



# Dedicated to the Discovery, Development and Commercialization of Small-Molecule Therapeutics for Severe Neuromuscular Diseases in Orphan Indications

Santhera (SWX:SANN) is a Swiss-based specialty pharmaceutical company focused on the discovery, development and commercialization of small-molecule pharmaceutical products for the treatment of severe neuromuscular diseases. The Company seeks to address the high unmet medical need associated with most neuromuscular diseases where few, if any, effective therapies currently exist.

## Strategy

Santhera's vision is to become a global market leader in the treatment of neuromuscular diseases which frequently qualify for orphan drug status. The Company's strategy includes the following key elements:

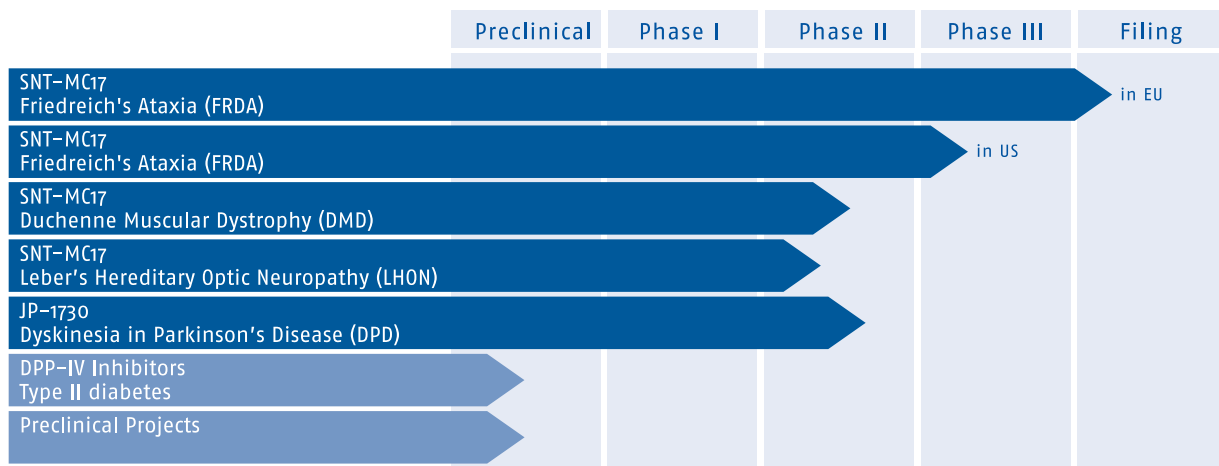
- Obtain marketing approval for SNT-MC17 (INN: idebenone) in Friedreich's Ataxia (FRDA) in the US and Europe;
- Leverage SNT-MC17 in Duchenne Muscular Dystrophy (DMD) and Leber's Hereditary Optic Neuropathy (LHON);
- Develop a specialist US sales force and global marketing franchise; and
- Further diversify the product portfolio by completing clinical development of JP-1730 (INN: fipamezole) in Dyskinesia in Parkinson's Disease (DPD).

Santhera is leveraging its in-house neuromuscular disease expertise to build a diversified product portfolio. Of equal importance to the active management of the current portfolio is finding new neuromuscular indications for well-characterized pharmaceutical compounds. The Company intends to pursue such candidates in multiple indications where overlapping disease mechanisms or other well-established scientific or medical rationales exist. Santhera also seeks to balance its portfolio by selectively in-licensing or acquiring attractive compounds within its area of therapeutic expertise and out-licensing internally developed programs beyond its core focus.

## Products in clinical development

### SNT-MC17 in Friedreich's Ataxia

FRDA is a serious genetic neuromuscular disorder, primarily affecting Caucasians. It causes degeneration of an individual's nerve and muscle tissue, loss of muscle control, uncoordinated movements (ataxia), muscle wasting, and thickening of heart walls tissue which often leads to premature death. A recent clinical trial



conducted in collaboration with the NIH has shown improvement on neurological parameters and activities of daily living scores in FRDA patients after treatment with SNT-MC17 for six months. Based on this positive data, Santhera intends to file for marketing approval in Europe in summer 2007. In the US, Santhera is preparing to start its pivotal Phase III trial in summer 2007. Marketing rights to SNT-MC17 in FRDA in the EU and Switzerland have been granted exclusively to Takeda.

#### **SNT-MC17 in Duchenne Muscular Dystrophy**

DMD is one of the most common genetic neuromuscular disorders, typically diagnosed young boys. This disorder causes the degeneration of muscle cells, resulting in progressive muscle loss, respiratory failure, weakening of the heart and, ultimately, premature death. Results of a Phase II proof-of-concept trial are expected in the second half of 2007.

#### **SNT-MC17 in Leber's Hereditary Optic Neuropathy**

LHON is a genetic neurological disease that usually affects otherwise healthy adults, the majority of whom are men. The disorder causes a sudden and rapid degeneration of optic nerve cells leading to vision loss

within months. Santhera is currently conducting a Phase II trial to obtain proof of concept that SNT-MC17 prevents or slows the progression of vision loss.

#### **JP-1730 in Dyskinesia in Parkinson's Disease**

Dyskinesia is a common, debilitating condition associated with long-term standard therapies for Parkinson's disease, such as levodopa. DPD is characterized by involuntary, irregular body movements that may result in severe incapacity. Santhera is currently preparing a Phase IIb trial for JP-1730 in the US in co-operation with the compound's owner, Juvantia, whom Santhera has an option to acquire.

## Products in preclinical development

In addition to its clinical pipeline, Santhera has several late-stage, preclinical programs in neuromuscular diseases with proof of concept in disease-relevant animal models. The DPP-IV inhibitor program for metabolic disorders for the treatment of diabetes has been out-licensed to Biovitrum.

#### **Shares**

Listing: SWX Swiss Exchange  
Symbol: SANN  
Total shares: 3,099,156 registered common shares  
Swiss security number: 002714864  
ISIN: CH0027148649  
Common code: 026905214  
First day of trading: November 3, 2006

#### **Address**

Santhera Pharmaceuticals Holding Ltd  
Hammerstrasse 47  
CH-4410 Liestal  
Switzerland

Phone +41 (0)61 906 89 50  
Fax +41 (0)61 906 89 51  
Email [communication@santhera.com](mailto:communication@santhera.com)  
[www.santhera.com](http://www.santhera.com)

#### **Management**

Klaus Schollmeier, Chief Executive Officer  
Barbara Heller, Chief Financial Officer  
Thomas Meier, Chief Scientific Officer  
Helmut Kessmann, Chief Business Officer

#### **Board of Directors**

Michael Lytton, Chairman, Oxford Bioscience Partners  
Hans Peter Hasler, Vice Chairman, Biogen Idec  
Martin Gertsch, CFO ESBATech, former CFO Straumann  
Ruedi Gygax, Novartis Venture Fund  
Georg Nebgen, NGN Capital  
Tim J Rink, former CEO Aurora Biosciences  
Bernd Seitzinger, CEO GPC Biotech

#### **Founded**

2004 through business combination of MyoContract Liestal, Switzerland, and Graffinity Pharmaceuticals, Heidelberg, Germany