This following testimony was delivery to congress on behalf of ALS TDI on April 23, 2009. For more information, please contact: Robert A. Goldstein, ALS TDI, 617-441-7295, rgoldstein@als.net

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Good Morning. My name is Dr. Stanley Appel. I would like to thank the Chairman and the Subcommittee for holding this hearing and allowing me the opportunity to address you today about the absence of a robust government funded program to develop therapies for Amyotrophic Lateral Sclerosis (ALS).

I am the Chair of the Department of Neurology at The Methodist Hospital in Houston, and the Co-Director of The Methodist Neurological Institute; where I am also the Director of the Muscular Dystrophy Association's ALS Research and Clinical Center. Through these roles, I am engaged in both research and clinical treatment of ALS. Over the last 40 years I have been privileged to participate in the care of over 3,000 patients with ALS. The experience has been made all the more meaningful because our ALS patients are extremely giving, and a deep well of courage fuels their daily actions.

Their inner strength has been a source of inspiration to us all, and has motivated me to help translate advances in the basic science laboratory to the bedside. My goal as a physician scientist is help ALS patients transform a profile in courage into a disease-free future.

I am here before this subcommittee responsible for supporting our veterans because this disease continues to take an inordinate toll on our nation's veterans.

Since 2000, research has continued to find that those with a history of military service are at a greater risk of developing ALS than those who have not served. A study jointly funded by the VA (Department of Veteran Affairs) and DOD (Department of Defense) found that those serving in the first Gulf War were nearly twice as likely to develop ALS as those not serving in the Gulf. In 2005, The Harvard School of Public Health broadened the case for military relevance. Its epidemiological study found that those with a history of any military service in the last century were 60% more likely to die of

ALS than those in the general population. In 2006, the Department of Veterans Affairs requested an independent assessment of the relationship between military service and ALS. Assigned with the task, The Institute of Medicine issued its report, supporting existing evidence of an association between military service and the later development of ALS. And most recently, the Research Advisory Committee on Gulf War Veterans Illnesses' report has called for a renewed federal commitment to identify effective treatments for Gulf War Illnesses, including ALS.

ALS, often known as Lou Gehrig's disease, is a progressive neurodegenerative disease that affects motor neurons in the brain and spinal cord. Motor neurons extend from the brain to the spinal cord, and to the muscles throughout the body. Eventually, in ALS, they die; disallowing the brain to initiate or control muscle movement. Ultimately, patients become paralyzed, and lose their ability to breathe independently or swallow. Throughout the course of the disease, the mind remains intact and unaffected. The patient becomes 'trapped' inside their own body.

In our country, it is estimated that ALS strikes between 5000 and 7000 people a year. The incidence rate is similar to that of Multiple Sclerosis, yet ALS patients seldom live beyond three to five years of their diagnoses, leaving at least 30,000 Americans suffering at any one time.

Though ALS was 'discovered' over 130 years ago, we have yet to offer a cure. Only one drug has ever been brought to market; offering no more than the hope for a few months of average life extension, and no improvement in quality of life.

I know this Committee has seen the evidence for itself. Many American heroes have sat in witness chairs, and wheelchairs, to ask Congress for help. Major Michael Donnelly was the first to bring attention to the issue. Major Donnelly was a Gulf War fighter pilot, awarded four Air Medals for completing 44 combat missions over Iraq in Operation Desert Storm. The Major worked successfully for the last years of his life to convince the government that his illness was service based. He died at 36. Four Star Brigadier General Tom Mikolajcik, USAF Ret., sat before you in July of 2007 imploring Congress to increase funding for the treatment of ALS, knowing that a treatment would come too late for him. The General is not well enough to be with us today, but conveys his appreciation for the Department of Veterans Affairs new regulations recognizing service connection to all veterans facing ALS.

At its inception (2003), the VA's voluntary ALS Registry registered 2000 veterans suffering from ALS. While most of those heroes have passed away, the numbers imply that at least one out of fifteen ALS patients has a history of military service. This is quite staggering. These veterans, and all the patients I treat, are facing a horrifying illness; a battle with no armor. Physically, emotionally, and financially the plight is overwhelming. The cost of caring for an ALS patient can reach $250,000 a year…yet we offer them no hope.

For these veterans, their families, and on behalf of all ALS patients, I urge the VA to consider a comprehensive approach to developing therapies.

There is often a gap in the process of bringing a drug to a patient's bedside. Translational research takes the excellent work done by the NIH and academic/basic researchers (my being one), and translates their findings toward the development of therapeutic targets. This is a particularly expensive and risky phase of research. Pharmaceutical companies; the entities that traditionally build drugs are not incentivized to assume this cost and risk, especially in the case of orphan diseases, such as ALS.

The ALS Therapy Development Institute (ALS TDI) in Cambridge, Massachusetts bridges this gap. It is the world's largest ALS research program; with a state of the art lab and a full time scientific staff of thirty working solely on developing therapeutics for the treatment of ALS.

I have been on the Board of Directors of The Muscular Dystrophy Association since the 1970's, chaired its Scientific Advisory Committee, and now chair its Medical Advisory Committee. I think you should know that the MDA believes so strongly in the work at ALS TDI that we committed the largest sum in our organization's history to support its program.

The ALS Therapy Development Institute is effectively a biotech company, although is set up as a non-profit. Its scientific team comes from industry, with team leaders bringing an average of 15 + years of experience from firms such as Biogen Idec, Genzyme, Wyeth, and AstraZeneca. Thus, this team, unlike any other, brings true pharmaceutical experience in building drugs to this effort.

The Institute's approach represents a multi-faceted methodology to understanding disease progression from a preclinical animal model, and efficiently translates findings to ALS clinical samples. Their capability to employ unbiased gene expression, proteomics, and genetics technologies will speed the identification of biological mechanisms amenable to therapeutic development. This type of comprehensive strategy has never before been applied to ALS research.

ALS TDI rigorously addresses the specific issues that can impact the quality of life for veterans predisposed to the possibility of developing ALS. Their program works to develop prognostic and diagnostic biomarkers to facilitate rapid treatment options, and offers a process to understand disease onset and progression. It is through such an approach that we stand the best chance to develop impactful treatments quickly.

I am not here to ask for funding for ALS TDI, although your support would accelerate the progress that I am convinced is forthcoming. I am here to at least urge your subcommittee to consider the benefits of adopting this approach.

We have the responsibility to arm our service members in the final battle they are currently only to lose. The thousands who have recently returned from engagement, and those soon to come home, may in fact encounter a final new enemy in ALS.

ALS is not an incurable disease. It is an underfunded disease. The science is ready, the technology is ready, we clinicians are ready.