

Santhera Pharmaceuticals Holding AG Hammerstrasse 49 4410 Liestal / Switzerland Phone +41 61 906 89 50 Fax +41 61 906 89 51 www.santhera.com

Santhera Signs Distribution and Supply Agreement for Raxone[®] (idebenone) with Pharmathen for Greece and Cyprus

Liestal, Switzerland, May 2, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces that it has signed an agreement with Pharmathen to market Raxone[®] for the treatment of Leber's hereditary optic neuropathy (LHON) in Greece and Cyprus. LHON is a rare, heritable, mitochondrial disease that leads to rapid, profound and usually permanent blindness in otherwise healthy patients. Raxone is the first treatment option for LHON and the first approved therapy for a mitochondrial disease.

"We are pleased to announce the completion of this strategic partnership with Santhera Pharmaceuticals, which allows us to offer an important treatment option to Greek and Cypriot patients affected with Leber's hereditary optic neuropathy," commented Pharmathen's President **Vasileios Katsos**. "This partnership demonstrates our strong commitment to providing innovative solutions in the field of rare diseases."

"The agreement with Pharmathen represents an important achievement for Santhera and underscores our commitment to making Raxone available to patients across all EU member states," said **Giovanni Stropoli**, Santhera's Chief Commercial Officer for Europe & ROW. "We are confident that Pharmathen's expertise in the field of rare diseases, coupled with their understanding of the region, will greatly benefit patients with this devastating disease."

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.

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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. Treatment should be initiated and supervised by a physician with experience in LHON. Efficacy data come from Santhera's randomized, placebo-controlled RHODOS trial and from the open label Expanded Access Program, which together have demonstrated that vision loss can be mitigated or reversed in patients treated with Raxone.

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[®] is authorized in the European Union, Norway, Iceland and Liechtenstein for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), the second indication for Raxone, Santhera has filed a Marketing Authorization Application (MAA) in the European Union and Switzerland. In collaboration with the US 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 <u>www.santhera.com</u>.

Raxone® is a trademark of Santhera Pharmaceuticals.

About Pharmathen

Pharmathen was founded in 1969 in Athens, as a private pharmaceutical company, and is focused on the development and marketing of pharmaceuticals, with a strong position in generics. With three state of the art research laboratories and two manufacturing units, Pharmathen is a completely vertically integrated company and its activities extend from the development of pharmaceutical products up to their distribution. Pharmathen owns a diversified portfolio of products available globally, including innovative and generic medicines as well as pharmaceutical and other healthcare products.

For further information, contact:

Thomas Meier, PhD, Chief Executive Officer Phone +41 61 906 89 64 thomas.meier@santhera.com Christoph Rentsch, Chief Financial Officer Phone +41 61 906 89 65 <u>christoph.rentsch@santhera.com</u>

Sue Schneidhorst, Head Group Communications Phone +41 61 906 89 26 sue.schneidhorst@santhera.com

US investor contact

Hans Vitzthum, LifeSci Advisors, LLC Phone +1 212 915 2568 hans@lifesciadvisors.com US Public Relations contact John Gillespie, Medical Dynamics Phone +1 646 599 8626 jgjillespie@rxmedyn.com

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