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Temporary Authorization for Use Granted for Santhera's Raxone® for the Treatment of Leber's Hereditary Optic Neuropathy (LHON) in France

Liestal, Switzerland, January 21, 2014 – Santhera Pharmaceuticals (SIX: SANN) announced today that the French National Agency for Medicines and Health Products Safety (ANSM) has granted an "Autorisation Temporaire d'Utilisation dite de cohorte" (cohort ATU), a Temporary Authorization for Use for Raxone®, in the treatment of Leber's Hereditary Optic Neuropathy (LHON). The ATU system allows patients in France to receive reimbursed treatment with a product before a marketing authorization is granted in the European Union.

Promising treatments that have not yet been granted a marketing authorization but where the benefit/risk balance is presumed to be positive, based on the submitted dossier, can be granted a cohort ATU where there is a genuine public health need in the absence of any suitable therapeutic alternative.

The temporary authorization for Raxone® was granted after an assessment by the ANSM and clinical experts of a full application dossier comprising quality, clinical efficacy and safety data, including new efficacy data collected from LHON patients participating in an ongoing Expanded Access Program. Santhera will provide Raxone® to LHON patients in France under the cohort ATU, for which government allocation to hospitals ensures reimbursement, until a full marketing authorization is granted in the EU.

"We are very pleased that the ANSM has positively reviewed the submitted dossier and has taken the step of granting a temporary authorization for use for Raxone® in the treatment of LHON, whilst we continue to work towards a full market approval with the European regulatory authorities", commented Thomas Meier, CEO of Santhera. "We will work diligently to assist LHON patients in France in gaining access to Raxone® as quickly as possible under this program. The revenues from the cohort ATU will assist in off-setting previous development costs for Raxone® in this indication."

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 adults, mostly men, as rapid, painless loss of central vision in one eye, followed by visual loss in the fellow 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.

Santhera plans to submit a marketing authorization application (MAA) for Raxone® in the European Union in the first quarter of 2014.

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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 orphan mitochondrial and neuromuscular diseases, areas of high unmet medical need with no current therapies. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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