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Santhera Announces Successful Outcome of Phase III Study with Catena®/Raxone® in Duchenne Muscular Dystrophy

Liestal, Switzerland, May 13, 2014 – Santhera Pharmaceuticals (SIX: SANN) announces today that its Phase III DELOS study of orally administered Catena®/Raxone® (INN: idebenone) in patients with Duchenne Muscular Dystrophy (DMD) met the primary endpoint and achieved its primary objective of delaying the loss of respiratory function compared to placebo.

The Phase III, double-blind, placebo-controlled DELOS study randomized 65 DMD patients who were 10-18 years of age and who were not using concomitant corticosteroids. The study met the primary endpoint, the difference between Catena®/Raxone® and placebo in the change from baseline to week 52 in Peak Expiratory Flow (p=0.04). Peak Expiratory Flow is a measure of respiratory muscle strength, the decline of which is a major contributing factor to morbidity and mortality in DMD. Catena®/Raxone® (900 mg/day) was safe and well tolerated with adverse event rates comparable to placebo. Other endpoint analyses are ongoing and results of these will be disclosed shortly.

“We are thrilled by these results which are consistent with the findings of our Phase II DELPHI and DELPHI Extension studies,” commented Thomas Meier, CEO of Santhera. “As acknowledged by clinicians and regulatory authorities, preservation of respiratory function is a major benefit for patients with DMD. On the basis of these results we will approach the US and European authorities for discussions on the most expeditious regulatory pathway to approval.”

“I am very enthusiastic about the positive outcome for the DELOS trial. This is tremendously good news for patients with DMD since it indicates that Catena®/Raxone® can mitigate respiratory weakness and dysfunction,” commented Gunnar Buyse, M.D., Ph.D., Professor of Child Neurology at the University Hospitals Leuven (Belgium) and Principal Investigator for the DELOS study.

Santhera holds global commercialization rights to the DMD program, which has been granted orphan drug designation and patent protection in the US and EU.

About Catena®/Raxone® as Treatment of Duchenne Muscular Dystrophy
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 approximately 1 in 3,500 live born males worldwide. DMD is characterized by a complete 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 due to respiratory failure. Idebenone is a synthetic short-chain benzoquinone and a cofactor for the enzyme NAD(P)H:quinone oxidoreductase (NQO1) capable of stimulating mitochondrial electron transport and supplementing cellular energy levels.

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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 Catena®/Raxone® as treatment for patients with Leber's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS), all of them areas of high unmet medical need with no current therapies. Santhera previously received temporary approval (cATU) for Raxone® in the treatment of LHON in France and has recently submitted a Marketing Authorization Application to the European Medicines Agency for the treatment of LHON in the European Union. For further information, please visit the Company's website www.santhera.com.

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