Ad hoc announcement pursuant to Art. 53 LR

Santhera Enters into Exclusive License Agreement with Sperogenix for Vamorolone in Rare Diseases in the Greater China Region

- *Sperogenix will receive exclusive rights for development and commercialization of vamorolone for the treatment of Duchenne muscular dystrophy (DMD) and any other rare disease*
- *Santhera to receive a double-digit upfront cash payment plus short-term US-regulatory milestones amounting to USD 20 million combined, in a deal valued at USD 124 million*
- *Santhera continues to focus with own organization on bringing vamorolone to patients in US and Europe, with a rolling NDA submission to commence in Q1-2022, while partnering for additional markets worldwide*

Pratteln, Switzerland, January 4, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces that it has entered into an exclusive license agreement with Sperogenix Therapeutics, a China-based company specializing in orphan diseases. Under this agreement, Sperogenix will in-license vamorolone for rare disease indications for a total consideration of up to USD 124 million, including a double-digit upfront cash compensation and DMD-related US-regulatory milestone payments amounting to a combined USD 20 million, as well as further double-digit royalties on net sales.

Under the terms of the agreement, Santhera will grant Sperogenix Therapeutics exclusive development and commercialization rights to vamorolone in DMD and all other rare disease indications for Greater China (including mainland China, Hong Kong, Macau, and Taiwan). Santhera will remain responsible for manufacturing and supply while Sperogenix, a China-based dedicated rare disease company financed by Lilly Asia Ventures and Morningside Ventures, will focus on regulatory and development work and future commercialization. Sperogenix plans to initiate a regulatory filing for vamorolone for DMD in China upon US FDA approval which could lead to market entry in China as early as in 2024.

There are significant opportunities in the healthcare business in China due to the large patient base and high unmet needs. Regulatory reforms have led to an increasing number of approvals and launches of innovative medicines [1]. DMD is on the Chinese list of rare disease with a high medical need recognized by the Chinese government [2]. The estimated prevalence of DMD could be as high as 70,000 patients with increasing rate of diagnosis giving more patients access to care in expert centers. There is currently no approved treatment for DMD in China. Sperogenix will proactively engage with the health authorities in China in order to achieve an accelerated regulatory pathway for vamorolone.

“We look forward to working with Sperogenix, a strong rare disease partner and well positioned for bringing vamorolone to patients in China,” said **Dario Eklund, Chief Executive Officer of Santhera**. “This agreement enables us to continue our focus on US and EU regulatory activities on our own while also gaining access to one of the largest pharmaceutical markets globally.”

"We are pleased to partner with Santhera. We believe in the potential of vamorolone as a treatment for Duchenne muscular dystrophy and are excited about its potential in the treatment of other rare diseases. The extensive regulatory, clinical and commercialization experience in rare disease products of our team will help to bring this differentiated product candidate to benefit patients in the Greater China region," said **Alan (Zhiyu) Yan, Co-founder, Chairman and Chief Executive Officer of Sperogenix**.

Santhera intends to commercialize vamorolone for the treatment of DMD through its own organization in the United States and main markets in Europe, and is seeking collaborations outside those regions for DMD and for additional indications worldwide. The Company will commence a rolling NDA submission in the US in Q1-2022, paving the way for a first launch as early as beginning of 2023 in the US, followed by a European marketing authorization application in Q2-2022. Santhera estimates the peak product sales potential for vamorolone in the indication DMD alone to be in excess of USD 500 million in the US and the largest five European countries combined.

About Vamorolone

Vamorolone is a first-in-class drug candidate that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is a dissociative agonist [3-5]. This mechanism has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescents with DMD. In the pivotal VISION-DMD study, vamorolone met the primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile. Compared to prednisone, vamorolone showed comparable efficacy, improvements on multiple safety parameters (including a reversal of growth impairment seen during prednisone treatment and a reduction of behavioral changes) and was associated with fewer adverse events. In clinical studies, vamorolone was generally well tolerated. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

Vamorolone has been granted Orphan Drug status in the US and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the US FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD. Vamorolone is an investigational medicine and is currently not approved for use by any health authority.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare inherited X-chromosome-linked disease, which almost exclusively affects males. DMD is characterized by inflammation which is present at birth or shortly thereafter. Inflammation leads to fibrosis of muscle and is clinically manifested by progressive muscle degeneration and weakness. Major milestones in the disease are the loss of ambulation, the loss of self-feeding, the start of assisted ventilation, and the development of cardiomyopathy. DMD reduces life expectancy to before the fourth decade due to respiratory and/or cardiac failure.

About Sperogenix Therapeutics

Sperogenix Therapeutics is a platform company dedicated to developing and commercializing rare disease therapeutics in China. With prioritized therapeutic areas, such as pulmonary vascular disorders, neuromuscular diseases, and inherited metabolic diseases, Sperogenix is dedicated to establishing an innovative commercial model tailored to the China rare disease field, in order to provide affordable and

reliable products and services to Chinese physicians and patients. Sperogenix was founded in 2019 and is backed by biopharma industry blue chip investors including Lilly Asia Ventures and Morningside Ventures. www.sperogenix.com

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 clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases as well as an exploratory gene therapy approach targeting congenital muscular dystrophies. 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.

References

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For further information please contact:

Santhera
Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, CH-4133 Pratteln
public-relations@santhera.com or
Eva Kalias, Head External Communications
Phone: +41 79 875 27 80
eva.kalias@santhera.com

Sperogenix
Lei Xiao, Associate Director of PR & Patient Support
Phone: +86-10-64620528
lei.xiao@sperogenix.com

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