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Santhera Receives European Marketing Authorization for Raxone® in Leber's Hereditary Optic Neuropathy (LHON)

First Approved Treatment for LHON and First Approved Treatment for a Mitochondrial Disease

Liestal, Switzerland, September 9, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces the European Commission (EC) has granted marketing authorization for Raxone® as the first approved medicine available in all 28 member states of the European Union (EU), Norway, Iceland and Liechtenstein for the treatment of visual impairment in adolescent and adult patients with Leber's Hereditary Optic Neuropathy (LHON). This rare condition is an inherited mitochondrial disease which if untreated usually leads to rapid, profound and permanent blindness in otherwise healthy patients.

"Our data demonstrate that Raxone treatment can prevent patients from further vision loss and can promote clinically relevant recovery of vision," said **Thomas Meier**, PhD, Chief Executive Officer of Santhera. "The European approval of Raxone as an efficacious treatment for LHON and as the first approved medication for a mitochondrial disease is a major milestone for Santhera as it marks our transition to a pharmaceutical company with a product on the market. I personally want to thank the team that achieved this tremendous success and the patients and doctors who supported our clinical development program over the past years. We will now ensure patients have a rapid access to Raxone, with immediate availability in some European countries."

"Raxone represents a major breakthrough in mitochondrial disease treatment, and its approval paves the way for patients with LHON to be treated and to achieve a meaningful improvement of their visual acuity," said **Thomas Klopstock**, MD, Professor for Neurology at the University of Munich, LHON researcher and coordinator of the German network for mitochondrial disorders, mitoNET. "LHON is a particularly devastating condition because sufferers, who are otherwise healthy and often young, rapidly become bilaterally blind within a few months. Most will remain permanently blind if untreated."

Raxone is an oral medication authorized at a daily dose of 900 mg (given as 2 tablets three times a day with food), for the treatment of visual impairment in adolescent and adult patients with LHON. Treatment should be initiated and supervised by a physician with experience in LHON. Efficacy data come from Santhera's randomized, placebo-controlled RHODOS trial and from the open label Expanded Access Program, which together have demonstrated that vision loss can be mitigated or reversed in patients treated with Raxone.

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, usually leading to permanent bilateral 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 reactive oxygen species (ROS) production 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, reducing and scavenging ROS 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's lead product Raxone® is authorized in the European Union for the treatment of Leber's Hereditary Optic Neuropathy (LHON). Santhera develops Raxone®/Catena® in two additional indications, Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS), and omigapil for Congenital Muscular Dystrophy (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

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