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UK's MHRA renews EAMS Scientific Opinion for Santhera's Idebenone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, June 23, 2020 – Santhera Pharmaceuticals (SIX: SANN) announces that the UK's Medicines and Healthcare products Regulatory Agency (MHRA) has renewed for a further year the Early Access to Medicines Scheme (EAMS) scientific opinion for idebenone for patients with Duchenne muscular dystrophy (DMD) in respiratory function decline who are not taking glucocorticoids. With this renewal, the MHRA again confirmed its positive scientific opinion for idebenone under the EAMS while a corresponding European marketing authorization application (MAA) is currently under review.

By renewing the EAMS[1], the MHRA continues to enable access to idebenone for DMD patients with respiratory function decline, a leading cause of increased morbidity and early death for which no approved therapy exists. A conditional marketing authorization (CMA) application for idebenone (under the trademark Puldysa®) to treat respiratory dysfunction in DMD is currently under regulatory review and Santhera expects an opinion by the Committee for Medicinal Products for Human Use (CHMP) in the fourth quarter 2020.

"This EAMS renewal for idebenone enables a much-needed therapeutic option for DMD patients with deteriorating respiratory function who have no real treatment alternative. We welcome the continued recognition by the UK's MHRA of the positive benefit-risk of idebenone in this patient population," said **Kristina Sjöblom Nygren, MD, Chief Medical Officer and Head of Development at Santhera**.

Idebenone has been available in the UK through EAMS since June 2017. At present, 84 patients with DMD are benefiting from early access to idebenone through EAMS at several specialized DMD centers across the UK.

Under the EAMS, as shown in the public assessment report[2], idebenone is indicated as a treatment for slowing the decline of respiratory function in patients with 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. Idebenone can be offered to patients previously treated with glucocorticoids or in patients in whom glucocorticoid treatment is not tolerated or is considered inadvisable.

[1] Annex to the Public Assessment report (3rd renewal, 2020). Available at: https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/893913/FINAL_ANNEX_to_PAR_Raxone_EAMS_THIRD_Renewal.pdf

[2] Public assessment report. Available at: https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/620862/Raxone_FINAL_Public_Assessment_Report.pdf

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 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 rare neuromuscular and pulmonary diseases with high unmet medical need. Santhera is building a Duchenne muscular dystrophy (DMD) product portfolio to treat patients irrespective of causative mutations, disease stage or age. A marketing authorization application for Puldysa® (idebenone) is currently under review by the European Medicines Agency. Santhera has an option to license vamorolone, a first-in-class anti-inflammatory drug candidate with novel mode of action, currently investigated in a pivotal study in patients with DMD to replace standard corticosteroids. The clinical stage pipeline also includes lonodelestat (POL6014) to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases, as well as omigapil and an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed ex-North American rights to its first approved product, Raxone® (idebenone), for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Puldysa® and Raxone® are trademarks of Santhera Pharmaceuticals.

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