

For a World Without Myasthenia Gravis

January 30, 2020

Research Funding Announcement

Targeted Research Opportunity: Advancing Knowledge of Treatment and Disease Management in Seronegative MG Patients

People diagnosed with seronegative myasthenia gravis – and their clinicians – face special challenges. Seronegative MG has been a relatively neglected area of research in the field, leading to uncertainty about patient outcomes and how to optimally manage patients. The Myasthenia Gravis Foundation of America requests submission of proposals that will lead to greater understanding of seronegative MG, particularly in determining treatment and disease management paradigms, and what may be most successful.

Thanks to a generous donation from MGFA Board member and philanthropist Susie Johnson, MGFA is able to offer <u>\$150,000 over 2 years</u> to support this project.

There is a great deal of latitude for creative approaches for this project. A project can build on other research that is already in process, but can also be innovative in design. Access to the MG Patient Registry can be provided. Projects may include translational and clinical approaches. Animal studies are not permitted under this funding mechanism. Given the rarity of seronegative MG, the engagement of multiple sites is highly encouraged.

Successful proposals will include:

- A clinical component, studying patients with a confirmed diagnosis of seronegative MG
- Inclusion and exclusion criteria, including clearly defined criteria for the diagnosis of seronegative MG
- Clear plan and timeline, including agreement from participating centers, plan for identification of study participants and planned sample size, and if the study will be retrospective or prospective
- Rigorous scientific methodology and experimental design

- Sound scientific premise, including consideration of the strengths and weaknesses of published research AND preliminary data crucial to the support of the proposal
- Clearly defined outcomes, and a detailed plan for how success will be measured
- Next steps for use of the data, and possible pathways for subsequent research and funding

Applicants are asked to submit a letter of intent (LOI) using the form provided by March 19, 2020. LOIs should be sent to <u>MGFA@myasthenia.org</u>. Please copy <u>nancy.law@myasthenia.org</u>. You will be informed by April 1, 2020 if you are invited to submit a full proposal.

The MGFA will fund highly meritorious projects with a maximum direct cost of \$150,000 inclusive of a maximum 10% indirect cost rate. Funds may be expended over 2 years from the time of the award.

For the full proposal, the applicant must provide a detailed budget in the format used for NIH budgets. See the list of forms and links at the end of this announcement. There are no specific budget restrictions but all aspects of the budget must be clearly justified. If Principal Investigator salary support is requested, then a letter from a department chair or equivalent must accompany the application to assure that the PI is afforded protected research time for the project.

The application consists of a five page research section, which should include specific aims, background/preliminary data, innovation, and approach sections. There is no limit to the reference section. A separate single page section should describe how the funds will benefit the goals of the MGFA. The application must also contain a *Lay Summary* of up to ½ a page in length or approximately 200 words.

Proposals will be reviewed by the MGFA research committee as well as ad hoc reviewers when required. The RFA is open to established and new investigators. The funding is restricted to principal investigators in academic institutions in the United States and Canada, although collaborators may reside outside North America.

Proposals should be submitted by email to MGFA at <u>MGFA@myasthenia.org</u> no later than **June 15, 2020**. A preliminary review to assure compliance with guidelines will be performed, followed by scientific review. Funding notification is expected by **October 15**, **2020** and funds provided by **January 2021**. Participation in MGFA key activities, such as presentations on their work at the MGFA Scientific Session at the AANEM Annual

Meeting and the MGFA National Conference are expected from award recipients. Recipients may also be asked for interviews on their project and its relevance to the MG Community for MGFA's lay oriented website and/or newsletter at both the start and completion of the project.

Applicants are required to use the following forms which can be accessed using the links below:

- <u>Letter of Intent</u>
- <u>Cover Letter</u>
- Lay Summary Form
- <u>Research Plan Form</u>
- <u>NIH Budget Form</u> & <u>MGFA Budget Justification Form</u>
- Development of Future Support Form
- <u>NIH Bio-sketch Form</u>

The Research Funding Application and Review Calendar can be found on the next page.

Myasthenia Gravis Foundation of America • 355 Lexington Avenue, 15th Floor • New York, NY 10017

Research Funding Application & Review Calendar

Activity	Timing
Research Funding Announcement	January 30, 2020
Letter of Intent Deadline	March 19, 2020
Application Deadline	June 15, 2020
Applications assigned to reviewers	June 16 to July 1
Review of applications	July 1 th to September 20 th
Funding notification	October 15, 2020
Funding provided	January 2021
First Year Progress Report due	January 2022
Final Report due	January 2023

Myasthenia Gravis Foundation of America • 355 Lexington Avenue, 15th Floor • New York, NY 10017



The research committee of the MGFA is committed to supporting research that will ultimately improve the lives of patients with myasthenia gravis and related neuromuscular junction disorders. The committee has identified four broad research priorities of unmet need in the field.

- Biomarkers
 - Early diagnosis
 - Predictors of clinical outcome
 - Predictors of response to immunosuppressive therapy
 - Biomarkers for use in clinical trials
- Mechanisms of disease
 - Basic mechanisms of disease
 - How loss of self-tolerance is sustained throughout the disease course
- Therapeutic strategies
 - Development of new therapeutic targets
 - Targeted therapies that hopefully prevent widespread immunosuppression and off-target side effects
 - Optimizing treatment strategies with existing therapies
- Improving patient outcomes
 - The patient perspective on their disease, its impact on their daily life, and treatment considerations that are most important to them
 - Collateral effects of MG and its' treatment such as associated medical conditions, treatment related side effects, and financial considerations.
- Pediatric treatment strategies, safety concerns, and long term outcome.

Though this research agenda is intended to guide researchers towards topics that are important to the MGFA MSAB, the research committee will accept proposals outside of these priority areas and is committed to supporting the best science.

Myasthenia Gravis Foundation of America • 355 Lexington Avenue, 15th Floor • New York, NY 10017