



SIDEROS trial currently enrolling Study of whether idebenone is safe and effective at delaying the loss of respiratory function

The SIDEROS clinical trial is currently recruiting boys with DMD ages 10 and older at 24 trial sites in the U.S. The study will compare the efficacy of idebenone to placebo in boys and men currently on steroids. Idebenone is an investigational drug in Phase 3 development for patients with DMD who are in respiratory decline.

To be eligible for the trial, boys must be: 10 or older; immunized against the flu and

pneumonia; have a FVC of between 35-80% predicted; and be on steroids for at least 12 months with no dose adjustments in the last 6 months (can switch between prednisone and deflazacort if it is a weight-equivalent dose).

To learn more about the trial, please visit www.clinicaltrials.gov/NCT02814019 and www.siderosdmd.com to find a trial site near you.

Santhera in the Community

Several organizations are holding educational events for families and people with DMD this fall, and we look forward to seeing you there. At these events, we will provide additional information and answer questions about the SIDEROS clinical trial and the importance of preserving respiratory muscle function in Duchenne.

Please join us at the following events:

- **Hartford, CT – Jan 20:**
Jett Foundation
- **Orange County – Jan 27:**
CureDuchenne
- **Morristown, NJ – Feb 3:**
PPMD
- **Philadelphia – Feb 24:**
Jett Foundation

Santhera is a proud sponsor of education programs from the following groups:

MDA, Parent Project Muscular Dystrophy, CureDuchenne, and Jett Foundation

Santhera was a proud sponsor of Parent Project Muscular Dystrophy's 2017 Connect Conference in Chicago, June 28-July 2.

Congratulations to the PPMD team who put on a fantastic educational opportunity for the Duchenne community!

To see Santhera CEO Thomas Meier's presentation, please visit: https://www.youtube.com/watch?v=ZEEteQY2i_E

SIDEROS Trial Sites Enrolling:

University of Alabama, Birmingham,
Dr. Troxler

Arkansas Children's Hospital
Little Rock, Dr. Stefans

Banner-University Medical Center,
Tucson, Dr. Daines

Phoenix Children's Hospital, Dr. Woodward

Loma Linda University Children's Hospital,
San Bernardino, Dr. Michelson

UC Davis
Sacramento, Dr. McDonald

UCLA
Los Angeles, Dr. Shieh

Children's Hospital of Los Angeles,
Dr. Ramos-Platt

Shriners Hospital for Children
Tampa, Dr. Couluris

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

University of Iowa
Iowa City, Dr. Mathews

University of Kansas
Kansas City, Dr. Statland

Johns Hopkins University
Baltimore, Dr. Crawford

Boston Children's Hospital, Dr. Darras

Gillette Children's Specialty Healthcare,
St Paul, Dr. Smith

Carolinas HealthCare System
Charlotte, Dr. Brooks

Cincinnati Children's Hospital
Dr. Tian

MetroHealth Medical Center
Cleveland, Dr. Prochoroff

Children's Hospital of Philadelphia
Dr. Mayer

Wesley Neurology Clinic
Memphis, Dr. Bertorini

Cook Children's Medical Center
Ft. Worth, Dr. Marks

University of Utah
Salt Lake City, Dr. Butterfield

Guidelines to Evaluate Breathing:

- Test pulmonary function at least once while ambulatory and every year after loss of ambulation
- Discuss with your doctor cough assist when cough peak flow is < 270 liters per minute or if cough becomes weaker (use cough assist machine during respiratory illnesses while ambulatory and daily and as needed after loss of ambulation)
- Discuss with your doctor nighttime ventilation as needed or when forced vital capacity (FVC) drops below 30% predicted
- Keep immunizations up-to-date (including pneumonia and annual flu)
- Treat respiratory infections promptly and aggressively
- Do NOT give supplemental oxygen without monitoring CO2

For more information about
Duchenne care guidelines, please visit:

<http://www.parentprojectmd.org/site/DocServer/provider-flyer14.pdf?docID=15003>

Lung function is important. So is feeling supported.
Visit our site for helpful information on respiratory health and to stay informed about the latest news in DMD.

Coming this December:

TakeabreathDMD.com



If you have questions for Santhera,
please contact:

patient.advocacy@santhera.com

For questions about the SIDEROS
clinical trial, please contact:

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