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European Medicines Agency Validates Santhera's Marketing Authorization Application for Raxone® in Leber's Hereditary Optic Neuropathy

Liestal, Switzerland, June 5, 2014 – Santhera Pharmaceuticals (SIX: SANN) today announced that the European Medicines Agency (EMA) has validated its Marketing Authorization Application (MAA) for Leber's Hereditary Optic Neuropathy (LHON). Validation confirms that the submission is complete and signifies that the CHMP review process has begun. Santhera expects a decision from the EMA in the first half of 2015.

The validation was based upon the pivotal RHODOS trial, which showed a significantly higher proportion of Raxone®-treated patients presented with clinically relevant recovery in visual acuity (VA) compared to placebo, and on two additional datasets from the Expanded Access Program (EAP) and a Case Record Survey (CRS) which provide supportive efficacy and natural history data.

Efficacy data from 48 consecutive patients enrolled in Santhera's EAP with Raxone® in the treatment of LHON show that 50% of patients achieved clinically relevant improvement in their vision and that 63% were protected from further vision loss following Raxone® treatment. The second dataset, a CRS established in collaboration with the European Vision Institute Clinical Research (EVICR) network, provides comparative data demonstrating profound vision loss and low rates of spontaneous recovery in untreated patients.

Inclusion of the data from the EAP and CRS in the current MAA increases the number of Raxone®/idebenone-treated LHON patients for whom VA outcomes data is available to 101 (RHODOS trial alone 53) and the number of placebo/untreated patients to 102 (RHODOS trial alone 28).

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

"The validation of the dossier by the EMA acknowledges that the additional data provided represent substantial new information for the clinical efficacy review process", commented Nick Coppard, SVP Development at Santhera. "The initiation of the CHMP review is an important milestone for patients with LHON. Over the last few months we have experienced increasing demand from LHON treating physicians for access to Raxone® via our ATU and Expanded Access Programs, which we have been able to provide only in France and elsewhere where local regulations allow. Patients now have the prospect of equitable access to Raxone® across the EU."

Professor Patrick Chinnery, principal investigator of RHODOS and Director of the Institute of Genetic Medicine at Newcastle University, United Kingdom added "The additional data on the effectiveness of Raxone® in the real world setting and on the comparative natural history of the disease provides a clear picture of the potential benefits of Raxone® in the treatment of LHON. We are hopeful that the availability of an effective treatment for patients with LHON has now been brought closer."

Santhera reported that the validation of the MAA for LHON also represents the start of the regulatory clock for an additional indication for Raxone® in the treatment of Duchenne Muscular Dystrophy (DMD) for which a clinically relevant and statistically significant benefit of Raxone® in the preservation of respiratory function was recently reported. The company currently plans to file a MAA for DMD as a variation to the LHON label and will work with the CHMP Rapporteurs to find the most expeditious regulatory approval pathway for this indication.

The compound has been granted orphan drug designation in the European Union (EU) for both LHON and DMD. Raxone® would become the first product authorized for the treatment of LHON, which is a rare inherited mitochondrial disease that otherwise invariably leads to blindness.

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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. Santhera develops Catena®/Raxone® as treatment for patients with Leber's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS), all areas of high unmet medical need for which no therapies are currently available. For further information, please visit the Company's website www.santhera.com.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

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