



**Dedicated to  
small-molecule therapeutics  
for neuromuscular diseases**

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# The vision – in 2000



**Discover and develop pharmacological treatments for Duchenne Muscular Dystrophy and other neuromuscular diseases.**



# A brief look back



- **Jan. 2000: MyoContract AG as first start-up life sciences company of Biozentrum**
  - Laboratory and office space available
  - Loan from Novartis Venture Fund
- **2002: transfer from Biozentrum to Liestal**
  - Implementation of own in-house chemistry facility
  - 24 FTE
- **Until 2004: CHF ~10m raised**
  - Ready to start clinical development of SNT-MC17/idebenone
- **2004: Business combination with Graffinity to form Santhera**
  - Restructuring of companies
  - Initiation of clinical development programs
- **November 2006: IPO and listing on Swiss Stock Exchange**
  - Raised CHF 88.5m; market capitalization CHF 266m
  - Currently approx. 60 employees

# Focus on neuromuscular diseases offers interesting business opportunity



**High unmet medical need**

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**Seriousness of disease**

+

**Costly disease progression**

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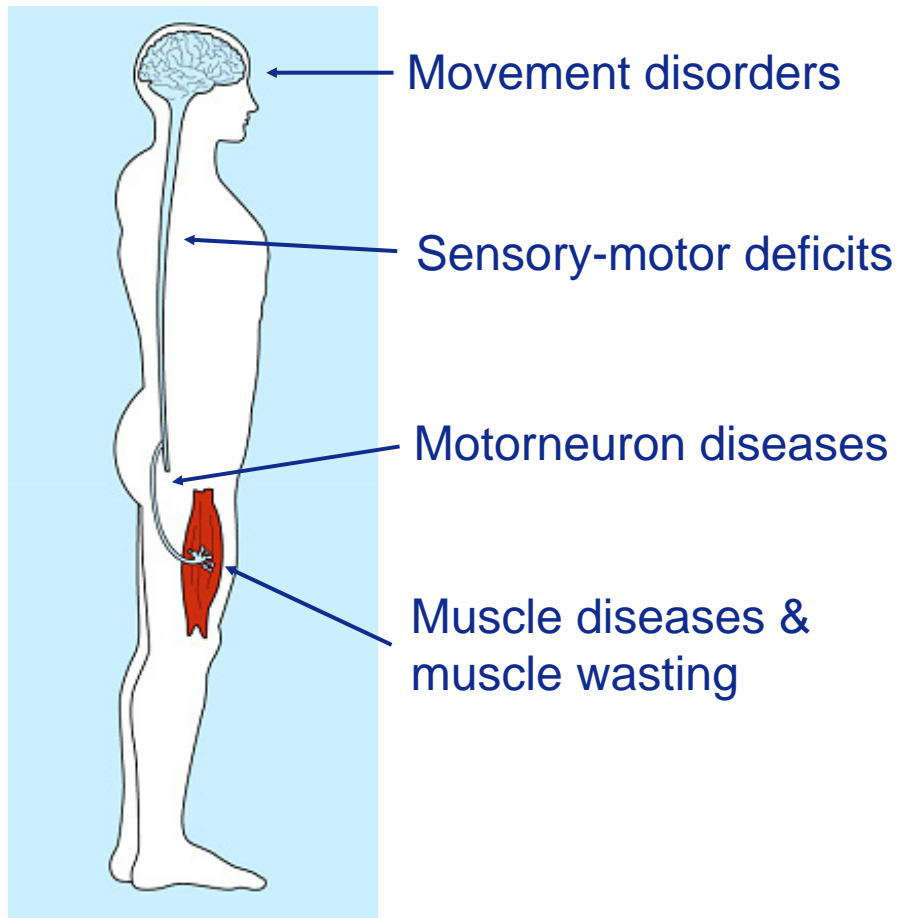
**Defined patient group and market**

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**Strong business opportunity**

- Orphan or “ultra orphan” diseases
- Few if any approved therapies
- Loss of muscle tissue leads to impaired movement control, loss of mobility and respiratory capacity and heart malfunctions
- Often leads to life threatening conditions
- Chronic diseases with severe impact on quality of life
- Significant patient care required
- High cost and social burden on family carers
- Genetically determined diseases
- Limited medical options
- **Unmet need, only few therapies available**
- **Opportunity for specialized marketing in niche markets**
- **Orphan drug protection**

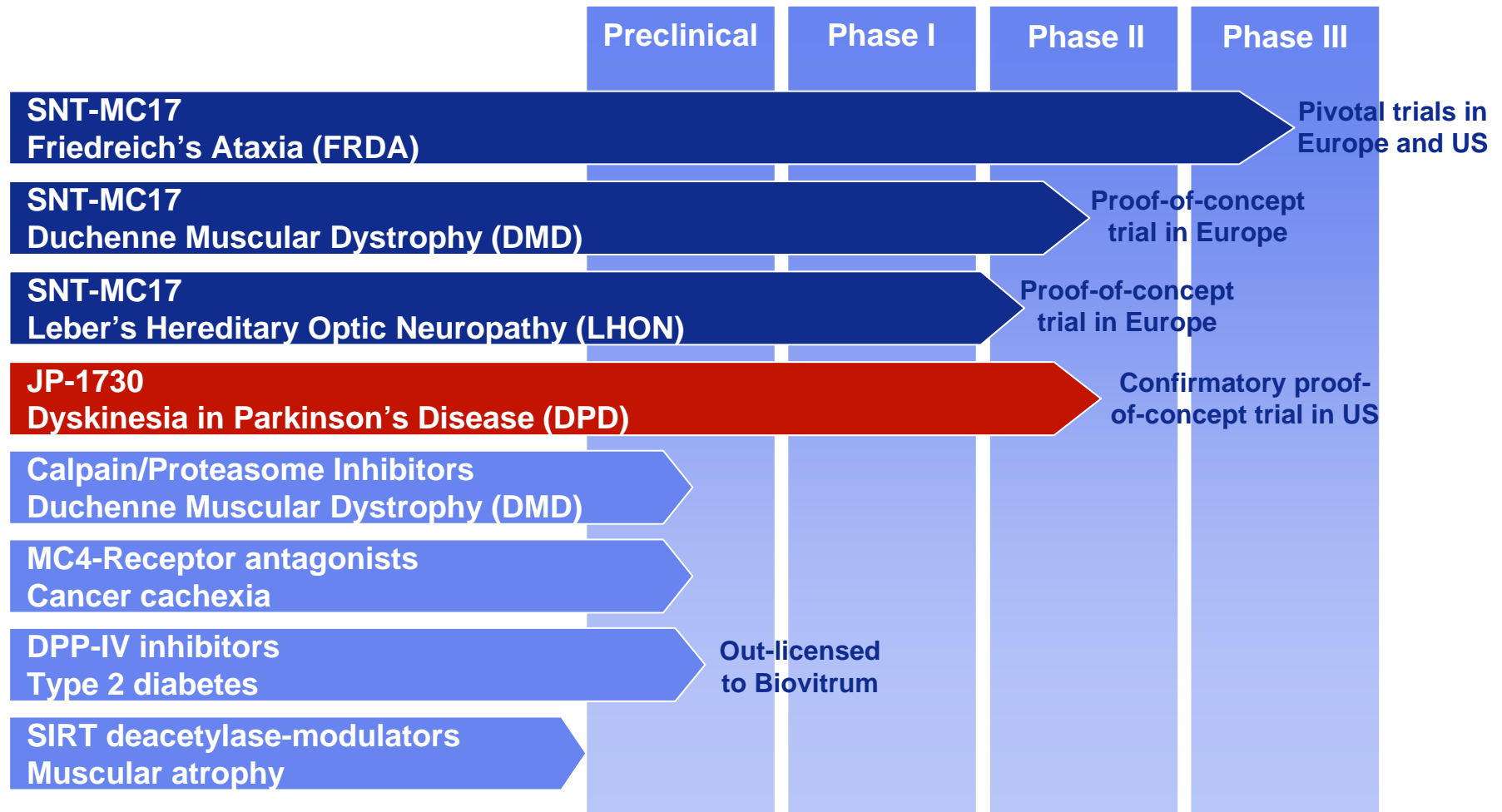
# Over 200 NMDs and movement disorders



- **Dyskinesia in Parkinson's Disease (DPD)**
- Huntington's Disease
- Spinocerebellar ataxias
- **Friedreich's Ataxia (FRDA)**
- Spinal cord injury
- Charcot-Marie-Tooth neuropathies
- Amyotrophic lateral sclerosis
- Guillain-Barre Syndrome
- Peripheral nerve injuries
- **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

# Current product development portfolio



# Positive NIH data with SNT-MC17/ idebenone



## Press Release

November 13, 2006

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### NIH and Santhera Announce Positive Results of Study with SNT-MC17/idebenone in Friedreich's Ataxia (FRDA)

Data Presented at the 3rd International Scientific Friedreich's Ataxia Conference

## Santhera's vision



**Our goal is to become a global market leader in the development and commercialization of therapeutics for severe neuromuscular diseases that frequently qualify for orphan drug status.**

# Lessons learned



- **Per ardua ad astra, but remember that failures are part of the game!**
- **Learn from past mistakes, preferably from those of others.**
- **Build on your area of expertise and get professional support where you lack knowledge.**
- **Networking is not for free drinks but the starting point of your business case.**
- **Persistence is OK, but be open for alternatives if changes arise.**