santhera Pharmaceuticals

Dedicated to small-molecule therapeutics for neuromuscular diseases

Klaus Schollmeier, CEO

Basel, April 23, 2007

Santhera at a glance

- Focus on small-molecule therapies for orphan neuromuscular diseases
- Lead compound SNT-MC17 with well-established safety profile in filing for EU marketing approval and clinical development in three indications (FRDA, DMD and LHON)
- Second compound JP-1730 with proof of concept in Dyskinesia in Parkinson's Disease (DPD) about to enter confirmatory Phase IIb trial
- Proven success in establishing and leveraging collaborations to maximize commercial potential
- Experienced management team with proven track-record
- Approx. 70 employees in Liestal

Today‘s agenda

1. Business overview / Equity story
2. Product pipeline
3. Key results 2006
4. News flow & concluding remarks

Focus on neuromuscular diseases offers interesting business opportunity

High unmet medical need

- Chronic diseases often leading to life threatening conditions
- Few if any approved therapies

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Genetic disorders

- Well-understood disease mechanisms
- New treatment opportunities offered by scientific advance

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Costly disease progression

- Significant patient care required
- High cost and social burden on family

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Defined patient group and market

- Orphan or ultra orphan diseases
- Well-organized medical communities and patient advocacy groups

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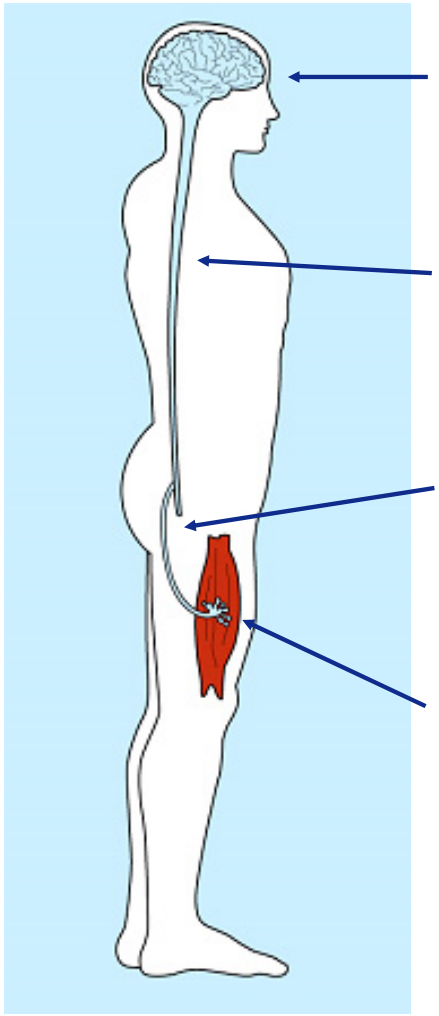
Strong business opportunity

- **High unmet medical need**
- **Scientific progress offers treatment opportunities**
- **Specialized niche marketing opportunities**
- **Market exclusivity through orphan drug protection**

Over 200 NMDs and movement disorders



Movement disorders

- Dyskinesia in Parkinson's Disease (DPD)
- Huntington's Disease
- Spinocerebellar ataxias

Sensory-motor deficits

- Friedreich's Ataxia (FRDA)
- 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 (DMD)
- Cachexia (e.g. Cancer cachexia)
- Congenital muscular dystrophies
- 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

Clear business strategy

Research & Development

- Grow in key area of expertise through own research as well as in-licensing and co-operations
- Leverage compounds into several indications
- Out-license non-core development programs

Marketing & Sales

- Launch products with own specialty marketing and sales team in the US
- Leverage sales organization with future products
- Build marketing partnerships in other territories

Become recognized by physicians, patients and payers as the premier company for introducing successful new therapies in severe NMDs

Significant market potential of current clinical portfolio

Friedreich's Ataxia

- ~ 20,000 patients in Europe and North America in total
- Affects primarily Caucasians
- Life-long treatment required
- ~ EUR 300 million market

Duchenne Muscular Dystrophy

- ~ 30,000 patients worldwide
- Affects men, diagnosed around 3 to 5 years of age
- Life-long treatment required
- ~ EUR 400 million market

Combined market opportunity of ~ EUR 1.6 billion

Leber's Hereditary Optic Neuropathy

- ~ 35,000 patients worldwide
- Affects mainly healthy adult males
- Chronic treatment expected
- ~ EUR 400 million market

Dyskinesia in Parkinson's Disease

- ~ 200,000 patients worldwide
- Affects Parkinson's patients after long-term treatment with levodopa
- Chronic treatment required
- ~ EUR 500 million market

Friedreich's Ataxia (FRDA)

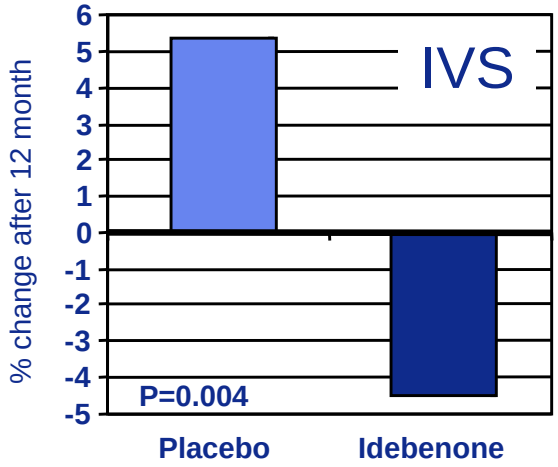
- Severe genetic disorder:
  - Degeneration of nerve and muscle tissue
  - Loss of muscle control
  - Impaired movements
  - Muscle wasting
  - Thickening of heart walls (cardiomyopathy)
- Caused by a reduced level of *frataxin*, a protein needed in mitochondria to facilitate energy production
- Average onset between 5 and 15 years; average life expectancy between 35 and 50 years

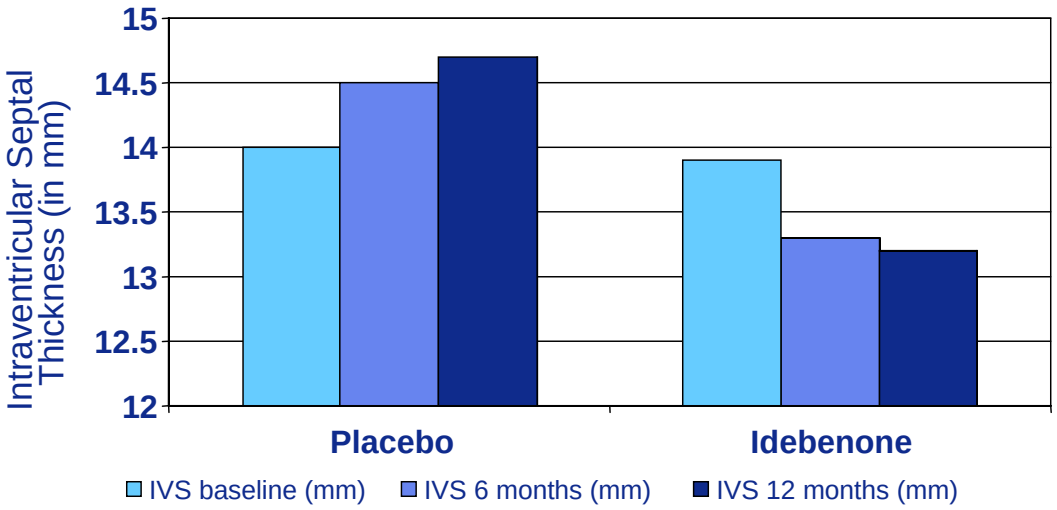
- Affects both males and females, predominantly Caucasian population
- ~ 20,000 patients in Europe and North America in total
- No approved pharmacological treatment available
- Chronic disorder, requires life-long treatment

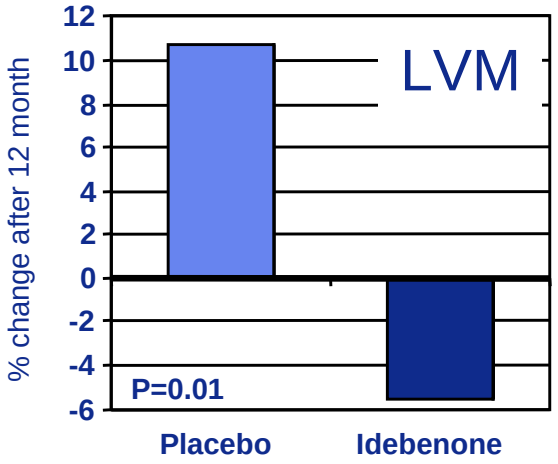
Idebenone improves cardiomyopathy in FRDA patients (Mariotti et al)

- Double-blind, placebo-controlled study with 14 + 14 patients
- Idebenone dose: 5 mg/kg/d (given in three doses)
- Duration of treatment: 12 months
- Endpoint: changes in IVS, LVM by echocardiography

IVS: Intraventricular septal thickness; LVM: left ventricle mass

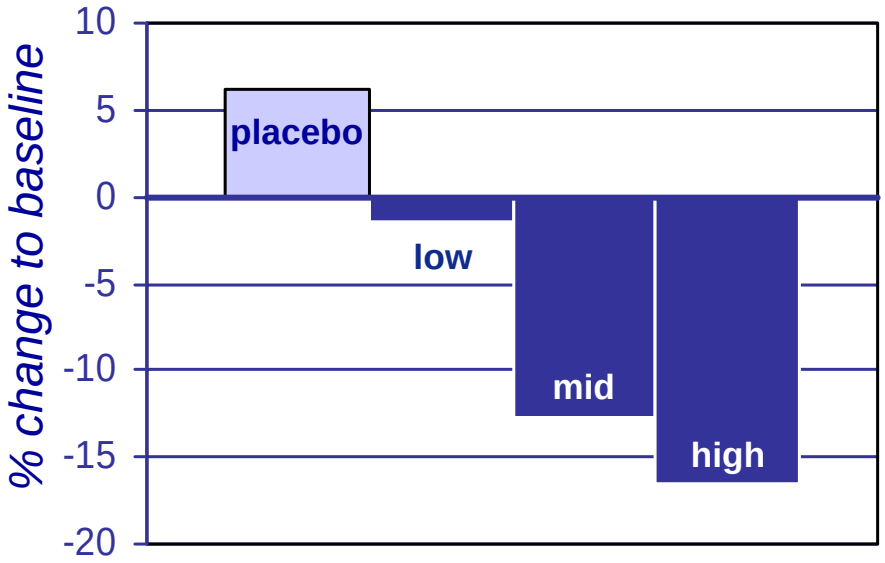






SNT-MC17 improves neurological functions in FRDA patients (NIH/Santhera)

- Patients with baseline ICARS >10 and <54
- p=0.002 for hypothesis that there is a dose-dependent effect (Jonckheere-test)

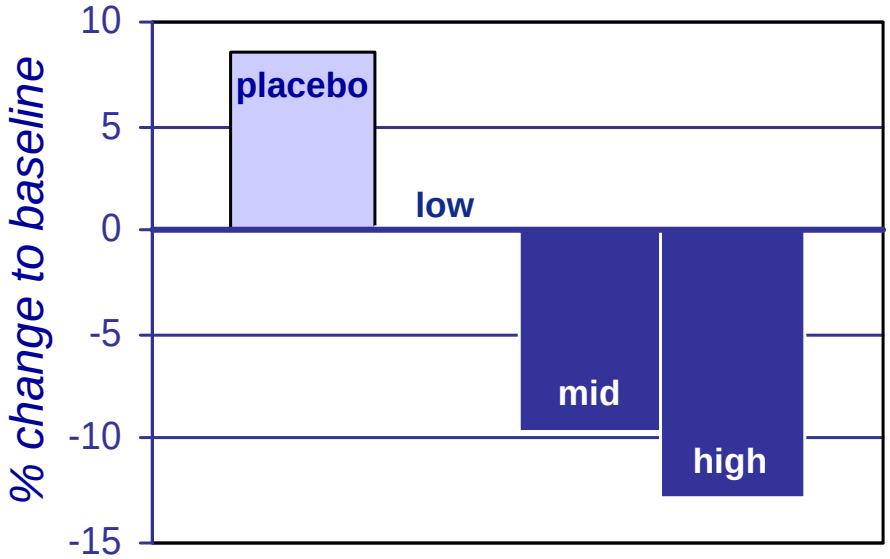


ICARS	placebo	low	mid	high
baseline[1]	32.12	34.66	33.85	35.04
change[1]	1.96	- 0.48	- 4.30	- 5.83
SEM[1]	1.68	2.01	1.49	1.60
P-value[2]		0.369	0.009	0.002
N	8	6	10	9

1: least square means; 2: pairwise comparison to placebo

SNT-MC17 improves quality of life in FRDA patients (NIH/Santhera)

- Patients with baseline ICARS >10 and <54
- ANOVA model with effects for treatment and allele category



ICARS	placebo	low	mid	high
baseline[1]	11.74	12.52	13.00	11.27
change[1]	0.81	0.02	- 1.25	- 1.45
SEM[1]	0.87	1.03	0.77	0.82
P-value[2]		0.570	0.086	0.066
N	8	6	10	9

1: least square means; 2: pairwise comparison to placebo

Orphan drug legislations in EU and US: EMEA strengthens support for orphan products

Orphan drug protection by FDA or EMEA

Marketing exclusivity after marketing approval; designed to encourage drug development for treatment of rare diseases or conditions

- affecting < 200,000 (US) or < 5 in 10,000 individuals (EU)
- 7 and 10 years in US and EU, respectively, for a compound in a specific indication

EMEA Guideline on clinical trials in small populations

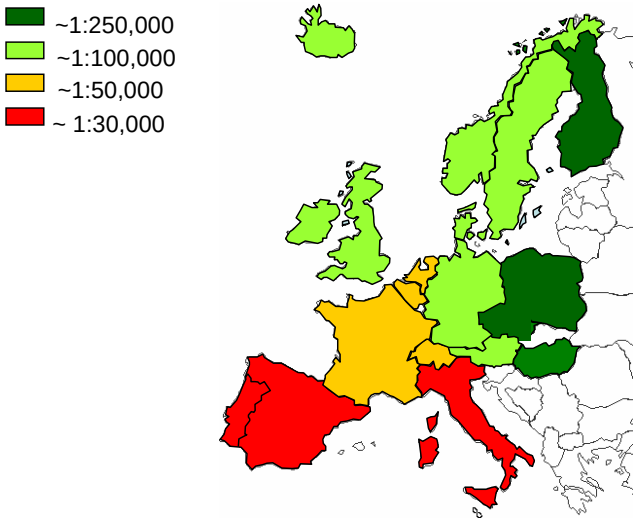
Facilitates approval of product candidates developed to treat diseases affecting only a small number of patients

- a single clinical trial with limited data can justify for market approval
- p-values of 0.05 not necessarily required due to small population
- pre-selection of primary endpoint not necessarily required if clinical studies can be judged on overall effect

SNT-MC17 in FRDA – accelerated clinical and regulatory status

- Consistent reporting of reduced cardiac hypertrophy
- Latest clinical data (NIH/Santhera) show improvement of neurological parameters
- Conclusion for going forward with new dataset from NIH/Santhera study:
  - in Europe
    - MAA filing in summer 2007 with expected market launch in H2 2008
    - Amendment of on-going Phase III trial to collect additional safety and efficacy data in wider population
  - in US
    - One additional pivotal Phase III trial needed before NDA filing; shorter study duration and fewer patients expected than originally planned
    - New protocol under open IND submitted to FDA for Special Protocol Assessment (SPA) review

Compassionate use and temporary registration of idebenone in Europe

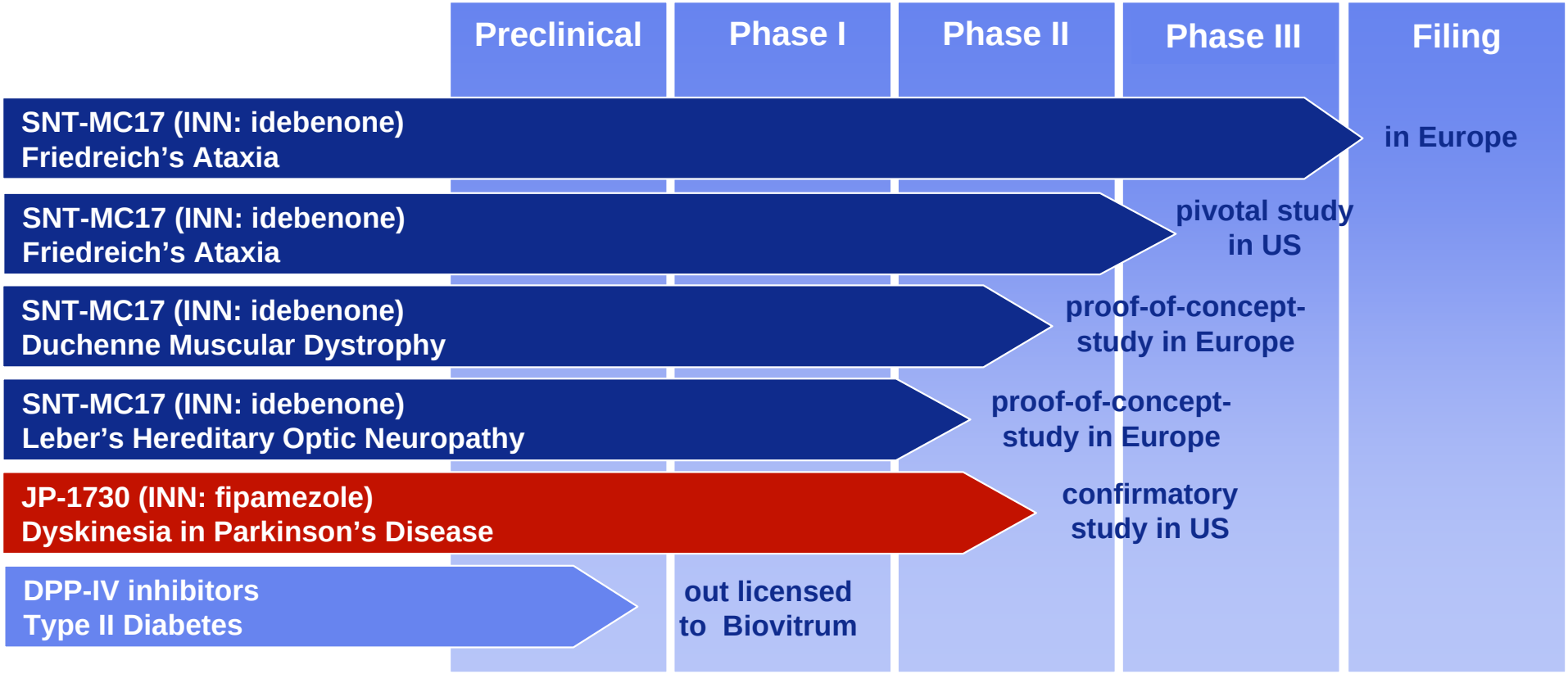


- In Switzerland: Provisional registration for treatment of cardiomyopathy in FRDA patients
- In France: Compassionate use in FRDA
- In Italy: Compassionate use in FRDA
- Other European countries: Treatment center based compassionate use programs

- Originator Takeda is exclusive European marketing partner for SNT-MC17 in FRDA

Takeda has achieved preliminary registration and reimbursement status for FRDA in several countries = existing business case

Today's portfolio



Strong cash position and tightly managed burn rate

- Financing since 2004:
  - CHF 86.7 million raised in three private financing rounds
  - CHF 101.8 million in IPO (greenshoe exercised)

- Cash and cash equivalents as of December 31, 2006:
  CHF 125.7 million

- Gross cash burn from operating and investing activities[1]:
  CHF 25.9 million in 2006 and CHF 22.5 million in 2005

- Group sales:
  CHF 0.8 million in 2006 and CHF 15.1 million in 2005, primarily through Takeda and Biovitrum partnerships (upfront payments and research funding)

[1] Net cash flow from operating activities plus cash flow from investing activities, net of gross profit

Key financial information 2006

Income statement

(IFRS, consolidated, in CHF thousands)

	2006	2005
Gross profit	781	13,756
R&D expenses	–17,985	–14,542
G&A expenses	–12,052	–6,012
Other expenses	617	–4,719
Operating result (EBIT)	–28,639	–11,517
Financial result	562	–867
Result before taxes	– 28,077	–12,384
Income taxes	–181	748
Net loss	**–28,259**	–11,636

Balance sheet

(IFRS, consolidated, in CHF thousands)

	Dec 31, 2006	Dec 31, 2005
Cash and cash equivalents	125,662	31,268
Noncurrent assets	34,260	32,993
Other current assets	2,472	14,451
Total assets	**162,394**	78,712
Equity	152,048	66,147
Noncurrent liabilities	1,758	4,773
Current liabilities	8,588	7,792
Total equity & liabilities	**162,394**	78,712

Changes to 2005 figures mainly due to changes in reporting currency from EUR to CHF

Share price development

- Performance*: 17.9% since IPO
- High/low: CHF 135/83.60
- Daily average volume: 7,900 shares
- Market cap*: CHF 322 million
- Member of SXI Life Sciences and SXI Bio+Medtech

Analyst coverage:

- Deutsche Bank, Brian White
- Piper Jaffray, Sally Bennett
- WestLB, Irina Stratan
- ZKB, Hernani L. de Faria

* as of April 20, 2007

Shareholder structure

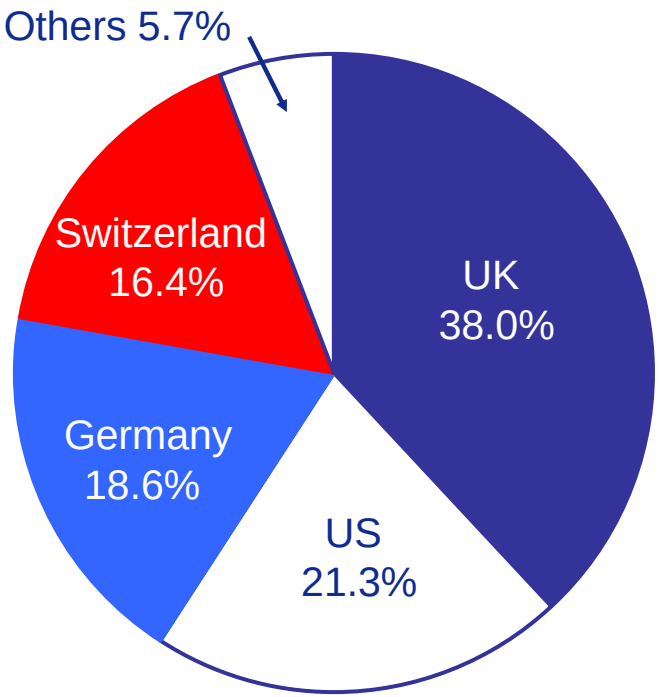
- 81.2% of shares registered*; total of 719 investors*
- 96.1% of shares held by institutional investors*
- Pre-IPO investors (61.7%) locked-up until May 2007 (hard lock-up) and November 2007 (soft lock-up) respectively;
- Free float: 38.3%

Largest shareholders*

NGN	12.2%
Merlin	7.7%
Oxford Bioscience	7.1%
3i	6.9%
Cominvest [1]	6.0%
Schroders [2]	5.1%
GIMV	5.0%

* as of February 27, 2007
[1] disclosed November 9, 2006
[2] disclosed January 25, 2007

Shareholders by Domicile



Expected milestones and news flow

H1 2007

- SPA meeting with FDA on US development of SNT-MC17 in FRDA

H2 2007

- MAA filing of SNT-MC17 for FRDA in Europe
- Start of US Phase III trial of SNT-MC17 in FRDA
- Results of Phase IIa trial of SNT-MC17 in DMD
- Start of Phase IIb trial of JP-1730 in DPD
- Further partnering with SNT-MC17

2008

- Results of Phase IIa trial of SNT-MC17 in LHON
- Results of Phase IIb trial of JP-1730 in DPD
- Results of US Phase III trial of SNT-MC17 in FRDA
- Market approval for SNT-MC17 for FRDA in Europe
- Biovitrum to start Phase II trial of DPP-IV in T2 diabetes