Santhera Pharmaceuticals Holding AG
Hammerstrasse 47
CH-4410 Liestal / Switzerland
Phone +41 61 906 89 50
Fax +41 61 906 89 51
www.santhera.com

Leading medical journal publishes treatment effect of Santhera's Raxone on inspiratory function in patients with Duchenne muscular dystrophy (DMD)

Liestal, Switzerland, September 13, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that additional data from the pivotal phase III trial (DELOS) demonstrating efficacy of Raxone (idebenone) on inspiratory function in patients with DMD were published online in the medical journal *Pediatric Pulmonology* (http://dx.doi.org/10.1002/ppul.23547).

The assessment of dynamic inspiratory function provides valuable information about the degree and progression of pulmonary involvement in patients with DMD. The data now published in *Pediatric Pulmonology* evaluated the effect of Raxone on the highest inspiratory flow generated during a forced vital capacity (FVC) maneuver (maximum inspiratory flow; V'I,max(FVC)). DMD patients in both treatment groups of the DELOS trial (Raxone, n=31; placebo: n=33) had comparable and abnormally low V'I,max(FVC) at baseline. During the study period, V'I,max(FVC) further declined by -0.29 L/s (liters/second) in patients on placebo (95% CI: -0.51, -0.08; p=0.008 at Week 52), whereas it remained stable in patients on Raxone (change from baseline to Week 52: 0.01 L/s; 95% CI: -0.22, 0.24; p=0.950). The between-group difference demonstrated a positive treatment effect for Raxone by 0.27 L/s (p=0.043) at Week 26 and 0.30 L/s (p=0.061) at Week 52. In addition, during the study period, the fraction of the maximum flow that is not used during tidal breathing, called Inspiratory Flow Reserve (IFR), improved by 2.8% in patients receiving Raxone and worsened by -3.0% among patients on placebo (between-group difference 5.8% at Week 52; p=0.040).

"These new data expand previously published results with Raxone from the successful phase III DELOS trial which enrolled patients not taking concomitant glucocorticoids," commented **Thomas Meier** PhD, CEO of Santhera Pharmaceuticals. "Since its completion, the DELOS Study Group has published data demonstrating a statistically significant and clinically relevant benefit of Raxone treatment on expiratory and inspiratory function, a reduced risk of patients treated with Raxone to experience bronchopulmonary complications and a reduced need for systemic antibiotics. These results clearly demonstrate an overall benefit for patients receiving Raxone on multiple pulmonary function outcomes, which is of relevance for patients with DMD."

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. The positive outcome of the Phase III DELOS study was so far published by Buyse et al., *The Lancet* 2015, 385:1748-1757 and McDonald et al., *Neuromuscular Disorders* 2016, 26: 473-480.

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.

For further information, contact:

Thomas Meier, PhD, Chief Executive Officer
Phone +41 61 906 89 64
thomas.meier@santhera.com

Christoph Rentsch, Chief Financial Officer
Phone +41 61 906 89 65
christoph.rentsch@santhera.com

Daniel Piller, Head Communications
Phone +41 61 906 89 26
daniel.piller@santhera.com

US investor contact:
Hans Vitzthum, LifeSci Advisors, LLC
Phone +1 212 915 2568
hans@lifesciadvisors.com

US Public Relations contact:
Deanne Eagle, Planet Communications
Phone +1 917 837 5866
deanne@planetcommunications.nyc

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