

Santhera Reports Outcome of Exploratory Trial with Idebenone in PPMS Conducted at the NIH

Pratteln, Switzerland, March 5, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces that the exploratory Phase I/II clinical trial in primary progressive multiple sclerosis (PPMS) conducted as an investigator-initiated trial at the National Institutes of Health (NIH) confirms the safety profile of idebenone at a dose of 2,250 mg daily over a treatment period of two years. In assessing the efficacy of idebenone on disease progression, no difference between the active treatment group and placebo was observed.

The National Institute of Neurological Disorders and Stroke (NINDS), part of the US NIH, sponsored this investigator-initiated, double-blind, placebo-controlled Phase I/II trial (IPPoMS) investigating the safety and therapeutic efficacy of idebenone in PPMS. A total of 77 patients were randomized and 66 (idebenone: 33, placebo: 33) completed the trial which combined a one-year observational pre-treatment phase, followed by a two-year placebo-controlled intervention period. There was no difference in the occurrence and severity of adverse events between the treatment groups indicating that idebenone at a daily dose of 2,250 mg was well tolerated.

The primary outcome to explore the efficacy of idebenone was the change in the CombiWISE, a rating scale recently developed by **Bibiana Bielekova**, MD, and colleagues at the NINDS. Top-line analysis of the CombiWISE data and other clinical assessments and biomarkers (such as the disability progression scale EDSS-plus and changes in ventricular volume) indicate that there was no difference between treatment groups for measures of disease progression.

“The long-term study in patients with PPMS confirms the favorable safety profile of idebenone given at higher dose than the currently approved dose for Raxone® in Leber’s hereditary optic neuropathy,” said **Thomas Meier**, PhD, CEO of Santhera. “We thank the NIH team for conducting this long-term pilot study which will add to the knowledge of disease progression and data collection instruments. Clearly, the small sample size is a limitation when studying a therapeutic intervention in such a complex, relentlessly progressing neurological disease.”

About PPMS

Multiple sclerosis (MS) is an inflammatory and neurodegenerative disorder of the central nervous system that causes a wide range of physical symptoms, such as impaired movement, fatigue, numbness, and pins and needles, as well as problems with memory and understanding. In MS, the outer coating of nerve fibers (called myelin) is damaged, preventing the nerves from functioning properly. Primary progressive multiple sclerosis (PPMS) is the more aggressive of two main subtypes of MS and affects about 10-15% of all patients with MS. In remittent relapsing MS (RRMS), patients experience symptoms intermittently, with a slower accumulation of permanent disability than in PPMS. In PPMS, physical disability progressively worsens over time without symptom-free intervals.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. The most advanced pipeline product, idebenone, is in clinical Phase III for the treatment of Duchenne muscular dystrophy (DMD). Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and currently commercialized in 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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