



Santhera Pharmaceuticals Holding AG

Hammerstrasse 49

4410 Liestal / Switzerland

Phone +41 61 906 89 50

Fax +41 61 906 89 51

www.santhera.com

Santhera Provides Update on Timeline for Application of Raxone® in Duchenne Muscular Dystrophy in Europe

Liestal, Switzerland, May 19, 2017 – Santhera Pharmaceuticals, a specialty pharmaceutical company focused on the development of innovative treatments for rare mitochondrial and neuromuscular diseases, announces an updated timeline for the ongoing assessment by the Committee for Medicinal Products for Human Use (CHMP) of its extension application for Raxone® (idebenone) in Duchenne muscular dystrophy (DMD).

"Santhera is in ongoing, constructive discussions with the CHMP, and we are now expecting to receive a request for supplementary information to further support the clinical relevance of our data. We are working closely with the CHMP to conclude the application process and anticipate an opinion in Q3 2017," said **Thomas Meier**, PhD, CEO of Santhera.

The application was filed as a Type II Variation of the existing marketing authorization and is based on data from Santhera's phase II (DELPHI) study and the successful pivotal phase III (DELOS) study, the latter in patients not taking concomitant glucocorticoids. This data demonstrated a statistically significant and clinically relevant benefit of Raxone in preserving respiratory function compared to placebo. This result is further substantiated by a natural history study, which shows that the benefits observed in the group treated with Raxone would not have been expected from the natural course of the disease.

The intended indication for Raxone is to slow the loss of respiratory function in patients with DMD who are currently not taking glucocorticoids. The indication would include patients who were previously treated with glucocorticoids or in whom glucocorticoid treatment is not desired, not tolerated, or is contraindicated.

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, early morbidity and mortality due to respiratory failure.

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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), which demonstrated that idebenone can slow the loss of respiratory function.

Idebenone was well tolerated in the DELOS study, with overall incidence of adverse events being similar to 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* 2017, 52:580-515.

The European Medicines Agency's CHMP and the Swiss regulatory authority Swissmedic are currently assessing a Marketing Authorization Application for idebenone under the name Raxone in patients with DMD with respiratory function decline who are not taking glucocorticoids.

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 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 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

Sue Schneidhorst, Head Group Communications
Phone +41 61 906 89 26
sue.schneidhorst@santhera.com

US investor contact

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

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