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First International Consensus Statement on the Clinical and Therapeutic Management of LHON published in the *Journal of Neuro-Ophthalmology*

Liestal, Switzerland, October 23, 2017 – Santhera Pharmaceuticals (SIX:SANN) announces that the first *International Consensus Statement on the Clinical and Therapeutic Management of Leber's Hereditary Optic Neuropathy (LHON)* has been published in the *Journal of Neuro-Ophthalmology*.

The Consensus Statement reflects the independent views from a panel of world-leading experts in the management of patients with LHON. It is intended to provide guidance for the clinical and therapeutic management of LHON based on the evidence currently available. Amongst other clinical aspects, the Consensus Statement focuses on the use of Raxone® (idebenone), the first and only treatment approved by the European Medicines Agency (EMA) in 2015 for the treatment of this heritable form of vision loss. The authors provide guidance on the optimal target population, timing, dose and frequency of administration of idebenone as well as other areas where there has previously been a lack of accepted definitions and general guidelines for the clinical management of patients with LHON.

“The expert consensus statement provides a much-needed set of recommendations for physicians who are treating patients with LHON, ultimately leading to improved patient care,” said **Francesco Bandello**, MD, Professor at University Vita Salute, Scientific Institute San Raffaele of Milano and Past-President of the European Society of Retina Specialists (EURETINA). “In March 2016, during an international meeting sponsored by the San Raffaele Scientific Institute, sixteen international experts came together to provide these recommendations so that treating physicians, health care providers, and health organizations can further advance the management of patients with LHON.”

Thomas Meier, PhD, CEO of Santhera, commented: “This is the first time that a consensus for the management of LHON has been developed, and this paper will generate important awareness for the disease and Raxone – the first approved treatment for this heritable form of vision loss. We hope that this publication will allow us to engage in further collaborations with the neuro-ophthalmological and optic nerve specialist community, and that we are able to advance knowledge and continue to improve the clinical outcomes for those suffering from this rare disease.”

The paper is available in electronic format to subscribers to the *Journal of Neuro-Ophthalmology*. The abstract is accessible under the following link: http://journals.lww.com/jneuro-ophthalmology/Abstract/publishahead/International_Consensus_Statement_on_the_Clinical.99345.aspx

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

Leber's hereditary optic neuropathy (LHON) is a heritable genetic disease causing profound vision loss and 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, currently authorized in the European Union, Norway, Iceland, Liechtenstein and Israel at a daily dose of 900 mg for the treatment of visual impairment in adolescent and adult patients with 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® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), Santhera has filed a Marketing Authorization Application in the European Union and Switzerland for DMD patients with respiratory function decline who are not taking glucocorticoids. In collaboration with the U.S. 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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