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**

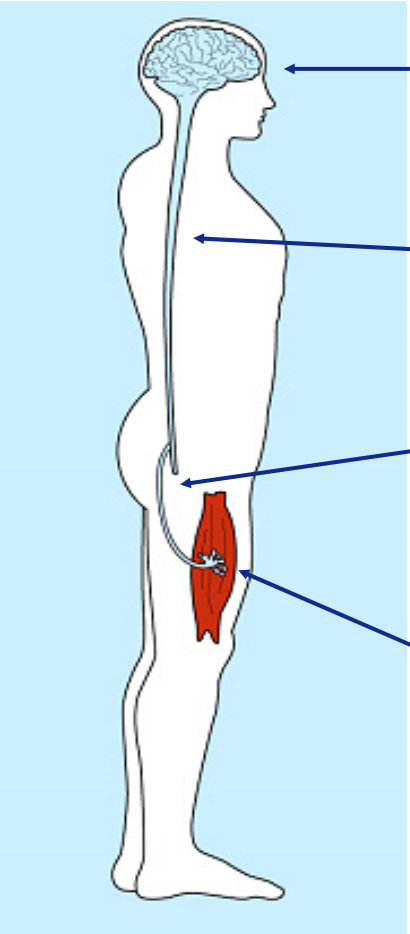
# Taking Advantage of Proven Business Model in Orphan Indications

- **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



**Movement disorders**

- Dyskinesia in Parkinson's Disease
- Huntington's disease
- Spinocerebellar ataxias

**Sensory-motor deficits**

- Friedreich's Ataxia
- Spinal cord injury
- Charcot-Marie-Tooth neuropathies

**Motorneuron diseases**

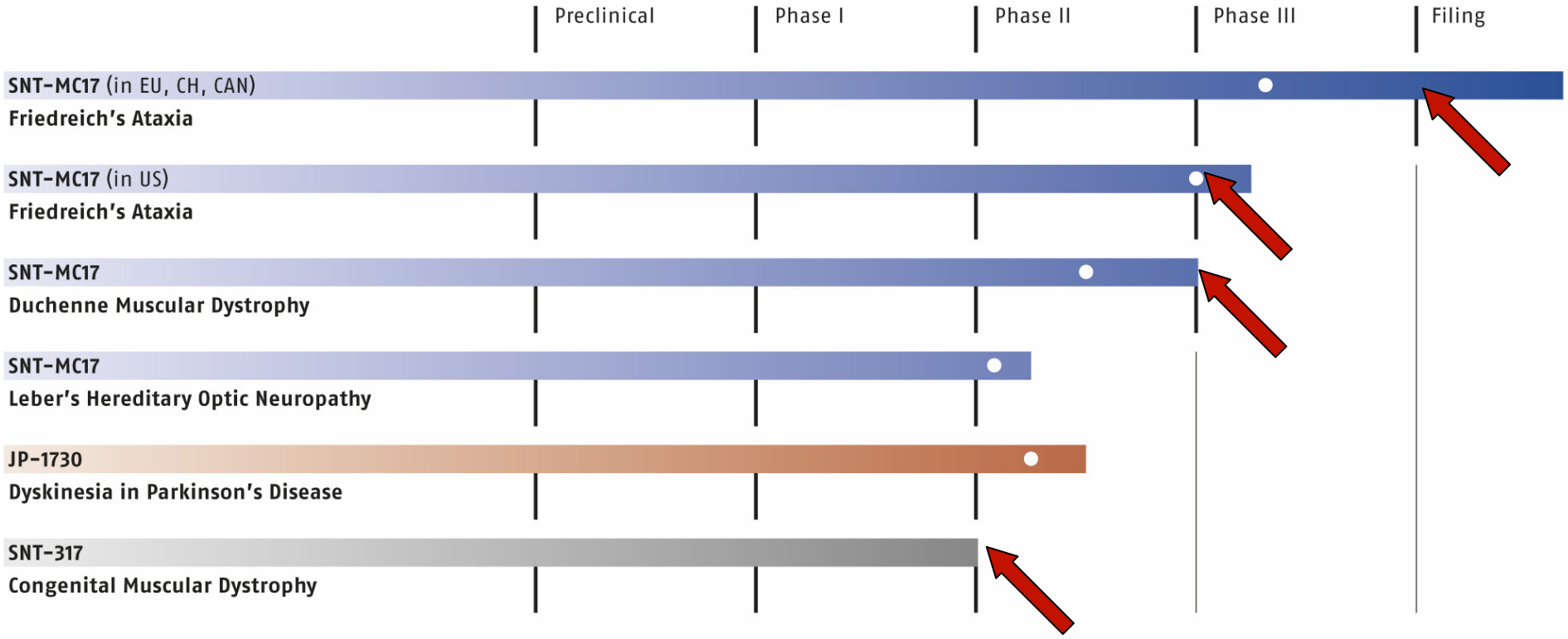
- Amyotrophic lateral sclerosis
- Guillain-Barre syndrome
- Peripheral nerve injuries

**Muscle diseases & muscle wasting**

- 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



# 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 together[1]
- 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 newborn[1]
- 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 |

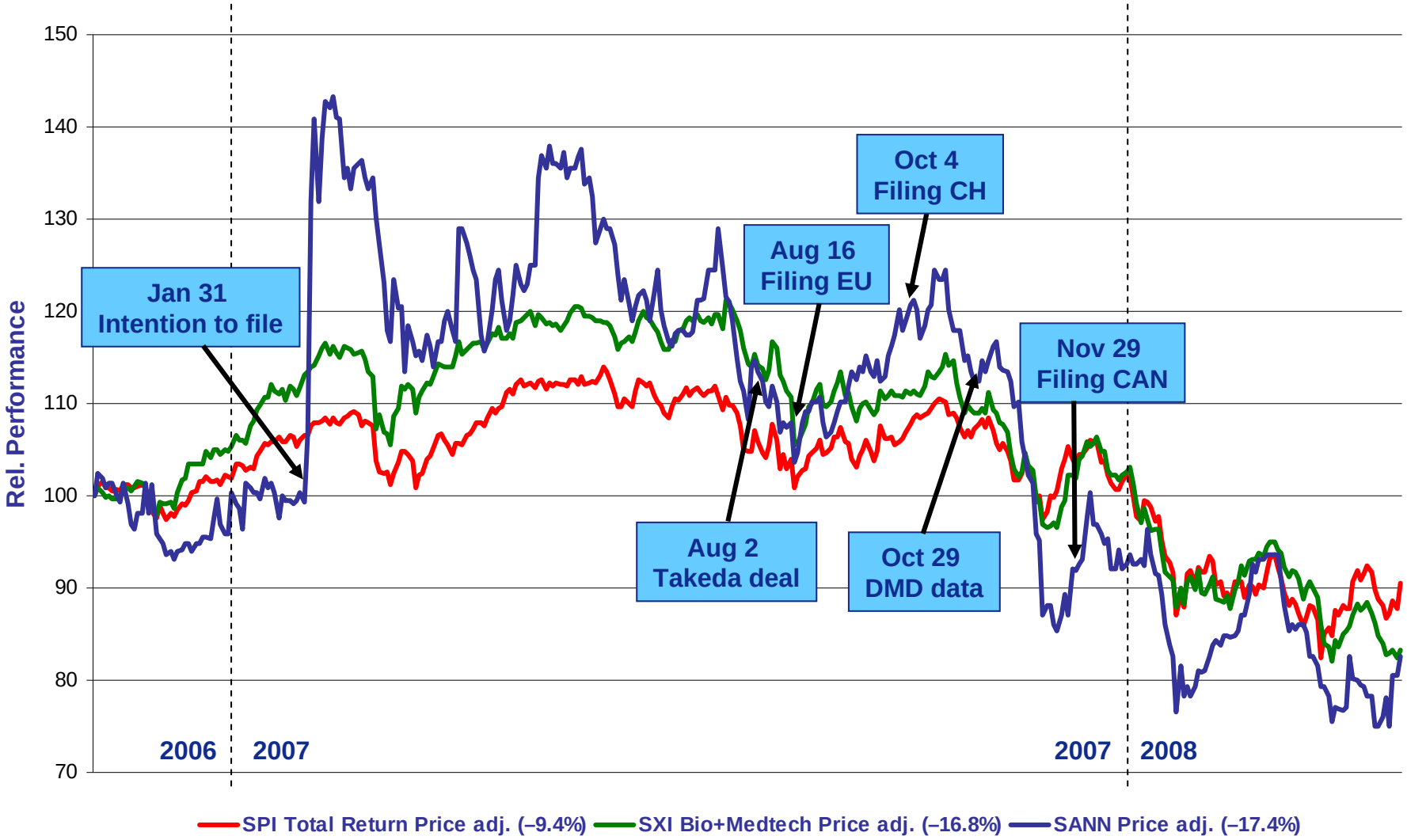
- **Total income of CHF 11.7m in 2007 (2006: CHF 1.4m)**
- **Monthly net cash-burn in 2007 CHF 1.6m compared to CHF 2.3m in 2006**

# 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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