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Santhera Signs Gene Therapy Agreement with SEAL Therapeutics

Pratteln, Switzerland, February 28, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces the signing of an agreement with SEAL Therapeutics for a gene therapy approach targeting congenital muscular dystrophy.

Santhera has entered into an agreement with SEAL Therapeutics, a spin-off company from the Biozentrum of the University of Basel, which will further develop a gene therapy approach intended for the treatment of LAMA2-deficient congenital muscular dystrophy (LAMA2 MD). Santhera will be eligible for payments based on future proceeds of SEAL Therapeutics.

Previous license agreements and preclinical collaborations that existed between Santhera and University Basel as well as Rutgers, The State University of New Jersey, have been terminated. SEAL Therapeutics will advance the gene therapy technology building on existing research progress from the Biozentrum Basel and Rutgers, both previously partially funded by Innosuisse and/or Santhera. The new company aims to team up with and support a pharmaceutical partner with experience in gene therapy technologies for clinical development and registration with the ultimate goal to make this innovative treatment approach available to patients with LAMA2 MD and their families.

The new arrangement allows Santhera to focus on its core clinical assets vamorolone and lonodelestat, freeing up resources to advance these platform compounds towards registration and market entry.

Dario Eklund, Chief Executive Officer of Santhera, said: “Whilst Santhera has been encouraged by the progress of this preclinical gene therapy program, we remain focused on developing our key pipeline projects vamorolone and lonodelestat. SEAL Therapeutics is well positioned to build on this groundbreaking research and attract a qualified pharmaceutical partner in the gene therapy area.”

The opportunities of a potential novel gene therapy approach for the treatment of so-called laminin-alpha 2 (LAMA2)-deficient congenital muscular dystrophy (“LAMA2 MD”, also known as “MDC1A”), a form of inherited and progressive muscle weakness with symptoms starting in newborns or infants, have been developed and evaluated in prior preclinical work conducted at the University of Basel and Rutgers [1-6]. The findings from this preclinical research indicate that the simultaneous expression of specifically designed small protein domains (structures which form part of a protein), so-called linker proteins, may exert disease-modifying potential in LAMA2 MD patients. The researchers have demonstrated that these linker proteins ameliorate muscle membrane stability by compensating the deficient functionality of laminin-alpha2 and lead to improved muscle histology and function and much increased lifespan in relevant mouse models.

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 has an exclusive license for all indications worldwide

to vamorolone, a first-in-class dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The Company is planning for filing for approval with the US FDA in Q1-2022. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. Santhera out-licensed rights to its first approved product, Raxone® (idebenone), outside North America and France for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

About SEAL Therapeutics

SEAL Therapeutics AG, a spin-off of the Biozentrum of University of Basel, develops its proprietary SEAL technology as potential gene therapy treatment of laminin-α2-deficient congenital muscular dystrophy (LAMA2 MD; also called MDC1A). SEAL has been founded by Prof. Rüegg and Dr. Reinhard from the Biozentrum and Dr. Meier, board member of Santhera Pharmaceuticals. SEAL Therapeutics intends to team-up with and support a qualified pharma partner with experience in advanced gene therapy technologies for clinical development and registration with the ultimate goal to make this innovative treatment approach available to LAMA2 MD patients and their families. For further information, please visit www.sealtherapeutics.com.

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