Santhera Presents Positive Data from IONIA Open-Label Extension Study in Friedreich’s Ataxia at the Fourth International Friedreich’s Ataxia Scientific Conference

Liestal, Switzerland, May 5, 2011 – Santhera Pharmaceuticals (SIX: SANN) announced today that it will present positive data from an open-label extension study (IONIA-E) evaluating Catena® for the treatment of Friedreich’s Ataxia. The findings indicate that Catena® can offer therapeutic benefit to pediatric patients by improving overall neurological function, particularly fine motor skills, and speech. Data will be presented by the principal investigator of the study, Dr. David Lynch from Children’s Hospital of Philadelphia (CHOP) on May 7 2011 in Strasbourg, France at the 4th International Friedreich’s Ataxia Scientific Conference [1].

The 12-month IONIA-E study was an open-label extension study following the 6-month randomized controlled IONIA trial. The 68 participants enrolled into the IONIA-E study received high dose (1350/2250 mg/day for patients below/above 45 kg body weight) Catena®. Changes in the International Cooperative Ataxia Rating Scale (ICARS) and Friedreich’s Ataxia Rating Scale (FARS) total scores and subscores were evaluated during the total of 18 months period for the combined IONIA and IONIA-E studies. Throughout the combined treatment period, all ICARS subscores showed an improvement with the exception of posture and stance subscore. Patients improved with statistical significance for the eye (p<0.0001), speech (p<0.0001) and upper/lower limb ataxia (p<0.0001) ICARS subscores over the 18 months combined study period. The subgroup of patients who received high dose Catena® from the beginning of the IONIA study (N=22) significantly improved in neurological function over the 18 months of the combined study period (change in total ICARS –3.0 over baseline, p=0.014) suggesting a positive long-term benefit for patients suffering from this chronic disease. The ICARS total score for all patients combined (N=68) showed a mean change of –1.03 points (p=0.132) from baseline, indicating a trend for improvement in neurological function. Comparable data were obtained with the FARS.

“Patients participating in the combined 18-month study period showed an overall trend for functional improvement, which is in clear contrast to the expected natural rate of decline. Generally, the best efficacy of Catena® was observed on ICARS and FARS items addressing fine motor skills and speech,” said Thomas Meier, Chief Scientific Officer of Santhera. “These findings provide valuable longer-term efficacy data with Catena® in children affected by this chronic, progressive disease.”
In addition, Santhera will present the design and outcome measures of the randomized withdrawal study (PROTI) [2], the results from a patient survey on resource utilization [3] and an analysis of ICARS rating based on over 600 patients [4].

References

[1] Lynch D.R., Perlman S.L., Coppard N., Rummey C., Meier T.: Efficacy of Idebenone (Catena®) in Pediatric Patients with FRDA: Data from a 6-month Controlled Study (IONIA) Followed by a 12-month Open Label Extension Study (IONIA-E). Oral presentation given at the 4th International Friedreich’s Ataxia Scientific Conference in Strasbourg, France, from May 5 to 7, 2011.


About Friedreich’s Ataxia

Friedreich’s Ataxia is a devastating inherited disease associated with progressive neurodegeneration. The disorder is caused by mutations in the gene that encodes for frataxin. Lack of this protein impairs the energy production in the mitochondria, the energy production centers in each cell, and damages nervous and cardiac tissue.

First symptoms typically develop from around 5 to 15 years of age. Coordination difficulties such as unsteady gait, frequent falls or clumsiness usually appear first. Gait ataxia then spreads to the arms and the trunk. Speech is almost always affected, making communication increasingly difficult. Patients become wheelchair-bound and require continuous care. Cardiomyopathy is a common complication of Friedreich’s Ataxia and while it may be asymptomatic early on, it remains a leading cause of death.

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About Santhera
Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative pharmaceutical products for the treatment of severe neuromuscular and neurodegenerative diseases, an area of high unmet medical need which includes many orphan and niche indications with no current therapy. Santhera’s first product, Catena®, to treat Friedreich’s Ataxia is marketed in Canada. For further information, please visit www.santhera.com.

Catena® is a trademark of Santhera Pharmaceuticals.

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