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Publication in European Heart Journal Highlights Potential of Santhera's SNT-MC17/Idebenone in Duchenne Muscular Dystrophy

Liestal, Switzerland, September 25, 2008 – Santhera Pharmaceuticals (SWX: SANN), a Swiss specialty pharmaceutical company focused on neuromuscular diseases, announces today the publication of study results in the European Heart Journal that support the potential efficacy of Santhera's lead compound SNT-MC17 (INN: idebenone) in Duchenne Muscular Dystrophy [1]. The authors concluded that early-initiated and long-term treatment of *mdx* mice, a well-established animal model for Duchenne Muscular Dystrophy, is cardioprotective and improves exercise performance. Santhera's recent Phase II proof-of-concept trial with SNT-MC17/idebenone in a population of young Duchenne Muscular Dystrophy patients showed positive efficacy on cardiac and respiratory functions. Meanwhile, the Company is in scientific discussions with clinical experts and health authorities from the European Union and the United States of America in preparation for a Phase III development program, the initiation of which is planned for the first half of 2009. Lead investigator, Prof. Gunnar Buyse (University Leuven, Belgium), will present data from the preclinical and clinical studies with SNT-MC17/idebenone in Duchenne Muscular Dystrophy at the upcoming 13th Congress of the World Muscle Society (September 29 to October 2 in Newcastle, UK).

The publication in the European Heart Journal reports on the results of a very long-term, blinded and placebo-controlled study in the *dystrophin* deficient *mdx* mouse, a disease relevant animal model. The main finding of the study is that presymptomatic-initiated and long-term idebenone treatment significantly prevented cardiac diastolic dysfunction, preserved cardiac systolic contractile reserve and, as such blocked the development of lethal acute heart failure during a dobutamine-mediated stress protocol, reduced cardiac inflammation and fibrosis, and improved voluntary running performance in the *mdx* mouse model.

In the authors' opinion the beneficial effects of idebenone can be explained by its ability to improve mitochondrial respiratory chain function and to reduce oxidative stress, pathways that have been implicated in the pathophysiology of *dystrophin* deficient muscular dystrophy. "We have identified a novel potential therapeutic strategy for human Duchenne Muscular Dystrophy, as SNT-MC17/idebenone was cardioprotective and improved exercise performance in the dystrophin-deficient *mdx* mouse", the authors concluded.

Thomas Meier, Santhera's Chief Scientific Officer, commenting on the European Heart Journal publication said: "Duchenne Muscular Dystrophy is a severe and still incurable disease. Heart failure is responsible for early death of a significant fraction of Duchenne Muscular Dystrophy

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patients. The reported cardioprotective data are in line with the evidence collected in our Phase II proof-of-concept trial and support SNT-MC17/idebenone as potential treatment option of Duchenne Muscular Dystrophy.”

Reference

[1] Gunnar M. Buyse, Gerry Van der Mieren, Michael Erb, Jan D'hooge, Paul Herijgers, Erik Verbeken, Alejandro Jara, An Van Den Bergh, Luc Mertens, Isabelle Courdier-Fruh, Patrizia Barzaghi, and Thomas Meier (2008). Long-term blinded placebo-controlled study of SNT-MC17/idebenone in the dystrophin deficient mdx mouse: cardiac protection and improved exercise performance. *European Heart Journal* (doi:10.1093/eurheartj/ehn406); Epub ahead of print 2008 Sep 10.

About Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy is the most common and a devastating type of muscular degeneration and results in rapidly progressive muscle weakness. It is a genetic, degenerative disease that is inherited in an X-linked recessive mode. Duchenne Muscular Dystrophy affects approximately 30,000 patients in the USA, EU, and Japan and its incidence is approximately 1 in 3,500 live born males worldwide. Duchenne Muscular Dystrophy is characterized by a loss of the protein *dystrophin*, leading to progressive muscle weakness and wasting through a complex cascade that involves impaired calcium homeostasis, mitochondrial dysfunction and oxidative stress. The average age of onset is between 3 and 5 years of age with a loss of ambulation in teenage patients. Dilated cardiomyopathy and respiratory failure are commonly associated with this chronic disease leading to early morbidity and mortality in Duchenne Muscular Dystrophy patients.

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About Santhera

Santhera Pharmaceuticals (SWX: SANN) is a Swiss specialty pharmaceutical company focused on the discovery, development and marketing of small-molecule pharmaceutical products for the treatment of severe neuromuscular diseases, an area of high unmet medical need which includes many orphan indications with no current therapy. Santhera currently investigates three compounds in six clinical-stage development programs. The Company's first product, SNT-MC17 (INN: idebenone), has received a marketing approval with conditions from Health Canada to treat Friedreich's Ataxia and will be marketed under its brand name CATENA®. The product is also under review by health authorities in the EU and in Switzerland, while in the United States, a pivotal Phase III trial is recruiting patients. The compound has also shown efficacy in a Phase II clinical trial as a potential treatment for Duchenne Muscular Dystrophy. For further information, please visit the Company's website www.santhera.com.

CATENA® is a trademark of Santhera Pharmaceuticals, registered in Canada and the United States.

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