Abstract

In 2005-2006, the National Tay-Sachs & Allied Diseases Association (NTSAD) focused its research funding on the Lysosomal Storage Disease Research Consortium (LSDRC) grants initiative. Co-founded by NTSAD and the National MPS Society in 2005, LSDRC was a collaborative research-funding group composed of patient advocacy groups representing lysosomal storage diseases (LSDs). LSDRC entered into an agreement with the National Institute of Neurological Disorders and Stroke (NINDS) to establish a three-year, jointly sponsored program to provide financial, scientific, and administrative support to preclinical or translational research specifically addressing the neurological aspects of LSDs.

Mission

LSDRC’s goal was to stimulate interest in and to solicit applications for LSD research focused on improving central nervous system 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.

The Review Committee prioritized applications needing additional targeted research that would improve the principal investigator’s opportunity of receiving National Institutes of Health (NIH) funding. Thirteen investigators received a total of $360,000 in LSDRC grants over the period 2006-2008. Of these investigators, six received NIH follow-on grants which is a higher than average success rate.

**Patent Engagement**

The LSDRC patient advocacy group partners were as follows:

- National MPS Society, Inc.
- National Tay-Sachs & Allied Diseases Association, Inc.
- The Canadian Society for Mucopolysaccharide & Related Diseases, Inc.
- The Sanfilippo Syndrome Medical Research Foundation, Inc.
- Hunter’s Hope Foundation
- National Niemann-Pick Disease Foundation

**Links/Social Media Feed**


**Points of Contact**

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**Sponsors & Partners**

Hunter’s Hope Foundation
National Institute of Neurological Disorders and
Stroke (NINDS)
National MPS Society
National Niemann-Pick Disease Foundation
National Tay-Sachs & Allied Diseases Association
The Canadian Society for Mucopolysaccharide & Related Diseases, Inc.
The Sanfilippo Syndrome Medical Research Foundation, Inc.

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