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Santhera Receives FDA Grant in Support of its Ongoing Phase I Trial with Omigapil in Congenital Muscular Dystrophy

Liestal, Switzerland, August 30, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces that the Office of Orphan Products Development (OOPD) at the US Food and Drug Administration (FDA) has granted Santhera an award of USD 246,000 in support of its ongoing Phase I trial with omigapil (CALLISTO) in patients with congenital muscular dystrophy (CMD). Santhera is conducting CALLISTO in collaboration with the US National Institutes of Health (NIH). The FDA awards grants through the Orphan Products Grants Program to support the clinical development of products for use in rare diseases where no current therapy exists.

”We are delighted that the FDA has awarded us this prestigious and highly competitive grant in support of our CALLISTO trial in CMD,” commented **Thomas Meier,** PhD, CEO of Santhera. “With this award the FDA emphasizes the need for a therapy for CMD and the contribution of the CALLISTO trial to the development of an effective treatment. After Raxone, omigapil is our second pipeline product which further demonstrates our dedication to developing effective medicines for the treatment of mitochondrial and neuromuscular diseases.”

“As the leading organization representing the interests of patients with CMD, we are very pleased that the FDA recognizes the need to advance medical research in this orphan disease. With currently no effective treatment available for patients, the CALLISTO trial offers hope to patients with this devastating disease,” added **Patrick May**, President of CureCMD.

Orphan Products Grants are intended for clinical studies evaluating the safety and/or effectiveness of products that could either result in, or substantially contribute to, market approval of these products. Santhera, in collaboration with the US National Institutes of Health (NIH), is currently conducting CALLISTO under the leadership of Prof. Carsten Bönnemann and Dr. Reghan Foley at the National Institute of Neurological Disorders and Stroke (NINDS) in Bethesda, Maryland. CALLISTO assesses the pharmacokinetics, safety and tolerability of omigapil in ambulatory and non-ambulatory children affected by either of two subtypes of CMD (COL6-RD or LAMA2-RD). The study is expected to be completed in the first half of 2017. The CALLISTO trial was previously supported 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).

In May 2016, Santhera received Fast Track Designation from the FDA for omigapil for the treatment of CMD. Omigapil was previously granted Orphan Drug Designation for CMD in both the EU and the US.

About Congenital Muscular Dystrophy

Congenital muscular dystrophy (CMD) is a heterogeneous and clinically distinct group of inherited neuromuscular diseases including the subtypes LAMA2-RD and COL6-RD and manifests with an early onset of symptoms that may include weakness, contractures, elevated creatine kinase levels, dystrophic changes on muscle histology, loss of ambulation or even inability to stand or walk, respiratory insufficiency, feeding difficulties and early death. Severe forms can affect newborns or young children with life-threatening progressive muscle weakness ("floppy infant syndrome"). A contributing pathway and factor in disease burden and muscle fibrosis is apoptosis through a cascade of cellular events involving the glyceraldehyde-3-phosphate dehydrogenase (GAPDH) pathways for transcription of pro-apoptotic genes. No pharmacological therapy is currently available or in advanced clinical development. Treatment options are limited 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, worldwide license for omigapil for the development in congenital muscular dystrophy (CMD). Omigapil binds to glyceraldehyde-3-phosphate dehydrogenase (GAPDH) and this interaction leads to its inhibition and is thought to underlie the compound's observed anti-apoptotic effect. This mechanism of action provides the rationale for the clinical development of omigapil in CMD and anti-apoptotic effects were demonstrated in animal models of CMD where omigapil inhibited cell death and reduced body weight loss and skeletal deformation, while increasing locomotive activity and protecting from early mortality.

About CureCMD

Cure CMD's mission is to bring research, treatments and in the future, a cure for congenital muscular dystrophy. 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. For further information, please visit www.curecmd.org.

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's lead product Raxone® is authorized in the European Union, Norway, Iceland and Liechtenstein for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), the second indication for Raxone®, Santhera has filed a Marketing Authorization Application (MAA) in the European Union. In collaboration with the US National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing Raxone® in a third indication, primary progressive multiple sclerosis (PPMS), and omigapil for congenital muscular dystrophy (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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