At Santhera, we’re studying a potential new treatment option to slow the progression of respiratory function decline in Duchenne.
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Educational Purposes

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 Duchenne 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

<table>
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<th>OUR PIPELINE</th>
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<tbody>
<tr>
<td><strong>idebenone in DMD</strong> (Duchenne muscular dystrophy)</td>
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<tr>
<td>Phase 3</td>
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<td><strong>idebenone in PPMS</strong> (Primary progressive multiple sclerosis)</td>
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<td>Phase 2</td>
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<td><strong>omigapil in CMD</strong> (Congenital muscular dystrophy)</td>
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<td>Phase 1</td>
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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 or go unnoticed 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
  - Shallow breathing at night
  - Trouble concentrating
  - Difficulty staying awake
  - Unexpected weight loss
- Protect against infections and clear airways on a regular basis
  - Get flu and pneumococcal vaccines
  - Watch colds carefully
Welcome to **TakeabreathDMD.com**

As a proud partner of the Duchenne community, we’re happy to introduce **TakeabreathDMD.com**

You'll find valuable information about DMD respiratory health including:

- Ways to help manage lung function and well-being
- How to address respiratory complications
- Helpful news about DMD when you sign up for updates

Take a look for yourself and see what **TakeabreathDMD.com** has to offer.
What Is Idebenone?

Idebenone is an investigational oral tablet that works within the 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.
Idebenone in Duchenne Clinical Trials (SIDEROS)

**SIDEROS**: Phase 3 trial in patients using steroids (ongoing)

**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** males with Duchenne
- **10** years of age and older who are using steroids
- **64** centers worldwide (23 in the US)
- **78 weeks**

Idebenone or placebo
## Idebenone in Duchenne Clinical Trials (SIDEROS)

### Inclusion Criteria:

<table>
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<tr>
<th>DNA symbol</th>
<th>FVC*</th>
<th>Boys and men with a forced vital capacity between 35% and 80% of predicted value</th>
<th>Any regimen of prednisone (prednisolone) or deflazacort (calcort)</th>
<th>Corticosteroids use for at least 12 months prior to randomization without any dose adjustments in last 6 months</th>
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<tr>
<td>35-80%</td>
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<td>Independent of dystrophin mutation type</td>
<td>12 months</td>
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### Exclusion Criteria:

- Those needing daytime ventilation assistance
- Cannot be part of any other ongoing therapeutic trial or any experimental drug within 90 days prior to start of SIDEROS participation (with exception of deflazacort)

*The total amount forcibly blown out after one big breath. FVC scores indicate the degree to which the lungs are inflating to full capacity.*
SIDEROS Clinical Trial Sites

Visit www.Siderosdmd.com for an in-depth trial overview

ClinicalTrials.gov identifier: NCT#02814019

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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.
APPENDIX
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<th>Dr. Bradley Troxler</th>
<th>University of Alabama</th>
<th>Birmingham, Alabama</th>
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<tr>
<td>Dr. James Woodward</td>
<td>Phoenix Children's Hospital</td>
<td>Phoenix, Arizona</td>
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<tr>
<td>Dr. Cori Daines</td>
<td>Banner–University Medical Center</td>
<td>Tucson, Arizona</td>
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<tr>
<td>Dr. Vikki Stefans</td>
<td>Arkansas Children's Hospital</td>
<td>Little Rock, Arkansas</td>
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<td>Dr. David Michelson</td>
<td>Loma Linda University Medical Center</td>
<td>Loma Linda, California</td>
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<td>Dr. Leigh Maria Ramos-Platt</td>
<td>Children's Hospital of Los Angeles</td>
<td>Los Angeles, California</td>
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<tr>
<td>Dr. Perry Shieh</td>
<td>David Geffen School of Medicine at UCLA</td>
<td>Los Angeles, California</td>
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<tr>
<td>Dr. Craig McDonald</td>
<td>UC Davis Department of Physical Medicine and Rehabilitation</td>
<td>Sacramento, California</td>
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<tr>
<td>Dr. Marisa Couluris</td>
<td>Shriners Hospitals for Children</td>
<td>Tampa, Florida</td>
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<tr>
<td>Dr. Han Phan</td>
<td>Center for Integrative Rare Disease Research</td>
<td>Atlanta, Georgia</td>
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<td>Dr. Kathy Mathews</td>
<td>University of Iowa</td>
<td>Iowa City, Iowa</td>
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<td>Dr. Jeffrey Statland</td>
<td>University of Kansas Medical Center</td>
<td>Kansas City, Kansas</td>
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<tr>
<td>Dr. Thomas Crawford</td>
<td>Johns Hopkins University Hospital</td>
<td>Baltimore, Maryland</td>
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<tr>
<td>Dr. Basil Darras</td>
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<tr>
<td>Dr. Stephen Smith</td>
<td>Gillette Children’s Specialty Healthcare</td>
<td>St Paul, Minnesota</td>
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<td>Dr. Emma Ciafaloni</td>
<td>University of Rochester</td>
<td>Rochester, New York</td>
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<tr>
<td>Dr. Benjamin Brooks</td>
<td>Carolinas HealthCare System</td>
<td>Charlotte, North Carolina</td>
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<tr>
<td>Dr. Cuixia Tian</td>
<td>Cincinnati Children's Hospital Medical Center</td>
<td>Cincinnati, Ohio</td>
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<td>Dr. Andre Prochoroff</td>
<td>MetroHealth Medical Center</td>
<td>Cleveland, Ohio</td>
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<td>Dr. Oscar Henry Mayer</td>
<td>Children's Hospital of Philadelphia</td>
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<td>Dr. Tulio Bertorini</td>
<td>Wesley Neurology Clinic</td>
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<tr>
<td>Dr. Warren Marks</td>
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<td>Fort Worth, Texas</td>
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<tr>
<td>Dr. Russell Butterfield</td>
<td>University of Utah School of Medicine</td>
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