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Santhera Update on Pre-NDA Meeting with FDA on Raxone®/ Catena® in Duchenne Muscular Dystrophy (DMD)

Liestal, Switzerland, June 10, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that it has discussed its plans to submit a New Drug Application (NDA) for Raxone®/Catena® (idebenone) in DMD with the US FDA in a pre-NDA meeting. The Agency and Santhera agreed that data presented at the meeting, including natural history data obtained under collaboration with the Cooperative International Neuromuscular Research Group (CINRG), and the possible implications of the natural history data on plans for this NDA will be further discussed during a second meeting.

"In our meeting with the FDA, we also communicated that we have recently started a collaboration with CINRG to compare the outcomes of the successful Phase III DELOS trial with a comprehensive data-set collected by CINRG in their Duchenne Natural History Study over the past years. We are confident that these comparative analyses will further support the clinical relevance of the DELOS trial results which showed a clinical benefit on respiratory function in patients not taking concomitant glucocorticoid steroids. Our Fast Track designation allows us regular interactions with the FDA, and we will now prepare for a second meeting with the Agency to discuss our plans for submitting an NDA in light of these emerging data," stated **Thomas Meier**, PhD, CEO of Santhera. "In parallel to these ongoing activities, we are continuing to prepare the NDA for submission."

Idebenone has been granted orphan drug designation for DMD in Europe and the US and has use patent protection until 2026 in Europe and 2027 in the US. The FDA recently granted Fast Track designation for Raxone/Catena (idebenone) for the treatment of DMD.

About Idebenone in Duchenne Muscular Dystrophy

DMD is one of the most common and devastating types of muscle degeneration and results in rapidly progressive muscle weakness. It is a genetic, degenerative disease that is inherited in an X-linked recessive mode with an incidence of up to 1 in 3,500 live born males worldwide. DMD is characterized by a loss of the protein dystrophin, leading to cell damage, impaired calcium homeostasis, elevated oxidative stress and reduced energy production in muscle cells. This results in progressive muscle weakness and wasting and early morbidity and mortality due to cardio-respiratory failure. Currently, glucocorticoid steroids are the only available medical treatment that can slow the decline in muscle strength and function irrespective of the disease-causing mutation. However, the effect is only partial and clinical use is limited by well-known side effects caused by glucocorticoid steroids. A recent study showed that ~42% of DMD patients 10 years and older had either never used glucocorticoid steroids or have discontinued their use.

The lack of the protein dystrophin leads to membrane instability and uncontrolled calcium influx and imbalance of intracellular calcium homeostasis in muscle cells. This results in reduced cellular energy production, increased reactive oxygen species (ROS) production and mitochondrial dysfunction, which contribute to muscle cell loss in DMD. Idebenone is a synthetic short-chain benzoquinone and a substrate for the enzyme NAD(P)H:quinone oxidoreductase (NQO1) capable of stimulating mitochondrial electron transport, supplementing cellular energy levels and inhibiting ROS production.

DELOS was a Phase III double-blind, placebo-controlled trial which randomized and treated 64 DMD patients not receiving concomitant glucocorticoid steroids. Patients 10-18 years of age received either Raxone/Catena tablets (900 mg/day) or matching placebo for 52 weeks. Data from the successful DELOS trial, which met its primary endpoint, were recently published in The Lancet (Buyse et al., (2015) Lancet. 385:1748-57).

About CINRG

The Cooperative International Neuromuscular Research Group was founded in 1999 as the Clinical Research Arm of the Duchenne Muscular Dystrophy Research Center and the Research Center for Genetic Medicine at the Children's National Medical Center in Washington DC. It started, and remains, a multi-disciplinary and cross-institutional network of clinicians and scientists with the shared goal of wanting to positively impact the lives of neuromuscular disorder patients and their families. Today, CINRG is a global, state-of-the-art clinical research network, with over 20 sites, that has conducted many studies and has access to over 4,000 neuromuscular patients. The primary goal of CINRG is to study the cause(s), pathogenesis and clinical outcomes of neuromuscular disorders and to conduct well-controlled clinical studies that examine promising, therapeutic interventions that may improve quality of life or extend life for patients. The CINRG Coordinating Center is located at the Children's National Medical Center in Washington, DC. The CINRG Duchenne Natural History Study (DNHS), led by a team of investigators at the University of California, Davis and funded through a combination of US Federal and patient advocacy foundation grants is presently the largest and most comprehensive multi-institution study ever conducted in individuals with DMD. The study has regularly followed more than 400 patients in 10 nations for up to 9 years each, and has combined genetic information, clinical measures of strength and function, pulmonary function, medical treatment and outcome data, and patient and family reports of activities, participation and quality of life. The data is being used to create a contemporary picture of individuals with DMD that guides the development of and interpretation of clinical trials, improves care practices, and validates clinical, biomarker and patient-reported assessment tools.

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 Dystrophies (CMD), all areas of high unmet medical need for which no therapies are currently available. For further information, please visit the Company's website www.santhera.com.

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