



2nd Research Grant Program (2009-2011) Request for Proposals

The Pediatric Neurotransmitter Disease (PND) Association is pleased to announce the availability of up to \$200,000 in grant awards for research involving the detection, diagnosis, and/or treatment of diseases related to neurotransmitter metabolism.

The grants will be divided among one or two year grants with a preliminary allocation among the diseases currently represented by the PND Association as follows:

Succinic Semialdehyde Dehydrogenase Deficiency(SSADH)	\$95,000
Tyrosine Hydroxylase Deficiency (TH)	\$55,000
Aromatic L Amino Acid Decarboxylase Deficiency (AADC)	\$33,000
GTP I Cyclohydrolase Deficiency (GTPCH)	\$ 0
Sepiapterin Reductase Deficiency (SR)	\$ 0
Non disease specific PND research	\$17,000

A summary of significant dates for this grant program appear below:

Letter of Intent Deadline	July 1, 2009
Application Deadline	October 1, 2009
Grants to be Announced	March 1, 2010

Background

Pediatric neurotransmitter disease is an umbrella term for a group of rare genetic disorders that affect the synthesis, metabolism and catabolism of neurotransmitters. Individually, these disorders may be considered rare, but cumulatively the incidence may be higher than observed due to under diagnosis. Because many clinical and biochemical abnormalities of PND's remain poorly understood, the PND Association is seeking proposals for research that will shed light on any aspect of these diseases.

Types of Proposals Sought

Basic Science and Research

- Understand the molecular and biochemical pathogenesis of PND's and response to treatment.
- Develop new approaches to neurotransmitter biology using cellular and animal systems.
- Develop animal models of PND mutations.

Clinical Research

General

- Understand the natural history of specific PND's
- Understand epidemiology of PND's (e.g. frequency, ethnicity, disparity)
- Establish common protocol for the evaluation of patients with PND's
- Understand pharmacological effects on PND's
- Develop clinical-biochemical correlations
- Create an international longitudinal database on PND's
- Develop improved genetic counseling, as well as research required to provide accurate information about prognosis and recurrence risk

Diagnosis

- Develop common definitions leading to diagnostic criteria and standards for all diagnostic testing, including imaging
- Develop least invasive methods of diagnosis
- Develop rapid and accurate detection of DNA mutations

Treatment

- Develop and test strategies leading to extended life span, improved quality of life, and cure for PND's
- Support clinical trials using appropriate, objective outcome criteria
- Understand the response to, and side effects of therapeutic strategies (i.e. pharmacological treatment, diet etc)

Application Process

Letters of Intent must be received by the PND Association by July 1, 2009.

Initial letters of intent will be evaluated by an independent Grant Review Committee (GRC) chaired by Dr. Darryl De Vivo, MD of Columbia University Medical Center. The GRC will evaluate all letters of intent and determine which ones will be selected for further consideration.

If selected, a full grant application must be received by the PND Association by October 1, 2009. The GRC will review and rank all grant applications and make its recommendations to the PND Board of Directors for final funding determinations.

Letters of Intent must include the following:

1. Objective
2. Which specific PND disease the research relates to and/or relevance to PND's
3. Hypothesis or hypotheses to be tested.
4. Approximate amount of funding requested
5. Approximate timeline for research project

Invited applications should include the following;

- Description of the proposed project (no longer than 5-10) pages.
- Curriculum Vitae for the principal investigator and other researchers.
- Financial accounting of how funds will be spent.
- Start date of the project and project timeline.
- A letter from an official of the sponsoring institution endorsing the project along with a commitment that adequate facilities will be available to conduct the research proposed.

Please note that we do not provide any indirect costs to the institution. Research and clinical protocols must conform to legal and regulatory standards established by the FDA and other governmental agencies.

The PND Association reserves the right to cancel grants for cause at any time and require the return of funds.

APPLICATIONS AND QUESTIONS SHOULD BE DIRECTED TO:

PND Association
c/o Carolyn Hoffman
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