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# Santhera and Parent Project Muscular Dystrophy (PPMD) Team Up on Benefit / Risk Study in Duchenne Muscular Dystrophy

**Liestal, Switzerland, November 25, 2014 – Santhera Pharmaceuticals (SIX: SANN) and Parent Project Muscular Dystrophy (PPMD), the leading US advocacy organization working to end Duchenne Muscular Dystrophy (DMD), will collaborate on a benefit/risk study in DMD. The study will focus specifically on patient and caregiver preferences regarding pulmonary therapies in the disease, and will be based on data from Santhera's successful phase III clinical trial with Catena®/Raxone® (idebenone).**

Earlier this year, PPMD convened a broad coalition of over 80 stakeholders to draft and submit the first-ever patient advocacy-initiated guidance for a rare disease to the U.S. Food and Drug Administration (FDA) to help accelerate development and review of potential therapies for DMD. One of the recommendations in the guidance was to create partnerships between patient groups and industry to study the benefit/risk preferences of the disease community to better inform the company's new drug application.

Pursuant to this recommendation, Santhera and PPMD have now initiated such a partnership and collaborate to study patient and caregiver benefit/risk preferences for pulmonary therapy with Santhera's Catena/Raxone. The study will be based on data from Santhera's successful Phase III (DELOS) trial which provided clear evidence of a clinical benefit for Catena/Raxone in delaying the loss of respiratory function in DMD patients not using concomitant glucocorticoid steroids. The preservation of respiratory function is considered to be of major clinical importance.

"Following the successful outcome of our Phase III trial, we are excited about this collaboration with PPMD to determine patients' and caregivers' views of the benefit/risk balance for Catena/Raxone", emphasized **Thomas Meier**, CEO of Santhera. "PPMD has become a powerful and reliable partner of industry in facilitating the development of promising treatment options. With this study of the benefit/risk preferences of the disease community, which is the first of its kind, we will proactively meet regulators' expectations with regard to the content of our NDA filing."

PPMD's Founding President and CEO, **Pat Furlong**, commented: "Since entering the Duchenne space, Santhera has proven their dedication to this community and to finding therapies to treat this disease. They are committed to provide thorough and reliable data and information in the submission to the FDA when they reach that stage. We are happy to collaborate with Santhera's team to ensure the patients' voice is included in the benefit/risk equation. The FDA has told us they want to know what the patients want and thanks to this study, we will be able to tell them."

**About Duchenne Muscular Dystrophy and DELOS**

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 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 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.

DELOS was a Phase III, double-blind, placebo-controlled trial which randomized 64 European and US patients, 10-18 years of age, to receive either Catena/Raxone tablets or matching placebo. The trial met its primary endpoint and demonstrated that Catena/Raxone can delay the loss of respiratory function in patients not taking concomitant glucocorticoid steroids. Detailed data from the DELOS trial were presented recently at the 19th International Congress of the World Muscle Society in Berlin (Germany).

**About Parent Project Muscular Dystrophy (PPMD)**

Parent Project Muscular Dystrophy (PPMD) is the largest most comprehensive nonprofit organization in the United States focused on finding a cure for Duchenne muscular dystrophy—our mission is to end Duchenne. We invest deeply in treatments for this generation of young men affected by Duchenne and in research that will benefit future generations. We advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. We demand optimal care, and we strengthen, unite and educate the global Duchenne community. Everything we do—and everything we have done since our founding in 1994—helps boys with Duchenne live longer, stronger lives. We will not rest until every young man has a treatment to end Duchenne.

Over the last few years, PPMD has implemented a detailed advocacy agenda that included: publication of Putting Patients First, a white paper submitted to the FDA outlining recommendations to speed responsible access to new therapies for Duchenne and other rare, serious and life-threatening neurologic disorders; publication of our benefit/risk study that quantified caregiver preferences regarding emerging therapies, which was shared with the FDA; the above-mentioned draft guidance submitted to the FDA; and now this collaboration with Santhera to best prepare their submission of new drug applications to the Agency.

Go to www.ParentProjectMD.org for more information or to learn how you can support our efforts and help families affected by Duchenne.

**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) 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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