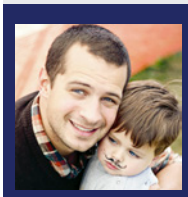
AMYOTROPHIC LATERAL SCLEROSIS RESEARCH PROGRAM

CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

MISSION: Fund impactful research to develop ALS treatments

Congressional Appropriations

FY07, FY09-FY24:
$269.4M total



"I was a pilot ...when I got sick. I had no genetic disposition, no family history of ALS. The ALSRP is a very promising program because it is focused on therapeutic ideas for the aggressive development of a treatment. I was blown away by the scientists working on this; they're trying to get us where we need to go. The ALSRP has really given me a lot of hope because I've learned that we know a lot more about what's going on than we did even last year.

Every year I go, I'm amazed at how much the body of knowledge has grown. It's exponential, so we're going to get there. I guarantee it."

Matt Bellina, former U.S. Navy Pilot, ALS Therapy Development Institute, FY14-FY16 Consumer Peer Reviewer

SCOPE OF THE PROBLEM[1]

30,000 people in the U.S. **live with ALS**

Average life expectancy is **2-5 years** from diagnosis

ALS is **always fatal**

No known therapies to effectively stop, significantly slow, or reverse progression

RELEVANCE TO MILITARY HEALTH[2]

1 in 6 people living with ALS are **Veterans**[3]

4,540 Veterans received care for ALS in 2020[4]

~1,055 Veterans with new onset ALS each year

- The Department of Veterans Affairs implemented regulations to establish a presumptive service connection for ALS
- Causes of the increased risk remain unknown; may result from chemical exposure, traumatic injury or viral infection

PROGRAM PRIORITIES

The program evaluates its priorities annually:

- Preclinical treatment discovery
- Preclinical treatment validation
- Better define ALS subtypes, therapeutic responses and prognosis
- Early-phase clinical trials to de-risk interventions and improve clinical care

[1] cdc.gov
[2] VHA directive 1101.07
[3] Weisskopf M, et al. *J. Neurol*, 64, no.1, 2005:32-37.
[4] Veterans Health Administration data

For more information, visit: **https://cdmrp.health.mil/alsrp/**

PROGRAM IMPACT AND OUTCOMES

The overall objective of the ALSRP is to expedite the pathway from bench science to clinical trials for new therapeutic approaches aimed at controlling or curing ALS.

The ALSRP invests congressional appropriations in distinct stages along the therapeutic development pipeline through stepwise translational award mechanisms.

International organizations are eligible to apply, and non-U.S. citizens are eligible to serve as principal investigators.

The ALSRP funds industry partners, non-profit organizations and more traditional academic research institutions.

Supporting Community Engagement

Equitable partnerships between researchers and community members and representatives

- The ALSRP encourages all investigators to consider collaboration with affected communities.
- The ALSRP requires effective and equitable collaborations with community partners in all clinical trials.

RESEARCH BREAKTHROUGHS — MAKING A DIFFERENCE

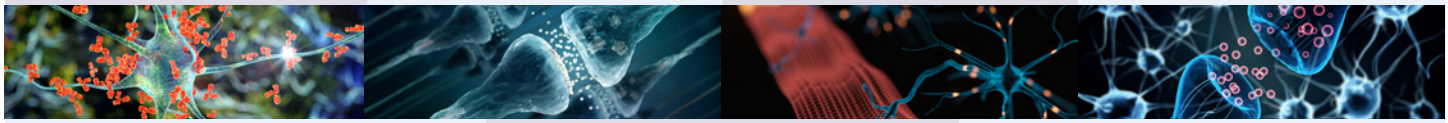
Treatment Approaches Advancing Through Clinical Trials

Tegoprubart: Humanized anti-CD40LG IgG antibody blocks damaging inflammation in motor neurons[5]

GDNF-Expressing Neural Progenitor Cells: Transplanted cells secrete growth factor GDNF, which protects and stimulates neurons[5]

Rasch-Built Outcome Measures to Improve Clinical Trials: Improved assessments of motor strength using a patient-reported questionnaire and an objective exam-based scale[5]

Prosetin: Oral therapy designed to revert cell stress and reduce damage to motor neurons[5]



Apilimod: Small molecule inhibitor to eliminate toxic protein aggregates that cause neurodegeneration[5]

Baricitnib: Assessing the safety and tolerability of this drug and biomarkers for responses to this medication[6]

Metformin: Adapting the FDA-approved drug into clinical use for ALS[6]

Treatment Ideas Advancing through Further Development

Combination Therapy: Increased penetration/bioavailability of FDA-approved riluzole in combination with the membrane pump inhibitor, Elacridar

microRNAs: Genetic manipulation of microRNAMir-155 to reduce inflammation

Drug Repurposing: Use of Duchenne muscular dystrophy drug, RASRx1902, to decrease neurological deficits and neuronal death

[5] ALSRP supported early stages of therapeutic development.
[6] ALSRP supported clinical trial.

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