



ALS ACT (Accelerated Therapeutics)

Request for Proposals: Early phase clinical development of novel, high-potential treatments for people with ALS

Release Date: 16 October 2017

Letter of intent due: 10 November 2017

Upload LOI and also email it to researchgrants@alsa-national.org

Link to online LOI submission:

<http://alsa.spectrumportal.net/IndividualApplicants/Application/0/25>

ALSA ALS ACT Clinical Phase I & II RFP 2017

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

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

Potential early phase clinical trials (Phase I and II) should include, where possible, therapeutic interventions that have:

- A. A pharmacodynamics marker that can measure whether pathway of interest is affected
- B. A plan to collect samples for biomarker studies
- C. A treatment planned in people with ALS

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

1. Scientific rationale and merit, novelty, and the value of the project.
2. Availability of appropriate facilities, regulatory approvals, and the technical ability to carry out the clinical study.
3. If co-funding is needed, availability of funding must be demonstrated.

The successful applicant will retain control of the trial as well as intellectual property relating to the therapeutic agent being investigated. Contacting ALSFAC/ALS Association prior to their grant submission is recommended to discuss opportunities and budget for The NCR Core facilities that can be utilized in the trial. 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. Should applicants require more than the allowed budget for the trial, match funds should already be secured at the time of the application.

Deadlines

Letter of Intent:	November 10, 2017
Notification to submit full application	December 1, 2017
Full Application	January 12, 2018
Recipients Announced:	March 15, 2018

Award starts once all necessary paperwork and administrative forms have been signed off by the institution(s) and the funding agencies.

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 are currently two FDA-approved treatments for ALS, riluzole (Rilutek) and edaravone (Radicava) that modestly change the disease course. 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 ALS Finding a Cure Team, composed of researchers from General Electric (GE) Healthcare and four academic sites, ALS ACT will enact a multi-pronged approach to expediting clinical trials in ALS.

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

About ALSFAC

The sole purpose of the ALS Finding A Cure®, a program of The Leandro P. Rizzuto Foundation, is funding research to find a cure. For more information about ALSFAC, visit our website at www.alsfindingacure.org

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. For more information about NEALS, visit our website at www.neals.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.
6. Applicants are not required to but can partner with two NEALS Cores: the MGH Neurological Clinical Research Institute and the Barrows Neurological Institute to access:
 - 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

Should you be invited to submit a full application, application forms will be provided and applications must include the following. Applications will be treated as confidential documents.

1. Research Plan

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 ALS FAC prior to submission to discuss budget planning. Lucie Bruijn, Ph.D. MBA; lucie@alsa-national.org Merit

Cudkowicz, MD mcudkowicz@partners.org

Budget and budget justification – direct and indirect costs

Biographical Sketch of key personnel

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 Annual NEALS Meeting in 2018.

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, and ALS Finding a Cure® Foundation'.

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.