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Santhera Presents Data and Hosts Symposium on Respiratory Function Decline in Duchenne Muscular Dystrophy at the World Muscle Society Congress

Liestal, Switzerland, October 3, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces that it will present additional data from the positive Phase III DELOS trial in patients with Duchenne muscular dystrophy (DMD) at the 22nd International Congress of the World Muscle Society (WMS) in Saint Malo, France

"We are pleased to have the opportunity to showcase our research and to further facilitate discussion surrounding respiratory function decline in DMD patients and emerging treatment strategies at this year's WMS Congress," commented **Thomas Meier**, PhD, CEO of Santhera. "We also confirm that we have requested re-examination of the negative opinion from the CHMP regarding our marketing authorization application for Raxone® in DMD, as previously announced. The procedure will be completed in Q1 2018. We are convinced that Raxone offers therapeutic benefit in preserving respiratory function in teenage patients not taking glucocorticoids – a patient population with high unmet medical need. We remain dedicated to work with the patient community and regulators to make Raxone available to patients as soon as possible."

The following posters will be on display for the entire duration of the International Congress of the World Muscle Society, October 3 – 7, 2017, at the Palais du Grand Large in Saint Malo, France:

- *Consistency of efficacy of idebenone in respiratory decline in Duchenne muscular dystrophy (DMD): Comparison of analysis methods* (Poster P.408)
- *Meta-analysis of two clinical trials with idebenone in patients with Duchenne muscular dystrophy (DMD): Impact on respiratory decline* (Poster P.409)
- *Impact of idebenone on pulmonary morbidity, including bronchopulmonary adverse events, in Duchenne muscular dystrophy (DMD)* (Poster P.410)

On Thursday, October 5, from 02:00 - 03:30 p.m., the Company will also host a symposium entitled *Respiratory Function Decline in Duchenne Muscular Dystrophy (DMD) – New insights and evolving treatment strategies* featuring an expert panel consisting of:

- **Rosaline Quinlivan**, MD, Consultant, MCR Centre for Neuromuscular Disease Institute of Neurology at National Hospital, London and Consultant, Dubowitz Neuromuscular Centre, Great Ormond Street Hospital, London, UK

- **Craig McDonald**, MD, Professor and Chair, Department of Physical Medicine & Rehabilitation and Director of Neuromuscular Disease Clinics, UC Davis Health, USA
- **Oscar Henry Mayer**, MD, Associate Professor of Clinical Pediatrics, Perelman School of Medicine at the University of Pennsylvania, Division of Pulmonary Medicine Medical and Director of Pulmonary Function Testing Laboratory, The Children's Hospital of Philadelphia, USA
- **Gunnar Buyse**, MD, PhD, Pediatric Neurologist, University Hospital Leuven, and Full Professor of Medicine, University of Leuven, Belgium

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, 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 52-week study which randomized 64 patients, not taking concomitant glucocorticoids, to receive either idebenone (900 mg/day) or matching placebo. Positive outcomes of the DELOS study were previously 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.

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 another product – omigapil – for congenital muscular dystrophy (CMD), both also 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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