ALS United

Together, We End ALS®

Reauthorize ACT for ALS: Accelerate Treatment Development and Protect Expanded Access

Overview

ALS is a rapidly progressive, fatal disease and for most people living with ALS, treatment options remain limited. Families don't have the luxury of waiting years for the traditional drug development timeline. That's why Congress enacted the **Accelerating Access to Critical Therapies for ALS Act** (**ACT for ALS**) in 2021, which established a practical, patient-centered approach to speed progress on two fronts:

- Building shared data infrastructure needed to move promising treatments to faster approval
- Expanding access to investigational therapies for people who can't enroll in trials, while collecting key evidence in the process

Congress must reauthorize the law to protect these programs, sustain momentum, and avoid disruption for patients and researchers.

Open Data and Research Infrastructure

ACT for ALS built the collaboration and data backbone that ALS research has long lacked. Specifically, it created three connected platforms plus a competitive grant program that make it easier to find targets, run studies, and move therapies toward approval faster:

- **AMP-ALS: Open Access 'Knowledge Portal.'** ACT for ALS established a partnership with the Foundation for the NIH (FNIH) to create the Accelerating Medicines Partnership for ALS (AMP-ALS), aligning federal agencies, academic scientists, companies, and nonprofits around shared data and shared priorities. A core output is the ALS Knowledge Portal, which provides open access to existing clinical datasets and integrates new data and biosamples as they are generated, supporting researchers worldwide.
- **CP-RND: Shared Data Standards and Drug-Development Tools.** The partnership between Critical Path for Rare Neurodegenerative Diseases (CP-RND) and the FDA is developing common data standards and fit-for-purpose tools to help the agency evaluate ALS treatments more efficiently—including biomarkers, modeling platforms, and clinical outcome assessments. Biomarkers are especially important because they can help characterize disease biology, track progression, monitor response to a therapy, and inform target selection.
- **ALL ALS: Natural History + Biomarker Collection.** Using ACT for ALS funding, NIH launched the ALL ALS Consortium, which is conducting a large natural history study with 1,200+ participants enrolled across dozens of sites and collecting tens of thousands of samples. Those samples and associated data are deposited into the ALS Knowledge Portal, creating the largest open natural history resource in ALS and helping make research participation and resulting datasets more representative.
- **Rare Disease Grant Program.** ACT for ALS established a competitive Rare Disease Grant Program at NIH, designed to support research approaches, tools, and infrastructure relevant to ALS and other rare neurodegenerative diseases. These grants fund work such as data collection methods, biomarker development, and clinical research capabilities.

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Evidence-Generating Expanded Access Programs

Most people living with ALS never enroll in a traditional clinical trial, often because they are further along in disease, have complex medical needs, or cannot meet restrictive eligibility criteria. That means two things happen at once: people who most need options are left out, and the evidence base skews toward a narrower slice of the ALS population.

ACT for ALS addressed this gap by funding research-based **Expanded Access Programs (EAPs)** at NIH. These EAPs are designed to do more than provide access, they intentionally pair access with structured learning, generating data on patients who are routinely excluded from trials and helping the field understand how investigational therapies perform in real-world ALS populations.

To date, NIH has launched five EAPs nationwide, providing **over 700 people living with ALS** access to investigational therapies they otherwise could not receive. Early experience also shows promise for tele-EAP models that can reach patients who face geographic or mobility barriers. Importantly, lessons from these programs are informing the next stages of development—two therapeutic candidates are advancing to Phase 3 trials.

Why Reauthorization Matters

Without reauthorization, the EAPs providing access to hundreds of people living with ALS will wind down, the ALL ALS natural history effort will lose support, and the shared data and collaboration infrastructure that is accelerating ALS therapy development will be disrupted.

Reauthorization will:

- **Sustain evidence generation from the full ALS population.** Continued enrollment and data collection—especially from people typically excluded from trials—makes research and regulatory decision-making more representative and clinically meaningful.
- **Keep the national ALS research ecosystem coordinated.** ACT for ALS created the framework that aligns NIH, FDA, researchers, industry, and advocates; reauthorization prevents backsliding into siloed, slower development.
- **Protect national data and biospecimen resources.** Maintaining shared platforms and repositories ensures discoveries build on prior contributions instead of restarting efforts and losing momentum.

Congress must act swiftly ahead of the **September 30, 2026 deadline to reauthorize ACT for ALS** and continue funding these critical programs, which have already demonstrated their value. The very systems ACT for ALS created to collect data, coordinate research, and share knowledge will expire and risk losing years of progress in the fight against this deadly disease. Reauthorization keeps these national research engines available to advance therapeutic interventions, honors the contributions of those who have participated in these studies, and ensures that people living with ALS benefit from them.

ALS United is a nationwide partnership of independent nonprofit organizations dedicated to serving the ALS community. Guided by our vision "Together, We End ALS," we unite 15 member organizations across the country to create meaningful impact through research, care, and advocacy. Our collaborative network serves more than half of the U.S. ALS population with personalized, community-driven support. Visit alsunited.org for more information.