santhera
Pharmaceuticals

Advancing Mitochondrial Medicine

Günther Metz, SVP Business Development

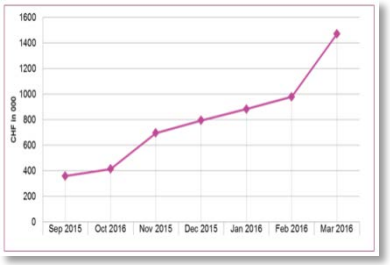
Disclaimer

This presentation is not and under no circumstances to be construed as a solicitation, offer, or recommendation, to buy or sell securities issued by Santhera Pharmaceuticals Holding AG. Santhera Pharmaceuticals Holding AG makes no representation (either express or implied) that the information and opinions expressed in this presentation are accurate, complete or up to date. Santhera Pharmaceuticals Holding AG disclaims, without limitation, all liability for any loss or damage of any kind, including any direct, indirect or consequential damages, which might be incurred in connection with the information contained in this presentation.

This presentation expressly or implicitly contains certain forward-looking statements concerning Santhera Pharmaceuticals Holding AG and its business. Certain of these forward-looking statements can be identified by the use of forward-looking terminology or by discussions of strategy, plans or intentions. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Santhera Pharmaceuticals Holding AG to be materially different from any expected results, performance or achievements expressed or implied by such forward-looking statements. There can be no guarantee that any of the research and/or development projects described will succeed or that any new products or indications will be brought to market. Similarly, there can be no guarantee that Santhera Pharmaceuticals Holding AG or any future product or indication will achieve any particular level of revenue. In particular, management's expectations could be affected by, among other things, uncertainties involved in the development of new pharmaceutical products, including unexpected preclinical and clinical trial results; unexpected regulatory actions or delays or government regulation generally; the Company's ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; government, industry, and general public pricing and other political pressures. Santhera Pharmaceuticals Holding AG is providing the information in this new release as of the date of the publication, and does not undertake any obligation to update any forward-looking statements contained herein as a result of new information, future events or otherwise.

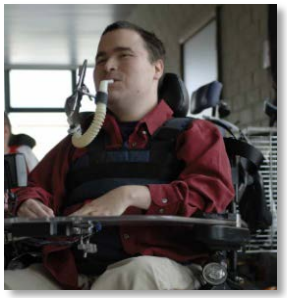
Highlights 2015

- Regulatory approval in EU for Raxone® as first treatment for LHON



- Successful product launch in first countries and increasing sales of Raxone®

- Advanced preparation for regulatory filings in EU and US for DMD



- Successful financing to support commercial launch and regulatory projects

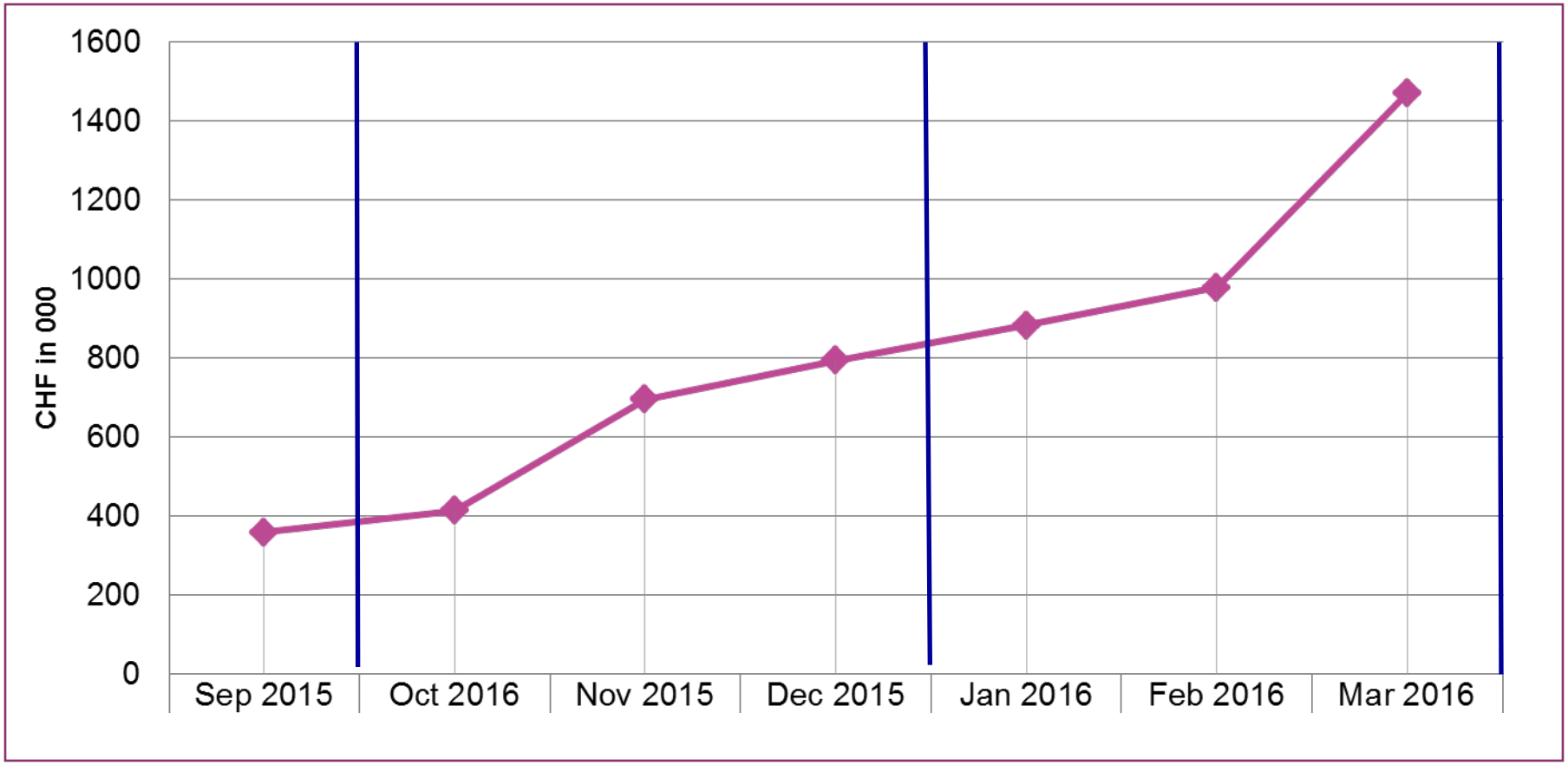
Key Financials 2015 and 1Q 2016

(IFRS, consolidated, in CHF million)	1Q 2016	**2015**	2014
Net sales	3.3	**4.3**	2.6
Operating expenses		**0**	–10.9
due to reversal impairment intangibles and inventory		*27.1*	
Net result		**5.9**	–7.5
Cash & cash equivalents	69.4	**76.9**	17.4
Net change in cash & cash equivalents	–7.5	**59.4**	12.4
Operating cash flow		**–22.4**	–6.1

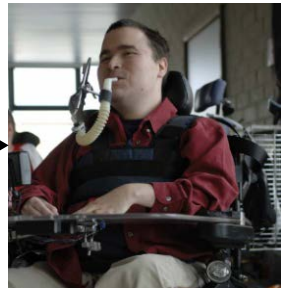
- Positive net result: CHF 5.9 million
- Financing and income from Raxone sales increased cash position (CHF 76.9 million)
  - Capital increases of total CHF 82.4 million (gross); CHF 80.5 million (net)
  - Revenues increased to CHF 4.3 million
- Current Headcount: 65 (end of 2014: 18)

Sales development of Raxone®

Patients on drug:	**~120**	**~220**
Quarterly net sales:	**CHF 1.9M**	**CHF 3.3M**



Pipeline with Raxone® (idebenone) in three indications with high unmet medical need



Leber's Hereditary Optic Neuropathy (LHON):

Commercial launch in EU

Duchenne Muscular Dystrophy (DMD):

NDA/MAA filings in preparation

Primary progressive MS (PPMS):

Phase II study ongoing

Raxone® in Leber's Hereditary Optic Neuropathy (LHON)

Leber's Hereditary Optic Neuropathy (LHON): Commercial launch in EU

Clinical presentation of LHON

Normal vision

Vision due to LHON

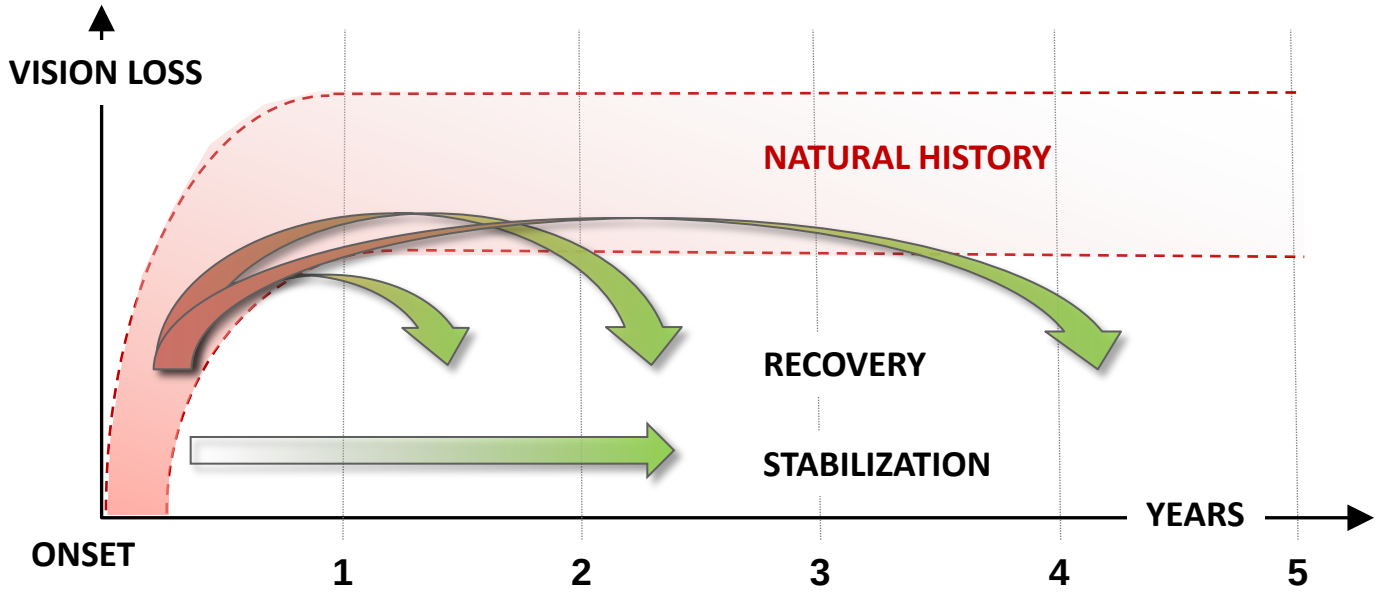
Days, weeks or few months

Effective therapy to improve visual acuity (VA)

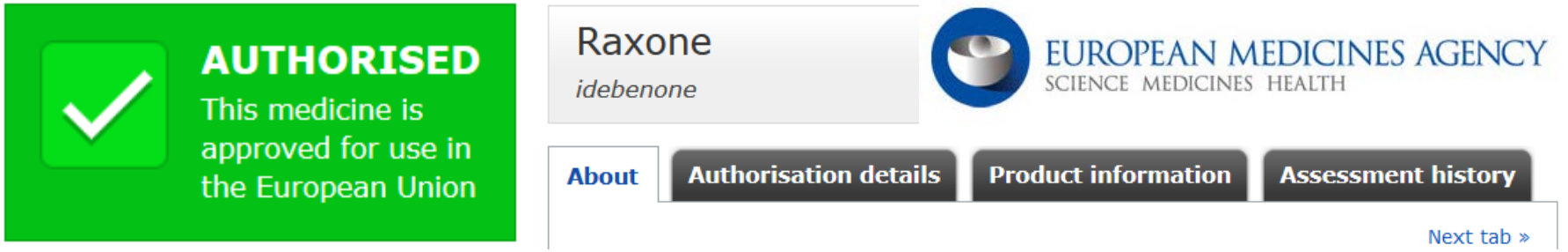
- Therapeutic objectives:
  - Prevention of further vision loss
  - Clinically relevant recovery of visual acuity (VA)

Efficacy demonstrated by Raxone

- There is a window of opportunity for treatment while retinal ganglion cells are still viable (up to several years after onset of vision loss)



Raxone® is the first and only approved treatment for LHON



- Authorized for the treatment of visual impairment in adolescent and adult patients with LHON
  - for all disease stages
  - for all LHON mutations
- 10 years market exclusivity due to Orphan Drug Status (until Q3 2025)
- Expected annual peak sales potential in EU: CHF ~60 M (by 2019)

European Commercialization of Raxone®

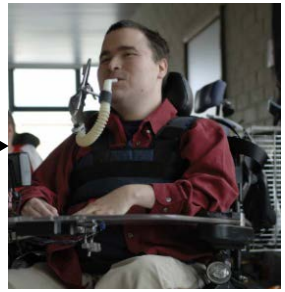
- Commercial presence in 4 regional clusters

  Headcount of commercial team currently 29 (45% of total)

- Distribution agreement with Ewopharma for Eastern Cluster countries

Western
Central
Southern
Northern
Distribution agreement

Raxone® in Duchenne Muscular Dystrophy



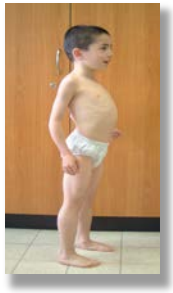
Duchenne Muscular Dystrophy (DMD):

Positive phase III study outcome,

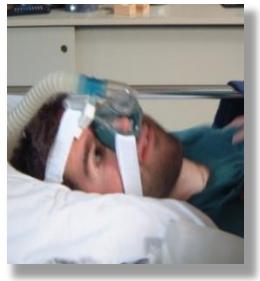
NDA/MAA filing in preparation

Medical need for effective treatment of respiratory illness in DMD

- Medical complications include ineffective cough, nocturnal hypoventilation, sleep disordered breathing, and ultimately daytime respiratory failure





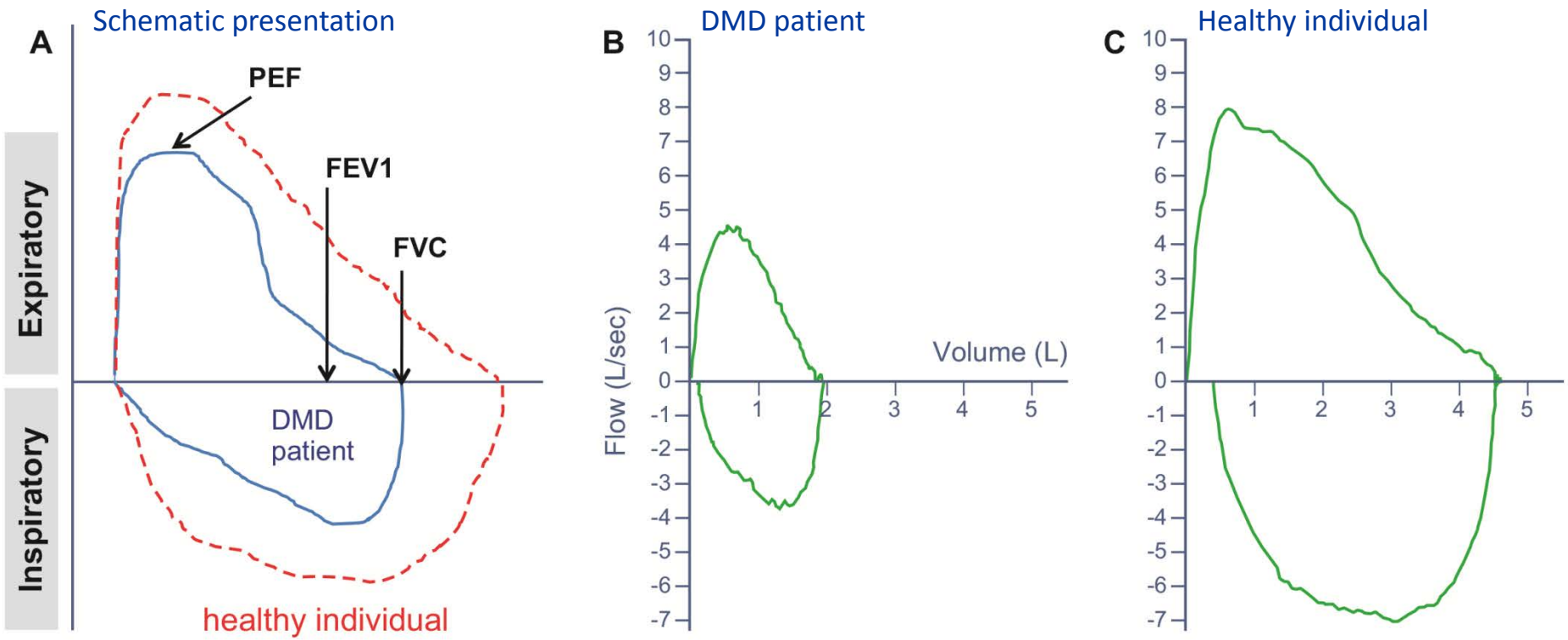


Loss of respiratory function
Assisted ventilation
Nocturnal ventilation

- DMD patients develop cardiac and respiratory complications that typically lead to early morbidity and mortality

Pictures courtesy: Nathalie Goemans, University Hospitals Leuven, Belgium

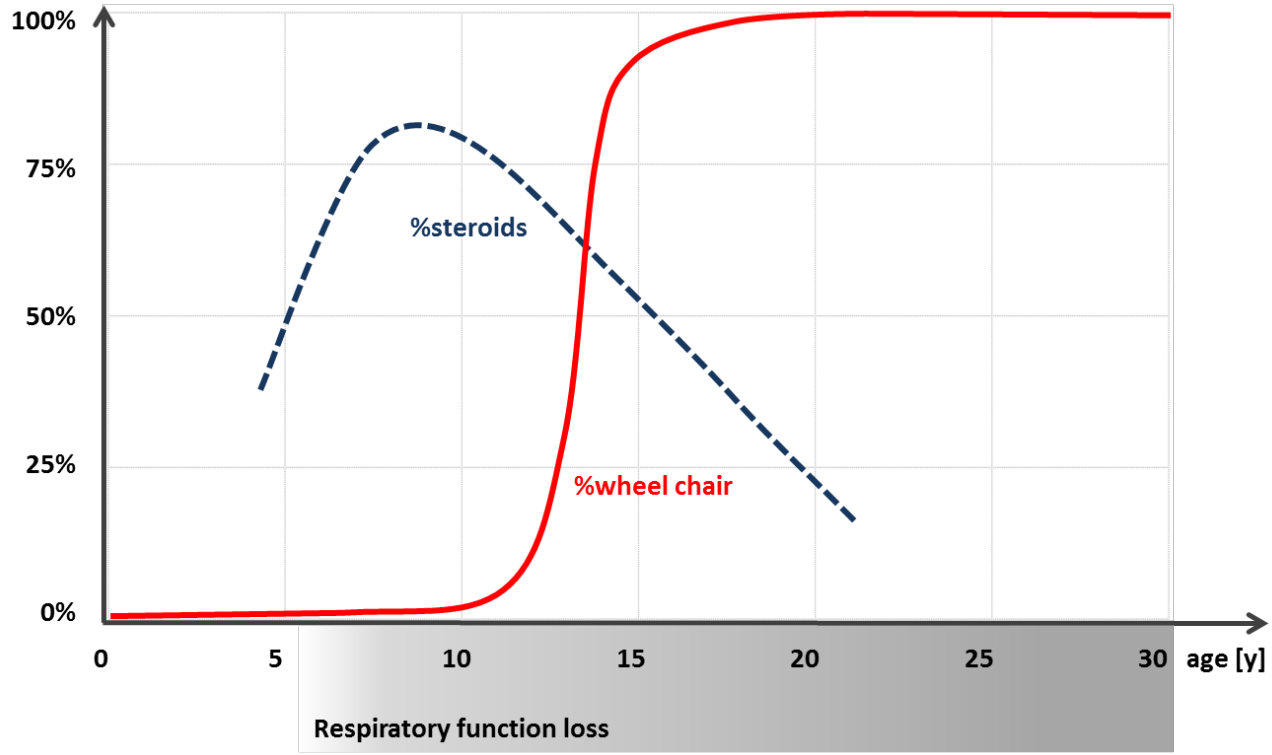
Measures of pulmonary function loss in DMD



Figures (B) and (C) courtesy of Dr. Oscar H. Mayer, Division of Pulmonology, The Children's Hospital of Philadelphia, USA.

Urgent medical need for patients unable to take glucocorticoid steroids

- With increasing age, fewer patients tolerate glucocorticoid steroids (side effects)
- Loss of respiratory function enters critical stage in early teenage years
- There is currently no treatment available for this group of DMD patients



Phase 3 DELOS trial

Efficacy of idebenone on respiratory function in patients with Duchenne muscular dystrophy not using glucocorticoids (DELOS): a double-blind randomised placebo-controlled phase 3 trial

Gunnar M Buyse, Thomas Voit, Ulrike Schara, Chiara S M Straathof, M Grazia D'Angelo, Günther Bernert, Jean-Marie Cuisset, Richard S Finkel, Nathalie Goemans, Craig M McDonald, Christian Rummey, Thomas Meier, for the DELOS Study Group

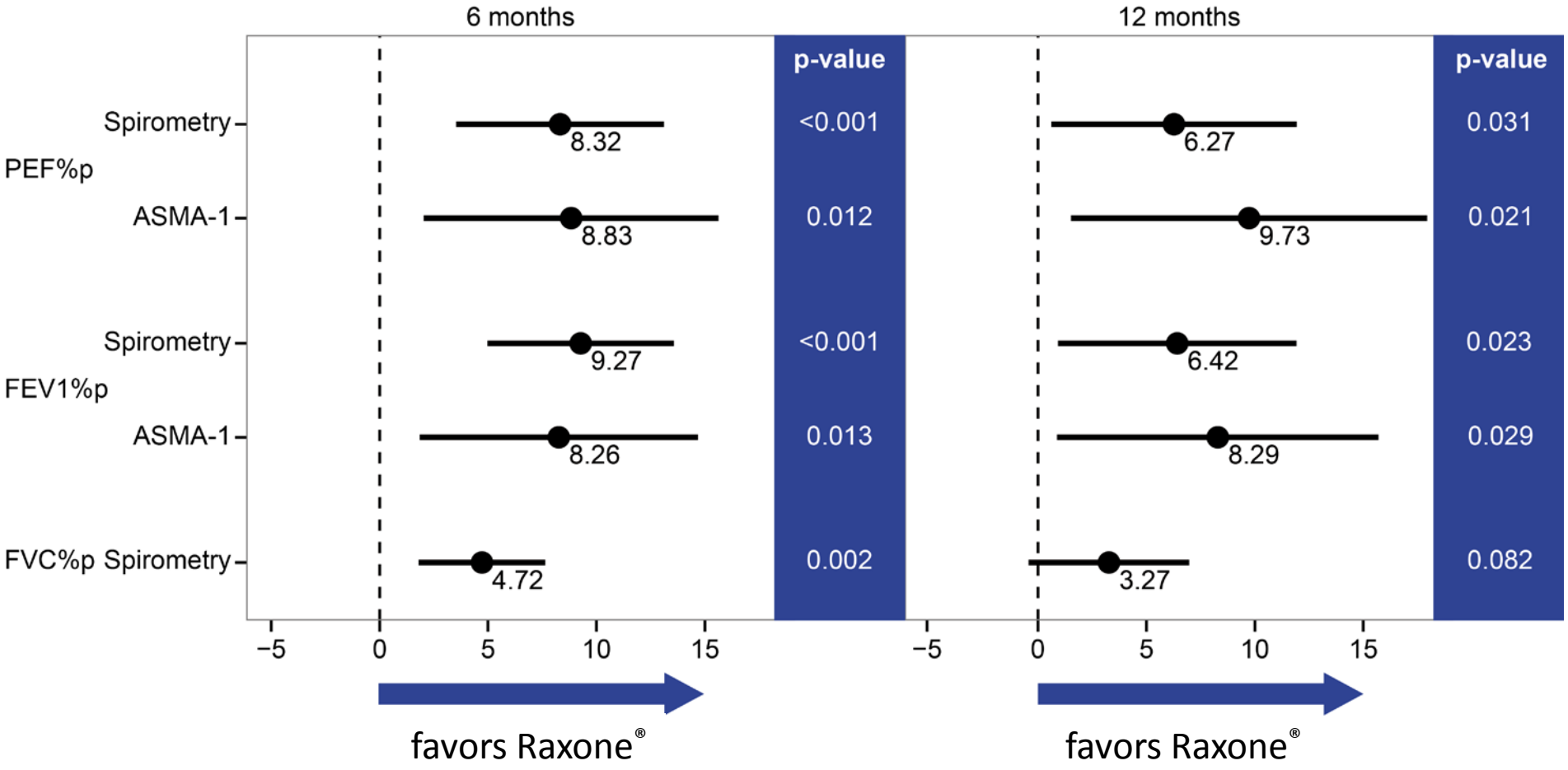
Patients:

- Age 10-18 years
- No selection for mutational status
- Patients had to be off chronic steroids
- 92% of patients were non-ambulatory
- Established respiratory function decline

Randomized treatment:

- Raxone (900 mg/d): N=31
- Placebo: N=33
- Mean Age: 14.3 y
- Treatment duration: 12 months

Raxone® delays the loss of respiratory function

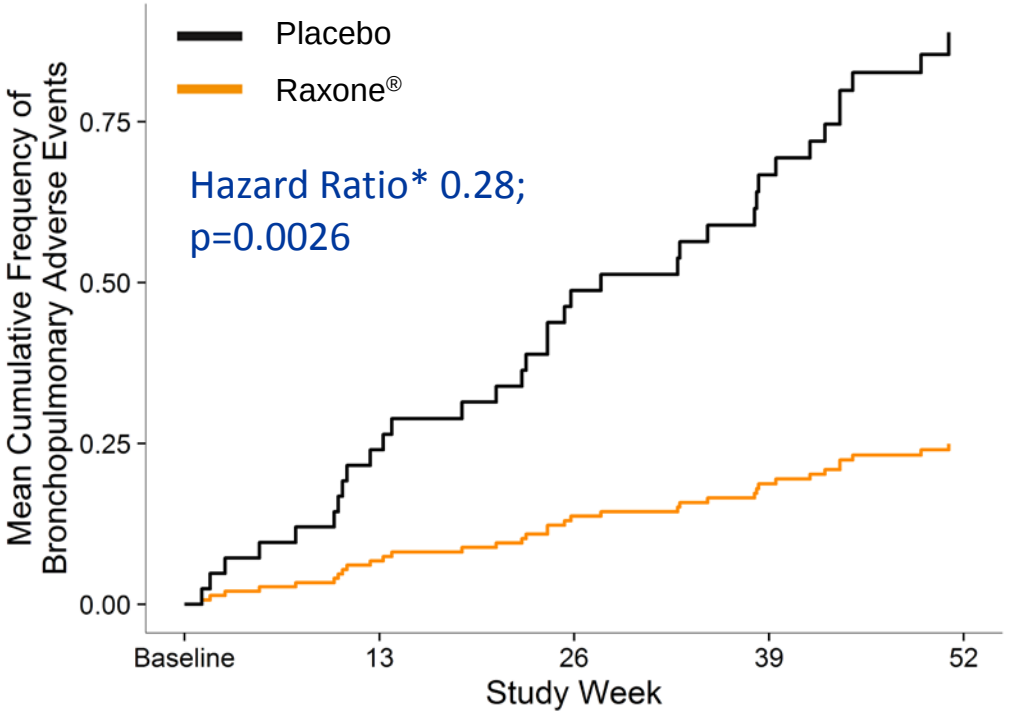


Fewer patients on Raxone® experience bronchopulmonary disease (e.g. airway infections)

	Raxone	Placebo
Subjects (%)	6 (19.4%)	17 (51.2%)
Events	7	28

Duration of bronchopulmonary disease

Total Days	**82**	**222**

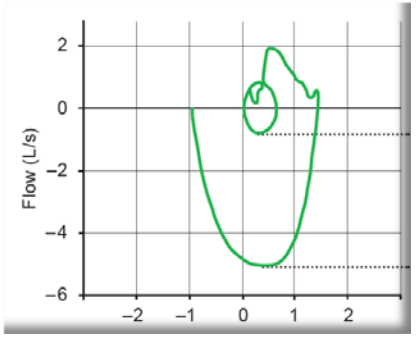


Comprehensive data package for MAA/NDA filings

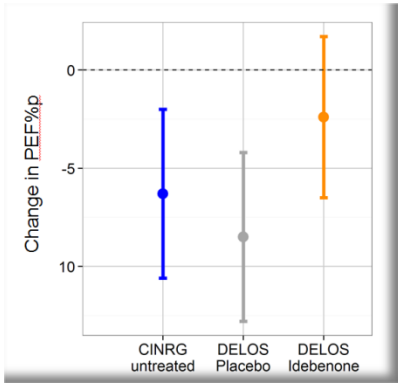
- Patient-centered benefit-risk survey highlights importance to treat pulmonary disease in patients with DMD

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

- Data from pivotal DELOS trial and phase II DELPHI program
  - Statistically significant and clinically relevant efficacy for Raxone on expiratory and inspiratory function
  - Treatment effect for bronchopulmonary complications and antibiotic use
  - Consistency and robustness of study results



- External validation of DELOS results: placebo group representative of natural history decline in respiratory function

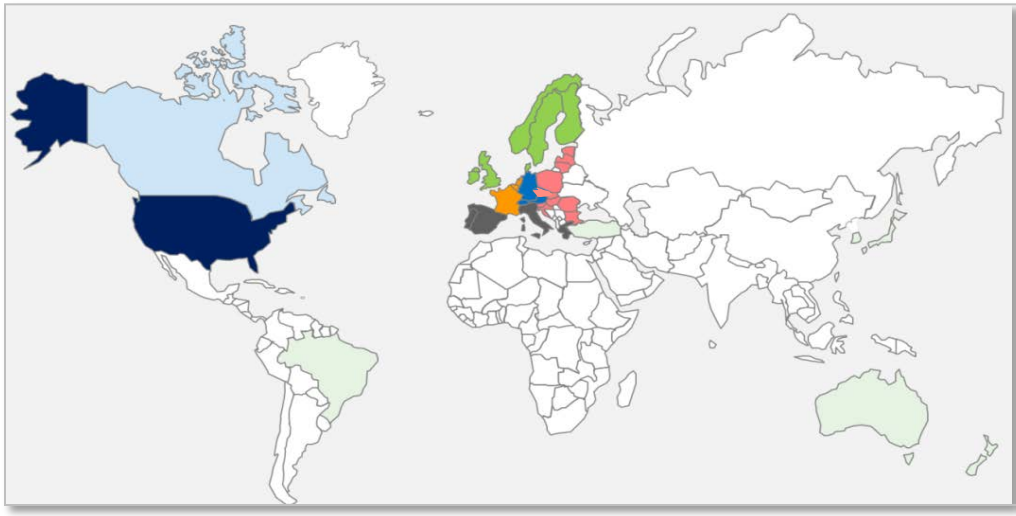


- Comprehensive safety data base (benign safety profile)

Raxone® - initial target population in DMD

- **Patients with respiratory function decline currently not using glucocorticoids**
  - ➢ ~42% of patients ≥ 10 years do not use glucocorticoids
- **Assumption for potential Raxone treated population: 6,500 patients in EU & US**

	Population [m]	DMD Patients	Raxone Target Pop.	Patients on drug
US	320	12,800	5,120	
CA	35	1,400	560	
	355	14,200	5,680	2,900
D-A-CH	100	4,000	1,600	
F-BeNeLx	95	3,800	1,520	
UK-Nordics	95	3,800	1,520	
Southern	125	5,000	2,000	
Eastern	105	4,200	1,680	
	520	20,800	8,320	3,600



Raxone® in the treatment of DMD

Clinical positioning

- First and only treatment for DMD patients not using steroids
- Patients eligible irrespective of mutation type or ambulatory status
- Convenient oral medication (2 tablets, 3 times/day)

Market protection

- Patent protection until March 2026 (EU, JP) and December 2027 (USA)
- Orphan Drug Designation granted in EU and US

Raxone® in primary progressive MS

Primary progressive MS (PPMS):

Phase II study in collaboration with NIH

Phase II study in PPMS with Raxone®

- Primary progressive MS
  - affects 10-15% of total MS population: patients in EU*: 580’000, US*: 460’000
  - no approved treatment available
  - associated with mitochondrial pathology

- Phase II trial (IPPoMS) in collaboration with NIH ongoing
- Study is fully recruited
- Santhera has exclusive license to granted use patent
- Not included in company valuations to date

*Source: Roche company presentation 2015

Outlook for 2016

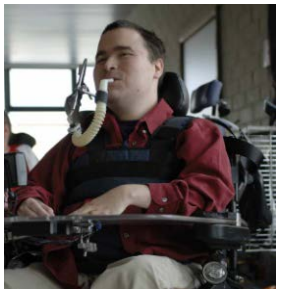
- **Continue commercial roll-out in EU for Raxone as treatment of LHON**

- **Filing for regulatory approvals for Raxone in DMD in EU and US**
  - **Meeting request submitted to FDA for discussion of NDA filing**
  - **Marketing Authorization Application in Europe to be submitted shortly**

- **Preparation of market entry for DMD in EU and US**

Advancing mitochondrial medicine towards treatments for

LHON



DMD

PPMS

Thank you for your attention