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

Santhera Reports Positive Outcome for Catena®/Raxone® in Phase III DMD Trial Supported by Additional Respiratory Function Data

Liestal, Switzerland, May 22, 2014 – Santhera Pharmaceuticals (SIX: SANN) today reported that the results of secondary respiratory function endpoints from the on-going analysis of the DELOS trial in Duchenne Muscular Dystrophy (DMD) corroborate the positive outcome for the primary endpoint. These data provide further supportive evidence of a treatment benefit for Catena®/Raxone® in DMD.

As previously announced, the DELOS trial met the primary endpoint, the difference between Catena®/Raxone® and placebo in the change from baseline to week 52 in Peak Expiratory Flow (PEF as percent predicted, PEF%p). Hospital-based spirometry assessments demonstrated that Catena®/Raxone® significantly reduced the annual decline in PEF%p by 66% compared to patients taking placebo. The average annual decline in PEF%p was 9.0% for placebo (Baseline: 54.3%; Week 52: 45.3% (n=27), p<0.001) versus 3.1% for Catena®/Raxone® (Baseline PEF%P: 53.1%; Week 52: 50.1% (n=30); p=0.13) for a treatment group difference in change from Baseline to Week 52 of 5.96% (p=0.04).

Santhera today announced that this finding has been corroborated by the results of secondary endpoints assessing respiratory function in all randomized and treated subjects. When measured weekly by the patient at home using the hand-held ASMA-1 device (secondary endpoint), Catena®/Raxone® significantly reduced the annual decline in PEF%p by 80% compared to patients taking placebo. The ASMA-1 device showed a significant 9.0% decline in PEF%p occurred between Baseline and Week 52 in the placebo group (n=31; p<0.001), compared to a non-significant decline of 1.8% in the Catena®/Raxone® group (n=31; p=0.44), for a treatment group difference in change from Baseline to Week 52 of 7.2% (p=0.03).

Furthermore, for Forced Expiratory Volume in 1 second (as percent predicted, FEV1%p), an additional endpoint for respiratory muscle strength, Catena®/Raxone® significantly reduced the annual decline by 78% compared to patients taking placebo. The annual decline in FEV1%p in the placebo group was 10.7% compared to 2.4% in the Catena®/Raxone® group (p=0.03).

Importantly, the outcome for Forced Vital Capacity (as percent predicted, FVC%p), a measure of restrictive lung disease predictive of morbidity and mortality in DMD, also supported a treatment benefit of Catena®/Raxone®. The annual decline in FVC%p was reduced by 37% in Catena®/Raxone®-treated patients (9.0% decline in FVC%p in the placebo group versus a 5.7% decline in the Catena/Raxone group; p=0.08).

No differences were observed between treatment groups in Maximal Inspiratory or Expiratory Pressures or in Peak Cough Flow.

In summary these outcomes provide clear evidence of a clinical benefit for Catena®/Raxone® in delaying the loss of respiratory function in patients with DMD compared to placebo.

As also reported previously, treatment with Catena®/Raxone® was safe and well tolerated. A total of 93.8% of Catena®/Raxone®-treated and 94.1% of placebo-treated patients experienced at least one Adverse Event (AE). Serious AEs were reported in 6.3% of Catena®/Raxone® treated and in 14.7% of placebo-treated patients. Nasopharyngitis (25.8%) and headache (19.7%) were the most common adverse events but there were no differences in the incidence of these events between the treatment groups. Diarrhoea was slightly more commonly observed in Catena®/Raxone®-treated patients (25.0% versus 11.8%), whilst upper respiratory tract infections were more frequently observed in placebo-treated patients (17.6% versus 6.3%). Overall adverse events were mild to moderate in intensity.

"We are very excited to observe this treatment benefit for Catena®/Raxone® in delaying respiratory deterioration in DMD, as evidenced by the significant outcome for the primary PEF endpoint in DELOS. The positive results of secondary outcomes, in addition to the primary endpoint, are clearly supportive and establish the clinical meaningfulness of Catena®/Raxone® treatment. This is of critical importance for the demonstration of substantial evidence of effectiveness in the regulatory process", explained Nick Coppard, SVP Development at Santhera. "Having demonstrated a significant and clinically relevant benefit without safety concerns, we believe we are well positioned to discuss regulatory approval with the authorities".

These data are presented today at the Bio€quity Europe 2014 Conference in Amsterdam and will be available for download on the Company's website www.santhera.com under *Investors/ Presentations.*

About DELOS
DELOS was a Phase III, double-blind, placebo-controlled trial which randomized and treated 64 European and US patients with DMD who were not receiving concomitant corticosteroids to either Catena®/Raxone® tablets (900mg: 300mg 3 times daily, N=31) or to matching placebo (N=33). Their average age was 14.3 years and 5 were ambulatory and 59 non-ambulatory at Baseline. There were no eligibility criteria for mutational status. The study was designed to assess the efficacy of Catena®/Raxone® in improving or delaying the loss of respiratory function in patients with DMD not using corticosteroids compared to placebo. The preservation of respiratory function is acknowledged by clinicians and by regulatory authorities as of major clinical importance for patients with DMD.

About 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 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.

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 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 (MAA) to the European Medicines Agency for the treatment of LHON in the European Union. Santhera recently estimated that the combined annual peak sales potential for Catena®/Raxone® in the LHON and DMD indications could reach CHF 600 million.

For further information, please visit the Company's website www.santhera.com.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

For further information, contact
Thomas Meier, Chief Executive Officer
Phone: +41 61 906 89 64
thomas.meier@santhera.com

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.