

#### NBIA Disorders Association

#### Announces

#### Research Grant

*For*

***PLA2G6-Associated Neurodegeneration (PLAN)***

####  Submission Deadline For Applications is March 31, 2024

**About NBIA Disorders Association**

# the NBIA Disorders Association’s goals 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 these ultra-rare disorders. If you would like further information about our mission, please email Amber Denton at adenton@NBIAdisorders.org, or telephone at (832) 835-NBIA.

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. These disorders typically first appear 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.

**About PLAN**

**PLA2G6-Associated Neurodegeneration (PLAN)**, an NBIA disorder caused by mutations in the *PLA2G6* gene, is made up of three distinct forms with differing characteristics:

* **INAD (Infantile Neuroaxonal Dystrophy) or infantile-onset PLAN**: early onset, rapidly progressive psychomotor deterioration.
* **Juvenile-onset PLAN**: later childhood onset with slower progression and predominant extrapyramidal (nerves that regulate motor control) findings, such as dystonia (involuntary muscle contractions that cause repetitive or twisting movements) and dysarthria (difficulty pronouncing words). It includes a broad range of presentations.
* **Adult-onset PLAN or PLA2G6-related dystonia-parkinsonism**: adult-onset dystonia-parkinsonism accompanied by cognitive decline and neuropsychiatric changes (mental disorder due to disease of the nervous system)

**Research Objectives**

The NBIA Disorders Association is currently accepting applications for a one or two-year grant not to exceed $100,000 for basic, translational, or clinical research studies related to the early detection, diagnosis, or treatment of people with PLAN.

The purpose of this grant call is to encourage meritorious research studies designed to improve the diagnosis or treatment of PLAN. 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 National Institutes of Health (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 PLAN 2024 Spring Grant Cycle are:**

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

All proposals for the treatment of PLAN are welcome with an emphasis on those with high potential impact. While gene therapy falls under that category, two AAV9 projects are currently underway. Therefore, proposals for gene therapy should focus on new research in this area in order to be considered. Priorities include identifying biomarkers or new therapeutic targets, delineating disease pathogenesis, and performing pre-clinical studies to evaluate compounds for safety and efficacy.

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 March 31, 2024**

* 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 and whether one or two-year project. (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 with any other source, 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 the 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 for the primary investigator (PI).
* Open application (pages 5-7) on your computer in Microsoft Word, do a “Save As” with the filename **“2024 PLAN 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.

## RFA Deadlines

* Applications due by March 31, 2024
* Award announcement by June 7, 2024
* Grant funding to begin July 15, 2024

**Further Information**

* All applications will be reviewed by NBIADA’s Scientific & Medical Advisory board (SMAB) for scientific merit.
* Indirect costs are allowed up to a maximum of 15% of the grant request as part of the total grant award not to exceed $100,000.
* PI salary of up to 10% effort per year will be allowed in addition to salaries for other essential personnel.
* 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 participant Informed Consent and the Institutional Review Board (Ethics Committee) approvals will be required from each site involved in the study before grant 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 recipient and NBIADA will enter into a grant agreement delineating the rights and responsbilities of the parties, including reporting requirements, the grant payment schedule, and project timeline.
* Grant recipients will receive one or two-year funding in three stages: 1/3 at start of project; 1/3 at six months (for 1 year projects) or 12 months (for 2 year projects) 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 grant manager 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. The grant agreement will contain the right on the part of NBIADA to terminate the grant agreement for an uncured violation of its terms and conditions by the grant recipient.
* 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/ZipCountry |  |
| 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 March 31, 2024** **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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