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Santhera Co-Sponsors and Presents at the First "Duchenne Pulmonary Outcomes Workshop" Organized by Parent Project Muscular Dystrophy (PPMD)

Liestal, Switzerland, April 14, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that it is co-sponsoring and will present clinical data at the "Duchenne Pulmonary Outcomes Workshop," organized by Parent Project Muscular Dystrophy (PPMD), the leading U.S. advocacy organization working to end Duchenne. The workshop takes place on April 14 and 15, 2016, in Bethesda, MD, and convenes over 50 experts from the US and Europe to discuss established and new methods for the assessment of pulmonary function in patients with Duchenne muscular dystrophy (DMD).

During the workshop, which is being co-chaired by **Jonathan Finder**, MD, Children's Hospital of Pittsburgh, and **Craig McDonald**, MD, UC Davis Health System, experts in the research and clinical care of DMD patients examine current and future assessments of pulmonary function. The workshop also summarizes available natural history data, addresses limitations of current pulmonary function tests, reviews correlations between pulmonary function measures and other clinical endpoints, and identifies clinically relevant efficacy thresholds for potential therapeutic interventions.

"Respiratory failure is one of the leading causes of early morbidity and mortality in Duchenne muscular dystrophy and pulmonary measures and outcomes are of key importance in developing treatments for DMD," commented **Thomas Meier**, PhD, Chief Executive Officer of Santhera. "We are delighted that PPMD is taking a leadership consensus-forming position by bringing experts together to advance the community's understanding in the assessment of pulmonary function changes in DMD patients. We are pleased to co-sponsor and to present data from our phase III DELOS trial, which demonstrated clinically relevant efficacy of idebenone (Raxone®) in preserving respiratory function."

About the PPMD Duchenne Pulmonary Outcomes Workshop
The workshop aims to consolidate the current knowledge amongst experts in DMD clinical care (including representatives of PPMD) about the use of pulmonary outcome measurements in disease monitoring, the need to implement novel pulmonary outcome measurements and their application during clinical development and routine care. The steering committee will publish a report on the outcome of the workshop.

About Duchenne Muscular Dystrophy and DELOS
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, 10-18 years of age, to receive either Raxone tablets or matching placebo. The trial met its primary end-point and demonstrated that Raxone can slow the loss of respiratory function in patients not taking concomitant glucocorticoid steroids. The positive outcome of the phase III DELOS study was published in *The Lancet* (Buyse et al., The Lancet 2015 385(9979):1748-57).

About Parent Project Muscular Dystrophy (PPMD)
Parent Project Muscular Dystrophy (PPMD) is the largest most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne Muscular Dystrophy – their mission is to end Duchenne. PPMD invests deeply in treatments for this generation of people affected by Duchenne and in research that will benefit future generations. They advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. They demand optimal care, and strengthen, unite and educate the global Duchenne community. Everything PPMD does – and everything they have done since their founding in 1994 – helps people with Duchenne live longer, stronger lives. For more information, visit www.parentprojectmd.org.

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 for the treatment of Leber's hereditary optic neuropathy (LHON). Santhera develops Raxone® in two additional indications, Duchenne muscular dystrophy (DMD) and 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.

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