

Santhera's Raxone[®] designated Promising Innovative Medicine (PIM) and suitable candidate for further evaluation under the UK Early Access to Medicines Scheme (EAMS) for treatment in Duchenne muscular dystrophy

Liestal, Switzerland, December 23, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that it has been informed that the UK's Medicines and Healthcare Products Regulatory Agency (MHRA) designated Raxone[®] (idebenone) for the treatment of Duchenne muscular dystrophy (DMD) in patients with respiratory function decline not taking concomitant glucocorticoids as Promising Innovative Medicine (PIM) and as a suitable candidate for entry into Step II of the EAMS process.

In the UK the Early Access to Medicines Scheme 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.

"We are delighted about MHRA's decision designating Raxone as a candidate for further consideration under the Early Access to Medicines Scheme as this underscores the high unmet medical need in DMD and acknowledges the innovative treatment approach of Raxone," said **Thomas Meier**, PhD, CEO of Santhera.

Nic Bungay, Director of Campaigns, Care and Information at Muscular Dystrophy UK, commented: "We welcome this very encouraging news that Raxone has been considered suitable for the second step of the EAMS process, especially as this is the first muscular dystrophy drug to reach this stage. This demonstrates that the introduction of the EAMS, which Muscular Dystrophy UK had called for, could help to fast track emerging treatments. We urge the Government to continue to support the use of EAMS by pharmaceutical companies to bring through potentially promising treatments."

The European Medicines Agency (EMA) and Swissmedic (the Swiss Agency for Therapeutic Products) are currently reviewing marketing authorization applications for Raxone for DMD patients in whom respiratory function has started to decline and who are not taking concomitant glucocorticoids. This indication would include patients who were previously treated with glucocorticoids or in whom glucocorticoid treatment is not desired, not tolerated or is contraindicated.

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December 23, 2016 / Page 2 of 2

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. The EAMS is a two-step process:

Step I is the Designation as a Promising Innovation Medicine (PIM). The PIM designation is an early indication that a medicinal product is a promising candidate for EAMS and gives reassurance that its clinical development is on track by having an early review of its data by the medicines regulator.

Step II is the Scientific Opinion by the Medicines and Healthcare products Regulatory Agency (MHRA, UK regulatory agency). The Scientific Opinion describes the benefits and risks of the medicine and supports the prescriber and patient to make a decision on using the medicine before its license is approved.

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 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), the second indication for Raxone, Santhera has filed a Marketing Authorization Application (MAA) in the European Union and Switzerland. In collaboration with the US 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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