

Important ALS News Briefs From Elsewhere

Retired Director ALS Clinic at UCSF to be honored by his colleagues



Dr. Richard Olney, recently retired director of the ALS Clinic at the University of California, San Francisco and noted EMG specialist, will be honored at a special professional symposium and dinner for his clinical and scientific accomplishments on Friday, December 17th from 1:30 – 5:30pm at UCSF's Genentech Hall.

In an especially cruel irony for reasons known only to the gods, and after caring for neuromuscular patients for many years, Dr. Olney a short time ago announced that he has now himself recently been diagnosed with ALS. This quietly modest and humane physician must now face the specter of debility presented by the disease for which he has treated many others.

In attendance at the symposium to talk about the advances of neurology, as well to pay homage to the professional achievements of Dr. Olney, will be Dr Stanley Prusiner, Nobel Laureate in Medicine and Director, Institute for Neurodegenerative Diseases; Dr. Arnold Kriegstein, Director, UCSF Developmental and Stem Cell Biology Program; and Dr. Jeffrey Rothstein, Director Robert Packard Center for ALS Research, Johns Hopkins University, Baltimore, Maryland.

The Forbes Norris ALS Center's Clinical and Basic Science Staff will be in attendance to honor Dr. Olney's career and contributions to ALS.

For further information, please call: UCSF Office of Continuing Education (415) 476-5808.

Scientists in Europe Seek License to Clone Human Cells to Study ALS Nerve Cells

With the United States, normally a leader in worldwide science, now restrained by governmental inhibitions on stem cell research, leadership in this field may now be taken by the European and Asian scientific community. This was made evident recently with announcement of an application for human therapeutic cell cloning by the neuroscientists in Scotland who led the creation of Dolly, the cloned sheep. Britain became the first nation to legalize research cloning in 2001, but only now are the developing techniques beginning to be used.

Dr. Ian Wilmut plans to clone cells from patients with ALS (Motor Neuron Disease), make them develop into nerve cells, and compare their development with nerve cells derived from healthy embryos. Dr Wilmut hopes for approval of his application authorizing this form of cell development by early next year and to begin work in the spring.

Dr. Brian Dickie, director of research development at the London-based Motor Neuron Disease Association, said, "At the moment, we have to rely on post-mortem tissue, and that's very much the end stage. What we don't have, and what we desperately need, is a way to study human motor neurons as the disease progresses. If we are going to develop a treatment, the chances are that we are going to have to catch the disease at a relatively early stage."

Report by: Emma Ross, Associated Press, September 2004

The Forbes Norris ALS Center News is published twice a year. For more information or to be removed from the mailing list, please call (415) 600-3608.



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The Gift of Giving

The Forbes Norris MDA/ALS Research Center is totally dependent upon private donations to support its patient services programs. It is supported, in part by the generous donors for ALS to the California Pacific Medical Center Foundation.

For more information on the Foundation or on ways you may give to the Forbes Norris MDA/ALS Research Center to support their services, please call Dee Norris at (415) 600-3608.

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The Forbes Norris MDA/ALS Research Center Forbes Norris ALS Center News

FALL | WINTER 2004

We've Been Very, Very Busy

Over the last two years the Forbes Norris ALS Research Center has been unusually busy! We have hosted visiting professors, national professional meetings, educational conferences, and fundraising events, in addition to continuously caring for our ALS patients. This is the reason you haven't heard from us since last year.

Here is some of what we did:

The American Neurology Association Annual Meeting held October 2003 in San Francisco.



"Ask the Experts" panel.

During this national meeting, the Forbes Norris ALS Center, in conjunction with the ALS Association, created and hosted an extraordinary "Ask the Experts" panel presentation of internationally recognized ALS experts discussing the latest in current ALS science. The Norris ALS Center's Medical Director, Dr. Robert Miller, ably chaired the presentation. Videotapes of this panel are available through the ALS Association, Bay Area Chapter offices at (415) 904-2572.

The American Academy of Neurology's Annual Meeting held April 2004 in San Francisco

The Norris ALS Center and the Muscular Dystrophy Association hosted another "Ask the Experts" panel presentation. Audiotapes of this presentation are available by contacting the Muscular Dystrophy Association at (415) 673-7500.

The new **National ALS Research Group** met during the American Academy of Neurology's Annual Meeting. The group was later hosted at the home of the Norris ALS Center Advisory Director, Dee Holden Norris. Over sixty neurologists and health care professionals enjoyed a lovely evening and gorgeous views overlooking San Francisco Bay. Larry and Wanda Alary and the Pedroncelli Family Vineyards of Healdsburg, California generously donated wines.

The 14th International ALS/MND Symposium and Alliance Meeting was held November 2003 in Milan, Italy

A report on the symposium proceedings can be read on the International ALS/MND website at www.alsmndalliance.org.

Visiting Lecturers in 2004

The Norris ALS Center hosted several noted visitors from prominent ALS academic institutions around the country to deliver educational lectures and present scientific papers.

Joseph S. Beckman, Ph.D.
Linus Pauling Institute, Oregon State University.

"Did Radicals Kill Lou Gehrig?" – Insights into zinc and the toxicity of SOD in ALS"

Jeffrey Elliot, M.D.
University of Texas Southwestern Medical Center.

"The Molecular Basis of SOD1 Aggregation in Familial ALS"

Stanley H. Appel, M.D.
Professor and Chairman, Dept. of Neurology, University of Texas Southwestern Medical Center
"Neurons Don't Die Alone: The Role of Inflammation in Amyotrophic Lateral Sclerosis"



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From the Desk of Robert Miller, M.D., Medical Director



At the Norris ALS Center, we are actively engaged in clinical research trials testing new pharmaceutical agents which we hope will have a substantial impact on the disease course. This is an unprecedented period for the National Institutes of Health (NIH) and federally funded clinical research in ALS. Until only a few years ago, federal funds were not available for ALS trials due to a perception that too little was understood about the disease.

Today, two new NIH funded trials will soon be launched, and the Norris ALS Center will play a key role in both. The first is high dose Co-enzyme, Q10, a drug that has already shown promise in Parkinson's disease. The Phase 2 study will determine the optimal CoQ10 dosage and effectiveness. The second trial will evaluate an antibiotic, ceftriaxone, a widely available

drug that has shown great potency in promoting the survival of motor neurons.

There is now ample evidence that oxidative stress, the build up of oxidized substances in the nervous system, plays an important role in ALS. Available antioxidants have thus far demonstrated very little beneficial effect against ALS in the mouse model. Now there is a novel antioxidant, manganese porphyrin, which has demonstrated a prolongation of survival of 250%. This huge impact in the mouse model compares with only a 10% prolongation of survival in the mouse from Rilutek. The drug will be tested first for human safety at the Norris ALS Center under the supervision of the Food and Drug Administration and Aeolus, a new pharmaceutical company.

With these exciting pharmaceutical agents, gene therapies, and stem cell research clearly on the horizon for future trials, we have never been so optimistic that soon we will find effective treatment for ALS.

UPCOMING EVENTS

15th International ALS/MND Symposium and Alliance Meeting

December 2-4, 2004
Philadelphia

This is the most important, and the only scientific meeting dedicated solely to ALS, to be held anywhere in the world. A report on its proceedings will be made available to anyone on the Alliance's website at the beginning of next year at www.alsmndalliance.org.

Our Partners in Care Show Their Support

Norris ALS Center Fundraising Events

ALS patients and their families generated an assortment of significantly successful fundraising events supporting the Norris ALS Center Patient Services and Research programs. It is through these events that the Norris ALS Center is able to deliver for our patients the most effective treatment options available. We are immensely grateful to all our donors and their families for their initiative, their caring support, their inspiration, and dedication to the Norris ALS Center staff and their work.

These successful events inspired three different ALS Family Funds specifically dedicated to supporting the Patient Services Programs at the Norris ALS Center.

Larry and Wanda Alary ALS Family Fund



The First Annual Alary Golf Tournament was held May 30th in Healdsburg, California. Many friends and family came to support The Alary family by playing golf and then donating auction gifts of wine, tours, and special events, such as an abalone feed and a boar hunt, all of which created a wonderfully successful event. Wine makers Phyllis and John Pedroncelli specially commissioned a local

Bottom to top: Dr. Deborah Gelinis, Larry Alary, Dee Norris, Wanda Alary

artist to create a personalized wine label dedicated to Larry, which captures the essence of his unique spirit.

The Scott and Annabel Lew ALS Family Fund

Hollywood producer, Scott Lew and his lovely wife, Ann, decided to launch their own ALS Family Fund through a special wine tasting benefit held July 15th at Universal Studios in Hollywood, California. Organized by the dynamic Susan Paley, this successful event featured over 15 different wineries and a number of notable Hollywood film personalities, all showing their highest regard for Scott and his family by supporting this cause.



The Scott Lew family on a recent trip.

Marshall Mathews ALS Family Fund



Nancy Mathews

With the inspiration of the indomitable Nancy Mathews, the Marshall Mathews ALS Family Fund, created in September 2002, continues its namesake's legacy. Nancy not only supports ALS but also continues to participate in antique car events around the west. She recently rode in a re-named "Marshall Mathews Memorial Ride" in honor of her late husband, which began in Scottsdale, Arizona.

The Norris ALS Center participated in several ALS organizations' events:

- The ALS Association, Bay Area Chapter's **All Star Gala Awards**, May 22nd at San Francisco's SBC Park
- The MDA "Gateway to a Cure: A Giant Swing at ALS", May 17th at San Francisco's SBC Park
- The ALS Association "Summer Games," in conjunction with the Ruth Bancroft Garden, July 17th, on a private estate in Alamo, California

RESEARCH AGENDA



ALS Basic Research



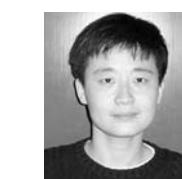
Nancy M. Lee, Ph.D

Her laboratory work continues on the role of abnormal inflammation as a destructive process on motor neurons in ALS. Dr. Lee has discovered that this destructive process occurs earlier than previously thought and this may be an important clue in the whole picture of the degeneration of the nerve cell. This work will be reported on at the next ALS/MND International Symposium in Philadelphia.



Mary Abood, Ph.D

She has shown that THC or Dronabinol, the active ingredient in marijuana, has been found effective in delaying motor impairment and prolonging survival in mice. This work led to a "Phase 1", or tolerability study in humans, which was reported on at the International ALS Symposium in Milan and was published in the journal of Amyotrophic Lateral Sclerosis and Other Motor Neuron Diseases in March of 2004. Dr. Abood continues her work on these apparent neuroprotective mechanisms seeking a clinically effective treatment to slow progression of ALS.



Jian Liu, Ph.D

Her work focuses on the role that malfunctioning mitochondria, the "energy factories" in cells, play in the destruction of motor nerve cells. Increasing evidence, both in human and animal models, has shown that abnormal mitochondria are one of the pathological features of ALS disease process.



Eric Beattie, Ph.D

His work focuses on the specialized receptors in nerve cells, abbreviated as AMPARS. These receptors aggravate inflammation, making it more intense, and thus more potentially destructive. These receptors interact with another chemical, TNF-alpha, in a complex way to enhance the effects of glutamate, a necessary neurotransmitter, causing it to be over produced, which seems to lead to the cell's death in ALS. This very complex mechanism remains under study.

ALS Clinical Research

Current Clinical Research Trials

Insulin-Like Growth Factor-1: Phase 3 study:

- Requires a breathing capacity of 60% or greater to participate.
- The study is 2 years long and requires seven visits over 24 months.
- Requires self injection twice daily, seven days per week.

Minocycline: Phase 3 study

- Requires a breathing capacity of 75% or greater to participate.
- The study is 13 months long; requires monthly visits.
- Oral medication twice daily.
- 50% of patients receive placebo, 50% of patients receive minocycline.

Creatine: Phase 3 study

- Requires adequate upper extremity strength.
- Study is 9 months long requires weekly visits the first 4 weeks, then monthly visits the next 4 months, then 2 additional visits at 2 month intervals.
- 50% active medication; 50% placebo.

Co-Enzyme Q-10: Phase 2 Study

- Breathing Capacity of 60% or greater.
- Study is 9 months long: Monthly visits.
- 2/3 of patients get active drug (either 1000mg or 2000mg), 1/3 of patients receive placebo.
- Cannot take medications being tested in other clinical trials.

Aeolus #10150: Phase 1 study

- Breathing capacity 60% or greater.
- 80% of patients receive study medication/20% placebo.
- Single dose of study medication.
- 3-day hospitalization.

For further information concerning these trials, please call the Norris ALS Center Research Staff: (415) 600-3938.