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Santhera Announces Approval of Raxone® for LHON in Israel

Liestal, Switzerland, August 31, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces approval of Raxone® (idebenone) for the treatment of visual impairment in adolescent and adult patients with Leber’s hereditary optic neuropathy (LHON) from the Ministry of Health Israel – the first approval for Raxone in LHON outside Europe.

Following the European Commission’s marketing authorization for Raxone, the first approved treatment in LHON and the first approved treatment for a mitochondrial disease, the Ministry of Health Israel granted Raxone a marketing authorization for the treatment of visual impairment in adolescent and adult patients with LHON.¹

“We are pleased that Raxone has received marketing authorization for the treatment of LHON in Israel, the first approval for the product outside of Europe,” said **Giovanni Stropoli**, Chief Commercial Officer Europe & ROW at Santhera. “As part of our strategy to provide treatment to patients in Europe and beyond, we have signed a collaboration agreement with Megapharm Ltd., a leading Israeli private pharma marketing company, who is committed to making Raxone available to patients with LHON in Israel.”

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

Leber’s hereditary optic neuropathy (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.

About Megapharm Ltd.

Megapharm Ltd. is a leading Israeli private pharma marketing company, exclusively representing a number of major American, European and Japanese pharmaceutical companies. Megapharm provides its partners with the full commercial capabilities, including registration, market access & reimbursement, and sales & marketing. Megapharm has demonstrated dynamic sales growth by developing a strong company presence and expertise in selected therapeutic areas and proven track record for obtaining reimbursement and inclusion of its products in all the Health Funds in Israel.

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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'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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¹https://www.old.health.gov.il/units/pharmacy/trufot/PerutTrufa.asp?Reg_Number=159%2018%2034904%2001&safa=h

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