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Publication in LANCET Neurology Highlights Potential of Santhera's SNT-MC17 for Friedreich's Ataxia

Santhera Pharmaceuticals (SWX:SANN), a Swiss specialty pharmaceutical company focused on neuromuscular diseases, announces today the publication of data from the clinical trial conducted by the US National Institutes of Health (NIH) with Santhera's lead compound SNT-MC17 (INN: idebenone) in Friedreich's Ataxia (FRDA) [1]. The authors concluded that treatment with higher doses of study drug was generally well tolerated and associated with improvement in neurological function, measured by the International Cooperative Ataxia Rating Scale (ICARS), and Activities of Daily Living (ADL) in patients with FRDA. The degree of improvement correlated with the dose of SNT-MC17, suggesting that higher doses may be necessary to have a beneficial effect on neurological function. Data from this collaborative study were included in the filing of Santhera's Marketing Authorization Application (MAA) recently accepted by the European Medicines Agency (EMA).

The publication in LANCET Neurology reports an indication of dose-dependent improvement in total ICARS scores in the study population. In the authors' opinion the slowing of the progression of disease has been widely held as the most that could be expected of a treatment for FRDA. "If these results are confirmed, then improvement in neurological function may be possible for FRDA patients, particularly those treated early in the course of the disease", concluded the authors. The article further reported a change in neurological scores at higher doses relative to the baseline of 10 to 17%, which in the opinion of the authors is likely to be clinically meaningful.

Thomas Meier, Santhera's Chief Scientific Officer, commenting on the LANCET Neurology publication said: "We are excited that the data from this study are now available to a wider audience. The reported effect on the ICARS endpoint are of particular interest to us as we use this scale as a primary endpoint in our ongoing Phase III clinical program with SNT-MC17. Data from this trial analyzing a variety of neurological and cardiac outcome measures are part of our MAA file currently under review at the EMA."

Study details

48 genetically confirmed FRDA patients, aged 9 to 17 years, were enrolled in a 6-month, randomized, double-blind, placebo-controlled study. The patients received placebo or one of three doses of the drug. The primary endpoint was change from baseline in urinary 8-hydroxy-2'-deoxyguanosine (8OH2'dG), a marker of oxidative DNA damage. Secondary endpoints included changes in ICARS, the Friedreich Ataxia Rating Scale (FARS), and a survey of ADL.

Reference

[1] Nicholas A Di Prospero, Angela Baker, Neal Jeffries, Kenneth H Fischbeck: Neurological effects of high-dose idebenone in patients with Friedreich's ataxia: a randomised, placebo-controlled trial; published online September 7, 2007, in LANCET Neurology (<http://neurology.thelancet.com>) DOI:10.1016/S1474-4422(07)70220-X.

About Friedreich's Ataxia (FRDA)

Friedreich's Ataxia (FRDA) is a rare but severe genetic neuromuscular disorder that results in the degeneration of an individual's nerve and muscle tissue. This disorder causes loss of muscle control, uncoordinated movements, muscle wasting and thickening of heart walls which frequently leads to a shortened life span. FRDA affects both Caucasian males and females equally and it is estimated that about 20,000 patients suffer from the disease in both North America and Europe. Average life expectancy for FRDA patients is limited to approximately 35 to 50 years.

The disorder results from a genetic defect in the gene encoding for *frataxin*. Reduced levels of this protein ultimately result in impaired energy production in mitochondria, the cells' energy production centers, and elevated oxidative stress. Tissues that have the highest need for energy, in particular nerve and cardiac tissues, are primarily affected by *frataxin* deficiency resulting in pathological changes in heart muscle anatomy and function and loss of nerve cells. SNT-MC17 is believed to improve the balance and flow of electrons within the mitochondria, therefore increasing the energy production within nerve and muscle cells of FRDA patients, protecting these cells from cell death. A number of clinical trials have provided strong evidence that SNT-MC17 may offer an effective treatment option for FRDA associated heart wall thickening (cardiomyopathy). In addition, data from the collaborative NIH clinical trial suggest positive effects on neurological function.

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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 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. The most advanced program, SNT-MC17 in FRDA, is currently in Marketing Authorization Application process in Europe and in Phase III clinical development in the US while the other clinical programs are in Phase II. For further information, please visit www.santhera.com.

For further information, contact

Thomas Meier, Chief Scientific Officer
Phone: +41 (0)61 906 89 87
thomas.meier@santhera.com

Thomas Staffelbach, VP Public & Investor Relations
Phone: +41 (0)61 906 89 47
thomas.staffelbach@santhera.com

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