Santhera Pharmaceuticals Holding AG
Hohenrainstrasse 24, 4133 Pratteln, Switzerland
Phone: +41 61 906 89 50 | Fax: +41 61 906 89 51
www.santhera.com

Santhera to Present Long-term Efficacy Results with Idebenone in DMD at the 2019 MDA Clinical and Scientific Conference

Pratteln, Switzerland, April 15, 2019 – Santhera Pharmaceuticals (SIX: SANN) and its collaborating clinical experts present results of long-term efficacy on respiratory function outcomes in idebenone-treated patients with Duchenne muscular dystrophy (DMD) at the *2019 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference* held in Orlando, USA, April 13-17, 2019.

Posters presented at the MDA conference show consistent long-term efficacy data from two randomized placebo-controlled studies and their extended long-term data collections in patients treated with idebenone (Posters #7 and #9). In addition, new natural history data demonstrates the predictive value of slowing the rate of respiratory function decline on delaying time to initiation of assisted ventilation or death (Poster #11). Collectively, these data support the long-term potential of treatment with idebenone to delay the time to clinically relevant outcomes.

Poster #7: **"**SYROS study – long-term reduction in rate of respiratory function decline in patients with Duchenne Muscular Dystrophy (DMD) treated with idebenone"
Presenter: Oscar H Mayer, MD, pediatric pulmonologist and Director of the Pulmonary Function Laboratory at Children's Hospital of Philadelphia, USA

Poster #9: "Consistent long-term effect of idebenone in reducing respiratory function decline in advanced patients with Duchenne Muscular Dystrophy (DMD)"
Presenter: Oscar H Mayer, MD

Poster #11: "Crossing thresholds and changing rates of respiratory function decline are predictive of clinical outcomes in Duchenne Muscular Dystrophy (DMD)"
Presenter: Craig McDonald, MD, Professor and Chair, Department of Physical Medicine & Rehabilitation and Director of Neuromuscular Disease Clinics, UC Davis Health, USA

Time/place: Monday, April 15, 2019, 6pm – 8pm, during the *Networking and Poster Reception* in the Exhibit Hall (on display all day).

Additionally, the new data will be presented at a Santhera hosted *Industry Forum Lunch* by a panel of clinical experts:

Title: "Respiratory function decline in Duchenne muscular dystrophy: insights and evolving treatment strategies"
Presenters: Craig McDonald, MD and Oscar H Mayer, MD
Time/place: Tuesday, April 16, 2019, 12:30 – 1:45pm, Hall Florida B-C.

In February, Santhera announced topline results from the SYROS study which demonstrated that long-term treatment with idebenone consistently reduced the rate of respiratory function loss in patients with Duchenne muscular dystrophy (DMD) for up to 6 years in a real-world setting. This long-term data further supports the potential for idebenone to modify the course of respiratory function decline in DMD and thereby delay the time to clinically relevant milestones.

The posters can be viewed here and the findings from the SYROS study will be submitted for publication in a peer-reviewed journal.

About Duchenne Muscular Dystrophy

DMD is one of the most common and devastating types of progressive muscle weakness and degeneration starting at an early age and leading to early morbidity and mortality due to respiratory failure. It 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. 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. With age, progressive respiratory muscle weakness affecting thoracic accessory muscles and the diaphragm causes respiratory disease, impaired clearance of airway secretions, recurrent pulmonary infections due to ineffective cough, and eventually respiratory failure. There is currently no treatment approved for slowing loss of respiratory function in patients with DMD.

About Idebenone in Duchenne Muscular Dystrophy

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 steroids, to receive either idebenone (900 mg/day) or matching placebo. The study met its primary endpoint, the change from baseline in peak expiratory flow (PEF) expressed as percent of predicted, which demonstrated that idebenone can slow the loss of respiratory function. Supportive data for idebenone were shown in the Phase II double-blind, placebo-controlled DELPHI study and its 2-year open-label extension study (DELPHI-E).

SYROS was a prospectively planned, retrospective collection of long-term respiratory function data from 18 patients who completed the DELOS study and subsequently received idebenone (900 mg/day) under Expanded Access Programs (EAPs). The SYROS study showed that the previously observed beneficial effect of idebenone in reducing the rate of respiratory function decline was maintained for up to six years during treatment.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein, Israel and Serbia for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

For further information please contact:
public-relations@santhera.com or
Eva Kalias, Head External Communications
Phone: +41 79 875 27 80
eva.kalias@santhera.com

For Investors:
investor-relations@santhera.com or
Christoph Rentsch, Chief Financial Officer
Europe: +41 61 906 89 65
christoph.rentsch@santhera.com

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

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.

# # #