December 2023

Technical Assistance Webinar

Amyotrophic Lateral Sclerosis (ALS) Intermediate Patient Population Expanded Access (U01 Clinical Trial Required) RFA-NS-24-029

NIH National Institute of Neurological Disorders and Stroke

Webinar Overview

Topics

- Purpose and Background
- Key Requirements & Considerations
- Eligibility Information
- How to Apply - Resources and Tips
- Peer Review
- Cooperative Agreement Awards
- Sharing of Biospecimens
- NIH Data Management and Sharing (DMS) Policy
- Q & A

Presenters/Moderator

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Purpose and Background
RFA-NS-24-029

Purpose and Background

- Conduct of **scientific research** utilizing data **from expanded access** (EA) for investigational drugs or biological products
- EA for intermediate size populations of patients living with amyotrophic lateral sclerosis (ALS) who are **not eligible for ongoing clinical trials** for the prevention, diagnosis, mitigation, treatment, or cure of ALS.
- FDA considers an **intermediate-size patient population** for an EA IND to be when more than one patient, but generally fewer patients than are treated under a typical treatment IND or protocol, will be treated with an investigational drug/biological product under the EA (21 CFR 312.315).

Key Dates

- Letter of Intent Due Date: January 22, 2024 – **note: a Letter of Intent is not required but helpful to the NINDS**
- Application Due Date: February 22, 2024
- Scientific Merit Review: March 2024

An Act

To direct the Secretary of Health and Human Services to support research on, and expanded access to, investigational drugs for amyotrophic lateral sclerosis, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Accelerating Access to Critical Therapies for ALS Act".

SEC. 2. GRANTS FOR RESEARCH ON THERAPIES FOR ALS.

(a) IN GENERAL.—The Secretary of Health and Human Services (referred to in this section as the "Secretary") shall award grants to participating entities for purposes of scientific research utilizing data from expanded access to investigational drugs for individuals who are not otherwise eligible for clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis. In the case of a participating entity seeking such a grant, an expanded access request must be submitted, and allowed to proceed by the Secretary, under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and part 312 of title 21, Code of Federal Regulations (or any successor regulations), before the application for such grant is submitted.

Key Requirements & Considerations
RFA-NS-24-029

- **Expanded Access (EA) New Drug Application (IND):**
  - Applicants must provide documentation such as a "may proceed" email or letter from the FDA prior to funding.
  - An intermediate-size patient population EA IND application must be submitted to the FDA prior to, or at a minimum by the day of submission of the grant application to the NINDS.
- **The investigational drug or biological product must not be approved under a New Drug Application (NDA) or licensed under a Biologics License Application (BLA).**
- **Institutional Review Board (IRB) approval of the protocol and the informed consent documents**
  - Not required at the time of application submission
  - Required prior to funding and the initiation of treatment of patients
- **Follow NINDS guidelines for monitoring clinical trials:** available here
- **Describe scientific research goal:** The EA protocol will provide patients treatment access to the investigational drug or biological product and generate data that will support research or development related to the prevention, diagnosis, treatment of ALS.
- **Describe how the proposed EA program will be designed not to interfere with patient enrollment in ongoing clinical trials for investigational therapies for the prevention, diagnosis, mitigation, treatment, or cure of ALS.**
- **Establish relationships with patient advocacy groups** to solicit input on recruitment, clinical meaningfulness of the question under study, relevance of the proposed clinical outcomes, and approaches to minimizing the burden on study participants.

Eligibility Criteria
RFA-NS-24-029

Eligible applicants must be **clinical trial sites that participate in a phase 3/efficacy clinical trial supported by a small business concern** that is the FDA-designated sponsor of a drug or biological product which is the subject of an IND under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) **to prevent, diagnose, mitigate, treat, or cure ALS.** (See ACT for ALS Sec. 2(e))

An intermediate-size patient population **Expanded Access IND application must be submitted to the FDA** prior to, or at a minimum no later than on the same day of, submission of the grant application to the NINDS (See ACT for ALS Sec. 2(a))

Additional Eligibility Information

Clinical Trial eligibility:

- **Phase 2/3 trials, ongoing phase 3 trials, and concluded phase 3 trials are eligible *until a regulatory determination has been made regarding approval***. The investigational drug proposed under RFA-NS-24-029 must not be approved under a New Drug Application (NDA) or licensed under a Biologics License Application (BLA).
- The **IND for the ALS phase 3/efficacy clinical trial must be active at the time of award** for applications selected for funding under RFA-NS-24-029.

Drug Sponsor eligibility:

- ACT for ALS defines a small business concern according to (15 U.S.C. 632(a)) Sec. 3(a)
  - ***Note that NINDS cannot determine whether the sponsor is a qualifying small business concern.*** The drug/biological product sponsor will be required to certify eligibility as a small business concern prior to award.
- **The small business concern drug sponsor cannot apply directly to RFA-NS-24-029**

How to Apply – Resources
RFA-NS-24-029

NIH GRANTS & FUNDING
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Home » About Grants » How To Apply Application Guide

How to Apply - Application Guide

Use the application instructions found on this page along with the guidance in the funding opportunity announcement to submit grant applications to NIH, the Centers for Disease Control and Prevention, the Food and Drug Administration, and the Agency for Healthcare Research and Quality.

Prepare to Apply

- Systems and Roles
- Register
- Find Grant Funding
- Understand Funding Opportunities
- Types of Applications
- Submission Options
- Obtain Software

Write Application

- Write Your Application
- How to Find Forms
- Develop Your Budget
- Format Attachments
- Rules for Text Fields
- Page Limits
- Data Tables
- Reference Letters
- Biosketches

Submit

- How to Submit, Track, and View
- How We Check for Completeness
- Changed/Corrected Applications
- Standard Due Dates
- Submission Policies
- Dealing with System Issues

FAQs

How to Apply – Tips
RFA-NS-24-029

- ✓ **Read the Notice of Funding Opportunity carefully**
- ✓ **Follow the SF424 (R&R) Application Guide**
- ✓ **Stick to page limits for U01 grant applications**
- ✓ **Include budget sheets for all years of the grant**
- ✓ **Include Data Management and Sharing Plan (within the "Other Plan(s)" field on the PHS 398 Research Plan)**
- ✓ **Feel free to reach out to Program staff at NINDS**

424 R&R and PHS-398 Specific
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Peer Review of Applications

- Applications submitted in response to the RFA will be evaluated for scientific and technical merit through the NIH peer review system.
- The peer review mission aims to see that all grant applications receive fair, independent, expert, and timely scientific reviews—free from inappropriate influences—so NIH can fund the most promising research.
- Only the review criteria described in section V in RFA will be considered in the review process. We are using the five standard criteria listed here, and additional criteria specific to the RFA can be found in the RFA.
- Reviewers will consider each of the review criteria in the determination of scientific merit and give a separate score for each.
- The review panel will include appropriate scientists, clinicians, and persons affected by ALS.

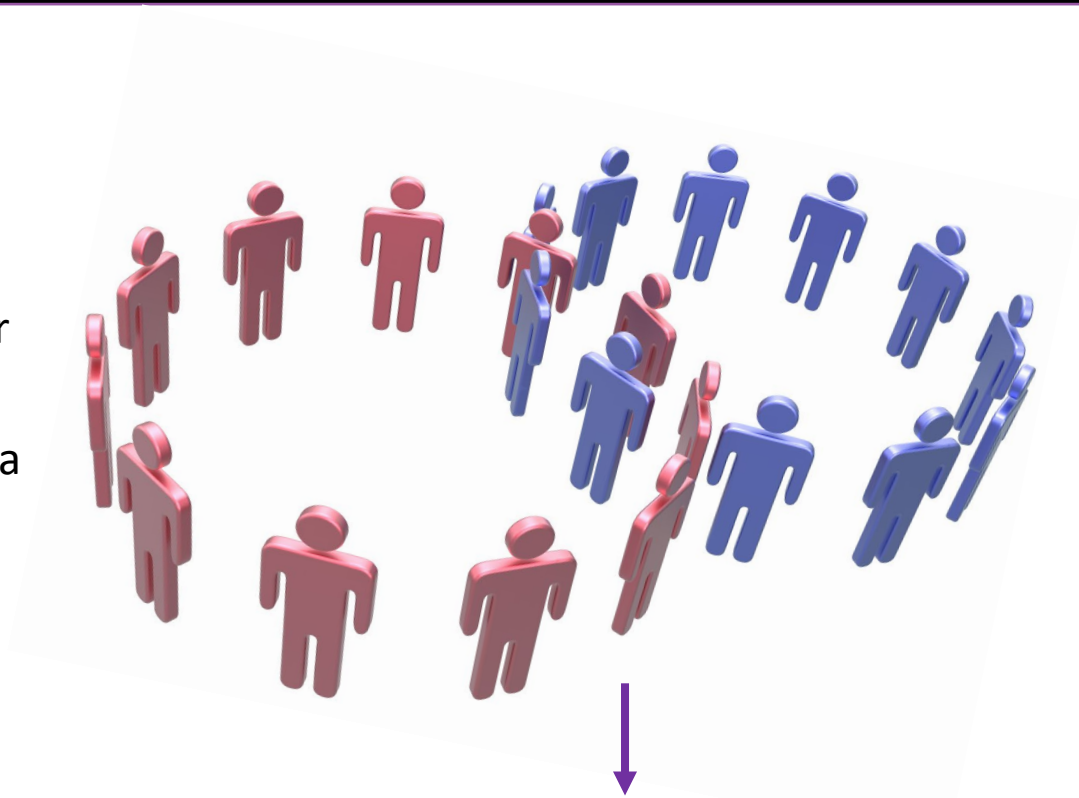
Cooperative Agreement Awards

- Cooperative agreement awards (U-series awards) are a support mechanism NIH frequently uses for high-priority research areas that require substantial involvement from NIH program/scientific staff.
- Active Partnership:
  - ✓ NIH program/scientific staff will participate in project activities; however, NIH staff will not play a dominant role nor assume direction or primary responsibility for awardee activities.
  - ✓ More frequent communication
  - ✓ Connect award recipients with resources across NIH to resolve potential challenges/barriers

Cooperative Agreement Awards – Roles & Responsibilities

PD's/PI's

- ✓ Overall scientific and administrative leadership
- ✓ Primary responsibilities for the project as a whole
- ✓ Reporting recruitment data to the NINDS Recruitment Planning & Monitoring System (RPMS)
- ✓ Publish and publicly disseminate results, data and other products



Areas of Joint Responsibility:

- ✓ Access to data generated
- ✓ Provide scientific/programmatic support
- ✓ Oversee adverse event management
- ✓ Monitor progress of study milestones

NIH/NINDS staff

- ✓ Assist, guide, coordinate, or participate in project activities
- ✓ Project Scientist: substantial programmatic involvement (part of your team)
- ✓ Administrative Program Director/Official: programmatic stewardship of the award
- ✓ Program Official: NINDS liaison to the DSMB

Sharing of Biospecimens — RFA-NS-24-029

- All biospecimens must come from study participants who have consented to banking and sharing broadly with academia and industry
- Costs for the biospecimen collection (collection kits, shipping kits to sites, shipping samples to the repository) are borne by the grantees

Biofluids:

- BioSEND will serve as the biofluid repository
- Request a quote from BioSEND and include this information in the application

Cells:

- The NINDS Human Cell and Data Repository (NHCDR) will serve as the human cell repository
- Request a quote from NHCDR and include this information in the application

NIH Data Management and Sharing (DMS) Policy

- ✓ All applications must include a DMS Plan according to the 2023 DMS policy
- ✓ Include the DMS Plan within the "Other Plan(s)" field on the PHS 398 Research Plan
- ✓ NIH has developed an optional DMS Plan format page that aligns with the recommended elements of a DMS Plan
- ✓ Investigators may request allowable funds toward DMS in the budget section of their applications
- ✓ Detailed information can be found at the NIH DMS policy website "Data Management and Sharing Policy | Data Sharing (nih.gov)"

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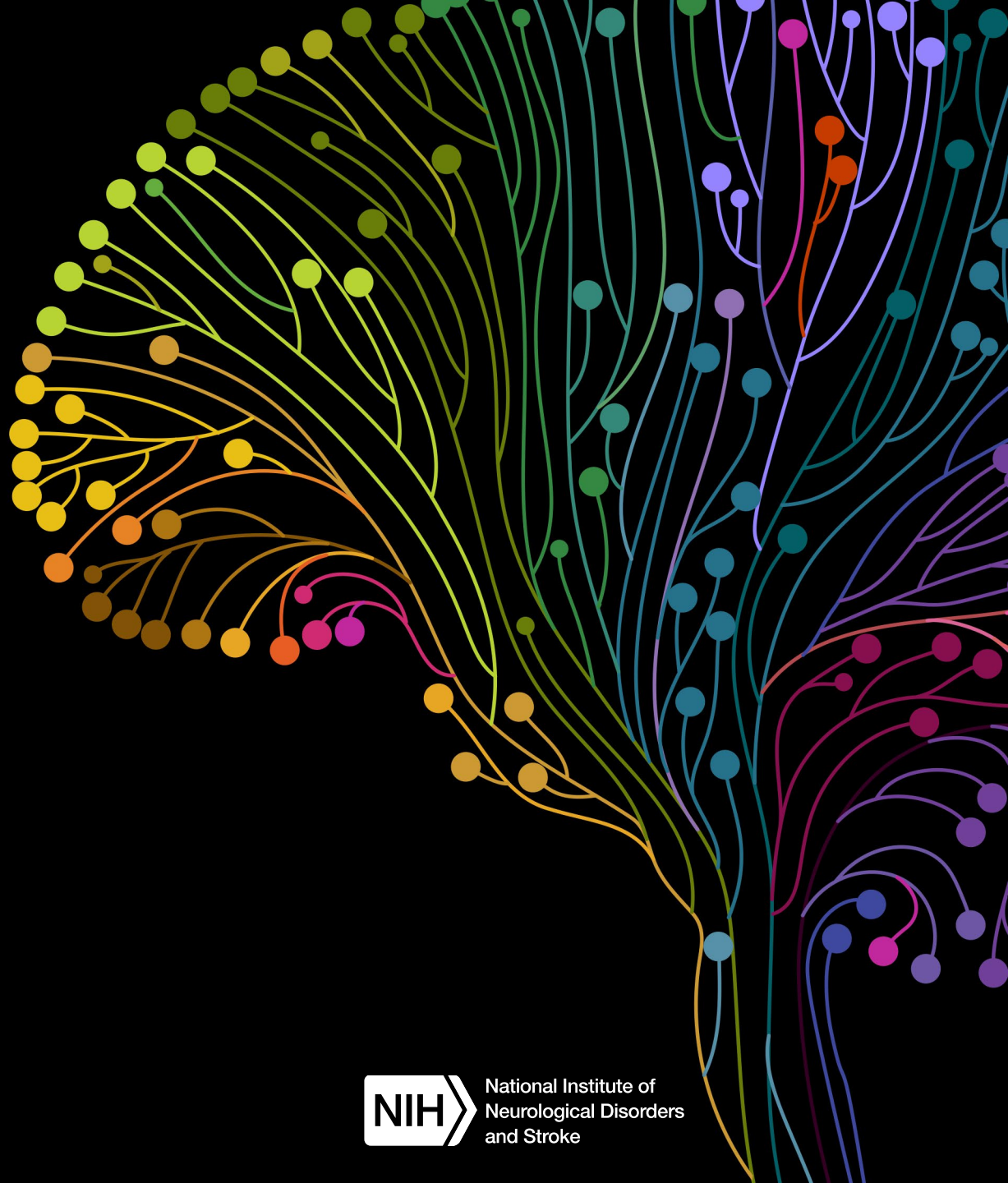
Questions & Answers



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