The Neurology Clinical Trials Unit (NCTU) at Massachusetts General Hospital is pleased to announce a call for clinical trial applications for novel, high-potential treatments for Amyotrophic Lateral Sclerosis (ALS). The call for clinical study proposals is intended for pharmaceutical, biotherapeutic/biotechnology companies, academic members of the Northeast ALS Consortium (NEALS), and ALS scientists throughout the world. A total of USD $480,000 (direct costs) in NCTU/NEALS clinical research support is available. The request for proposals is independent of the recent call for clinical pilot studies from The ALS Association, however requests for use of the NEALS clinical infrastructure as part of that program will also be considered.

Potential studies may include phase 0, I, and II clinical trials. Selection of the top candidates to bring forward will be made by a Science Review Board. Applications will be judged on:

- The basis of scientific merit, novelty, and the value of the project.
- Availability of appropriate facilities and the technical ability to carry out the project.
- Use of NCTU and NEALS clinical trial cores.

We strongly encourage applicants to examine the shared resources and core facilities available within the NCTU and NEALS and to develop applications that make use of these facilities. Importantly, the successful applicant will retain control of the trial as well as any intellectual property relating to the therapeutic agent being investigated. NCTU/NEALS are extremely well-positioned to work with both academic and industry partners to rapidly bring the best ALS science into clinical testing.

**Deadlines**

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**Background**

There is a critically urgent need for better treatments. ALS affects one in approximately 40,000 Americans, with 5,000 new diagnoses each year in the United States. Currently, there is only one marketed therapy (Riluzole®) for ALS patients. The goal of this Request for Proposal is to expedite the process of bringing new treatments forward for testing in people with ALS.

**About the Neurology Clinical Trials Unit and the Northeast ALS Consortium**

The NCTU at Massachusetts General Hospital is an Academic Contract Research Organization (ACRO) experienced in developing and conducting multicenter clinical trials in neurological diseases. It has an extensive history in leading clinical research projects aimed at finding new treatments for people with ALS.

The NCTU serves as a Coordinating Center for NEALS, a non-profit, research consortium committed to the cooperative implementation, timely completion, analysis, and reporting of clinical trials and other research studies aimed at improving patient care.
and understanding of the disease process of ALS. Together, the two entities have supported/conducted 19 clinical trials in ALS.

Selection Criteria

The Science Review Board will evaluate proposals based on the following criteria:

1. **Significance.** Does this study bring forward a new potential therapy for people with ALS? Can the proposed clinical trial be initiated expeditiously? Applicants should demonstrate that there is sufficient preclinical data (pharmacology and toxicology) to support initiation of the proposed trial.

2. **Approach.** Are the conceptual framework, design, methods, and analyses adequately developed, well-integrated, and appropriate to the aims of the project? Does the applicant acknowledge potential problem areas and consider alternative approaches?

3. **Innovation.** Does the project employ novel concepts, approaches, or methods?

4. **Investigator/Sponsor.** Is the investigator or sponsor appropriately trained/qualified to carry out this work?

5. **Environment/Collaborative Potential.** Does the scientific environment in which the work will be done contribute to the probability of success? Does the proposed clinical trial take advantage of useful collaborative arrangements? Will the proposed trial appropriately utilize resources available through the NCTU/NEALS clinical trial cores?

NCTU/NEALS Clinical Trial Cores

- Protocol Development and Consultation
- Regulatory Guidance
- Project Management
- Grants & Contract Management
- Data Management
- Study Monitoring
- Outcome Measure Development and Training
- Site selection, start up, regulatory document review, and ongoing site management
- Site Trainings: Good Clinical Practice, Regulatory Compliance, and Site Management

Please visit our websites for further details and core leadership contact information:
NEALS: http://www.alsconsortium.org

Applications Must Include the Following:

1. **Research Plan (maximum of 7 pages)**
   - Abstract
   - Specific Aims
   - Background and Significance
   - Preliminary Studies
• Research Design and Methods (study design; description of subjects; data to be collected; analysis plan)
• Supportive Documentation including a proposed timeline demonstrating that the therapeutic trial can begin expeditiously
• References

Appendix materials are allowed including material to demonstrate that the treatment is ready for human testing. For studies requiring an IND, please indicate in the proposal the status of the IND application.

2. Budget and Ancillary Documents
• Budget and Budget Justification – Direct Costs Only
• Biographical Sketch of Key Personnel
• Applicants are strongly encouraged to contact MGH NCTU prior to submission to discuss budget

Rules and Requirements
1. The deadline for the receipt of the application is 5:00 pm EST on September 1st, 2011.

2. Applications must be submitted electronically in a single PDF document to bpower@partners.org.

3. Format restrictions:
   a. Application text must be in Arial or Times New Roman font and can be no smaller than 12-point type. Figures, charts, tables, figure legends, and footnotes must be readily legible.
   b. Single-spacing is allowed.
   c. Margins, in all directions, must be at least 0.5 inches.

4. Decisions will be final and will be announced by October 2011.

Awardees will be invited to present their proposal at the NEALS Annual Meeting on October 27th, 2011 in Clearwater Beach, Florida.

Applications will be treated as confidential documents.

Questions about the RFP or NEALS/NCTU capabilities are encouraged and may be directed towards:

Breen Power
Outreach Manager
Neurology Clinical Trials Unit
bpower@partners.org
617-643-2456

and

Merit Cudkowicz, MD, MSc
Director
Neurology Clinical Trials Unit
mcudkowicz@partners.org
617-724-1873
Conditions of the Grant

1. Proprietary Rights
   The PI/Sponsor will maintain full control of scientific work and all corresponding responsibilities. A scope of work will be agreed upon by all parties prior to initiation of funding. Confidentiality agreements will be used if necessary.

2. Reporting
   For all projects, quarterly reports and a final report will be required.

3. Presentation
   Awardees are invited to make a platform or poster presentation at the 10th Annual NEALS Meeting on October 27th, 2011.

4. Publications
   All publications, as well as abstracts of presentations at scientific meetings, posters, or any other form of publication that results from a project supported by this award must carry the following acknowledgement: “This research was in part supported by a grant from the Massachusetts General Hospital Neurology Clinical Trials Unit and the Northeast ALS Consortium.”