

## **NNPDF-Funded Research Grant # 46**

**TITLE: Additional contribution to LSD Research Consortium for research into issues of common interest to all LSD support groups**

**(See also Grant # 40)**

**PROJECT INVESTIGATOR: Multiple**

**PERIOD: 4/1/2007 - 3/31/2008**

### **PROJECT DESCRIPTION**

The LSD Research Consortium (LSDRC) is a collaborative research-funding group, comprised of LSD patient support groups and private family research foundations. The LSDRC has entered into an agreement with the National Institute of Neurological Disorders and Stroke (NINDS) for the purpose of a jointly sponsored program to provide financial, scientific and administrative support towards preclinical or translational research specifically addressing the neurological aspects of lysosomal storage disorders (LSD).

The goal of this mechanism is to stimulate interest in and to solicit applications for lysosomal storage disorder research focused on improving CNS treatment outcomes, enhancing the effectiveness of delivery and targeting of cells, enzymes, drugs and genes into the brain. This funding opportunity specifically encourages the transition from basic studies in LSDs to translational research for improved delivery of therapeutic cells, proteins, genes and small molecules across the blood-brain barrier.

### **Final STATUS REPORT**

**Dated 5/31/2008**

Three researchers were funded last year for proposals that target novel ways of conquering the blood-brain barrier (BBB). Of those three Dr Eain Cornford was awarded a five year NIH grant for his research "Trojan Horse Gene Therapy of Inclusion Body Disease" in which it was proposed to use recently developed immunoliposome BBB delivery systems to deliver a normal gene therapeutically through the BBB via intravenous administration.

In early 2008 the LSDRC review committee received the grants from eleven researchers who did not receive NIH funding. In May 2008 the committee awarded \$92,000 to four of those researchers:

Dr. Mark Haskins, University of Pennsylvania Research title: "Gene therapy for alpha-mannosidosis

Dr. Alessandra d'Azzo, St. Jude Children's Research Hospital Research title: "GM1 as apoptotic signal in neurodegenerative GM1 gangliosidosis"

Dr. Douglas Martin, Auburn University Research title: "Enzyme replacement therapy and role of unfolded proteins in response to feline GM1Ga"

Dr. Bruce Bunnell, Tulane University Research title: "Mesenchymal stem cell therapy for Krabbe disease"

These are the last grants to be awarded by the LSDRC since the NIH Grant has come to the end of its three years.

**PUBLICATIONS:**

No Publications on this Work To Date