



santhera
Pharmaceuticals

At the Edge of Transition into a Product Company

2008 Shareholders' Meeting
Basel, April 21, 2008

Track Record in 2007

Santhera Delivered on All Key Milestones (1)

- **Regulatory**
 - Filing for marketing authorization of SNT-MC17 in FRDA in EU, Switzerland and Canada
- **Clinical**
 - Initiation of Phase III study with SNT-MC17 in FRDA in US; recruiting
 - First time clinical relevant efficacy of SNT-MC17 in DMD
 - Initiation of Phase IIb study with JP-1730 in DPD in US; recruiting
- **Operational**
 - Expansion of partnership with Takeda:
European marketing rights of SNT-MC17 in DMD licensed to Takeda
 - In-licensing of SNT-317 for CMD from Novartis

Track Record in 2007

Santhera Delivered on All Key Milestones (2)



- **Intellectual Property Rights**
 - Orphan drug designation granted in EU and US; now for all 3 indications of SNT-MC17
 - Use patent granted in Canada for SNT-MC17 in FRDA
 - 6 patent families filed for preclinical compounds
- **Financials**
 - Total income of CHF 11.7m
 - Efficient cash management; expenses focused on R&D
 - IPO proceeds untouched at 2007 year-end

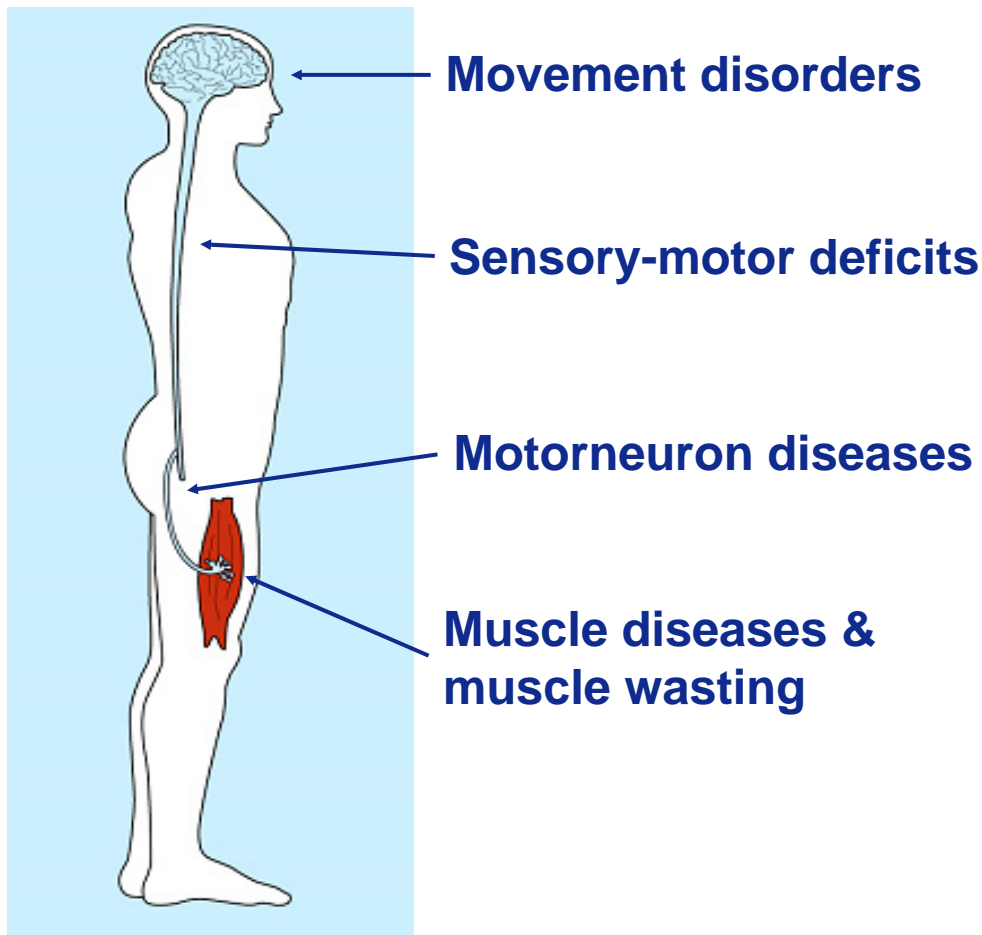
Taking Advantage of Proven Business Model in Orphan Indications

- **Small-molecule drugs**
- **Rare to very rare (orphan) indications**
- **Market exclusivity through orphan drug protection**
- **Well-organized medical communities, patient advocacy groups**
- **High pricing opportunity, niche markets**

Specific business drivers of Santhera

- **Focus on neuromuscular and muscle wasting diseases**
- **Chronic, mostly life-threatening conditions**
- **Limited competition, if any**
- **Own marketing and sales activities in North America, partnerships in other territories**

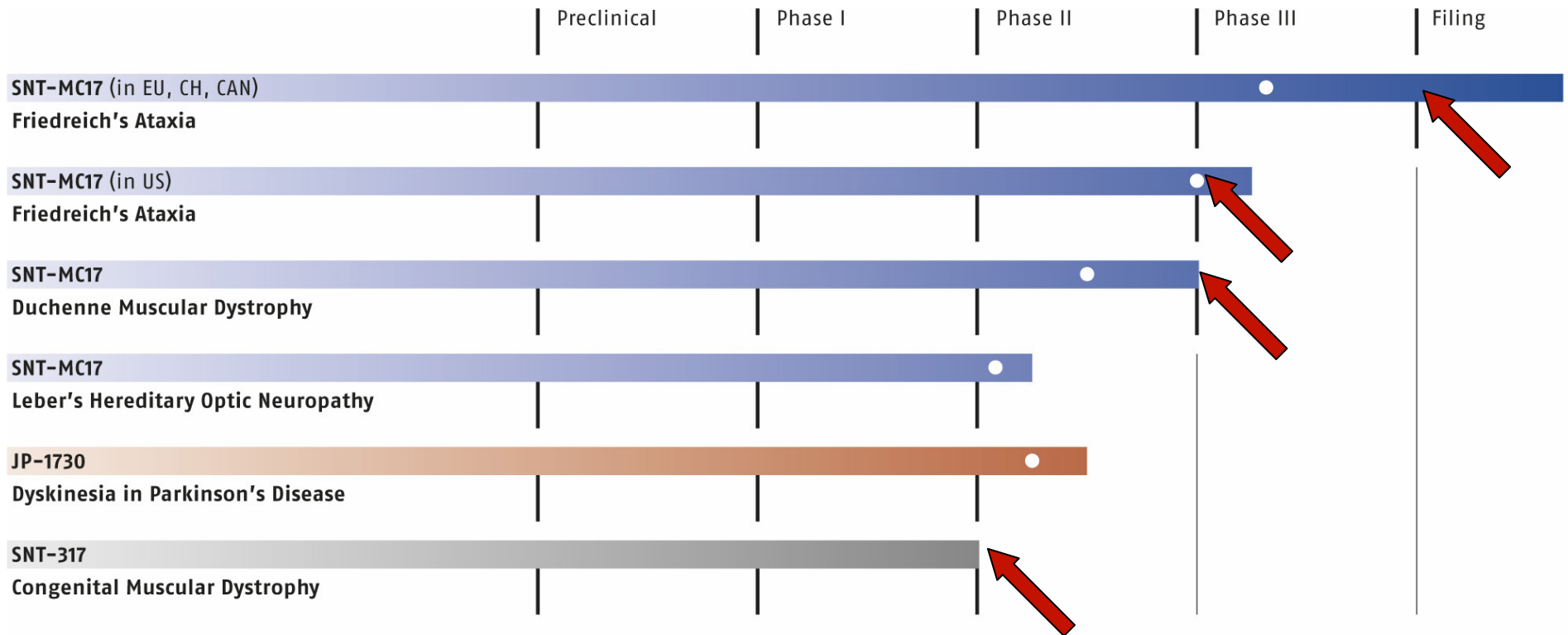
Over 200 NMDs and Movement Disorders Known, Diagnosed but Mostly Not Treated Today



- **Dyskinesia in Parkinson's Disease**
- Huntington's disease
- Spinocerebellar ataxias
- **Friedreich's Ataxia**
- Spinal cord injury
- Charcot-Marie-Tooth neuropathies
- Amyotrophic lateral sclerosis
- Guillain-Barre syndrome
- Peripheral nerve injuries
- **Duchenne Muscular Dystrophy**
- Cachexia (e.g. **Cancer cachexia**)
- **Congenital Muscular Dystrophy**
- Myopathies
- Myasthenia gravis
- Myotonic syndromes
- Ion channel muscle diseases
- Spinal muscular atrophies

Selected examples, areas highlighted in red reflect Santhera's current areas of focus

Clinical Pipeline Reflects Significant Progress in 2007



○ Status January 1, 2007

SNT-MC17 in FRDA: Potentially First Approved Product to Help Patients



SNT-MC17 has shown to improve

- life-threatening heart condition
- neurological functions
- fine motor skills
- quality of life

of patient suffering from Friedreich's Ataxia



SNT-MC17 in FRDA: Santhera's First Key Value Driver



- **Product file submitted for marketing authorization in EU, Switzerland and Canada**
- **Joint pre-launch activities on-going with Takeda, marketing partner in Europe**
- **Pivotal Phase III trial in the US initiated, patient recruitment ongoing in both centers**
- **Market potential for SNT-MC17 in FRDA estimated to be EUR 300m for Europe and US together¹**
- **Product already accepted by medical community**
- **European marketing approval would trigger milestone payment from Takeda**

¹ Company estimate

SNT-MC17 in DMD: First Ever Demonstration of Clinical Efficacy

Data obtained in a small double-blind, placebo-controlled trial shows potential of SNT-MC17 to improve life-threatening

- respiratory functions
- heart parameters

of patients suffering from Duchenne Muscular Dystrophy



SNT-MC17 in DMD: Potential Second Indication for Santhera's Lead Product



- **First time demonstration of potential efficacy in a clinical trial in DMD**
- **Study participants offered a two-year open-label extension study**
- **Meetings pending with European and US health authorities to discuss Phase III program**
- **Begin of Phase III clinical trials would trigger milestone payment from partner Takeda**

SNT-317 in CMD: Novel Molecule in Core Area In-licensed from Novartis



- **A group of severe muscle weaknesses at birth (“floppy infant syndrome“) or early childhood**
- **Prevalence estimated to be 1 in 20,000 to 50,000 newborn¹**
- **No approved pharmaceutical treatment available or in advanced clinical development**
- **Research at Santhera in animal model shows potential of SNT-317 to**
 - **improve muscle strength**
 - **ameliorate skeletal deformation**
 - **reduce early mortality**
- **Exclusive license to develop and market SNT-317 for the treatment of CMD (and other neuromuscular diseases) obtained in July 2007**

¹ British Muscular Dystrophy Campaign

Key Talent Added - Company Still Lean



- **Full time staff of 54 employees in Liestal**
 - 27 in Preclinical Development
 - 14 in Clinical Development
 - 2 in Business Development
 - 2 in Marketing & Sales
 - 10 in General & Administration
- **Two employees in North America (February and April 2008)**
- **44 academics**
- **Average age of 41 years**
- **Additional 17 part-timers, mostly academics in Research & Development and Business Development**

Key Financials 2007



	2007	2006
Cash and cash equivalents	106,618	125,662
Net cash burn (excl. capital increases)	- 19,100	- 27,501
Gross operating and investing cash flow	- 29,646	- 26,534
Revenue / Other operating income	11,665	1,418
Total operating expenses	- 42,792	- 30,057
- whereof R&D expenses	- 23,335	- 17,985
- whereof noncash-relevant share-based payments	- 10,154	- 2,566
Net loss	- 27,871	- 28,258

- **Total income of CHF 11.7m in 2007 (2006: CHF 1.4m)**
- **Monthly net cash-burn in 2007 CHF 1.6m compared to CHF 2.3m in 2006**

Condensed Balance Sheet



IFRS, consolidated, in CHF thousands,
as of December 31

2007 **2006**

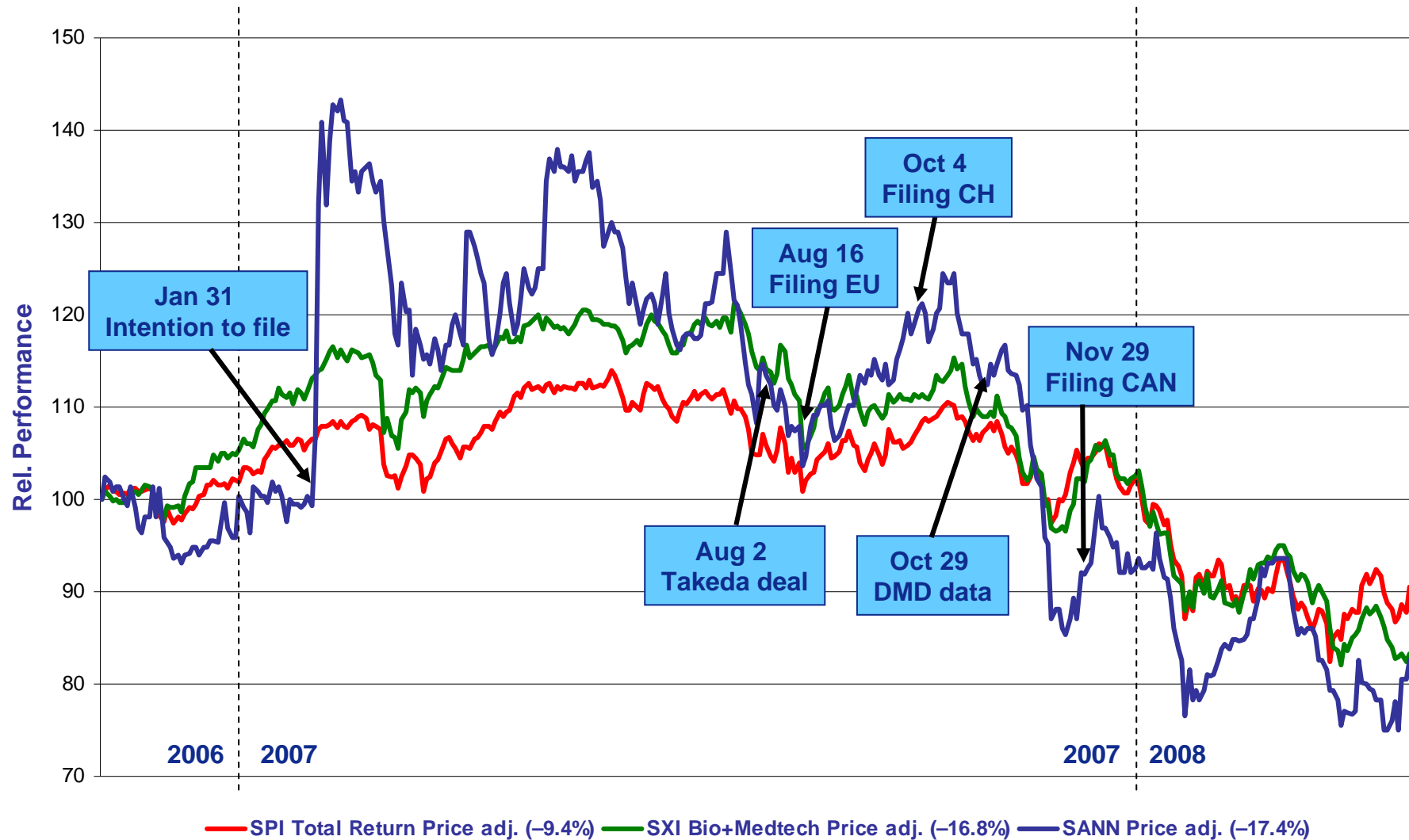
Cash and cash equivalents	106,618	125,662
Other current assets	2,969	2,472
Noncurrent assets	34,588	34,260
Total assets	144,175	162,394
Equity	135,514	152,048
Noncurrent liabilities	272	1,758
Current liabilities	8,389	8,588
Total equity and liabilities	144,175	162,394
Cash per Share (in CHF)	34.20	40.55

- IPO proceeds still available

- Remaining outstanding loans (CHF 1.4m) from tbg fully repaid

- Fully equity financed

Share Performance since IPO





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