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Santhera Launches U.S. Expanded Access Program with Idebenone for Patients with Duchenne Muscular Dystrophy (DMD)

Liestal, Switzerland, February 6, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces the launch of a U.S. Expanded Access Program (EAP) referred to as BreatheDMD with idebenone for patients with DMD.

Through the BreatheDMD program, eligible patients in the U.S. with DMD who are 10 years and older and in respiratory function decline, can obtain access to investigational idebenone, at no cost, through a growing network of research centers across the U.S.

EAPs such as **BreatheDMD** are permitted by the U.S. Food and Drug Administration (FDA). Such programs allow eligible patients with serious or life-threatening diseases or conditions, where there is a lack of satisfactory therapeutic alternatives, to gain access to a medicine under investigation before it is approved by regulatory authorities.

Details regarding this EAP, including eligibility requirements, can be obtained by visiting the website www.breatheDMD.com.

Idebenone is a medicine that is under investigation for the treatment of DMD. It has not yet been approved by the U.S. FDA and the safety and efficacy continue to be evaluated in clinical trials.

"I commend Santhera for developing the BreatheDMD program, allowing eligible patients to access idebenone, an investigational medicine that has shown the ability to slow respiratory function decline in a completed Phase III (DELOS) trial. We desperately need alternative treatment options for patients with DMD that target the respiratory decline component of the disease," said **Oscar H. Mayer**, MD, a pediatric pulmonologist focused on treating neuromuscular diseases.

"Several patient advocacy organizations in the U.S., including Parent Project Muscular Dystrophy (PPMD), Jett Foundation, CureDuchenne and the Muscular Dystrophy Association (MDA) have consistently mentioned that patients need access to additional treatment options, especially older patients in respiratory decline, and that these patients cannot afford to continue to wait. To help address this important unmet need, Santhera is proud to be launching this EAP, allowing eligible patients to obtain access to investigational idebenone," said **Thomas Meier**, PhD, CEO of Santhera.

BreatheDMD follows shortly after Santhera has launched its educational website, www.TakeABreathDMD.com, where patients and families residing in the U.S. can find practical, in-depth information in one centralized location to help manage respiratory complications, including information about breathing, coughing and pulmonary care.

About Duchenne Muscular Dystrophy
DMD is one of the most common and devastating types of muscle degeneration and leads to progressive muscle weakness starting at an early age. DMD is a genetic, degenerative disease that occurs almost exclusively in males with an incidence of up to 1 in 3,500 live male births worldwide. With age, progressive

respiratory muscle weakness affecting thoracic accessory muscles and the diaphragm causes respiratory disease, impaired clearance of airway secretions, recurrent pulmonary infections due to ineffective cough, and eventually respiratory failure. There is currently no treatment approved for slowing loss of respiratory function in patients with DMD.

About Idebenone in Duchenne Muscular Dystrophy

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, muscle wasting, 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 52-week study which randomized 64 patients, not taking concomitant steroids, to receive either idebenone (900 mg/day) or matching placebo. The study met its primary endpoint, the change from baseline in Peak Expiratory Flow (PEF), which demonstrated that idebenone can slow the loss of respiratory function. Data from the DELOS trial are published in peer-reviewed medical journals: Buyse et al., The Lancet 2015, 385:1748-1757; McDonald et al., Neuromuscular Disorders 2016, 26:473-480; Buyse et al., Pediatric Pulmonology 2017, 52:580-515; Mayer et al., Journal of Neuromuscular Diseases 2017, 4:189-198; Meier et al., Neuromuscular Disorders 2017, 27:307-314.

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® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON). Santhera is currently conducting the Phase III SIDEROS trial with idebenone in patients with Duchenne muscular dystrophy (DMD) in respiratory function decline and using concomitant glucocorticoids. In collaboration with the U.S. National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing idebenone in a third indication, primary progressive multiple sclerosis (PPMS), and another product – omigapil – for congenital muscular dystrophy (CMD), both also areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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