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CHMP recommends granting a marketing authorization for Santhera's Raxone® for the treatment of Leber's Hereditary Optic Neuropathy (LHON)

Liestal, Switzerland, June 26, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) has recommended granting a marketing authorization for Raxone® (idebenone) for the treatment of visual impairment in adolescent and adult patients with LHON.

LHON is a heritable mitochondrial disease that leads to rapid, profound and usually permanent blindness in otherwise healthy patients. Raxone® will be the first treatment option for LHON and the first approved therapy for a mitochondrial disease.

"This is a major breakthrough as it paves the way for the first medicinal product to become available for the treatment of a mitochondrial disease," stated **Thomas Klopstock**, MD (Professor for Neurology at the University of Munich, LHON investigator and coordinator of the German network for mitochondrial disorders, mitoNET). "LHON is a severe form of vision loss caused by mitochondrial dysfunction. Affected patients, usually young and otherwise healthy, rapidly lose central vision and become bilaterally blind within a few months from the onset of symptoms. Although there is a chance for partial or even full spontaneous recovery, most patients remain permanently blind if untreated. The mode of action of idebenone provides a clear biochemical and medical rationale and the clinical data demonstrate that vision of affected patients can substantially improve upon treatment with Raxone. This recommendation is a landmark in mitochondrial disease research worldwide and will undoubtedly spur further research in this direction."

"We are very excited about the CHMP's positive opinion, which recognizes the urgent medical need for a treatment for this devastating disease," stated **Thomas Meier**, PhD, CEO of Santhera. "We can now execute on our plans to ensure Raxone is made available to patients in the EU as soon as the European Commission marketing authorization is received."

The CHMP based its recommendation for Raxone on data from the randomized, placebo controlled RHODOS trial, Santhera's Expanded Access Program, and comparative natural history data from a comprehensive case record survey. The Committee considered that the totality of the data provided for an orphan disease as severe as LHON with no available treatment options warranted a recommendation for approval under Exceptional Circumstances. Santhera has undertaken to gather additional long-term efficacy and safety data in LHON patients as post-authorization measures.

The positive opinion by the CHMP for Raxone will now be forwarded to the European Commission (EC) for the adoption of a decision on EU-wide marketing authorization, applicable to all 28 member states of the European Union as well as Iceland, Liechtenstein and Norway. Raxone has orphan designation which provides 10 years of market exclusivity from the date of EC approval.

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

Leber's Hereditary Optic Neuropathy (LHON) is a heritable genetic disease causing blindness. The disease typically presents in young, otherwise healthy adults, mostly men, as rapid, painless loss of central vision in one eye, followed by visual loss in the other eye within a few months of the onset of symptoms, leading to blindness. Over 95% of patients harbor one of three pathogenic mutations of the mitochondrial DNA, which cause 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 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. Santhera develops Raxone®/Catena® as treatment for patients with Leber's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS) and omigapil for Congenital Muscular Dystrophies (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

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