



BPAN Research and Collaborative Exchange (RACE)

Targeted Research to Advance a Cure

BPAN RACE Awards

PURPOSE

BPAN RACE's grants aim to encourage research focused on finding a specific treatment for BPAN. BPAN RACE is committed to assisting individuals living with BPAN to realize their full potential. Our goal is to bring practical treatment into current medical practice as quickly as possible; therefore, preference will be given to research projects that have a clear practical application and the results of which will be shared in a timely fashion. We also hope that grants we fund will lead to additional research support from government agencies and other funding sources.

The BPAN RACE program will provide targeted funding to enable research projects on BPAN to proceed at a rapid pace. These awards seek to stimulate the development of potential therapeutics for the treatment of BPAN. Particular areas of interest are drug discovery, gene therapies, and identification of BPAN-specific biomarkers. All proposals must be scientifically linked to BPAN. Applications that receive the strongest consideration are those that have the potential for rapid impact on and translation to the BPAN community.

KEY DATES

Release Date: January 1, 2020

Applications will be accepted on a rolling basis.

2020 BPAN RACE RESEARCH AWARD PRIORITY AREAS:

Basic Research track:

1. Research dedicated to furthering the understanding of WDR45 function to inform the development of targeted, novel therapies.
2. Transformative research to enhance our understanding of the cellular, molecular, genetic and systems-level mechanisms contributing to the pathogenesis of BPAN, facilitating the continued investigation of disease-modifying strategies.
3. Research aimed at improving BPAN disease models (e.g., cell-based, tissue-based, or animal models) in an effort to assess the potential efficacy of therapeutic interventions against phenotypic deficits in the BPAN patient population.

Clinical Research track:

1. Clinical research that will facilitate elevated understanding of the natural history of BPAN as it relates to symptom progression (including, but not limited to, seizures and cognitive delay), pharmacosensitivity, and response to surgical/behavioral/supportive therapies.
2. Development of clinical biomarkers, severity scales, or patient-reported outcome measures for BPAN, particularly those that (1) assess disease burden, that (2) may be prognostic indicators of an individual's risk for disease progression, or that (3) contribute to clinical trial readiness.
3. Development of a scalable collaborative infrastructure for a BPAN Clinical Research network. Beyond establishing and maintaining the infrastructure for collecting and sharing data, a priority will be to support the activities of clinical research fellows in ongoing collaborative projects within the network.

ELIGIBILITY

Researchers from non-profit institutions (including academic institutions) are eligible to apply. Researchers from for-profit institutions will NOT be considered. Primary Investigator(s) should have training and experience at least equal to the Ph.D. or M.D. level. BPAN RACE encourages research projects that are collaborative and those with a multidisciplinary approach.

BPAN RACE'S Guiding Principles include a commitment to collaboration. Research funding is contingent upon sharing of resources, data, and outcomes.

DURATION OF SUPPORT and ALLOWABLE COSTS

BPAN RACE Grants are typically awarded as either one or two year grants, made in one-year allocations, with a maximum funding of \$250,000 per year. Second year funding is non-competitive but is based on review of the first year's progress report.

A mid and end-of-year progress report (including a financial report) are required for all grants. Progress report guidelines will be distributed to awardees with the grant agreement. The financial report and final progress report must be submitted within 8 weeks from the close of the grant period. Grant renewal applications must be submitted at least 8 weeks before the second year of funding. Renewal applications must include a detailed progress and financial report.

Potential allowable costs include:

- Reagents and Services - These may include but are not limited to: gene chips, chemical libraries, acquisition of cell lines, acquisition of mouse lines.
- Services - These may include, but are not limited to: deep sequencing, proteomics, metabolomics, mass spectrometry, and antibody production.
- Equipment - BPAN RACE will consider the purchase of specialized pieces of equipment or software purchases for specific projects.
- Personnel – Including salary and fringe benefits.
- Travel Costs – For travel costs directly related to achieving the objectives of the grant and to present at a BPAN RACE designated patient or research summit to be announced.
- Indirect Costs - Up to 10-12% to be included in total cost of project.

LETTERS OF INTENT

Unless an invitation to apply for funding has been extended, applicants are required to submit a Letter of Intent (LOI) to determine the feasibility of a potential request prior to submitting a full proposal. LOIs will be reviewed by members of the BPAN Warriors Scientific Medical Advisory Board and invited scientific reviewers.

LOIs must be submitted as one PDF file containing the following information:

1. A brief description of the project including objectives and hypothesis/rationale
2. An estimate of proposed budget
3. Preliminary data
4. CV/Biosketch of PI(s)

LOIs can be submitted to Sarah Chisholm at science@bpanwarriors.org.

APPLICATION FORMAT and SUBMISSION INFORMATION

The research proposal must include and be ordered as follows:

- I. Title Page: Proposal title and PI information.
- II. Abstract: Provide a lay summary and a scientific summary of the project.
The lay summary is an important part of the application and will be considered when determining funding decisions. The lay summary will be posted on our website if your proposal is funded.
- III. Proposal (NIH format is acceptable)
 - A. Hypothesis
 - B. Specific Aims - include research questions and/or hypotheses.
 - C. Background and Significance - provide rationale for the research with emphasis on what is needed and why this project would benefit BPAN research.
 - D. Preliminary Studies - describe any preliminary data you have gathered which bears on the specific research question(s).
- IV. Research Design and Methods (3-page maximum):
 - A. Study Design
 - B. Analysis Plan
 - C. Expected Results
 - D. Limitations and Alternative Approaches
- V. Timeline and Milestones (Deliverables)
- VI. Budget: Provide a detailed budget and justification.
- VII. Biosketch for each PI and any collaborative personnel for the project (use basic format followed by NIH).
- VIII. Letters of support or collaboration as relevant (optional)

Applications can be submitted as one PDF file to Sarah Chisholm at science@bpanwarriors.org.

BPAN RACE GRANT REVIEW PROCESS

Applications will be reviewed by the highly esteemed scientists that form our Scientific Medical Advisory Board (SMAB) based on scientific merit and the alignment of the applications with the BPAN RACE priorities. Applications will also be sent to an external review panel who will evaluate the proposals based on established BPAN RACE funding criteria.

Final approval is required by the BPAN Warriors Board of Directors before funding may occur. BPAN Warriors' Board of Directors will select the grant recipients based on the recommendations of the Scientific Medical Advisory Board, the external review panel, the impact of the research on the mission of BPAN RACE, and available funds.

CONFLICTS OF INTEREST

All reviewer conflicts of interest must be revealed to the Scientific Medical Advisory Board and/or external review panel prior to any evaluations being conducted. Conflicts of interest include, but are not limited to, employment at the same sponsoring institution and collaboration on recent or current research projects. All discussions of scientific merit are conducted in the absence of any reviewers who have declared conflicts of interest.

INSTITUTIONAL REVIEW BOARD

BPAN RACE requires recipients to demonstrate Institutional Review Board approval of the research project prior to dispersal of award funds. IRB approval is NOT required at the time of application submission. If IRB approval cannot be obtained within six months of the award, the award may be rescinded by the Board of Directors.

PROHIBITION OF DISCRIMINATION

No person shall be excluded from participation in or denied the benefits of any program or activity receiving financial assistance from BPAN RACE because of race, ethnic origin, religion, sex, or sexual orientation.

AMENDMENT OF POLICIES

BPAN RACE reserves the right to modify its policies governing research awards at any time. The recipient agrees to abide by any changes or to terminate the grant at the time when such changes become effective. Failure to abide by the policies governing awards shall be considered sufficient grounds for cancellation of an award or refusal to consider any pending application by the grantee.

FINAL DOCUMENT FORMATTING

All pages should be single spaced, 11 pt. Arial font with 0.5 inch margins. Number pages at top right or bottom center prior to converting to pdf.

INQUIRIES

Questions regarding these guidelines are welcome and should be directed to Sarah Chisholm at science@bpanwarriors.org.