

# The ALS Hope Foundation Funds Basic Research at the Drexel University ALS Research Laboratory



The research lab is actively pursuing ways to better understand ALS and to treat and modify disease progression. The laboratory is small, so that collaborations with other researchers and with industry are very important. Most of the work uses the SOD1 mouse model of ALS, which has been genetically engineered to carry the mutated human gene for SOD1 (superoxide dismutase). Mutant SOD1 is responsible for 20% of the cases of hereditary ALS. These transgenic mice develop weakness and pathology that is very similar to people with ALS, providing an excellent model with which to study the disease. All supporters of the ALS Hope Foundation are invited to visit the labs and get a first hand glimpse of the research efforts. Ongoing research projects in the laboratory include:

## **Developing a cell culture model of ALS**

Grant title: “*In Vitro Studies of Cellular Interactions and Trophic Factor Receptor Function in a Cell Culture Model of ALS.*” (Co-funded by the Murray Abrams Memorial Research Fund).

This project uses the highly technical advantage of studying neurons in a culture dish to understand what is going wrong inside the individual motor neurons and to test potential therapeutics that can keep motor neurons healthy.

## **Examining the role of inflammation in ALS**

Grant title: “*A Novel Neuron Survival-Promoting sPLA2 Inhibitor as Treatment for ALS.*” (Co-funded by ALSA.)

Inflammatory signals have been discovered in the spinal cord of people with ALS and in the animal model of ALS. Previous trials with anti-inflammatory drugs have not helped, but they also may not have been the right kind of drug. We are collaborating with Tim Cunningham of the Neurobiology Dept. at Drexel, who has developed a potent anti-inflammatory agent that shows effectiveness in other neurological conditions such as stroke and multiple sclerosis. We want to test this drug on our mouse model of ALS, and eventually, in our PALS.

## **Transplanting stem cells to replace damaged motor neurons**

This is highly speculative and early work, but we are developing plans with a biotechnology company in California to figure out how to get stem cells to grow into motor neurons and reconnect with muscles in the mouse model of ALS.

## **Searching for genetic modifiers of ALS**

Grant title: “*Genetic Analysis of ALS in Inbred Transgenic Mice.*” (Co-funded by MDA).

This has been our main project for several years. The onset and progression of ALS is very different in different people, even if they are carrying the same mutation that causes ALS. This suggests to us that other genes may affect how (or whether) one gets ALS. The same is found in mice. We are mapping the genome of the mouse to look for the “good” genes that can slow down or stop the degeneration.

## **Maintenance of a Colony of Transgenic Mice for Amyotrophic Lateral Sclerosis Research**

Probably one of the simplest but most important things we do is to maintain this colony of mice expressing mutant SOD1, so that researchers working on other diseases can easily switch to do pilot projects on ALS. In addition, we have bred a unique mouse with very few copies of the mutant SOD1 gene, making it a model for onset later in life and slow progression of disease.