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UK's MHRA renews Early Access to Medicines Scheme Scientific Opinion for Santhera's Raxone® (idebenone) in Duchenne Muscular Dystrophy

Pratteln, Switzerland, June 22, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces that the UK's Medicines and Healthcare products Regulatory Agency (MHRA) has renewed the Early Access to Medicines Scheme (EAMS) scientific opinion for Raxone® (idebenone) for patients with Duchenne muscular dystrophy (DMD) in respiratory function decline who are not taking glucocorticoids. At the occasion of the annual review, the MHRA confirmed its positive scientific opinion for Raxone under the EAMS and renewed the EAMS for a further year.

The aim of EAMS is to provide patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. Inclusion in EAMS allows eligible patients with DMD, who meet criteria defined under this scheme, to gain access to Raxone, an investigational medicinal product for which Santhera is currently preparing marketing authorization applications in Europe and the USA.

Raxone has been available in the UK through EAMS since June 2017. At the present time over 40 DMD patients are enrolled in EAMS at several specialized DMD centers in the UK.

"We are very pleased about the renewal of the EAMS for Raxone which allows patients with DMD who have progressed to the stage of respiratory decline and have no alternative therapeutic options available, to receive treatment," said **Thomas Meier**, PhD, CEO of Santhera. "The MHRA renewal comes as a sign of continued recognition of both the high unmet medical need of DMD patients and the positive benefit-risk of Raxone in this population. We are committed to addressing this urgent medical need and will make Raxone available to all patients in the UK who meet the eligibility criteria for the EAMS upon request of their treating physician."

Janet Bloor, the chair of Action Duchenne and the mother of a young man living with Duchenne, said: "We have waited many years to realize the opportunity for early access to potential new treatments. It has taken a company like Santhera to focus on a huge unmet need and seek to address the underlying problems with respiratory failure. Time is something we don't have with Duchenne and I welcome the renewal of the EAMS by the MHRA after effective consultation with the patient community."

Under the EAMS, as shown in the public assessment report[1], Raxone is indicated as a treatment for slowing the decline of respiratory function in patients with Duchenne muscular dystrophy (DMD) from the age of 10 years who are currently not taking glucocorticoids. Patients will need to meet the clinical criteria for entry into EAMS, including showing evidence of active decline of respiratory function prior to initiation of treatment. Raxone can be offered to patients previously treated with glucocorticoids or in patients in whom glucocorticoid treatment is not tolerated or is considered inadvisable.

[1] Public assessment report. Available at https://www.gov.uk/government/publications/early-access-to-medicines-scheme-eams-scientific-opinion-raxone-to-treat-the-decline-of-respiratory-function-in-patients-with-duchenne-muscular-dys

About the UK Early Access to Medicines Scheme (EAMS)

The UK's EAMS aims to give patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization when there is a clear unmet medical need. Under the scheme, the MHRA provides a scientific opinion on the benefit-risk balance of the medicine, based on the data available when the EAMS submission was made. The opinion lasts for a year and can be renewed. The scheme is voluntary and the opinion from MHRA does not replace the normal licensing procedures for medicines.

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. 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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