

## GENE THERAPY UPDATE

It began with a phone call. Three years ago, Fred H. Gage, Ph.D. of the Salk Institute, called Project A.L.S. to say that a gene therapy approach he was developing for Alzheimer's disease might be worth trying in ALS. Project A.L.S. funded a pilot experiment immediately. Results were so promising that Project A.L.S. funded the next phase of Dr. Gage's work. The broader investigation showed that ALS mice receiving the gene therapy lived up to 33% longer than untreated ALS mice. Results of the Brian Kaspar, Fred Gage et al. study were published in August, 2003, in the journal *Science*.

Project A.L.S. continued working hand-in-hand with the Gage Lab, and with Ceregene, a biotechnology company that came on board to create this gene therapy for human trial. The pre-clinical development program, as overseen by Ceregene, and funded by Project A.L.S., is now nearly completed.

**The broader investigation showed that ALS mice receiving the gene therapy lived up to 33% longer than untreated ALS mice.**

Project A.L.S. recently partnered with the Packard Center for ALS Research, and the ALS Association., to support the further development of this potential therapeutic as it moves closer to human trial, currently slated for 2005. Please stay in touch with Project A.L.S. for up-to-the-minute news. ★

## Project A.L.S. Stem Cell Breakthrough: Nerve Connects With Muscle

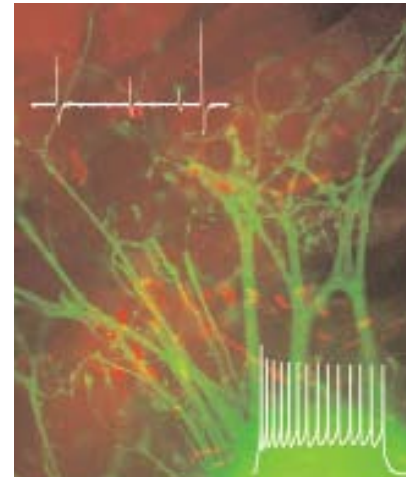
**T**wo years ago, Project A.L.S.-funded scientists led by Hynes Wichterle and Thomas M. Jessell, of Columbia University, showed they could direct stem cells to become motor neurons—the nerve cells that die in ALS—and, further, that these stem-cell derived motor neurons could survive in animals. Now Project A.L.S.-funded collaborator Robert Brownstone, of Dalhousie University, Nova Scotia, has taken that work to the next level.

Brownstone and colleagues reported in the September 15, 2004 issue of the *Journal of Neuroscience*, that stem

**Brownstone et al:**  
**Motor axons (green) extending from embryonic stem cell derived motor neurons connect with muscle fibers (red) and elicit action potentials.**

cell derived motor neurons can form functional connections with target muscles *in vitro*.

Having identified the electrophysiological properties of stem cell derived motor neurons, Dr. Brownstone now aims to establish similar nerve-muscle connections in live animals.



Project A.L.S. is proud to be funding this ongoing investigation in its entirety. ★

## Spinal Cord Provides New Clues

**S**ally Temple wasn't thinking about ALS in 2001. She was working with neural stem cells, which she observed could differentiate into various cells of the nervous system. That's precisely what captured the interest of Project A.L.S.



Dr. Sally Temple, an investigator at Albany Medical College

Project A.L.S. commissioned Dr. Temple, an investigator at Albany Medical College, to identify the minute population of stem cells that reside in the spinal cord, and help them to become motor neurons, the cells that are lost in ALS—a tall order to be sure.

Dr. Temple now shows that the spinal cord, long considered a poor source of stem cells, may be somewhat fertile

ground. In collaboration with Project A.L.S.-funded scientist and research advisor, Thomas M. Jessell, Dr. Temple has identified a small window of time during which spinal stem cells are likely to differentiate into motor neurons. She has also

devised an efficient method for extracting the spinal stem cells at that precise time, growing them up to greater numbers in culture, and directing them to become motor neurons.

Says Dr. Temple, "We hope to be able to use our technique to study how motor neurons develop and survive. In addition, we intend to transplant these cells back into the spinal cord to find out whether they

(CONTINUED ON PAGE 3)

## ALS: A RETROVIRAL COMPONENT?

**A team of scientists led by Ammar Al-Chalabi (Kings College London) and Jeremy A. Garson (University College London) has just completed a study funded in full by Project A.L.S. investigating the prevalence of reverse transcriptase — an enzyme present in retroviral infection — in the serum of ALS patients. The findings of the study will be published in February in the scientific journal *Neurology*. Project A.L.S. is funding the team to expand this investigation to elucidate a possible role of exogenous or endogenous retroviruses in ALS. ★**

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# WEIZMANN INSTITUTE HONORS



**Bruce Pollack,** chairman of the New York chapter of the American Committee for the Weizmann Institute of Science.

On the evening of November 4, at the Mandarin Oriental Hotel in New York, the American Committee for the Weizmann Institute of Science honored its Women of Vision. Meredith and Valerie Estess, co-founders of Project A.L.S., were two of those women.

The esteemed crowd, which included Nobel laureates and their colleagues from research science, and generous supporters of scientific research at the Weizmann Institute, paid tribute to the Estess sisters and noted the accomplishments of Project A.L.S. Additional honorees were Barbara Levinson, a leading fundraiser, and Professor Hadassa Degani, a researcher working in breast cancer imaging.

“Jennifer Estess has and always will be *our* Woman of Vision,” said Project A.L.S. president Meredith Estess. “Valerie and I accept this award from the Weizmann Institute on her behalf, and on behalf of sisterhood, which we always knew was powerful.”



**Larry Tarica, COO**  
 The Frye Company

## Frye Kicks Off Jenifer Boot

### Company Previews Estess Collection

Over 150 members of the media, retailers and special guests, attended the Frye Boots Spring 2005 preview at Ruby Falls, in New York City, on October 26. Project A.L.S. board member, Rob Morrow, hosted the event.

Jim McCormick, President of Frye, and Guy Marshall, Creative Director, announced the expansion of its two-year partnership with Project A.L.S., “A Step Toward A Cure,” the brainchild of Project A.L.S. angel, Larry Tarica.

The announcement was followed by the preview of a signature collection of women’s and men’s boots slated for Fall 2005 delivery, in honor of Project A.L.S. co-founder, Jennifer Estess.

“In honor of our friend, Jennifer, we are proud to unveil the limited edition Jenifer Boot Collection to raise proceeds on behalf of Project A.L.S.,” said Jim McCormick. According to Frye designer, Guy Marshall, “This collection is a tribute to Jennifer’s amazing outlook and incredible strength.” ★



## New York Gala Nets \$1 Million

Ben Stiller and Christine Taylor hosted the seventh annual Project A.L.S. *Tomorrow is Tonight* event, sponsored by InStyle, which netted \$1 million. The quintessential couple hosted the quintessential New York evening, a moving tribute to the life and work of Jennifer Estess. Over seven hundred turned out for cocktails, dinner and a show at New York’s fabled Cipriani.

Participants included Edie Falco, Julianna Margulies, Marisa Tomei, William Baldwin, Toby Parker, and Katie Couric. Robert H. Brown, of Massachusetts General Hospital delivered a research update. Project A.L.S. board chairman Robert S. Kaplan spoke. Allison Moorer sang. ★

**ABOVE: Hosts Ben Stiller and Christine Taylor with Bobby Cannavale and Annabella Sciorra. RIGHT: Marisa Tomei and Katie Couric with board chairman Rob Kaplan.**



# Coldwell Banker Walks On for Research

**O**n October 23, a glorious Saturday, Coldwell Banker Residential Brokerage in Connecticut/Westchester walked the walk for Project A.L.S. — and raised over \$105,000 for cutting edge research. The event, a 3-kilometer affair on the grounds of SUNY Purchase, featured palpable team spirit, crisp autumn weather, and memorable motivational words from the race's official starter, William Baldwin.

Over four hundred walkers, many representing Coldwell Banker offices throughout Connecticut and southern Westchester, participated, raising funds through the walk, and satellite events like tag sales, holiday gift bazaars, a concert, and office raffles.

"We have become passionate about Project ALS," says Peggy Doepper, president and chief operating officer of Coldwell Residential Brokerage in Connecticut/Westchester. "I couldn't be prouder of my management team, sales associates and staff — all of whom are committed to making a difference. We're in this for the long run!" ★



**TOP:** Over 400 walkers helped raise \$105,000 for ALS research  
**ABOVE:** Jude Kravitz and Peggy Doepper of Coldwell Banker Residential Brokerage join Billy Baldwin, Marilyn, Valerie and Meredith Estess in kicking off the Walk for Project A.L.S.

For more information or to make a donation, please write to:  
**Project A.L.S.**  
 900 Broadway, Suite 901  
 New York, NY 10003  
 or call 1-800-603-0270  
 or 212-969-0329  
 email: info@projectals.org  
 visit our website at  
 www.projectals.org

## SPINAL CORD PROVIDES NEW CLUES

CONTINUED FROM PAGE 1

**Dr. Temple now shows that the spinal cord, long considered a poor source of stem cells, may be somewhat fertile ground.**

can be encouraged to generate motor neurons in the adult."  
 Dr. Temple and her collaborators look forward to reporting next discoveries. "I have to say that working with Project A.L.S. is different from working with others. Project A.L.S. promotes collaborations. The personal involvement is also a strong motivator, and we are working very hard to push this forward." ★

## FUNDRAISING ROUND-UP:

GIFTS CELEBRATING THE EXQUISITE FRANK SELVAGGI-WILLIAM SHEA WEDDING TOTALED \$18,000...ACTRESS AND COMEDIAN CAROLINE RHEA WON CELEBRITY BLACKJACK HANDS DOWN, DONATING

HER \$50,000 TV ACTORS RICHARD POKER FACES WON \$5,000 AND CUT-A-THON



Paul Rudd

WINNINGS TO PROJECT A.L.S... RICHARD KIND AND PAUL RUDD BROUGHT THEIR BEST TO CELEBRITY POKER SHOWDOWN ON BRAVO, AND AND \$12,500, RESPECTIVELY...THE JANINE ROSE COLOR IN HARRISON, NY NETTED \$3,600...MORE SOON. ★



Richard Kind



**Call for our new research summary: 1-800-603-0270**

# Project A.L.S. Hosts Preview Party for The New York Design Fair



**P**roject A.L.S. will host the Benefit Preview of the first New York Design Fair, sponsored by House & Garden Magazine. The event will take place on Wednesday, February 9th, 2005. The cocktail reception at the Seventh Regiment Armory, Park Avenue and 67th Street, New York City, will provide a first glimpse at the wide range of antiques, modern décor, home accessories and garden design from seventy-five international dealers. Dominique

Browning, Editor-in-Chief of *House & Garden* magazine, will be the Honorary Chair of the Preview Party. Proceeds from the preview will go to Project A.L.S.

This new style show emphasizes today's trend to mix period and contemporary pieces for indoor and outdoor living. The Seventh Regiment

Armory is recognized worldwide as the finest exhibition hall in the country. The Design Fair will attract collectors, interior designers and people for whom personal style knows no bounds.

The New York Design Fair, produced by Wendy Management, will run from February 10 through the 13th.

For Benefit Preview ticket information, call: Project A.L.S. at 212-969-0329 ★



## SUPPORT THE PROJECT A.L.S. SOD-1 COLONY

**M**ice are often considered a nuisance—especially by researchers who wait up to several months just to receive shipments of the all important SOD1 transgenic mouse. When these shipments are delayed—so is crucial research science.

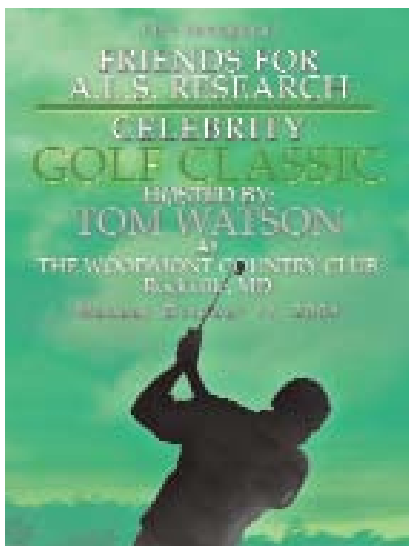
Now the Project A.L.S. SOD1 Colony is speeding research dramatically by providing this valuable tool instantly and free of charge. To date, the Colony has shipped nearly 5,000 mice to scientists everywhere.

To support the colony, or for more information, call 800-603-0270. ★

**“It is difficult to overstate the impact that Project ALS continues to have on understanding how ALS is caused and how it may be treated. Its hallmark is an ability to find “out-of-the-box” solutions and to recruit top flight investigators to the research program. The combination of creative ideas and world-class scientists has been remarkably catalytic.”**

**ROBERT H. BROWN, JR.,**

M.D., D.PHIL., HARVARD MEDICAL SCHOOL, MASSACHUSETTS GENERAL HOSPITAL



## Golf Legend Tom Watson Supports Gene Therapy

Golf great Tom Watson, who lost his caddy Bruce Edwards to ALS, hosted the inaugural Friends For ALS Research Golf Classic on October 11, at the prestigious Woodmont Country Club in Rockville, Maryland. Project A.L.S. board member Linda Wertlieb and her incredible daughters were driving forces behind the event, which netted \$200,000 for Project A.L.S. and its partner in gene therapy fundraising, the Robert Packard Center for ALS Research. ★

**A GREAT HOLIDAY GIFT IDEA**



## TALES FROM THE BED

A MEMOIR  
by Jenifer Estess  
as told to Valerie Estess  
with a foreword by  
Katie Couric

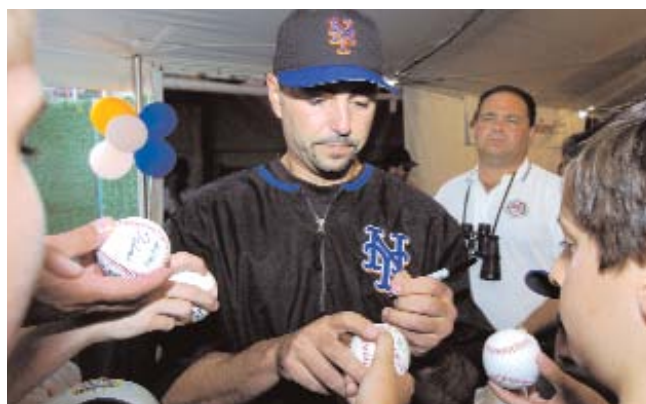
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# Amazin's Are #1 in Supporting Research

**T**he New York Mets continue to lead the majors in the fight against ALS. The second annual Night to Believe, held last on June 4, at Shea Stadium, and presented by the Frye Company, raised over \$100,000. Family fun began with celebrity batting practice, and continued with player autographs for the kids, and a barbecue and sushi extravaganza under the big tent in the picnic area. Eight hundred children of all ages cheered from the Project A.L.S. bleachers as the Mets gave their all against the Florida Marlins.

"Project A.L.S. is thrilled to be working with the New York Mets. I'd like to acknowledge Al and Lori Leiter, John and Rose Franco, all of the Mets players' wives, the entire Mets organization and our cherished board member, Debbie Wilpon, for creating this dynamic partnership," said Meredith Estess, Project A.L.S. president.

The third annual Night to Believe is slated for next June 3, with the Mets taking on the San Francisco Giants. It'll



be bigger and better, with an expanded player autograph session, and a chance for one lucky raffle winner to throw out the first pitch of the game. See you there. ★

**TOP: Mets owner Fred Wilpon welcomes the Second Annual Night to Believe crowd. ABOVE: John Franco autographs baseballs for kids of all ages.**

## Boca Raton Hits Hole-In-One

**T**he third time's the charm. That's what they're saying in Boca Raton, Florida, where Alan Jacobson, Jack Bernstein, Tony Wollins, Beth Schwartz, and Project A.L.S. board member, Howard Hirsch, are gearing up to make the third annual golf tournament to benefit Project A.L.S., even more successful.

Last winter's event netted over \$150,000, and attracted competitive golfers, and the comedian Caroline Rhea, who entertained the crowd after dinner. All paid tribute to Andy Decter, 42, a son of New Jersey and Florida, who fights against ALS every day.

The third annual Boca tournament is set for February 28 at Deer Creek Country Club. For more information: 800-603-0270. ★

## Americana Manhasset's Champions for Charity® 2004 Welcomes Project A.L.S.



Two fabulous pre-holiday events at Americana Manhasset, one of the New York area's premiere shopping experiences, brought awareness and crucial funds to Project A.L.S.

On November 18, Dolce & Gabbana held a grand opening celebration of its new boutique, featuring complete

men's and women's collections, with 20% of pre-tax purchases going to Project A.L.S.

Then, on December 2, the legendary outfitter Hirshleifer's hosted a holiday reception for Project A.L.S., the newest participating charity in **Champions For Charity®**, Americana Manhasset's dynamic giving program that has raised over \$1.8 million for non-profits. ★



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## WINTER NEWS 2004-2005

FINDING AND FUNDING TREATMENTS AND A CURE FOR ALS



Dear Friend,



This is the first holiday season we've spent without our sister Jenifer. She died from ALS at the age of forty last December 16. The world is a different place without Jenifer Estess. Her nieces and nephews miss her so much. Our family is finding its way. It has been difficult.

Yet Jenifer continues to guide and inspire us—and to help Project A.L.S. move scientific research forward. We are proud of the accomplishments summarized here, and we look forward to working with you to make 2005 even more productive.

Project A.L.S. is committed to fighting on, without fear, using every rational means available to identify the causes and the first effective treatments for ALS. We won't stop or slow down until that day when our children, parents, and friends, live in a safer world.

The brain remains the most misunderstood organ. It's time we understood it. With your help and thoughtful support this holiday season, we will move closer to knowledge, which, as Jenifer always taught us, is power.



Happy Holidays,  
Valerie and Meredith Estess