



santhera
Pharmaceuticals

Building the Company on Two Pillars for Growth

Company Presentation
July 2010

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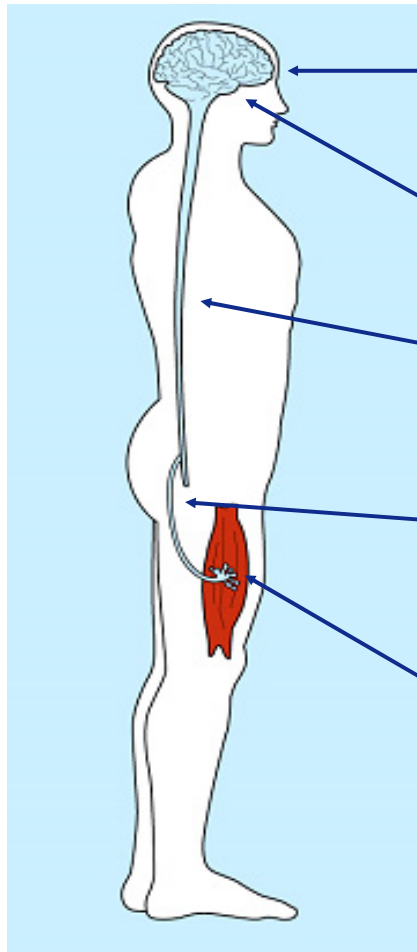
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An Emerging Product Company

- **Focus on innovative therapies for orphan neuromuscular diseases**
- **Robust clinical pipeline of 3 compounds in 7 indications**
- **Lead compound Catena®**
 - Successfully launched in Canada for treatment of Friedreich's Ataxia
 - Positive results from RHODOS study in Leber's Hereditary Optic Neuropathy
- **Catena® in clinical development in 3 additional indications in Phase II and III**
 - Data of MELTIMI Phase II in MELAS syndrome in 2H 2010
 - Interim analysis of DELOS Phase III in Duchenne Muscular Dystrophy in 2H 2010
- **Second franchise fipamezole in Dyskinesia in Parkinson's Disease:**
 - Positive FJORD Phase IIb data reported in June 2009; Licensed to Biovail in the US and Canada
 - Start of Phase III (Biovail) in 2011
 - Partnering of ex-North American rights expected by 2H 2010
- **Listed on SIX Swiss Exchange (SIX: SANN)**
- **Headquartered in Liestal/Basel (Switzerland), approx. 55 employees**



Over 200 Neuromuscular Diseases and Movement Disorders Known Today



Movement Disorders

Mitochondrial Diseases

Sensory-motor Deficits

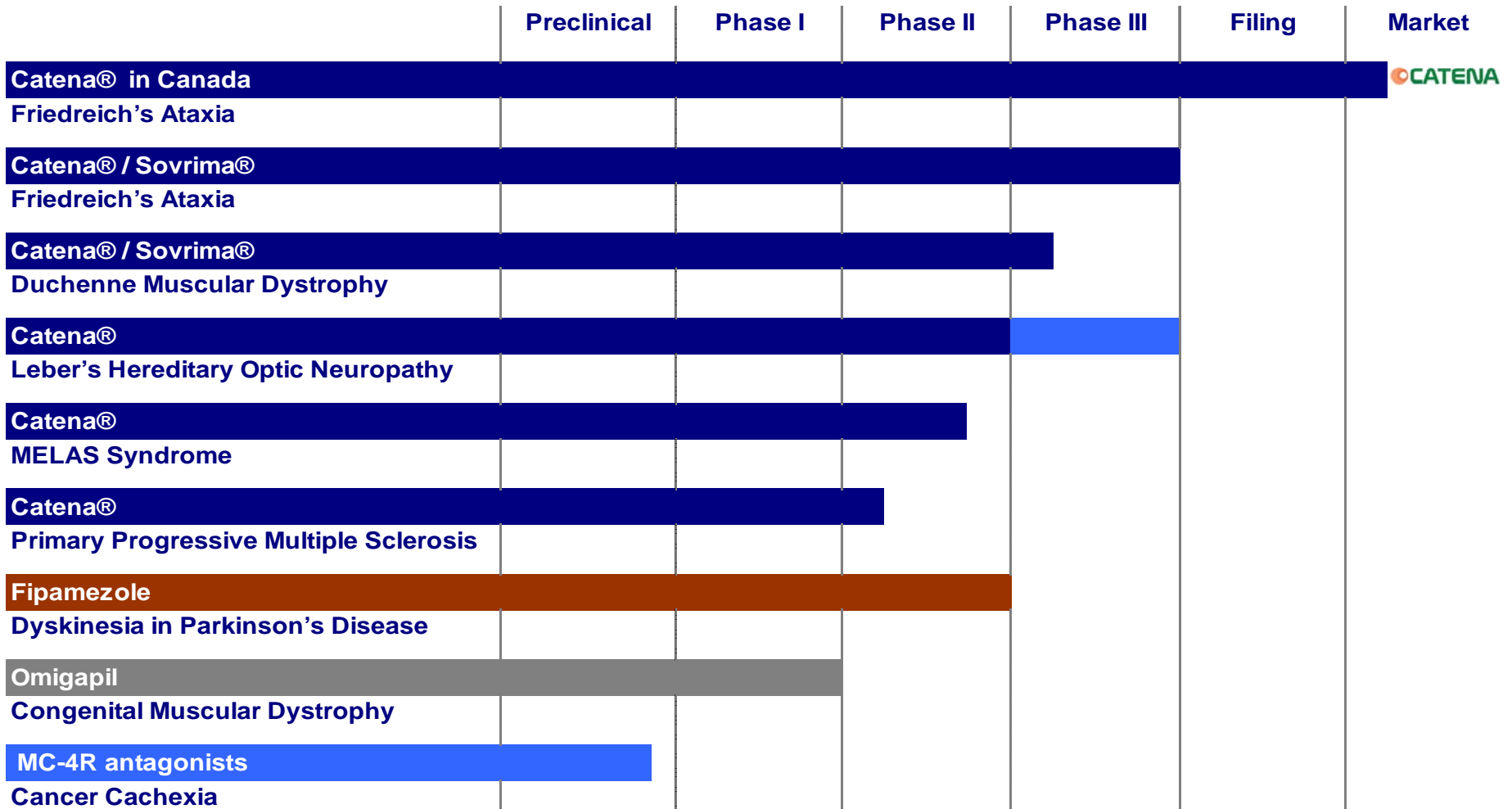
Motorneuron Diseases

Muscle Diseases & Muscle Wasting

- **Dyskinesia in Parkinson's Disease**
- **Primary Progressive Multiple Sclerosis**
- **Huntington's Disease**
- **Leber's Hereditary Optic Neuropathy**
- **MELAS Syndrome**
- **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**
- **Myasthenia Gravis**
- **Spinal Muscular Atrophies**

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

Late-stage Product Portfolio Focused on Orphan Neuromuscular Indications



Strategic Focus and Business Drivers



▪ Innovative therapies in orphan indications

- Market exclusivity through orphan drug protection
- High margin opportunities
- Low competition
- Well-organized medical communities and patient advocacy groups

▪ Specific value drivers of Santhera

- Focus on neuromuscular diseases, motor dysfunctions and muscle wasting
- High unmet medical needs, life-threatening diseases requiring chronic or life-long treatment
- Focus marketing on North American markets, partner in other territories
- Partner in larger indications worldwide
- Portfolio and growth management tools along the value chain: leveraging into several indications, partnering, re-profiling, own development, M&A

Financial Information 2009

Key Financials

(IFRS, consolidated, in CHF thousands)

	2009	2008
Cash and cash equivalents	53,320	75,006
Net decrease in cash and cash equivalents	-21,686	-31,612
Total revenues	22,339	48
Total operating expenses	-48,544	-45,642
- whereof R&D	-31,536	-31,467
- whereof noncash-relevant share-based payments	-1,830	-1,680
Net loss	-25,923	-44,656

- **Cash flow neutral second half 2009**
- **Strong partnering income, product sales with Catena® in Canada and from NPP outside North America**
- **Cost saving measures and restructuring**
- **Average monthly net decrease in cash and cash equivalents in 2009: CHF 1.8m, below earlier guidance (2008: CHF 2.6m)**

Financial Information 2009



Consolidated Income Statements

(IFRS, condensed, in CHF thousands)

	2009	2008	
Total revenues	22,339	48	
Gross Profit	22,095	25	
R&D	-31,536	-31,467	+/-0%
whereof noncash-relevant share-based payments	-600	-401	
M&S	-3,752	-3,484	+8%
whereof noncash-relevant share-based payments	-350	-261	
G&A	-13,206	-10,624	+24%
whereof noncash-relevant share-based payments	-880	-1,018	
Other operating expenses	-50	-67	
Total operating expenses	-48,544	-45,642	+6%
whereof noncash-relevant share-based payments	-1,830	-1,680	
Operating result	-25,895	-45,591	-43%
Financial result	39	814	
Result before taxes	-25,856	-44,777	
Income taxes	-67	121	
Net loss	-25,923	-44,656	-42%
Basic and diluted loss per share	-7,31	-14.11	

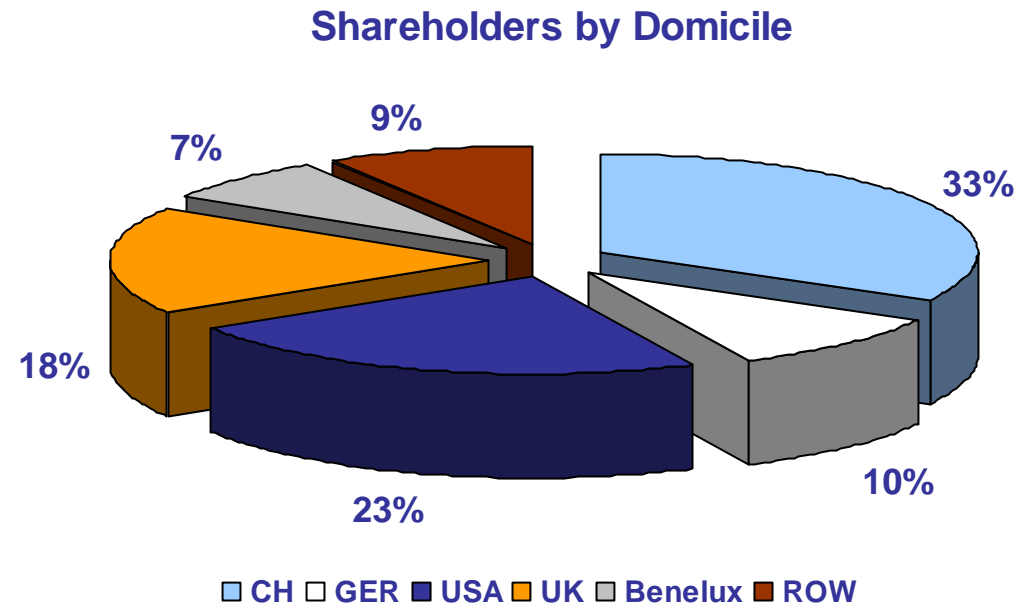
- Partnering Income: CHF 20.7m
- Net product sales: CHF 1.6m

- R&D: unchanged, late-stage clinical trials and extension studies ongoing through 2009; restructuring effects from 4Q 2009
- M&S: slight increase in line with product uptake in Canada
- G&A: increased due to Business development/partnering activities and restructuring expenses
- Net loss: substantial reduction compared to 2008

Our Shareholders

- >82% of shares registered; ~1,400 shareholders in total
- >95% of shares held by institutional investors
- Largest shareholders (>3%) control 49.8%

– Ares Life Sciences	14.9%
– NGN Capital	10.5%
– Excalibur / Merlin	6.6%
– Oxford Bioscience	6.0%
– GIMV	4.3%
– Varuma	4.0%
– Heidelberg Innovation	3.5%



All figures as of March 2010

Catena[®]/Sovrima[®]

Friedreich's Ataxia (FA): Lead Indication for Catena[®]/Sovrima[®]

- **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 to 15 years, reduced life expectancy**
- **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**



Catena[®]/Sovrima[®] in FA: Generating Sales in Canada

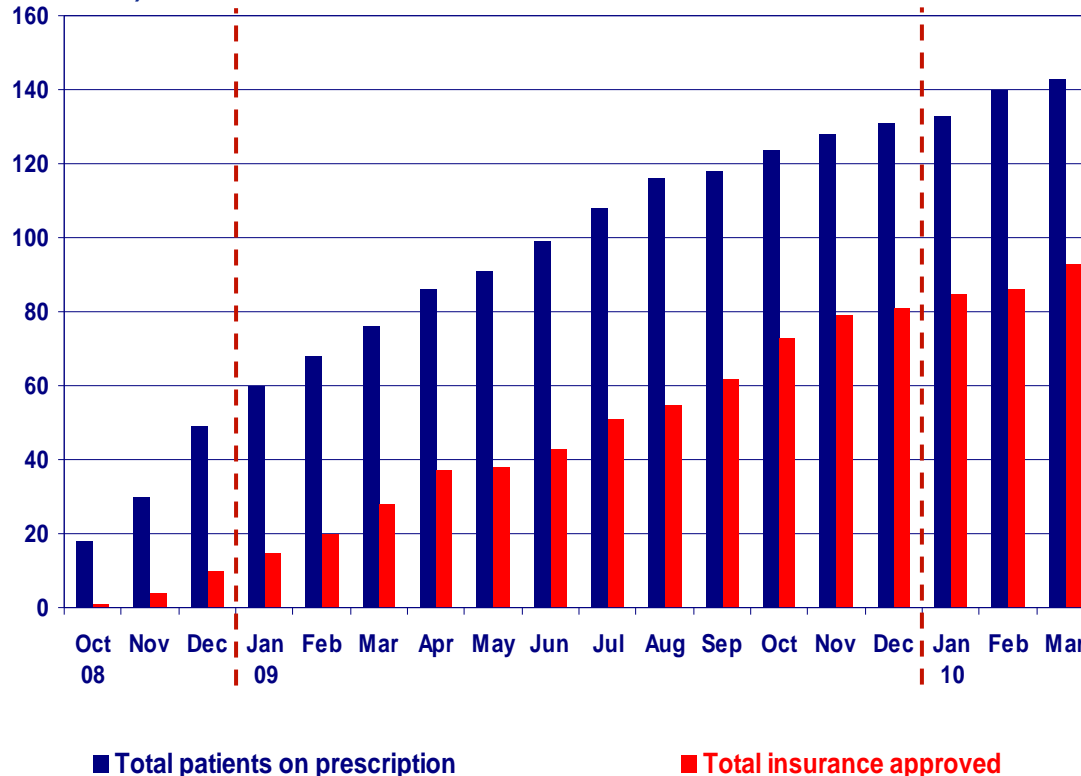
- Catena[®] approved by Health Canada for treatment of symptoms of Friedreich's Ataxia
- Supporting evidence for business model and larger than expected market size:
 - High medical need translated into fast uptake
 - Premium-priced product
 - Reimbursed by private & first public payers
- North American rights with Santhera; European commercialization rights partnered to Takeda
- Named Patient Program started in Europe in September 2009 in collaboration with marketing partner Takeda
- Data of MICONOS Phase III delivered conflicting picture



Catena® in Canada: Driven by Strong Demand from Medical Community



Patient numbers
(cumulative)

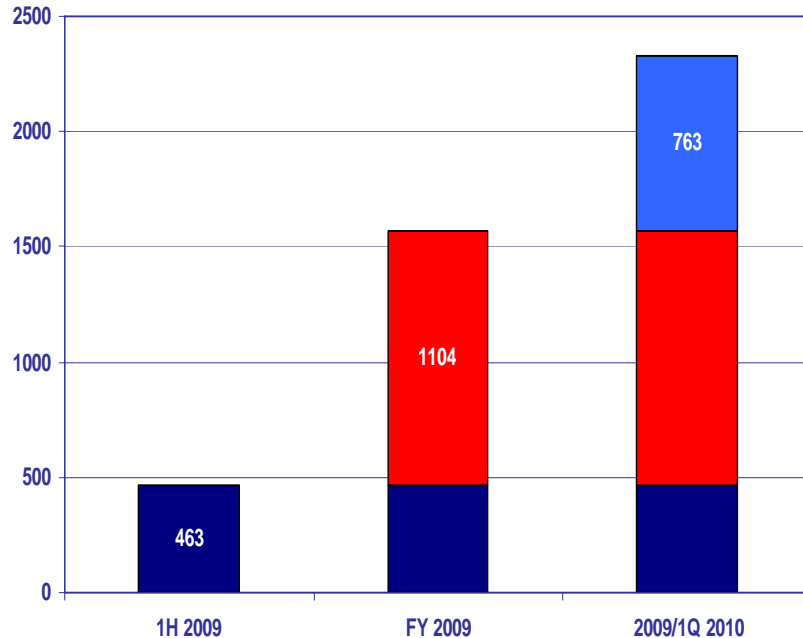


- ~50% market penetration (expected patient population of 300 individuals)
- >60% of prescriptions have reimbursement secured
- Continuous growth of prescriptions, reimbursements and shipments

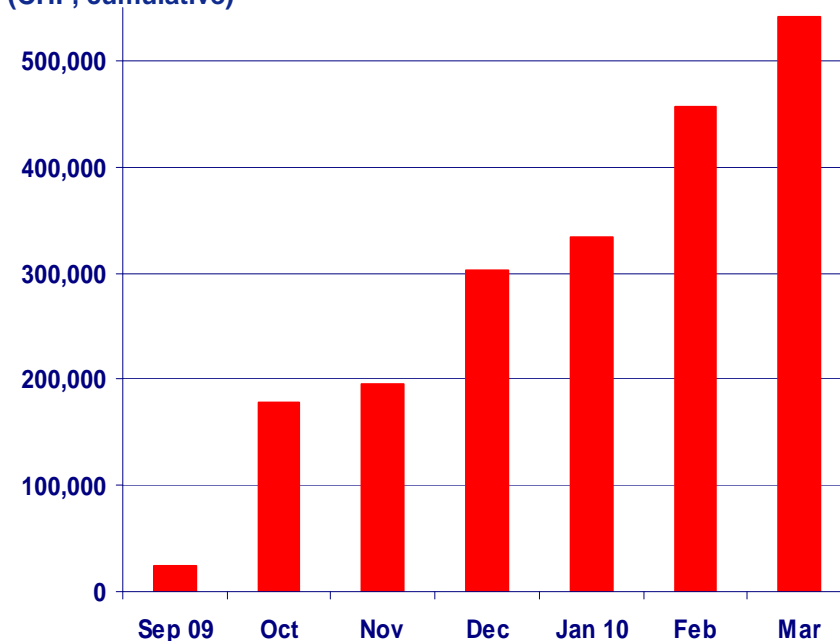
Strong Demand from Medical Community Drives Catena[®] Sales in Canada and NPP Europe



Net sales in TCHF
(cumulative)



Gross sales
Takeda & Santhera
(CHF, cumulative)



- FY2009 net sales: CHF 1.6m
- 1Q 2010 net sales: CHF 0.8m
- Average ex-factory price: CAD ~45k

- NPP Agreement signed with Takeda in August 2009
- Orders from Germany, Austria, Sweden, Greece
- Sales split 75:25 Takeda / Santhera

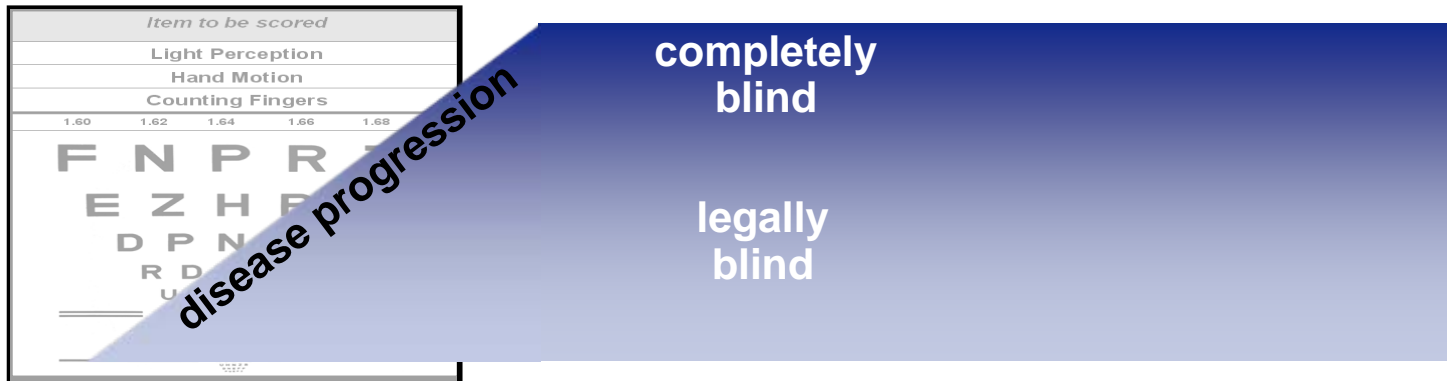
Leveraging Catena[®] into Leber's Hereditary Optic Neuropathy (LHON)

- **Genetic disease caused by a degeneration of nerve cells in retina**
 - Rapid loss of central vision, subsequent blindness
 - First symptoms in one eye, second eye involvement within a few months
 - Legal blindness in both eyes usually within 12 months
- **Onset 20 to 30 years**
- **Predominantly young otherwise healthy males in all ethnic groups**
- **~20,000 patients in Europe and US**
- **CHF 500m market in EU & US (Company estimate)**
- **No cure or therapeutic treatment available**



Catena[®] in Leber's Hereditary Optic Neuropathy: A Progressive Form of Vision Loss

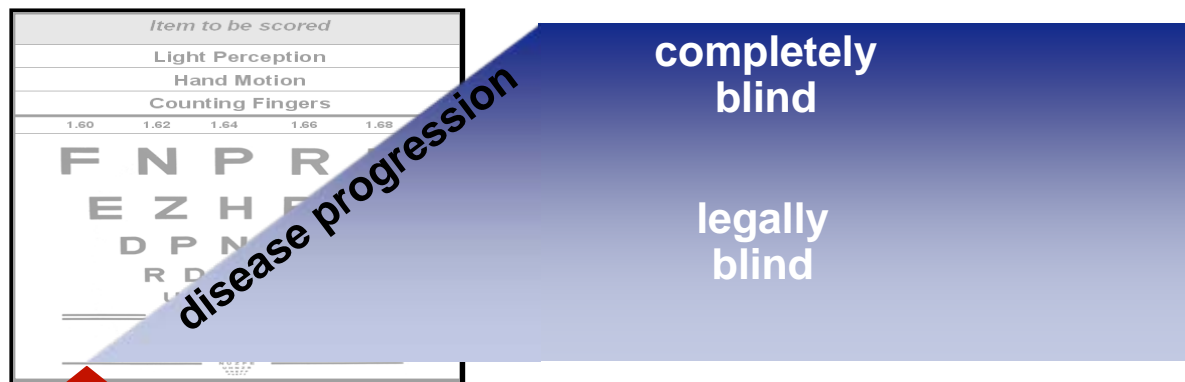
First eye



Time (in months)

0 3 6 9 12 15 18

Second eye

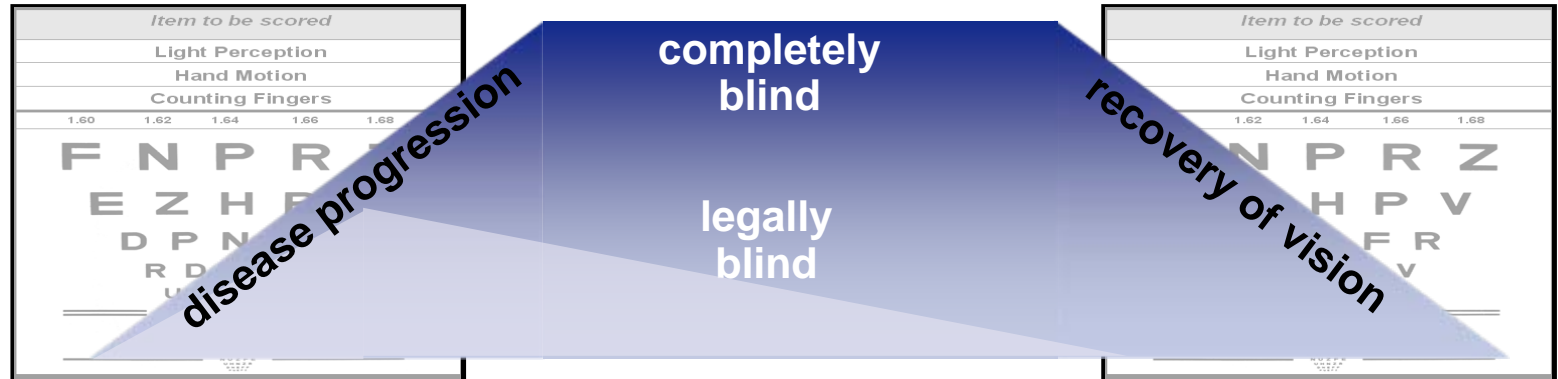


↑ Beginning of symptoms

spontaneous remissions exist but are rare

Therapeutic Goals

First eye



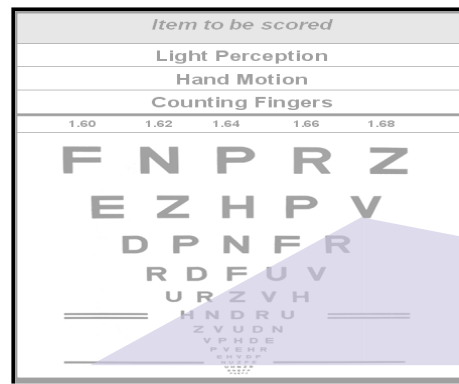
↑ Early Intervention

↑ Late Intervention

Time (in months)



Second eye



↑ Early Intervention

RHODOS Study: Effect of Catena[®] on Visual Acuity Endpoint

Mean difference and p-values comparing Catena[®] with placebo for change in visual acuity from baseline to week 24

Population	Endpoint		
	Best recovery (primary)	Best acuity (secondary)	Acuity in both eyes (secondary)
N Total (N active/placebo)			
Intent To Treat (ITT) N=82 (53/29)	diff = 3 letters p = 0.291	diff = 5 letters p = 0.078	diff = 4 letters p = 0.026
ITT, excluding patients not legally blind* N=77, (50/27)	diff = 5 letters p = 0.075	diff = 7 letters p = 0.018	diff = 6 letters p = 0.002
ITT, only patients at highest risk** N=30, (20/10)	diff = 13 letters p = 0.011	diff = 20 letters p = 0.003	diff = 16 letters p = 0.0001
	Improvement	Protection	Overall vision

* Subpopulation of patients with both eyes having a logMAR value of less than 1.0

** Subpopulation of patients in progressive disease stage defined as two eyes having a difference in > 0.2 logMAR (~target population of initial protocol)

Note: A difference of 5 letters is equivalent to 1 line on a standard eye-chart or 0.1 logMAR.

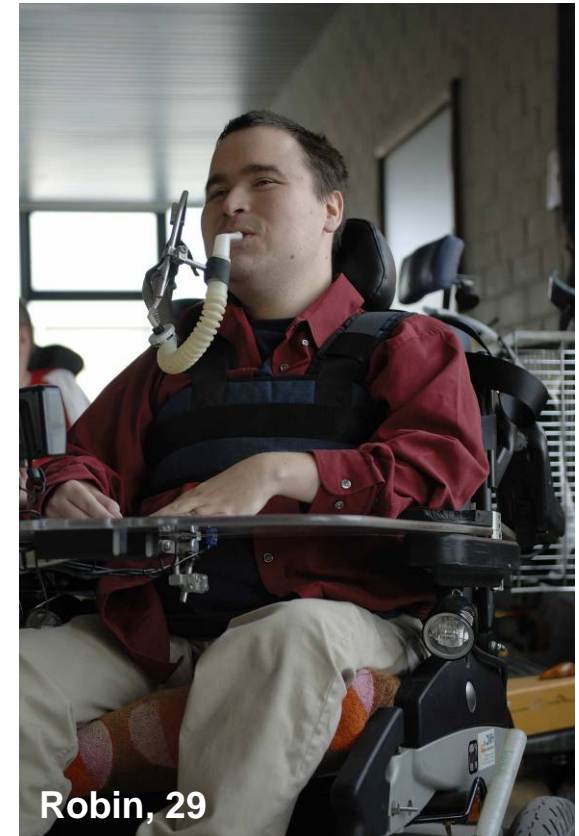
Key Findings of RHODOS Study

- RHODOS is the first randomized, placebo-controlled intervention study in LHON
- Catena® patients improved in vision when compared to placebo
- Catena® patients who were almost completely blind recovered sufficiently to read at least 5 letters on the standard eye-chart
- Catena® patients with residual vision (=patients at highest risk) were significantly protected from further disease progression
- Excellent safety profile of Catena® confirmed, i.e. drug is safe and well tolerated

Based on RHODOS results, considering scientific advice provided by regulatory authorities in EU, Santhera intends to apply for marketing authorization in 1H2011

Third Indication for Catena®/Sovrima®: Duchenne Muscular Dystrophy (DMD)

- **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 to 5 years, life expectancy 35 to 40 years**
- **Males, all ethnicities**
- **~30,000 total patients worldwide**
- **No approved pharmacological treatment available**
- **Chronic disorder, requires life-long treatment**



Catena[®]/Sovrima[®] in DMD: Clinical Development Program

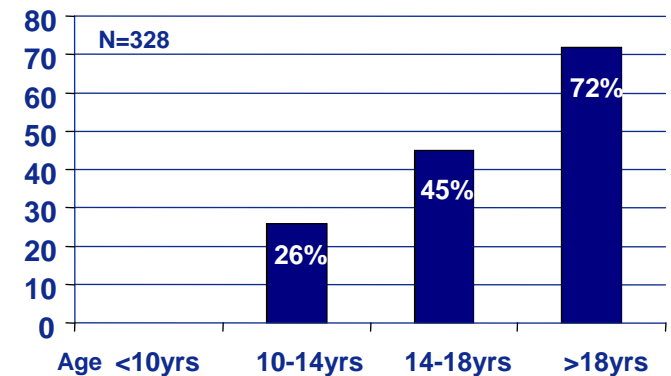
- **Proof of concept established in single-center DELPHI Phase IIa study**

- Efficacy on cardiac and respiratory function
- 12-month treatment
- 21 boys, aged 8 to 16 years

- **Pivotal DELOS Phase III study recruiting**

- 12-month treatment
- 8 study centers in Europe and US; open IND
- Group sequential design; 40 non-glucocorticoid users enrolled first; futility analysis after 6 month treatment in 2H 2010
- Primary endpoint: improvement in % predicted Peak Expiratory Flow (PEF)
- Secondary endpoints: pulmonary function, muscle strength and motor functions, quality of life

Development of dilated cardiomyopathy



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

Takeda Partnership: Commercialization of Sovrima[®] in Europe in DMD



- **Upfront and milestone payments**
 - EUR 2m upfront and EUR 5m milestone payment received
 - Up to EUR 13m further development and regulatory milestones
- **Takeda to pay 25% supply price on its net sales for product supply by Santhera plus 5% royalties on product sales**
- **Santhera has full access to all preclinical and clinical data and right to cross reference for regulatory filings**

Fipamezole

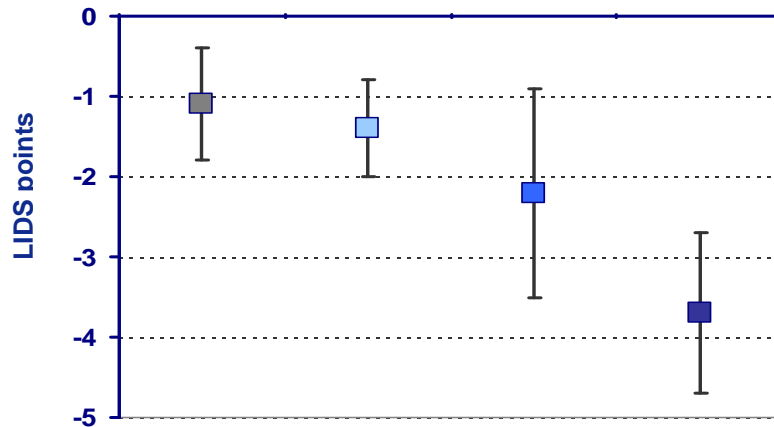
Dyskinesia in Parkinson's Disease: Severe Side-Effect of Standard Treatment

- **Motor dysfunction such as dyskinesia**
 - Caused by chronic use of dopaminergic drugs, gold standard in Parkinson's Disease
 - Periodically occurring chaotic movements of arms, legs, trunk and face
 - Increase in frequency, severity over time
- **~400,000 patients in North America and Europe suffer from levodopa-induced troublesome dyskinesia**
- **No approved pharmacological treatment available**
- **Chronic treatment required**
- **USD ~500m peak sales** (Source: Decision Resources)

FJORD Phase IIb: Fipamezole Reduces Levodopa-Induced Dyskinesia in All Body Parts

Average Dyskinesia – LIDS

(US sites, ITT-OC); Change: D28-BL

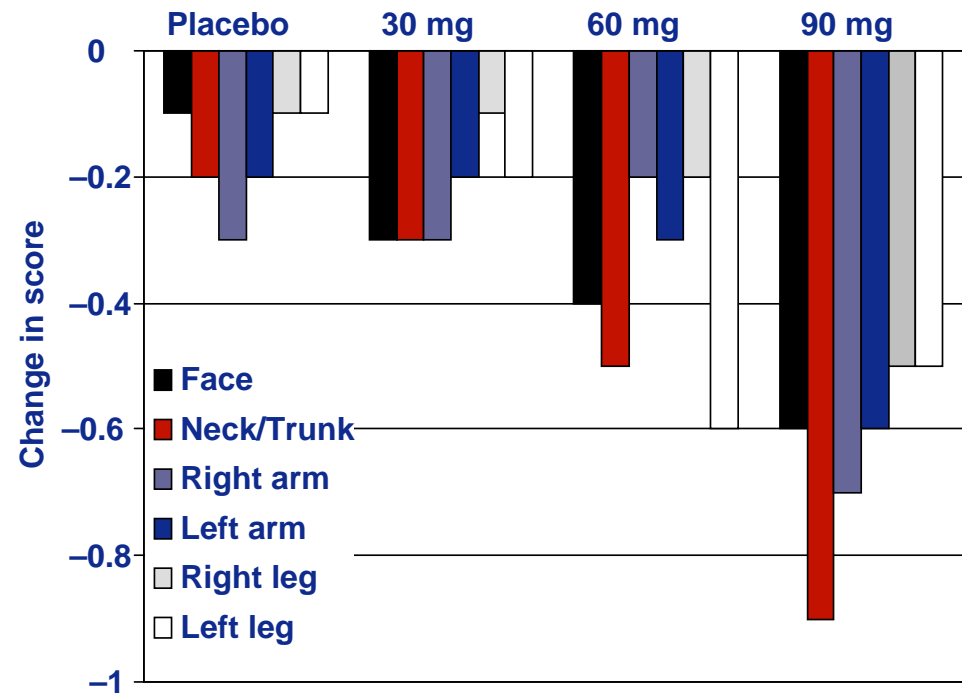


LIDS	Placebo	30 mg	60 mg	90 mg
Baseline ¹	9.0 ± 0.7	8.9 ± 0.7	10.1 ± 0.8	10.7 ± 0.8
D28-BL ¹	-1.1 ± 0.7	-1.4 ± 0.6	-2.2 ± 1.3	-3.7 ± 1.0
N at D28 ¹	27	19	14	19
Difference ²	-	-0.3 ± 0.9	-0.7 ± 1.1	-1.9 ± 0.9
P-value ²	-	0.790	0.521	0.047

	P-value
Linear regression model	0.0187
Jonckheere's test for group	0.0066

Average LIDS per body part

(US sites, ITT-OC); Change: D28-BL



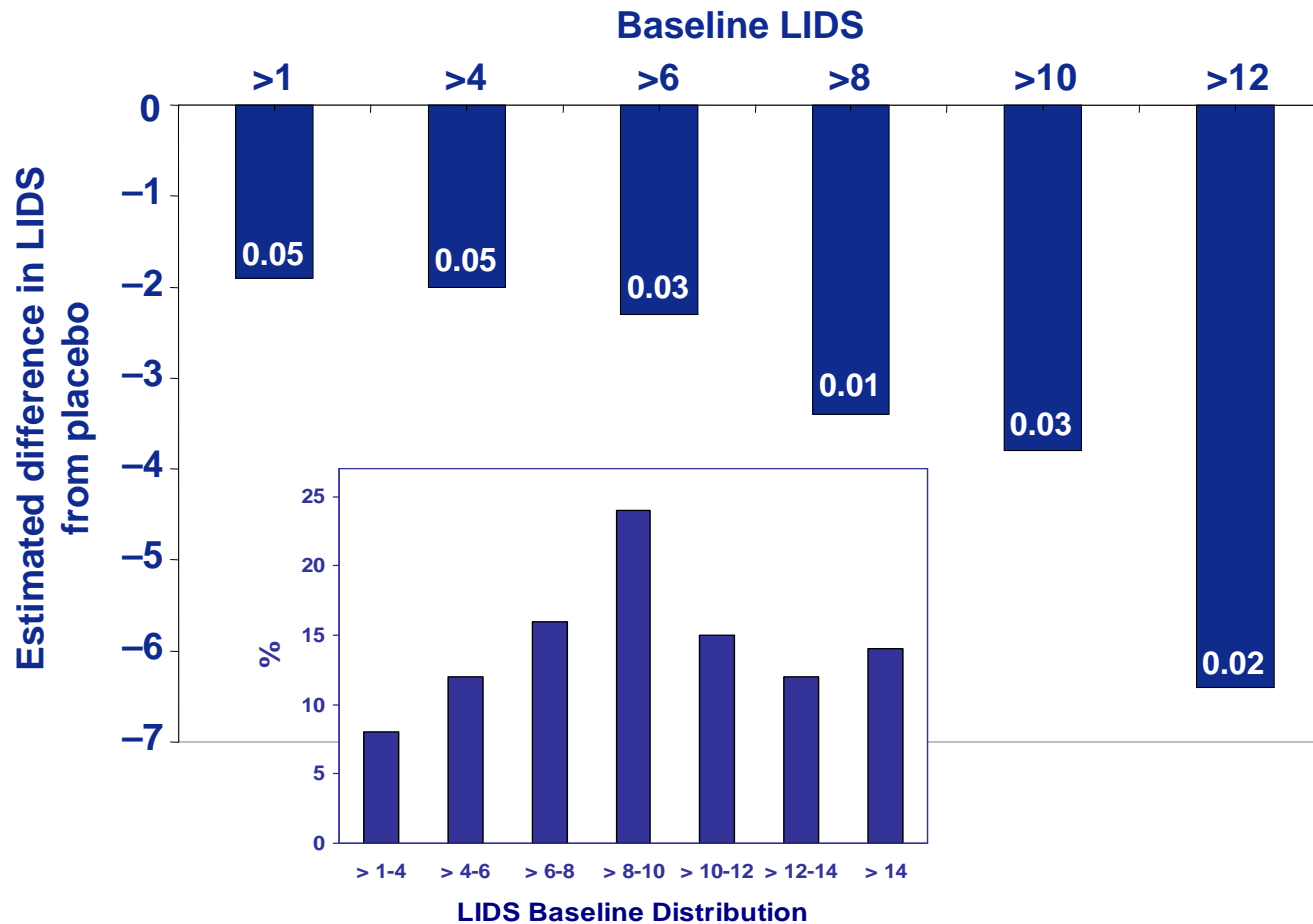
1: descriptive statistics: raw means ± SEM;

2: RMANCOVA analysis comparison to placebo (estimate ± SEM)

FJORD Phase IIb: Fipamezole Reduces Dyskinesia More Efficiently in PD Patients with Higher LIDS



Average LIDS change from baseline
(US Sites; D28-BL in Average Dyskinesia; ITT-OC)



Key Findings from FJORD Phase IIb



- **Highest dose (90mg) effective for reducing levodopa-induced dyskinesia in pre-specified secondary analysis, assessed by LIDS (p=0.047 vs placebo, n=29)**
- **Significant treatment dose effect for reducing dyskinesia across all dose groups (p=0.0066)**
- **Beneficial effect on dyskinesia not associated with a clinically relevant worsening of Parkinsonian features, assessed by UPDRS III**
- **Significant treatment dose effect regarding reduction in hours spent with diminished mobility (“off time”)**
- **High percentage of completers: 78%; expected 65 to 70%**
- **Strong trend to improvement in cognitive function**
- **Favorable clinical global impression in improvement (CGI-I) of dyskinesia**
- **Compound safe and well tolerated**

Partnering Fipamezole for Development & Commercialization

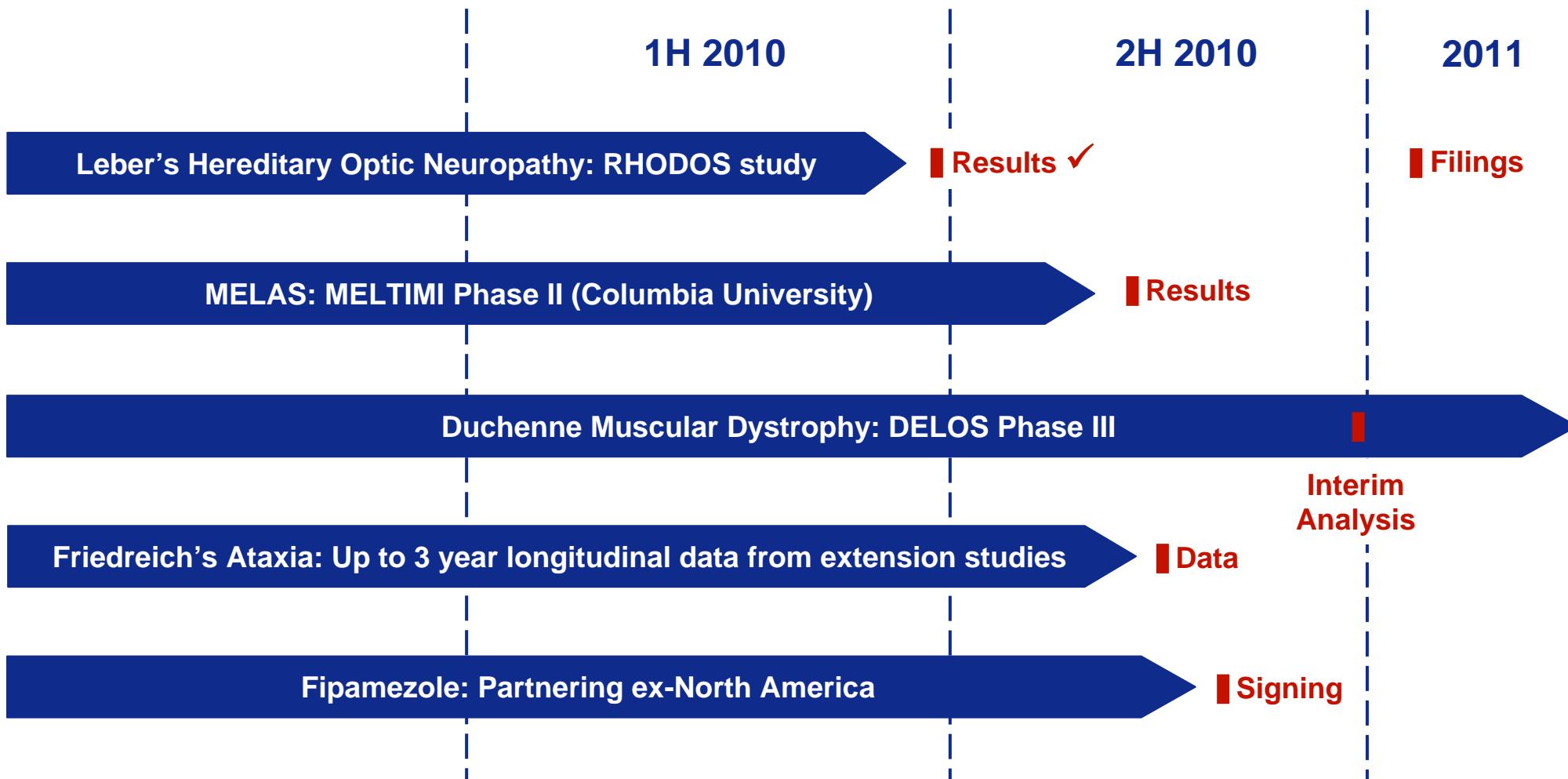


- **Licensing agreement with Biovail signed in August 2009**
 - Development and commercialization in US and Canada, Initiation of Phase III in 2011
 - Financial Terms: USD 12m upfront payment, up to USD 35m development/regulatory milestones and up to USD 145m sales milestones; 8 to 15% royalties on net sale
 - Access for Santhera to all data generated by Biovail and right to sublicense data to partners outside North America
 - Co-promotion rights retained for US
 - Biovail has right to develop compound in additional neurological diseases (up to USD 20m success milestone due upon approval of second indications)

- **Partnering activities ongoing for ex-North American rights; Signing expected in 2H 2010**

Outlook

Expected Milestones and Short-Term News Flow



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Hopefully she'll never
hear the name Santhera
as she grows up...



...but if she needs us,
we'll be with her
every step of the way.

As an emerging speciality pharmaceutical company, Santhera believes that the development of small molecules has a big future.

Unmet medical need is what drives our work. Particularly rare diseases and the development of orphan drugs where there are no current alternatives.

That's why we are busy developing novel solutions to improve the lives of patients with severe neuromuscular diseases.

At Santhera we care about people – every step of the way.

Date of preparation: August 2008

when it comes to science

we know how to move people

