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Santhera receives FDA Fast Track Designation for Raxone[®]/Catena[®] (idebenone) for the Treatment of Duchenne Muscular Dystrophy (DMD)

Liestal, Switzerland, April 9, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for Santhera's Raxone[®]/Catena[®] (idebenone) for the treatment of Duchenne Muscular Dystrophy (DMD). FDA's Fast Track program facilitates the development and review of important drugs intended to treat serious conditions and fill an unmet medical need for the purpose of getting them to the patient earlier. Santhera previously announced that the Phase III trial (DELOS) in DMD met its primary endpoint and demonstrated that Raxone/Catena delayed the loss of respiratory function.

"We are very pleased that the FDA has granted Fast Track designation for Raxone/Catena, which further underscores the unmet medical need for effective treatments for patients with DMD," commented **Thomas Meier**, PhD, CEO of Santhera. "On the basis of the positive data from our Phase III trial with Raxone/Catena in DMD, we have started to prepare a New Drug Application and plan to meet with the FDA in the coming weeks to discuss our NDA dossier in a pre-NDA meeting."

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 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.

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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 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 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.

Raxone[®] and Catena[®] are trademarks of Santhera Pharmaceuticals.

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