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

Pratteln, Switzerland, June 24, 2019 – 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) has recently been submitted.

When applying for the EAMS renewal, Santhera submitted new efficacy data (including results from the long-term SYROS study) supporting the potential for a clinically relevant preservation of respiratory function during idebenone treatment for up to six years in a real-world setting. By renewing the EAMS[1], the MHRA has enabled access to idebenone for DMD patients with the highest need. Recently, Santhera has submitted a conditional marketing authorization (CMA) application to the European Medicines Agency (EMA) for idebenone (under the trademark Puldysa®) to treat respiratory function decline in DMD.

"We welcome the EAMS renewal for idebenone as it provides a therapeutic option for DMD-patients with deteriorating respiratory function in the UK who really have no treatment alternative," said **Kristina Sjöblom Nygren, MD, Chief Medical Officer and Head of Development at Santhera**. "Last month, we submitted a European MAA for idebenone in DMD. In this context, the MHRA renewal not only comes as a continued recognition of the positive benefit-risk of idebenone in this patient population but also helps bridge the time until drug approval."

Idebenone has been available in the UK through EAMS since June 2017. At present, 67 patients with DMD are benefiting from access to idebenone through EAMS at several specialized DMD centers in 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 (2nd renewal, 2019). Available at: https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/809856/ANNEX_to_PAR_Raxone_EAMS_Second_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 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, Israel and Serbia for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.

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