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Scottish Medicines Consortium Approves Santhera's Raxone® (idebenone) for Restricted Use in Patients with LHON within NHS Scotland

Liestal, Switzerland, May 9, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces approval by the Scottish Medicines Consortium for restricted use of Raxone® (idebenone) in patients with LHON, with effect May 8, 2017.

Patients with Leber's hereditary optic neuropathy (LHON), an extremely rare, inherited cause of blindness, could benefit from the only licensed treatment to date, following advice issued by the Scottish Medicines Consortium (SMC) under the ultra-orphan medicine process. The SMC accepted Raxone® (idebenone) for restricted use by NHS Scotland in the treatment of visual impairment in adolescent and adult patients with LHON who are not yet blind (i.e., they do not meet the UK criteria to be registered as severely sight impaired). Raxone was accepted following consideration through SMC's Patient and Clinician Engagement (PACE) process, for medicines used at the end of life and for very rare conditions. Scotland is the first country in the UK to make Raxone available for the treatment of LHON. The SMC advice takes into account the benefits of a Patient Access Scheme (PAS), which improves the cost-effectiveness of Raxone.

LHON is a devastating disease that causes blindness mainly in young men during their late teens/early adulthood. Patients suddenly lose central vision and can no longer see fine details, read print or recognize faces. Within 12 months from onset, about 80% of untreated patients will have become legally blind. This is exceptionally debilitating and isolating in young adults, affecting their education, career plans and overall quality of life. **Russell Wheeler**, Trustee of the UK based LHON Society said: "Despite LHON being identified almost 150 years ago, the lack of any approved treatment for this devastating condition until now has led to many patients feeling helpless and abandoned by the health service. We applaud the pragmatism of the SMC's decision following a rigorous and transparent process. The availability of Raxone to affected families in Scotland is a welcome step towards avoiding preventable blindness in patients with LHON and we hope it will be followed by similar availability in other parts of the UK."

Raxone treatment is now available to LHON patients under different reimbursement models in several European countries. **Giovanni Stropoli**, EVP, Chief Commercial Officer Europe & ROW commented, "We are grateful to the SMC for their comprehensive evaluation of our dossier for Raxone. As we continue working with the health services in other parts of the UK we hope that this positive advice to NHS Scotland will be followed soon by similar availability for LHON patients across the United Kingdom."

The full SMC advice can be reviewed at the following link: http://www.scottishmedicines.org.uk/SMC_Advice/Advice/1226_17_idebenone_Raxone/idebenone_Raxone. Please also refer to the full Summary of Product Characteristics at: http://www.medicines.org.uk/emc/medicine/32655.

About Leber's Hereditary Optic Neuropathy and the Therapeutic Use of Raxone

LHON is a heritable genetic disease causing blindness. The disease presents predominantly in young, otherwise healthy adult males as rapid, painless loss of central vision, usually leading to permanent bilateral blindness within a few months of the onset of symptoms. About 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), circumvents the complex I defect, reduces and scavenges ROS, restores cellular energy levels in retinal ganglion cells and promotes recovery of visual acuity.

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. Efficacy data come from Santhera's randomized, placebo-controlled 24-week RHODOS trial, from the open label Expanded Access Program of 'real-life' usage and from a natural history study of untreated patients, which together have demonstrated that vision loss can be mitigated or reversed in patients treated with Raxone. Treatment should be initiated and supervised by a physician with experience in LHON.

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, Norway, Iceland and Liechtenstein for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), the second indication for Raxone, Santhera has filed a Marketing Authorization Application (MAA) in the European Union and Switzerland. In collaboration with the US National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing Raxone in a third indication, 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® is a trademark of Santhera Pharmaceuticals.

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