

# The ALS Hope Foundation Supports the Clinical Research Infrastructure at the MDA/ALS Center of Hope



Delivering excellent care to people living with ALS is of primary importance to the clinic team at the MDA/ALS Center of Hope. However, that is not all that they do. An active clinical research program is an integral part of the activities at the Center of Hope. Without research, there would be no advances in our ability to offer the best care for people with ALS or progress in the search for a cure. The ALS Center of Hope believes in using the treatments and technology that are available now for optimizing the care and quality of life in people who are living with ALS.

Research includes clinical trials, which study the effects of an experimental compound on disease progression or symptoms, as well as clinical studies, which aim to better understand the disease and its effects. Clinical research at the Center of Hope complements our basic research program. We are working toward the day when we can bring our basic research discoveries directly to the clinic for trial.

Funding for clinical research, even clinical trials, is not always available. Sometimes the funding that is available is not enough to fully carry out the research. The ALS Hope Foundation fills in the funding gaps, so that clinical research never stops at the Center of Hope. Some of the research made possible by the ALS Hope Foundation is outlined below:

## Clinical Trials: Hope is on the Horizon

**Co-enzyme Q10:** Co-enzyme Q10 (Co-Q) acts as an antioxidant and protects mitochondria (the energy producer of nerve cells) from damage. Treatment with Co-Q It extends life in ALS mice. The Center of Hope is now part of a high dose Phase III trial of Co-Q. Funded by NIH.

**Arimoclomol:** Arimoclomol increases production of a type of protein, known as a “heat shock protein,” that helps to protect damaged or stressed nerve cells. The center recently completed a Phase II trial of arimoclomol in ALS patients examining the side effects of the medication and will be starting the Phase IIb trial in the summer to determine efficacy. Funded by CytRx.

**Ceftriaxone:** Ceftriaxone is best known as an antibiotic that treats Lyme disease but has also shown efficacy in models of ALS and nerve degeneration by blocking glutamate toxicity. A small trial of Ceftriaxone was recently initiated to assess any side-effects that may appear with long term administration in ALS patients. Once this is completed, the Center of Hope will participate in a larger trial to look at the effect of Ceftriaxone on survival in ALS. Funded by NIH.

**Minocycline:** Minocycline has the potential to be a therapeutic in ALS by two mechanisms: as an anti-inflammatory agent and as a neuroprotectant blocking programmed cell death of nerve cells. A large Phase III trial was recently completed at the Center of Hope and results will be released soon. Funded by NIH.

**Neurodex:** Neurodex is a combination of Dextromethorphan (DM) and Quinidine (Q). This drug is directed at the emotional lability (laughing and crying too easily) experienced by some people living with ALS. Funded by Avanir Pharmaceuticals.

## Clinical Studies Relating to Respiratory Management

**NIPPV and Nutrition in ALS:** This study is examining how to optimize both NIPPV (noninvasive positive pressure ventilation) and nutrition evaluation for PEG placement in people living with ALS. The study is a large multi-center effort with the long-term goals of determining the best time for each of these treatments to begin. The results will also tell us whether the effects of optimizing nutrition and respiratory support are additive on survival and quality of life. Funded by NIH and supplemented by ALS Hope Foundation.

**Work of Breathing:** This study is examining the calories that people burn on and off of the NIPPV machine. This will allow us to calculate the differences in calorie needs and savings by ventilation. The information will be helpful in optimizing nutrition. Funded by the ALS Hope Foundation.

## Other Clinical Research Programs Funded Solely by the ALS Hope Foundation

**Nutritional Needs In ALS:** This project is a collaboration with the Pulmonary department and examines what types of foodstuffs (carbohydrate, fats, proteins) people with ALS burn at various times during the disease.

**GI Motility in ALS:** In many people living with ALS, constipation is a major problem. This may be due to medications. Alternatively, there may be involvement of nerve cells in the GI tract causing slowing of the GI motility. In collaboration with Dr. Michael Sherman, we are examining the speed at which food travels down the GI Tract in ALS using a technique to measure hydrogen production with a measured bolus of food.

**Inflammation in ALS:** This project is examining an enzyme, soluble phospholipase A2 (sPLA2), which is one of the first mediators of the inflammatory reaction. We have found an increase in the levels of sPLA2 in urine from people with ALS and in the mouse model. Further studies on sPLA2 in humans are being conducted, and a pre-clinical trial of an sPLA2 inhibitor is underway in the animal model.

**Assistive Technology in ALS:** The center has an active interest in developing assistive technology for use by people living with ALS. This program is part of the Kevin O'Donnell Independent Living Initiative and includes use of the brain-computer interface (BCI) in ALS, use of the Cyberlink® to access computers for speech, and other environmental controls. In addition we have built two fully handicapped accessible hospital rooms at Hahnemann Hospital that are outfitted to allow people with ALS full environmental control in a home-like environment. These rooms are the "The Sanctuary" in memory of Bo Leboutillier and the "Dr. Bruce Rosenberg Suite of Hope" made possible by Dr. Rosenberg and his family.

**Tissue Repository:** The Center of Hope now houses a Tissue Bank for blood, urine, and spinal fluid specimens from people living with ALS as well as an Autopsy Tissue Bank. These tissues are critical for research into the cause of ALS.