



**Santhera Pharmaceuticals Holding AG**  
Hammerstrasse 47  
CH-4410 Liestal / Switzerland  
Phone +41 (0)61 906 89 50  
Fax +41 (0)61 906 89 51  
[www.santhera.com](http://www.santhera.com)

## **Santhera Presents Clinical Efficacy Data of SNT-MC17/idebenone in Duchenne Muscular Dystrophy at the American Academy of Neurology Annual Meeting**

**Liestal, Switzerland, April 11, 2008 – Santhera Pharmaceuticals (SWX:SANN), a Swiss specialty pharmaceutical company focused on neuromuscular diseases, announces today that the positive efficacy data from a 12-month Phase II clinical trial with SNT-MC17 (INN: idebenone) in Duchenne Muscular Dystrophy (DMD) are being presented at the 60th American Academy of Neurology (AAN) Annual Meeting in Chicago, first, in a poster session on April 15 and later, in a highlight session on April 17. The efficacy data on cardiac and respiratory function provide the basis for a planned pivotal clinical development program expected to start later in 2008.**

Prof Gunnar Buyse, principal investigator of the DELPHI trial, will present the results of this study (P02.115: "Double-Blind Randomized Controlled Trial of SNT-MC17/Idebenone in Duchenne Muscular Dystrophy"; Abstract 1736) at the 60th AAN Annual Meeting during the Poster Session on April 15 from 11:30 am to 2:30 pm. In addition, this paper has been selected by the organizing committee for inclusion in the session "Neuromuscular, EMG, and Autonomic Disorders Scientific Topic Highlights at the 60th AAN Annual Meeting" on April 17 from 6:00 to 7:00 pm. These are the first presentations of the full details of the efficacy data obtained in the DELPHI trial. Preliminary efficacy findings have already been announced in October 2007.

In the DELPHI trial patients on SNT-MC17/idebenone improved during the 52-week treatment period on the primary endpoint, peak systolic radial strain of the left ventricular (LV) inferolateral cardiac wall, the region of the heart that is most severely affected in DMD patients. Expressed as percent change from baseline, the patient group treated with SNT-MC17/idebenone improved by 104% which was significantly different from placebo treated patients who improved only by 29% ( $p=0.03$ ). In addition, peak systolic longitudinal strain of the LV lateral-mid cardiac region also improved significantly, indicating a beneficial effect of SNT-MC17/idebenone on early and systolic myocardial dysfunction in DMD.

Secondary outcome measures of this study also included respiratory function tests. Direct measures of respiratory weakness (peak expiratory flow, maximal inspiratory pressure) improved in patients on SNT-MC17/idebenone, indicating efficacy of SNT-MC17/idebenone to improve early signs of respiratory weakness and insufficiency. For example, peak flow expressed as percentage of the predicted value for patients on SNT-MC17/idebenone improved by 2.8% while patients on placebo deteriorated by 8.5% ( $p=0.042$ ).

Prof Gunnar Buyse, associate professor of pediatrics and child neurology at the University of Leuven and principal investigator of the study, comments: "These are the first indications of clinical efficacy of SNT-MC17/idebenone on functional cardiac and respiratory parameters in DMD. It is particularly encouraging to see that SNT-MC17/idebenone may protect from the potentially life-threatening complications in DMD patients. The positive results provide a clear basis for further clinical development studies to confirm the potential therapeutic benefit of SNT-MC17/idebenone for this devastating neuromuscular disease."

**About the DELPHI (Duchenne Efficacy Study In Long-Term Protocol Of High Dose Idebenone) trial**

The 12-month Phase II trial has evaluated the efficacy and tolerability of treatment with SNT-MC17/idebenone at a dose of 450mg/day compared to placebo in children with DMD. In total 21 patients, aged 8 to 16 years, with cardiac dysfunction were enrolled in the double-blind, randomized, place-controlled study conducted at the University of Leuven, Belgium. Thirteen patients received SNT-MC17/idebenone while eight patients were randomized to the placebo group. There were no drop-outs in the study and the compliance was very good. Importantly, there was no difference in the safety and tolerability of SNT-MC17/idebenone compared to placebo underlining the excellent safety profile of the compound in particular also in a pediatric population. Clinical efficacy of SNT-MC17/idebenone was demonstrated on functional cardiac and respiratory parameters (including the primary endpoint), that are sensitive markers of cardiac disease and respiratory insufficiency.

**About Duchenne Muscular Dystrophy (DMD)**

DMD 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. DMD affects approximately 30,000 patients in the USA, EU, and Japan and its incidence is approximately 1 in 3,500 live born males. Women can be carriers of DMD but usually exhibit no symptoms. DMD is characterized by a complete loss of the protein *dystrophin*, leading to progressive muscle weakness and wasting. 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 DMD 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. Santhera's vision is to become a leading specialty pharmaceutical company offering therapies for a number of indications in this area of high unmet medical need which includes many orphan indications with no current therapy.

Santhera currently has five clinical-stage development programs, three of which are investigating its lead compound, SNT-MC17 (INN: idebenone), for the treatment of Friedreich's Ataxia (FRDA), Duchenne Muscular Dystrophy (DMD) and Leber's Hereditary Optic Neuropathy (LHON). Another

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clinical program is investigating JP-1730 (INN: fipamezole) for the treatment of Dyskinesia in Parkinson's Disease (DPD) in cooperation with Juvantia, the compound's owner. The fifth program comprises SNT-317 (INN: omigapil) in Congenital Muscular Dystrophies (CMD), a compound in-licensed from Novartis. For the most advanced program, SNT-MC17 in FRDA, the Company has applied for marketing authorization in the EU, Switzerland and Canada. In the US, the compound is in Phase III clinical development for FRDA. The other clinical programs are in Phase II. For further information, please visit [www.santhera.com](http://www.santhera.com).

### **For further information, contact**

Klaus Schollmeier, Chief Executive Officer

Phone: +41 (0)61 906 89 52

[klaus.schollmeier@santhera.com](mailto:klaus.schollmeier@santhera.com)

Barbara Heller, Chief Financial Officer

Phone: +41 (0)61 906 89 54

[barbara.heller@santhera.com](mailto:barbara.heller@santhera.com)

Thomas Staffelbach, VP Public & Investor Relations

Phone: +41 (0)61 906 89 47

[thomas.staffelbach@santhera.com](mailto:thomas.staffelbach@santhera.com)

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