Santhera Pharmaceuticals Holding AG
Hammerstrasse 49
CH-4410 Liestal / Switzerland
Phone +41 61 906 89 50
Fax +41 61 906 89 51
www.santhera.com

Santhera Announces First Patient Dosing with Omigapil in Congenital Muscular Dystrophy (CMD) and Full Patient Recruitment of CALLISTO Study

Liestal, Switzerland, July 20, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that the first patient in the CALLISTO Phase I study assessing the pharmacokinetics, safety and tolerability of oral omigapil in patients with Congenital Muscular Dystrophy (CMD) has been dosed and all participating patients have been recruited. This study, which is being conducted at the US National Institutes of Health (NIH), will also evaluate the feasibility of conducting disease-relevant clinical assessments that could be used as endpoints in future efficacy trials in pediatric and adolescent CMD patients. Omigapil is a drug candidate in-licensed from Novartis and repositioned by Santhera for development in CMD.

The study is being conducted at the NIH's National Institute of Neurological Disorders and Stroke (NINDS) in Bethesda, Maryland, and will evaluate use of the compound in 20 ambulatory and non-ambulatory patients aged 5 to 16 years affected by either Ullrich or MDC1A subtypes of CMD. All patients have been selected, pre-screened and randomly assigned to one of the three study cohorts that are starting sequentially with ascending doses of omigapil. Following a 4-week vehicle run-in phase, each patient will receive one of three doses of omigapil (0.02, 0.08 or 0.2 mg/kg/day) for 12 weeks, during which period the pharmacokinetics of omigapil will be assessed as well as the patients' respiratory function, muscle strength and motor function. To ensure the collection of as much information as possible from both subtypes of CMD, Ullrich or MDC1A, all patients have been identified and randomized prior to the first dosing.

The first patient received the starting dose of 0.02 mg/kg/day, applied in a new liquid formulation developed by Santhera for this patient population. Only after an independent data and safety monitoring board (DSMB) has assessed the pharmacokinetic and safety data in each completed cohort, dosing in subsequent cohorts will follow at ascending doses. Due to the staggered dosing of patients, the study is expected to run until the end of 2016.

"We are very excited about the start of this trial with the first investigational new drug for this group of severe neuromuscular diseases where children are affected with ultimately devastating loss of functional muscle and no treatment currently available to slow down or stop progression of the disease", commented **Carsten Bönnemann,** MD, a senior investigator in the NINDS Neuromuscular and Neurogenetic Disorders of Childhood Section and Principal Investigator of this study. "To ensure as much information as possible can be collected from patients with both Ullrich or MDC1A subtypes of CMD in this trial, it was important to have all patients identified and randomized prior to the first patient commencing treatment."

"We are very proud of being able to collaborate with the NIH in developing omigapil for CMDs", emphasized **Thomas Meier**, PhD, Chief Executive Officer of Santhera. "We are thankful to the patients and families who are making tremendous efforts to enroll into CALLISTO, which is the first intervention trial with an investigational medicinal product in this group of neuromuscular diseases."

About Congenital Muscular Dystrophies

Congenital Muscular Dystrophies refer to a variety of inherited neuromuscular conditions characterized by different forms of progressive loss of muscle tissue. Severe forms can affect newborns or young children with life-threatening progressive muscle weakness ("floppy infant syndrome"). Complications associated with the disorder such as loss of body weight, skeletal deformations and respiratory distress result in immobility at young age and early mortality. No pharmacological therapy is currently available or in advanced clinical development. Treatment options are confined to respiratory support and orthopedic surgery for scoliosis as well as supplementary nutrition to avoid malnutrition.

About Omigapil

Omigapil is a deprenyl-analog with anti-apoptotic properties, originally developed by Novartis. Santhera obtained an exclusive license for omigapil for the development in Congenital Muscular Dystrophies. 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. Clinical development of omigapil is sponsored by Santhera under an open IND granted by the US Food and Drug Administration.

Omigapil has been granted orphan drug designation for CMD in both the EU and the US.

About CALLISTO

CALLISTO (**C**ongenital Muscular Dystrophy **A**scending Multiple Dose Cohort Study ana**L**yzing Pharmacokinetics at three dose **L**evels **I**n Children and Adolescents with assessment of **S**afety and **T**olerability of **O**migapil) is a Phase I study in Congenital Muscular Dystrophy patients. The CALLISTO trial is 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 and EndoStem, an EU 7th Framework program. More details on the study are available from www.clinicaltrials.gov (Identifier NCT01805024).

About the NINDS

The National Institute of Neurological Disorders and Stroke (www.ninds.nih.gov) is the nation's leading funder of research on the brain and nervous system. The mission of NINDS is to seek fundamental knowledge about the brain and nervous system and to use that knowledge to reduce the burden of neurological disease. The National Institutes of Health (NIH), the nation's medical research agency, includes 27 Institutes and Centers and is a component of the U.S. Department of Health and Human Services. NIH is the primary federal agency conducting and supporting basic, clinical, and translational medical research, and is investigating the causes, treatments, and cures for both common and rare diseases. For more information about NIH and its programs, visit www.nih.gov.

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 develops Raxone®/Catena® as treatment for patients with Leber's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS) and omigapil for Congenital Muscular Dystrophy (CMD), all areas of high unmet medical need for which no therapies are currently available. In June 2015, the Committee for Medicinal Products for Human Use (CHMP) recommended granting a marketing authorization in Europe for Raxone® for the treatment of LHON. For further information, please visit the Company's website www.santhera.com.

Raxone®/Catena® are trademarks of Santhera Pharmaceuticals.

For further information, contact:
Thomas Meier, PhD, Chief Executive Officer
Phone +41 61 906 89 64
thomas.meier@santhera.com

US investor contact:
Andrew McDonald, LifeSci Advisors, LLC
Phone +1 646 597 6979
andrew@lifesciadvisors.com

US Public Relations contact:
Deanne Eagle, Planet Communications
Phone +1 917 837 5866
deanne@planetcommunications.nyc

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.

# # #