



## **Press Release**

**October 19, 2005**

---

### **Santhera Targets New Indication with its Lead Compound**

#### ***SNT-MC17 (idebenone) enters Phase IIa study in Duchenne muscular dystrophy***

Liestal, Switzerland, October 19, 2005 -- Santhera Pharmaceuticals AG ("Santhera") of Switzerland announced today that it has started a Phase IIa clinical study with its lead product SNT-MC17 (idebenone) to evaluate its benefits in the treatment of Duchenne muscular dystrophy (DMD). DMD is the most common and devastating type of muscular dystrophy, causing weakness and muscle wasting in young boys for which there is no cure or effective treatment. This is a new potential indication for Santhera's SNT-MC17 (idebenone), which is expected to enter Phase III clinical trials in Europe later this year for Friedreich's Ataxia, another life-threatening neuromuscular disease.

The Phase IIa DMD study is a double-blind, randomised, placebo-controlled trial which aims to assess the efficacy of SNT-MC17 in 10 to 16 year old males with cardiac dysfunction associated with DMD. The primary endpoint is to evaluate cardiac function improvement in DMD patients after one year of treatment. The effect of SNT-MC17 on muscle strength in the limbs and respiratory muscles will also be assessed as secondary endpoints. This study will take place at the University of Leuven in Belgium, and will enroll a total of 21 patients. The principal investigator of this study is Prof. Gunnar Buyse, a distinguished physician in the field of neuromuscular disease.

Thomas Meier, Ph.D., Chief Scientific Officer of Santhera commented: "Duchenne muscular dystrophy is a devastating neuromuscular disease, which affects all of the voluntary muscles, as well as the muscles of the heart and the respiratory system. In recent years, progress has been made by using ventilation support systems to prevent respiratory failure, leaving heart failure as a major cause of mortality in this patient group. By protecting the muscle cells from oxidative stress, a major pathological factor in the disease, with SNT-MC17, we believe that we can improve cardiac function. We will also study whether the general muscle weakening in DMD patients can be slowed down."

Klaus Schollmeier, Ph.D., Chief Executive Officer of Santhera declared: "Santhera's strategy is to investigate and capitalize on the full potential of our lead product in as wide a range of indications as possible. Our strong expertise in the field of neuromuscular disease together with the experts at University of Leuven will enable us to evaluate the potential benefits of our compound in patients affected with Duchenne muscular dystrophy."

- Ends -

#### **About Duchenne muscular dystrophy (DMD)**

Duchenne muscular dystrophy is the most common and devastating type of muscular dystrophy. It is a genetic, degenerative disease that is inherited in an X-linked recessive mode. DMD affects approximately 30,000 to 50,000 patients worldwide and its incidence is approximately 1 in 3,500 live born males. Women can be carriers of DMD but usually exhibit

no symptoms. DMD is characterized by a complete loss of the dystrophin protein, leading to impaired calcium homeostasis and elevated oxidative stress in muscle cells. This results in progressive muscle weakness and wasting and the loss of ambulation in teenage patients. Dilated cardiomyopathy is commonly associated with this disease leading to early morbidity and mortality in DMD patients, usually in their twenties.

### **About Santhera**

Santhera Pharmaceuticals AG is a Swiss biopharmaceutical company focused on the discovery, development and marketing of small molecule pharmaceutical products for the treatment of neuromuscular diseases. The Company's lead product, SNT-MC17 (idebenone) is about to enter Phase III for the treatment of Friedreich's Ataxia, a rare but devastating disease which is ultimately fatal. Santhera has orphan drug designation for this indication in both the US and EU. The Company intends to market the product in the US, and has exclusively licensed to Takeda rights to market the product in Europe. Santhera has developed a pipeline of preclinical drug candidates which it will progress in neuromuscular diseases and out license in areas outside its core therapeutic focus. Santhera's program on novel DPP IV inhibitors for the treatment of metabolic diseases, including Type II diabetes, is licensed to Biovitrum (Sweden).

Santhera was formed in 2004 through the merger of MyoContract AG and Graffinity Pharmaceuticals AG providing it with a fully integrated platform for the discovery and development of drug candidates. The Company has operations in Basel, Switzerland and Heidelberg, Germany. Santhera has attracted investment from leading global industry investors including Merlin Biosciences Limited, Oxford Bioscience Partners, NGN Capital, 3i Group plc, Carnegie Asset Management, The Novartis Venture Fund, Varuma AG, GIMV, Heidelberg Innovation, Clariden Bank, The Dow Chemical Company, TechnoStart, tbg, Altana Innovationsfonds, the Swiss Foundation for Research on Muscle Diseases, and private investors. Since its formation in 2004 Santhera has raised €28 million.

For further information, please visit [www.santhera.com](http://www.santhera.com).

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

#### **Santhera Pharmaceuticals**

Klaus Schollmeier, CEO  
Thomas Meier, Chief Scientific Officer

Tel: +41 (0) 61 906 89 52  
Tel: +41 (0) 61 906 89 87  
[thomas.meier@santhera.com](mailto:thomas.meier@santhera.com)

#### **Media contact: Citigate**

Chris Gardner  
David Dible

Tel : +44 (0) 207 638 9571  
Tel : +44 (0) 207 638 9571  
[david.dible@citigatedr.co.uk](mailto:david.dible@citigatedr.co.uk)