

Clinical Trials

The MDA/ALS Center of Hope at Drexel University College of Medicine is involved with several sponsored clinical trials. They include both drug studies and investigational devices.

Arimoclomol

This study will test the safety and tolerability of arimoclomol along with its effects on ALS. Arimoclomol causes your body to make more heat shock proteins, which are normally increased in the body as part of its response to a variety of stressful events. Studies in animals have shown that these proteins may play an important role in helping to protect nerves from damage.

Zenvia

The safety, tolerability and efficacy of Zenvia, a combination drug consisting of dextromethorphan and quinidine for the treatment of patients with pseudobulbar affect (PBA), will be evaluated in this study. PBA is a condition characterized by frequent episodes of abnormal laughing or crying (not matching a person's emotions). Previous studies have shown this drug was successful in reducing PBA episodes.

Ceftriaxone

This study will ascertain whether chronic intravenous treatment with ceftriaxone prolongs survival with ALS. Ceftriaxone may increase the level of a protein that decreases glutamate levels near nerves. Researchers think that increased levels of glutamate may be related to motor neuron death. Participation in this study requires placement of a central venous catheter.

KNS-760704

This trial will study the safety and efficacy of drug KNS-760-704. This is a "neuroprotective" drug that protects nerve cells from being destroyed and was developed from a known drug that has significant side-effects at concentrations needed for motor neuron protection. Knopp Neurosciences Inc. has developed KNS-760704 which is similar to the original drug, but has greater penetration into the nervous system at lower concentrations thereby resulting in fewer side effects. This allows KNS-760704 to potentially serve as a safer treatment for ALS.

NeuRx Motor-Point Stimulation

This trial will assess the safety and feasibility of the NeuRx Diaphragm Pacing Stimulation System (DPS). It has been shown to improve diaphragm function in ALS patients with Forced Vital Capacity (FVC) above 50% predicted. In this study we will assess whether it is also useful for people with lower FVC, and even those people who cannot perform the test but have diaphragm movement under x-ray fluoroscopy. Participation in this study will require surgical implantation of the DPS.



Additional Studies

There is still much to learn about ALS. As such, we have several ongoing studies evaluating various aspects of this disease.

Quality of Life (QoL) Questionnaire

The current ALS QoL Questionnaire is a 59-item survey used nationally to evaluate the QoL in ALS patients. Our goal is to develop a shorter, more easily administered 46-item version to gain an overall better understanding of the QoL in ALS patients. We hope this will also be useful in ALS clinical management and in other studies.

Substrate (sugar/fat/protein) Utilization

This study investigates the substrate utilization, using a metabolic cart to measure the calories you need and whether you are using sugar, fat or protein calories. Our goal is to understand shifts in substrate utilization through the progression of ALS, helping patients attain a healthier lifestyle and QoL. This study will also elucidate the influence of improved nutrition via PEG tube.

Work of Breathing

We are evaluating how many calories are used by ALS patients while at rest, while placed on NIPPV and while breathing against resistance. This data will aid in calculating the work of breathing and effects of NIPPV on the work of breathing. In addition, this study will help to resolve mismatches between caloric intake and nutritional needs.

GI Dysmotility

Studies have shown delayed gastric emptying may be associated with ALS; possibly attributable

to GI complaints. This study will quantify GI transit time in a large cohort of patients and controls in an effort to gain further insight into the pathophysiology of GI dysmotility in ALS.

sPLA-2

The process of inflammation may have a critical role in the development and progression of many neurodegenerative disorders like ALS. The enzyme sPLA-2 is thought to be either directly or indirectly involved in this process. The purpose of this study is to measure the activity levels of sPLA-2 in patients with ALS and controls to determine if this protein is altered in ALS. Our hope is that this study will help to understand inflammation in ALS and identify targets for treatment.

Tissue Donation

There are animal models of ALS, but understanding the human disease relies on research performed on human tissue. We are currently collecting blood, urine, CSF and other tissue samples. Appropriate information regarding diagnosis, disease course and patient demographics are not readily available to maximize these precious materials. We would like to increase availability of human tissue for research with pertinent corresponding clinical information.

ALS & Technology

Technological developments are giving people with disabilities alternate communication and control options. The following are projects aimed at evaluating the effectiveness of some of these new means of communication and control for ALS patients.

EEG-Based Brain-Computer Interface Project

By recording EEG (brain wave) signals from the scalp, the Brain-Computer Interface (BCI) allows people to make selections from the computer screen. The study is intended to evaluate both the complexity of the system and the degree to which each participant will be able to communicate. Trials will consist of asking the subject to follow a series of simple instructions and to complete certain tasks while using the BCI.

Cyberlink Control System

We are interested in using the Cyberlink Control System as a hands free means to access a computer for people with ALS. The goal of this project is to determine whether this device is a practical and realistic means of communication using only the facial muscles, brain waves and eye movements. The effectiveness of the Cyberlink Control System will be compared to that of the standard manual letter board.



If you have any further questions regarding these studies, feel free to ask any of the staff at the MDA/ALS Center of Hope. We can be reached at:

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**Thank you for your
time!**



**Clinical Trials,
Studies &
Upcoming
Technology**