

TREAT ALS NEALS Clinical Trials Network

by Breen Power, Merit Cudkowicz, M.D.,
Alex Sherman, and Jeremy Shefner, M.D., Ph.D.



Breen Power Merit Cudkowicz Alex Sherman Jeremy Shefner

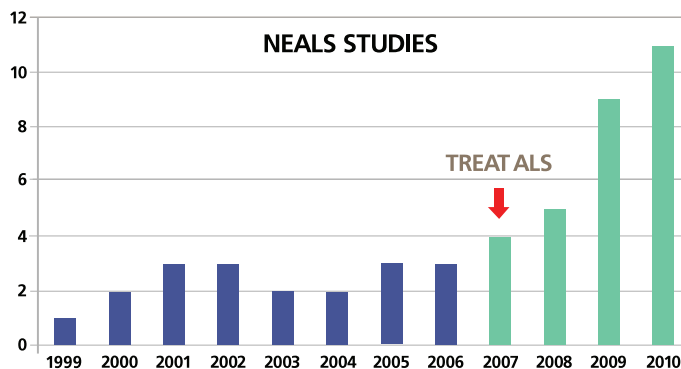


Chart shows the rise in the number of clinical trials since the TREAT ALS network was established.

For the last two years, The ALS Association has funded the **Translational Research Advancing Therapy for Amyotrophic Lateral Sclerosis (TREAT ALS)** Northeast ALS Clinical Trials Network to develop the infrastructure necessary to rapidly complete clinical trials of new therapeutic agents, and to provide partial funding of individual trials. The Northeast ALS Clinical Trials Consortium (NEALS) is comprised of 92 clinical sites throughout the U.S. and Canada. Founded in 1996, NEALS has developed rigorous standards for clinical trial performance, standards for training and validation of individual sites, and much of the infrastructure to perform trials.

The TREAT ALS NEALS Network Platform, a sophisticated clinical trials Web-based system, was customized and re-designed to accommodate the network's goals. The platform helps to start new trials more quickly. An integrated project management system reduces much of the burden of running a clinical trial. This system makes tracking important trial documents easier. Another component of the system facilitates the collection and tracking of fluid and tissue samples collected as part of all trials. Additionally, an ALS biorepository is being developed, which will aid in the search for biomarkers. This repository is intended to be a resource for the entire ALS community.

Currently, the TREAT ALS NEALS Network is actively involved in 12 trials that are either ongoing or in final

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First ALS Patient Receives ISIS-SOD1_{RX} Antisense Treatment

In March, Isis Pharmaceuticals, Inc., initiated a Phase 1 study of ISIS-SOD1_{RX} in patients with an inherited, aggressive form of Lou Gehrig's Disease. ISIS-SOD1_{RX} is an antisense drug designed to inhibit the production of SOD1. Antisense techniques are used to deactivate disease-causing or undesirable genes so they cannot produce harmful or unwanted proteins. The ALS Association provided funding for the development of ISIS-SOD1_{RX}.

"This therapy will be the first ALS treatment that is specifically aimed at the target, SOD1, known to cause ALS. The development of new treatments for ALS is an extremely challenging and costly process. The ALS Association has been pleased to co-fund this study and partner with Isis on this extremely promising treatment approach. It is only through the support of our generous donors that this type of advancement is made possible," commented Lucie Bruijn, Ph.D., Chief Scientist, The ALS Association.

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**VISION
GOES
GREEN!**

For details, turn to Jane Gilbert's message, "With Change Hope Remains," on page 2.

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2.

With Change Hope Remains A Message from Jane H. Gilbert, President and CEO

On January 7, 1985, The ALS Association was chartered when the California-based ALS Society of America merged with the New York-based National ALS Foundation to form our current organization. Our founders shared the vision that continues to guide our efforts today, which is to create a world without ALS.

Today, more than ever, we share the hope with the people and families living with Lou Gehrig's Disease that one day very soon we will announce significant breakthroughs in ALS research, and there are many new technologies, research categories and clinical trials that provide such reason for hope. This knowledge and your continued support drive our daily activities.

While our dedication to leading the fight to find a cure and effective treatments remains steadfast, some things at The Association are changing. For instance, we have relocated our national headquarters to Washington, D.C., to be closer to many of the government agencies that make decisions about the direction of many activities important to the ALS community. These agencies include the National Institutes of Health, the Department of Defense, Department of Veteran Affairs, and Congress.

In addition, we are looking to make our operations more efficient and yet continue to be the primary source of information about Lou Gehrig's Disease to the ALS community. One way to accomplish this is by moving our *Vision* publication to electronic distribution. I encourage each one of you to provide us with your e-mail address so you can continue to receive this publication and help us avoid the costs of printing and mailing. This will allow us to dedicate more dollars toward research and patient and family services.

To sign up for the electronic distribution, go to <http://web.alsa.org/Vision>. It's that simple.

And, if you prefer to read *Vision* in hard-copy form, you can easily print the publication on your home or office printer. We will also provide limited numbers of the publication for those of you who want to receive *Vision* through the mail.

With your support, The ALS Association will continue to fuel the engine of hope, driving us closer and closer to new therapies to treat ALS and ultimately a cure to end it. Together, we can make this a reality.



Jane H. Gilbert

Vision goes electronic. Send us your e-mail. Don't miss an issue!

A Song for Dad

by Katie Sweeney

Dr. Lewis Jassey is using his passion for music to honor his late father—and help find a cure for ALS.



Growing up, Lewis Jassey watched his father, Marvin Jassey, go out of his way to help others. A family doctor in Long Island, N.Y., his dad would run out in the middle of the night or even heavy snowstorms to tend to his patients. At home, the elder Jassey was just as devoted, encouraging Lewis and his other two sons to pursue their passions in life.

So when his father passed away in 1996 after a five-year battle with ALS, Lewis Jassey was determined to keep his dad's spirit alive. And late one night in 2004, he found a way to do just that.

While his family slept, Jassey began to pour out his memories and feelings about his dad into a song, "Hey Dad." A pediatrician by day, Jassey is equally passionate about music and works as hip hop artist "Doc J." Backed by his musical crew, The Raw Poets, he writes and performs rap lyrics with positive and profound themes.

"I'm passionate about music, and I loved my dad so much," he explains. "I couldn't think of a better way to honor him."

Today, "Hey Dad" is serving not only as a personal tribute, but also as a vehicle for helping others. Jassey recently teamed up with the Greater New York Chapter of The ALS Association to use the song as a way to raise money and awareness for ALS research.

He's donating 100 percent of the proceeds of the song's online sales to the Chapter to fund ALS research. He and The Raw Poets also performed at the Chapter's Walk to Defeat ALS last fall and have been invited to perform at the Keith Worthington Chapter's walk in Kansas City, Mo., in September.

In addition, he's talking with professional sports organizations and high-profile athletes to coordinate and organize more fund-raising efforts for ALS research. His story has attracted the attention of local media and even NBC News.

"The work that The ALS Association does is so important," Jassey adds. "They genuinely care about patients and families, and they're fighting every day to find a cure for this disease."

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TREAT ALS NEALS

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planning stages. Among the most exciting is the first FDA-approved stem cell trial for ALS in the U.S. This trial, supported by Neuralstem, is a Phase 1 study whose purpose is to determine the safety and tolerability of surgical implantation of embryonic stem cells within the spinal cord of ALS patients. This trial is being performed at Emory University in Atlanta under the supervision of Drs. Nick Boulis and Jonathan Glass, employing the TREAT ALS NEALS infrastructure.

Another trial that is evaluating a novel treatment is the trial of antisense oligonucleotides in ALS patients who carry the SOD1 gene. The study is supported by Isis, The ALS Association, and the Muscular Dystrophy Association. This trial is also a Phase 1 study and is based on very exciting preclinical data showing that small molecules designed to interfere with the production of mutant SOD1 can in fact reduce SOD1 protein levels and exert a positive effect on the SOD1 transgenic mouse. The human

study is directed by Drs. Tim Miller of Washington University in Saint Louis, and Merit Cudkowicz of Massachusetts General Hospital in Boston. The initial study will test the safety of a single infusion of anti-sense into the intrathecal space (spinal canal), with increasing doses planned as safety is shown. This trial also takes full advantage of the TREAT ALS NEALS clinical trials infrastructure.

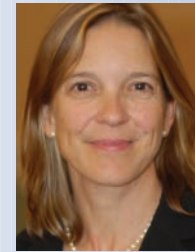
Trials funded with help from the network, including studies of biomarkers and nutrition as well as Phase 1-3 studies of experimental agents. The NEALS/ALS Association collaboration has almost tripled the number of ongoing trials in less than three years. We fervently hope that, in addition to facilitating more clinical trials, we are coming closer to demonstrating a clear benefit in subjects that will translate into improved care for people with ALS.

Please visit www.nealsconsortium.org for more information on NEALS and the TREAT ALS NEALS Clinical Trials Network.

This article was written as a collaboration among NEALS Co-Chairs Merit Cudkowicz, M.D., M.S.C. and Jeremy Schefner, M.D., Ph.D., and Breen A. Power, program manager; and Alex Sherman, director of systems (NEALS).

A Promising Time for ALS Research

The ALS Association's TREAT ALS (Translation Research Advancing Therapies for ALS) pipeline is a



Lucie Bruijn

comprehensive and strategic research program ensuring that important laboratory findings receive the financial and

scientific support to take discoveries from the bench to the bedside.

This year proves to be an extremely promising year for ALS research and the advances in the field would not be possible without your generous contributions. The TREAT ALS NEALS Clinical Trials Network, highlighted in this publication has facilitated an increased number of clinical trials, including two very novel approaches to treating ALS: a superoxide dismutase (SOD1) antisense approach that lowers the unwanted mutant protein in some familial forms of ALS and spinal cord stem cell transplants to test for safety of the procedure and ultimately to help protect surviving motor neurons.

With the discovery of new genes for ALS, scientists are currently developing new model systems for ALS, which will be invaluable to better understand the disease and develop new treatments. These new discoveries have brought new scientists into ALS research. Furthermore, advances in technologies, such as

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Song for Dad

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ALS has touched his life in more ways than one. His grandmother and two aunts also passed away from the disease. He knows that there may be a genetic connection in his family.

"It's scary," says Jassey, who is married with two daughters, Julia, 9, and Mya, 4. "There's a whole new generation of people who could be affected, not just my family, but other families, too. That really pushes me to get the word out."

He says his efforts are just getting started, and like his dad, he doesn't give up easily.

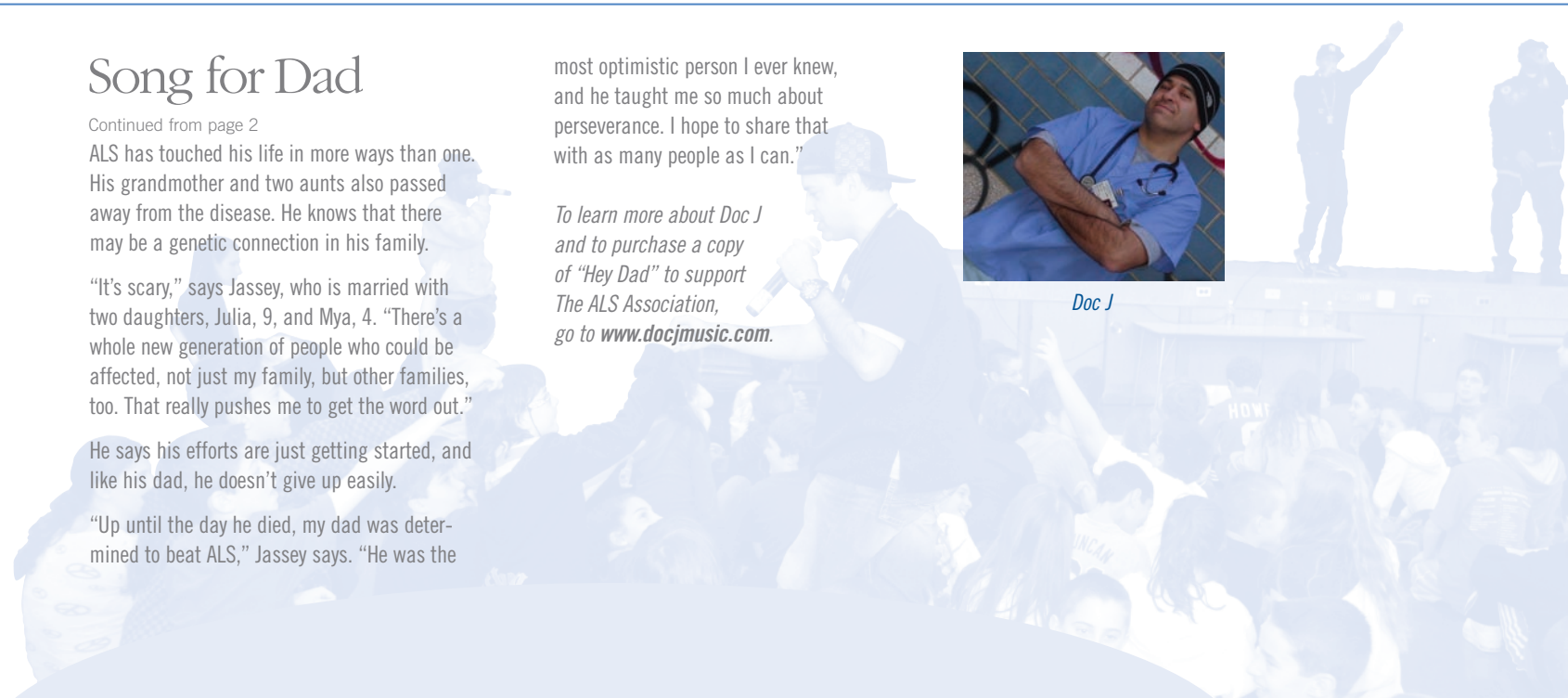
"Up until the day he died, my dad was determined to beat ALS," Jassey says. "He was the

most optimistic person I ever knew, and he taught me so much about perseverance. I hope to share that with as many people as I can."

To learn more about Doc J and to purchase a copy of "Hey Dad" to support The ALS Association, go to www.docjmusic.com.



Doc J



Enduring Love Advances the Fight Against ALS



Richard P. Essey didn't know what amyotrophic lateral sclerosis was until his wife, Sheila, was diagnosed with it the day before Thanksgiving in 1994. For the next 15-plus years, Essey would become one of the most active and influential forces in the fight to rid the world of Lou Gehrig's Disease, which claimed Sheila's life in 2004.

Essey retires this spring from The ALS Association Board of Trustees, but his legacy – from the founding of The Greater Bay Area Chapter to the establishment of the premier worldwide award in ALS research, named after his wife – will endure for years to come.

“There are few who have given so selflessly over a protracted period of time as Dick Essey,” says Allen L. Finkelstein, Esq., one of Essey's board colleagues.

4. Adds Hon. Jay Daugherty, The ALS Association National Board of Trustees Chair: “Dick always stepped forward when something was needed – whether it was money, volunteering for a project, or coming forward with a creative idea. He combines business sense with a quiet passion, fueled by his love for his wife.”

Essey first made his mark in business in 1962 when he founded TemPositions, a company specializing in supplying high-quality personnel to provide the flexible work force needed by rapidly expanding businesses. At his side was the woman he had met at a charity cocktail party nine years earlier before proposing on the couple's third date.

Essey would describe Sheila as “a wonderful wife, mother, confidant, and philanthropist who celebrated each day with courage, tenacity, and grace and never lost the twinkle in her eye.” Their marriage would last more than 50 years, during which they raised two children and had three grandchildren.

After all of their success, the Esseys wanted to give back. In 1990 they established the Essey Family

Fund, which over the next two decades would provide charitable contributions of more than \$14 million to important causes. A few years later, one cause would take on special meaning.

Essey might not have known what ALS was before his wife was diagnosed with the disease, but once he learned he was determined to do something about it. Within six months he had made the first of many gifts to The ALS Association and had joined the Board of Trustees. He established The Greater Bay Area Chapter in San Francisco, where the Esseys resided.

Having learned how little the medical community knew about ALS, Essey and his family next established the Sheila Essey Award for ALS Research, given by The ALS Association in partnership with the American Academy of Neurology (AAN). The award, presented each year since 1996 during the AAN's annual meeting, recognizes individuals, chosen through a selection process by their peers, who have made significant contributions in the search for the cause, prevention of, and cure for ALS. A \$25,000 prize is awarded to further the recipient's ALS research, and the prestige associated with the award has provided a boost to many promising careers. This year's award was presented to Clive Svendsen, Ph.D., director of the Cedars-Sinai Regenerative Medicine Institute.

Even after his wife's death, Essey continued to devote countless volunteer hours and ideas to The ALS Association. “Dick Essey deserves much credit for his contribution to the success of The ALS Association's research program,” says Robert V. Abendroth, Esq., chair of the board's Research Committee. “He has continually proposed new ideas and repeatedly challenged and inspired the trustees of The ALS Association to fund research.”

As for Essey, he is most pleased to know that, although his wife is no longer with him, the fight for a cure endures in her name.



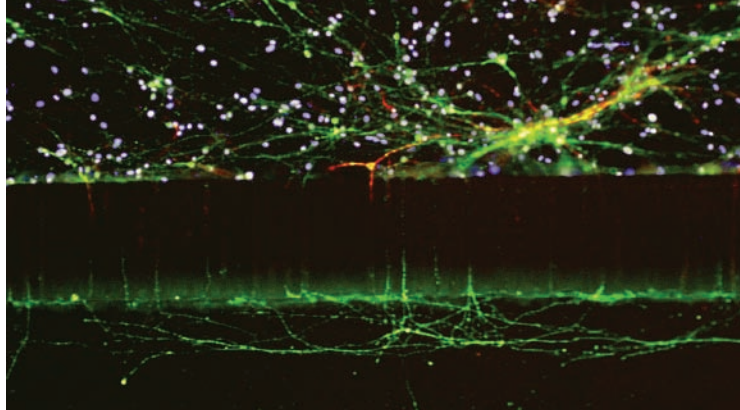
Sheila Essey



Richard P. Essey

Motor neurons (SMI32, red) derived from patient induced pluripotent stem cells put out axons (Tau, green) into chambers of a microfluidics device.

Clive Svendsen Wins Prestigious Essey Award



—Photo courtesy of Clive Svendsen, Ph.D., and Jered McGivern, Ph.D.

The ALS Association joins the American Academy of Neurology in presenting the 2010 Sheila Essey Award for ALS Research to Dr. Clive Svendsen, a global leader in stem cell research. Dr. Svendsen has been instrumental in bringing stem cell approaches to the clinic.

Currently the Director of the Cedars-Sinai Regenerative Medicine Institute in California, Dr. Svendsen received funding through The ALS Association to establish stem cell approaches for the treatment of ALS. He and his group have also harnessed the induced pluripotent stem cell technology, using adult skin cells and reprogramming these cells to be able to generate a variety of neuronal cell types including motor neurons. Using this technology he is developing model systems for diseases such as Spinal Muscular Atrophy, Huntington's disease and ALS.

"I'm very honored to have received this award and

would like to dedicate it to my friend Jeff Kaufman, who devoted his life to raising funds for ALS before finally falling to this horrific disease. We will use the award money to continue exploring novel stem cell and growth factor treatments," commented Dr. Svendsen.

Inaugurated in 1996, the Sheila Essey Award for ALS Research acknowledges and honors an individual who is actively engaged in ALS research and is making significant contributions in research for the cause, treatment, prevention or cure for ALS. The recipient receives a \$25,000 prize to be used specifically for continuing his/her ALS research. Funding of the award is made possible through The Essey Family Fund and The ALS Association. For a complete listing of previous recipients visit <http://www.alsa.org/research/essey.cfm?CFID>.



Dr. Clive Svendsen, Ph.D.

Research Council Named in Honor of Lawrence R. Barnett

In a tribute to Lawrence R. Barnett's tireless support of The ALS Association and its work to find a cure for ALS, the National Board of Trustees has renamed the Research Council in his honor.

The Lawrence R. Barnett Research Council is a group of supporters who contribute a minimum of \$1,000 a year to fund ALS research. Members receive research updates and exclusive invitations to interact with top researchers.

Barnett's support for ALS research began more than three decades ago, when the wife of a friend, Robert V. Abendroth, Esq., was diagnosed with ALS and passed away. Barnett, a former president of Music Corporation of America (MCA), eagerly joined the effort to find a cure.

The first chairman of The ALS Association in 1985, Barnett is often called the grandfather of The ALS Association and has been an

extraordinary and steadfast supporter with his wife, Isabel, who died in 2006. Even today, at age 96, he still serves on the Board of Trustees.

"I remember my father always explaining how much The ALS Association meetings meant," says his son, Lawrence R. Barnett, Esq., who also recently joined the Board of Trustees. "He's been determined to find a cure and help people suffering from this disease."

Abendroth, the Council's original founder and longtime chairman of The Association's Research Committee, says re-naming the Council is a fitting tribute for his friend.

"The common intersection in Larry's philanthropy has always been cutting-edge research," he notes. "We are extremely grateful for his support."

To learn more about The Lawrence R. Barnett Research Council, please contact Karen Starleaf at (888) 949-2577, ext. 211, or kstarleaf@alsa-national.org.

ALS Research

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more rapid and cost-effective gene sequencing and the generation of motor neurons and their surrounding cells from adult skin cells using induced pluripotent stem cell technology, will accelerate the development of new treatment approaches to slow the progression of ALS.

Thank you for your support and for making these advances possible. Together, we can make a difference in the fight against Lou Gehrig's Disease by finding therapies and ultimately a cure.

—Lucie Bruijn, Ph.D.
Chief Scientist
The ALS Association

Phi Delta Theta Launches “Iron Phi” to Fight Lou Gehrig’s Disease

For more than a decade, Phi Delta Theta Fraternity’s undergraduate chapters and alumni clubs have raised money, awareness, and support for the fight against Lou Gehrig’s Disease. Recently, Phi Delta Theta launched an initiative called Iron Phi. The concept of Iron Phi was developed as a way to strengthen both Phi Delta Theta and its impact on the fight against ALS.

Through the fundraising efforts of members participating in marathons, triathlons, bike races or other athletic challenges, the Fraternity is mobilizing its membership base to help the Phi Delta Theta Foundation and The ALS Association achieve their missions. The goal is to raise more than \$1 million within the first four years of the program.

Phi Delta Theta’s connection with The ALS Association is through Lou Gehrig, who was a member of the Fraternity’s chapter at Columbia University. To learn more about Phi Delta Theta’s Iron Phi initiative, visit www.ironphi.org.

Caring and Quality Are What Motivates National Partner Permobil

By Gary Wosk
Staff Writer

Permobil, one of the most well respected mobility companies in the world, also is one of The ALS Association’s national partners of choice for its national signature fundraising event, the Walk to Defeat ALS.®

The Sweden-based company approached The Association about becoming a national partner in 2006 after one of its power wheelchairs was used by actor James Woods when he portrayed a doctor living with ALS on NBC’s “ER.” The overture made sense because many of the company’s customers are people living with the disease.

Since Permobil became a national partner of The Association, the company has become a familiar sight at many of The Association’s events. Permobil technicians who attend The Association’s ALS Advocacy Day and Public Policy Conference Day in Washington, D.C., provide free repairs and replacements of power wheelchairs used by people with ALS.

“Permobil is a vital partner to our advocacy conference,” said Steve Gibson, vice president of government

relations for The Association. “By helping ensure that people with ALS can navigate the halls of Congress, Permobil is empowering them with the ability to make a difference in the fight for a treatment and cure on Capitol Hill.”

A world leader in the developing and manufacturing power wheelchairs, Permobil, founded 40 years ago, wanted to be known as more than the company that sold the most power wheelchairs.

“We wanted to become involved with an organization such as The ALS Association to give something back to the community,” said Barry Steelman, the director of marketing for the U.S. division of Permobil. “We

also wanted our employees to feel good about their company.”

Each year the company donates \$150,000 to The ALS Association as well as four power wheelchairs valued at as much as \$25,000 each to The Association’s chapters, which then add the wheelchairs to loan closets. In addition, the company provides \$3,000 staff scholarships to the chapters whose Walks have improved the most compared to the previous year.

“We have 38 sales representatives in the United States and we encourage them to get involved with local chapters,” Steelman continued. “They make sure the power wheelchairs in the loan closets are in good working order.”

The Association’s chapters are very appreciative of Permobil’s support.

“Permobil is the premier company we partner with to implement our mission,” said Lance Christian, executive director of The Association’s Oregon and Southwest Washington Chapter. “The company has been a sponsor and has donated equipment to our chapter in association with the Walk to Defeat ALS.”

Many people with ALS use Permobil power wheelchairs because they have enhanced the quality of life.

“The Permobil wheelchair provides me with freedom and independence,” said Ken Kinder, who has ALS and is helped by The Association’s DC/MD/VA Chapter.

ALS Association chapters are happy that people with ALS select Permobil chairs, because they come highly recommended by physicians and physical therapists. “Chapters want people with ALS to know that when they own a Permobil they will enjoy the most mobility possible and receive dependable service as well,” said Ashton Chase, senior director of National Signature Events for The Association.

For information about Permobil visit www.permobil.com.



“By helping ensure that people with ALS can navigate the halls of Congress...”
—Steve Gibson



A Love Story



Inspires a Heartfelt Gift to The ALS Association

*“I hope this story
might cause
other people to
join the fight.”*

It was a fateful day in 2008 when Bill Robinson, 84, met Betty Scharf, 77, at their retirement village in Southern California. Their shared love of duplicate bridge spawned an unexpected romance. Then, just a little more than a year later, their close relationship led to a lovely, generous gift to The ALS Association.

In December 2009, Bill’s sister, Nancy Reckinger, passed away. In her will, Nancy asked something rather unusual of Bill. She wanted him to select charitable organizations worthy of receiving her estate, which was surprisingly large for a woman who’d dedicated her life to public education.

Bill turned for advice to Betty, his “sweetheart.” Betty shared her hopes that Bill might consider a gift to The ALS Association. Her husband died of ALS in 1990 at age 58, and Betty was concerned her three children, all now in their fifties, could possibly face a similar fate.

Bill, who said he shares a “big, emotional attachment” to Betty’s children, agreed immediately to give one-third of Nancy’s estate to The ALS Association. With Betty, Bill chose to support the Northeast ALS Consortium (NEALS), a network of clinical trial centers throughout the U.S., which stands ready to test promising compounds in people with ALS, as well as provide opportunities for training of newer investigators to participate in clinical trials for ALS.

Betty is excited to be making this difference in the lives of others. “I just can’t tell you how thrilling it is to me,” she commented. “Not just for my children and grandchildren, but I’ve worked very hard for The ALS Association, and I saw all the wonderful people whose lives were touched by this terrible, terrible disease.”

Bill is philosophical about honoring his sister’s memory, noting, “I hope this story might cause other people to join the fight, and that it inspires and motivates others to follow suit.”

While Nancy’s gift to The ALS Association came about in a unique way, it is a warm reminder that others may wish to consider The ALS Association when planning their own estate. To discuss your estate or to ask any questions, please contact David Moses, Director of Gift Planning, at (888) 949-2577, ext.212, or dmoses@alsa-national.org



ISIS-SOD1_{RX} Antisense Treatment

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The study is a placebo-controlled, dose-escalation study designed to assess the safety, tolerability and pharmacokinetic profile of ISIS-SOD1_{RX} in patients with familial ALS that is caused by mutations within the SOD1 gene. The study consists of four cohorts with eight patients each. In this study, ISIS-SOD1_{RX} will be administered intrathecally (in the spinal canal) using an external pump to deliver the drug directly into the spinal fluid during a single, 12-hour infusion. The study will be conducted at multiple centers within the United States.

ALS Association Top Rated by Charity Navigator

Charity Navigator (www.charitynavigator.org), the nation's largest and most-utilized charity evaluation organization, gives The ALS Association four stars – its highest possible ranking – and says The Association “exceeds industry standards and outperforms most charities in its cause.” When you support The ALS Association, you can be confident you are fighting Lou Gehrig’s Disease.

The ALS Association

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Walk to Defeat ALS Raises Millions

The ALS Association’s Walk to Defeat ALS experienced continued growth in 2009, raising more than 16.6 million nationwide. During a year when many nonprofit Walk programs experienced revenue decreases, The Association’s growth in participation and revenue confirms there is much to celebrate across the organization.

The 2010 Walk season is currently underway. The ALS Association’s goal for 2010 is to encourage greater participation in every community across the country.

Visit www.walktodefateals.org to register for a Walk event near you or to make a contribution.

