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## **Santhera and NIH Collaborate To Evaluate Catena® in Primary Progressive Multiple Sclerosis**

**Liestal, Switzerland, June 24, 2009 – Santhera Pharmaceuticals (SIX: SANN), a Swiss specialty pharmaceutical company focused on orphan neuromuscular diseases, announced today a collaboration with the US National Institutes of Health (NIH) to investigate Catena® as potential treatment of Primary Progressive Multiple Sclerosis (PPMS). An estimated 150,000 to 300,000 patients worldwide suffer from this rare but devastating form of Multiple Sclerosis. The Phase I/II trial consists of a one-year observational and a two-year interventional period.**

The IPPoMS (Idebenone in Patients with Primary Progressive Multiple Sclerosis) trial is a Phase I/II study with a 12-month pre-treatment baseline period followed by a double-blind, randomized, placebo-controlled treatment of 24 months duration investigating the safety and efficacy of one dose of Catena® (INN: idebenone) versus placebo. During the baseline period up to 80 patients will be enrolled to collect patient-specific biomarkers of disease progression as well as longitudinal neuroimaging and clinical data. Selection of the primary outcome measure as well as potential adjustments of the sample size will be based on the analysis of data from pre-randomization baseline period for the first 30 patients. The adaptive trial design allows for the selection of the most sensitive measures of tissue destruction of the central nervous system as key outcome parameters. In addition, this specific design was selected to reduce the number of patients usually required for clinical studies in MS.

The IPPoMS study is performed as collaboration between the US National Institute of Neurological Disorders and Stroke (NINDS) at the NIH and Santhera. Under the collaboration, the NIH will conduct the clinical trial while Santhera will supply study medication.

Bibiana Bielekova, Chief of the Neuroimmunological Diseases Unit at the NINDS and principal investigator of the study, declared: "PPMS is a debilitating disease for which no effective treatment is approved. Lack of demonstrated efficacy of immunomodulatory or immunosuppressive treatments suggests that alternative mechanisms, such as mitochondrial dysfunction and oxidative damage may underlie development of clinical disability in these patients. Previous studies have suggested that idebenone can increase the cellular energy production in the mitochondria and protect cells from oxidative stress. By conducting this study, we want to investigate the drug's potential as first efficacious therapy to treat patients suffering from PPMS."

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Thomas Meier, Chief Scientific Officer of Santhera, commented: "We are very pleased to be collaborating with Dr. Bielekova and her colleagues at the NIH in this intervention study in PPMS. There is a compelling scientific rationale that suggests that Catena® may protect from neuronal damage in patients suffering from PPMS. This is based on the principal mode of action of Catena® as an ATP production modulator and powerful antioxidant which is generally considered as safe and well tolerated in the dose that the NIH will use in the IPPoMS-study."

### **About Primary Progressive Multiple Sclerosis**

Multiple Sclerosis (MS) is an inflammatory and demyelinating disorder of the central nervous system. The majority of newly-diagnosed MS patients develop the Relapsing Remitting form of MS (RRMS), which is characterized by periods of neurological worsening followed by periods of spontaneous remission. About 10-15% of all MS sufferers develop a Primary Progressive Multiple Sclerosis (PPMS). This subform is characterized by a gradual progression of the neurological disability from its onset with no superimposed relapses or remissions. While the cause of MS remains unclear, accumulating data indicate that, especially in progressive stages, mitochondrial dysfunction and oxidative stress may play a major role, particularly in the pathogenesis of PPMS. Catena® has been shown to enhance the electron flow in the electron transport chain and increase cellular energy production. The drug is also active in scavenging reactive oxygen species thereby protecting the cell from oxidative stress. Catena® is crossing the brain-blood barrier and is readily taken up by cells. There are currently no treatments with proven therapeutic efficacy for PPMS, indicating a high unmet medical need for this devastating disease.

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### **About the National Institute of Neurological Disorders and Stroke**

The National Institutes of Neurological Disorders and Stroke (NINDS) is the nation's primary supporter of biomedical research on the brain and nervous system and a component of the National Institutes of Health (NIH). The NIH – the Nation's Medical Research Agency – is made up of 27 Institutes and Centers and is a component of the US Department of Health and Human Services. NIH is the primary federal agency for conducting and supporting basic, clinical and translational medical research, and it investigates the causes, treatments, and cures for both common and rare diseases. For more information about the NINDS and its programs visit [www.ninds.nih.gov](http://www.ninds.nih.gov); for more information about the NIH visit [www.nih.gov](http://www.nih.gov).

### **About Santhera**

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the discovery, development and commercialization 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's first product, Catena® to treat Friedreich's Ataxia is marketed in Canada. Data of a second pivotal Phase III trial in Europe are expected for the first half of 2010. The same compound has also shown efficacy in a clinical trial as a potential treatment for Duchenne Muscular Dystrophy. For further information, please visit the Company's Web site [www.santhera.com](http://www.santhera.com).

*Catena® is a trademark of Santhera Pharmaceuticals.*

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**National Institute of Neurological Disorders and Stroke**

For interviews with Dr. Bibiana Bielekova, please contact the NINDS Office of Communications and Public Liaison at +1 (301) 496-5924 or email [NINDSPressTeam@ninds.nih.gov](mailto:NINDSPressTeam@ninds.nih.gov).

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