Clinical Research Pilot Study and Clinical Management Grants
Request for Proposals

Deadline for brief study outline: May 28, 2012

Email researchgrants@alsa-national.org for study outline form.

A. Clinical Research Pilot Study

- The ALS Association’s scientific research program TREAT ALS (Translational Research Advancing Therapy for ALS) encourages translational research to bring new treatments to patients.
- Currently there is only one FDA approved drug for ALS, Riluzole, which has a modest effect on patient survival.
- This request for proposals (RFP) seeks to fund pilot clinical studies to obtain preliminary clinical data which will support applications to the National Institutes of Neurological Disorders and Stroke (NINDS) for subsequent larger clinical trials of an intervention to treat or prevent ALS.
- The proposed study must address questions that, when answered, will optimize the design of more definitive clinical trials rather than simply addressing the clinical question with lower power. The research proposal should directly address how the preliminary study will advance the design of a subsequent definitive clinical trial for efficacy.

Examples of relevant clinical research for this mechanism include, but are not limited to, the following:

Note that in each of the above examples, the study is designed to provide specific data that will be essential to design the subsequent definitive efficacy trial. In many cases, a control group is not necessary, nor preferable in order to achieve these objectives.

- Studies to optimize the intervention strategy (e.g., dose, duration and frequency of dosing). For example, studies designed to investigate dose-concentration, dose-response, or concentration-response relationships may contribute to optimal dosage selection for definitive trials.
- Studies to assess the appropriate delivery system or parameter settings of an electronic device or surgical technique.
• Studies to assess the safety and tolerability at various doses or concentrations of a specific intervention or studies designed to select the best of two or more potential interventions or dosing regimens to evaluate in a subsequent definitive trial, based on tolerability or evidence of biological activity.

• Studies to identify improved biomarkers for clinical prognosis

• Studies to address compounds or interventions that may enhance quality of life

**BUDGET:** A maximum of $150,000/year for a maximum of 2 years

No indirect costs will be paid for these awards

### B. Clinical Management Grants

The aim of the TREAT ALS Clinical Management Grant Program is to improve care and living with ALS with a focus on clinical, psychological and/or social management of ALS. Examples of relevant clinical management studies for this mechanism include, but are not limited to, the following:

• Studies that address the gaps in the delivery of care (as outlined in the ALS practice parameters).

• Studies that explore and develop telemedicine for the care of individuals with ALS.

• Psychological interventions in ALS to address the significant mental health issues facing ALS patients and caregivers.

• Nutritional and respiratory intervention

• Studies to address the needs of caregivers for patients with ALS and cognitive/behavioral impairment.

**BUDGET:** A maximum of $100,000/year for a maximum of 2 years

No indirect costs will be paid for these awards

### APPLICATION RECEIPT DATES FOR BOTH A. CLINICAL PILOT STUDIES AND B. CLINICAL MANAGEMENT GRANTS:

- Brief study outline: May 28, 2012
- Request to submit full application: June 29, 2012
- Submission of full application: September 10, 2012
- Notification of award: November, 2012
- Funds begin on receipt of all relevant signatures
REVIEW CRITERIA FOR BOTH A. CLINICAL PILOT STUDIES AND B. CLINICAL MANAGEMENT GRANTS.

- Significance
- Approach
- Innovation
- Investigator
- Environment
- Appropriateness of Budget

(1) SIGNIFICANCE: Does this study address an important problem? Has the applicant addressed both the significance of the eventual definitive clinical trial AND the significance of this study in providing knowledge needed to proceed to the definitive clinical trial? Is there a sufficient body of high quality preclinical or clinical research that supports the rationale for the proposed study? What is the potential impact of the proposed intervention on health care and quality of life? If the aims of the study are achieved, how will these results contribute to the design and implementation of the definitive clinical trial? For Clinical Management Grants, how will the findings impact the future care of patients? What are the hurdles to implementing the findings and how might they be overcome?

(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 tactics?

For the proposed preliminary study, reviewers will evaluate whether the approach is adequate in each of the following areas:

- The scientific soundness of the proposed methodology, such as translation of the clinical question into statistical hypotheses, selection of outcome measure(s), inclusion and exclusion criteria, plans for identifying, screening, and enrolling subjects, plans for randomization and masking, if appropriate, implementation of intervention (including determination of treatment dose), and plans for follow-up of patients;

- The soundness of plans for analysis of the primary and secondary questions, including the adequacy of the target sample size for achieving the goals of the study;

- The completeness and quality of the protocol and standardized procedures that will be used for this study;

- The ethical aspects of the study, including risks and benefits to patients, the adequacy of subject education and consent procedures, and safety protections;

- The adequacy of plans for safety monitoring of the study;

- The adequacy of data management and quality control procedures.

(3) INNOVATION: Are the aims of the study original? While the proposed study design, methods, and interventions may not necessarily be innovative, the underlying aims should represent an advancement in the field.
(4) INVESTIGATOR: Is the investigator appropriately trained and well suited to carry out this work? Is the work proposed appropriate to the experience level of the principal investigator and other researchers (if any)? The reviewers should consider training and expertise in the clinical problem and the proposed intervention, and training and expertise in clinical trials. If a multicenter study is proposed, the reviewers should also evaluate the investigator's ability to organize and manage the research group.

(5) ENVIRONMENT: Does the scientific environment in which the work will be done contribute to the probability of success? Do the proposed experiments take advantage of unique features of the scientific environment or employ useful collaborative arrangements? Is there evidence of institutional support? Have appropriate agreements with participating industry sponsors, if any, been established?

(6) BUDGET: The reasonableness of the proposed budget and the requested period of support in relation to the proposed research.