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Santhera Repositions Omigapil in Congenital Muscular Dystrophy and Initiates Clinical Development Program with Public-Private Partners

Liestal, Switzerland, July 17, 2014 – Santhera Pharmaceuticals (SIX: SANN) announced today the initiation of a clinical program with omigapil, a drug candidate in-licensed from Novartis and repositioned for therapeutic use in Congenital Muscular Dystrophy (CMD). The clinical development program will be initiated with a Phase I study in pediatric CMD patients, to be conducted at the National Institute of Neurological Disorders and Stroke (NINDS), a component of the US National Institutes of Health (NIH). The program is supported financially in the amount of CHF 1.3 million by EndoStem, an EU 7th Framework Programme, and two patient organizations, the US-based Cure CMD and the Swiss Foundation for Research on Muscle Diseases. Patient enrolment is expected to start in late 2014.

Following Santhera's successful repositioning of idebenone for the treatment of Leber's Hereditary Optic Neuropathy (LHON) and for Duchenne Muscular Dystrophy (DMD), omigapil, as an anti-apoptotic which previously reached late-stage clinical development for other neurological indications, represents an ideal candidate for the treatment of CMD in which muscle cell death by apoptosis is a major contributing factor.

The Phase I study (CALLISTO) will evaluate the pharmacokinetic profile, safety and tolerability of oral omigapil in pediatric and adolescent CMD patients and establish the feasibility of conducting disease-relevant clinical assessments for the design of future efficacy trials. A new liquid formulation of omigapil has been developed by Santhera specifically for this patient population. CALLISTO will be conducted at the NIH's National Institute of Neurological Disorders and Stroke (NINDS) in Bethesda, Maryland, and will include 20 ambulatory and non-ambulatory patients aged between 5 and 16 years suffering either from the Ullrich or from MDC1A subtypes of CMD who will be treated for 12 weeks. An independent drug safety monitoring board (DSMB) will monitor patient safety and study progress.

"Congenital Muscular Dystrophies are inherited neuromuscular conditions characterized by progressive loss of muscle tissue. Frequently, these children are affected with devastating muscle loss and no treatment is currently available to slow down or stop progression of the disease. The NIH is pleased to participate in a study that will explore the feasibility of omigapil in pediatric and adolescent patients with CMD", commented **Carsten Bönnemann,** MD, PhD, Senior investigator and Chief of the NINDS Neuromuscular and Neurogenetic Disorders of Childhood Section at NINDS who will serve as the Principal Investigator of this study.

"The initiation of the CALLISTO trial constitutes the fulfillment of one major goal for the EndoStem project, namely to get new drugs into the clinic that target diseased muscle. Funded by the European Commission and now in its 5th year, EndoStem is a collaborative network of leaders in stem cell biology and private entities that came together to drive research from the laboratory to the clinic with a particular focus on muscular dystrophies. A central strategy is to target the diseased tissue in order to promote regeneration and prevent degeneration, for which omigapil is a perfect example", explained Professor **David Sassoon**, coordinator of the EndoStem Programme.

"With currently no effective treatment available for patients, the CALLISTO trial offers hope to patients with Congenital Muscular Dystrophies. As the leading organization representing the interests of patients with this disease, Cure CMD welcomes CALLISTO as a major milestone for our foundation", added **Patrick May**, President of Cure CMD.

"Our foundation is dedicated to support clinical research for the development of therapies for rare neuromuscular diseases both in Switzerland and internationally. We are glad to support the consortium of experts in order to conduct the CALLISTO trial", added **Jacques Rognon**, Chairman of Swiss Foundation for Research on Muscle Diseases (FSRMM).

"Combined international efforts of private and public institutions are needed to advance medical research into orphan diseases. This externally funded collaboration is a perfect strategic fit for Santhera to which it brings orphan drug and muscular dystrophy clinical development expertise whilst retaining global commercial rights for omigapil in CMD", concluded **Thomas Meier**, Chief Executive Officer of Santhera.

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About Congenital Muscular Dystrophies
Congenital Muscular Dystrophies refer to a wide 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
Originally developed by Novartis, Santhera obtained an exclusive license for omigapil, a deprenyl-analog with anti-apoptotic properties, to develop the molecule in Congenital Muscular Dystrophies. Preclinical 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 et al., 2009. Journal of Pharmacology and Experimental Therapeutics 331:787–795). Clinical development of omigapil is sponsored by Santhera under an open IND granted by the US Food and Drug Administration.

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. Study preparation is ongoing while NINDS and Santhera are in the final stage of negotiating an agreement for the conduct of this trial. 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 NINDS mission is to reduce the burden of neurological disease – a burden borne by every age group, by every segment of society, by people all over the world.

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 EndoStem

EndoStem (www.endostem.eu) is a partnership of 15 research and clinical teams from globally recognized academic centers, small biotech and large pharmaceutical companies working together to develop new strategies aimed at stimulating stem cells that are resident in damaged tissue to repair it in situ. This approach is recognized as one of the most promising approaches to targeting stem cells for regenerative medicine due to the alignment with existing therapeutic development approaches used by large industry and recent advances in understanding the key barriers for tissue regeneration. Coordinated by Professor David Sassoon, co-financed by the European Commission via the 7th Framework Programme the aims of the project over are: (i) the implementation of clinical trials, with muscular dystrophies as the primary clinical target using innovative biopharmaceuticals; (ii) the development of novel best in class biopharmaceuticals with highly specific and well defined modes of action; (iii) the fast-track clinical translation based on a constant feedback loop between emerging patient responsiveness to new drugs and the development of the next generation of therapeutics; and (iv) the better understanding of the key issues preventing effective tissue repair matched with approaches to circumvent them.

About Cure CMD

Cure CMD's (www.curecmd.org) mission is to bring research, treatments and in the future, a cure for Congenital Muscular Dystrophies. Cure CMD will achieve this mission by working globally together with dedicated parent, government and research advocates. By focusing on this mission, Cure CMD will find and fund high potential research and clinical trials. Success will be determined by clinical applications that improve the lives of those afflicted with CMD's.

About FSRMM

The Swiss Foundation for Research on Muscle Diseases (www.fsrmm.ch) is active in Switzerland since 1985. Its main purpose is to implement the development of therapies for patients with genetic neuromuscular diseases by supporting excellent research projects and by facilitating communication and contacts on national and international level.

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, areas of high unmet medical need with no current therapies. As its most advanced asset, Santhera develops Catena®/Raxone® (idebenone) as treatment for patients with Leber's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS). Santhera previously received temporary approval (cATU) for Raxone® in the treatment of LHON in France and in May this year submitted a Marketing Authorization Application (MAA) for this indication to the European Medicines Agency. Recently, Santhera reported that its Phase III (DELOS) study met its primary endpoint of delaying the progression of respiratory function loss in patients with DMD not taking concomitant corticosteroids. For further information, please visit www.santhera.com.

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