

Santhera Submits NDA in South Korea for Raxone® for the Treatment of LHON

Pratteln, Switzerland, June 12, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces that the Korean Ministry of Food and Drug Safety (MFDS) has accepted for review Santhera’s new drug application (NDA) for Raxone® (idebenone) for the treatment of Leber’s hereditary optic neuropathy (LHON). Raxone was granted orphan drug designation for LHON in South Korea.

Raxone® (idebenone) 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. It is the first and only medicine approved for this rare inherited disease which, if untreated, invariably leads to profound vision loss and blindness.

The NDA now submitted to the MFDS was prepared on the basis of the European marketing authorization and Santhera expects a decision from the South Korean drug regulatory authorities in approximately one year.

“The regulatory submission of Raxone for LHON in South Korea, one of the major Asian markets, underlines our geographical expansion and our commitment to providing treatment to patients worldwide, addressing a major unmet medical need in this rare disease,” said **Thomas Meier**, PhD, CEO of Santhera.

Raxone has also been granted orphan drug designation for LHON in South Korea, which provides up to 10 years market exclusivity from the date of approval.

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. Current data demonstrate that up to 50% of patients benefit from treatment and are protected from progression of visual acuity loss or experience a clinically relevant recovery of visual acuity.

Raxone for the treatment of LHON was granted orphan drug status in the EU, US, Switzerland and South Korea.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. The most advanced pipeline product, idebenone, is in clinical Phase III for the treatment of Duchenne muscular dystrophy (DMD). Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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