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Santhera's US Phase III IONIA Trial in Friedreich's Ataxia Misses Primary Endpoint

Data from second Phase III study expected in the first half of 2010

Liestal, Switzerland, May 19, 2009 – Santhera Pharmaceuticals (SIX: SANN), a Swiss specialty pharmaceutical company focused on orphan neuromuscular diseases, announced today that its US Phase III clinical trial evaluating Catena® for the treatment of Friedreich's Ataxia missed its primary endpoint as measured by the International Cooperative Ataxia Rating Scale (ICARS). The study also did not show statistical significance on the second neurological endpoint, the Friedreich's Ataxia Rating Scale (FARS). On both endpoints, the active treatment arms showed a consistent improvement over baseline and placebo, as seen in prior studies. However, due to a lower than expected effect size combined with the fact that patients on placebo improved unexpectedly, statistical significance could not be achieved in this study population. The safety results were consistent with published data suggesting that Catena® is safe and well tolerated at doses up to 2250 mg/day.

The primary endpoint in the IONIA (Idebenone effects On Neurological ICARS Assessments) study compared the effect at six months of two treatment arms with placebo on the baseline ICARS score. For both treatment arms, patients on Catena® improved on average by 2.4 points on the ICARS scale at six months over baseline. This is about half of the improvement seen in the prior US Phase II study named NICOSIA (NIH Collaboration With Santhera In Ataxia) which was used to design the IONIA study. Patients on Catena®, however, improved by only 1.2 points over placebo, because patients on placebo did not deteriorate to the extent expected from the NICOSIA study or as described in the literature. These phenomena combined to produce an effect size that did not reach statistical significance over the six-month study period.

Teleconference

At **15.00 CET / 14.00 UKT / 09.00 EST** on **May 19, 2009**, Santhera's management will discuss the IONIA results at a teleconference. Anyone interested in participating may join using one of the following dial-ins (**conference ID: 10747196**):

Germany	0692 222 3479 (local call)
Switzerland	056 580 00 07 (local call)
United Kingdom	0871 700 0345 (national call) or +44 1452 555 566 (standard international)
United States	1866 966 9439 (free call)

The teleconference will be available for playback one hour after the presentation ends.

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"We are of course tremendously disappointed by the IONIA results reported today," commented Klaus Schollmeier, Chief Executive Officer of Santhera. "The study met neither our expectations, nor those of the patients or the investigators. Everybody involved was highly motivated to demonstrate the drug's efficacy again. The results have not dampened our confidence in the drug's potential in Friedreich's Ataxia. Because of its larger patient population and longer study duration, we expect that the ongoing European Phase III MICONOS study will finally provide the efficacy data necessary to support marketing approval in the US and Europe."

The design of the ongoing European Phase III trial named MICONOS (Mitochondrial Protection With Idebenone In Cardiological Or Neurological Outcome Study) is different from the IONIA study reported today. The MICONOS study is a twelve-month trial of 232 predominantly adult patients with three active treatment arms against placebo. Enrollment was completed in December 2008 and results are expected in the first half of 2010. If positive, these results will form the basis of filings for a New Drug Application and a Marketing Authorization Application in the United States and Europe, respectively. As a consequence of the IONIA study results, the application for marketing approval earlier filed in Switzerland will be withdrawn.

Sue Perlman, Clinical Professor of Neurology at the University of California, Los Angeles and one of the two IONIA study investigators, comments: "I still strongly support the disease-modifying effect of Catena® in Friedreich's Ataxia. I believe it slows the progression of the neurological and cardiac aspects of this condition over time, and I strongly recommend that patients continue in the open-label extension study arm of the Phase III IONIA trial to enable us to gather as much longer term data as possible."

"Patients on drug improved over placebo on the primary endpoint (ICARS) in the IONIA study. This is supported by the second neurological endpoint (FARS) which also showed an improvement of treated patients over patients on placebo. In addition, a prespecified responder analysis of the active arms showed effect levels comparable to the NICOSIA study in which 60% of the treated patients showed a clinically meaningful improvement on ICARS. However, due to a larger than expected placebo response rate, statistical significance could not be achieved in this six month trial," said Thomas Meier, Chief Scientific Officer of Santhera. "Therefore, we are eager to see the additional data from the twelve-month MICONOS study as well as our two open-label extension studies. We remain confident in our development programs with Catena® in Friedreich's Ataxia and other neuromuscular and mitochondrial disorders and look forward to the upcoming data from several ongoing clinical trials."

About the IONIA Phase III trial

The IONIA (Idebenone effects On Neurological ICARS Assessments) trial was a double-blind, randomized, placebo-controlled Phase III study of six months duration investigating the efficacy, safety and tolerability of two doses of Catena® compared to placebo. The first dose group was

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450 mg/day for patients below 45 kg body weight and a corresponding dose of 900 mg/day for patients above 45 kg body weight. The second dose group was 1350 mg/day for patients below 45 kg of body weight and 2250 mg/day for patients above 45 kg. 70 ambulatory Friedreich's Ataxia patients between the ages of 8 and 17 years were recruited into two clinical centers in the US – the Children's Hospital of Philadelphia and the School of Medicine of the University of California, Los Angeles.

The primary endpoint was the change in the International Cooperative Ataxia Rating Scale (ICARS), a neurological scale, where the difference between baseline and end of treatment for each of the dosing groups was compared with the change in the placebo group. The IONIA study also investigated Activities of Daily Living parameters (ADL) as well as additional neurological (FARS) and cardiac outcomes. The study incorporated advice provided by the US Food and Drug Administration under Special Protocol Assessment.

About the NICOSIA trial

In the Phase II study conducted in collaboration with the US National Institutes of Health named NICOSIA (NIH Collaboration With Santhera In Ataxia), Catena® showed a positive effect in particular on the ICARS as well as the ADL scales. Data from the NICOSIA study demonstrated the drug's significant potential to improve neurological functions after a six-month treatment [1]. 48 patients were recruited at the NIH into three active dosage arms against placebo.

About Friedreich's Ataxia

Friedreich's Ataxia 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. Friedreich's Ataxia affects both Caucasian males and females equally and it is estimated that about 20,000 patients suffer from the disease in North America and Europe. The average life expectancy for Friedreich's Ataxia 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.

References

[1] Nicholas Di Prospero et al; (2007) Lancet Neurology; 6: 878-886.

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 the second pivotal Phase III trial in Europe are expected for the first half of 2010. The drug 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.

Catena® is a trademark of Santhera Pharmaceuticals.

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