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Santhera's Raxone® Receives First Positive EAMS Scientific Opinion from UK's MHRA in Duchenne Muscular Dystrophy

Liestal, Switzerland, June 22, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces that the UK's Medicines and Healthcare products Regulatory Agency (MHRA) has granted Raxone (idebenone) a positive scientific opinion through the Early Access to Medicines Scheme (EAMS) for patients with respiratory function decline not taking glucocorticoids in Duchenne Muscular Dystrophy (DMD).

The aim of the 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.[1] The MHRA decision allows patients with DMD, who meet criteria defined under this scheme, to gain access to Raxone, an investigational medicinal product currently under review for DMD for Marketing Authorization by the European Medicines Agency (EMA).

Under the EAMS, and as shown in the public assessment report,[2] Raxone is indicated for slowing the decline of respiratory function in patients with DMD from the age of 10 years who are currently not taking glucocorticoids. The decline of respiratory function must be confirmed by repeated measurements prior to initiation of treatment. Raxone can be used in patients previously treated with glucocorticoids or in patients in whom glucocorticoid treatment is not tolerated or is considered inadvisable.

"We're proud to receive the positive EAMS scientific opinion for Raxone in the UK and to have our lead compound designated as a promising innovative medicine, the first for a drug intended for the treatment of DMD," said **Thomas Meier**, PhD, CEO of Santhera. "This decision allows patients with DMD to receive treatment for respiratory function decline who otherwise would not have access to such treatment options."

"This is excellent news for patients with respiratory decline in Duchenne muscular dystrophy," said **Janet Bloor**, Chair of the Board of Trustees at Action Duchenne. "The need for new treatments in DMD is very great and the EAMS can help to accelerate access for patients. Action Duchenne was pleased to provide advice during the development of the EAMS program and we are delighted to see this first positive opinion in DMD."

About the UK Early Access to Medicines Scheme (EAMS)

The UK's industry-sponsored 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 pharmaceutical products for the treatment of orphan mitochondrial and neuromuscular diseases. Santhera's lead product Raxone® (idebenone) 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), Santhera has filed a Marketing Authorization Application in the European Union and Switzerland for DMD patients with respiratory function decline who are not taking glucocorticoids. In collaboration with the U.S. 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 www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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References

[1] Medicines and Healthcare products Regulatory Agency Patient safety and Marketing authorisations, variations and licensing guidance. Available at: https://www.gov.uk/guidance/apply-for-the-early-access-to-medicines-scheme-eams (June 2017)

[2] 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

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