News and Announcements

The Diaphragm Pacing System (DPS) Trial has Enrolled its First Participant
In collaboration with Synapse Biomedical, the Muscular Dystrophy Association (MDA), and the ALS Association, NEALS is leading a study to assess the ability of the NeuRx Diaphragm Pacing System (DPS) to improve respiratory function and quality of life in people with ALS. Survival and shortness of breath will also be determined. A group of people with ALS will receive the DPS and another group of people with ALS will receive the current standard treatment. The first patient was enrolled in August 2013. Learn more about the trial here.

Statement on Future U.S. BrainStorm Trial - UPDATE
We have had inquiries regarding the BrainStorm Stem Cell Therapeutics Clinical Trial. BrainStorm is currently conducting a Phase IIa dose-escalating trial at the Hadassah Medical Center in Israel. Three U.S. sites are working with BrainStorm on a planned Phase II study in the U.S. We are continuing to follow the progress of the study in Israel and will keep the NEALS website current on all new trials and start dates. Protocol information and eligibility criteria for the BrainStorm trial were recently posted on the US Government's clinical trials website. Please note, however, that the study has not yet been approved by the FDA, and
Recruitment has not started.

**Immunosuppression Trial Phase II**

This trial will investigate the disease-modifying effect of a novel immunosuppression regimen. The primary objective of this 15-month, phase II study is to evaluate the effect of immunosuppression treatment on the rate of change on ALSFRS-R score in 30 subjects with ALS. Subjects will be assigned to receive the following study drugs: Basiliximab, Methylprednisolone, Prednisone, Tacrolimus, and Mycophenolate mofetil.

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**12th Annual NEALS Meeting**

**Highlights Clinical Trials Progress**

The 12th Annual Meeting of NEALS was the largest and most information-packed meeting yet. Over 300 clinicians, nurses, therapists, and researchers came together to take stock of the many efforts being coordinated by NEALS, and to accelerate the pace of clinical research in ALS. Read more [here](#).

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Enrolling NEALS Clinical Trials

**Phase II Trials**

**Diaphragm Pacing System (DPS) Trial**

In collaboration with Synapse Biomedical, the Muscular Dystrophy Association (MDA), and The ALS Association, NEALS is leading a study to assess the ability of the NeuRx Diaphragm Pacing System (DPS) to improve respiratory function and quality of life in people with ALS. Survival and shortness of breath will also be determined. A group of people with ALS will receive the DPS and another group of people with ALS will receive the current standard treatment. The first patient was enrolled in August 2013. Learn more [here](#).
about the trial [here](#) -- and register to attend our upcoming informational webinar [here](#).

**The Experimental Treatment of Bulbar Dysfunction in ALS**

*Nuedexa* is a drug approved by the FDA for improving emotional lability in ALS and other disorders. Based on patient experience, a NEALS trial is seeking to evaluate whether this drug improves bulbar functions such as swallowing and speaking. The trial will enroll 60 participants.

**Mexiletine in Sporadic ALS**

"A Safety and Tolerability of Mexiletine in Patients with Sporadic Amyotrophic Lateral Sclerosis" is a phase II randomized, double blind, placebo controlled trial investigating the safety and tolerability of two oral dosage levels of mexiletine, an anti-arrhythmic medication, versus placebo. The study will enroll 60 participants with sporadic ALS. Research sites Mass General, UCLA, and University of Iowa are now enrolling. Learn more about the trial and see other research sites [here](#).

**Phase IIa Double-Blind, Placebo-Controlled Study to Evaluate the Safety of Oral Fingolimod in Patients With Amyotrophic Lateral Sclerosis**

The purpose of the research study is to determine whether Fingolimod, a drug that is believed to reduce the number of lymphocytes by blocking them from leaving the lymph nodes, is safe and tolerable for patients with (ALS). Participants will be treated for a total of four weeks (approximately 28 days). For more information, contact Study Coordinator [Christina Dheel](#).

**Other Interventional Trials**

**Resistance and Endurance Exercise in ALS Trial**

Researchers at Johns Hopkins University, Massachusetts General Hospital, Carolinas Medical Center, and Washington University in St. Louis, Missouri are currently investigating 3 forms of exercise in people with ALS: weightlifting (resistance exercise), stationary bicycling (endurance exercise), and range of motion exercise (the current "standard of care" for ALS patients). The researchers will determine whether one type of exercise is better tolerated and/or safer than another form of exercise. They will also collect information about how the bodies of ALS participants respond to exercise. The trial will enroll 60 participants across the country. Study participants will take part in at-home regimen with the guidance of a study physical therapist during visits to the study center.
Familial ALS
Arimoclomol in SOD1 Positive Familial ALS
Arimoclomol is under investigation for safety and efficacy in patients with SOD1 positive familial ALS as part of a seamless, adaptive, randomized, double-blind, placebo-controlled phase II/III trial. Participants will be randomized 1:1 to receive either placebo or arimoclomol at a dose of 200 mg t.i.d. The trial is currently enrolling 80 participants at two sites: the University of Miami and Massachusetts General Hospital. The trial consists of both visits at the study site and visits in the participant's home.

Observational Studies
Longitudinal Study Comparing the Responsiveness of Outcomes Measures in ALS Trials
A new strength measurement device called Accurate Test of Limb Isometric Strength (ATLIS) was developed to precisely and conveniently measure static limb strength in patients with ALS. We will compare ATLIS data with data from two commonly used ALS outcomes measures, the ALS Functional Rating Scale-Revised (ALSFRS-R) and slow vital capacity (SVC) in a prospective, longitudinal study. All three outcomes measures will be performed on 100 subjects collected preferably at bi-monthly clinic visits during the study period.

SOD1 Natural History Study
Dr. Miller and colleagues at Washington University in collaboration with Dr. Cudkowicz at Massachusetts General Hospital, and Isis Pharmaceuticals are continuing to develop a novel therapy (antisense oligonucleotides) for ALS caused by mutations in the SOD1 gene. An important part of planning for the next clinical trial for SOD1 ALS patients is to gather clinical information about ALS in this group of patients. If you or someone you know has ALS and a mutation in the SOD1 gene, please contact Jennifer Jockel-Balsarotti at Washington University for details regarding how to participate in the study.

A Multicenter Study for the Discovery and Validation of Biomarkers in ALS
Biomarkers are markers of disease or responses to therapy that might be used to diagnose ALS, monitor disease progression, or monitor treatment effectiveness. To learn more about ALS, researchers are collecting blood and cerebrospinal fluid (CSF) from patients with ALS. Samples will be collected approximately every 4 months. This clinical study is enrolling at 6 NEALS sites: Boston MA, Worcester
MA, Pittsburgh PA, Atlanta GA, Jacksonville FL, and Phoenix AZ.

The archived Biomarkers Webinar discusses the importance of biomarkers research into finding a cure for ALS. Through comparison of these specimens, the researchers hope to learn more about the underlying cause of ALS, as well as find unique biological markers, which could be used to develop new therapies.

Enrollment Closures

The following studies have closed to enrollment:

**Phase II High Fat/High Calorie Trial**  
For information about this trial, click [here](#).

**GM604 Phase 2A Randomized Double-blind Placebo Controlled Pilot Trial in ALS**  
For information about this trial, click [here](#).

**A Phase IIb, Multi-National, Double-Blind, Randomized, Placebo-Controlled Study to Evaluate the Safety, Tolerability and Efficacy of CK-2017357 in Patients With ALS (BENEFIT-ALS)**  
For information about this trial, click [here](#).

NEALS Researcher Featured in CNN Series on ALS

Suzanne Malveaux, an Emmy award-winning journalist and host of CNN's *Around the World* and *CNN Newsroom*, produced a three-part series in 2013 about the current fight against ALS. The series, entitled "Fighting ALS," chronicled her mother's journey from thriving family matriarch to ALS patient, and also features the stories of former NFL player Steve Gleason and Life Fitness Founder Augie Nieto. Dr. Jeffrey Rothstein, Packard Center founder and director, is featured throughout the series providing the latest medical and research insights.

A companion website, [www.malveauxmission.org](http://www.malveauxmission.org), was established to accompany the series. The site was co-founded by Malveaux and the Packard Center to share the Malveaux
story, educate people about the disease, share the latest treatments, technologies and research, and provide opportunities for people to make a gift to aid in research efforts.

Since her mother's diagnosis, Malveaux has worked to learn everything she can about the causes, treatments and potential cures for ALS. See more at [www.cnn.com](http://www.cnn.com).

The ALS Association/NEALS
PALS Webinar Series

NEALS offers live, online seminars (or "webinars") for people with ALS and other motor neuron diseases. Experts explain the scientific rational and design of current ALS clinical research. Our webinars also offer participants the opportunity to ask questions directly to the experts running trials. Below is the information for our next webinar:

**Diaphragm Pacing System (DPS) in Participants with ALS - Clinical Trial: An Informational NEALS Webinar**

Principal Investigators Dr. Jonathan Katz and Dr. Jeremy Shefner will explain the rationale and background of the DPS study, describe the patient population being recruited, and discuss what is expected of those who enroll. Please join us for this very informative session on Thursday, January 16 at 3:00pm. [Register here.](http://register.)

NEALS also has a [library](http://library) of previously-recorded webinars available to view at any time.

Quick Links

[Recruiting Trials](http://recruiting)
[Resources for Patients and Families](http://resources)
[Clinical Research Blog](http://clinicalresearch)
[Clinical Trial Glossary](http://clinicaltrial)
[Online NEALS Resources](http://onlineneals)
Our Clinical Trial Liaison service is available to help people with motor neuron disease and their caregivers navigate the NEALS website and answer questions about clinical research.

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Stay Connected

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