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The Eleanor & Lou Gehrig MDA/ALS Research Center at Columbia University

This issue of the Eleanor and Lou Gehrig MDA/ALS Newsletter is dedicated to the courage and strength demonstrated by:

Linda Barrett, James Bungerz, Lewis Cole, Luisa Gomez, Norma Katcher, Eve Lodge, Mary Marcasciano, Gladys Orans, Eleanor Penziner, Beryl Quinerly, Marvin Rasnick, Marc Raeff, Allan Rosenfield, Ines Schoot, Zevie Schiezer, Ralph Wieskus, Perry Wagner

From the desk of Hiroshi Mitsumoto, MD



Dear Friends,

From Nov 2nd - 5th, the 19th International ALS/MND Symposium was held in an airport hotel in Birmingham, UK. The chilly, soggy, and dark weather combined with the meeting location created the perfect conditions for enjoying the 4-day meeting. This meeting is the only international meeting solely devoted to ALS. Almost every ALS expert, health care professionals and some patient delegates got together to report the results of their research studies, learn about new progress, and exchange their ideas. A day before the real symposium begins, it functions as a magnet, so a number of clinical and research meetings are held. For example, the European ALS group meets and discusses their annual research direction. The World Federation of Neurology (WFN) Motor Neuron Disease Subcommittee also has a meeting. This year's topic was ALS biomarkers. The North American ALS Study Group, for which I was the first elected chair, had a meeting to discuss the DNA repository and providing follow-up data on patient status, advocacy issues as to how to increase patient participation in clinical trials. Allied Health Professionals also had their conference to present their study on how to improve patient care. A number of regional ALS study groups also had their meetings.

The Symposium began with a brief welcome speech made by the British Health and Science Minister. The entire symposium was divided into simultaneous parallel clinical and basic science sessions. Basic science sessions included Cell Biology, Pathology, Translational Strategies, TDP-43 (a new disease marker

of ALS), Use of the SOD1 mouse in therapeutic testing, Role of non-neuronal cells, Genetics, In vivo models mostly on SOD1 mice, and SOD1 Pathogenesis. Clinical sessions included MND phenotypes (in this session, we presented a couple of our studies on ALS subtypes such as primary lateral sclerosis and progressive muscular atrophy), Quality of life and decision making, Communication, Managing ALS and clinical practice, Multidisciplinary care management, Clinical electrophysiology and imaging, Cognitive and psychological change, Beyond Guam: new aspects of the cyanobacteria and BMAA hypothesis, Clinical trials and trial design (in this session, Dr. Kaufmann presented the results of CoQ10 study), and Respiratory management (Dr. Kasarskis presented results of the nutrition study in which we participated).

The second morning, more than 170 posters ranging from pure basic science topics of ALS to all clinical issues were presented in one room. There were many people discussing sciences in front of every poster. The last day, there was a plenary session discussing the highlight of clinical and science sessions of the symposium, and two scientists gave a talk on the new strategies of ALS therapy on stem cells and vaccination strategies. The meeting organizer, British MND Association, reminded us that there were only 47 people who attended the first symposium in Solihull, a suburb of Birmingham (I have been attending this meeting since the second symposium). This year, there were more than 800 delegates who are working on ALS at this symposium from all over the (continued on page 2)



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world. It is pretty amazing to realize that this symposium has become the most important ALS symposium for the whole ALS community.

This is why I talk in such detail about this meeting in this newsletter. Our Center has contributed to this symposium by continually presenting the results of our studies and participating in the program in past years. Looking at the sessions and research directions, our own research covers many important areas. For example, our Center (Columbia Motor Neuron Center and Project ALS Stem Cell Laboratory) is forefront in the research of stem cell development, translational research and motor neuron biology. The Eleanor and Lou Gehrig MDA/ALS Research Center participates in a number of clinical trials including KN704, Talampanel, Lithium, and Zenvir. We are testing oxidative stress biomarkers, another biomarker for skin elasticity, genetic environmental epidemiology and oxidative stress and disease progression, development of clinically meaningful changes that should be essential for clinical trials, and a prospective study of the noninvasive ventilator. By looking at the symposium, there are many areas we are not investigating. I believe the research fields are strongly influenced by the interest and expertise the investigators and their team have. There is a lot of fierce competition in finding out the cause and cure of ALS, which is a great force for more studies.

What kind of funding do we have to support such research activities? At our Center, the MDA has initiated a new ALS Clinical Network, which promotes new ALS research. We were chosen as one of the first five Centers to participate in new research projects including a project we have proposed, that is, a development of new scale called a clinically meaningful changes scale. That is clearly a positive note; on the other hand, Federal research grants are becoming harder and harder to obtain. The MDA Wings Over Street funding which has greatly supported our overall research has now been reduced to a half of the funds raised, which is a big blow. We are facing a very serious challenge our financial ability to continue our research activity at the current level. The insurance reimbursement for patient care at our ALS Multidisciplinary Clinic has not improved, that is, none of the costs incurred from health care professionals such as the nurse, physical therapist, dietician, speech therapist, or social worker are reimbursed. We are only reimbursed with a doctor's follow-up fee by the insurance. Although we receive support from the MDA, it is certainly not sufficient. Yet we believe the multi-disciplinary clinic is the most effective and helpful way to provide the best care. Non-Profit and academic institutions and ALS Centers like ours need your support and help, more now than ever. We will continue to provide the best possible care and find the cause and cure of this disease.

Traveling with ALS

by Jinsy Andrews, M.D.

Along with summer comes time for vacation and family trips. Many patients with ALS decide to travel by plane or by car for many reasons. However, when traveling for a prolonged period of time, whether by plane or by car, there are certain risks involved for patients with ALS.

Recently, we have seen several patients develop a deep venous thrombosis (blood clot in the leg) after taking a long car ride or plane trip. It has been reported in the literature that the incidence of deep venous thrombosis can be anywhere from 2.7 to 3% (Qureshi, et al., 2007) which is higher than the 0.1% incidence in the general population. The higher incidence in patients with ALS is likely related to impaired mobility. A deep venous thrombosis needs to be treated urgently since it can lead to a pulmonary embolus (blood clot in the lung) which can be life threatening.

Certain precautions can be taken to prevent a deep venous thrombosis including: moving the legs or massaging them during travel, wearing compression stockings or taking medications to thin the blood prior to traveling.

If you are planning to travel, make sure that you discuss your plans with your primary care doctor or neurologist and any medical therapy should be initiated under the supervision of your treating physician.

Reference:

Qureshi MM, et al. Increased incidence of deep venous thrombosis in ALS. *Neurology* 2007; 68: 76-77.





DON'T FORGET YOUR LUNCH!

A lot of our patients travel from far away. ALS Clinic can last for several hours, so by the time it is over, you will be hungry. Remember that it is important to eat. We wish we could provide coffee and cookies, but, unfortunately, we don't have the room and finances for it. Please bring your own lunch and soft drinks. Think ahead and be prepared.

PREVENTING FALLS IN OUR CLINIC

In the past couple of months a few of our patients have experienced falls while arriving or leaving from their clinic visits in the Neurological Institute. Our staff and patients are concerned that these unfortunate accidents occurred. If a fall occurs on the hospital's property, it is our responsibility to ensure that each of our patients visit the emergency department to assess for internal injuries. Due to falls, patients have sustained injuries, missed their appointments and spent many hours in the emergency department. Most of these falls could have been avoided by taking advantage of services offered in the building to assist our patients.

Patients who have difficulty with balance or walking or who have experienced falls in the past should always travel with a companion for assistance. You should not walk to the clinic on your own. Please bring any assistive device that you typically use (walker, cane, or bracing). Not only will this ensure your safety, but will allow us to better evaluate your walking and balance. We know parking is difficult in this area but there are many ways to avoid walking on your own:

- You can wait in the lobby of the building while your companion parks the car.
- Your companion may pull into the driveway of the Neurological Institute and leave the car unattended while assisting you up to the 9th floor for your appointment.
- A security officer on the 1st floor of our building is always available to assist patients to their appointments.
- Wheelchairs are available on the 1st floor for use by any patient attending an appointment in the Neurological Institute.
- Valet parking is available in the front of the Milstein building and patients can avoid walking outside altogether by going through the hospital. Take the patient elevators to the 4th floor of Milstein (corresponds to the 5th floor of the Neurological Institute) and follow signs for the Neurological Institute.

We welcome any further suggestions and hope that we can avoid all falls in the future.

Gabby, Ronit, and the Team
Eleanor and Lou Gehrig MDA/ALS Research Center



CURRENT CLINICAL TRIALS

Actively Enrolling:

- Dextromethorphan/Quinidine (DMQ) in the Treatment of Pseudobulbar Affect in ALS
- Exercise and Oxidative Stress in ALS
- Genetic and Epidemiology Study
- Ambispective case-control study of oxidative stress
- Skin and ALS
- Non-Invasive Ventilation
- Talampanel in ALS

Ongoing (closed to enrollment):

- Stage II Early Treatment of ALS with Nutrition
- 2-Part, Randomized, Double-Blind, Safety and Tolerability Study Evaluating KNS-760704 in Patients with ALS

Upcoming Fall 2008:

- Clinical Trial of Ceftriaxone in Subjects with ALS
- Arimoclomol in ALS

Completed:

- Modafinil for Fatigue in ALS
- Clinical Trial of High Dose CoQ10 in ALS
- Longitudinal study of Cognitive Function in ALS

Please call **Kate Bednarz**
at 212-305-2027 for more information.

Pseudobulbar Affect by **Kate Dalton, R.D.**

Pseudobulbar affect (PBA) is a distinct neurologic disorder that causes sudden and unpredictable episodes of crying and laughing. PBA impacts more than 1 million people in the United States diagnosed with neurologic disease or brain injury. PBA may occur when disease or injury damages the area of the brain that controls normal expression of emotion such as in ALS. This damage can disrupt brain signaling causing a "short circuit" triggering episodes of involuntary emotional expression. It is not life threatening nor does it exacerbate an underlying neurologic disease or brain injury. Please keep in mind that PBA is a distinct neurologic disorder, which can be diagnosed and treated separately from underlying neurologic disease of brain injury.

PBA episodes may look like signs of depression, and as a result, PBA is often misdiagnosed. But unlike depression, PBA episodes are often sudden, unpredictable, and contrary to the person's mood. Some people have both depression and PBA.

The STAR Trial, sponsored by Avanir Pharmaceuticals, is enrolling patients who have PBA.

STAR Trial Participation Criteria

- The patient is 18-80 years of age
- The patient has a clinical diagnosis of pseudobulbar affect (PBA)
- The patient exhibits uncontrollable episodes of laughing and/or crying.
- The patient has a confirmed diagnosis of ALS
- If female, must not be pregnant, breastfeeding, or planning a pregnancy during the course of the study, and must have a negative urine pregnancy test prior to the start of the study
- If female, must have been practicing an established method of birth control for at least the prior month, or be surgically sterile of post-menopausal
- The patient must be willing to not take any prohibited medications during participation in the study

Special thanks to the MDA/ALS Division for their continuing support of our patients and their caregivers.

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