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Santhera to Present Data and Host Symposium on Pulmonary Function in Duchenne Muscular Dystrophy at the American Thoracic Society 113th Annual Conference

Liestal, Switzerland, May 16, 2017 – Santhera Pharmaceuticals, a specialty pharmaceutical company focused on the development of innovative treatments for rare mitochondrial and neuromuscular diseases, announces that two posters with data from the positive phase III DELOS trial in patients with Duchenne muscular dystrophy (DMD) will be presented at the 113th American Thoracic Society (ATS) International Conference in Washington D.C.

Throughout the conference, which runs May 19-24, 2017, Santhera will host a clinical trial booth to inform clinicians on the new SIDEROS trial. The SIDEROS trial is a phase III, double-blind, randomized, placebo-controlled trial that evaluates the efficacy, safety and tolerability of idebenone in slowing the rate of respiratory function decline in DMD patients. Patients with reduced respiratory function on any stable glucocorticoid treatment regimen are eligible, regardless of their underlying dystrophin mutation or ambulatory status. More information is available on the new SIDEROS study website at www.siderosdmd.com.

Santhera will also facilitate a non-CME symposium featuring an expert panel consisting of:

- **Oscar H. Mayer, MD**, Associate Professor of Clinical Pediatrics Perelman School of Medicine at the University of Pennsylvania, Division of Pulmonary Medicine Medical Director, Pulmonary Function Testing Laboratory The Children's Hospital of Philadelphia
- **Erik Henricson, PhD, MPH**, Assistant Professor Associate Director for Clinical Research UC Davis Department of Physical Medicine & Rehabilitation Neuromuscular Research Center
- **Hemant Sawhani, MD**, Associate Professor, Division of Pulmonary Medicine, Section of Sleep Medicine Comprehensive Neuromuscular Program Cincinnati Children's Hospital Medical Center

Topics of the symposium include the pathophysiology of DMD and data around the importance of preserving respiratory function in DMD.

"Santhera is pleased to have the opportunity to showcase our focus on research to address pulmonary function decline in DMD patients at this year's ATS conference," said **Thomas Meier, PhD**, CEO of Santhera. "Pulmonary function decline is a serious medical complication in patients with DMD. However, it's often overlooked, particularly in the early stage of the disease. Our placebo-controlled phase III DELOS trial in 10 to 18 year old patients with respiratory function decline and not on steroids, met its primary endpoint, demonstrating that idebenone treatment slows pulmonary function decline. Santhera is proud to be leader in this important aspect of clinical DMD research."

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Event Details

Non-CME Symposium

Date: Tuesday, May 23, 6:30 - 9:30 p.m.

Location: Constitution Ballroom C-E (Constitution Level 3B)

Title: Duchenne Muscular Dystrophy (DMD): Importance of Preserving Pulmonary Function and Impact of Idebenone (An Investigational Treatment) in Boys with DMD

Speakers: Oscar H. Mayer, MD; Erik Henricson, PhD, MPH; Hemant Sawnani, MD

About: Please join us for an informative and interactive session on one of the most common and devastating types of muscle degeneration, Duchenne muscular dystrophy (DMD). This expert panel will review the pathophysiology of DMD, data from a DMD natural history study, and data around the importance of preserving pulmonary function in DMD.

Posters with data from phase III DELOS trial

Date: May 22, 9:15 - 4:15 p.m.

Session: B69 - WHEN KIDS CANNOT SLEEP (OR BREATHE)

Title: A4125 - Correlation Between Volume- and Flow-Related Pulmonary Function Outcomes in Patients with DMD

Location: Walter E. Washington Convention Center, Area F, Hall B-C

Session Label: Thematic Poster Session; **Poster Board Number:** P676

Authors: O. H. Mayer, MD; E. Henricson, PhD; M. Leinonen, MS; J. Karafilidis, PharmD; C. McDonald, MD; G. Buyse, MD, PhD

Date: May 23, 2:15 - 4:15 p.m.

Session: C105 - DISORDERS OF RESPIRATORY PHYSIOLOGY AND SLEEP IN CHILDREN

Title: A6875 - Effect of Idebenone on Bronchopulmonary Adverse Events and Hospitalizations in Patients with Duchenne Muscular Dystrophy (DMD)

Location: Walter E. Washington Convention Center, Room 201 (South Building, Level 2)

Session Label: Poster Discussion Session; **Poster Board Number:** 502

Authors: O. H. Mayer, MD; C. McDonald, MD; T. Meier, PhD; M. Leinonen, MS; G. Buyse, MD, PhD

About the SIDEROS Trial

SIDEROS is a phase III, double-blind, randomized, placebo-controlled trial with idebenone in 266 DMD patients receiving concomitant glucocorticoid steroids. Patients with declining respiratory function on any stable steroid treatment scheme and irrespective of the underlying dystrophin mutation or ambulatory status are eligible for enrolment. Study participants will receive either idebenone (900 mg/day; given as 2 tablets 3 times a day with meals) or placebo for 78 weeks (18 months). The primary endpoint of the trial is change from baseline to week 78 in forced vital capacity % predicted (FVC%p). Secondary endpoints include changes from baseline in % predicted peak expiratory flow (PEF%p), time to first 10% decline in FVC and change from baseline in inspiratory flow reserve. Patients completing the trial will be offered the opportunity to enroll in an open label extension study where all patients receive idebenone. The study will be conducted in 63 centers in the United States, Europe and Israel. Further information about the study is available under www.clinicaltrials.gov and www.siderosdmd.com.

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About Duchenne Muscular Dystrophy

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 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 52-week trial which randomized 64 patients, not taking concomitant steroids, to receive either idebenone (900 mg/day) or matching placebo. The trial met its primary endpoint which was a change from baseline in Peak Expiratory Flow (PEF) demonstrating that idebenone can slow the loss of respiratory function.

Idebenone was well tolerated in the DELOS trial with overall incidence of adverse events being similar to placebo. Most commonly reported adverse events were: diarrhea (idebenone 25% vs. 12% for placebo), constipation (idebenone 9% vs. 18% for placebo) and abdominal pain (idebenone 9% and 9% for placebo).

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>.

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) and the Swiss regulatory authority Swissmedic are currently assessing a Marketing Authorization Application (MAA) for idebenone under the name RAXONE® in DMD patients with respiratory function decline who are not taking concomitant steroids.

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 and Liechtenstein for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), Santhera has filed a Marketing Authorization Application (MAA) in the European Union and Switzerland for DMD patients with respiratory function decline who are not taking concomitant steroids. 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 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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