At Santhera, we’re studying a potential new treatment option to slow the progression of respiratory function decline in Duchenne.
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.
The following presentation is for educational purposes. It features information about:

- Duchenne muscular dystrophy
- Understanding the importance of respiratory health in Duchenne
- Santhera’s role in studying a potential treatment option
- Potential participation in DMD clinical trials

This presentation is intended for educational purposes only. It is intended for informational purposes only, and should not be used in place of a discussion with a health care professional. All decisions regarding patient care must be handled by a health care professional, and be made based on the unique needs of each patient.
Meet Santhera

US headquarters in Burlington, MA with global headquarters in Liestal, Switzerland

Working to develop idebenone for Duchenne since 2008

3 completed studies and 1 ongoing study in Duchenne

Majority of clinical trial patients were non-ambulatory

Approved for Leber’s Hereditary Optic Neuropathy (LHON) in Europe

---

OUR PIPELINE

idebenone in DMD (Duchenne muscular dystrophy)
  Phase 3

idebenone in PPMS (Primary progressive multiple sclerosis)
  Phase 2

omigapil in CMD (Congenital muscular dystrophy)
  Phase 1
In Duchenne, the muscles that support breathing weaken in the same way that leg and heart muscles weaken.

**Over time, little-by-little, the respiratory system loses strength which means:**

- Supporting muscles continue to weaken
  - This can make the lungs less effective at moving air in and out
- Cough gets weaker
- Changes that seem minor can become more serious complications

Respiratory complications are one of the leading causes of death in Duchenne.
How to Protect Respiratory Health: Good Care and Be Aware

Protecting respiratory health means being mindful of small changes through regular testing and symptom management.

**GOOD CARE**

- Have a care team of experts starting with a neurologist
- See pulmonologist every year, 2 times/year if non-ambulatory
  - While still able to walk, boys should have a respiratory test
  - Lung function tests can help signal if there is trouble breathing now or in the future

**BE AWARE**

- Watch symptoms closely when non-ambulatory for early signs that respiratory muscles are weakening:
  - Headaches
  - Restless sleep
  - Trouble concentrating
  - Difficulty staying awake
- Protect against infections and clear airways on a regular basis
  - Get flu and pneumococcal vaccines
  - Watch colds carefully
What Is Idebenone?

Idebenone is an investigational oral tablet that works within the mitochondria.

Healthy mitochondria

Unhealthy mitochondria
**DELOS:** Phase 3 trial in patients in respiratory decline not using steroids (complete)

**Objective:** to study the efficacy of idebenone compared to placebo, on respiratory function in patients with Duchenne not using steroids.

**Study design and patients**

- **64** males with Duchenne
- **92%** of patients were non-ambulatory
- **17** centers around the world
- **52** weeks

Idebenone or placebo
DELOS Primary Endpoint: The primary endpoint was a change from baseline to week 52 in PEF%p (percent predicted peak expiratory flow)
  • PEF measures peak or maximum flow of air when a boy breathes out as hard as he can

DELOS PEF Outcome: The decline in PEF%p from baseline to week 52 was 3.05% for the idebenone group versus 9.01% for the placebo group ($p=0.044$)
  • There was a 66% reduction in loss of PEF%p between the treatment group and the placebo group

DELOS Side Effects: The most common side effects were diarrhea, abdominal pain, nasopharyngitis (common cold), and headache. Overall treatment was well-tolerated.
**Objective:** to study the efficacy of idebenone compared to placebo, in delaying the loss of respiratory function in patients with Duchenne who are using steroids.

**Study design and patients**

| 266 | 10 | 64 | 78 |
| males with Duchenne | years of age and older who are using steroids | centers worldwide (24 in the US) | weeks |

**SIDEROS:** Phase 3 trial in patients using steroids (ongoing)
Idebenone in Duchenne Clinical Trials (SIDEROS)

Inclusion Criteria

30-80% FVC*

Boys and men with a forced vital capacity between 30% and 80% of predicted value

NO

Prednisone or deflazacort, any regimen

daytime ventilation assistance

*The total amount forcibly blown out after one big breath. High FVC scores are a sign that the lungs are inflating to full capacity.
SIDEROS Clinical Trial Sites

Visit [www.siderosdmd.com](http://www.siderosdmd.com) for an in-depth trial overview

ClinicalTrials.gov identifier: NCT#02814019

[Contact Information]

Jodi Wolff, PhD
Jodi.wolff@santhera.com
Sideros@santhera.com
Santhera is a proud partner of the Duchenne community.
Thank you for joining us today.

At Santhera, we believe information and support can be empowering.
SIDEROS – US Clinical Trial Sites

Dr. Bradley Troxler
University of Alabama
Birmingham, Alabama

Dr. James Woodward
Phoenix Children’s Hospital
Phoenix, Arizona

Dr. Cori Daines
Banner–University Medical Center
Tucson, Arizona

Dr. Vikki Stefans
Arkansas Children’s Hospital
Little Rock, Arkansas

Dr. David Michelson
Loma Linda University Medical Center
Loma Linda, California

Dr. Leigh Maria Ramos-Platt
Childrens Hospital of Los Angeles
Los Angeles, California

Dr. Perry Shieh
David Geffen School of Medicine at UCLA
Los Angeles, California

Dr. Craig McDonald
UC Davis Department of Physical Medicine and Rehabilitation
Sacramento, California

Dr. Marisa Couluris
Shriners Hospitals for Children
Tampa, Florida

Dr. Han Phan
Center for Integrative Rare Disease Research
Atlanta, Georgia

Dr. Marcia Felker
Indiana University School of Medicine
Indianapolis, Indiana

Dr. Kathy Mathews
University of Iowa
Iowa City, Iowa

Dr. Jeffrey Statland
University of Kansas Medical Center
Kansas City, Kansas

Dr. Thomas Crawford
Johns Hopkins University Hospital
Baltimore, Maryland

Dr. Basil Darras
Boston Children’s Hospital
Boston, Massachusetts

Dr. Stephen Smith
Gillette Children’s Specialty Healthcare
St Paul, Minnesota

Dr. Emma Ciafaloni
University of Rochester
Rochester, New York

Dr. Benjamin Brooks
Carolinas HealthCare System
Charlotte, North Carolina

Dr. Cuixia Tian
Cincinnati Children's Hospital Medical Center
Cincinnati, Ohio

Dr. Andre Prochoroff
MetroHealth Medical Center
Cleveland, Ohio

Dr. Oscar Henry Mayer
Children’s Hospital of Philadelphia
Philadelphia, Pennsylvania

Dr. Tulio Bertorini
Wesley Neurology Clinic
Memphis, Tennessee

Dr. Warren Marks
Cook Children’s Medical Center
Fort Worth, Texas

Dr. Russell Butterfield
University of Utah School of Medicine
Salt Lake City, Utah