australia

Liestal, Switzerland, September 8, 2016 – Santhera Pharmaceuticals (SWX:SANN), announces today that the Australian Therapeutic Goods Administration (TGA) has granted orphan drug designation (ODD) to idebenone (Raxone) for the treatment of Duchenne muscular dystrophy (DMD). The product has already received ODD from European, Swiss and US authorities.

"We are delighted that the Australian authority has granted the orphan drug designation for Raxone in DMD," commented **Thomas Meier**, PhD, CEO of Santhera. "This decision underlines the need for a therapy for DMD and the potential role Raxone might have as an effective treatment. We are grateful to the DMD patient organization 'Save Our Sons-Duchenne Australia' which actively supported us in this successful application."

Orphan drug legislation is designed to encourage pharmaceutical companies to develop treatments for rare conditions. The TGA defines orphan diseases as affecting fewer than 2000 individuals in Australia. Orphan drug designation is an important first step in gaining regulatory approval and a drug with this designation has market exclusivity in Australia of up to five years as per the date of approval. The application for orphan drug designation was submitted on Santhera's behalf by TudorRose Consulting Ltd, which is acting as Santhera's legal sponsor in Australia.

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.

Following an exploratory Phase II trial (DELPHI), the safety and efficacy of Raxone (idebenone) was investigated in the confirmatory phase III, double-blind, placebo-controlled DELOS trial. DELOS 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. Results of the Phase II DELPHI trial were published by Buyse et al. *Neuromuscular Disorders* 2011, 21: 396–405 and *Pediatric Pulmonology* 2013, 48: 912–20. The positive outcome of the Phase III

DELOS study was published by Buyse et al., *The Lancet* 2015, 385:1748-1757 and McDonald et al., *Neuromuscular Disorders* 2016, 26: 473-480.

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) is currently assessing a Marketing Authorization Application (MAA) for Raxone in DMD patients with respiratory function decline who are 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. The MAA was submitted as a Type II variation of the company's existing marketing authorization for Raxone for the treatment of visual impairment in patients with Leber's hereditary optic neuropathy (LHON).

About TudorRose Consulting Pty Ltd

TudorRose Consulting Pty Ltd has been operating as a Regulatory and Quality consulting company, based in Melbourne, Australia since July 2010. The company is specialized in international product development and provides a broad range of services including Regulatory Affairs, QA, Project Management and Strategy. For further information, please visit the Company's website www.tudorroseconsulting.com.au.

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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