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Principal Investigator to Present Phase III Data on Idebenone in Duchenne Muscular Dystrophy (DMD) at the Annual Meeting of the American Academy of Neurology (AAN)

Liestal, Switzerland, March 11, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that Gunnar M. Buyse, PhD, MD, Professor of Child Neurology at the University Hospitals Leuven (Belgium), and principal investigator, will present outcome data of the Phase III trial with Raxone®/Catena® (idebenone) in Duchenne Muscular Dystrophy (DMD) at the upcoming annual meeting of the American Academy of Neurology (AAN).

The oral presentation titled, "Idebenone Reduces Loss of Respiratory Function in Duchenne Muscular Dystrophy—Outcome of a Phase III Double Blind, Randomised, Placebo controlled Trial (DELOS)," will be held during the Clinical Trials Plenary Session on Friday, April 24, 12:00 pm-1:30 pm. This session was designed by the AAN to address important clinical topics identified throughout the neurology community that affect patient care, and the Science Committee considered the DELOS outcome a critical advancement in the field of neuroscience.

"Progressive lung disease and irreversible loss of lung function are a main cause of morbidity and early mortality in DMD", stated Professor **Gunnar M. Buyse**, invited presenter at the AAN conference. "The statistically significant and clinically relevant outcomes of primary and secondary endpoints of this Phase III trial coherently demonstrate the therapeutic potential of idebenone in reducing the loss of respiratory function."

"I am excited that Professor Buyse was invited by the organizers of this year's AAN conference to present the outcome of this first successful phase III trial in DMD as a prominent lecture to a general audience of neurologists," commented **Thomas Meier**, PhD, CEO of Santhera. "This is as much a tribute to the positive outcome of the trial as it is tribute to the scientists and patients and families conducting it."

About the DELOS trial

DELOS was a Phase III, double-blind, placebo-controlled trial which randomized and treated 64 European and US DMD patients not receiving concomitant corticosteroids. Patients 10-18 years of age received either Raxone/Catena tablets (900 mg/day) or matching placebo for 52 weeks. The primary endpoint was change in Peak Expiratory Flow % predicted (PEF%p) from baseline to week 52. PEF%p declined significantly (-9.01%p; 95% CI: -13.2, -4.8; $p<0.001$) from baseline to week 52 in the placebo group compared to a non-significant decline (-3.05%p; 95% CI: -7.1, 0.97; $p=0.134$) in the Raxone/Catena group, resulting in a statistically significant difference between treatment

groups of 5.96%p (95% CI: 0.16, 11.8; p=0.044) at week 52 and representing a 66% reduction in loss of PEF%p. A statistically significant treatment effect was also seen at week 26 (p=0.007) and week 39 (p=0.034) and across all assessment timepoints (p=0.018). Data for the primary endpoint were robust across multiple sensitivity analyses and supported by positive outcomes of additional respiratory endpoints.

About Duchenne Muscular Dystrophy (DMD)

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 steroids. A recent study showed that ~42% of DMD patients 10 years and older had either never used steroids or have discontinued their use.

About Idebenone in Duchenne Muscular Dystrophy

Raxone/Catena (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 and supplementing cellular energy levels. A prior phase II randomized placebo-controlled trial (DELPHI) demonstrated trends for beneficial effects of Raxone/Catena on early functional cardiac and respiratory parameters. An important finding of the DELPHI trial was that patients treated with idebenone stabilized in PEF%p, a marker of expiratory muscle strength, compared to patients receiving placebo who declined as expected from the natural history of the disease. Additional analyses indicated that the Raxone/Catena treatment effect on respiratory function outcomes was larger in patients not taking concomitant glucocorticoid steroids.

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.

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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