

Santhera's Catena® Improved Vision in Patients with Leber's Hereditary Optic Neuropathy; Regulatory Submissions Planned in First Half of 2011

- Catena® patients who were almost completely blind recovered sufficiently to read at least 5 letters on a standard eye-chart
- Catena® patients with residual vision were significantly protected from further disease progression

Liestal, Switzerland, June 16, 2010 – Santhera Pharmaceuticals (SIX: SANN) announced today that the RHODOS study demonstrated that Catena® improved vision in patients with Leber's Hereditary Optic Neuropathy (LHON). LHON is a rare genetic eye disease that leads to a rapid loss of central vision and ultimately to blindness as a result of nerve cell degeneration in the retina and optic nerve. The goal of an efficacious treatment is therefore to prevent the progression of disease and to recover vision already lost. Catena® supports cellular energy production and has the potential to mitigate the effects of the genetic mutations which lead to blindness in LHON. Data from this first-ever double-blind, placebo-controlled intervention study in LHON consistently demonstrated benefit of Catena® in all endpoints related to visual acuity. Santhera will now approach health authorities to discuss strategies to ensure Catena® is made available to LHON patients as soon as possible.

Estimated mean difference and p-values comparing Catena® with placebo for change in visual acuity from baseline to week 24

Population	Endpoint		
	Best recovery (primary)	Best acuity (secondary)	Acuity in both eyes (secondary)
N total (N active/placebo)			
Intent To Treat (ITT) N=82 (53/29)	diff = 3 letters p = 0.291	diff = 5 letters p = 0.078	diff = 4 letters p = 0.026
ITT, excluding patients not legally blind* N=77 (50/27)	diff = 5 letters p = 0.075	diff = 7 letters p = 0.018	diff = 6 letters p = 0.002
ITT, only patients at highest risk** N=30 (20/10)	diff = 13 letters p = 0.011	diff = 20 letters p = 0.003	diff = 16 letters p = 0.0001

* Subpopulation of patients with both eyes having a logMAR value of less than 1.0

** Subpopulation of patients in progressive disease stage defined as two eyes having a difference of more than logMAR 0.2

Note: A difference of 5 letters is equivalent to 1 line on a standard eye-chart or 0.1 logMAR

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The 6-month RHODOS study enrolled 85 patients and evaluated the safety and efficacy of 900mg/d of Catena® compared to that of placebo. Santhera obtained scientific advice and protocol assistance from the British Medicines and Healthcare Regulatory Agency (MHRA) and the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) respectively. This advice defined conditions under which standard interpretation of p-values may not be required to demonstrate a positive risk-benefit. Santhera believes that the RHODOS study and its results meet these conditions and could support a regulatory filing.

The primary endpoint of the study was the best recovery in visual acuity in either eye measured by change in logMAR (logarithm of Minimal Ange of Resolution) between baseline and week 24. Patients in the intent-to-treat (ITT, N=82) population receiving Catena® (N=53) improved on average by 6 letters whilst patients receiving placebo (N=29) improved by 3 letters (p=0.291).

A prespecified secondary endpoint was the patients' best visual acuity at week 24 compared to best visual acuity at baseline. The visual acuity of patients in the Catena® group was 5 letters better at week 24 than patients in the placebo group (p=0.078). Scientific advice from the CHMP indicated that this endpoint will be of special importance in the assessment of the results.

In a further prespecified secondary endpoint analyzing the change of all patients' eyes separately to increase the power of the study, the visual acuity of eyes of patients receiving Catena® significantly improved compared to those receiving placebo (p=0.026).

A subsequent analysis of a subgroup of patients excluding 5 patients who were only mildly affected at baseline (defined as logMAR < 1.0) strengthens the outcome of the RHODOS study. In this subgroup, the improvement in the Catena® group approached significance for the primary endpoint (p=0.075) and reached significance for the secondary endpoints (p=0.018 and p=0.002).

A further subgroup of patients was defined as those with significant loss of visual acuity in the first eye and at highest risk of disease progression in the second eye (N=30). Generally, these patients were more recently diagnosed and were the initial target group of the RHODOS study. The visual acuity of patients in the Catena® group (N=20) was protected from deterioration whilst those on placebo (N=10) deteriorated by 20 letters (p=0.003).

A responder analyses of patients who were so severely affected that they were unable to read the eye-chart at baseline (N=38) shows that 7 out of 25 (28%) individuals receiving Catena® recovered sufficient visual acuity to read at least 5 letters on the eye chart compared to 0 out of 13 (0%) individuals in the placebo group (p=0.072).

The RHODOS study also confirmed the excellent safety profile of Catena® observed in previous clinical trials. The nature, severity and frequency of the adverse events were very similar in patients

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treated with Catena® and patients receiving placebo. No potential safety signal emerged from the review of vital signs, laboratory analyses or ECG data.

"We would like to thank the participating patients and the clinical investigators for their contributions to make the RHODOS study such an encouraging success," commented Thomas Meier, Santhera's Chief Scientific Officer. "Catena® could be the first treatment for this devastating and rapidly progressing form of blindness. The RHODOS results consistently demonstrate superiority of Catena® over placebo in endpoints related to visual acuity. Patients in the study benefited from the drug in particular the more severely affected individuals."

"I am very optimistic about the RHODOS results. For the first time, there is the very real possibility of an effective treatment for patients suffering from LHON. Catena® holds the promise to both protect against the loss of vision and also encourage recovery, moving LHON from an incurable disease to a disorder with a good chance of treatment", said Professor Patrick Chinnery, principal investigator of RHODOS who is Director of the Institute of Human Genetics at Newcastle University, and consultant neurologist at Newcastle Hospitals NHS Foundation Trust, United Kingdom.

Professor Thomas Klopstock, study investigator at the University of Munich, Germany, added: "The RHODOS data announced today establish Catena® as the first treatment option for LHON. Larger trials may not be feasible in such a rare disease, and another placebo-controlled trial may not be acceptable to patients or ethics committees in light of these results. I hope that Catena® becomes available to my patients as soon as possible."

The RHODOS study was the first-ever placebo-controlled trial in LHON patients and is the second largest trial ever conducted with Catena®. It was originally designed as a proof-of-concept study, the inclusion criteria of which were expanded to facilitate enrollment and the use of the study for regulatory purposes. Santhera will now discuss a filing strategy with the European health authorities and with the US Food and Drug Administration. Both agencies have already granted orphan drug designation to Catena® in LHON. Regulatory submissions for marketing approval are currently anticipated for the first half of 2011.

Klaus Schollmeier, Santhera's Chief Executive Officer, commented: "Today's announcement is good news for all LHON patients and a major milestone in the development of Catena® in this indication. Conducting another placebo-controlled trial in such a rare and progressive disorder after evidence of efficacy will be difficult if not impossible. We intend to file for marketing approval with these data as expeditiously as possible."

The RHODOS data announced today will be presented by Professor Chinnery at the upcoming Mitochondrial Medicine 2010 conference in Scottsdale, Arizona, on June 18, 2010.

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About the RHODOS study

The RHODOS study (Rescue of Hereditary Optic Disease Outpatient Study) was a randomized, double blind, placebo controlled trial testing the efficacy and safety of Catena® and placebo over a six-month treatment period. The dose tested was 900 mg/day. A total of 85 patients were enrolled into the study and were randomized in a 2:1 ratio to receive either Catena® or placebo. At baseline, participants were between 14 and 64 years of age with loss of visual acuity not longer than five years prior to enrolment. The study was conducted at centers in Newcastle (UK), Munich (Germany) and Montréal (Canada).

About Leber's Hereditary Optic Neuropathy

Leber's Hereditary Optic Neuropathy (LHON) is an inherited atrophy of certain cells in the retina and optic nerves that leads to rapid loss of central vision and ultimately to blindness. Blurring of central vision and color desaturation usually mark the beginning of the symptomatic phase of this neuro-ophthalmological disorder. The effects of LHON are rapid and severe, typically leading to blindness within a few months of the onset. While symptoms initially develop in one eye, the second eye is usually involved within a few months. Patients are predominantly young adult males who typically have one of three different point mutations of the mitochondrial genome. These mutations lead to the reduction in cellular energy production, which in turn results in cell damage and death of certain optic nerve cells.

Catena® is the first drug that has ever been clinically investigated in a randomized, placebo-controlled intervention study in LHON. Given the drug's principal mode of action, Catena® may protect the retinal and optic nerve cells and thereby delay, lessen or prevent vision loss. LHON is found in all ethnic groups; an estimated 20,000 patients live in Europe and in the United States.

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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 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. The drug is also being investigated in a Phase III study in Duchenne Muscular Dystrophy. Commercial rights in Europe for Friedreich's Ataxia and Duchenne Muscular Dystrophy are licensed to Takeda Pharmaceutical. Santhera's second compound fipamezole has demonstrated efficacy in reducing levodopa-induced Dyskinesia in Parkinson's Disease. Phase III development and commercialization rights in the United States and Canada are partnered with Biovail. For further information, please visit the Company's web site www.santhera.com.

Catena® is a trademark of Santhera Pharmaceuticals.

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Webcast/Teleconference

At **15:00 CET / 14:00 UKT / 09:00 EST** on **June 16, 2010**, Santhera's management will host a teleconference/webcast. You can either join the **webcast on www.santhera.com/webcast** or the **teleconference** using the conference **ID 82011795** and one of the following dial-ins:

Schweiz/Switzerland: 0445 803 409
Deutschland/Germany: 0692 222 204 47
UK: 0844 871 9395
Europe (international): +44 (0) 1452 560 068
USA: 1866 691 1171
Canada: 1866 231 3592

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

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