REQUEST FOR PROPOSALS

The National Organization for Rare Disorders (NORD)'s Research Grants Program

Announces a research grant opportunity for one grant
up to $35,000 US for

Arteriovenous Malformation (AVM)

DEADLINE FOR INITIAL APPLICATIONS: October 16, 2023 (11:59 pm PT)

NORD, with fundraising by the Tyler James Abizeid Foundation is accepting applications for one grant, up to $35,000 US, for scientific and/or clinical research studies related to Arteriovenous Malformation (AVM).

Arteriovenous malformations (AVMs) are abnormal tangles of blood vessels, varying in number and size, that cause irregular connections between arteries and veins. AVMs most frequently form in the brain or the spinal cord but can occur in many different parts of the body-wherever arteries and veins are present. AVMs can cause damage to the brain or spinal cord by altering the amount of oxygen that reaches neurological tissue, causing bleeding in surrounding tissues, and compressing parts of the brain or spinal cord. In most cases, people with AVMs experience few, if any symptoms until the AVM ruptures. Symptoms of AVM may only occur after some amount of damage has been done to the brain or spinal cord and range in severity from a minor headache to a devastating rupture with hemorrhagic stroke. Other physical symptoms may include seizures, headache, pain on one or both sides of the head, visual problems, muscle weakness, problems with speech, problems with movement and abnormal sensations. Neurological symptoms depend on the location of the lesion and may include dizziness, loss of consciousness, memory deficits, confusion, hallucinations, or dementia. In addition to arteriovenous malformations, three other types of vascular lesions can affect the central nervous system including cavernous malformations, capillary telangiectasis and venous malformations.

The cause of AVMs is not yet well understood, but most often AVMs are congenital and form during fetal development. AVMs can appear early or later in life. Several types of AVMs are known to be hereditary and have a genetic basis (including hereditary hemorrhagic telangiectasia (HHT), Sturge-Weber syndrome, and Klippel-Trenaunay syndrome). Some current research studies related to the advancement of knowledge in AVM include: identifying risk factors for bleeding inside the brain in people who have HHT; testing beta-blockers to evaluate their effect on HHT; developing biomarkers for AVM; identifying molecular pathways that are fundamental to the formation of brain AVMs; and testing the Rhoa/ROCK protein signaling pathway in relation to inhibiting CCMs (cerebral cavernous malformations) development and hemorrhage.

Research Objectives

The NORD Rare Disease Research Grant Program was established in 1989 to encourage meritorious scientific and clinical studies designed to improve the diagnosis, understanding of underlying disease mechanisms, or therapy of specific rare diseases. Studies related to Arteriovenous Malformation (AVM) will be considered. Grants will be awarded to qualified researchers to initiate small scientific research studies or clinical trials, the results of which could be used to obtain funding from the NIH, FDA, or other funding agencies, or to attract a corporate sponsor. Ideally, the proposed research should have the potential to lead to the ultimate development of a new or better therapy. Evaluation of proposals will include careful consideration of protocol design, objectiveness of parameters measured, and statistical evaluation proposed.

About NORD

The National Organization for Rare Disorders (NORD) is the leading independent advocacy organization representing all patients and families affected by rare diseases. NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90 percent are still without an FDA-approved treatment or therapy. Rare diseases affect 25-30 million Americans. More than half of those affected are children. NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For more than 35 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research and providing patient and family services for those who need them most. NORD is made strong together with over 300 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease patient community.

APPLICATION PROCESS OVERVIEW

INITIAL APPLICATIONS

- Requests for proposals will be released by NORD.
- Letters of intent will be due **October 16, 2023**.
- Letters of intent will be reviewed by NORD reviewers.
- Requests for full proposals will be issued via email in early December 2023.

FULL PROPOSALS

- Full proposal invitations will be issued via email.
- Application requirements for full proposals will accompany these invitations.

AWARDING OF GRANT

- Award announcements will be made via email and posted on NORD's website in April 2024.
- Funding will begin after all necessary documents (e.g., IRB forms, patient consent forms, signed grant agreements) have been received by NORD.

FURTHER INFORMATION

- If the study involves human or animal subjects, copies of governance documents will be required from each site involved in the study before payment can be issued.
- Clinical drug trials must meet requirements established by the U.S. Food & Drug Administration (FDA).
- Duplicate/overlapping funds from any other private or public source are not to be used.
- All applications determined to have scientific merit will be considered, however before an award is issued compliance with international funding regulations must be confirmed when applicable.

INITIAL APPLICATION

Interested applicants should submit a completed application and letter of intent electronically to research-programs@rarediseases.org with "NORD Letter of Intent" as the subject line. Applicants can complete the requested information directly in the document provided below and/or merge any additional required documents into a single PDF file. Incomplete applications may not be considered. All applications must be received by **October 16, 2023 (11:59 pm PT)**.

REQUIRED ELEMENTS CHECKLIST	**PAGE**	✓
Application summary	4	
Letter of Intent (Maximum Length 2 Pages)	5	
Biographical sketch	6	
List of co-investigators, if applicable	7	
Budget outline	7	
OPTIONAL ELEMENTS	**PAGE**	✓
Reviewer information	8	
Letter of support	8	

APPLICATION SUMMARY

Deadline for Initial Applications: October 16, 2023 (11:59 pm PT)

PRINCIPAL INVESTIGATOR INFORMATION	
Name	
Position/Title	
Email	
Mailing Address	
Telephone	
PROPOSAL INFORMATION	
Project Title	
Project Term	2 YEARS
Funding Amount Requested ($ US) *not to exceed $35,000 US*	
Institution(s) where research will be conducted	
City, State/Province, Country of Institution(s)	
Will research involve human subjects?	[ ] YES [ ] NO
Will research involve animals?	[ ] YES [ ] NO
How did you hear about this RFP?	☐ NORD ☐ Rare Disease Organization ☐ Professional Organization ☐ Academic Organization ☐ Referral from Colleague ☐ Other (please specify)
Please be as specific as possible:	
Did you hear about this RFP via:	☐ NORD Website ☐ Email ☐ Google ☐ Medical/Research Publication ☐ Social Media (Facebook, Twitter, LinkedIn, Instagram) ☐ Rare Action Network ☐ Other (please specify)
Please be as specific as possible:	

LETTER OF INTENT

Please provide a Letter of Intent addressing the following elements of your research. Applicants may use the form below or attach a separate document to address the following details.

Letter of Intent (Not to exceed 2 pages)	
Summary Statement	
Statement of Need • What issue will the research address • Significance of the work • Why you have chosen this issue • Who will benefit from this research • Why this funding is essential	
Project Activity • Overview of research activities • Why this approach is novel • How the research builds upon and differs from previous research (if applicable)	
Credentials • Why your institution/program is best equipped to do this research	
Closing	
Principal Investigator Signature **REQUIRED**	

BIOGRAPHICAL SKETCH

Please provide a biographical sketch and bibliography for the principal investigator. Applicants may use this form or the NIH Biosketch form. Please modify the form to include, when applicable, the following:

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				

CO-INVESTIGATOR(S)

Name of Co-Investigator	
Position/Title	
Institution	
Email	

Name of Co-Investigator	
Position/Title	
Institution	
Email	

Name of Co-Investigator	
Position/Title	
Institution	
Email	

BUDGET

Please provide a brief budget outline describing how the funding will be used. Do not include PI salary, overhead, or indirect costs. Funding can be used to cover expenses such as staff salary, technical assistance, supplies, and small equipment.

REVIEWER INFORMATION (OPTIONAL)

Please list up to five areas of scientific/medical expertise needed to review this application (optional). Do not list names of individuals.

1.
2.
3.
4.
5.

Please list below any individuals who should not review this application (optional).

NAME	INSTITUTION	JUSTIFICATION

LETTER OF SUPPORT (OPTIONAL)

Please include a letter of support for your research from an individual advocate (e.g., patient, care partner) or patient advocacy group in this disease space (optional, but encouraged).

To receive notification of future funding opportunities through NORD, sign up for NORD research news here: https://rarediseases.org/communications-sign-up/