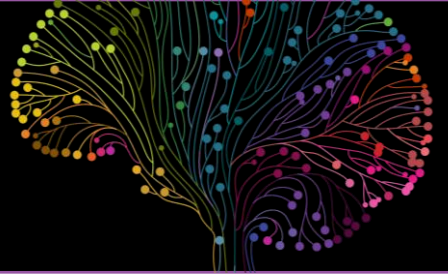
Expanded Access Trials in ALS

Amy Tsou, MD, MSc

January 2024

National Institute of Neurological Disorders and Stroke

Overview



- **ACT for ALS**
- **NINDS funding for expanded access in ALS**
- **Review process and involvement of patients/people with lived experience (PWLE)**
- **Unique opportunities with expanded access ALS trials**

Access to Critical Therapies for ALS Act

On December 23, 2021, President Biden signed into law the Accelerating Access to Critical Therapies for ALS Act, more commonly known as the **ACT for ALS**

The law sunsets September 2026.

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.

https://www.congress.gov/117/plaws/publ79/PLAW-117publ79.pdf

Expanded Access

FDA U.S. FOOD & DRUG ADMINISTRATION

← Home / News & Events / Public Health Focus / Expanded Access

" a potential pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available."

Regulated by the FDA

Requires sponsor to file an **expanded access investigational new drug (IND)** application

Information for Patients, Physicians and Industry

Patients	Learn about expanded access, including information about the expanded access process, what FDA considers, and what costs may be involved.
Physicians	Learn about expanded access, including information about the different types of expanded access, how to submit expanded access requests, and reporting requirements.
Industry	Learn about expanded access, including information about posting your expanded access policy, how to submit expanded access requests, and reporting requirements.
Forms	Learn about how to complete and submit forms needed for each type of expanded access request.

https://www.fda.gov/news-events/public-health-focus/expanded-access

NIH Funding for Expanded Access Trials in ALS

Drug/Biological Product

- Must have an active Phase 2/3 or Phase 3 clinical trial (assessing how effective it is)

Applicant

- Must be one of the clinical sites for the Phase 2/3 or Phase 3 clinical trial

Sponsor

- Must meet criteria as a small business (criteria set by Small Business Act)

Proposed Study

- Should support **scientific research** utilizing data **from expanded access** (EA) for investigational drugs or biological products
- Should address EA for an **intermediate-size patient population** (defined by the FDA) to be treated with an investigational drug/biological product.
- Should provide EA for patients living with amyotrophic lateral sclerosis (ALS) **not eligible for ongoing clinical trials** for the prevention, diagnosis, mitigation, treatment, or cure of ALS.

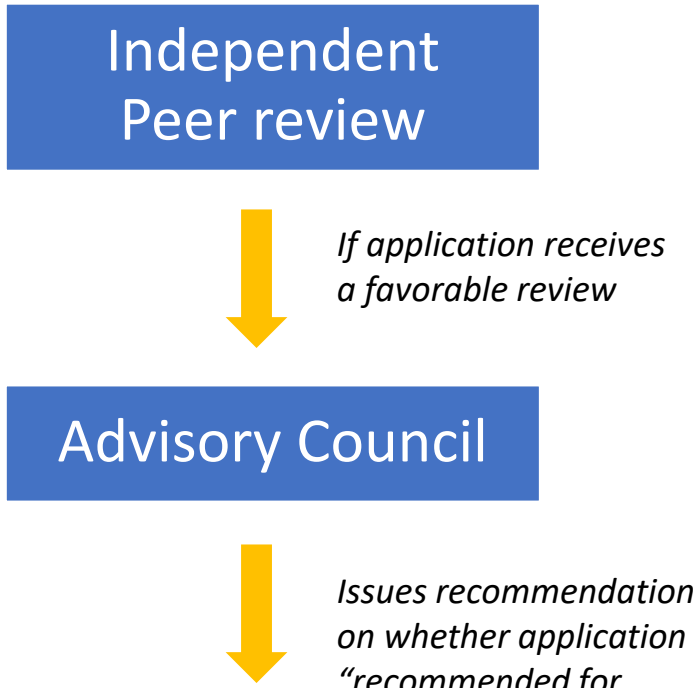
Accepting applications through February 22, 2024

Technical Assistance Webinar: https://www.ninds.nih.gov/news-events/events/informational-webinar-applicants-ninds-als-expanded-access-u01-12122023

RFA-NS-24-029 https://grants.nih.gov/grants/guide/rfa-files/RFA-NS-24-029.html

National Institute of Neurological Disorders and Stroke

NIH Review Process



How are patients/people with lived experience (PWLE) involved?

- Applicants are encouraged to seek input from people with ALS to inform study design and outcomes
- PWLEs provide input as part of peer review along with other experts

ACT for ALS: Expanded Access Research for ALS

FY 2022

- **An Expanded Access Protocol of Intravenous Trehalose Injection 90 mg/mL Treatment of Patients with ALS**
  - Collaboration between MGH, 20+ enrollment sites, and Seelos Therapeutics

FY 2023

- **An Intermediate-Size Expanded Access Protocol for ALS with Pridopidine**
  - Collaboration between MGH at Harvard University, Prilenia Therapeutics, Inc., 45 clinical sites within HEALEY Platform, and 200 participants
- **An Intermediate-Size Expanded Access Trial of Autologous Hybrid TREG/Th2 Cell Therapy (RAPA-501) of ALS**
  - Collaboration between MGH at Harvard University, Rapa Therapeutics, LLC, 7 clinical sites, and 40 participants
- **An Intermediate-Size Expanded Access Protocol for CNM-Au8 in ALS**
  - Collaboration between Columbia University and Clene Nanomedicine, 10 clinical sites, and 100 participants

NIH

EA Research: Unique opportunities

- Capture “**real world**” clinical and safety data (for example, data patients on stacked therapies)
- Capture biomarkers and natural history data from patients **previously missing** from clinical trials
- Pilot **new methods** (remote data collection, recruitment from community clinics)
- Data captured in **centralized knowledge portal**, available to researchers and the public, supporting future research

ALS Team at NINDS

Division of Clinical Research (Extramural Research)

Lumy Sawaki-Adams, MD, PhD: lumy.sawaki-adams@nih.gov

Amy Tsou, MD, MSc: amy.tsou@nih.gov

Division of Neuroscience (Extramural Research)

Amelie Gubitz, PhD: gubitza@nih.gov (ALS & ALS/FTD)

Frank Shewmaker, PhD: frank.shewmaker@nih.gov (FTD & ALS/FTD)

Division of Translational Research (Extramural Research)

Emily Caporello, PhD: emily.caporello@nih.gov (SBIR/STTR)

Office of Neuroscience Communications & Engagement

Rebekah Corlew, PhD: rebekah.corlew@nih.gov (Patient Engagement Strategist)

Office of Science Policy & Planning

Christine Torborg, PhD: christine.torborg@nih.gov

Elio Peraza, MS: elio.peraza@nih.gov

Visit the **NINDS Focus on ALS** webpage to learn more about ALS and join the **NINDS ALS Listserv** for ALS-related updates on current activities, research opportunities, and NIH-funded science advances

Link to **NINDS Focus on ALS** webpage

National Institute of Neurological Disorders and Stroke

Thank You!