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AMYOTROPHIC LATERAL SCLEROSIS (ALS)

HEARING

BEFORE A

SUBCOMMITTEE OF THE

COMMITTEE ON APPROPRIATIONS

UNITED STATES SENATE

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AMYOTROPHIC LATERAL SCLEROSIS (ALS)

WEDNESDAY, MAY 11, 2005

U.S. SENATE,
SUBCOMMITTEE ON LABOR, HEALTH AND HUMAN SERVICES, EDUCATION, AND RELATED AGENCIES,
COMMITTEE ON APPROPRIATIONS,
Washington, DC.

The subcommittee met at 10 a.m., in room SDG–50, Dirksen Senate Office Building, Senator Richard C. Shelby presiding.

Present: Senators Shelby, Harkin, and Murray.

OPENING STATEMENT OF SENATOR RICHARD C. SHELBY

Senator SHELBY. I want to take this opportunity to thank all of you for being here to discuss the issues surrounding ALS. This month is ALS awareness month and today, the ALS Association's advocacy day. So I can think of no better time to be holding this hearing.

I want to take a minute to thank Senator Specter and also Senator Harkin because I am not the chairman of this subcommittee. I am a chairman of another subcommittee in the Appropriations chain and so I am indebted to Senator Specter and the ranking member Senator Harkin, for letting us conduct this hearing today.

This is an important issue that affects many of our constituents. I am pleased that so many folks were able to join us here today including some of my constituents from my home State of Alabama.

Appearing before us today are two ALS patients, a number of their advocates, both local and celebrity, as well as a leading researcher in the field. I look forward to hearing from each of you.

ALS is a tragic disease that we know little about and have no treatment for at this time. I have seen firsthand how rapidly this disease robs one's ability to function and how quickly it can take our loved ones from us.

Just last year, I met with some Alabamians about ALS and I am sad to report that one of the individuals that we spoke about during that meeting is no longer with us. His wife, who is here today, continues her efforts to promote increased research for ALS and I for one greatly appreciate her efforts on behalf of those suffering from ALS.

Federal funding for ALS research is critical. While we work diligently to increase funding, there is much more to do. I am hopeful that this hearing today will provide a forum to discuss the issues that must be addressed by researchers.

There is so much basic research that must first be done if we are to find the cause and the cure of ALS. I also believe that we must continue to work to find immediate opportunities to slow its progression and provide patients with every option available.

Again, I hope this hearing will serve to increase awareness of ALS and our need to do more on behalf of the ALS patients and their families.

Thank you all for being here today and I look forward to hearing from each of you.

Senator Harkin.

OPENING STATEMENT OF SENATOR TOM HARKIN

Senator HARKIN. Mr. Chairman, thank you very much, Senator Shelby, for calling this hearing today.

Senator Shelby and I have been friends for a long time. We served in the House together many—well, a few years ago. Probably longer ago than we care to admit, I suppose. But we have been great friends for many, many years.

I want to thank you for calling this hearing today.

Let me also thank the hundreds of patients and their loved ones who have packed this room today to demonstrate their support for ALS research. I just shook the hands of a man from Alaska and I said you get the prize for coming the farthest. He said, no, there is someone here from Hawaii.

So people have come a great distance and I know the difficulty of traveling today, so we just really appreciate this show of support.

Later today this morning, you will be fanning out across Capitol Hill to make your case for more aggressive ALS research. I want to tell you how important it is that you are here. You remind us and those that you will be seeing that ALS is not just about statistics. It is about the suffering of real human beings, our friends and our family.

It makes all the difference in the world for you to bring that message here today in person. Your presence could not come at a more crucial time. It has been 6 decades since Lou Gehrig died, but we still have only one FDA approved drug to treat this disease and that drug only extends life for a few months. This is unacceptable.

Five thousand Americans are diagnosed with ALS each year. For some reason, no one knows why, our military veterans are especially susceptible to this disease. So we have a responsibility to do more to find an effective treatment and cure.

Regrettably, the administration has taken a hard line against one of the most promising avenues of research, stem cells. Scientists tell me that ALS is exactly the kind of disease that could benefit from stem cell research.

They have already figured out how to direct stem cells to develop into motor neurons. If they could solve that next critical step, replacing motor neurons that have died off in a person with ALS, then we would be looking at a cure.

Instead the administration has put a choke hold on research by limiting the number of stem cell lines that federally-funded scientists can study.

We were initially told that more than 70 lines would be eligible for federally-funded research. Today there are just 21 and all of them, every single one, are contaminated with mouse feeder cells, meaning that there is really no chance that they will ever be used for any human therapy.

That is why Senator Specter here in the Senate, Congressman Castle on the House side have introduced bipartisan legislation to expand the number of stem cell lines that are eligible for federally-funded research.

The House will vote on this measure sometime in May. I was talking with Congressman Castle who is a member of the Republican party on the House side and he believes that they have the votes to pass it, but, please, any help you can give on the House side, I would appreciate that. Then we will take it up later on.

So I hope that in your meetings today with senators and their staffs that you will also push for this legislation.

In the meantime, I was pleased to learn that NIH is supporting five clinical trials on ALS that are either ongoing or about to start. I am sure we will hear about that from Dr. Landis. That is more than we have ever had before. So these are clearly more hopeful times for ALS and I look forward to hearing more about the trials this morning from Dr. Landis.

Again, I want to join Senator Shelby in welcoming an outstanding group of witnesses. In particular, I would just take the time right now to recognize Rob Borsellino, a renowned columnist for the Des Moines Register.

Rob has been in the news business for 30 years in TV and radio and newspapers. He has worked for a number of newspapers across the country from New York to Florida and decided that Iowa was the best place to live, so settled in Iowa.

In the mid nineties, he was a talk show host on ABC, a TV affiliate in Des Moines. He and his wife have two sons, one 18, one 15. So Rob, I just want to say, Rob Borsellino has an intensely loyal following all across Iowa. I am one of his biggest fans.

So it came as a blow to all of us when we opened the newspaper one morning and Rob announced in his column earlier this year that he has ALS. Since then, he has been writing and speaking about this disease with his customary humor and grace.

So I want to thank you, Rob, for the effort you showed to be here with us in person today.

I also want to recognize Roger Gold and his wife who are here from Iowa, also from Ames. Roger has had ALS now for 10 years and is one of those that has fought this disease. I just want to recognize him for coming here today also.

So with that, Mr. Chairman, again, thank you for having us here.

Senator SHELBY. Senator Murray.

OPENING STATEMENT OF SENATOR PATTY MURRAY

Senator MURRAY. Well, Mr. Chairman, I want to thank you for scheduling this really important hearing on ALS. I think it is really important for this subcommittee to hear from the National Institute of Neurological Disorders and Stroke as well as all of those who are suffering from this devastating disease.

It is important that everyone affected is informed about what we know today about ALS, about what we can hope for in the future, and what we need to do to find a cure.

We know that ALS is a tragic neurological disease that often strikes the patient in the prime of life and causes tremendous suffering and pain both for the victims and for their families.

Yet, while we all know this disease can be overwhelming and challenging, there is hope. I think it is really appropriate that the ALS Association has chosen their theme this year to be a flickering light of hope.

I believe that the source of that light is the work being done by so many dedicated research and health care professionals who each day bring us closer to a cure.

We have a responsibility to fuel the light of hope by funding research through NIH and avoiding political or ideological battles that might stand in the way of facilitating ethically sound research and progress on this really crucial issue.

I hope that this hearing today, Mr. Chairman, serves to raise awareness of this disease and the impact on both the patient and their families.

I want to thank Senator Shelby for chairing this and I want to thank Chairman Specter as well and Senator Harkin for their work on behalf of NIH funding. I look forward to working with everyone at the table to maintain our investment in sound biomedical research.

Thank you, Mr. Chairman.

STATEMENT OF STORY C. LANDIS, Ph.D., DIRECTOR, NATIONAL INSTITUTE OF NEUROLOGICAL DISORDERS AND STROKE, NATIONAL INSTITUTES OF HEALTH, DEPARTMENT OF HEALTH AND HUMAN SERVICES

Senator SHELBY. Dr. Landis, welcome to the committee.

Dr. Landis is the director of the National Institute of Neurological Disorders and Stroke. She earned her Master's and Ph.D. from Harvard University. She later served on the faculty of the Harvard Medical School and was the chairman of the Department of Neurosciences at the Case Western Reserve University School of Medicine. She has been the director of NINDS since 2003.

We welcome you. We understand you have a written statement and you also have an opening statement. You proceed as you wish.

Dr. LANDIS. Right. Thank you.

Mr. Chairman and members of the committee, I am Dr. Story Landis, Director of the National Institute of Neurological Disorders and Stroke, one of the components of the National Institutes of Health. I want to thank you for offering me the opportunity to speak with you about our research programs in ALS.

As you have already heard and will hear from other panel members, ALS is a devastating disease, results from the death of motor neurons in the brain, brain stem, and spinal cord.

These nerve cells relay control signals from the brain to the muscles throughout the body and when these nerve cells die, patients lose the ability to move. They lose the ability to swallow, to speak, and ultimately to breathe. For many patients, 5 years from the diagnosis of ALS, they die. It is a terrible disease.

Scientists do not fully understand what triggers motor neuron death in this disease. Most believe that it is an interaction between genes, environmental influences, and aging.

There are a number of specific hypotheses that include oxidative stress, over-excitation, lack of trophic support, and/or aberrant signaling within a nerve cell or between nerve cells.

About 10 percent of ALS cases are genetic in origin and the identification of these disease genes has actually given us very important clues as to why motor neurons die in this disease and also have given us tools to increase our ability to understand the disease and to develop therapies.

There is a desperate need for new therapies for ALS. At present, as you have already heard, there is only one drug approved by the FDA for ALS. That is Riluzole and it is not very effective.

CLINICAL TRIALS

We have four clinical trials now. It is rare for a disease that we are able to say this. We have four clinical trials that are already enrolling patients and a fifth that will start enrolling patients in the fall.

These clinical trials are based on very promising pre-clinical data and are a result directly and indirectly of the doubling of the NIH budget. We did actually do something good with those monies.

One of them is a phase three clinical trial looking at a trophic factor, IGF-1. A second is a phase three clinical trial looking at minocycline, an antibiotic which turns out to have an additional activity which is reducing inflammation and nerve cell loss.

We have a phase two clinical trial that is looking at CoQ10, an antioxidant which works in pre-clinical studies.

A fourth trial is actually one aimed at helping patients who are in mid stages of ALS which is an effort to look at when nutritional therapy and respiratory therapy is best undertaken.

BASIC SCIENCE RESEARCH

Now, in order to have good clinical trials, you really have to have a very good understanding of the basic underpinnings of motor neuron biology and cell death. A very useful strategy in understanding motor neuron survival and the death of these motor neurons in ALS has been the identification of genes.

We now have three genes which are known to cause ALS and the first and most informative of these is one in copper/zinc superoxide dismutase or SOD1. This accounts for about 2 percent of ALS patients. There are over 100 mutations in this gene which can cause ALS.

So the discovery of this gene and the protein which is influenced has given us very interesting and testable hypotheses about why motor neurons die and examination of a mouse model that has been created has informed us that, in fact, normal glial cells, support cells can help motor neurons which have the mutation survive. An interesting finding that is relevant to stem cell therapy.

It also turns out that this mouse model has been a critical piece in the pre-clinical studies that allow us to look at what drugs might be useful for human trials.

We believe that discovering other ALS genes is critical to increasing our understanding of why motor neurons die and to develop additional pre-clinical models. It is not enough to just have one.

So using funds that we got from the doubling, the increase in NINDS funding, we created a repository for genetic samples of patients with ALS so that those samples can be shared and genes can be discovered more quickly. We are working in particular with ALSA to encourage investigators to deposit samples in the repository.

So I have given you an example of clinical trials we are running and of very basic research. One of the challenges for NIH has been how you bridge the gap between those two.

TRANSLATIONAL RESEARCH

We have with our doubling invested funds in a number of translational research programs helping investigators move promising leads from basic research to the point where they would be ready for clinical trials.

ALS has been a particular benefactor from this new program and because of monies invested in this program, the fifth clinical trial is going to look at an antibiotic which no one would have ever guessed would be useful in ALS. We are very optimistic that we will see benefit from that.

PREPARED STATEMENT

So I have focused on a very small number of efforts that NINDS is making in the area of ALS with seven other institutes and centers at the NIH and in collaboration with a number of very effective voluntary organizations.

I would be pleased to take questions. Thank you.

[The statement follows:]

PREPARED STATEMENT OF STORY C. LANDIS

Mr. Chairman and Members of the Committee, I am Dr. Story Landis, Director of the National Institute of Neurological Disorders and Stroke (NINDS), a component of the National Institutes of Health (NIH) within the Department of Health and Human Services (HHS). The NINDS is investing aggressively in new approaches to understand and treat amyotrophic lateral sclerosis (ALS), and I am pleased to be here today to share our research priorities, plans, and advances with you.

Without question, ALS—or Lou Gehrig's disease—is one of the most debilitating and devastating of all diseases, and NINDS takes the need for treatments in this community very seriously. As many of you already know, ALS is caused primarily by the loss of nerve cells called motor neurons. These cells reside in the brain and spinal cord, and relay control signals from the brain to muscles throughout the body—including those of the limbs, face and respiratory system. Although the clinical presentation varies widely, the death of motor neurons in ALS eventually leads to increasing difficulties with movement. These often occur first in the hands or feet, but occasionally begin in muscles such as those that control the tongue and swallowing. Regardless of the site of onset, the disease is relentless, and it gradually robs affected individuals of all motor function over a period of months or years. Approximately 5,000 people in the United States are diagnosed with ALS each year, and only 10 percent survive beyond five years after the onset of symptoms. Despite the advances made in ALS research and continued improvements in supportive therapy, current options for disease-modifying treatment are quite limited. Only one drug—Riluzole®, an inhibitor of the excitatory neurochemical glutamate—is approved by the U.S. Food and Drug Administration (FDA) for treating ALS, and it only extends survival by a few months.

Researchers do not fully understand what triggers motor neurons to die in people with ALS, but several factors have been implicated, including the increased cellular stress caused by the need for these neurons to maintain tremendously long connections with distant cells; overstimulation by excitatory nerve chemicals, like glutamate; and the activation of specific signals inside the cells that lead to their destruction. In 1991, researchers funded in part by NINDS published the initial discovery of a genetic link to ALS, specifically the chromosomal mapping of a gene that was believed to contribute to the hereditary form of ALS. This discovery later led to the identification of more than 100 mutations in this gene, along with the recognition that it normally codes for an enzyme called superoxide dismutase (SOD1), which helps clear damaging free radicals from cells. Only ten percent of ALS cases have been found to be associated with inherited genetic mutations, and mutations in SOD1 only a small fraction of those. In addition, researchers still do not fully understand how SOD1 mutations make people more susceptible to developing ALS. However, the discovery of this first gene energized investigators by providing the scientific community with new insights into possible mechanisms of disease, and by offering a means to create useful animal models of ALS that could be used for studying disease causality and testing treatments.

To adequately address all of the issues that impact the ALS community, NINDS supports a continuum of research: clinical research to rapidly test available therapies; translational research to move basic science towards clinical applications; and basic science research to expand our understanding of the causes of ALS.

CLINICAL RESEARCH

Despite the difficulties presented by this disease, NINDS is greatly encouraged by the enhanced interest and creativity among researchers in exploring approaches that may reduce the burden of ALS by intervening in the progression of the disease and/or improving the supportive care of affected individuals. Not only do these different approaches have the potential to extend survival and improve quality of life, but by targeting different aspects of the disease, they may ultimately be explored as part of a combination therapy approach.

Several ALS clinical trials are actively recruiting subjects, including a NINDS-funded Phase III trial of insulin-like growth factor-1 for ALS, for which enrollment is nearly complete. This trial takes advantage of the potential for a naturally occurring protein that promotes nerve cell growth and survival to delay loss of muscle strength, improve function, and extend survival in people with the disease. Enrollment is also underway for a Phase III trial of the antibiotic minocycline for treating ALS. In addition to its antibiotic properties, minocycline can also suppress cell death signals and inflammation—and has shown promise in delaying disease progression in several animal models of neurodegenerative disease, including ALS. The data from preclinical work in animals, in combination with safety and tolerability information provided in a Phase I/II human study of minocycline, provided support for its advancement into Phase III testing.

In addition to these ongoing studies, the Institute is also supporting several new translational research programs and clinical studies that herald a new era of patient-focused research at NINDS. As a prime example of the momentum in the field, ALS researchers have recently participated in the screening of a number of drug candidates with possible effectiveness in treating the disease. NINDS conceived of this novel program for academic researchers, and designed it to enable rapid screening of potential therapeutic compounds with known bioactivity and/or safety in humans, so that the most viable candidates could be quickly moved forward into clinical trials. Initiated in 2001 in collaboration with the Amyotrophic Lateral Sclerosis Association (ALSA) and two other private funding organizations, the program supported the screening of more than a thousand bioactive compounds in nearly 30 laboratory models of nerve cell degeneration. Over 75 percent of these drugs were already FDA-approved, which means that researchers could also access some information on the toxicity of these compounds in humans. The availability of toxicity information is a significant advantage for the research community, since it can save years of time in the drug development process for any agents that are moved forward into subsequent testing. In the models that were most relevant to ALS, a group of antibiotics related to the penicillins emerged as the candidates with the most potential for further study. These antibiotics are not only effective in killing bacteria; they were also found to protect cells from the toxicity of mutant SOD1 and to activate a gene for a glutamate transporter—a protein found on the surface of glial cells (non-neuronal cells that provide support and nutrition to nerve cells) that helps remove excess glutamate from the spaces surrounding nerve cell connections. Too much glutamate in these spaces can stress cells via overexcitation; and this

process may occur in people with hereditary ALS as well as the sporadic form of the disease. After identifying this family of compounds in the large drug screen, researchers moved quickly to evaluate the most promising member of this family—ceftriaxone—in additional laboratory tests and preclinical studies of neuroprotection. Recent results indicate that ceftriaxone can stimulate the glutamate transporter gene in intact animals, and can protect neurons both in animal models of oxygen deprivation injury and ALS. Moreover, NINDS, in partnership with ALSA and Project ALS, contracted a study that demonstrated that ceftriaxone can delay the loss of muscle strength and death in an animal model of ALS as well.

With these results in hand, clinical investigators designed an integrated clinical trial to explore the safety, tolerability, and ultimately the efficacy of ceftriaxone in people with ALS. Typically, NINDS relies on investigator-initiated research proposals to attain most of the Institute's clinical research goals. In most cases, these investigators conduct Phase I and Phase II studies to explore dosing, safety, and tolerability, then analyze the results before submitting a separate application for a Phase III trial to explore the efficacy of a particular therapy. In the ceftriaxone trial, NINDS worked with the applicant to use a design feature that is new to ALS and neurology, but not to the field of clinical research, that allowed the separate trial phases to be combined into one integrated study. In the three-step ceftriaxone trial, investigators will first determine the optimal dosage of ceftriaxone in a small group of 60 subjects, and will continue in a second step to examine the safety and tolerability of the drug in these same individuals. If sufficient levels of the drug are well tolerated in these participants, the researchers will expand the trial to 600 participants, in order to determine if the drug prolongs survival. The advantages of this approach are that it can eliminate the 9–22 months that are often required for the review of a Phase III trial application, and the study can still be stopped early if the equivalent of the Phase II results are negative or discouraging. NINDS has recently initiated funding for this trial and expects to begin recruitment in the fall; the Institute hopes this trial will serve as a first step in the successful translation of this therapy into clinical practice.

Many researchers exploring potential treatments for neurodegenerative diseases have also considered the antioxidant and health supplement coenzyme Q10 (CoQ10) as a promising candidate. Its ability to penetrate the nervous system and protect cells from oxidative stress, combined with its excellent safety profile, has stimulated interest in the drug for the treatment of Parkinson's disease, Huntington's disease, and ALS. In April 2005, enrollment began for a Phase II trial supported by NINDS that is designed to examine the potential of high-dose CoQ10 to treat ALS. Like the ceftriaxone study, this trial will also be conducted in several sequential steps. In the first part of the trial, investigators will identify the optimal dose of the drug; in the second, they will collect preliminary evidence of efficacy using a number of different outcomes, including functions needed for daily living, and measures of lung capacity, fatigue, and quality of life. Although a conclusive determination of efficacy may not be available at the end of this study, NINDS hopes that it will facilitate the collection of data needed to plan a phase III trial.

Although the need for therapies designed to intervene in the cellular events that cause ALS is essential, NINDS also supports strategies to prolong survival by improving the clinical care of ALS patients. Specifically, the clinical literature suggests that respiratory and nutritional support can independently improve survival in people with ALS. However, issues such as the identification of the optimal timing for initiating respiratory support; the best method for improving the tolerability of appliances that facilitate respiration; and the development of better techniques to assess the balance of energy consumption and use have not been addressed in well-designed clinical studies. NINDS has recently funded a Phase II trial to collect data on these and other issues; this information will enable investigators to design a Phase III trial of combined respiratory and nutritional therapy. This trial is slated to begin enrollment in the very near future, and NINDS is enthusiastic about the possibility that these two approaches might have synergistic effects in treating ALS, and may in the future be combined with therapies that target the cellular mechanisms of the disease.

TRANSLATIONAL RESEARCH

Across its areas of responsibility, NINDS actively promotes translational research, which links findings in basic science laboratories with early-phase clinical trials. Specifically, NINDS has designed a large translational research program to facilitate the development of goal-directed, milestone-driven research projects, and issued three Program Announcements (PAs) in July 2002 as part of this project. These PAs target high-risk exploratory studies; large research programs that could be con-

ducted under cooperative agreements with NINDS; and mentored research scientist awards—all focused solely on translational research goals. NINDS also established special review panels to evaluate applications received in response to these PAs, to ensure that the unique needs of translational research would be taken into consideration. To date, this program has provided support for a cooperative agreement that is enabling the research group responsible for the preclinical data on ceftriaxone to identify additional antibiotic and non-antibiotic compounds that may also have stimulatory effects on the glutamate transporter gene. In addition, NINDS is also supporting an exploratory project designed to evaluate another series of promising compounds originally tested in the drug screening program described above. In the latter project, investigators will assess eight compounds that can potentially protect neurons by enhancing glutamate uptake, validate any observed cellular effects in intact mice, and then test their downstream impact on disease in a mouse model of ALS.

As a complement to the translational grants program, NINDS has also established a facility at the Southern Research Institute in Birmingham, Alabama, that is miniaturizing laboratory tests relevant to neurodegeneration; automating them via robotic technology; and then using them to rapidly screen a collection of approximately 100,000 chemically diverse, non-proprietary compounds. By enabling the academic research community to have access to the type of drug screening resource that is normally only available to the pharmaceutical industry, the Institute is hoping to accelerate the identification of potentially useful therapeutics for a number of neurodegenerative diseases, including ALS. To date, two "test-tube" models of ALS are already being used at the facility to screen for drugs that may be useful in treating ALS, and researchers at the facility have already identified several drugs as possible "hits" in these screens.

In addition to these specific programs, NINDS-funded researchers continue to independently explore potential therapies for ALS that target a wide range of cellular processes. As suggested above, inflammation is one potential contributor to ALS that is gaining attention among translational and clinical investigators. Along these lines, recent data have suggested that a novel anti-inflammatory compound called pioglitazone can improve motor performance, delay the death of motor neurons, and extend survival in a mouse model of ALS. Further work will be required to confirm these effects in animals, but the continued success of these researchers in identifying novel therapeutic compounds is encouraging.

BASIC SCIENCE RESEARCH

As discussed earlier, research has implicated multiple cellular mechanisms—including cellular stress, overexcitation, and activation of cell death signals—in the motor neuron degeneration that causes ALS. Researchers are actively investigating these and many other leads in an effort to understand the disease and develop new strategies for treatment. In one example, they have found that in animals that are a chimera—having some cells with the SOD1 mutation, and others without it—the presence of normal glial support cells can delay the degeneration of motor neurons that harbor the mutation. This finding not only helps to explain the cause of ALS, but also suggests that glial support cells might be a reasonable target for the development of therapeutics.

Mitochondria, the energy generators of the cell, are also emerging as a target of enhanced interest among ALS researchers, including those studying how mutations in the SOD1 gene cause motor neurons to be uniquely vulnerable in ALS. In 2004, two independent groups of researchers discovered important clues that suggest a prominent role for these cellular structures in the death of motor neurons. In one study, investigators explored the effects of SOD1 mutations on mitochondria, and found that the vulnerability of motor neurons in ALS may be linked to an unexpected buildup of mutant SOD1 proteins inside the mitochondria of the spinal cord, where they can cause the subsequent degeneration of affected neurons. In a second study, a separate group of researchers explored the function of the mutant SOD1 protein and found that within spinal mitochondria, these proteins may bind to and specifically trap other proteins that are necessary for cells to survive. By taking the cell survival proteins out of circulation, the mutant SOD1 proteins may be contributing to the neuron's ultimate demise.

NINDS-funded investigators also continue to study the genetics of ALS—specifically the genetic changes that lead to inherited forms of disease. In addition to the mutations in SOD1 that cause an adult-onset form of ALS (ALS1), researchers also discovered in 2001 that a mutation in a different chromosome can cause a rare juvenile-onset form of ALS (ALS2). Then, in 2004, investigators found a link between a second form of childhood-onset ALS (ALS4)—one with a clinical syndrome distinct

from ALS2—and a known gene called Senataxin that is believed to play a role in the control of protein production following the activation of specific genes. This discovery involved the analysis of genetic material from four different families in the United States and Europe, highlighting the importance of the contributions from affected individuals to the research process.

Although researchers now have a better understanding of the genetic contributors to ALS, the search for genes that play a role in this disease is not yet over. For many years, NINDS has recognized that genetic researchers would benefit from having access to many well-characterized DNA samples from people with specific neurological conditions. To address this need, the Institute established a Human Genetic Resource Center at the Coriell Cell Repositories in New Jersey, in September 2002. As part of their contract, Coriell maintains a repository of data, cell lines, and DNA samples for the study of the genetic factors contributing to neurological diseases, including conditions like ALS that affect motor neurons. Genetic information is absolutely critical for the study of these conditions, as an understanding of the mutations in genes linked to ALS can help clinicians identify who is at risk for the disease, and can aid researchers in characterizing the disease process and identifying potential points of intervention. The positive effects of the repository on research in other neurological conditions are already evident, and NINDS is working with ALSA and the extramural research community to try to accelerate the rate of contributions of samples that are useful for ALS research.

As mentioned above, an understanding of the genes that play a role in the development of ALS can serve as a springboard for researchers searching for new strategies to treat the disease. The promising technique of RNA interference (RNAi) is one such strategy that is receiving a great deal of attention across many fields of medical research for its potential in treating diseases caused by known genetic mutations. Investigators both here in the U.S. and overseas have already begun to explore its applicability to inherited forms of ALS. Researchers initiate RNAi by delivering small pieces of genetic material that match those coding for an unwanted protein in a cell. By "binding up" the genetic intermediates that lead to toxic protein production, these proteins can be reduced or eliminated in the target cells. Although U.S. researchers are still in the very early stages of exploring this therapy for ALS, results from a recent study suggest that a well-designed RNAi strategy might be capable of simultaneously counteracting the more than 100 possible mutations in the SOD1 gene that can contribute to the development of hereditary forms of ALS. This proof-of-principle study is encouraging, and will hopefully lead to additional tests of this approach in animal models of the disease.

Though much of the basic science research described above is focused on improving our understanding of the causes of ALS, NINDS also supports other areas of fundamental neuroscience that may have an impact on the disease. For example, nervous system plasticity and stem cell research are particularly promising fields of study for ALS researchers, since the replacement of the motor neurons lost to the disease and the stimulation of these replacement cells to make contact with their original targets offer a reasonable therapeutic strategy.

It is widely recognized that the brain and spinal cord of adult mammals, including people, show a very limited capacity to regrow following injury. However, in recent years, researchers have shown that even the brains of adult mammals can generate new nerve cells under the right conditions. As an example, investigators have recently found that the brains of adult mice can generate new corticospinal motor neurons—which control voluntary movement via long nerve fibers they extend from the brain to the spinal cord—if the normal motor neurons are destroyed in a particular way. Importantly, these new nerve cells were also able to regrow their long extensions and make distant connections with their spinal cord targets. These findings are encouraging, as they suggest that in some cases, the body may be able to produce its own replacement cells, and that these cells may be able to make the contacts needed to restore lost function. Further understanding of what controls the generation of these cells and the growth of their long nerve fibers may facilitate the development and optimization of repair strategies for conditions like ALS and spinal cord injuries.

While promising, using "internal" replacement cells is only one approach to restoring the nerve cells lost in ALS. Investigators are continuing to explore other possible approaches as well, such as stimulating stem cells to produce a pool of motor neurons for nerve cell replacement therapy. Recently, one such group of researchers has found that motor neurons derived from human fetal neural stem cells can survive transplantation, make connections with target muscles, and support improvements in motor function in a rat model of motor neuron degeneration. In addition, a separate group of investigators has shown that they can successfully cause a line of federally-approved human embryonic stem cells to specialize to become motor neurons.

Their step-wise procedure involved a sequential application of growth-stimulating molecules to the cells—molecules that researchers had previously identified as being important during nervous system development. Potential uses for these types of cells include additional studies on the development of motor neurons, the screening of drugs that could be useful in treating ALS, the testing of therapeutics in animal models of disease, or ultimately, the replacement of motor neurons in people with ALS.

TOPICS FOR FUTURE STUDY

While ALS researchers understand more than ever about the causes of and potential treatments for this disease, many questions remain unanswered. Are there additional genes involved in hereditary ALS? How are the rare inherited forms of the disease linked to the more common sporadic forms? What triggers cell death in the sporadic cases? How can we intervene early in the disease process to protect motor neurons when they first become vulnerable to the cellular events underlying ALS? Why are these cells uniquely vulnerable? Are there additional drugs already available that might reduce the burden of ALS?

Recently-developed programs at NINDS, including the accelerated drug screening efforts and DNA repository, are fueling a more aggressive approach to these questions within the research community. To complement these efforts, NINDS also sponsored a workshop in January 2003 to explore the remaining gaps in our understanding of ALS and motor neuron biology, and released a Request for Applications (RFA) in August 2003, jointly with the Department of Veterans Affairs and ALSA. This solicitation encouraged studies of ALS in a broad range of research areas, including the causes of disease across broad populations, including genetic and environmental causes; the cellular interactions that contribute to the disease; the cellular and sub-cellular problems in affected tissues; novel approaches to delivery of therapies; and biomarkers for early disease detection. NINDS funded five applications that were responsive to this solicitation, and NIH's National Institute of Environmental Health Sciences contributed funds to support two additional awards.

CONCLUSIONS

NINDS has a history of funding strong basic science research on ALS, but the translation of these findings into effective clinical therapies for this disease has been extremely challenging. We realize that these challenges must be overcome, and we believe that the multifaceted approach we have taken will be the key. While still maintaining a vibrant program of preclinical research, we have now complemented this work with multiple opportunities for translational research to flourish, an active drug screening program that is leaving no stone unturned in the search for readily available drugs with promise against ALS, and several new and exciting clinical trials. While it is still not possible to guarantee when a cure for ALS will be developed, we are extremely encouraged by the progress of the research community, and we hope that this excitement extends to our most important constituents—the community of people with ALS and their families.

Mr. Chairman and Members of the Subcommittee, thank you for the opportunity to share this information with you. I will be happy to answer any questions you may have.

Senator SHELBY. Dr. Landis, you have touched on this already, but specifically has the doubling of the NIH budget and the significant increase in ALS specific funding helped accelerate clinical research on ALS and, if so, just briefly tell us how?

Dr. LANDIS. It absolutely has. Before these five new clinical trials were undertaken, we had only ever been able to do one clinical trial in ALS. We now have four underway and recruiting patients. A fifth that will begin.

That fifth trial is a direct result of a new program in translational research that we funded with the doubling. It involved a screen of a thousand FDA-approved compounds. Out of that, a number of hits arose. Through this translational research program that we were able to initiate with additional funds, we have three separate projects that are looking at different strategies for treating ALS.

Senator SHELBY. How many Americans are being treated for ALS roughly?

Dr. LANDIS. So the best figures that we have are that there are 5,000 patients that are diagnosed each year. Because this is a very rapidly-progressing disease, probably any one time, there are between 15,000 and 20,000 patients with ALS in this country.

Senator SHELBY. What are you doing to slow the progression of ALS? I know you said basically there is no cure yet——

Dr. LANDIS. Right.

Senator SHELBY [continuing]. For it. But you have made some—you have had a head start in that direction.

Dr. LANDIS. So all of the—out of the clinical trials that we are running, four are directed at neuroprotection, at slowing the progression. These drugs which are being tested in these clinical trials showed efficacy in this mouse model, SOD1 mouse model that I told you about.

Senator SHELBY. Doctor, is it true that only one drug, one FDA approved drug is on the market to treat ALS?

Dr. LANDIS. Yes. Just one.

Senator SHELBY. Just one?

Dr. LANDIS. Riluzole was approved 10 years ago. I would say that it is always depressing when you look at a disease as devastating as this one to have not made further progress.

The identification of the SOD1 mutation has allowed us to come up with better hypotheses and better animal models which has resulted in this significant increase in the number of strategies to develop therapies for ALS and these clinical trials.

We anticipate if we had more genes, we would have better ideas about how to design therapies and more clinical trials.

Senator SHELBY. What would you need to accelerate the research? I know you need more resources. We understand that and this is an appropriations committee——

Dr. LANDIS. Right.

Senator SHELBY [continuing]. There is a big fight everywhere for dollars today and always has been. But what could you do with additional money and what would you be thinking about?

Dr. LANDIS. So I have already indicated gene discovery which allows one to develop better pre-clinical models. We have a number of investigators who have assays which can be used for pre-clinical screening of potential therapeutic molecules.

The problem is oftentimes those initial screens, the compounds in those initials screens are not appropriate for direct movement to people. The FDA drug screen was important because all those were FDA approved. But in these new screens, we are coming up with molecules that are known to be safe, efficacious and get to the brain. So medicinal chemistry is a very important piece of what we would spend money on.

Senator SHELBY. Additional money would be very helpful.

Dr. LANDIS. Absolutely. We would put those monies to very good use. I think if I had been asked to testify at a hearing like this 5 years ago, I would have not had nearly as much positive to report.

I can tell you that we have invested the dollars, as you can see from the progress in ALS, very wisely. I hope that the fruits of that

investment will make a difference for patients now and patients in the future.

Senator SHELBY. Doctor, you referenced translational research.

Dr. LANDIS. Right.

Senator SHELBY. Research that bridges basic science findings with clinical therapies. Is any of this research relevant to ALS and, if so, how?

Dr. LANDIS. Absolutely. I will give you an example of one project which will begin funding on June 1.

We know that in some animal—in culture, tissue culture and in some animal models, insulin-like growth factor can make a difference for motor neuron survival. We have had one clinical trial that was successful, a second that was unsuccessful. We are conducting a third where it is given systemically.

But there is very good evidence that if you deliver it specifically to the terminals of motor neurons it helps survival. We are funding a translational project which will help investigators take this basic science finding to the point where they could apply for an IND for the FDA and begin a clinical trial.

Senator SHELBY. Thank you.

Senator Harkin.

Senator HARKIN. Thank you, Mr. Chairman.

Dr. Landis, my first question is very simple. Why has it taken so long to do anything about this disease? Forty years—60 years now since Lou Gehrig had it. We just know very little about it, what causes it, how to cure it.

Now I am finding out there is a whole subset of veterans who seem to be more susceptible to it. Why? I mean, why do we know so little about it? After all these years, why do we know so little about it?

Dr. LANDIS. Well, I do not know that I would say we do not know so little. It is that we do not know enough to actually make as big a difference as we would like to for patients.

Any of the neurodegenerative diseases, and we are responsible for a host of them, target specific populations of neurons. A puzzle has been why are motor neurons susceptible in ALS, why are dopamine neurons susceptible in Parkinson's. It is difficult to come up with strategies that allow you to look at individual populations of neurons and understand why they are susceptible and also to understand how to make them better.

For any nervous system disease, a major problem in delivering therapy is something called the blood-brain barrier. So there is a cellular barrier between the blood and the brain parenchyma which in most cases is protective, keeps toxins out. But if you want to deliver a therapy to the brain, you have to figure out a way to get it across the blood-brain barrier. This has been very challenging.

Therapy that we need that would be given systemically needs to be something that would get across the barrier or it needs to be delivered directly to parenchyma. We have made extraordinary strides and we will continue to make them. It is just not enough yet.

Senator HARKIN. One of the things I have learned about ALS is there is no real single test to determine whether someone has ALS.

Again, referring to my friend, Rob Borsellino, he talks about how you go from doctor to doctor to doctor and no one really knows what is going on until a lot of time passes. Are we making any progress in terms of early detection?

Dr. LANDIS. This is a very challenging problem. Again, it is not unique to ALS. It is a problem for many of our neurological diseases. There is a lot of redundancy and extra capacity in the nervous system and oftentimes symptoms do not appear until 20, 30, 40, 50, or 60 percent of the nerve cells are gone.

People often go to a general practitioner. They complain of weakness and it is not until they get to a neurologist that they actually get the appropriate tests done to determine what is wrong. If we had a blood test, it would be spectacular and we do not have that. So let me give you——

Senator HARKIN. Is your department looking at this?

Dr. LANDIS. So let me give you an example of one of the things that we could do if we had more money. In these ALS clinical trials, we will be enrolling a thousand or more patients. If we could collect biological samples from——

AMYOTROPHIC LATERAL SCLEROSIS

Senator HARKIN. How much more money do you need?

Dr. LANDIS. We would need $9 million.

But the biomarkers are a very interesting strategy. If we could collect blood, biological samples from each of these patients who were in these trials, and that is not part of the funding presently planned for those trials, we could then use proteomics and genomics to look at what markers are present, what markers are present in these patients that are absent from controls.

Senator HARKIN. Let me ask you another question about clinical trials. A couple of people have asked about clinical trials. If you enter a clinical trial, you do not know whether you are getting the drug or not.

Dr. LANDIS. Right.

Senator HARKIN. When my brother was dying of cancer and he was looking around for different things, there were some experimental drugs at the National Cancer Institute.

Dr. LANDIS. Right. Different institute.

Senator HARKIN. I know it is not yours. I understand that.

But my point is they were doing some clinical trials on some new drugs. My brother said, well, wait a minute. I am dying anyway. Why put me in a control group. Why not just give me the drug and see what happens.

So I mean, ALS, let us be frank about it, I mean, except for a few cases like Roger who has lived for 10 years, I mean, people are facing a death sentence.

Dr. LANDIS. Right.

Senator HARKIN. My brother knew he was dying of cancer and he said I do not care. If I die of the drug, I die of the drug. I am going to die one way or the other. I might as well take the drug.

My point is for this class of people, why—I can see doing some things—why not if someone really says, look, I am willing to be a guinea pig—my brother, for example, was willing to be a guinea pig—why not let them be a guinea pig? Why not?

Dr. LANDIS. So one of the basic tenets of clinical trials research is that you need to have a double blind control group——

Senator HARKIN. I understand that.

Dr. LANDIS [continuing]. To determine whether or not the effects that you are seeing are a function of placebo. There are very good examples where patients receiving the placebo have their health status improve as much as patients who are receiving a drug because of wiring in the brain that is able to influence health. The only way we are going to know if something is efficacious is to compare it to either best treatment or a control group. There is no simple way around that.

Senator HARKIN. I understand that. But I am just saying that if someone was willing, of sound mind, and could make that informed decision, why not let them? If there is an experimental drug out there that may have some hope and someone is willing as my brother was—I mean, he took a chance. Did not help him, but—and they did. They gave him an experimental drug because he was willing to do it.

Dr. LANDIS. So that is a strategy whereby the person could receive the drug knowing that they were receiving it.

Senator HARKIN. Right.

Dr. LANDIS. But not be part of a clinical trial because in the clinical trial, you have to——

Senator HARKIN. That is right. That is what I am saying.

Dr. LANDIS. Right. But then that person would not be participating in a clinical trial. If one of the goals——

Senator HARKIN. I am saying can that—I am asking a practical question. Could someone who is willing to take an experimental drug that someone has at least indicated may have some hope, could they get—we used to call it a——

Dr. LANDIS. Compassionate use of the drug.

Senator HARKIN. Compassionate release or whatever. Could that happen?

Dr. LANDIS. That could happen. That participation, taking a drug under circumstances like that could—and having access to drugs like that, through that kind of mechanism could slow the development of therapeutics because one of the most difficult problems in clinical research is patient recruitment. We would have much more information about therapies for diseases if we were able to speed recruitment.

We have on occasion closed trials because people will not volunteer. If you could get what you perceived to be the drug that would be effective outside of the trial, I think it would decrease recruitment.

Senator HARKIN. Thank you. I see my time is up.

Mr. Chairman, my time is up. But I hope that before Dr. Landis is excused, I would like to have one follow-up question.

Senator SHELBY. Senator Murray.

Senator MURRAY. Thank you, Mr. Chairman.

I have worked very hard to increase access to health care for our veterans because I believe we owe them nothing less than quality affordable health care for the service to our country. I think that is especially true of our veterans with service connected injuries or illness.

Recently the VA recognized that ALS as a possible service connected disability for our Persian Gulf veterans. I really applaud their decision to quickly recognize that link and to provide the necessary benefits for the veterans who have been affected.

I have also worked very hard to support expanded DOD research, Department of Defense research on surveillance of neurological diseases within the population of people who have served our country.

Can you tell me if NINDS has been working with the VA and the DOD on these efforts?

Dr. LANDIS. Absolutely. In the case of ALS, we have sponsored grant solicitations with the VA. We have a group of institutes and centers that interact with the DOD and with the VA more generally for neurodegenerative diseases. We have strong ties to them.

It is very clear when money is tight, we need to make sure that there is not duplication and also that all possible opportunities for advances are exploited.

Senator MURRAY. Is anybody at NINDS or any other institute at NIH working on research involving potential toxic exposure triggers for ALS?

Dr. LANDIS. Yes. This is within the purview of the National Institute of Environmental Health Sciences and they have recently funded a grant that will be looking at exposures and genetic susceptibilities to exposures that veterans might have.

Senator MURRAY. What are we learning?

Dr. LANDIS. It just got funded. So it will be unfortunately several years before we have results of that.

Senator MURRAY. Is there anything we can do to help better coordinate NIH research and DOD and VA research?

Dr. LANDIS. I think that we recognize the importance of coordination and that each part of the government has opportunities and strategies for expediting research and that we try to communicate often enough to make sure that each of us is doing the things that we can do best.

This also is true for the health voluntary organizations. We have significant coordination with them. They can sometimes be a bit more nimble to undertake novel and innovative strategies which then the NIH can pick up.

Senator MURRAY. Okay. If you can help us do the right thing so we can better coordinate this because I think this has tremendous potential. I think we have a responsibility obviously to make sure we are doing it correctly.

Chairman Shelby asked you a question about funding and what you could do with more funding. Let me ask you kind of the reverse of that. President's budget calls for 1 percent increase for NIH. I am deeply concerned about how the Appropriations Committee is going to be able to deal with the difficult task of balancing a lot of very, very difficult requests this year.

Can you tell us how this limited budget that has been proposed is going to impact your ongoing research?

Dr. LANDIS. So during the time when we received significant increases, it was possible for my institute to undertake a number of novel programs including translational research, expanding our support for clinical trials, increasing the number of physicians, sci-

entists that we are training, loan repayment, developing strategies for clinical trials to be undertaken in rare diseases.

At the present time with the President's budget, any time we do something new, a creative, innovative program, we are going to have to stop doing something that we have been doing. For example, some of the compounds that are being tried in clinical trials for ALS are also being looked at for other neurodegenerative diseases. They could have wide applicability.

Senator MURRAY. This budget, if it is enacted, is going to be a step backwards for you?

Dr. LANDIS. Yes.

Senator MURRAY. Will any researchers who are currently working be reduced? Are you going to have to lay off any researchers as a result?

Dr. LANDIS. If we are to fund new programs, we will have to stop funding old programs. One of the issues that the NIH is dealing with now is we know that we are not going to solve, sad to say, our diseases which are devastating, difficult, challenging diseases in the next 5 years.

To have investigators who will be able to carry the banner forward, we need to be investing in new and young investigators. For every young investigator, a senior investigator will be unfunded. For every senior investigator who is refunded, it means a junior investigator——

Senator MURRAY. So we know, if the President's budget is enacted, will any of the research that is currently being done be delayed?

Dr. LANDIS. We have gone from being able to fund 25 percent of grants at the height of the doubling to being able to fund 16 percent. What this means is that investigators will oftentimes have to submit their grant two or three times before they get money.

Senator MURRAY. So it will have a significant impact?

Dr. LANDIS. It will have a significant impact.

Senator MURRAY. Thank you. I really appreciate that.

Dr. LANDIS. You're welcome.

Senator MURRAY. Thank you, Mr. Chairman.

Senator SHELBY. Just one quick follow-up, Dr. Landis. Would it be helpful for ALS research if NIH funded researchers could access more stem cell lines than are currently allowed?

Dr. LANDIS. Scientists believe and make very compelling arguments that more stem cell lines would be better. Up until a year and a half ago when I took this job, I myself was a practicing scientist and know that it would be more useful. You have already outlined some of the issues: Mouse feeder layers, change in the stem cell lines with time. Not all stem cell lines are equally able to be turned into different derivatives. An issue which has not been discussed here is that it would be very helpful to have stem cell lines that have the same mutations in them that cause disease in humans. Right now the models that we have are at best models.

In order to get a mouse to have an ALS-like syndrome, you have to significantly over express the SOD1 mutation. Well, in people, one copy of a mutant SOD1 mutation causes disease. So we could make significant use of those lines were they to be—if more lines were to be available.

Senator SHELBY. Thank you, Dr. Landis, and we are going to continue to work with you to get appropriate funding——

Dr. LANDIS. Thank you very much.

Senator SHELBY [continuing]. To find some breakthrough in this disease.

Dr. LANDIS. We will put the money to good use.

Senator SHELBY. I know you will. Thank you very much.

Our second panel will be composed of Dr. Lucie Bruijn. She is the ALS Association science director and vice president. Before coming to the association, Dr. Bruijn focused her research on the treatment of neurodegenerative diseases. She received her Master's in Neuroscience and a Ph.D. in biochemistry from the University of London Institute of Psychiatry.

Dr. Bruijn, thank you for being here today. We look forward to your testimony.

We also will have on the second panel Mr. Eric Obermann of Huntsville, Alabama. He was diagnosed with ALS when he was 20 years old. Prior to his diagnosis, Eric was a student at Georgia Tech University where he studied computer science and played in the University symphony.

Now 23 years of age, Eric is unable to walk or talk and requires the use of a ventilator to help him breathe. I consider Eric to be an extraordinary young man and I am glad to have here today to hear his testimony.

Eric's father, Stewart Obermann, will accompany him. Mr. Obermann is the Chairman of the ALS Association, the North Alabama Friends Group. He is also the former chairman and CEO of Mobular Technologies, a Huntsville, Alabama based Internet company.

Mr. Obermann, I appreciate your willingness to sit at the table with Eric. I believe you can provide us all some important perspective on what effect ALS has had on the families of ALS patients.

Eric, I understand that you have prepared an opening statement today. From what I understand, it was only with significant time and effort. We all greatly appreciate your willingness to come today to tell us about your experience and to help us understand what ALS is and what it does.

Eric will be using a computer to give his testimony today. I understand it has taken him hours to put this together.

Then we have—you want to introduce Mr. Borsellino? You alluded to him earlier.

Senator HARKIN. I can run through the whole thing again, but I think I gave you Rob Borsellino's background and the fact that, again, what really strikes me is he has got two young kids. As I said, he has got a dedicated following in the State of Iowa.

We have all just been shaken by this happening to someone that we know and we follow daily. I guess we all have the same question: Why? Why just out of the clear blue sky does this happen to someone? I just want to thank Rob for being here today.

Senator SHELBY. Dr. Bruijn.

STATEMENT OF LUCIE BRUIJN, SCIENCE DIRECTOR AND VICE PRESIDENT, ALS ASSOCIATION

Dr. BRUIJN. Senator Shelby, Senator Harkin, and members of the subcommittee, thank you for inviting me today to appear before you. I commend you for holding this important hearing and for your efforts to support people with ALS in your home States and across the country.

My name is Lucie Bruijn. I am the science director and vice president for the ALS Association. The ALS Association is the only national not-for-profit health association dedicated solely to the fight against ALS or Lou Gehrig's disease.

In addition to serving as a resource for people with ALS and their families, the association and more than 41 chapters and affiliates across the country advocate for increased funding for ALS research and other health care reforms that respond to the needs of people with ALS.

The ALS Association is the largest private source of funding for ALS specific scientific research in the world and having awarded nearly over $30 million since 1995 to fund the best research to identify the cause, means of prevention, and cure for ALS.

ALSA funded scientists are currently looking at over 15 areas of research relevant to the disease including stem cell therapy, gene therapy approaches to treatment, biomarkers for early diagnosis of the disease, and identification, as Dr. Landis so well described, of new genes linked to the disease.

Currently ALSA is funding over a hundred promising research projects in 14 different countries in the world.

I am particularly pleased today to be appearing before you with my colleague, Dr. Story Landis, the Director of the National Institute of Neurological Disorders and Stroke. Dr. Landis is one of the world's leading experts in neurological disorders and has been one of ALSA's champions through the years.

It is through collaboration and with ALS experts throughout the world and together with the NIH that we can and we will make real progress in the disease.

I am pleased to say that through the efforts of the subcommittee and the Congress as a whole the ALS community has been able to benefit from vital funding provided to the NIH.

In fiscal year 2005 NIH and NINDS will provide more than $48 million in funding for ALS specific research and we hope that both the Congress and NIH will continue to make this funding a priority again in fiscal year 2006.

Collaborations between ALSA and NINDS have been extremely successful as Dr. Landis so clearly put today. As she mentioned, the screening of a thousand FDA approved compounds and model systems of ALS has led to one important compound being tested in clinical trials which the NINDS is funding.

In addition, I will emphasize again the importance of the establishment of this repository that the NINDS has done and this will provide us with important genetic clues. This is a resource that will be available widely for all investigators here and internationally to learn more about the genetics of the disease and ultimately to have new targets for therapies.

Why do we only have one FDA approved compound? We need to have much better and novel compounds and this is a huge challenge.

I am pleased, therefore, today to announce that the ALS Association is launching a new program, TREAT ALS, Translational Research Advancing Therapy for ALS.

Researchers have made tremendous progress over the years both scientifically and in technological fields. This knowledge has enabled us to design the laboratory models as described and lead to innovative ideas. The time is right to translate these more rapidly into clinical trials for patients.

TREAT ALS will be led by a steering committee of biologists and chemists and by noted Dr. Tom Maniatis of the Molecular and Cellular Biology Department of Harvard who lost his sister to the disease and testified here in May 2000.

So tangible progress will be turned towards the treatment of cures for the patients. Translational research and clinical trials will find the drugs that will prevent, halt, or significantly slow the disease. We will support the development of lead compounds.

We are extremely excited about this program, but it will need significant financial resources. We hope that both Congress and the NINDS will work with us so that this program will be a success and will benefit the ALS community in the United States and around the world.

In addition, we are currently working with Senator Reid and Members of the Congress to offer the establishment of a national ALS registry at the Centers for Disease Control.

A national patient registry does not exist today. The value of such a registry cannot be overstated. It is vital to understand the disease, its management, and the development of standards of care.

Another issue mentioned today is the need for more research of the veterans. One way in which the Congress can not only help veterans with ALS but all people with ALS is to support funding through the peer reviewed medical research program at the Department of Defense and recommend that ALS continue to be a disease that is studied under the program.

I have before you today a summary or white paper of ALS and the military for the records.

Finally, we hope that the promise of stem cell research will open up new avenues for ALS to understand the disease, as a drug screening tool, and in the future as a therapy for ALS.

We are very excited about the new and promising avenues for ALS research. Thank you again for this opportunity to appear before the subcommittee.

PREPARED STATEMENT

The ALS Association appreciates your previous support for our cause, urges your continued support, and we look forward to a Nation that commits itself to finding a treatment and ultimately a cure for this horrific disease.

I'm very happy to answer any questions you may have.

[The statement follows:]

PREPARED STATEMENT OF LUCIE BRUIJN

Chairman Specter, Senator Harkin, and Members of the Subcommittee, thank you for inviting me to appear before you today. I commend you for holding this important hearing and for your efforts to support people with ALS in your home states and across the country.

My name is Lucie Bruijn and I am the Science Director and Vice President for The ALS Association. The ALS Association (ALSA) is the only national not-for-profit health association dedicated solely to the fight against ALS, or Lou Gehrig's disease. In addition to serving as a resource for people with ALS (PALS) and their families, The Association and our more than 41 Chapters and affiliates across the country advocate for increased funding for ALS research and other health care reforms that respond to the needs of people with ALS. The ALS Association also is the largest private source of funding for ALS-specific scientific research in the world, having awarded nearly $30 million since 1995 to fund research seeking to identify the cause, means of prevention and cure for ALS.

ALS is a progressive, neurodegenerative disease that attacks nerve cells and pathways in the brain and spinal cord. Motor neurons reach from the brain to the spinal cord and from the spinal cord to muscles throughout the body. It is through these neurons that we are able to control all muscle movement, whether it be moving our arms and legs, or simply breathing or opening and closing our eyelids. As the disease progresses and these motor neurons cease to function and die, our ability to initiate and control muscle movement is lost, ultimately resulting in total paralysis in the later stages of the disease.

However, what makes ALS particularly devastating is that as people progressively lose the ability to walk, move their arms, talk and even breathe, their minds remain sharp; acutely aware of the limits ALS has imposed on their lives. That is to say, people in the later stages of the disease continue to think, reason, and have the same emotions, but they are trapped inside a body they no longer can control or use to communicate.

Early symptoms of ALS often include increasing muscle weakness, especially involving the arms and legs, slurred speech, and difficulty swallowing or breathing. When muscles no longer receive the messages from the motor neurons that they require to function, the muscles begin to atrophy (become smaller) and our arms and legs also begin to look thinner.

The average life expectancy for a person with ALS is two to five years from the time of diagnosis. We currently do not know what causes ALS or how it can be prevented and cured. Moreover, only one drug, approved by the FDA in late 1995, currently is available to treat ALS. Thus far, the drug, Rilutek, only has shown limited effects, prolonging life by just a few months.

As I mentioned, The ALS Association is the largest private source of funding for ALS-specific scientific research in the world. ALSA-funded scientists currently are looking into 15 different research areas relevant to ALS including stem cell and gene therapy approaches to treatment, biomarkers for early diagnosis of the disease and identification of new genes linked to the disease.

In May 2000, ALSA announced an aggressive, new initiative to rapidly accelerate the search for a cure for ALS—The Lou Gehrig Challenge: Cure ALS. In just 4 years, $16 million has been raised toward the initiative's $25 million goal. The strategy of this program is to recruit outstanding investigators, identify the most promising directions in ALS research and develop new ALS therapies. Currently, ALSA is funding more than 100 promising research projects around the world.

ALSA also regularly convenes scientific workshops to examine new trends in ALS research, providing vital leadership in areas ranging from examining research on ALS and the environment to engaging and educating young investigators.

ALSA's clinical management research program focuses on managing the care of ALS patients in areas such as nutrition, respiration, mobility, quality of life, and psychosocial needs. Currently, ALSA is funding ten clinical management research grants, representing a commitment of more than $414,000.

Although The Association continues to initiate, fund and support ALS research, our most effective and promising programs have occurred as the result of collaboration with ALS experts—individuals and institutions—throughout the world. I am pleased to say that through the efforts of this Subcommittee specifically, and the Congress as a whole, the ALS community has been able to benefit from vital funding provided to the National Institutes of Health and, within NIH, to the National Institute of Neurological Disorders and Stroke (NINDS). In Fiscal Year 2005, NIH and NINDS will provide more than $48 million in funding for ALS specific research and we hope that both the Congress and NIH will continue to make this funding a priority again in Fiscal Year 2006.

I am particularly pleased to be appearing before you today along side my colleague Dr. Story Landis, the Director of NINDS. As you know Dr. Landis is one of the world's leading experts on neurological disorders and has been one of ALSA's champions through the years.

Collaborations between ALSA and NINDS, including funding for research projects, have been extremely successful in recent years. For example, NINDS and ALSA partnered to screen 1,040 Food and Drug Administration (FDA) approved compounds in model systems of ALS, one of which will be funded by the NINDS in clinical trials later this year. In addition we are working with NINDS to establish a repository of genetic materials from ALS patients. This will be a vital resource internationally for investigators to understand more about the mechanisms leading to cell death in ALS.

As you can see, the leadership and expertise provided by NIH and NINDS have enabled us to move forward in the fight against ALS. But much more work remains to be done to learn more about the disease, its method of action, what causes ALS, and how it can prevented, treated, and ultimately cured.

It is with those goals in mind that The ALS Association today is launching TREAT ALS, a comprehensive new initiative that will concentrate efforts toward the rapid discovery of new therapeutics for ALS. TREAT ALS stands for Translational Research Advancing Therapy for ALS, and entails a two-pronged approach to discovering new treatments for the disease. First, the initiative will prioritize existing drugs that may be candidates for clinical trials, and second, it will take on the task of drug discovery by identifying new compounds with promise for the disease.

Researchers have made tremendous scientific and technological advances in the ALS field. We understand far more about the biological basis of the disease. This knowledge has enabled design of laboratory models of ALS that have yielded innovative ideas and clinical candidates. The time is right to translate these advances into effective therapeutics for ALS patients.

Already partnerships have formed, between The ALS Association (ALSA), other ALS organizations, and biotech to bring new and existing compounds to clinical trials. The National Institutes of Health in 2003 launched its own road map program that emphasizes the search for new medicines. ALSA is building on this momentum.

A steering committee is in place to guide TREAT ALS of prominent biologists, chemists and business advisors including noted biologist Tom Maniatis, Ph.D., of the Molecular and Cellular Biology Department at Harvard University, who lost his sister to ALS and testified before this Subcommittee in May 2000.

Now tangible progress will be turned towards patients to produce treatment success. Translational research and clinical trials will find the drugs which will prevent, halt or significantly slow down disease progression.

We anticipate supporting the development of lead compounds which can be ready for large scale, U.S. Food and Drug Administration approved clinical trials. This means the identification of small molecular entities that demonstrate bioactivity in animal models of ALS, and an acceptable safety profile, for evaluation in a Phase 1 clinical trial. At the same time, ALSA seeks to engage in one or two small pilot trials each year, of known or existing compounds, such as FDA-approved drugs.

TREAT ALS will align closely with existing trial centers as well as the National Institute of Neurological Disorders and Stroke clinical trials groups. NINDS and ALSA are already discussing the need for trials that span a larger geographic region and recognize the importance of involving many more clinical centers.

To create a powerful clinical trials process, ALSA will partner with the NINDS and other disease organizations. TREAT ALS will fund an annual clinical investigator award, will seek collaboration by multiple centers, and will sponsor educational workshops on effective design of clinical trials. ALSA can play a leading role in recruiting expertise outside the ALS field to help improve clinical trials design and outcome measures.

Concrete steps along the TREAT ALS path are already taken. ALSA has established a partnership with NINDS to identify and prioritize existing compounds with relevance to ALS and to move them forward expeditiously into clinical trials. ALSA is already funding several biotech companies with programs in ALS. Treat ALS enables further support of those efforts that are most likely to develop promising leads for ALS.

Treat ALS will consolidate these efforts, launch from them a series of clinical trials, and will translate a decade of progress into real promise for ALS patients.

We are extremely excited about TREAT ALS and we hope that both Congress and NINDS will work with us on the program so that together we can realize its full potential to benefit the ALS community in the United States and around the world.

In addition to research funding, this Subcommittee will, or soon will, have before it several other opportunities to help us make progress in defeating ALS. For example, we currently are working with Senator Reid and other Members of Congress to authorize the establishment of a national ALS registry at the Centers for Disease Control and Prevention. As you may know, a single national patient registry does not exist in the United States today. However it is urgently needed for ALS research, disease management and the development of standards of care. In addition to collecting data on the number of people living with ALS and the rate at which ALS occurs in the United States, the registry would collect other data, including information on environmental factors that may be associated with the disease, the age, race and ethnicity of individuals with ALS, family history, and additional information that will promote a better understanding of the disease. The registry would identify, build upon, and coordinate with existing data, surveillance systems and registries, such as state-based ALS registries, the Department of Veterans Affairs ALS registry and the National Institute of Neurological Disorders and Stroke (NINDS) repository. We hope that when the opportunity arises, the Committee will support funding for this critical effort.

While not under the jurisdiction of this Subcommittee, but under the jurisdiction of the full Committee, are other initiatives supported by ALSA that are focused on learning more about the apparent connection between ALS and military service. As you may know, several studies, including studies funded by the Department of Defense and the Veterans Administration, have found that veterans of the 1991 Gulf War are nearly twice as likely to die of ALS as veterans who did not serve in the Gulf War. Another study, published earlier this year by epidemiologists from the Harvard School of Public Health, found that men with a history of any military service, whether Vietnam, Korea, or World War II, are nearly 60 percent more likely to die of ALS than men in the general population.

We believe additional funding is necessary to further explore the questions that arise as a result of these studies, including to definitively determine what is the risk of ALS for our military men and women, and what is causing these elevated risks. The Committee can help in this endeavor by supporting funding for the Peer Reviewed Medical Research Program (PRMRP) at the Department of Defense, and recommending that ALS continue to be a disease that is studied under the program, funding for which is provided in the Senate through the DOD Appropriations bill. You also can support other research and programs at the DOD and VA, including the VA ALS registry and other initiatives that will respond to the recommendations to Congress given by the Research Advisory Committee on Gulf War Veterans' Illnesses. Those recommendations called on Congress to set a national goal to develop treatments for the diseases affecting Gulf War veterans within five years.

Finally, we are hopeful that funding stem cell research will allow us to explore the potential it holds for ALS. While it remains unclear whether this research will produce immediate treatments for ALS, we believe that any effort which may further our understanding of the disease must be explored. Moreover, as is the case with all research, advances in stem cell research may lead to unexpected developments that could help us bring new treatments from the lab to the patient earlier than if this research was discouraged.

Clearly we have challenges before us if we are to continue the advances we have made in ALS research. There are many areas of research to explore and many opportunities for us as a country to make progress in improving our understanding of the disease, how it can be prevented, treated and cured. The work of The ALS Association and our new TREAT ALS program is one example of what can be done. The work conducted and supported by NIH and NINDS is another as are the efforts of the Department of Defense of the Department of Veterans Affairs. However, it is your work here, in this Subcommittee—in Congress—that can make possible our collective goal of relegating ALS to a disease of the past. And I urge you provide the support that is needed in this effort. Equally important, I urge you and the Congress to provide the leadership that is needed to encourage and facilitate collaboration among these varied interests who share the common goal of finding a treatment and cure for ALS. Together we can light the way in finding a treatment and cure for ALS.

Thank you again for inviting me to appear before the Subcommittee. The ALS Association appreciates your previous support for our cause and we look forward to continuing to work with you as the nation commits itself to finding a treatment and cure for this horrific disease.

I am happy to answer any questions you may have.

Senator SHELBY. Mr. Obermann, could you just tell the audience, if you could, how your son put this statement together. I think this would be interesting.

Mr. OBERMANN. Certainly, Mr. Chairman. Under difficult circumstances. Certainly Eric has been asked in the past to write several articles, mostly in medical journals.

He is a ventilator user. There is great interest in how people can try to proceed with as normal a daily life as possible doing things like traveling which just a few years was not possible. So he had written some articles previously for a journal there. He had actually done a brief article that the ALS Association had used as part of their fund raising.

We pieced together parts of those previous stories that he had written along with some comments and responses that he gave in response to some questions that my wife, Marcia, and I asked Eric. Then I did most of the word processing, but all of the thoughts and the emotions that you are able to hear are from Eric.

Senator SHELBY. Thank you.

STATEMENT OF ERIC OBERMANN

Mr. OBERMANN. Good morning. Can everyone hear me?

My name is Eric Obermann and I live in Huntsville, Alabama. I am 23 years old and I have ALS.

I would first like to thank you all for giving me the opportunity to talk with you about this disease. I think it is very important for all of you to hear and see firsthand how ALS affects both patients and their families. I am truly honored to be here.

To begin my story, I am going to take you back to May 2000. I was a typical 18-year-old, excited to graduate from high school and nervous about going to college.

I went to Grissom High, a fine public school named in honor of astronaut Gus Grissom. Grissom is known throughout the State for its outstanding bands and I was a first chair clarinetist and enjoyed playing with the marching band. I worked hard in high school, got good grades, and was accepted into Georgia Tech.

There have been computers in our home since I was 7 or 8 years old, so I became an avid computer user from early on. Computer science seemed to be a very promising field, so that fall I enrolled at Tech as a CS major. I expected to graduate in about 4 years with a Bachelor's Degree in computer science and management.

I also enjoyed a lot of outdoor activities, such as helping my parents with landscaping projects, riding my bike, and taking backpacking trips with my dad. Life was good and my future looked great.

The first signs of trouble came in the fall of 2000 just as I was entering my freshman year at Georgia Tech. I had noticed that my ability to play clarinet was beginning to slip as I was developing weakness in my mouth and tongue.

Soon I also started to have a speech impedient, very subtle at first, but it quickly became quite noticeable. My words were slurred and I could not enunciate. It was very frustrating as people started to not understand what I was saying.

We decided to seek medical advice, starting with an ear, nose and throat doctor. He noticed my tongue was atrophied or shrinking which explained my speech problem.

Fearing a brain tumor or disease, he scheduled an MRI for that same day and referred us immediately to a neurologist. It was then that I realized that this may be a serious issue. But my family remained optimistic that it was something the doctors could fix and my life could go on as normal.

The first neurologist ran a few tests and referred us to a neuromuscular specialist who understands what he called these unusual cases.

Since I was in Atlanta going to school, we made an appointment at Emory University. The specialist completed a thorough examination, reviewed the notes from the other neurologist, and left the room.

When he came back, he delivered the bad news. Eric, I think you have ALS. Do you know what that is? I had no idea and looked at my mom, a nurse, who was with me. She looked stunned, blank.

The doctor continued, it is a progressive neuromuscular disease. It is fatal, but you probably have 3 to 5 years to live. Keep doing what you are doing. Get exercise and build up your body and muscles. That may allow you to last a little longer.

My mom and I left in total silence. I felt as though I had just received a death sentence. Mom took me out to eat at one of our favorite places. But feeling sick to our stomachs, neither of us could eat.

We just looked at each other wondering what had just happened to us. We were in shock. An hour later, mom took me back to the dorm to drop me off. I had to go back to class that afternoon.

Over the next few weeks, we thought about what the doctor at Emory had said. It did not make sense. We were sure the doctors were all wrong. So we decided to seek a second opinion and then a third and a fourth.

We went to four neurology specialists across the country. None of them said the same thing other than I was a very unusual case. They all essentially told me, I am sorry. There is nothing we can do for you. Check back in 6 months and we will see where you are then.

I was in denial, unwilling to accept the fact that I had this beast of a disease they call ALS.

Over time, we concluded that even if what I had was not typical ALS, it was progressing as they had predicted. My speech became unintelligible. I had shortness of breath. I had a hard time chewing and swallowing my food. Eating a normal meal became a huge effort, taking almost an hour.

I often would have a choking fit when I ate right there in front of my new college friends. I started losing weight rapidly.

Through it all, I kept going to class full time, hanging out with my friends at school and was starting to really enjoy my new life as a college student. But there was no ignoring the obvious signs of deterioration that my body was undergoing. My friends noticed it too, although none of them knew exactly what was wrong. I could not bring myself to tell them.

When I came home for Christmas break during my sophomore year, we decided to have a feeding tube inserted so I could take my food without having to chew or swallow. This worked fine for a while, but soon I began choking on my saliva. Sometimes I would cough and gag for several minutes before I could breathe again.

In the spring of 2002, I came down with severe pneumonia caused by aspiration of fluids from my mouth. I spent my spring break in the ICU.

After a few weeks, I recovered and we drove over to Atlanta to move me out of my dorm room. I realized at that time that I would never be going back to school at Georgia Tech. This was a very painful experience for me.

Living back at home again, I had repeated bouts of pneumonia that spring and was hospitalized three times, spending a total of 45 days in the ICU. The cause each time was aspiration of fluids from my mouth.

The only solution was to do a laryngectomy, a radical procedure involving removal of my voice box and routing my trachea out through a hole in my neck. This was the only way to prevent the pneumonia, my doctors told me.

On June 1, 2002, I had the surgery. I knew I would never speak, drink, eat, or smell again. I then started using a respirator, also called a ventilator, at night as I could no longer breathe on my own when I was lying down.

My disease progressed rapidly in distinct downward steps. After each step, there would be a temporary halt to the progression and my condition would stabilize. I remember that with each of these phases, my family and I would think, okay, we can deal with this. If it only would stop here, we will be all right. But it never stopped. There was always another slide coming. We just did not know exactly what it would be or when.

Concealing my laryngectomy stoma with a foam filter, I went back to school that fall at the University of Alabama at Huntsville to continue my degree. I soon had to begin wearing a neck brace as my neck could no longer support my head.

My face became paralyzed so I could not laugh, smile, or grimace from pain. Without the ability to speak, I got a speech synthesis computer similar to the one I am using today, but with which I could talk by typing in words on a keyboard.

My family and I also took American Sign Language courses which allowed us to communicate without a machine. Unfortunately, the next symptom was that my hands and arms began to lose strength and dexterity.

Soon I was unable to type and my sign language became as garbled as my speech had been.

My new computer which I operate using a switch under my toe was given to me by Jimmy McDonald, one of my friends from our ALS support group who passed away 1 year ago. Jimmy's wife, Bonnie, who along with Jimmy started our support group in Huntsville 3 years ago is here with me today.

In 2003, I had to begin using the ventilator 24 hours a day as I could no longer breathe at all without assistance. Fortunately we found a portable ventilator which can be strapped to the back of

my wheelchair. Very soon my legs also gave out. No longer able to drive my car, I had to withdraw from school again.

That was it. I could no longer walk, talk, eat, or even breathe without assistance. Trying to be optimistic, I concluded that having lost so much there is not really much else that can happen.

That brings us up to the present, May 2005. I am a ventilator-dependent quadriplegic who requires 24 hour skilled nursing care and I am fully dependent on my parents. This is certainly not what I had in mind as I first headed off to school only 5 years ago.

I was just starting a new life as a young adult. By now I thought I would have my degree, a new profession in perhaps a new city, maybe a girlfriend, and the financial independence that every young person dreams of. Obviously ALS changed all those plans dramatically.

But two things ALS cannot take from me are my mind and my spirit. Despite the radical changes my body has undergone, I am still the same guy inside. I am actually much tougher mentally and have learned to appreciate many things I used to take for granted.

My faith has grown stronger as I have a lot of time to listen to what God has to say to me. I now have many wonderful friends in the ALS community that I might not have met. These are a few of the many blessings that I have received during my illness.

Despite my severe disability and thanks to the determination and commitment of my loving parents and the support of my sister, Lauren, I am able to do things that few people in my condition ever dreamed of.

I can travel and go on trips with my family, go to movies and concerts, attend swim therapy, and take walks in the woods. I am very blessed that there is technology to enable me to do these activities. My family has dedicated themselves to helping me live with ALS rather than simply waiting to die from it.

Which brings me to the reason I am here today. I have never been a very outgoing person or one who seeks the limelight. Rather, I have always been quiet, analytical, and somewhat introspective.

But my situation today is such that having an opportunity to speak out on behalf of all ALS patients is very important to me. This is something I can do and something I want to do because it is so important to so many people who are suffering.

There are hundreds of advocates from the ALS Association who traveled here this week to emphasize to you what a devastating disease ALS is. Hopefully, after hearing my brief story, you will have a better understanding of how the patient as well as the family is impacted in such a devastating way.

I come here with hopes that we can make a difference in how Congress will provide for ALS patients and their families. We are all very fortunate to live in a country that has risen to great challenges time after time and conquered them.

In 1960, our country embarked on a program to put a man on the moon and return him safely and did this in less than a decade. ALS was first identified in 1869, 136 years ago. Yet, the prognosis today is exactly the same as it was then.

How can this be? The answer is simple. We need to dedicate more funding to researching treatments and a cure. We have bril-

liant researchers working on this disease, but they simply need more resources to accelerate the progress they are making.

PREPARED STATEMENT

It may be too late for me and for many of my friends here, but hopefully any positive results from our visit this week will help hundreds of thousands of Americans with ALS in the future.

Thank you again for your time. I am very grateful that I have had this opportunity to speak to you.

[The statement follows:]

PREPARED STATEMENT OF ERIC OBERMANN

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It may be too late for me, and for many of my friends here, but hopefully any positive results from our visit this week will help hundreds of thousands of Americans with ALS in the future.

Thank you again for your time, I am very grateful that I have had this opportunity to speak to you.

Senator SHELBY. Thank you.

Mr. Borsellino.

STATEMENT OF ROBERT BORSELLINO

Mr. BORSELLINO. Well, Eric, need I say, was about as articulate as one can be about this illness.

My name is Rob Borsellino. I do live in Iowa, but I am from the Bronx, so I just want to point out when a U.S. Senator calls and asks you to testify, if you are an Italian from the Bronx, there's that moment of hesitation, uncomfortableness.

But Senator Harkin's staff assured me that they basically wanted to, you know, put a face on this. I would not have to name names.

I am 55. I have a wife, two teenage sons, and I have been in pretty good health up until now. I do not drink. I do not smoke. I cannot remember the last time I had a joint.

Anyway, back in November, I started slurring my words. I was tired all the time. I felt weak. Friends kept asking me if I was drinking again. Eventually I went to see a doctor. He ran some tests and he asked me to go see another doctor, a nerve specialist.

That guy ran more tests. At the end of the session, he is telling me I have this fatal, incurable, exotic-sounding disease, ALS, also called Lou Gehrig's disease. Then he is telling me most people live 2 to 5 years with this.

It has been 6 months. I have been to see three other nerve specialists in various parts of the country. I am hearing the same

thing and every time I hear it, I am in denial. I find myself sitting there avoiding the important stuff.

I will not be there for my kids' weddings. I will not know my grand kids. I have got to do a will. You have got to deal with life insurance, all that stuff.

What is most shocking to me is that conventional medicine has nothing to offer people in my situation except this one FDA approved medicine that will keep you alive another 2 or 3 months.

So, you know, I am doing—and I would not like this to be public—I am doing Yoga and acupuncture and massage therapy. I am taking Chinese herbal medicines, anti something or others. It is the only way I feel I can engage in this stuff rather than just sit and wait for things to get worse.

Something else that is making me nuts. I am hearing about and from Americans with these new degenerative diseases, they have to go to Portugal. They got to go to India for surgery. ALS patients in Iowa are going to China for stem cell surgery because they cannot get it here in the United States.

Anyway, and when you talk to people about that, most people do not know what this is, so they are stunned. They are appalled that this is going on and it is a complete mystery to them and to me why this illness remains a death sentence which is pretty much what it is.

One theory is that it is not that so few people have it. It is that people live such a short time that there has not been a movement in effect to deal with it. That is something we got to look at.

One other quick thing. You go through life. You talk to your kids. You say, you know, be careful, use sun screen, mosquito repellant, do not drink and drive, practice safe sex. With ALS, that stuff is worthless. It is always a death sentence.

We need more information about what factors are common among ALS patients. I am told—and, again, I am not an authority on this sort of thing, but we need to have a database to keep track of those things that are affecting people. We need hopeful, meaningful treatment. We need a cure.

One last thing I did want to say real quick. When Senator Harkin was saying he wanted me to put a face on this, I have been looking for some sort of analogy and the best thing I could come up with from me personally is the fact that it is little league season right now and I cannot play catch with my 15-year-old.

Thank you.

Senator SHELBY. Dr. Bruijn, in your opinion, what is the most promising research currently being conducted with respect to ALS in your judgment?

Dr. BRUIJN. I think there are a few if I may mention. I think one exciting thing is the potential of gene therapy as Dr. Landis mentioned. We know that the motor neurons need to be nurtured by the right factors. We are working hard on the pre-clinical work to try and get the first gene therapy trial for ALS. It is difficult and challenging.

I think the other area is the potential of finding new genes. So I do think that the most promising would be to capture all the samples from the population of ALS through a registry and through the repository that we are working so hard on because I do believe that

we are close, but there are a lot of tools that we still need to make that difference.

Senator SHELBY. What do you need to do to put that database together? I know you need money.

Dr. BRUIJN. One is money and a commitment from the community to participate. I think that the patient community——

Senator SHELBY. How much money roughly?

Dr. BRUIJN. I know that the Veteran Affairs Registry is something in the order of $5 million, at least now, for 5 years. So we recognize that that is only the beginning and the Veteran Registry is only a part of our ALS population.

So I think it is going to be several million. But to even get it started, a million or so. The exact figures are difficult, but to get it started would be a huge commitment.

Senator SHELBY. The ALS Association has had a lot of experience working cooperatively with the NIH. From that experience, could you tell us what forms of specific cooperation work well and where, if anywhere, are the impediments? Where are the problems?

Dr. BRUIJN. I think what works well is that we are able to through our programs and our granting get very exciting new and sometimes risky projects on the table. Certainly those small amounts of funding seed the ideas that then enable larger funding from the NIH.

Our partnerships as well in asking for proposals has been great. The biggest impediment is going to be the lack of increased funding because I think that we have now an investigator pool both young and more established that are outstanding. The thought that those could be restricted would be a real shame.

Senator SHELBY. What conditions have come together to make it possible? You are talking about your research and the doubling of the NIH budget. Has that enabled you to get to this point?

Dr. BRUIJN. I think it has made a huge impact. I think that what has also helped is the awareness through the patients and through the community, the recognition that the veterans are affected, and the excitement that we have the first tantalizing models to be able to do the research.

So I am proud to say that we have investigators worldwide, people that would maybe not have thought about researching ALS that are now working on the disease.

Senator SHELBY. Is there cooperative research now between the VA medical researchers, DOD researchers, NIH people?

Dr. BRUIJN. I hope that there could be an increase. An example is that the VA Registry is a very exciting resource and it is not entirely clear how we can capitalize on that and combine that repository that is being done there with the NINDS repository.

I think with leadership such as Dr. Landis, we can make it happen. But I do think that we have to all be talking very seriously.

Senator SHELBY. Senator Harkin has spoken to me about this and he is in a position and hope Senator Specter will be to lead that effort.

Dr. BRUIJN. Excellent. Thank you.

Senator SHELBY. Doctor, would you give us a couple of examples of the type of research that would be possible maybe if an ALS registry were created? In other words, what do you get from that?

Dr. BRUIJN. Well, one thing that is so striking about the disease—and I wanted to touch on the question you have, why do we not have therapies—it is a very challenging one because as you see here in the room today, we have those that have impaired speech, those that have less mobility, and those that live a long time and a short time.

It is a real mystery to us that this is all really quite a variety under one umbrella. So if we had a registry, we could start to understand how we could perhaps subdivide the groups and maybe the failure in getting good therapies is that we are not targeting the right population with the right therapy.

So I think that this is going to be a critical and important need that will take time because it will take several years and commitments over several years to get all that data together.

Additionally, if we can get the genetics, we would confine new targets. So the mystery of the disease and the tardiness is because of its challenge.

Senator SHELBY. Thank you.

Mr. Obermann, when you first learned of your son's diagnosis, did you have the information you needed to make choices regarding his care? In other words, what type of information was available to you and was it adequate or was it trial and error?

Mr. OBERMANN. It was probably really the latter. We turned initially to the Internet and we found tons of information about the disease and probably too much information. It became very difficult to sort out what might be useful or reasonable information from—there is a lot of stuff out in the Internet that truly is both inaccurate and not useful at all for patient care. We spent time going to these clinics and we got good information there as well.

But I would say that even though we are a small organization, our ALSA chapter is relatively young, serving about 10 families right now, that the exchange of information we do in our monthly support group probably was the best source of real practical information about where to get DME, you know, which physicians are doing which prescriptions and things of that sort.

So I think turning to the other patients is where the real bulk of valuable information was available to us.

Senator SHELBY. What has been the impact—it has had to have had an impact—of your son's diagnosis and care on your family?

Mr. OBERMANN. I guess to try to sum it up in a word, it is profound. As Eric said, he said he requires 24-hour skilled nursing care. I do not think it would shock any of you to know that neither insurance nor Medicare will provide that for a family. So my family like most of the families that you see in this room here, the primary caregiver, the bulk of that burden falls to the spouse, the parent, or the children.

I think we are very fortunate in our family that Eric has both parents available to care for him and actually the intensity of his care as his disease has progressed has required both my wife and I to leave our full-time professions and spend more time at home working with him.

Senator SHELBY. Thank you.

Mr. Borsellino, how do we encourage ALS patients to participate in clinical trials if only half of them will receive the medication?

You know, they have got to have a database. They have got to have some way to manage.

Mr. BORSELLINO. Well, I am the wrong one to ask because up to now I have avoided clinical trials for that very reason.

The weird thing about this illness is that there is so little in terms of hope, in terms of positive, good news. To the extent that we can come up with some good news—and I realize if you do not do the clinical trials, you are not going to have the results. I understand that.

But as I say, I am the wrong one to ask that particular question. I am not going to try to finesse it.

Senator SHELBY. Dr. Bruijn, you want to comment on that?

Dr. BRUIJN. I would like to make a comment and I think really to honor as well a good colleague and friend, Dr. Rick Ulney, who is a physician who was struck by ALS. He himself is in a controlled double-blinded study, recognizing the need of it.

But I also do think that a solution to this might be better design of clinical trials and we are committed to working with investigators on that. There are cross-over trials in which there is a much shorter period of time that a patient needs to be on that placebo and then they can go onto the drug.

I think that by creative design, we can meet both challenges, that we really find a drug that makes a difference and help the patients.

Senator SHELBY. Mr. Borsellino, you have tried alternative medicines in your struggle. Any of them seem to help and what has helped, if any?

Mr. BORSELLINO. No. I mean, I have tried these herbal Chinese anti something or other and, you know, drinking vegetable juice and, you know, washing down the soy, whatever. I have tried whatever was put in front of me, whatever I heard was out there.

Again, I do not know if it works. I do not know if I would be sitting here in a wheelchair unable to speak had I not done some of the stuff.

Senator SHELBY. Sure.

Mr. BORSELLINO. So I will say that I have rapidly deteriorated in my voice in the past 6 months, weakness in my hands, a lot of the traditional problems with ALS. So, again, I do not know if it has helped.

Senator SHELBY. Okay. Thank you.

Senator Harkin, thanks for your indulgence.

Senator HARKIN. No. Thank you, Mr. Chairman. Those are all good questions and many of those I wanted to ask myself.

But following up, Dr. Bruijn, just on that last question and what Rob was saying, does your association have any even anecdotal types of information from other countries, China, other countries, where obviously they have people that have ALS too that they may have treated with other kinds of interventions? Do you have any information on that at all?

Dr. BRUIJN. Sir, we have an international research program. In fact, many of our investigators are funded worldwide. We do keep a close eye on what is going on.

I think it is important to note that although intriguing and, of course, I understand in a desperate situation without therapies, we

would try and go anywhere, I am concerned about the kinds of therapies going on abroad which are not done in any kind of trial and are not providing any kind of data and at huge cost, $20,000 plus to a patient.

So I think that our challenge here is not that there are better therapies out there that we cannot get to, but to be able to learn from it. I think that it is not appropriate to suddenly be doing these kinds of things in patients here. But we do need to engage in the laboratory's research, especially for stem cells in an aggressive way.

So I think if there was something really promising, we will go out there and find it. I think that these kinds of studies are difficult without any rigorous follow-up.

Senator HARKIN. I am glad you mentioned stem cells. As you heard from Dr. Landis earlier, after we doubled—and, again, as often as I can, I pay tribute to Senator Specter for helping lead that charge here and the two Senators you see here, Senator Murray and Senator Shelby, both very supportive of doubling the funding for NIH. Got the job done. We were at this plateau where, as you heard Dr. Landis say, about one out of four peer reviewed research were funded and now it is down to 16 percent and falling.

So, again, whatever you can do to help us get the funding back up for NIH would be very helpful. We did not double the funding to then fall of a cliff. We doubled the funding to keep going.

Basically the budget that was sent to us is the lowest increase, I think, in NIH in, well, a long time. I do not know how many years it is, but we need to do better than that.

Second, on stem cells, you mentioned that—you heard Dr. Landis' response to me—we have these clinical trials and everything going on. But, again, it just seems to me a lot of these drugs that we are investigating are interventions that will keep you alive a couple more months or 5 more months or maybe relieve some of the symptomatic symptoms of ALS.

In order to get at the root cause, many scientists believe that stem cells really hold the answer and that this could be, as my staff person said, the low hanging fruit for stem cell research. Even Dr. Wilmot who cloned dolly, has said that of all of the candidates for stem cell intervention, ALS could be the primary one.

So, again, if you would just address that just for a couple minutes about the need to more aggressively have stem cell research, I would appreciate that.

Dr. BRUIJN. I absolutely agree that it has huge promise. I think that we must not lose the fact that we have many other avenues that we need to be focused on.

I think that the challenge of stem cells as a therapy, we need to diagnose the disease earlier because as we heard, at the point that 50 percent our motor neurons are lost, it is a hard task then to replace all those neurons with stem cells.

Having said that, I absolutely endorse that stem cells are a vital opportunity not to replace the neurons themselves. I think that is a bigger challenge. If we remember that we have got to connect our spinal cord to our muscle in meter and length. We have done it in dishes not on patients.

But the stem cells are an absolutely vital tool as a pump and a resource like gene therapy to put the right things around those motor neurons. So there is a huge promise and the biggest problem is the lines are available because we cannot even use them as a pump to facilitate the right factors with the contamination of the mouse feeders.

So what I find so frustrating as a scientist and with my colleagues is that we have to have separate buildings to be able to do studies that are either government funded or privately funded. I think that there has to be an openness to move exciting avenues with appropriate ethical guidelines forward. And so the stem cell lines are a real limitation at this point.

Senator HARKIN. Thank you, Dr. Bruijn.

Again, I just want to thank Eric for your testimony, for taking the effort I know that it took to be here today and to your family.

Let me just express my high esteem for you, Mr. Obermann, and your wife and your family for what you have done to support your son. It takes its toll and I do not know you personally. I think is probably the first time we have ever met. But, again, just my highest esteem for what you are doing. You provide a great example of what a family ought to be about. Appreciate it very much.

Mr. OBERMANN. Thank you.

Senator HARKIN. Again, Eric, you mentioned about we did this moon shot. We did the moon thing in the sixties. Well, we need that kind of moon shot again in medical research. We could if we set the goals and we funded it.

See, biomedical research is basically like opening doors. If you've got 10 doors to open, you do not know what is behind them.

Now, if you are going to open five doors and leave five unopened, then what are your odds? I mean, maybe the answer lies behind one of the doors you have not opened. If you could open 8 out of 10, your odds go up that you are going to find what you are looking for.

So when we say we are down to 16 percent, we are opening 1 out of 7 doors. You see what the odds are. So that is why we have got to get the research. You have to open more of those doors. A lot of them will come to dead ends. But the more we open, the more our chances are that we are going to find something.

So I hope that we can sort of use what Eric said about a moon shot and look at it in that way, that within 10 years, we could open more of these doors and try to get at some of the answers.

To my friend, Rob Borsellino, thank you.

I just have to admit in front of everyone here that when I found out about Rob—I had had dinner with he and his wife last summer and no indication about anything. When I had read in his column that he had ALS, I could not even call him up.

Here is a guy that has been poking fun at me a long time, see. Usually when I call him, I try to get back at him. As you can tell, he is a man of great humor. But how do you respond to something like this?

So, again, a lot of us feel we know Rob better than we know others because we read him every day and we read his columns and his columns are always very funny and sometimes at our expense.

So you really know someone like this. You feel that it is a part of your family that something has happened to them.

So I do not know if I have any questions, Rob, other than just, you know, as all of you can tell, here is someone still who can express humor. To me, I just think that is a great attribute.

Mr. BORSELLINO. I was not trying to be funny.

Senator HARKIN. No. I know that.

You just are naturally. But you have great courage and I hope you keep writing about it.

Mr. BORSELLINO. Thank you, Senator. I appreciate it.

Senator HARKIN. Keep writing about it. Keep telling people what is happening out there.

Mr. BORSELLINO. Is it appropriate to put in a pitch with the book I have coming out——

Senator HARKIN. Absolutely.

Mr. BORSELLINO [continuing]. Next month, at a time like this?

Senator HARKIN. You have a book coming out?

Mr. BORSELLINO. Yeah.

Senator HARKIN. See, that is how little I know.

Senator SHELBY. Talk about it. When is it coming?

Mr. BORSELLINO. It should be available May 25. On the Des Moines Register, web site, you can find what you need. I cannot believe I am doing this.

Senator HARKIN. Rob, thank you very much for being here. Thank you.

Senator SHELBY. Thank you.

Senator Murray.

Senator MURRAY. Well, thank you, Mr. Chairman. Thank you to all of our witnesses today for your courage and coming and sharing with us what we need to know to help make life better for those that are here, but really for those that are coming after as we know the history of this disease. But your being here does make a difference.

I know we have a vote coming up in a minute and we also have another panel that we want to hear from today. So I will just be really brief. I just want to focus on one area and that's respite care.

My own father had multiple sclerosis and I know well that when a patient gets a diagnosis of a disease like that, it is the entire family that gets the diagnosis. I know what a struggle it was caring for my dad throughout the rest of his life and for my own mother and for all of us. We were seven young kids when he was diagnosed.

I often think that the family gets left behind, forgotten, but mostly because families, I know how hard it was for us to say we need help because you want the person who has been diagnosed to know you are there every single minute and you do not want to look like you need help.

But I do think respite care is especially important. I think those who are suffering from ALS want to know that their families are well cared for as well. I think for families, it is extremely important so you can be there 100 percent when you have to.

Mr. Obermann, you are here with your son and I know you just said that you and your wife are both taking leaves from your jobs to care for him.

Can you talk for a minute just about what we can do as Members of Congress? We have a bill called "Respite Care for Life," things like that that will help families be able to be the best they can for the person they are caring for.

Mr. OBERMANN. I think that bill is probably the brightest thing on the visible horizon for families like ours and like yours earlier. You really cannot overstate how challenging it is on a family.

You are dealing with this devastating disease of a loved one. It has great emotional toll obviously, also great financial toll. They estimate that I think in the later stages of the disease, it costs roughly $200,000 per year to maintain a person.

Eric is certainly at that stage being ventilator dependent. We just cannot up and leave him with anybody. People are intimidated by a ventilator. Even skilled nurses and even physicians just have not had that type of experience. We obviously have.

I am not a trained medical professional, but my wife fortunately is a registered nurse and so that is another blessing that our family has had because she can train even an engineer to take care of my son.

But it is very difficult to, even if you can pay for respite care, it is very difficult to find qualified individuals to do that.

My sister flies down from Massachusetts once or twice a year to give us a break. She does not have to do that because—she does that because she loves Eric and realizes how important it is that Marcia and I have a few days away now and then.

I can tell you, and my wife would say the same thing, that when we come from a long weekend or, you know, 3 or 4 days just out of town doing nothing, our ability to take care of Eric is—it increases 10-fold.

Sometimes you do not realize how raw and fatigued and just emotionally drained you are until you go away for a few days and come back. Suddenly you have got tolerance and patience again. Eric understands that too.

In fact, I remember over a year ago, he got down on his computer, you know, dad, are you and mom getting enough time away. This is coming from a kid that requires our help every day. We were touched by that and we tried to understand that as much as we want to be with him every day, we do need to get away now and then in order to be good caregivers.

Senator MURRAY. Thank you very much for that.

Mr. Chairman, thank you.

Senator SHELBY. Thank you. We thank the panel very much for your appearance today and I think we are getting a lot out of what you had to say and your experiences. Thank you very much.

Our third panel will consist of Mr. Tommy John, everybody knows him, former major league baseball player; Mr. David Cone, former major league baseball player; Kate Linder, actress.

If you will proceed to the table, I am going to talk about all of you for just a minute.

First, Mr. Tommy John is a former major league baseball player. During his 26-season career, he played for the New York Yankees and the Los Angeles Dodgers. In 1976, he earned the Come-back Player of the Year Award. His career continued with three trips to the World Series.

Currently he is a minor league coach in the Yankee organization and an ALS advocate. Mr. John along with his wife and son are the 2005 recipients of the ALS Association's All Star Award.

We appreciate, Mr. John, you being here today.

Your written testimony, if any, will be made part of the record.

Our second panelist is Mr. David Cone. Mr. Cone is also well-known in America, all over the world. He is a former major league baseball player who played for 16 seasons with the Kansas City Royals, the New York Mets, the Toronto Blue Jays, and the New York Yankees.

Over that period, he earned five World Series champion rings. He is an honorary board member of the Greater New York Chapter of the ALS Association and was recognized as the 2004 ALS Association All Star Award recipient.

We appreciate you being here.

We also have with us today Ms. Kate Linder. Ms. Linder is a star of the daytime television drama "The Young and the Restless." Ms. Linder is helping to lead the fight against ALS through Kate's Club, a new public awareness campaign for the ALS Association. In December 2004, Kate's 49-year-old brother-in-law was diagnosed with ALS.

We appreciate all of your testimony here today. Our problem is going to be in a few minutes. We just got notice that we have a vote on the floor of the U.S. Senate.

So I'm going to start with you, Mr. John. You sum up whatever you want to say as quickly as you can here today. Thank you.

STATEMENT OF TOMMY JOHN

Mr. JOHN. Thank you, Mr. Chairman.

Members of the subcommittee, my name is Tommy John. I appreciate this opportunity to appear today alongside another great Yankee, David Cone, to talk about a disease I care about so deeply, a disease which was named after a former Yankee, Lou Gehrig.

The reason I mention my affiliation with the Yankees is because of my career with the Yankees is the reason I am involved in this fight. It is because I was aware of Lou Gehrig's disease during my playing days, but I did not know what the disease was or what it would do to one of my teammates, Jim Catfish Hunter. That is how I got involved in this fight. It is a personal fight with me.

Many of you remember Catfish as a great pitcher, Hall of Fame pitcher, who like David and one of our colleagues, Jim Bunning, threw a perfect game back in 1968. You may remember him as a larger-than-life figure with his North Carolina drawl and his big handlebar mustache that Charlie Finley gave him the nickname Catfish.

Catfish was a great teammate and a great person. ALS took his life in 1999 at the age of 53. As a friend of Catfish's back in North Carolina, I saw firsthand what ALS can do to a body that was just a strapping young man, that would hunt every day, and would make him totally powerless. It is a horrific disease and we must find a cure for it.

My entire family including my son, Taylor, and my wife, Sally, who are here with me today, we are committed to finding a cure for this disease.

As you said, I am an honorary member of the New York Chapter and each year, I am proud to attend the annual New York Sports Banquet. We do a fund raiser in Raleigh, North Carolina, the Jim Catfish Hunter Chapter.

We are also co-chairing a little league baseball tournament to benefit ALS, the first ever. That is out in Los Angeles July 23 to July 30.

However, what I want to do is I want to focus the remainder of my remarks today not on what I have done or what my family has done to raise the awareness of ALS. What I want to discuss is why military veterans, people who seem to be so strong like Catfish, are diagnosed with ALS and dying from the disease at a greater rate than other Americans.

Although I am not a researcher, I am aware of at least three recent studies which have found that military veterans are at an increased risk of dying from ALS. Two of those studies focused on the 1991 Persian Gulf War.

One found that those who served in the gulf were nearly twice as likely to contract ALS as veterans who did not serve in the gulf.

The other study found that young gulf veterans, those under the age of 45 were more likely, twice as likely to develop ALS.

The third study which was conducted by researchers at Harvard found out that people with a history of any military service, Vietnam, Korea, World War II, are at a more than 50 percent greater risk to get ALS than people who never served in the military.

As a veteran myself having served in the Air Force from 1966 to 1973, I believe that we owe it to our Nation's veterans to find out why there seems to be an increased risk of ALS with military service.

We owe it to people like Daniel Borsen, a veteran diagnosed with ALS who is sitting behind me with his daughter, Erica. We owe it to people like Charles Diser, a veteran who is also here, to the other veterans here today and across the country who are fighting ALS.

In this effort, we should also remember that ALS can strike anyone at any time as we have seen here earlier today. Therefore, I urge the subcommittee while it is important to discover what may be causing the increased risk of ALS in the military, we still must focus on ALS research as a whole for any progress we realize in ALS research certainly will benefit the entire ALS community.

Catfish Hunter was a pitcher back in his era and like David and like myself, when we took the mound, we took the mound to go nine innings to do a complete game. Catfish could not finish this game. He has turned the ball over to David, myself, Curt Schilling. We ask the subcommittee to take part in this and help us out of the bullpen to get that game complete so that we can find a cure for ALS.

PREPARED STATEMENT

Thank you for providing me with this opportunity and I am pleased to answer any questions you may have.

[The statement follows:]

PREPARED STATEMENT OF TOMMY JOHN

Mr. Chairman, Members of the Subcommittee, my name is Tommy John. I appreciate the opportunity to appear before you today, alongside another former New York Yankee, to talk about a disease I care so deeply about—a disease which was named after a former Yankee—Lou Gehrig.

The reason I mention our affiliation with the New York Yankees is because it is through my career with the Yankees that I am involved in the fight to find a cure for ALS. While I was aware of Lou Gehrig's disease during my playing days, what I did not know was that the disease would take one of my teammates—Jim "Catfish" Hunter. That's why I'm involved in this fight. It is personal.

Many of you may remember Catfish Hunter as a great pitcher—a hall of fame pitcher who, like David and one of your colleagues, Jim Bunning, threw a perfect game back in 1968. You may remember him as a larger than life figure with his trademark mustache and Carolina accent. Jim was a great teammate. ALS took his life in 1999, at the age of 53.

As a friend of Jim's back in North Carolina, I saw first hand what ALS is and how the disease can whittle away at the human body and how it can take a once powerful man and make him powerless. It is a horrific disease and we must find a cure for it.

My entire family, including my son Taylor and my wife Sally, who are with me here today, is committed to finding a cure for the disease that took the life of Catfish Hunter and thousands of others. I am an honorary member of the Greater New York Chapter and each year I am proud to attend the annual New York Sports Banquet, which is the single largest fund raising event for The ALS Association. My family and I also work closely with The ALSA Chapter in North Carolina—the Catfish Hunter Chapter—to increase awareness of the disease and raise funds for ALS research. In fact, Taylor, who is a professional singer, performed at the Candlelight Vigil that was held on Monday evening at the Jefferson Memorial. He performed at last year's vigil as well.

However, what I want to focus the remainder of my remarks on today is not what I have done or what my family has done to raise awareness of ALS. What I want to discuss is why military veterans—people who seem to be so strong, like Catfish Hunter—are being diagnosed with ALS and dying from the disease at a greater rate than other Americans.

Although I am not a researcher, I am aware of at least three recent studies, which have found that military veterans are at an increased risk of dying from ALS. Two of those studies focused on the 1991 Persian Gulf War. One found that those who served in the Gulf were nearly twice as likely to contract ALS as veterans who did not serve in the Gulf. The other study found that young Gulf War veterans—those under age 45—were more than twice as likely to develop ALS. The third study, which was conducted by researchers at Harvard, found that people with a history of any military service—Vietnam, Korea, and World War II—are at a more than 50 percent greater risk of ALS than people who have never served in the military.

I believe that we owe it to our nation's veterans to find out why there seems to be an increased risk of ALS with military service. We owe it to people like Daniel Bourson, a veteran diagnosed with ALS who is sitting behind me with his daughter Erika. We owe it to people like Charles Dysart a veteran who also is. And to other veterans here today and across the country who are fighting ALS.

In this effort, we should also remember that ALS can strike anyone of us at anytime. Therefore, I urge the Subcommittee that while it is important to discover what may be causing an increased risk of ALS in the military, we still must focus on ALS research as a whole, for any progress we can realize in ALS research certainly will benefit the entire ALS community.

Thank you for providing me with the opportunity to appear before you today. I am pleased to answer any questions you may have.

Senator SHELBY. Mr. Cone, you are welcome here, both of you. I just think if we could put you on the mound at the same time in your prime, would that not be a spectacle? Go ahead.

Mr. CONE. That would be nice.

Senator SHELBY. Thank you for being on this mound today though.

STATEMENT OF DAVID CONE

Mr. CONE. Senator Shelby, Senator Harkin, thank you for helping make this happen today and doing your part.

I have had the privilege of working with and meeting a great many people in the ALS community through my association with the Greater New York Chapter. You know, I was also a Yankee spokesman. You know, there has always been a Yankee player connected with ALS back to Lou Gehrig and I certainly am humbled to be one of those players along with Tommy John.

I am proud to have helped raise awareness of the disease and funding for ALS research and patient services. But I have to say that each time I participate in one of the chapter's events, each time I meet a person with ALS or a family whose lives have been touched by ALS, I am truly humbled.

After all, I am just an ex-ball player who is willing to help out. The people out there on the front lines living with ALS helping to fight this disease are the true heros. Everyone you see sitting here, all the families behind us. Many of the people in this room with us today and who are meeting with their Members of Congress as we speak are heros and they are the reason why I am so honored to be here today.

Before I continue with my remarks, I want to mention another baseball player and his wife who have been tremendous supporters in the fight against ALS, Curt Schilling of the Boston Red Socks and his wife, Shanda, who I would like to introduce is here.

They have helped raise millions of dollars for ALS research and patient services throughout Curt's career from Philadelphia and Arizona to Boston. They have also raised awareness of the disease.

You probably saw the KALS written on Curt's shoe during the World Series last year. Every time the camera focused on Curt's shoe and the now infamous bloody sock, you saw the words strike out KLS or KALS.

As you know, ALS is a disease that can strike anyone whether a seemingly invincible Hall of Fame ball player like Lou Gehrig or your next-door neighbor. It does not discriminate.

Unfortunately, we do not know what causes ALS, how it can be effectively treated, how to prevent the disease, or how to cure it. But we have an opportunity to change that.

In addition to increased funding for ALS research, which I strongly urge the subcommittee to support, we need to arm ALS researchers with the information and data they need to identify and pursue promising avenues of research that may provide new insights into the disease and lead us closer to finding treatment and cure.

The Greater New York Chapter is working with the New York State legislature on an effort that would identify and collect information about people living with the disease in New York State. While this is an important step in providing researchers with valuable information about ALS, more needs to be done on a national basis.

As Dr. Bruijn mentioned earlier, a national ALS registry will allow us to learn more about ALS, not only in terms of how many people have ALS in the United States today, but also those people and their ages, the family history and other information that is so vital to gaining a better understanding of the disease.

By collecting this information, we may be able to learn what causes ALS and why people like Lou Gehrig, Catfish Hunter, or

Eric Obermann, the young man who testified before me, contracted the disease, why ALS chose to strike them.

Equally important, this information may help us to prevent ALS from occurring in the first place or to discover new and better ways to treat the disease and improve the quality of life of people with ALS.

Although I am not a scientist, it is apparent to me that a national ALS registry could yield important clues about ALS and bring some significant benefits to the people sitting behind me in this room and as well as people living with ALS back home in your States.

I hope that this committee will provide the funding that not only makes additional research possible but also empowers those who conduct this research with the information and data they need to find a treatment and cure for ALS.

PREPARED STATEMENT

Senator Shelby, Senator Harkin, obviously thank you again for inviting me to testify today and I welcome the opportunity to work with you and the subcommittee to fight to strike out ALS. Thank you.

[The statement follows:]

PREPARED STATEMENT OF DAVID CONE

Good morning, Chairman Specter, Senator Harkin, Senator Shelby, and members of the Subcommittee. My name is David Cone, and I am pleased to be here today.

I have had the privilege of working with and meeting a great many people in the ALS community through my association with the Greater New York Chapter of The ALS Association and as the New York Yankee spokesman for The ALS Association. I am proud to have helped raise awareness of the disease and funding for ALS research and patient services.

But I have to say that each time I participate in one of the Chapter's events. Each time I meet a person with ALS or a family whose lives have been touched by ALS, I truly am humbled. After all, I am just an ex-ballplayer who's willing to help out. The people out there on the front lines living with ALS and helping to fight this disease are heroes. Many of the people in this room with us today and who are meeting with their Members of Congress as we speak are heroes. And they are the reason why I am so honored to testify before you today.

Before I continue with my remarks, I wanted to mention another baseball player and his wife who have been tremendous supporters in the fight against ALS. Curt Schilling of the Boston Red Sox and his wife Shonda, who is joining us today, have helped raise millions of dollars for ALS research and patient services throughout Curt's career—from Philadelphia to Arizona to Boston. They've also raised awareness of the disease. You probably saw the K-ALS written on Curt's shoe during the World Series last year. Every time the camera focused on Curt's now famous bloody sock, the world also saw strikeout ALS.

As you know, ALS is a disease that can strike anyone—whether a seemingly invincible hall-of-fame ballplayer like Lou Gehrig, or your next door neighbor. It does not discriminate. Unfortunately, we do not know what causes ALS, how it can be effectively treated, how to prevent the disease or how to cure it. But we have an opportunity to change that.

In addition to increased funding for ALS research, which I strongly urge the Subcommittee to support, we need to arm ALS researchers with the information and data they need to identify and pursue promising avenues of research that may provide new insights into the disease and lead us closer to finding a treatment and cure.

The Greater New York Chapter is working with the New York State legislature on an effort that would identify and collect information about people living with the disease in New York State. While this is an important step in providing researchers with valuable information about ALS, more needs to be done on a national basis.

As Dr. Bruijn mentioned earlier, a national ALS registry will allow us to learn more about ALS, not only in terms of how many people have ALS in the United States today, but also who those people are, their ages, family history, and other information that is so vital to gaining a better understanding of the disease. By collecting this information we may be able to learn what causes ALS and why people like Lou Gehrig, Catfish Hunter or Eric Obermann—the young man who testified before me—contracted the disease. Why ALS chose to strike them.

Equally important, this information may help us to prevent ALS from occurring in the first place or to discover new and better ways to treat the disease and improve the quality of life for people with ALS. Although I am not a scientist, it is apparent to me that a national ALS registry could yield important clues about ALS and bring significant benefits to the people sitting behind me in this room as well as the people living with ALS back home in your states.

I hope that this committee will provide the funding that not only makes additional research possible, but also empowers those who conduct this research with the information and data they need to find a treatment and cure for ALS.

Mr. Chairman, thank you again for inviting me to testify today. I welcome the opportunity to work with you and the Subcommittee in the fight to strikeout ALS.

STATEMENT OF KATE LINDER

Ms. LINDER. Good morning, Mr. Chairman, members of the subcommittee. Thank you for inviting me to participate in this important hearing today. I am truly honored to be here.

As you know, my name is Kate Linder. I am an actress. I play the role of Esther Valentine on the number one daytime drama "The Young and the Restless" for the past 23 years.

But I am also an advocate for people with ALS, their families, and caregivers. I am pleased to be here today to share with you my personal relationship with this terrible disease.

I also hope that through this hearing and in our meetings with your colleagues in both the Senate and House we will be able to raise awareness of the disease and support for our cause.

Five months ago, on December 8, 2004, my 49-year-old brother-in-law was diagnosed with ALS in Seattle, Washington. Now, at the time, I only had a passing knowledge of ALS. That is to say I had heard of ALS or Lou Gehrig's disease. But like many Americans, I did not know the nature of the disease or how it would change the lives of our entire family.

I did not know that ALS robs a person of the ability to move their arms and legs, to speak, and to breathe, nor was I aware that there was no cure for the disease. That is why I am here today, to raise awareness and to help advance the effort to find a treatment and cure for ALS.

Scott and his wife have two daughters and his diagnosis has devastated our entire family. But we will continue to fight ALS. It is still really hard for me to believe that even now, 5 months later, how someone so young and so full of life could be stricken with such a terrible disease.

Since Scott's diagnosis, I have made it my mission to do everything I can to support Scott, his wife, Georgianne, his daughters, Kristin and Sandra, in their fight against ALS.

Working with the ALS Association, ALSA, we have created a new public awareness campaign called Kate's Club which will focus much-needed attention on the disease. Through the ALS Association and Kate's Club, I am networking with fellow actors, fans, and volunteers to raise awareness and encourage them to join us in this important fight.

I have also filmed a public service announcement to promote one of ALSA's signature events, the Walk to Defeat ALS. This year, ALSA chapters are staging more than 147 walks in cities across the country to raise funding to support ALS research and patient services.

Last year, these walks collectively raised more than $9 million and more than 90,000 people participated in the events.

I hope that our work with Kate's Club will help raise even more funding to support programs and research that are so essential to finding a treatment and cure for ALS and to improving the quality of life for people with ALS and their families and caregivers.

I also wanted to praise the efforts of a fellow actor, Allen Rosenberg, who is also with us today for this hearing. Allen who portrayed a person with ALS on the hit show, "The Guardian," also has helped raise awareness of ALS and funding for our fight, having filmed a recent public service announcement and participated in numerous ALSA events and events organized by the Greater Los Angeles Chapter of the ALS Association.

But as actors and actresses, people in my profession are used to playing many roles as part of our every-day life. But what I would also like to focus attention on here today is the important role that family caregivers play in the lives of people with ALS.

Specifically I would like to highlight the need for respite care. As people with ALS lose the ability to walk, move their arms, talk, and even breathe, the disease requires them to rely on caregivers, usually their families, to provide the care and assistance that is needed to perform normal activities of daily living.

In many cases, particularly in the latter stages of the disease, people with ALS have a need for continuous care 24 hours a day, 7 days a week. The burden placed on caregivers and family members is tremendous.

Therefore, these families and caregivers have a significant need for respite care services. Respite care provides temporary relief to caregivers of individuals with chronic illnesses and disabilities and is a key component of quality long-term care.

Respite care may take place in the home or in a facility and allows caregivers much needed time off while providing quality care for the loved one. Respite care helps keep families together, helps prevent abuse and neglect, and forestalls premature costly institutionalization and possible impoverishment. Virtually every family who is touched by ALS has a need for respite care services.

Legislation such as "Life Span Respite Care Act," which passed the Senate in 2003, is needed to help expand the availability of respite care services for people with ALS and their families. The bill is expected to be introduced again this year by Senators Clinton and Warner and I hope Congress will pass this much-needed legislation.

When it does pass, I also hope that this subcommittee will support funding for the programs included in the bill. They would be a tremendous benefit to people with ALS and their families across the country. They would benefit Scott and his family as they continue their fight against ALS.

PREPARED STATEMENT

I just want to personally thank you so much for inviting me to participate in this hearing. Thank you.

[The statement follows:]

PREPARED STATEMENT OF KATE LINDER

Good morning. Mr. Chairman, Members of the Subcommittee, thank you for inviting me to participate in this important hearing today. I am honored to be here.

My name is Kate Linder and I am an actress, having played the role of Esther Valentine on the daytime television drama, The Young and the Restless, for the past 23 years. I also am an advocate for people with ALS, their families and caregivers and I am pleased to be here today to share with you my personal relationship with this terrible disease. I also hope that through this hearing, and in our meetings with your colleagues in both the Senate and House, we will be able to raise awareness of the disease and support for our cause.

Five months ago, on December 8, 2004, my 49 year old brother-in-law was diagnosed with ALS in Seattle, Washington. At the time, I only had a passing knowledge of ALS—that is to say, I had heard of ALS, or Lou Gehrig's disease. Like many, many Americans, I did not know the nature of the disease or how it would change the lives of our entire family. I did not know that ALS robs a person of the ability to move their arms and legs, to speak and to breathe. Nor was I aware that there was no cure for the disease. And that is why I am here today: To raise awareness and to help advance the effort to find a treatment and cure for ALS.

Scott and his wife have two daughters and his diagnosis has devastated our entire family. But we will continue to fight ALS. Yet, it still is hard for me to believe—even now, 5 months later—how someone so young and so full of life could be stricken with such a terrible disease.

Since Scott's diagnosis, I have made it my mission to do everything I can to support Scott in their fight against ALS. Working with The ALS Association (ALSA), we have created a new public awareness campaign called "Kate's Club[sm]," which will focus much needed attention on the disease. Through The ALS Association and Kate's Club I am networking with fellow actors, fans, and volunteers, to raise awareness and encourage them to join us in this important fight. I also have filmed a public service announcement to promote one of ALSA's signature events, the Walk to D'Feet ALS. This year, ALSA's Chapters are staging more than 147 walks in cities across the country to raise funding to support ALS research and patient services. Last year, these walks collectively raised more than $9 million and more than 90,000 people participated in the events. I hope that our work with Kate's Club will help raise even more funding to support programs and research that are so essential to finding a treatment and cure for ALS and to improving the quality of life for PALS and their families and caregivers.

I also wanted to briefly praise the efforts of a fellow actor, Alan Rosenberg, who also is with us today for this hearing. Alan, who portrayed a person with ALS on the hit show The Guardian, also has helped raise awareness of ALS and funding for our fight, having filmed a recent public service announcement and participated in numerous ALSA events, and events organized by the Greater Los Angeles Chapter of The ALS Association.

As actors and actresses, people in my profession are used to playing many roles as part of our everyday lives. But what I also would like to focus attention on here today is the important role that family caregivers play in the lives of people with ALS. Specifically I would like to highlight the need for respite care.

As people with ALS lose the ability to walk, move their arms, talk and even breathe, the disease requires them to rely on caregivers, usually their families, to provide the care and assistance that is needed to perform normal activities of daily living. In many cases, particularly in the later stages of the disease, people with ALS have a need for continuous care, 24 hours a day, seven days a week. The burden placed on caregivers and family members is tremendous. Therefore, these families and caregivers have a significant need for respite care services.

Respite care provides temporary relief to caregivers of individuals with chronic illnesses and disabilities and is a key component of quality long-term care. Respite care may take place in the home or in a facility and allows caregivers much needed time off, while providing quality care for the loved one. Respite care helps keep families together, helps prevent abuse and neglect, and forestalls premature, costly institutionalization and possible impoverishment. Virtually every family who is touched by ALS has a need for respite care services.

Legislation, such as the Lifespan Respite Care Act, which passed the Senate in 2003, is needed to help expand the availability of respite care services for people with ALS and their families. The bill is expected to be introduced again this year by Senators Clinton and Warner and I hope Congress will pass this much needed legislation. When it does pass, I also hope that this Subcommittee will support funding for the programs included in the bill. They would be a tremendous benefit to people with ALS and their families across the country. And they would benefit Scott and his family as they continue their fight against ALS.

Thank you again for inviting me to participate in this hearing.

Senator SHELBY. Thank you.

I want to thank the panel.

I want to recognize again Mrs. Schilling. Thank you for coming. We all appreciate the courage and the competitiveness of your husband and the fact that he is involved in this fight, too, ALS, and you too.

Mr. John, you know firsthand the benefit of biomedical advances. I will tell Dr. Andrews, who is a constituent of mine, that you were here today. He would rebuild your arm if you were probably a couple years younger, you know.

Mr. JOHN. I need somebody to—a body would due right now.

Senator SHELBY. A body.

Mr. JOHN. The arm is fine. The body is gone.

Senator SHELBY. Mr. Cone, we appreciate your involvement here and the background of both of you.

Ms. Linder, you are very articulate.

So we are going to do everything we can to properly fund this research to try to find a cure to a dreaded disease.

We have got a vote on the floor. They are holding the vote for us. Senator Harkin and I are going to have to leave.

Senator, you may have the last word.

Senator HARKIN. I just want to thank you, Mr. Chairman. I want to thank you, Mr. John, Mr. Cone, Ms. Linder. Obviously a lot of people look up to you. You are heros or heroines to a lot of people in this country, rightfully so, and your leadership in this really has a great impact.

So I just want to thank you for lending your names and your status to this effort. It means a lot. It really does. It motivates a lot of people. They look up to you and they respect you. When they hear that you are involved in this fight, that gives them courage too. So thank you for doing this.

Finally, I ask unanimous consent to include a written statement from Adrienne Hallett in the hearing record.

[The statement follows:]

STATEMENT FOR THE RECORD FROM ADRIENNE HALLETT

There's no question about when my father, Albert Arthur Hallett, lost his battle with ALS. He died early in the morning on August 5, 2004, at the age of 67. What's harder to determine is when his battle began.

Did he carry a nefarious gene when he was born in 1936, the first son of a farmer and a teacher in a small town in northeast Iowa? Did it have something to do with the chemicals and pesticides he was exposed to as a teenager on a struggling family farm?

Did ALS creep in when he enlisted in the Army, filled with all of the cocky pride of an 18-year-old about to see the world? He trained at Fort Hood, Texas, and spent four years in Korea. Was there something in the Army rations—those industrial meals he reminded me of every time I as a child "forgot how good I had it" with my mother's cooking?

Was ALS lurking when he got back from the Army and courted Shirley Ann Schnieders over dinner at the Cozy Inn? My father liked a challenge, and this strong, spunky young woman was a challenge worth winning. She used to say that by making her a Hallett, my father took the "SASS" out of her. He would wink at me and say, "not by half." Her feistiness would come in handy when his body started failing, but no one had any answers, and when someone needed to stand up to the HMO, and he didn't have the strength to do it himself.

Did he already have ALS as he raised two children? Three times in his life, my father made a beeline from a doctor's office to a bank. In 1971, the doctor informed him he was going to have a son. He went to the bank to start a college fund. Three years later, a little girl was on the way, and college was on his mind again. The third trip to the bank was the day of the ALS diagnosis. It was no surprise to anyone that Al Hallett's first thought was to make sure his family would be taken care of.

Did ALS take root in the manufacturing plants where my father walked the floors before returning to the office where he worked as an industrial purchaser? Or was the toll taken in the back-breaking years of laying carpet in people's houses on Saturdays—a side business he ran to make ends meet?

One thing I know now, ALS was there when he retired in 1998. An Internet search at the time convinced him that the choking feeling in his throat was the result of a hiatal hernia. He would regularly complain that his left thumb tingled, as he spent countless hours volunteering with the Optimist Club, SCORE, the Boy Scouts, and any other organization willing to put up with his corny phrases in return for his service. As I think back on his symptoms, my father's voice is in my head: "Adrienne, hindsight is always 20/20."

But in 1998, no one thought twice about those symptoms. How could they? This was the tough man whose right index finger was permanently bent at the first joint because of a disagreement with a corn husker. I used to joke that my father was clearly enjoying retirement because he spent his Saturday morning at the diner with all of the other retired guys, complaining about their various aches and pains. Meanwhile, he had eight years to wait until his wife could retire. He would delight in saying, "That's what I get for robbing the cradle." He planned the trips they would take and the property they might buy.

Soon, however, he would tell us that his hand would periodically go numb. He visited his primary care doctor, an otolaryngologist for his throat, a neurologist for his hand and finally a neurosurgeon. We never thought to tell the neurologist about the otolaryngologist, or vice versa. No one ever asked. We knew nothing about ALS and no health professional even mentioned the possibility. An MRI turned up a small bone spur in his fourth vertebrae. There was no accompanying loss of strength or range of motion, and so my mother was adamantly opposed to a surgery that would cut open her husband's spine.

Yet, five years after retiring, his left arm had further weakened and his feet would sometimes drag. Yes, Dad, hindsight is always 20/20. He convinced my mother that he needed to get the surgery before she retired and their health insurance changed. So in October 2003, a surgeon removed a section of his fourth vertebrae and replaced it with a bone graft. A metal plate held it all in place. We were told it was a common procedure—no reason to gather the family. With a son in Seattle, working for a genetics company, and a daughter in Washington, working for this subcommittee, Al Hallett underwent a procedure that would change our world forever.

When my father woke up from surgery, his left hand felt no better. More alarming, he couldn't move his right arm at all. While this wasn't typical, the doctors assured us that there was nothing to worry about. The nerve was simply bruised from the procedure, they said; with physical therapy, motion would return in four to six weeks.

He tried to comply, but his growing fatigue made physical therapy all but impossible. His feet began losing function and he began having falls. My mother hammered at the neurosurgeon in follow-up visits, convinced that something dreadful had happened in the surgery. Whatever it was, it was getting worse.

A month later, just before Thanksgiving, the neurosurgeon finally agreed and sent him to a neurologist. Brain scans, MRIs, and spinal taps ensued. Tests ruled out everything. Weeks went by with doctor appointments galore and no answers. When my father called me after an MRI, he sounded ecstatic. In keeping with the Optimist oath he took every Saturday at the diner, my father cheerfully announced that he had a vitamin deficiency. He passed the phone to my mother, who left the room and told me tearfully that he had multiple sclerosis. Further conversation revealed that the doctor had told them it could be anything from a vitamin deficiency to MS. The optimist and the pessimist—opposites will forever attract.

The final diagnosis came in March 2004, five months after the surgery. A neurologist who had defensively brushed off the questions posed by my mother, my brother and me for months delivered the devastating news to my mother in the middle of a crowded waiting room: "I'm sorry but there is no treatment. I suppose you will want a second opinion but I assure you this is it. There is a drug out there but I think it is a waste of money for him." Words can't describe that moment. My strong, spunky mother crumpled in front of me and I couldn't seem to find oxygen in the room. Anger: who was this doctor, this indifferent man sitting there throwing this news at us like an executioner in front of 50 total strangers? Fear: we cannot do this, it is too much, it is too hard and we will lose in the end. Disbelief: a terminal diagnosis based on ruling everything else out? How do they know? They can't know for sure, there wasn't a test, it could be something else. It must be something else, anything else. Shock: please, God, this isn't real. Please let it not be true. I want my father back.

The University of Iowa, located just 45 minutes away from our home in Cedar Rapids, had an expert in ALS and a comprehensive Center on Disability. Unfortunately, the HMO agreed to cover a visit there for a second opinion only; as far as they were concerned, there were plenty of neurologists in town. But none of those doctors offered the comprehensive approach of the UI center, and my parents couldn't pass up its services, even if it meant draining their retirement savings.

In one afternoon at the center, my father saw a neurologist who immediately confirmed the diagnosis, a nutritionist, an assistive technology specialist and a mental health coordinator who checked both my father and mother for signs of depression. A follow-up appointment was scheduled for three months later, the normal timeframe for ALS patients.

But the ALS took over so quickly, and I learned that progressive diseases don't fit the HMO model. My father's HMO wouldn't approve the purchase of any equipment until after he had lost the skill or function it served. Once that happened, we had to go to the doctor for a prescription, then submit it to the insurance company, then purchase the equipment—a process that could take weeks. In the meantime, he suffered and went without.

The two bright stars in this very dark night were the University Center on Disability and the local Muscular Dystrophy Association. The Center gave my father respect and choices. There was so much he couldn't control, but they told him of the limited options he had and let him decide. The MDA group loaned us equipment when we asked for it, so it was there when he would finally swallow his pride enough to use it. Leg braces were replaced by a walker, which led to an electric scooter. It took multiple trips to the emergency room with injuries from falling down before my dad submitted to using the scooter. Persuading him to use a wheelchair would take longer, but as is the case with ALS, it was inescapable.

My father, who had built our deck and repaired almost everything in our house, had to watch others build an accessible entrance, raise the chairs so it was easier to get in and out, and widen the doorways for a wheelchair. The only part of it the former purchaser liked was picking out the wheelchair lift and haggling with the salesman over gears and resistance and loads. None of it was eligible for insurance.

The HMO insisted that my father go to an approved physical therapist. This therapist ran him through a series of activities such as getting up from a lying position, standing, sitting, and negotiating stairs. My father was helped to the floor and directed to stand up. He tried in vain. When finally he was humbled enough to admit defeat, the therapist told my father to drag himself across the floor to a chair to pull himself up. With his wife watching, he labored to make his arms—the source of his initial symptoms—pull his body weight across the floor. The report to the insurance company indicated that my father was able to take care of himself.

My father went home that night exhausted and utterly humiliated. The therapist seemed totally unaware that fatigue is dangerous for ALS patients. Whenever my father tired himself out, it would take days to get merely a semblance of his energy and function back. We lost a little piece of him each time he became fatigued.

Meanwhile, the University nutritionist helped my mother as she tried in vain to keep weight on my father. She tried to make things as normal as possible, even putting a bratwurst in the blender on the Fourth of July.

In late June, the doctor appointment showed that he was losing lung capacity and his weight loss was unsustainable. He wasn't quite to the designated marker for the insertion of a feeding or breathing tube, but the next appointment wouldn't be for three months. Out of fear and frustration, my mother arranged for a consultation with the ALS center scheduled for the second week of August. Hospice workers checked on him, but said he wasn't sick enough for their services.

A week later, on August 4, my father informed his nurse that he wanted to go to the SCORE volunteer awards. He had had to quit the group months before, but

he wanted to cheer on his friends. He even insisted that my mother take him to the barber for a haircut—an Army man to the end.

After the haircut, he went home and went to sleep. My mother woke him twice for meals. He quietly died in the middle of the night. A week after being too well for hospice, a week before his chance for a breathing tube, my father went to sleep and never woke up.

A day later, the postman brought another denial from the HMO: The condition was not serious enough to warrant the June doctor appointment.

Did ALS come in from chemicals, from the environment, from the Army, from a gene? Or was it simply random? Can a good man, who does everything our society tells him is the right and responsible way to live, be struck down by the toss of the dice? I don't know the answer to that. But a man who told me repeatedly "Can't never did nothin', Adrienne. I don't want to hear that word from you" was eventually faced with a room full of doctors who had nothing for him but the word he so despised. No treatment, no options—we can't help you. He deserved more.

My father never complained and never let the rest of us complain. At his funeral, one of his buddies told me with tears in his eyes that my father had spoken to them just once about ALS. As he was walking out of the diner for the last time, just after he had come back from the emergency room following a fall, my father looked back, sadly shook his head and said, "What did I ever do that was so wrong?"

He did nothing wrong. He was a good man. He helped his parents, he served his country, he saved for his kids' education and his retirement, and he volunteered in his community. Like everyone here today, I have a thousand more questions than answers. I leave conclusions to be drawn by this Subcommittee and by the test of history. I will say though, that for all of the years my father talked about it, ALS taught me once and for all to hate the word can't. We can find a cure for ALS. We just have to try.

CONCLUSION OF HEARING

Senator SHELBY. Thank all of you. That concludes our hearing.

[Whereupon, at 11:50 a.m., Wednesday, May 11, the hearing was concluded, and the subcommittee was recessed, to reconvene subject to the call of the Chair.]

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