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Santhera Announces Successful Completion of First Clinical Trial with Omigapil in Patients with Congenital Muscular Dystrophy

Pratteln, Switzerland, April 5, 2018 – Santhera Pharmaceuticals (SIX: SANN) reports the successful completion of the first clinical trial with omigapil in patients with two forms of congenital muscular dystrophy (CMD) conducted in the US at the National Institutes of Health (NIH). The ascending multiple dose cohort study (CALLISTO) met its primary objective to establish a favorable pharmacokinetic profile of omigapil and demonstrated that the study drug was safe and well tolerated in children and adolescents with CMD. Following further data analysis, the Company will discuss these results with clinical experts and regulatory authorities to prepare for a pivotal trial in patients with CMD.

The single-center interventional trial to establish the pharmacokinetic profile and to evaluate the safety and tolerability of omigapil in pediatric and adolescent patients with CMD was conducted at the NIH's clinical center in Bethesda, Maryland (USA), and led by Carsten Bönnemann, MD, and A. Reghan Foley, MD, of the NIH's National Institute of Neurological Disorders and Stroke. Ambulant and non-ambulant patients aged 5-16 years with either of two of the most common forms of CMD resulting from collagen VI-deficiency (COL6-related dystrophies or COL6-RDs) or laminin alpha2-deficiency (LAMA2-related dystrophy or LAMA-RD) were eligible to participate in the trial. A total of 20 patients were enrolled in this ascending multiple dose cohort study. Participants were randomized to one of five groups and received omigapil at a once-daily dose ranging from 0.02 mg/kg to 0.08 mg/kg body weight as a liquid oral formulation for a period of 3 months.

The trial met its primary objective and established that the pharmacokinetic profile of omigapil is suitable for further development in pediatric patients and demonstrated that omigapil was safe and well tolerated in this fragile population of CMD patients.

"We are grateful to participating patients and their families for enrolling in this first interventional trial with a drug candidate for CMD and to the clinical researchers at the NIH for their dedication to this milestone trial for these forms of CMD," said **Thomas Meier**, PhD, CEO of Santhera. "This is an important step towards profiling the therapeutic potential of omigapil for the LAMA2 and COL6 related forms of CMD for which there is currently no treatment available. We will now collaborate with international experts and seek advice from regulators to further advance the clinical development of omigapil towards a pivotal trial."

"This collaboration with Santhera and the patient community allowed us to test for the first time an investigational therapy in children with these more common types of CMD for which no other treatment options are currently available," said **Carsten Bönnemann**. "With the help of Ken Cheung, PhD at Columbia University, this clinical trial applied an innovative design by utilizing a novel adaptive dose-finding algorithm. Upon full analysis, we will share detailed data from the CALLISTO trial at upcoming scientific conferences and with the patient community. We look forward to continue working with Santhera, all stakeholders and regulators to define the fastest development path towards pivotal efficacy studies for this drug candidate."

"Cure CMD and the CMD community are thrilled that this first-ever interventional trial for congenital muscular dystrophy has been successfully completed, in partnership with the NIH and Santhera Pharmaceuticals," added **Rachel Alvarez**, Director of Operations for Cure CMD, a leading non-profit organization focused on finding treatments and supporting the CMD community. "For the affected individuals and their families who enrolled in CALLISTO, trial participation represented a considerable burden, and we are forever grateful for their commitment to seeing this through to the end."

About Omigapil and the CALLISTO study

Omigapil is a deprenyl-analog with anti-apoptotic properties. Santhera obtained an exclusive license for omigapil from Novartis for the development in congenital muscular dystrophies (CMDs). Nonclinical studies in a disease-relevant model showed that omigapil inhibits cell death and reduces body weight loss and skeletal deformation, while increasing locomotive activity and protecting from early mortality (Erb M et al., J Pharmacol Exp Ther 2009, 331:787-795).

Omigapil has orphan drug designations for CMD in the US and Europe and was granted Fast Track Designation by the FDA.

The preparation and conduct of the Phase I CALLISTO trial was supported financially by a public-private partnership including two patient organizations, the US-based Cure CMD and the Swiss Foundation for Research on Muscle Diseases (FRSMM), EndoStem, an EU 7th Framework program, and NIH clinical resources. In addition, the CALLISTO study was supported by an award from the Office of Orphan Products Development (OOPD) at the US Food and Drug Administration (FDA).

About Congenital Muscular Dystrophy

Congenital muscular dystrophies (CMDs) are inherited neuromuscular conditions characterized by congenital-onset weakness and hypotonia and have associated dystrophic findings on muscle biopsy. Progressive muscle weakness, joint contractures and respiratory insufficiency characterize most CMDs. LAMA2-related and COL6-related dystrophies are the most common forms of CMD for which no pharmacological therapy is currently available or in advanced clinical development.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. The most advanced pipeline product, idebenone, is in clinical Phase III for the treatment of Duchenne muscular dystrophy (DMD). Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and currently commercialized in 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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