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Santhera's Positive Phase III Trial (DELOS) in Patients with Duchenne Muscular Dystrophy (DMD) Published in *The Lancet*

First successful Phase III trial in DMD shows Raxone®/Catena® preserves respiratory function

Liestal, Switzerland, April 21, 2015 – Santhera Pharmaceuticals (SIX: SANN) announced that the full results of the double-blind placebo-controlled Phase III trial (DELOS) demonstrating efficacy and safety of Raxone®/Catena® (INN: idebenone) in patients with DMD have been published in *The Lancet* (Lancet 2015; 385: 1748–57. Online publication: http://dx.doi.org/10.1016/S0140-6736(15)60025-3).

The results of the DELOS trial demonstrated that Raxone/Catena significantly reduced the annual decline in Peak Expiratory Flow (PEF as percent predicted, PEF%p) by 66% compared to patients taking placebo. Other respiratory function endpoints such as Forced Vital Capacity (FVC) and Forced Expiratory Volume (FEV1) corroborated these results and showed a consistent pattern with treatment differences supporting efficacy of Raxone/Catena over placebo in the preservation of respiratory function. Researchers concluded that Raxone/Catena represents a new treatment option for DMD patients.

"Publication of the DELOS trial outcome in *The Lancet,* one of the most prestigious medical journals worldwide, is an extraordinary tribute to this first ever successful phase III trial in DMD", said **Gunnar M. Buyse**, MD, PhD, Professor of Child Neurology at the University Hospitals Leuven (Belgium) and Principal Investigator for the DELOS trial and lead author of the publication. "It's also a tribute to the hard work of so many scientists, patients and families involved in the 10 years of innovative research in which we have brought idebenone from the lab bench to the patient. Statistically significant and clinically relevant outcomes of primary and secondary endpoints coherently demonstrated that Raxone/Catena reduced the loss of respiratory function and that it was safe and well tolerated. I am very enthusiastic about the positive data from the trial which demonstrate that this drug represents a suitable treatment option to ameliorate a life-threatening complication of the disease."

"With morbidity and mortality in DMD being associated with progressive restrictive lung disease and irreversible loss of lung function, these findings represent an important treatment effect and are of major clinical relevance for patients with DMD", added **Craig McDonald**, MD, Professor and Chair of the Department of Physical Medicine & Rehabilitation at UC Davis (USA), investigator of the DELOS trial and co-author of the *Lancet* publication.

"The degree of slowing of respiratory function loss demonstrated in DELOS is of major clinical relevance for patients with DMD", commented **Nicholas Coppard**, PhD, SVP Development at

Santhera. "Based on this benefit and its well-established safety profile, we are very excited about the prospects of Raxone/Catena as a treatment option for DMD patients and we are currently preparing the regulatory filing dossier for application of marketing authorization both in the US and Europe."

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 course 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 Fast Track designation by the US FDA. Use patent protection extends 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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