



MARCH 2018



Santhera launches “Take a Breath DMD” respiratory awareness campaign

The “Take a Breath DMD” campaign will underscore the importance of respiratory care for people living with DMD. The mission of this educational campaign is to help people living with DMD and their families receive information to help manage respiratory complications, including information about breathing, coughing, and pulmonary care.

On the new educational website, TakeABreathDMD.com, patients and families residing in the U.S. can find practical, in-depth information in one centralized location. Visitors to the site can also sign up to receive additional interactive tools and resources in the future, as well as stay informed about new advances.

Updated Guidelines to Evaluate Breathing:

The updated guidelines were funded and coordinated by the Centers for Disease Control and Prevention and recently published in Lancet Neurology

- Test pulmonary function at least once while ambulatory and every year after loss of ambulation
- Daily lung volume recruitment when Forced Vital Capacity (FVC) is less than 60% predicted
- Discuss with your doctor cough assist when cough peak flow is less than 270 liters per minute, FVC is less than 50% predicted, or MEP is less than 60 cm H₂O+
- Discuss with your doctor nighttime ventilation when there are signs or symptoms of sleep hypoventilation or abnormal sleep study, when FVC drops below 50% predicted, MIP <60cm H₂O or awake baseline SpO₂ 95% or pCO₂ >45mm Hg

The updated care standards state that men with Duchenne should be using most or all of these interventions by ages of 18-22

For more information about the updated Duchenne care guidelines:
http://www.parentprojectmd.org/site/PageServer?pagename=Care_guidelines

Santhera in the Community

Several organizations hold educational events for families and people with DMD, 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:

- **Richmond – Mar 24:**
Jett Foundation
- **Grand Rapids – Mar 24:**
PPMD
- **Iowa City – April 7:**
CureDuchenne
- **Denver – April 21:**
CureDuchenne
- **Charlotte – April 21:**
Jett Foundation
- **Minneapolis – April 28:**
PPMD

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

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 more than 20 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 have a decline in respiratory function.

To be eligible for the trial, boys must be: 10 or older; immunized against the flu and pneumonia; have an FVC of between 35-80% predicted; and be on steroids for at least 12 months with no dose adjustments in the past 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](http://www.clinicaltrials.gov/NCT02814019)

or <http://www.siderosdmd.com/> or contact
sideros@santhera.com

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

TakeabreathDMD.com



If you have questions for Santhera,
please contact:

patient.advocacy@santhera.com

For questions about the SIDEROS
clinical trial, please contact:

SIDEROS@santhera.com

