



Santhera Pharmaceuticals Holding AG

Hammerstrasse 49

CH-4410 Liestal / Switzerland

Phone +41 61 906 89 50

Fax +41 61 906 89 51

[www.santhera.com](http://www.santhera.com)

## **Santhera and Parent Project Muscular Dystrophy (PPMD) Announce Results of Benefit / Risk Survey in patients with Duchenne Muscular Dystrophy (DMD)**

Liestal, Switzerland, October 22, 2015 – Santhera Pharmaceuticals (SIX: SANN) and Parent Project Muscular Dystrophy (PPMD), the leading U.S. advocacy organization working to end Duchenne Muscular Dystrophy (DMD), announce the results of a benefit/risk survey in patients DMD and parents of individuals with DMD. The study focused specifically on patient and caregiver preferences for treatments that address disease symptoms that are not directly related to skeletal muscle function. PPMD has submitted the outcomes report of this survey to the FDA in their continuous efforts to share patients' and caregivers' view on emerging treatment options.

This first industry-supported benefit/risk study for patients with Duchenne Muscular Dystrophy (DMD) was inspired and based on data from Santhera's successful Phase III (DELOS) trial, which demonstrated a clinical benefit for Raxone<sup>®</sup>/Catena<sup>®</sup> (idebenone) in delaying the loss of respiratory function in patients with DMD not using concomitant glucocorticoid steroids. The results of the benefit/risk study confirm that the preservation of respiratory function is prioritized by caregivers and boys/men with DMD.

"Since entering the Duchenne space, Santhera has become a reliable partner and proven both its dedication to this community and commitment to incorporate the voices of patients into its regulatory submissions," PPMD's President, **Pat Furlong** remarked. "We are proud having partnered with Santhera for this project, as we see communities' treatment preference and risk tolerance as an important aspect of FDA's decision-making process. This study provides us with data that supports the need for patients to have access to medications that address the pulmonary and cardiac complications of Duchenne and Becker muscular dystrophy."

"PPMD has been a pioneer in the rare disease community and has provided groundbreaking leadership to ensure that patients have a voice in the regulatory process," commented **Thomas Meier**, PhD, Chief Executive Officer of Santhera. "We commend PPMD for elucidating the unique considerations of caregivers and boys and men with DMD related to treatment of pulmonary disease associated with their muscular dystrophy. As PPMD has already submitted the outcomes report of this survey to the FDA, we will be able to make reference to it in the course of our regulatory submission."

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“We hear from parents and people with Duchenne or Becker muscular dystrophy that they want faster drug approvals and regulatory agencies to accept more uncertainty and risk,” explained **Holly Peay**, PhD, lead investigator of the survey and co-principal investigator of PPMD’s Duchenne Connect registry. “But it was vital to conduct a study to systematically assess the preferences and priorities of a large number of patients and their parents.”

Researchers from the Johns Hopkins Bloomberg School of Public Health provided expertise in health economics and methodology for the study.

The results of this benefit/risk study were forwarded today by PPMD to the FDA providing the Agency with important information on the treatment priorities and risk tolerance of the DMD community for treatments that address disease symptoms not directly related to skeletal muscle function. Through this study, the regulators will have access to scientifically-robust data illustrating that study participants value interventions to slow cardiac and pulmonary disease progression highly, and would be willing to accept a higher risk of side effects and treatment burden for pulmonary benefits.

### **About the PPMD Benefit/Risk Survey Study**

The study consisted of three separate research activities to assess: (i) priorities of treatment targets; (ii) treatment preferences and (iii) treatment acceptance. Best/worst scaling was used for the survey design: respondents were presented with a series of hypothetical treatment profiles and asked to choose the best and worst features of each. One hundred fifty-five individuals participated in this study. Most affected individuals had a diagnosis of Duchenne (85%). The study included caregivers of patients who were at least 10 years old, as well as teenagers and adults.

Respondents prioritized treatment targets that address cardiovascular and pulmonary benefits. The greatest priority was for treatments that address weaker heart pumping. Treatments that address lung infections and cough strength were rated as the second and third-most prioritized, respectively. Stratified samples revealed that caregivers and patients had similar priorities.

The analysis of preference scores revealed greatest preference for treatments that target cough strength. Patients and their caregivers demonstrated the greatest preference for treatment that maintains cough strength underlining the importance to maintain adequate airway clearance.

In the treatment acceptance analysis, in which participants answered whether they would use a hypothetical drug if it were available, respondents almost always chose to accept the treatment profile with a strong benefit and little risk, while almost three quarters would accept a treatment with a strong benefit despite the highest risk/burden profile.

Study investigators from PPMD and the Johns Hopkins Bloomberg School of Public Health will disseminate detailed survey results to the patients and caregiver community and scientific community through conferences and publications.

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### **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 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 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, reducing and scavenging reactive oxygen species (ROS) and supplementing cellular energy levels.

DELOS was a Phase III, double-blind, placebo-controlled trial which randomized 64 patients, 10-18 years of age, to receive either Raxone/Catena tablets or matching placebo. The trial met its primary endpoint and demonstrated that Raxone/Catena can delay the loss of respiratory function in patients not taking concomitant glucocorticoid steroids. The positive outcome of the Phase III DELOS study was published in *The Lancet* and presented at the 2015 American Academy of Neurology meeting.

### **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 – their mission is to end Duchenne. PPMD invests deeply in treatments for this generation of people affected by Duchenne and in research that will benefit future generations. They advocate in Washington, DC, and have secured hundreds of millions of dollars in funding. They demand optimal care, and strengthen, unite and educate the global Duchenne community. Everything PPMD does – and everything they have done since their founding in 1994 – helps people with Duchenne live longer, stronger lives. For more information, visit [www.parentprojectmd.org](http://www.parentprojectmd.org).

### **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's lead product Raxone<sup>®</sup> is authorized in the European Union for the treatment of Leber's Hereditary Optic Neuropathy (LHON). Santhera develops Raxone<sup>®</sup>/Catena<sup>®</sup> in two additional indications, Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS), and omigapil for Congenital Muscular Dystrophy (CMD), all areas of high unmet medical need. For further information, please visit the Company's website [www.santhera.com](http://www.santhera.com).

*Raxone<sup>®</sup> and Catena<sup>®</sup> are trademarks of Santhera Pharmaceuticals.*

### **For further information, contact:**

Thomas Meier, PhD, Chief Executive Officer  
Phone +41 61 906 89 64  
[thomas.meier@santhera.com](mailto:thomas.meier@santhera.com)

Christoph Rentsch, Chief Financial Officer  
Phone +41 61 906 89 65  
[christoph.rentsch@santhera.com](mailto:christoph.rentsch@santhera.com)

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**US investor contact:**

Hans Vitzthum, LifeSci Advisors, LLC  
Phone +1 212 915 2568  
[hans@lifesciadvisors.com](mailto:hans@lifesciadvisors.com)

**US Public Relations contact:**

Deanne Eagle, Planet Communications  
Phone +1 917 837 5866  
[deanne@planetcommunications.nyc](mailto:deanne@planetcommunications.nyc)

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