NBIA Disorders Association
Announces
Research Grants
for
Pantothenate Kinase-Associated Neurodegeneration (PKAN)

Submission Deadline For Applications – September 15, 2014

About NBIA Disorders Association

The NBIA Disorders Association, formerly known as Hallervorden-Spatz Syndrome Association, (HSSA) was originally founded in 1996 by President, Patricia Wood. The goals of the association are to raise funds to support research pertinent to Neurodegeneration with Brain Iron Accumulation (NBIA); to provide emotional support to those afflicted with NBIA and their families; and to raise public awareness of NBIA. If you would like further information, please email at pwood@NBIAdisorders.org, or telephone at (619) 588-2315.

The NBIA Disorders Association is now accepting applications for one-year grants for clinical and translational research studies related to the early detection, diagnosis, or treatment of patients with Pantothenate Kinase-Associated Neurodegeneration (PKAN), the most common of the NBIA disorders. NBIA is a group of rare, genetic, neurological disorders characterized by the accumulation of iron deposits in the brain and progressive degeneration of the nervous system.

Between 35 and 50 percent of the NBIA population has PKAN. It is caused by mutations in the \textit{PANK2} gene. This gene provides the instruction for making an enzyme called pantothenate kinase. Researchers are investigating how this missing enzyme damages nerve cells in the brain and causes iron to build up.

There are two forms of PKAN: classic and atypical, although some people have symptoms that place them in between the two categories. At the present time, symptoms for the disorder may be treated, but there is no cure.

Research Objectives

The purpose of the NBIA Disorders Association Research Grant Program is to encourage meritorious research studies designed to improve the diagnosis or treatment of NBIA. The research can be conducted in the United States, countries of the European Union, Canada, Australia, New Zealand, Brazil, Argentina, Chile, South Africa, Japan, or Israel, and in other countries where adequate supervision of grant administration is possible.

Evaluation of proposals will follow NIH guidelines and include careful consideration of experimental or protocol design, objectivity or relevance of parameters measured, and statistical analysis plan.

Our research priorities reflect our goal to find cures for NBIA disorders and the understanding that strategic work in basic, translational and clinical research will be required to reach this goal. We
strive to accelerate the pace of discoveries that lead to new therapeutics by supporting pilot and high-risk projects. The projects we support are expected to generate essential resources for the scientific community, advance knowledge about NBIA disease processes, and produce preliminary data to enable national and international funding to carry the work forward.

Our research priorities for the 2014 NBIA-PKAN grant cycle are:

Support studies to:

- Develop rational therapeutics for PKAN
- Discover and validate biomarkers of disease in PKAN
- Delineate the molecular cascades that lead to early cellular changes in PKAN
- Advance understanding of why the globus pallidus is selectively vulnerable to defective PANK2
- Propose novel ideas to advance the understanding or treatment of PKAN

We are not considering applications for mouse models, stem cell studies or natural history studies this grant cycle.

A grant not to exceed $45,000 for one year (with exceptions if warranted for multi-year funding) will be awarded by the NBIA Disorders Association. Research resources developed under this funding initiative must be shared per National Institutes of Health guidelines. These guidelines can be found at [http://grants.nih.gov/grants/policy/model_organism](http://grants.nih.gov/grants/policy/model_organism).

**RFP Deadlines**

- Applications due by September 15, 2014
- Award announcements by November 15, 2014
- Funding to begin December 15, 2014

**Further Information**

- The number of research grants to be awarded will depend upon the availability of funds and the quality of applications.
- All applications will be reviewed for scientific merit.
- Indirect costs are allowed up to a maximum of 15% of the grant request.
- PI salary of up to $10,000 per year will be allowed in addition to other lab personnel salaries.
- If study involves humans, copies of the Informed Consent and the Institutional Review Board (Ethics Committee) approvals will be required from each site involved in the study, before payment.
- Clinical drug trials must meet the requirements established by the Food and Drug Administration (FDA).
- If study involves human gene therapy, a copy of the NIH Recombinant DNA Advisory Committee (RAC) review or waiver of review is required.
- All publications resulting from research funded by our organization must acknowledge NBIADA and should be provided to us when published.
Grant recipients will receive funding in three stages: 1/3 at start of project; 1/3 at six months after an interim scientific and financial report is submitted and approved; final payment at the conclusion of the grant after final scientific and financial reports are submitted and approved.

The PI must submit a final scientific & financial report within three months of the end of the Grant Period unless an extension is granted. Those not in compliance with this requirement may forfeit their final payment. Grant period starts with receipt of the first payment.

The organization must be informed immediately of any change to the status of the PI or other important contributors to the project which might affect their ability to comply with grant conditions.

If the interim or final reports reflect serious deviations from the grant objectives and cannot be explained adequately, forfeiture of funds due or stoppage of the grant may be warranted.

All research papers that have been accepted for publication in a peer-reviewed journal, and are supported in whole or in part by the Grant, must be made available at NIH PubMed Central as soon as possible, and in any event within six months of publication, in line with an Open Access policy.

Application Procedure – Deadline September 15, 2014

- Application must be submitted with all of the following documents to be considered:
- Narrative of proposal suitable for peer review and statement why the funds from NBIA Disorders Association would be essential to the project. (One page)
- Specific goals: State the specific aims of the proposed research. (One Page)
- Background and significance. (One page)
- Methods. (Five pages)
- Facilities available and Budget. All amounts must be in U.S. dollars. (One page)
- Provide statement that there is no funding overlap in monies requested from NBIADA, or explain in detail if such overlap exists and why funding is still essential from our organization.
- Provide explicit explanation of future plans to secure funding to take project forward.
- Research resources developed under this funding initiative must be shared per National Institutes of Health guidelines. Please provide a paragraph describing a sharing plan.
- Up-to-date Curriculum vitae and Bibliography.
- Attach copy of patients Informed Consent approved by Institution. (Required before payment)
- Attach copy of Institutional Review Board (Ethics Committee) approval. (Required before payment)
- If a clinical study involves human gene therapy, please remit a copy of the NIH Recombinant DNA Advisory Committee (RAC) review, or waiver of review. (Required before payment)
- If this is a multi-site study, Informed consent document and IRB approval is required from each.
- Open application (pages 4-6) on your computer in Microsoft Word, do a “Save As” with the filename “2014 NBIA-PKAN Grant Application-your last name” and convert document with all required information to a pdf file before sending. Submission of all pertinent documents must be a single pdf document attached to an email addressed to pwood@NBIAdisorders.org.
## GRANT APPLICATION
2014 NBIA-PKAN Grant Cycle

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<tr>
<th>Name of Researcher</th>
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<td>Institution(s) Where Research Will Be Conducted.</td>
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<td>Title of Project</td>
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<td>Funding Amount Requested</td>
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<td>Will Research Involve Human Subjects?</td>
<td>[ ] YES [ ] NO</td>
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<td>How Did You Hear About This RFP?</td>
<td>__ U.S. Postal Service Notice __ E-mail Subscription Service __ NBIADA email __ Website Posting __ Other</td>
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<td>Applicant Signature (required) e-signature acceptable</td>
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Please provide all supporting documentation required with your application and submit by September 15, 2014.

Document must be a single pdf and emailed to pwood@NBIAdisorders.org
**Biographical Sketch**
Not to exceed 2 pages

Applicant may use this form or the NIH Biosketch form. Please modify the form to include, when applicable, the following:

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<th>Name</th>
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<th>Education/Training</th>
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<td>Begin with baccalaureate or other initial professional education and include postdoctoral training.</td>
<td>Institution and Location</td>
<td>Degree</td>
<td>Year(s)</td>
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<td>Concluding with present position, list in chronological order, previous employment, experience, and honors. Include present membership on any advisory committee.</td>
<td>Institution and Location</td>
<td>Degree</td>
<td>Year(s)</td>
<td>Field of Study</td>
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| Honors and Awards | |
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BIBLIOGRAPHY
Not to exceed 2 pages

Publications: List in chronological order, the titles, all authors, and complete references to all publications in the last three years and to representative earlier publications pertinent to this application. If the list of publications exceeds two pages, select the most pertinent publications.