Funding Opportunity: The Lawrence and Isabel Barnett Drug Development Program

Table of Contents

Article I. Overview Information 1
Section 1.01 Funding Opportunity in Brief 1
Section 1.02 Key Dates 1
Section 1.03 Grants Policy Statement 2
Article II. Full Text of the Announcement 2
Section 2.01 Funding Opportunity Description 2
Section 2.02 Award Information 3
Section 2.03 Eligibility 3
Section 2.04 Content and Review of Applications 4
Section 2.05 Application and Submission Information 5
Section 2.06 Award Administration Information 7
Section 2.07 ALS Association Contacts 7

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Article I. Overview Information

Section 1.01 Funding Opportunity in Brief

Title: The Lawrence and Isabel Barnett Drug Development Program

Award: $500,000 in total funding across two years for the preclinical assessment of therapeutics for ALS

Section 1.02 Key Dates

Request for Application Open: February 23, 2022
Letter of Intent Due Date: March 30, 2022, 5 p.m. US ET

Full Application Due Date (by invite only): June 1, 2022, 5 p.m. US ET
Anticipated Award Decision: July 2022
Anticipated Earliest Start Date: August 2022

Our expectation is that contracting is completed within 60 days of award offer. If not, we reserve the right to rescind the award offer and redirect the funds to other projects.

Section 1.03 Grants Policy Statement

For more detail on all policies described in this document, please consult our Grants Policy Statement, available here.

Article II. Full Text of the Announcement

Section 2.01 Funding Opportunity Description

(a) Overview

There is an urgent need for new and improved therapies for ALS, as there is still no cure. The Lawrence and Isabel Barnett Drug Development Program is open to industry and academic investigators proposing to develop novel or repositioning approaches for ALS. The Association seeks applications for the preclinical assessment of therapeutics for ALS that have a high probability of reaching the clinic within three years.

(b) Topics of Interest

Both novel programs and repurposing of approved or clinically safe therapies from other disease indications are appropriate for this RFA. Studies can be performed by a contract research organization in collaboration with the PI. We are especially interested in drug discovery projects supporting the following:

- In vivo efficacy testing of therapeutic candidates (pharmacological treatments, biological therapies, or gene therapy) in models of ALS.
- Studies on pharmacokinetics, pharmacodynamics, preclinical toxicology/safety (ADME/Tox), dose-range finding, and target engagement.
- Other IND enabling studies

Successful applications will provide strong and compelling data supporting the biological rationale for the proposed treatment and a clear plan, including essential “Go/No-Go” decision milestones, for moving the approach through the essential stages of development.

Please Note: Clinical trials will not be supported with this funding opportunity. Early drug discovery studies proposing high-throughput screening, hit-to-lead, and lead optimization will

also not be supported through this funding opportunity. **Applications focusing on these topics will be administratively withdrawn.**

Section 2.02 Award Information

(a) Funding Instrument

- Grant: A support mechanism providing money to an eligible entity to carry out an approved project or activity.
- If the funded research contributes to revenue generation, the Association expects to share, proportionally, in that revenue (see Grants Policy Statement for details).

(b) Funds Available and Anticipated Number of Awards:

- The number of awards is contingent upon The ALS Association's budget allocation and the submission of a sufficient number of meritorious applications.

(c) Award Budget

- Budgets for total costs up to $500,000 (inclusive of both direct and indirect costs) may be requested (up to $250,000 per year across two years).
- Indirect costs are limited to 10% of total direct costs.

(d) Award Period of Performance:

- The maximum period of performance is two (2) years.

Section 2.03 Eligibility

(a) Eligible Individuals

- Individuals with the skills, knowledge, and resources necessary to carry out the proposed research may apply as a principal investigator.
- Post-doctoral fellows are not eligible to apply as a principal investigator.

(b) Eligible Organizations

- U.S. and non-U.S. biotechnology/pharmaceutical companies, or other publicly or privately held for-profit entities.
- U.S. and non-U.S. public and private non-profit entities, such as universities, colleges, hospitals, laboratories, units of state and local governments and eligible agencies of the federal government.

(c) Collaborations

- The preclinical drug development process often requires resources beyond those available at a single organization. Therefore, applications are open to investigators participating in synergistic collaborations focused on testing and developing lead agents for the treatment of ALS.
- If a collaboration is proposed, letters confirming/supporting the collaboration are **required**. Specific roles and responsibilities for each collaborator need to be clearly

articulated. If the collaboration is multi-organizational, participating organizations will ensure the success of the collaboration by resolving potential intellectual and material property issues and by removing organizational barriers that might interfere with achieving high levels of cooperation.

Section 2.04 Content and Review of Applications

(a) Content of Letter of Intent

By the date listed in Key Dates above, prospective applicants are required to submit a letter of intent that includes the following information:

- Descriptive title of proposed activity
- Name, address, and telephone number of the Principal Investigator
- Names of other key personnel
- Participating Institution(s)
- Number and title of this funding opportunity
- A scientific rationale for the proposed study and its relevance to ALS
- A brief description of the study hypothesis and/or objectives and overall experimental plan to meet those objectives

(b) Review Criteria for Letter of Intent

- **Fit**: The letter of intent should allow reviewers to assess whether or not there is a good match between The ALS Association's interests and the project.
- **Relevance & Rationale**: The Letter of intent should provide appropriate scientific rationale for the proposed study and its relevance to ALS.
- **Overall Experimental Objectives**: The letter of intent should provide study hypothesis and/or objectives and overall experimental plan to meet those objectives.

The letter of intent can either be accepted or declined. If the letter of intent is accepted, the applicant will be invited to submit a full application. Results from the letter of intent review phase will not be provided to the applicant.

(c) Content of Full Application

Application instructions for the full application will be provided when/if the applicant is invited to submit the full application.

(d) Review Criteria for Full Application

- **Impact/contribution to ALS:** Potential for the proposed studies to make an important contribution to ALS therapeutic development and how does the approach fit the current landscape of ALS treatments in development.
- **Target/Pathway:** Rationale for developing compounds against the given target/pathway.
- **Research Plan:** Feasibility, timeline and design of experiments, and whether the development plan will result in go-no go decisions in moving the therapeutic forward.

- **Research Team:** Qualifications of the PI, key personnel, and collaborators/consultants are appropriate to perform the proposed research project.
- **IP/Patent landscape:** Impact on future development/commercialization of the therapeutic.

(e) Peer Review and Selection Process

- All applications are peer reviewed by an ad hoc Scientific Review Committee constituted by The ALS Association
- The Scientific Review Committee's priority scores are forwarded only to The ALS Association Board of Trustees, which has the sole authority for approving the funding of research grants
- For applications selected for funding, The ALS Association will send the reviewer comments to the PI. To the extent practical and within the scope of the budget, The ALS Association recommends that the PI integrate any recommendations that the reviewers may have suggested to further optimize the project and outcomes.

Section 2.05 Application and Submission Information

(a) Format of Application Submission

- All applications are to be submitted through our online grants management platform, Proposal Central: https://proposalcentral.com/
- To find this funding opportunity on Proposal Central, navigate to the Grant Opportunities tab (https://proposalcentral.com/GrantOpportunities.asp) and search for Grant Maker: The ALS Association
- Instructions on how to register as a new user of Proposal Central: https://docs.proposalcentral.com/RegUser.pdf
- Applications must be prepared according to Proposal Central's instructions: https://docs.proposalcentral.com/CreateApp.pdf
- Upon receipt, applications will be evaluated for completeness and compliance with application instructions. Applications that are incomplete, non-compliant and/or nonresponsive will not be reviewed.

(b) Funding Restrictions

- The ALS Association awards are subject to the terms and conditions described in The ALS Association Grants Policy Statement (see: Grants Policy Statement) and the award Agreement.
- Awards will be limited $500,000 total costs for the entire 2-year period of performance.
- Salary and wages and fringe benefits of personnel other than the salary of the Principal Investigator are allowed
- Salary and wages and fringe benefits of the Principal Investigator are allowed only when indirect costs are waived
- Direct salary for individuals not to exceed the salary limitation for Executive Level II of the Federal Executive pay scale provided at https://grants.nih.gov/grants/policy/salcap_summary.htm.

- Indirect costs are limited to 10 percent of total direct cost
- Economy travel up to $2,000 per year to attend scientific conferences is allowed
- Moveable equipment costs not to exceed 20 percent of the annual budget
- Computer hardware and software costs up to $2,000
- Recurring annual cost-of-living/inflationary increase up to 3% for personnel and consumable supplies
- All funds must be expended within the approved period of support
- All unexpended funds remaining at the end of the project must be returned to The ALS Association

(c) Administrative and National Policy Requirements

All ALS Association grants include The ALS Association Grants Policy Statement (see: Grants Policy Statement) as part of the Research Grant Agreement. The Grants Policy Statement includes the requirements applicable to animal welfare, human subject protections, data sharing, research resource sharing, publications, etc. All necessary ethical and regulatory approvals must be in place before experiments are initiated.

- **Animal Welfare Requirements:** The ALS Association requires its grantees to establish appropriate policies and procedures to ensure the humane care and use of animals. Domestic grantees also bear the ultimate responsibility for compliance with The *Public Health Service (PHS) Policy on Human Care and Use of Laboratory Animals.* Foreign grantees also bear the responsibility to comply with applicable laws, regulations, and policies of the jurisdiction in which the research will be conducted, and a commitment to follow the *International Guiding Principles for Biomedical Research Involving Animals* developed by the Council for International Organizations of Medical Sciences.
- **Human Subject Requirements**: The ALS Association requires its grantees to establish appropriate policies and procedures to ensure the protection of human subjects used in research supported by The ALS Association. The ALS Association grantee must comply with the Department of Health and Human Services (HHS) regulation Title 45 Code of Federal Regulation Part 46. Clinical research involving investigational drugs and devices or products regulated by the Food and Drug Administration (FDA), must comply with all FDA requirements in 21 CFR Parts 50, 56, 312 and 812.
- **Health Insurance Portability and Accountability Act (HIPAA):** The ALS Association requires its domestic grantees to comply with the requirements of HIPAA to ensure the protection of individually identifiable health information for ensuring confidentiality of patient records.
- **General Data Protection Regulation (E.U. 2016/679):** The ALS Association requires its foreign grantees to comply with the Regulation E.U. 2016/679, or its amended regulation, of the European Parliament and of the Council of 27 April 2016 on the protection of natural persons with regard to processing of personal data and on the free movement of such data.
- **Intellectual Property/Royalty**: Grantees shall have the first right, but not the obligation, to prepare, file, prosecute, and maintain all registerable rights in Intellectual Property arising from implementation of the Research Scope of Work at its sole expense. If the

funded research contributes to revenue generation, the Association expects to share, proportionally, in that revenue (see Grants Policy Statement for details).

Section 2.06 Award Administration

(a) Award Agreement and Payment Schedule

- A formal notification in the form of an ALS Association Research Grant Agreement (Agreement) is the authorizing document and will be provided to the applicant organization for successful applicants.
- The Agreement, signed by the applicant's authorized organizational representative (AOR), principal investigator and The ALS Association, will include the negotiated terms and conditions of the award between The ALS Association and the Grantee.
- Milestone driven award payments will be made to the Grantee on an annual or semi-annual basis as outlined within the Agreement.

(b) Administrative Requirements

- The Grantee is expected to utilize The ALS Association funds in direct support of the research project and expend funds in accordance with the established organizational policies and procedures.
- Funds charged to The ALS Association award must be for allowable project costs that are determined to be reasonable, allocable, consistently applied and conform to the program guidelines and/or limitations outlined in The ALS Association Funding Opportunity Announcement and the Agreement.
- The Grantee is expected to seek The ALS Association's prior approval before making certain types of post-award changes.
- Requests for prior approval should be submitted via email to researchgrants@alsa-national.org at least 30 days before the proposed change.

(c) Reporting Requirements

- The Grantee will be required to submit semi-annual Research Progress Reports and annual Grant Expenditure Reports during the period of an award unless otherwise noted in the Agreement.
- Future grant payments are contingent upon the grantee's submission of and The ALS Association's acceptance of the report(s).
- The ALS Association's issuance of the next award payment, when applicable, serves as confirmation that the information provided has been reviewed and approved by The ALS Association.
- Final closeout reports (i.e., final research progress and expenditure reports) are due within 90 days following the termination date of the Agreement.

Section 2.07 ALS Association Contacts

We encourage inquiries concerning this funding opportunity and welcome the opportunity to answer questions from potential applicants. Submit all correspondence to researchgrants@alsa-national.org.