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Santhera and Biozentrum Basel participate in the pan-European TREAT-NMD initiative to advance the treatment of rare neuromuscular diseases

Liestal and Basel, Switzerland – Santhera Pharmaceuticals, a Swiss specialty pharmaceutical company with a focus on neuromuscular diseases, and the Biozentrum at the University of Basel today announced their participation in a pan-European network initiative, called TREAT-NMD (Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases). The program aims at coordinating research into rare neuromuscular diseases (NMDs) and seeks to accelerate the development of effective therapies for these often fatal conditions. NMDs are an area of high unmet medical need with no current therapies.

The TREAT-NMD is a network of excellence funded by a CHF 16 million (EUR 10 million) grant from the European Union's 6th Framework Programme, which will be phased over five years. The initiative brings together eminent scientific researchers, medical doctors, patient organizations and industry specialists from 21 organizations in 11 European countries. TREAT-NMD will lay the foundation for a long-term collaboration between partners in Europe and abroad to advance the development of effective therapies for these devastating diseases.

NMDs are mostly inherited diseases and affect around 200,000 people in Europe in a large number of conditions, including Duchenne Muscular Dystrophy and others. They are present in all populations and affect both children and adults. Most NMDs result in chronic long-term disability and pose a significant psychological, social and financial burden for the patients and their families.

Santhera is leading a TREAT-NMD activity package aimed at accelerating the preclinical phase of new therapeutic treatment development, to which CHF 1 million (EUR 650.000) of the overall budget has been allocated. The Company will select disease-relevant efficacy readout parameters and develop standardized protocols and procedures for harmonizing and accelerating preclinical studies of drug candidates. In parallel, Professor Rüegg's group at the Biozentrum will select appropriate preclinical disease models. Standardized procedures and disease models will allow researchers to appropriately compare results

which will clearly reduce the number of experiments necessary to identify possible treatment strategies and predict clinical outcome.

Santhera's participation in the TREAT-NMD network is based on its focus on developing therapies for NMDs and its expertise in advancing drug candidates through preclinical and clinical development programs for these diseases. The Company is currently investigating two compounds in four Phase II and III clinical studies and has several other preclinical programs in NMDs and movement disorders.

Dr Thomas Meier, CSO of Santhera said: "We are very proud to be participating in this initiative as it brings together some of the world's leading doctors and scientists. TREAT-NMD aims at improving treatment and finding cures for thousands of patients. Our inclusion in this program reflects both our scientific and clinical expertise in this specialist therapeutic area."

The Biozentrum at the University of Basel has a long tradition as an interdisciplinary institute working in fundamental to applied research. Important achievements related to muscular dystrophies are proof-of-concept studies in disease-relevant animal models that might open new possibilities for the treatment of congenital muscular dystrophies.

Markus A. Rüegg, Professor for Neurobiology at the Biozentrum, said: "We look forward to working closely with scientists from academia and the industry to test and apply new research into NMDs. TREAT-NMD has ambitious goals to coordinate this research and provide a framework that will accelerate the translation of basic research into the development of new therapies for these devastating diseases."

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About Santhera

Santhera Pharmaceuticals (SWX: SANN) is a Swiss specialty pharmaceutical company focusing on the discovery, development and marketing of small molecule pharmaceutical products for the treatment of severe neuromuscular diseases. Santhera's vision is to become a leading specialty pharmaceutical company offering therapies for a number of indications in this area of high unmet medical need which includes many orphan indications with no current therapy.

Santhera currently has four clinical-stage development programs, three of which are investigating its lead compound, SNT-MC17/idebenone, in the treatment of Friedreich's Ataxia (FRDA), Duchenne Muscular Dystrophy (DMD) and Leber's Hereditary Optic Neuropathy (LHON). The fourth clinical program is investigating JP-1730/fipamezole for the treatment of Dyskinesia in Parkinson's Disease (DPD) in cooperation with Juvantia, the compound's owner. The most advanced program, SNT-MC17/idebenone in FRDA, has entered pivotal Phase III clinical development; the other clinical programs are in Phase II. Santhera's drug pipeline comprises another three preclinical programs in cancer cachexia, DMD and type 2 diabetes (out licensed to Biovitrum).

For further information on Santhera, please visit www.santhera.com.

About the Biozentrum at the University of Basel

The Biozentrum, founded in 1971, was among the first institutions world-wide dedicated to molecular biology. It hosts Nobel laureates and world-renowned developmental biologists and biochemists. Scientists at the Biozentrum investigate the mechanisms important for neuromuscular function and dysfunction and the degeneration of neurons. In particular, the laboratory of Markus A. Rüegg is engaged in developing therapeutic approaches for the treatment of congenital muscular dystrophy.

For further information on the Biozentrum, please visit www.biozentrum.unibas.ch.

About TREAT-NMD

TREAT-NMD will encourage experts in the field of neuromuscular diseases to work together to share good practice and to improve global standards of care. The network of excellence is launched 20 years after scientists found the gene that caused Duchenne Muscular Dystrophy (DMD) but as yet no cure has been found. The initiative is co-coordinated by Kate Bushby and Volker Straub, Professors of Neuromuscular Genetics with Newcastle University's Institute of Human Genetics, who are based at Newcastle's Centre for Life. The 11 countries involved are the UK, Spain, France, Germany, Switzerland, Belgium, Holland, Italy, Finland, Sweden and Hungary.

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