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First Patients Enrolled in UK's Early Access to Medicines Scheme for Santhera's Raxone® in Duchenne Muscular Dystrophy (DMD)

Liestal, Switzerland, September 11, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces enrollment of first DMD patients with respiratory function decline not taking glucocorticoids into UK's Early Access to Medicines Scheme (EAMS) for Raxone®.

In June, Raxone® (idebenone) was granted a positive scientific opinion through the Early Access to Medicines Scheme (EAMS) by the UK's Medicines and Healthcare products Regulatory Agency (MHRA) – the first drug approved under the EAMS for patients with DMD. 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 Marketing Authorization in DMD by the European Medicines Agency (EMA). Enrollment into the EAMS documents the first use of Raxone outside of a clinical trial for patients with DMD.

"I am pleased to be able to offer Raxone to several of my patients in respiratory decline, as there are no other medical treatments available," said **Dr. Dipansu Ghosh**, a respiratory physician at a DMD center based in Leeds.

To date, 15 specialist DMD centers in the UK have received training under the requirements of the EAMS. Several additional sites have expressed an interest to be trained and are currently undergoing local approval processes.

"At Action Duchenne we were encouraged by the positive EAMS opinion, earlier this summer. Particularly, for young people living with Duchenne who have respiratory decline," said **Janet Bloor**, Chair of Trustees of Action Duchenne. "I am delighted that respiratory function is being considered by the regulatory agencies. This will hopefully pave the way for more potential treatments that may benefit the wider spectrum of DMD patients."

Under the EAMS, and as shown in the public assessment report, [1] 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.

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, Liechtenstein and Israel 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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Reference

[1] Public EAMS 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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