

## **LSDRC 2008 Grants Funded**

In 2004 the National MPS Society joined with other lysosomal storage disease (LSD) support groups to form the LSDRC (lysosomal storage disease research consortium). Our research philosophy that the treatment or cure developed for one disease can benefit the other MPS diseases also applies to the general category of LSDs. The LSDRC focus is on funding research to find or improve central nervous system (CNS) treatments for LSDs. The LSDRC entered an agreement with the National Institute of Neurological Disorders and Stroke (NINDS) for the purpose of a jointly sponsored program, CNS Therapy Development for Lysosomal Storage Disorders.

In July 2004 the NINDS announced the grant offering, CNS Therapy Development for Lysosomal Storage Disorders, NIH PAS-04-120. This announcement 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. In 2006 the LSDRC funded seven researchers who applied for the NIH PAS-04-120 but who did not receive NIH funding. Three additional grants were funded in 2006.

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?"

## **LSDRC 2007 Grants funded**

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In late 2006 the LSDRC review committee received the grants from six researchers who did not receive NIH funding. In January 2007 the committee awarded \$79,400 to three of the six researchers:

Dr. Eain Cornford, UCLA School of Medicine  
Research title: Trojan horse gene therapy of inclusion body disease

N. Matthew Ellinwood, Iowa State University  
Research title: Pathogenesis of murine MPS IIIB

Xiaogyang Qi, Children's Hospital Research Foundation  
Research title: The physiological importance of saponin C's fusogenicity

## **LSDRC 2006 Grants Funded**

The LSDRC funded seven researchers in 2006 who applied for the NIH PAS-04-120 but who did not receive NIH funding. The total amount of grants awarded is \$233,000 from the available \$310,000. The decisions for funding from the LSDRC were made by an independent review board of scientists and clinicians who serve on the advisory boards of the participating LSDRC groups. These grants will allow the researchers to expand their work so they can reapply for NIH funding.

Eain M. Cornford, PhD, Professor of Neurology  
David Geffen School of Medicine at UCLA, Veterans Affairs Greater Los Angeles Healthcare System  
"Gene delivery across the blood-brain barrier in Lafora knockout mice"

Philip E. Dawson, PhD, Assoc. Professor, Cell Biology  
Scripps Research Institute, LaJolla, CA  
"Potential of chemical chaperones and thioester reactive small molecules as potential therapeutic approaches in the treatment of infantile Batten disease"

Kostantin Dobrenis, PhD, Asst. Professor, Neuroscience, Co-Director, Center for Disorders of Lysosomal Metabolism  
Albert Einstein College of Medicine  
"GM2 Gangliosidosis Therapy Using Neuronotropic Enzyme"

Angela Gritti, PhD,  
Institute for Stem Cell Research (SCRI), Milan, Italy  
"Neural Stem Cell Based Therapy for GM2 Gangliosidosis"

Synthia H. Mellon, PhD, Prof, Ob/Gyn. Reproductive Sciences  
Univ. of California at San Francisco  
"Neurosteroid Therapy for Lysosomal Storage Disorders"

Thomas N Seyfried, PhD, Prof. of Biology  
Boston College  
"Evaluate MJ-DGJ as a substrate reduction therapy, neural stem cells (NSCs), as a cross-correctional therapy, and caloric restriction (CR) as an anti-inflammatory therapy for ganglioside storage diseases"

Brian W. Soper, PhD, Research Staff Scientist  
The Jackson Laboratory  
"MPS VII CNS Gene Therapy Using Neuronal Stem Cells"