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Santhera receives FDA Fast Track Designation for Omigapil for the Treatment of Congenital Muscular Dystrophies (CMD)

Liestal, Switzerland, May 20, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that it has received Fast Track Designation from the US Food and Drug Administration (FDA) for omigapil for the treatment of congenital muscular dystrophies (CMD). FDA's Fast Track process is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need for the purpose of getting them to the patient earlier. Previously, omigapil has already been granted Orphan Drug Designation for CMD in both the EU and the US. Santhera, in collaboration with the US National Institutes of Health (NIH), is currently conducting a clinical phase I study (CALLISTO) with omigapil in CMD patients.

"We are delighted that the FDA has granted Fast Track Designation for omigapil thereby underlining the unmet medical need for an effective therapy for CMD patients and the severity of this class of diseases," commented **Thomas Meier**, PhD, CEO of Santhera. "After Raxone®, omigapil is our second neuromuscular pipeline product and underpins our dedication to developing effective medicines to satisfy the needs of patients suffering from mitochondrial and other rare diseases."

CMD is a group of inherited, severe neuromuscular diseases characterized by different forms of progressive and ultimately devastating loss of muscle tissue, frequently affecting young children. Currently, no treatment is available to slow down or stop progression of the disease. Santhera evaluates omigapil in the phase I study (CALLISTO) which investigates the safety, tolerability and pharmacokinetic profile of a new liquid formulation of omigapil in pediatric and adolescent patients with CMD. The study, which is being conducted at the US NIH's National Institute of Neurological Disorders and Stroke (NINDS) in Bethesda, Maryland, with Prof. **Carsten Bönnemann** as Principal Investigator, is fully enrolled and expected to be completed early in 2017. As previously reported, the CALLISTO study 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.

About FDA Fast Track Designation

The FDA established the Fast Track Drug Development Program under the FDA Modernization Act of 1997. The program is designed to facilitate the development and expedite the review of therapies intended to treat serious or life-threatening conditions, and that demonstrate the potential to address unmet medical needs. The advantages of Fast Track designation include more frequent meetings with the FDA, eligibility for Accelerated Approval and Priority Review, if supported by clinical data and Rolling Review, which allows a company to submit its NDA in sections, as they are completed. Usually the FDA does not begin review until it has received a complete application.

About Congenital Muscular Dystrophies

Congenital muscular dystrophies (CMD) 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 (CMD). 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.

About CALLISTO

CALLISTO is a phase I study evaluating the pharmacokinetics, safety and tolerability of omigapil in 20 ambulatory and non-ambulatory patients aged 5 to 16 years affected by either Ullrich or MDC1A subtypes of CMD. The study is being conducted at the NIH's National Institute of Neurological Disorders and Stroke (NINDS) in Bethesda, Maryland, and has been fully enrolled. More details on the study are available from www.clinicaltrials.gov (Identifier NCT01805024).

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 for the treatment of Leber's hereditary optic neuropathy (LHON). Santhera develops Raxone® in two additional indications, Duchenne muscular dystrophy (DMD) and primary progressive multiple sclerosis (PPMS), and omigapil for congenital muscular dystrophies (CMD), all areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

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