

#### NBIA Disorders Association

#### Announces

#### Research Grant

*for*

##### Neurodegeneration with Brain Iron Accumulation (NBIA)

#### Submission Deadline For Applications is June 15, 2023

**About NBIA Disorders Association**

# the NBIA Disorders Association’s goals are to raise funds to support research pertinent to NBIA; to provide emotional support to those afflicted with NBIA and their families; and to raise public awareness of these ultra-rare disorders. If you would like further information about our mission, please email Patricia Wood at [pwood@NBIAdisorders.org](mailto:pwood@NBIAdisorders.org), or telephone at (619) 588-2315.

Neurodegeneration with Brain Iron Accumulation (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. It typically first appears in childhood.

Presenting signs and symptoms may include difficulty walking, loss of balance, developmental delay, intellectual disability, expressive language delay, sleep disorders, seizures, occular defects, and other problems related to speech. Those affected may suffer a progressive loss of muscle control, sudden involuntary muscle spasms, and uncontrolled tightening of the muscles. At the present time, symptoms for the disorders may be treated but there are no cures.

The NBIA Disorders Association is currently accepting applications for a one-year grant not to exceed $60,000 for clinical and translational research studies related to the early detection, diagnosis, or treatment of patients with **Beta-propeller Protein-Associated Neurodegeneration (BPAN)**, caused by a mutation in the *WDR45* gene on the X chromosome. BPAN typically is recognized in early childhood with delayed development and seizures. In adulthood, people with BPAN develop rapidly progressive parkinsonism.BPAN is the most common NBIA disorder at 35-45% of the NBIA population, with an estimated prevalence of 2 to 3 per million individuals.

**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 and advance knowledge about NBIA disease processes.

**Our research priorities for the BPAN 2023 Spring Grant Cycle are:**

Grants are expected to generate essential information for the scientific community to advance knowledge about BPAN disease processes, and to produce preliminary data to enable national and international funding to carry the work forward.

Examples of priority topic areas include: developing and exploiting disease models including computer models, identifying biomarkers, delineating the molecular cascade that leads to early cellular changes, developing rational therapeutics, establishing outcome measures to be used in clinical trials, and developing other essential resources to substantially prepare the BPAN community for clinical trials.

Natural history studies proposals should reflect knowledge of existing, ongoing studies and include a statement indicating how the proposed study would complement or integrate with existing studies. Moreover, proposals for natural history studies must have a component that includes participation in the TIRCON International NBIA Patient Registry & Biobank.

Research resources, including model organisms 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>. In addition, investigators are encouraged to share novel cell and animal models or technologies that are not yet defined in the NIH guidelines.

**Application Deadline June 15, 2023**

* 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 5-7) on your computer in Microsoft Word, do a “Save As” with the filename “2023 BPAN 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](mailto:pwood@NBIAdisorders.org).

## RFA Deadlines

* Applications due by June 15, 2023
* Award announcement by July 25, 2023
* Grant funding to begin September 1, 2023

**Further Information**

* All applications will be reviewed for scientific merit.
* Indirect costs are allowed up to a maxium 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.
* All awardees are expected to attend NBIADA family conference or regional meeting events during or shortly after the award time frame to present their work and meet NBIA families. Travel costs will be reimbursed.
* Applicants who currently have grant awards with NBIADA must be current on all required reports before submitting a new grant proposal.
* 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 1 year 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 acceptance of grant award within specified time by NBIADA.
* 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.

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| Name of Researcher |  | |
| Researcher’s Email |  | |
| Researcher’s Mailing Address |  | |
| City/State/Zip  Country |  | |
| Researcher’s Telephone |  | |
| Researcher’s Fax |  | |
| Institution(s) Where Research Will Be Conducted. |  | |
| City, State, Country of Institution(s) |  | |
| Title of Project |  | |
| Funding Amount Requested |  | |
| Will Research Involve Human Subjects? | [ ] YES [ ] NO | |
| How Did You Hear About This RFP? | \_\_ U.S. Postal Service Notice \_\_ E-mail Subscription Service  \_\_ NBIADA email \_\_ Website Posting \_\_ Other | |
| If you checked “Other” above, please explain. |  | |
| Applicant Signature (required)  e-signature acceptable |  | |
| **Please provide all supporting documentation required**  **with your application and submit by June 15, 2023**  **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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| --- | --- |
| **Name**  Position/Title |  |
| **Education/Training**  Begin with baccalaureate or other initial professional education and include postdoctoral training. | Institution and Location Degree Year(s) Field of Study |
| **Research and Professional Experience**  Concluding with present position, list in chronological order, previous employment, experience, and honors. Include present membership on any advisory committee. |  |
| **Honors and Awards** |  |

**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 torepresentative earlier publications pertinent to this application. If the list of publications exceeds two pages, select the most pertinent publications.

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