

CDMRP

XII. Amyotrophic Lateral Sclerosis Research Program

DEVELOPING NEW THERAPIES

The Disease

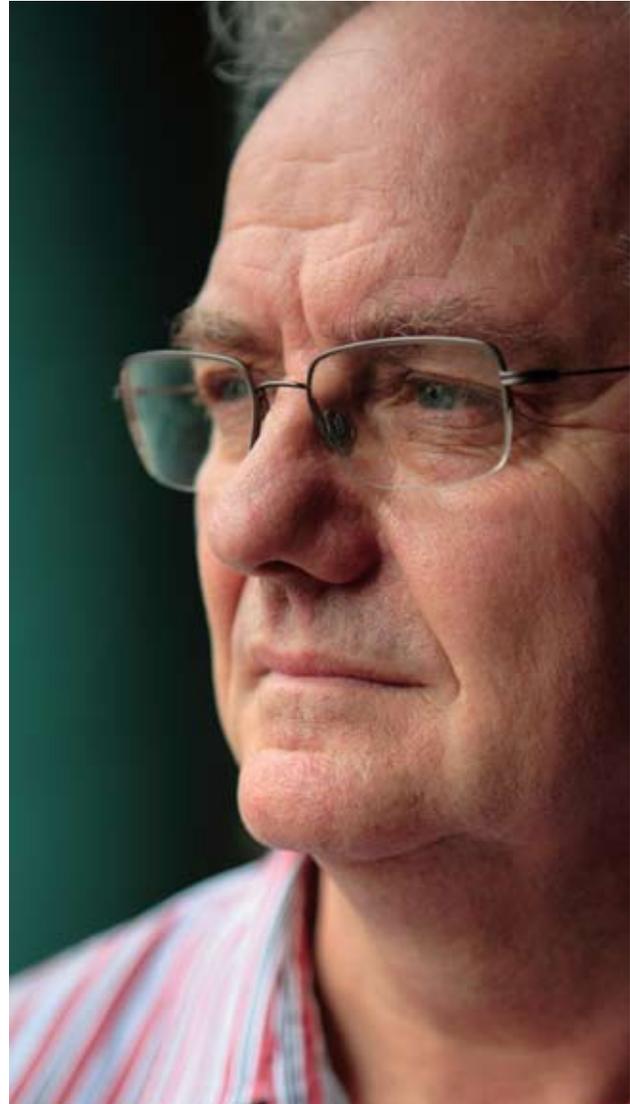
Amyotrophic Lateral Sclerosis (ALS), also known as “Lou Gehrig’s disease,” is an incurable, degenerative neurological disorder. For reasons that are not understood, the nerve cells of the brain and spinal cord that control voluntary muscle movement gradually deteriorate. Average life expectancy after diagnosis ranges on average from 2 to 5 years.¹ There are no known therapies to effectively halt the progression of ALS. Men and women who have served in the U.S. military are 60% more likely to develop a fatal muscle-wasting disease such as ALS than civilians.²

Signs and Symptoms

The early onset of ALS is often subtle that signs and symptoms are overlooked. However, emerging physical symptoms of the disease include:

- ❖ Twitching, cramping, or stiffness of muscles
- ❖ Muscle fatigue and weakness affecting an arm or leg
- ❖ Slurred and nasal speech
- ❖ Difficulty chewing or swallowing

These general symptoms then develop into more obvious signs of muscle weakness or atrophy. As the disease progresses, patients experience difficulties moving, swallowing, and speaking. Eventually, patients afflicted with ALS are unable to stand, walk, swallow, chew, and experience difficulty breathing. The disease does not usually affect cognitive abilities.



¹ ALS Association, <http://www.alsa.org/als/facts.cfm?CFID=4240363&CFTOKEN=81327833>

² Weisskopf M, et al. 2004. Annual Meeting of the American Academy of Neurology, San Francisco, California



Program Background

In June 2007, the DOD redirected \$5M of FY07 Army Research, Development, Test, and Evaluation funding for the Congressionally Directed Medical Research Programs (CDMRP) to initiate the Amyotrophic Lateral Sclerosis Research Program (ALSRP) as a broadly competed, peer-reviewed research program. Similar to other programs within the CDMRP, the ALSRP is being conducted according to the two-tier review model recommended by the National Academy of Sciences Institute of Medicine. The first ALSRP Integration Panel (IP) meeting was held in September 2007 to determine the FY07 vision and investment strategy for this new program.

The Program Today: Developing New Therapies

Fiscal Year 2007 Summary

The FY07 ALSRP IP envisioned the program to explore the basic biological mechanisms of the disease as well as accelerate translational research in the field of ALS. To capture this need, members of the IP proposed one award mechanism, the Therapeutic Development Award, to support the preclinical assessment of therapeutics for ALS. The overall goal of this award mechanism is to

promote the introduction of improved therapies for ALS by encouraging ALS investigators to undertake preclinical studies of novel and existing agents. Scientific peer review and programmatic review are scheduled for February and March 2008, respectively. It is anticipated that one or two awards will be recommended for funding.

Fiscal Year
2007
40 Proposals Anticipated
\$5M in Appropriations
~2 Awards



A Team of Outstanding People

Consumer Advocates

Consumer advocates for the ALSRP are individuals living with or family members of individuals living with ALS who are active participants in an ALS-related support, outreach, or advocacy organization. Similar to other CDMRP programs, consumer advocates are active participants in practically all aspects of program execution. They work collaboratively with leading scientists and clinicians in recommending program priorities and proposals where they contribute their unique perspectives and sense of urgency. Consumer advocates also serve as liaisons between their constituencies and the scientific community and are able to increase awareness about the program. More information about consumer advocate participation can be found in Section I, Overview.



Ellyn Phillips
ALS Association
FY07 Consumer Integration Panel
Member

“The ALS Association and the ALS community are delighted to partner with CDMRP through an ALSRP. Since the 1860s when ALS was first identified, the need for more research has been apparent. With the knowledge that those in the military have a higher incidence of ALS, we are optimistic that this research program will advance the efforts for more effective treatments and a cure.”

Peer Review Panel Members

The first ALSRP scientific peer review panels will be composed of outstanding investigators from scientific and clinical disciplines, as well as consumer advocates. The primary responsibility of scientific peer review is to provide unbiased, expert advice on the scientific and technical merit of proposals submitted to the program. Scientific reviewers for peer review are selected for their subject matter expertise and experience with scientific peer review. Consumer reviewers are nominated by a support or advocacy organization and are selected on the basis of their leadership skills, commitment to advocacy, and interest in science. Further details about peer review can be found in Section I.

Integration Panel Members

The ALSRP IP is made up of distinguished scientists, clinicians, and consumer advocates. They work to serve the interest of the ALS community by recommending critical research priorities, cutting-edge investment strategies, and multidisciplinary research portfolios (for more information about the functions of the IP, see Section I). Members of the program's first IP are helping to advance progress in the field of ALS.

FY07 ALSRP IP Members

Air Force

Hendrick Ruck, Ph.D., (Chair), Director, Human Effectiveness Directorate, Air Force Research Laboratory

Major David Watson, Ph.D., Flight Commander, Air Force Laboratory Services

Department of Veterans Affairs

Brenda Cuccherini, Ph.D., (Alternate Chair), Program Specialist, Office of Research and Development

Navy

Captain Richard Haberberger, Ph.D., Executive Officer, Naval Medical Research Center

Army

Colonel Cornelius Maher, M.D., Ph.D., Deputy Commander of Europe Regional Medical Command

ALS Association

Ellyn Phillips, Chair, Greater Philadelphia Chapter

Lucie Bruijn, Ph.D.

Ad Hoc Representative

Walter Bradley, Ph.D., University of Miami

Dr. Brenda Cuccherini Department of Veterans Affairs FY07 Alternate Chair

"A November 2006 Institute of Medicine report concluded that Gulf War veterans and other combat veterans may be at an increased risk for ALS when compared with the civilian population. The Veterans Health Administration's Office of Research and Development has expanded its research efforts related to ALS and supports cooperative efforts to advance discoveries related to early diagnosis, therapeutic interventions, and mechanisms to improve the quality of life. CDMRP's Amyotrophic Lateral Sclerosis Research Program represents a very valuable resource that will allow current scientists with expertise in ALS to pursue their research efforts to both develop novel therapeutics and advance medical knowledge about ALS."



Lucie Bruijn, Ph.D.
ALS Association
FY07 Integration Panel Member

"Amyotrophic Lateral Sclerosis, commonly known in the United States as Lou Gehrig's disease, is a devastating disease affecting nerve cells, leading to paralysis and death. The ALS Association is very excited about the recent commitment by the Department of Defense to fund researchers to find treatments for the disease."



Scientific Community

The scientific community is an integral part of the ALSRP. Scientists and clinicians are providing their expertise on setting the program's vision, reviewing proposals, and conducting the necessary laboratory and clinical studies to find effective treatments for this disease.

