Santhera Pharmaceuticals Holding AG
Hammerstrasse 49
CH-4410 Liestal / Switzerland
Phone +41 61 906 89 50
Fax +41 61 906 89 51
www.santhera.com

Santhera Presents New Efficacy Data from an Expanded Access Program with Raxone® for the Treatment of Leber's Hereditary Optic Neuropathy (LHON)

Liestal, Switzerland, March 4, 2014 – Santhera Pharmaceuticals (SIX: SANN) announced today that it will present data from its ongoing Expanded Access Program (EAP) with Raxone® in the treatment of LHON at the 2014 Annual Meeting of the North American Neuro-Ophthalmology Society (NANOS) in Rio Grande, Puerto Rico. Current data from the EAP demonstrate that 50% of patients have achieved a clinically relevant improvement in their vision and 63% of the patients were protected from further vision loss following Raxone® treatment. Raxone® was safe and well tolerated following long-term use in the EAP, which provides further evidence of safety and efficacy for Raxone® in the treatment of LHON in the routine clinical setting.

Safety and efficacy data were collected in an ongoing EAP for LHON patients with recent onset of symptoms in which they were given access to Raxone® in response to an unsolicited request from their treating physician. As of January 31, 2014, 29 physicians in Europe, the USA, Australia and New Zealand had enrolled 61 LHON patients. Participants received Raxone® typically at 900 mg/day and were assessed for safety and visual acuity (VA) during routine clinical care for up to 21 months. Currently data from 48 patients carrying the G11778A, G3460A or T14484C mutation have provided best corrected VA data from at least one on-treatment visit. Patient demographics such as distribution of mtDNA mutation carried, gender, age and extent of VA loss of enrolled patients were consistent with the known natural history of LHON. Efficacy analyses assessed (i) the proportion of patients with clinically relevant and stable improvement in VA and (ii) the proportion of patients without further deterioration in VA.

LHON patients treated with Raxone® experienced rapid, clinically relevant improvement in vision

In the EAP, 24 of 48 patients (50%) have so far experienced a clinically relevant and stable improvement in VA defined as either (i) improvement from VA nadir of at least 10 letters (logMAR 0.2) on the ETDRS chart or (ii) improvement to reading at least 5 letters on-chart in patients with severe vision loss unable to read any letters on the ETDRS chart at nadir. In both cases, VA recovery had to be stable until the last available visit. Patients have been treated for an average of 11 months (range 3 to 21 months). Analyzed by disease-causing mtDNA mutation carried, 89% of patients with

the T14484C mutation, 70% with the G3460A and 31% with the G11778A mutation have had clinically relevant recovery of vision. The percentage of Raxone® treated patients with VA recovery is markedly higher than that observed in a comparable population from a recently completed natural history case record survey of over 350 LHON cases collected from centers in Europe and the USA.

In the EAP, the average treatment effect size in patients with VA recovery was 29 letters from the VA nadir across all mtDNA mutations (49 letters for patients with the T14484C, 26 letters for patients with the G3460A and 13 letters for patients with the G11778A mtDNA mutation). The efficacy of Raxone® in promoting recovery of VA occurs rapidly, as 75% of patients have responded within the first 6 months and 83 % within 12 months of the initiation of Raxone® treatment.

Raxone® treatment protects LHON patients from further vision loss

In the EAP, there were 38 patients able to read at least 5 letters on the ETDRS chart at the time of enrolment (baseline). Of those 24 (63%) had not worsened by the clinically relevant margin (defined as 10 letters of on-chart VA or transition from on-chart VA to off-chart vision) in either eye at the last available visit. Moreover, 9 of 13 patients (69%) who were not legally blind at baseline were protected from legal blindness (defined as logMAR ≥ 1.0) during their Raxone® treatment period.

Data will be presented at this weeks' 40th Annual Meeting of the North American Neuro-Ophthalmology Society (NANOS) in Puerto Rico.

"The clinical experience with Raxone® from the ongoing Expanded Access Program clearly supports previously published evidence of the safety and efficacy of Raxone® in the treatment of LHON", commented Thomas Meier, CEO of Santhera. "With this program, we have increased greatly the long-term data available for Raxone® in this disease. These new efficacy and natural history data will be important supporting elements in the European Marketing Authorization Application which we plan to submit in the coming weeks."

About Leber's Hereditary Optic Neuropathy and the use of Raxone®

Leber's Hereditary Optic Neuropathy (LHON) is a heritable genetic disease causing blindness. The disease typically presents in young adult men as rapid, painless loss of central vision in one eye, followed by the fellow eye within a few weeks or months of the onset of symptoms, usually leading to complete and permanent blindness. Over 95% of patients harbour one of three pathogenic mutations of their mitochondrial DNA which causes a defect in the complex I subunit of the mitochondrial respiratory chain. This defect leads to decreased cellular energy (ATP) production, increased oxidative stress and retinal ganglion cell dysfunction which cause progressive loss of visual acuity and blindness.

Raxone® (idebenone), a synthetic short-chain benzoquinone and a cofactor for the enzyme NAD(P)H:quinone oxidoreductase (NQO1), is capable of transferring electrons directly onto complex III of the mitochondrial electron transport chain, thereby circumventing the complex I defect and restoring cellular energy levels in retinal ganglion cells and promoting recovery of visual acuity.

About the Expanded Access Program

The EAP was established to provide access to Raxone® treatment to individual "named" LHON patients at the request and under the personal care of a registered physician according to applicable regulations. All patients receive Raxone® (usually 900 mg/day) and there is no control group. Visual acuity and safety information (adverse events) is collected at the patient's regular (generally 3-monthly) clinic visits and reported to Santhera.

Together with the contributing physicians, Santhera intends to submit the results of the EAP to a peer reviewed journal for publication.

About NANOS

The North American Neuro-Ophthalmology Society (NANOS) is a professional organization of more than 500 ophthalmologists or neurologists. The Society seeks to promote the field of neuro-ophthalmology by supporting all forms of education, encouraging research and fostering clinical expertise. The 40th Annual NANOS Meeting is held March 1-6, 2014 in Rio Grande, Puerto Rico.

* * *

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 orphan mitochondrial and neuromuscular diseases, such as Leber's Hereditary Optic Neuropathy, Duchenne Muscular Dystrophy and primary progressive Multiple Sclerosis, all of them areas of high unmet medical need with no current therapies. For further information, please visit www.santhera.com.

For further information, contact
Thomas Meier, Chief Executive Officer
Phone: +41 61 906 89 64
thomas.meier@santhera.com

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.