The ALS Research Program (ALSRP)

Congressionally Directed Medical Research Programs
CDMRP
Department of Defense

Established in 2007, funding for the ALSRP is designated for innovative research projects that will help lead to the discovery of new treatments for ALS.

Since 2007, over $61 Million has been appropriated for the ALSRP at the Department of Defense. That funding has led to tremendous progress in ALS research, including:

- 700+ applications for ALSRP funding
- 44 projects funded FY07—FY15
- 25 peer-reviewed publications
- 4 drug candidates

2x

Our military heroes are twice as likely to develop ALS as the general population, regardless of the branch or era during which they served our country.

The ALSRP funds only the most promising pre-clinical research, which can then be used to development treatments for ALS.

Public Sector—Private Sector Partnership

The ALSRP Programmatic panel, which directs the program, is a partnership between the Department of Defense, Department of Veterans Affairs, the National Institutes of Health and the private sector. **The FY 2016 Programmatic Panel includes:**

Dr. Lucie Bruijn, The ALS Association
Dr. Lyle Ostrow, Johns Hopkins University
Dr. Amelie Gubitz, National Institute of Neurological Disorders and Stroke
Mr. Jim Humay, Les Turner ALS Foundation
Dr. Pierre Drapeau, University of Montreal
Major Carlos Maldonado, United States Air Force
Dr. John Ravits, University of California, San Diego
Dr. Larry Mink, The ALS Association
Dr. Bryan Traynor, National Institute on Aging
Dr. Kristy Lidie, Program Manager, CDMRP

Drug Candidates Developed through the ALSRP:

Apocynin: Completed pre-Investigational New Drug (IND) studies. Prepared for submission of IND application to the FDA.
Vardenafil HCl: Ready for pre-IND testing in SOD1 animal model.
Compound 11: Shows superior biological performance as a disease modifying therapeutic candidate.
Pimozide: Initiated a Stage IIb randomized clinical trial to look at effects of pimozide in ALS patients.

Studies Funded: 44 since the start of the ALSRP in 2007

1. Preclinical Development of Therapeutics for ALS: **University of Iowa, 2007**
2. Neuroprotective Small Molecules for the Treatment of ALS: **Columbia University, 2007**
3. Development of Lead Agents for ALS Treatment in Preclinical Model Systems Based on Differential Gene Expression of IGF-II: **McLean Hospital, 2007**
4. Protein Aggregation Inhibitors for ALS Therapy: **Northwestern University, 2009**
5. Preclinical Studies of Induced Pluripotent Stem Cell-Derived Astrocyte Transplantation in ALS: **Johns Hopkins University, 2009**
6. Inhibitors of TDP-43 Aggregation and Toxicity: **Mayo Clinic and Foundation, Jacksonville, 2009**
7. Apo-Ferritin as a Therapeutic Treatment for Amyotrophic Lateral Sclerosis: **Pennsylvania State University, Milton S. Hershey Medical Center, 2010**
8. Hypermetabolism as a Risk Factor for ALS: **Johns Hopkins University, 2010**
9. Chemical Genetic Screens for TDP-43 Modifiers and ALS Drug Discovery: **University of Montreal, 2010**
10. Designing Pharmacological Agents that Inhibit the Aggregation of SOD1 by Increasing the Net Negative Charge of the Protein: **Baylor University, 2010**
11. AAV-Mediated Inclusion Formation as a Novel Gene Therapy Strategy for ALS: **McLean Hospital, 2010**
12. Overcoming the Practical Barriers to Spinal Cord Cell Transplantation for ALS: **Emory University, 2010**
13. TDP-43 Acetylation and ALS: **Pennsylvania State University, 2010**
14. Rethinking Drug Treatment Approaches in ALS by Targeting ABC Efflux Transporters: **Jefferson Medical College, 2010**
15. c-jun-N-Terminal Kinase (JNK) for the Treatment of ALS: **Scripps Research Institute, 2011**
16. Developing ER Stress Inhibitors for Treating ALS: **Sanford-Burnham Medical Research Institute, 2011**
17. Targeting Protein Adduction by Reactive Aldehydes in ALS: **Stanford University, 2011**
18. Developing Wide-Spectrum Antiproteotoxicity Agents to Treat ALS: **Johns Hopkins University, 2011**
19. The Role of NG2 Glial Cells in ALS Pathogenesis: **Johns Hopkins University, 2011**
20. Targeted Riluzole Delivery by Antioxidant Nanovectors for Treating ALS: **University of Texas, Health Science Center at Houston, 2011**
21. Targeting miR-155 in Peripheral Monocytes for the Treatment of ALS: **Brigham and Women's Hospital, 2012**
22. A Cell-Based Assay To Identify Neuroprotective Molecules for the Treatment of Amyotrophic Lateral Sclerosis: **Columbia University, 2012**
23. Therapeutic Targeting of Expanded C9ORF72 Transcripts in ALS: **University of Miami School of Medicine, 2012**
24. Preclinical Testing of a Translocator Protein Ligand for the Treatment of Amyotrophic Lateral Sclerosis: **Columbia University, 2013**
25. Muscle-Derived GDNF: A Gene Therapeutic Approach for Preserving Motor Neuron Function in ALS: **Cedars-Sinai Medical Center, 2013**
26. Live Imaging of the RNA Hexanucleotide Repeat Expansion in C9ORF72-Linked ALS to Enable Therapeutic Screening and Development: **Cornell University, Weill Medical College, 2013**
27. Exosome-Mediated Transmission of Neurodegeneration in Amyotrophic Lateral Sclerosis Using Patient-Induced Pluripotent Stem Cell-Derived Neurons and Astrocytes: **J. David Gladstone Institutes, 2013**
28. Inhibitors of c9RAN Translated Peptides and Toxicity in c9FTD/ALS: **Mayo Clinic and Foundation, 2013**
29. Pathogen-Inspired Nanoparticle Drug Delivery to Motor Neurons: **St. Joseph's Hospital and Medical Center, 2013**
30. Establishment of a Human-Based in Vitro Functional NMJ System for ALS Drug Screening: **University of Central Florida, 2013**
31. Development of Copper ATSM as a Therapeutic for SOD-Familial and Sporadic ALS: **Oregon State University, 2014**
32. A High-Throughput Phenotypic Screen for C9ORF72 ALS Therapeutics Using Patient-Specific Motor Neurons: **University of Southern California, 2014**
33. Developing Therapeutic Agents for Nucleotide Repeat Expansion-Mediated ALS: **Johns Hopkins University, 2014**
34. Inhibitors of SOD1 Interaction as an Approach to Slow the Progressive Spread of ALS Symptoms: **University of Florida, 2014**
35. Development of Novel Neuronal Autophagy Inducers to Block Neurodegeneration and Treat ALS: **J. David Gladstone Institutes, 2014**
36. Small-Molecule Efflux Inhibitor for Enhanced Treatment of ALS: **Izumi Biosciences, Inc. 2015**
37. Targeting the ER Stress Sensor IRE1 to Treat ALS: **Biomedical Neuroscience Institute, 2015**
38. Moto Neuron-Protecting Agents as Therapeutics for Treating ALS: **Columbia University, 2015**
39. Reprogramming Reactive Astrocytes Directly into Functional Motor Neurons in the Spinal Cord of ALS Model: **Pennsylvania State University, 2015**
40. High-Throughput, High-Dimensional (HT-HD) Phenotyping of C. elegans for ALS Drug Discovery: **Rice University, 2015**
41. Enabling Widespread, Minimally Invasive Distribution of Multimodal Therapeutic hNSCs throughout the Neuroaxis of ALS Mice: **Sanford-Burnham Medical Research Institute, 2015**
42. Chemical Library Screening for Potential Therapeutics Using Novel Cell-Based Models of ALS: **Southern Illinois University, 2015**
43. RAN Translation as a Therapeutic in ALS: **Stanford University, 2015**
44. Delivery of Ubiquitin to Motor Neurons Using a Targeted, Sterically Stabilized Liposome Delivery System: **University of Wollongong, 2015**