



als FINDING a CURE®

ALS ACT (Accelerated Therapeutics)

Request for Proposals: Phase II Clinical development of novel, high-potential treatments for people with ALS

Release Date 23 October 2015

Letter of intent due: December 30, 2015

Submit letter of intent to researchgrants@alsa-national.org

ALS ACT and the Northeast ALS Consortium (NEALS) are pleased to announce a call for phase II clinical trial applications for novel, high-potential treatments in Amyotrophic Lateral Sclerosis. The call for clinical study proposals is intended for academic-industry partnerships, including pharmaceutical, biotherapeutic/biotechnology companies, academic members of the Northeast ALS Consortium (NEALS), and ALS scientists throughout the world. Up to USD \$1,500,000 (10% indirect costs can be included in this amount) in ALS ACT clinical research support is available.

Potential Phase II clinical trials should include therapeutic interventions that have:

- A. a pharmacodynamics marker that can measure whether pathway of interest has been affected, and
- B. a plan to collect samples for biomarker studies.

Applications will be reviewed by an ALS ACT steering committee and will be judged on:

- Scientific rationale and merit, novelty, and the value of the project.
- Availability of appropriate facilities and the technical ability to carry out the clinical study.

Funds will be awarded in the form of infrastructure support provided by NEALS and funds for per subject fee, sample collection, pharmacodynamic marker testing and other trial related costs as needed. Applicants may apply for a combination of any of the following clinical research support services available through NEALS:

- Project Management

- Grants & Contracts Management
- Data Management
- Study Monitoring
- Outcome Measure Development and Training
- Biostatistical Support
- Site Selection, Start Up, Regulatory Document Review, and Ongoing Site Management
- Site Trainings: Good Clinical Practice, Regulatory Compliance, and Site Management

The successful applicant will retain control of the trial as well as intellectual property relating to the therapeutic agent being investigated. Since infrastructure support is provided by this grant, applicants are required to contact NEALS/ALS Association prior to their grant submission to collaborate on the budget portion. Applicants may request the full \$1,500,000 in research support or may request a smaller amount, depending on the appropriate needs of the proposed study. A maximum of 10% indirect costs is allowable and should be included in the \$1,500,000.

Industry partnership applications are strongly encouraged with shared funding proposals.

Deadlines:

<u>Letter of Intent:</u>	November 30, 2015
Notification to submit full application	December 16, 2015
Full Application	February 1, 2016
Recipients Announced:	April 2016
Award starts once all necessary paperwork and administrative forms have been signed off by the institution(s).	

Download the Letter of Intent here: <http://www.alsa.org/assets/pdfs/loi-final-2015-call.pdf>

Background:

There is an urgent need for better ALS treatments and therapeutic agents. In the United States, ALS affects one in approximately 40,000 people, with 5,000 new diagnoses each year. There is currently one FDA-approved treatment for ALS, Riluzole (Rilutek). The goal of this Request for Proposal is to expedite the process of bringing new treatments forward for testing in people with ALS and to measure if that therapeutic agent is reaching its target.

About ALS ACT

ALS ACT is a novel academic-foundation-industry partnership to accelerate treatments for people living with ALS. In partnership with The ALS Association and the recently formed The ALS Finding a Cure Team, composed of researchers from General Electric (GE) Healthcare and four academic Northeast ALS Consortium (NEALS) sites, ALS ACT will enact a multi-pronged approach to expediting clinical trials in ALS.

About NEALS

The Northeast ALS Consortium (NEALS) is an international, independent, non-profit group of researchers who collaboratively conduct clinical research in Amyotrophic Lateral Sclerosis (ALS) and other motor neuron diseases. NEALS mission is to translate scientific advances into new treatments for people with ALS and motor neuron disease as rapidly as possible. NEALS has over 100 member sites in the United States, Canada, Ireland, and Israel.

About The ALS Association

The ALS Association is the only national non-profit organization fighting Lou Gehrig's Disease on every front. By leading the way in global research, providing assistance for people with ALS through a nationwide network of chapters, coordinating multidisciplinary care through certified clinical care centers, and fostering government partnerships, The Association builds hope and enhances quality of life while aggressively searching for new treatments and a cure. For more information about The ALS Association, visit our website at www.alsa.org

Selection Criteria

The ALS ACT steering committee will evaluate proposals based on the following criteria:

1. **Significance:** Does the proposed study bring forward a new potential therapy for people with ALS? Can the proposed clinical trial be initiated expeditiously? Does the study have a pharmacodynamics marker that can measure whether pathway of interest has been affected? Is there a proposed plan to collect samples for biomarker studies? 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 study? Have potential problem areas and alternative approaches been considered?
3. **Innovation:** Does the clinical trial employ novel concepts, approaches, and/or methods?
4. **Investigator/Sponsor:** Is the investigator or sponsor appropriately trained/qualified to carry out the study?
5. **Environment/Collaborative Potential:** does the scientific environment in which the work will be done contribute to the probability of success? Does the proposed study take advantage of useful collaborative arrangements? Will the study utilize resources available through available the clinical trial cores? Partnerships with Industry for partial support are encouraged.

Available NEALS Clinical Trial Cores

- Project Management
- Grants & Contracts Management
- Data Management
- Study Monitoring
- Outcome Measure Development and Training
- Biostatistical Support
- 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/>

The ALS Association: <http://www.alsa.org/>

Should you be invited to submit a full application, application forms will be provided and 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; participant description; data to be collected; plan of analysis)
- Supportive Documentation including a proposed timeline demonstrating that the trial will be conducted expeditiously
- References

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

2. Budget and Ancillary Documents

- Applicants are strongly encouraged to contact NEALS prior to submission to discuss budget planning. Tara Lincoln (617) 858-4277 tlincoln@partners.org; Merit Cudkowicz, MD mcudkowicz@partners.org
- Budget and budget justification – direct costs only
- Biographical Sketch of key personnel

Rules and Requirements

1. The application deadlines are described above. Applicants must be submitted electronically in a single PDF document to researchgrants@alsa-national.org
2. 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.

Awardees will be invited to present their proposal at the NEALS Annual Meeting in 2015.

Applications will be treated as confidential documents.

Questions about the RFP, ALS ACT, NEALS or The ALS Association capabilities are encouraged. Inquiries may be directed to:

Tara Lincoln
NEALS Program Director
tlincoln@partners.org
617-858-4277

And

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Co-Chair NEALS
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Lucie Bruijn, Ph.D. MBA
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Conditions of the Grant

1. Proprietary Rights

The Principal Investigator/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. If necessary, confidentiality agreements will be incorporated.

2. Reporting

For all projects, quarterly reports and a final report are required.

3. Presentation

Awardees are invited to make a platform or poster presentation at the 15th Annual NEALS Meeting in 2016.

4. Publications

All publications, as well as abstracts of presentations at scientific meetings, posters, or any other form of publication that results from a study supported by this award must carry the following acknowledgement: "This research was in part supported by a grant from the ALS Association, NCRI and the Northeast ALS Consortium (NEALS)."

5. Funds

Funds will be restricted until evidence of IND (or IND exemption) is presented. Additional restrictions will be in place until IRB approval is obtained.