



santhera
Pharmaceuticals

Strong Operational and Financial Results in 2007

Presentation of FY 2007 results
Zurich, February 29, 2008

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Today's Agenda



1. Key Achievements 2007

2. Business Overview

3. Advance of Clinical Development Programs

4. 2007 Financial Results

5. Outlook

Strong Operational and Financial Results in 2007



- **Santhera progresses according to plan**
- **All key operational milestones met**
- **Strong financial position due to increased income and focused spending**

Santhera at Year-end 2007



At the Edge of Transition into Product Company

- **Focus on discovery, development and marketing of small molecules for treatment of orphan neuromuscular diseases**
- **Clinical portfolio of 3 compounds in 5 indications**
- **Lead compound SNT-MC17 in Friedreich's Ataxia filed for market approval in EU, Switzerland and Canada; Launch expected in H2 2008**
- **Multiple clinical projects provide critical mass, momentum, news flow**
- **High quality partnerships along the value chain: Takeda (marketing), Novartis (in-licensing), Juvantia (collaboration), Biovitrum (out-licensing)**
- **Strong financial position, proven management**

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**
 - **Initiation of Phase IIb study with JP-1730 in DPD in US; recruiting**
 - **Clinical relevant efficacy of SNT-MC17 in DMD**
- **Operational**
 - **Partnering of European marketing rights of SNT-MC17 in DMD with Takeda**
 - **In-licensing of SNT-317 for CMD from Novartis**
 - **Expansion of management capacity and skills**

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 focus on R&D
 - IPO proceeds untouched

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Taking Advantage of Proven Business Model in Orphan Markets

- **Small-molecule drugs in 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
 - High unmet medical need, chronic diseases, often life-threatening
 - Limited competition
 - Regional partnerships with strong companies
 - Focus on North America as home market for specialty sales

Current Clinical Portfolio

Promising Market Potential in Orphan Indications

SNT-MC17 in FRDA

- ~20,000 patients in Europe and North America; primarily Caucasians
- Life-long treatment required
- ~EUR 300m market¹

SNT-MC17 in LHON

- ~35,000 patients worldwide; mainly healthy adult males
- Chronic treatment expected
- ~EUR 400m market¹

SNT-317 in CMD

- Incidence of 1 in 20,000 to 50,000 newborn³
- Life-long treatment required
- >EUR 100m market¹

SNT-MC17 in DMD

- ~30,000 patients worldwide; boys, diagnosed at age of 3-5 years
- Life-long treatment required
- ~EUR 400m market²

Combined market opportunity of EUR 1.7bn

JP-1730 in DPD

- ~200,000 patients worldwide
- Chronic treatment required
- ~EUR 500m market²

¹ Company estimates

² Frost & Sullivan

³ British Muscular Dystrophy Campaign

Today's Agenda



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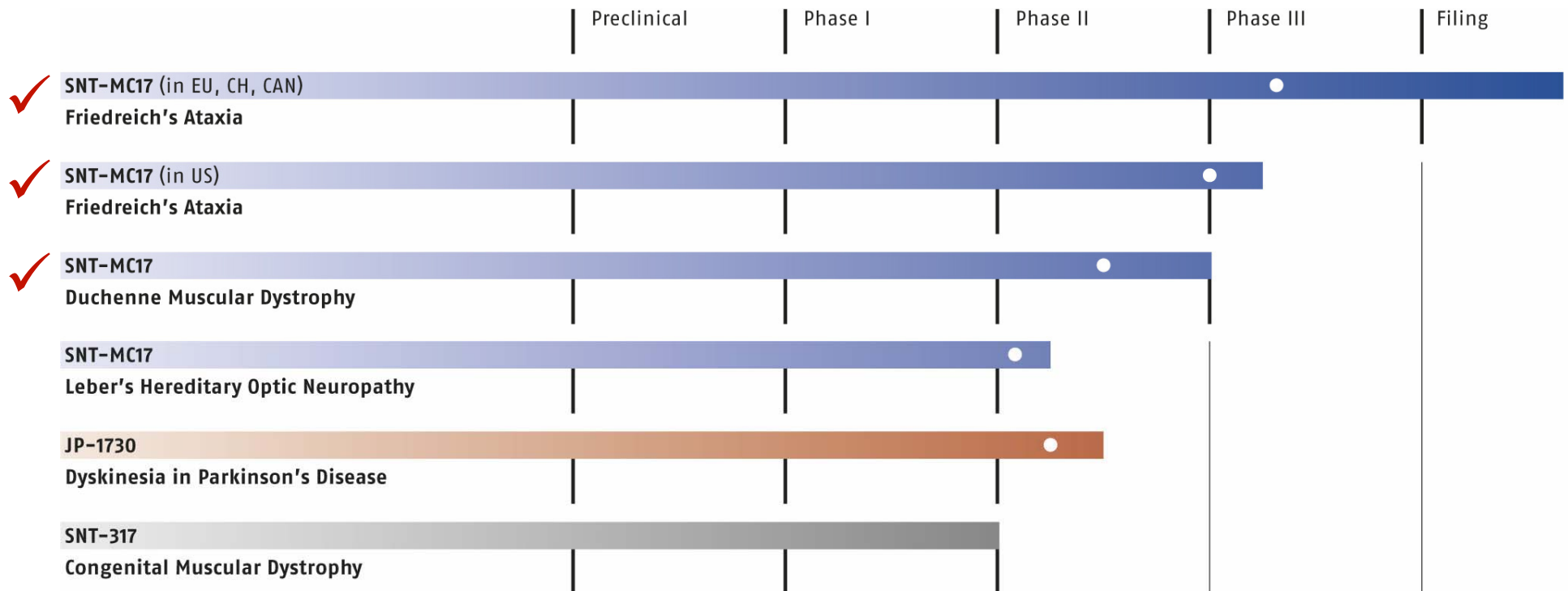
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Clinical Pipeline

Significant Progress in 2007



○ Status January 1, 2007

Friedreich's Ataxia (FRDA) – No Therapy Available for Severe Neuromuscular Disease

- **Severe genetic disorder**
 - Degeneration of nerve and muscle tissue, loss of muscle control
 - Impaired movements, muscle wasting
 - Thickening of heart walls (cardiomyopathy)
- **Caused by reduced level of *frataxin*, key energy protein in mitochondria**
- **Onset 5–15 years, life expectancy 35–50 years**
- **Females and males, predominantly Caucasians**
- **~ 20,000 total patients in Europe and North America**
- **No approved pharmacological treatment available**
- **Chronic disorder, requires life-long treatment**
- **~ EUR 300m market (Company estimate)**



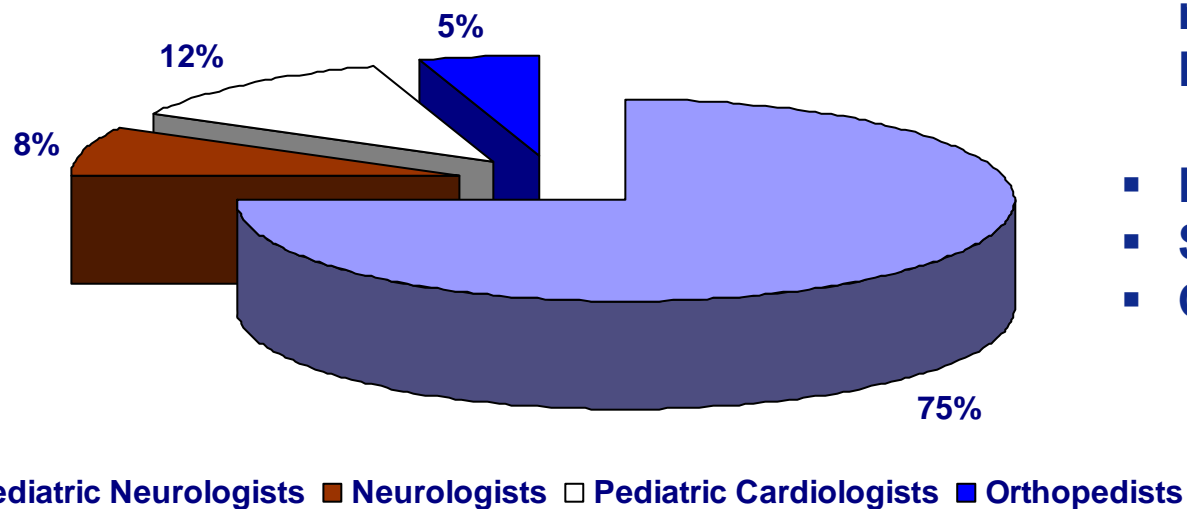
SNT-MC17 in FRDA

Santhera's First Key Value Driver



- **File submitted for marketing authorization in EU, Switzerland, Canada**
- **Joint pre-launch activities on-going with Takeda, marketing partner in Europe**
- **Pivotal Phase III in the US initiated after SPA process with FDA; recruiting into 2 centers**
- **Supporting evidence for business case provided by market research**
- **In US, premium-priced product expected by managed care**

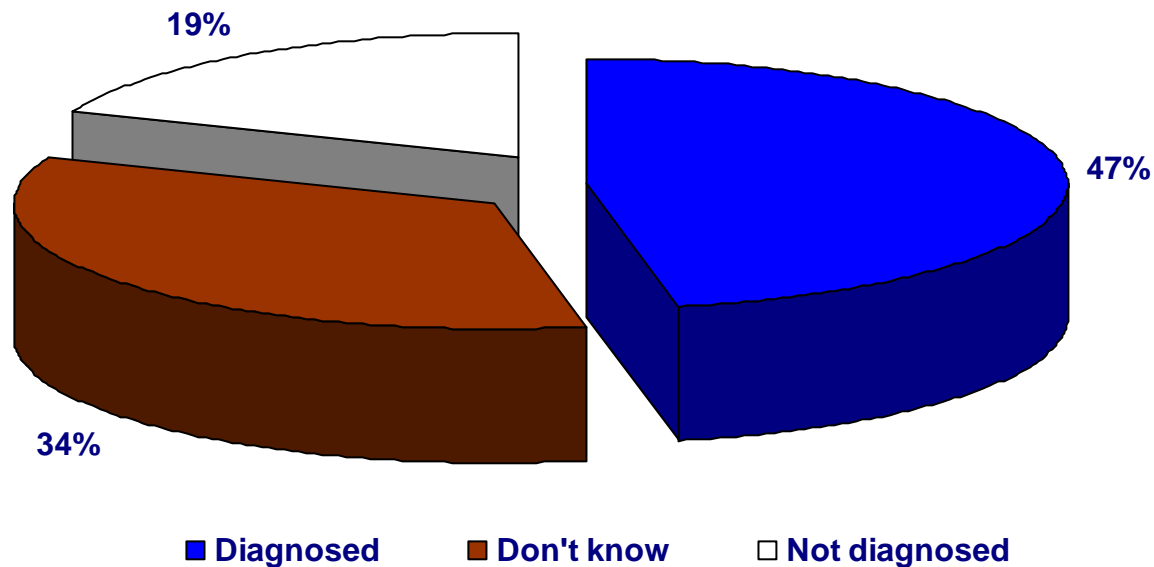
Neurologists and Pediatric Neurologists Prime Diagnosing Specialties for FRDA



- Pediatric neurologists and neurologists diagnose 4 out of 5 FRDA patients
- Defined target group
- Small number of specialists
- Opportunity for niche marketing

Source: Independent market research

Physicians Estimate – Up to Half of All FRDA Patients Not Diagnosed



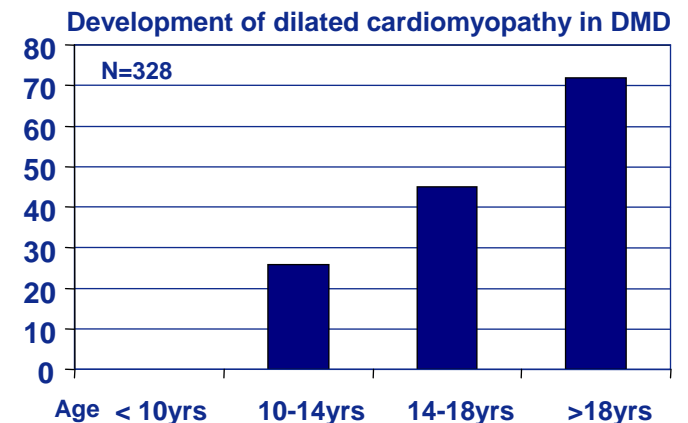
- Fast market access/penetration
- Long-term growth opportunities

Source: Independent market research

Duchenne Muscular Dystrophy (DMD)

Second treatment opportunity with SNT-MC17

- **Most common form of muscular dystrophy**
 - Progressive muscle weakness, skeletal deformities
 - Affected motor skills
 - Respiratory failure, cardiac complications
- **Caused by a deficiency of dystrophin**
- **Onset 3–5 years, life expectancy 30–35 years**
- **Males, all ethnicities**
- **~ 30,000 total patients worldwide**
- **No approved pharmacological treatment available**
- **Chronic disorder, requires life-long treatment**
- **~ EUR 400m market (Frost & Sullivan)**



Source: Nigro G., Int. J. Cardiol 1990

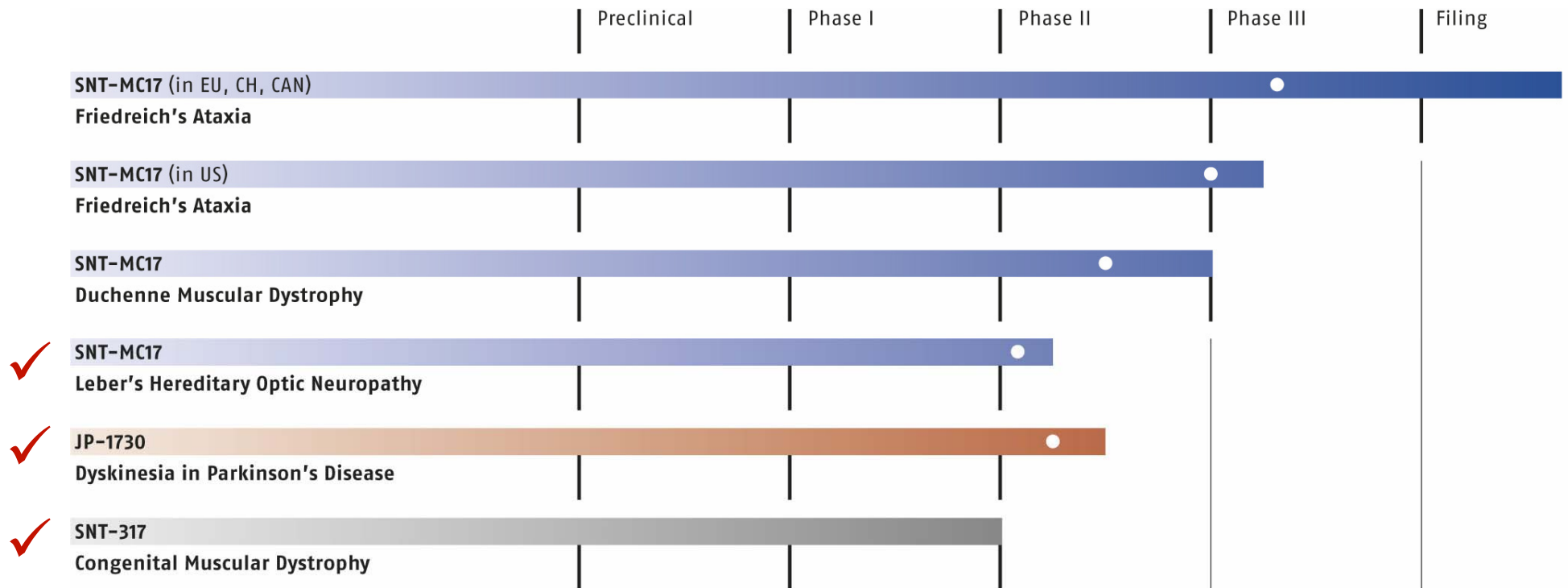
SNT-MC17 in DMD

Clinically Relevant Efficacy Data

- **Strong scientific rationale for mode of action**
- **Convincing data from long term studies in mdx mouse, a disease-relevant model**
- **Efficacy in double blind, placebo-controlled Phase II of 12 months duration**
 - On functional cardiac parameter, e.g. peak systolic radial strain of the LV inferolateral wall
 - On functional respiratory parameter, e.g. peak flow
- **Medication was well tolerated in pediatric DMD patients**
- **Participants offered long-term extension study**

Clinical Pipeline

Significant Progress in 2007



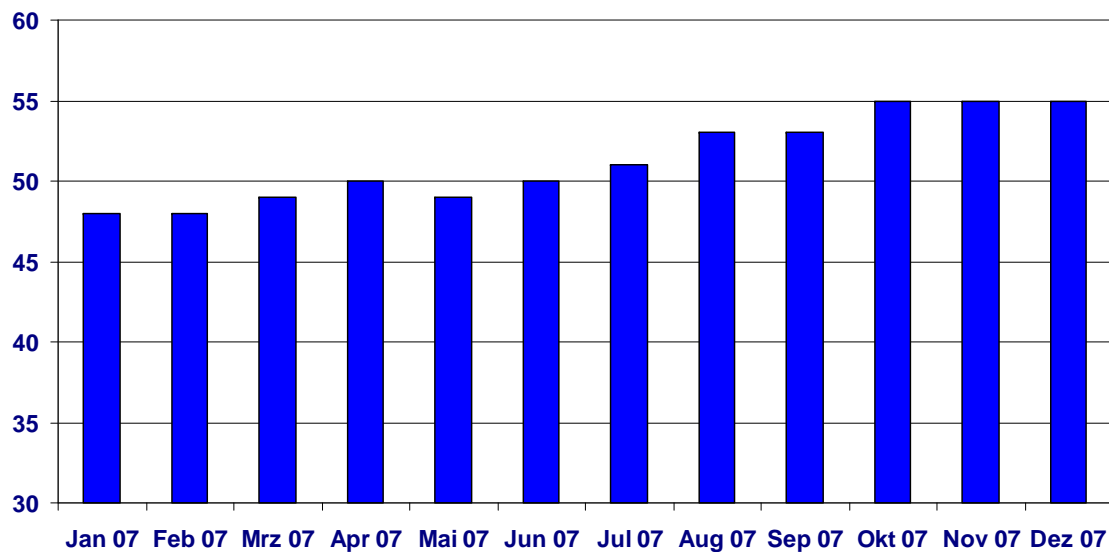
○ Status January 1, 2007

Key Talent Added in 2007

Company still lean



Full time employees



Preclinical development:	27
Clinical development:	14
Business development:	2
Marketing:	2
General and administration:	10
Academics:	44
Average age (years):	41

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Financial Information 2007

Key Financials

(IFRS, consolidated, in CHF thousands)

	2007	2006
Cash and cash equivalents	106,618	125,662
Net cash burn¹⁾	-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

1) without capital increases

- **Santhera delivered on all key milestones since IPO in November 2006**
- **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**
- **Strong financial position and efficient cash management**

Takeda Partnership

Commercialization of SNT-MC17 in FRDA and DMD

- **Exclusive commercialization licenses for EU and Switzerland, signed in July 2005 (FRDA) and in August 2007 (DMD), respectively**
- **Total of up to EUR 32m upfront and development milestones payable by Takeda (FRDA: EUR 12m; DMD: EUR 20m)**
- **Revenue potential: Takeda to pay 25% supply price on its net sales for product supply by Santhera to Takeda plus 5% royalties on net sales**
- **Full access to all preclinical and clinical data and right to cross reference for regulatory filings for both indications**

Financial Information 2007

Consolidated income statements

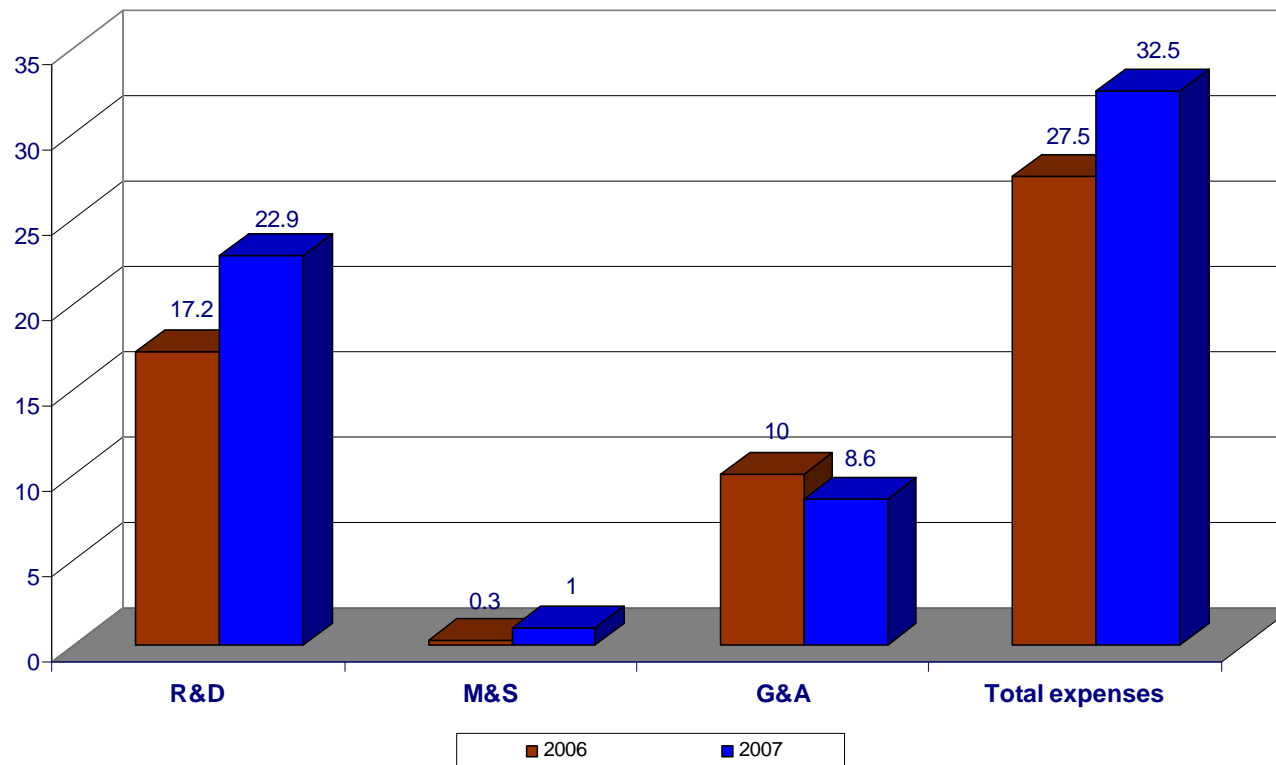
(IFRS, consolidated, in CHF thousands)

	2007	2006	
Revenue	9,226	781	
Other operating income	2,439	637	
R&D	-23,335	-17,985	+ 29.7%
incl. noncash-relevant share-based payments	-417	-808	
M&S	-1,170	-323	+ 262.2%
incl. noncash-relevant share-based payments	-167	-42	
G&A	-18,151	-11,729	+ 54.8%
incl. noncash-relevant share-based payments	-9,570	-1,716	
Other operating expenses	-136	-20	
Total expenses	-42,792	-30,057	+ 42.4%
incl. noncash-relevant share-based payments	-10,154	-2,566	
Operating result	-31,127	-28,639	+ 8.7%
Financial result	2,573	562	
Result before taxes	-28,554	-28,077	
Income taxes	683	-181	
Net loss	-27,871	-28,258	- 1.4%
Basic and diluted loss per share	-8.99	-14.44	

- Income from partners in the amount of CHF 11.7m
- Overall increase in expenses primarily due to noncash-relevant share based payments
- Slightly improved net loss

Financial Information 2007

Cash expenses 2007/2006 in CHF m (excluding noncash-relevant share-based payments)

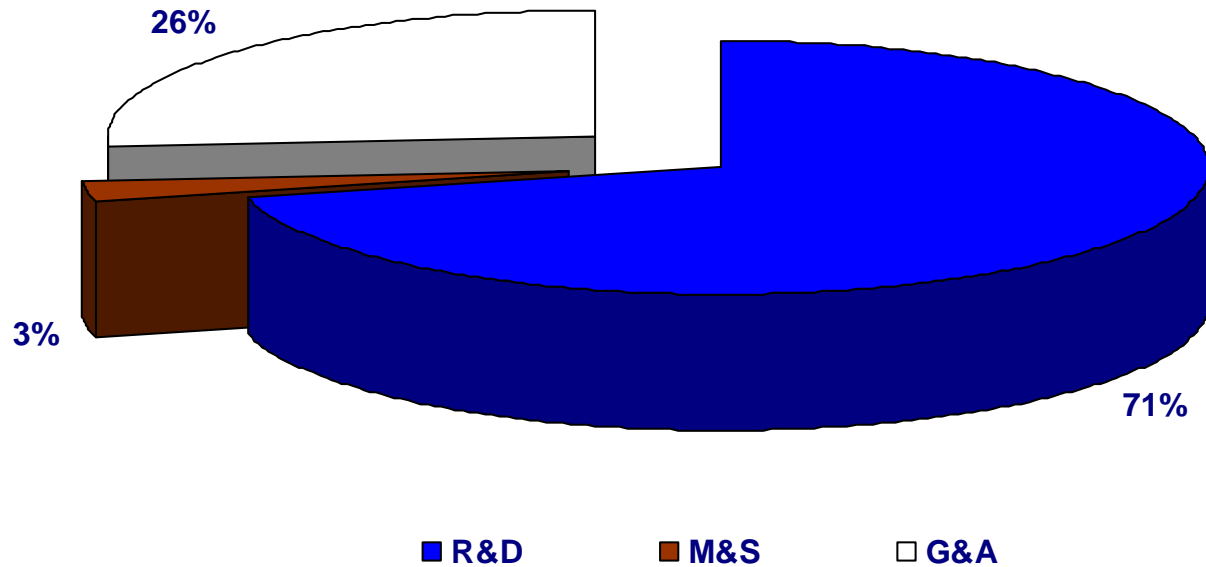


- Total expenses increased by 18%
- R&D expenses reached CHF 22.9m in 2007, an increase by 33% from CHF 17.2m in 2006
- Reduced G&A expenses from CHF 10.0m in 2006 to CHF 8.6m in 2007 (-14%)
- First time M&S expenses reported separately

Please note that these numbers do not take into consideration noncash-relevant share-based payments in accordance with IFRS 2, allocated to each expense category, but include depreciation/amortization (non material)

Financial Information 2007

Cash expenses allocation 2007 in CHF m
(excluding noncash-relevant share-based payments)



R&D expenses amount to 71% of total expenses in 2007 (excluding noncash-relevant share-based payments), 63% in 2006

Financial Information 2007

Condensed balance sheets

(IFRS, consolidated, in CHF thousands)

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 & liabilities	144,175	162,394

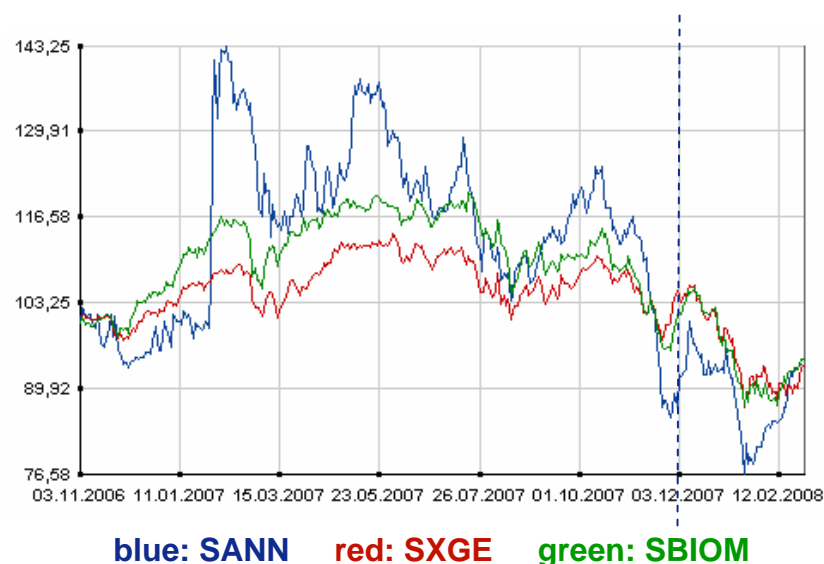
Cash per Share (in CHF)	34.20	40.55
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- IPO proceeds still available
- Remaining outstanding loans from tbg fully repaid in the amount of CHF 1.4m
- Fully equity financed

Financial information 2007

Share Capital and Share Price

December 31	2007	2006
Listed share capital	3,118,861	3,099,156
Cond. capital for stock options	351,971	371,676
Cond. capital for convertible rights	230,000	230,000
whereof allocated to Juvantia investors (warrants)	9,818	9,818
Authorized capital	561,092	561,092
whereof reserved for potential Juvantia acquisition	105,973	105,973
Share Price	85	91
High during year ¹⁾	135	94
Low during year ¹⁾	68	83.60
Market capitalization (CHF m)	265	282
Ø daily trading volume during year²⁾	3,323	7,890
Cash per share (in CHF)	34.20	40.55



1) 2006: from first trading day on November 3 until December 31

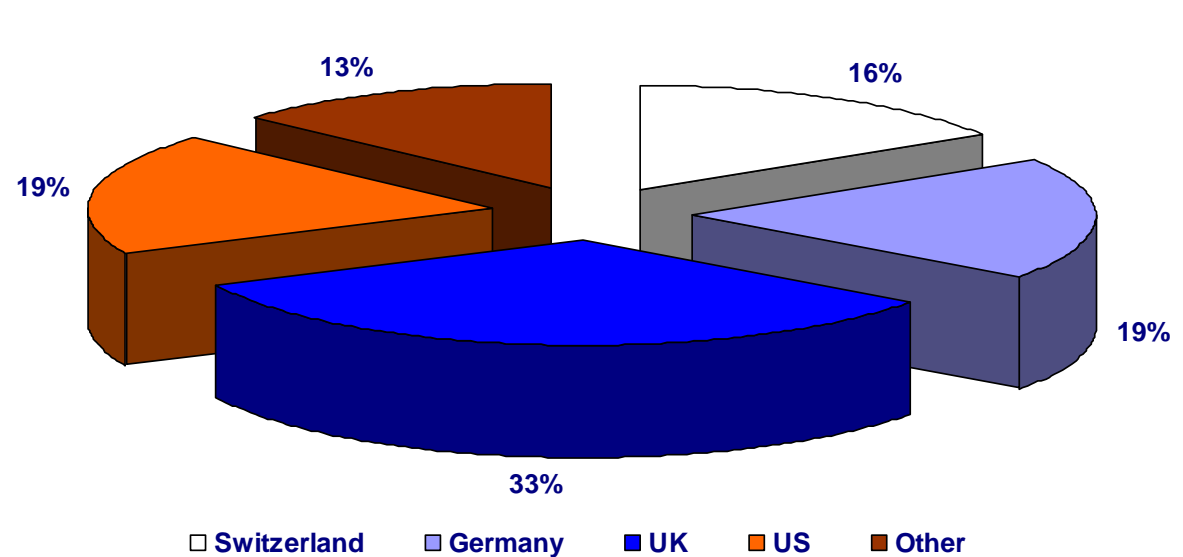
2) Source: www.swx.com

International, Institutional Shareholders



- 87.7% of shares registered; 846 shareholders in total
- 95.5% of shares held by institutional investors
- Largest shareholders (>3%)
 - NGN 12.2%
 - Merlin 7.7%
 - Oxford Bioscience 7.1%
 - 3i 6.9%
 - Schroders 5.6%
 - GIMV 5.0%
 - Varuma 4.7%
 - Cominvest 4.6%
 - Heidelberg Innovation 4.1%
 - Carnegie 3.8%
 - Neomed 3.2%

By domicile



controlling 64.9%

All figures as of December 31, 2007

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Expected Milestones and News Flow



1H 2008

- Annual Shareholders' Meeting (April 21)
- DMD data presentation at AAN, Chicago (April 12-19)
- FRDA symposia/ data presentation at ENS, Nice (June 10)
- Start extension study with SNT-MC17 in DMD

2H 2008

- Interim Report 1H 2008 (August 22)
- FRDA data presentation at EFNS, Madrid (August 23-26)
- Market approval for SNT-MC17 for FRDA in EU, Switzerland and Canada
- Start Phase III with SNT-MC17 in DMD
- Start Phase II/III program with SNT-317 in CMD
- Results of US Phase III with SNT-MC17 in FRDA

1H 2009

- Results of Phase IIb with JP-1730 in DPD
- NDA filing with SNT-MC17 for FRDA in the US



santhera
Pharmaceuticals

**Focused on Small-molecule
Therapeutics for Orphan
Neuromuscular Diseases**