

A FOUNDATION BUILDING STRENGTH FOR NEMALINE MYOPATHY

MISSION

Our Mission is to advance the science of treating the nemaline myopathies while supporting the families of Nemaline Myopathy with information, resources and solutions.

A Foundation Building Strength (AFBS) is a non-profit organization striving to stimulate, advance and move forward the science of treating the nemaline myopathies while supporting the community of Nemaline Myopathy (NM) with information, resources and solutions.

The foundation's primary goals are to:

1. Fund and stimulate basic and translational research into treatment and a potential cure for NM
2. Partner with companies to provide necessary medical equipment for those in need or when health insurance will not cover related expenses
3. Create a panel of experts to answer medical and developmental questions for individuals and families affected by NM
4. Provide resources to bring NM communities together for bi-annual conferences.
5. Raise awareness about this rare disease
6. Develop a clinical consensus on the standardization of care for individuals with NM

Nemaline Myopathy Research Grant

The objective of the Nemaline Myopathy Research Grant is to provide funding to investigators performing research that will:

- Advance our understanding of the pathogenesis of NM in order to design and optimize treatment and management strategies
- Explore the effectiveness of potential therapies and/or treatments for NM
- Investigate ways to improve muscle strength and quality of life for those living with NM by conducting clinical trials

AFBS encourages research that addresses fundamental questions with regard to:

- The way in which mutations in the actin, nebulin, tropomyosin and other genes cause disease
- The use of stem cells and small molecule therapy in NM disease models
- The development of research protocols to evaluate the role of exercise in improving muscle strength in patients with NM

Treatment related research is especially encouraged.

These projects may include, but are not limited to:

1. Discovery of new genetic mutations expressed in NM
2. Studies of the functional relationship between these mutations and impaired muscle function at the cellular and/or molecular level
3. Development of strategies for replacement or repair of mutant muscle proteins using stem cells or small molecule therapy
4. Clinical studies of the role of exercise in improving muscle strength in patients with NM
5. Clinical studies of the role of tyrosine supplementation, creatine supplementation and other approaches to increase energy and muscle strength in patients with NM

ELIGIBILITY CRITERIA

To be eligible to apply for the Nermaline Myopathy Research Grant, an applicant must:

- Hold a Doctor of Medicine (M.D.), Doctor of Philosophy (Ph.D.) or equivalent degree (e.g. D.O.)
- Be a professional researcher or faculty member (Professor, Associate or Assistant Professor) at an appropriate educational, medical or research institution
- Be qualified to conduct and mentor a program of original research within their own laboratory
- Assume both administrative and financial responsibility for the grant
- Have access to institutional resources necessary to conduct the proposed research properly
- There are no geographic restrictions for applicants.

REQUIREMENTS FOR GRANT APPLICATION

Lay Abstract

Please provide a statement of no more than 300 words explaining the subject of the research proposal and its relationship to NM. This should be written using relatively simple lay terminology so that an individual without scientific training will understand the statement.

Scientific Abstract

Please provide a statement of no more than 300 words explaining the subject of the research proposal and its relationship to NM. This statement will inform the scientific community of the nature of this work.

Research Plan (No longer than 5 pages of text plus references)

1. Hypothesis and Specific Aims: (maximum of one page)
State clearly and concisely the intent of the proposed research and the hypothesis to be tested.
2. Background and Significance: (maximum of one page)

Briefly describe the background of the present proposal. Explain the potential significance and relevance of the proposed work to advancing knowledge of NM. Please include a clear description of the scientific background and a demonstration of techniques needed to make the proposed project a success.
3. Preliminary Studies: (maximum of one page)

Cite prior work in your laboratory and published papers directly related to the proposed project. Include descriptions of established techniques and collaboration with other scientists.
4. Research Design and Methods : (maximum of two pages)

Provide a detailed discussion of the experimental design and methods to be used to accomplish the specific aims. Please include a detailed timeline for experiments included in the project.

Budget

Please include a detailed budget and justification that addresses personnel, equipment, supplies, travel, patient care, and any other relevant costs. The allowable indirect cost rate for institutional overhead will be negotiated.

Terms of Award

Award Period

Grants will be awarded for a one-year time interval. Funding recommendations will be made by the AFBS Scientific Advisory Board and funding decisions will be made by the AFBS Board of Advisors.

Amount of Award

Funding for the award period will be made for up to \$75,000 per year. Proposals for less than the maximum amount are encouraged.

Funding for additional years may be at an increased level and will not be competitive but rather based upon the approval of the AFBS Scientific Advisory Board of the first year's progress reports.

Other Policies and Guidelines

Written progress reports will be expected on a semiannual basis. AFBS will contact the grant recipient for an update via telephone or email on a quarterly basis.

Regulatory approvals for proposed clinical investigations or animal studies do not have to be in place at the time an application is submitted. However, funding will not commence until all applicable human subjects or animal protocol approvals have been obtained.

Proposals will be reviewed by the Scientific Advisory Board of AFBS and applicants will be notified of the status of their proposals by February 27, 2009. Funding will commence on or about April 1, 2009.

SUBMISSION INFORMATION

Application Deadline

Applications may be submitted electronically via the application form at www.buildingstrength.org. The deadline for submission is January 31, 2009 at 5:00 P.M. Pacific Standard Time.

A Foundation Building Strength

for Nemaline Myopathy

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