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Santhera Files Marketing Authorization Application in European Union for Raxone® in the Treatment of LHON

Liestal, Switzerland, May 7, 2014 – Santhera Pharmaceuticals (SIX: SANN) announced today that it has re-filed with the European Medicines Agency (EMA) a Marketing Authorization Application (MAA) for Raxone® (idebenone) in the treatment of Leber's Hereditary Optic Neuropathy (LHON). An earlier application was withdrawn in March 2013 in order to include additional clinical efficacy data. The compound has been granted orphan drug designation in the European Union (EU). Raxone® would become the first product authorized for the treatment of this rare, inherited disease which otherwise invariably leads to blindness. Santhera expects a decision from the EMA in the first half of 2015.

Santhera discussed the additional clinical efficacy data and the overall content of the revised MAA dossier with several EU member states prior to proceeding with the re-filing. Earlier this year the French National Agency for the Safety of Medicine and Health Products (ANSM) granted a temporary authorization for use for Raxone® in LHON patients in France based on a data package comparable to the submitted MAA dossier.

"We are optimistic that the new data on Raxone® which we have submitted in this MAA dossier will address the issues raised by the CHMP during the previous procedure" commented Thomas Meier, Chief Executive Officer of Santhera. "The totality of data included in the re-submission provides compelling evidence that Raxone® can protect against vision loss in the early stages of the disease and promote recovery of vision already lost."

Clinical data presented at ARVO conference in the US

Efficacy data on Raxone® in LHON patients from an ongoing Expanded Access Program will be presented this week at the Annual Meeting of the Association for Research in Vision and Ophthalmology (ARVO) in Orlando, Florida. The poster can be downloaded from www.santhera.com under *Products & Pipeline/Publications*.

US regulatory strategy

Santhera has previously been granted an US orphan drug designation for the treatment of LHON and will now approach the US Food and Drug Administration (FDA) for discussions on a regulatory path to an US approval based on the data package filed in the European MAA.

About LHON and the treatment with Raxone®

Leber's Hereditary Optic Neuropathy (LHON) is a heritable genetic disease which usually leads to permanent 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. Over 90% of patients harbour one of three pathogenic mutations of their 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 to 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 bypassing the complex I defect, restoring ATP production in retinal ganglion cells and promoting recovery of visual acuity.

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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, 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 the Company's website www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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