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New York
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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:

Joanne Becker, Arthur Borchert, Virginia Brunke, Robert Cespedes, Genevieve Dodsworth, Joseph Giannone, Hilda Kohl, Margaret Leary, Oddrun Lyngstad, Michael McLaughlin, Anne Poli, Roberto Rosado, William Sheluck, John Smith, and Pierina Stroud

From the desk of Hiroshi Mitsumoto, MD

Dear Friends,

It is time to bring you up-to-date on our Center and overall ALS research activity. The 18th International ALS/MND Symposium took place in Toronto in early December 2007. The meeting had one of the largest delegations, more than 800 people from all over the world, and one of the largest numbers of presentations. In particular, poster presentations have increased not only in numbers but also drastically in quality in the past few years. Throughout the Symposium, I had a clear sense that basic and clinical research in ALS is progressing steadily, but at the same time, I had a feeling that we must have more studies that could fill in the right pieces of this complex jigsaw puzzle that is the mysterious ALS.

One day before this International Symposium began, the World Federation of Neurology ALS Committee held a special symposium on Recent Progress in Genetic Studies in ALS. The last few years, an enormous effort has been made to collect DNA samples from the patients in the USA and throughout the world as well. As the first elected chair of the ALS Research Group, we made a unique effort to collect DNA (joint effort of the National Institute of Neurological Diseases and Stroke, MDA and ALS Association, along with nearly 70 ALS Centers in the USA), resulting in 2000 patient DNAs and 2000 DNAs from healthy controls. Independently, MDA researchers collected several hundred DNAs including controls. Similar efforts have been made in the UK and Europe. As the results of these DNA samples, a number of innovative genome-wise association studies (looking for a clue of single nucleotide polymorphism, SNP,

abnormal genes associated with ALS) were carried out independently by at least 4 sites (NIH, MDA, European, and UK studies). Those results were published in leading medical journals last year. A puzzling fact is that potential genes associated with ALS that the researchers found in their studies were different from study to study. Although it is possible that ALS is likely to be made of many gene mutations and that country and racial differences caused such different results, these utterly different results have made us uneasy.

I don't think such conflicting results mean a setback or regression in ALS research at all. A few years ago, we could not even imagine that these types of studies would be possible. But we now know that they are not only possible, we already have a few studies reported. We have been making enormous progress in building a strong foundation for the future of new genetic studies. No ALS geneticists doubt that there will be more genetic causes of ALS to be discovered or genes that suggest individual disease susceptibility, that is, anyone is prone to ALS. At the same time, we know a single gene is not enough to cause ALS or determine the ALS type but a combination of genes and factors beyond genes such as environment and lifestyle factors that would clearly influence not only the cause but also the onset and prognosis. We already have the right technology to study more genetic factors of this disease. We need more genetic epidemiology studies. In fact, 1000 or 2000 DNA samples may not be sufficient to resolve the questions just raised.

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The Eleanor and Lou Gehrig MDA/ALS Research Center



From the Director's Desk (from page 2)

We need to collect more DNAs along with healthy controls. That means that we need to have an effective system and sufficient funding to support tissue and body fluid banking. Such patient and control material will surely increase the research pace. Furthermore, we need to establish strong collaboration not just within our country, but clearly with international researchers. It is time to collaborate more cohesively.

In this sense, on the last day of the Symposium, the Co-Directors of our Columbia Motor Neuron Center (Drs. Henderson, Przedborski and DeVivo) and myself (as the head of ALS Center) held an exploratory meeting with the researchers from the two principle ALS research centers in UK. Closer collaboration is another step in moving the research process faster and making it more diverse.

During the Symposium, a number of satellite meetings were held. The ALS Research Group (now headed by Dr. Shefner, Syracuse) met to discuss how investigators can develop a mechanism of effective tissue collection. Clinical trial groups such as Northeastern ALS, Western ALS and Columbia CoQ10 Study groups had their own meetings to update the current status and future plans of clinical trials in ALS. Two very intriguing meetings were held: one was about a new potential clinical trial which is being developed in England using a type of food supplement. Another is a human embryonal stem cell therapy. Several clinical trialists who were invited all agreed that more basic animal studies are needed before we can consider clinical studies. Therefore, I feel there are exciting developments and I learned a lot at this Symposium. I hope you share my strong feeling that a lot is going on, far more than I can summarize in this Newsletter.

For the New Year, I reviewed my commitment to continue to provide the best care and management for those who have this difficult disease along with the spouses and families who care for their loved ones. Research work and clinical care must come in tandem.

Hiroshi Mitsumoto, M.D.

Special Thanks to Patrick Gorham, Sheila Noonan, and Stephen Mulcahy of the Scarsdale Uniformed Firefighters Association

On the behalf of the Eleanor and Lou Gehrig MDA/ALS Research Center at Columbia University, we express deep gratitude for the Scarsdale Uniformed Firefighters Association, and acknowledge the generosity and hard-work of many people who on November 30, 2007 created an unforgettable evening filled with music, songs, dance, and laughter, to raise funds and awareness in the fight against ALS.

Representing the Columbia ALS center were Dr. Andrews, Mary, Sasha, Aisha, and myself. The evening was titled "A Musical Taste of Ireland", with performances by the Firefighter Emerald Society Pipes and Drums, Paddy Noonan, Sheila Noonan, Tommy Mulvihill and the Band, Brian Hunter, Eddie Devine, and The O'Rourke School of Irish Dancers. What an evening of spectacular performances that was! We were all enthralled by the atmosphere of that evening, and I am honored to be a part of the medical and scientific community, working with patients in our Center, to help further our understanding in the causes of ALS and to improve patient care.

The event was in honor of Geraldine Oldak and Lucy Ann Branstron.

Thank you.
Amy Chen, MD





Multidisciplinary Center Planning Tool

At the Multidisciplinary Center we are always looking for ways to provide optimal care for our patients. We are aware that our Tuesday clinic visits can be quite long and tiring and we want to make sure all patients and caregivers have their needs met and all their questions answered prior to leaving. In an effort to provide the best care for our patients, we are providing patients with a list of topics at the beginning of their clinic visit. Here patients may indicate issues that are most pressing and need to be addressed. With this tool, we can assure that the patient see the appropriate clinicians at the beginning of their visit. At the end of the visit we will ask patients and caregivers to let us know if further follow-up is needed.

Please let Gabriela Harrington-Moroney, RN know if you have any questions:
gharrington@neuro.columbia.edu

ALS in Korea

There is no available official data showing the number of the patients suffering from ALS. According to a 2005 report, about 1300 patients were struggling with this condition.

The evaluation process and care for ALS patients in Korea are basically similar to those in the US. Doctors, most often neurologists, and allied health professionals devote themselves to help patients. Most of the diagnostic tests and drugs are also available.

Patients usually receive their care in a regular neurology office setting. In the past, and even today, patients have frequently remained in the hospital for long-term if they have respiratory

issues or advanced weakness. Because multidisciplinary care centers like the Eleanor team approach clinics are now launching. and Lou Gehrig MDA/ALS Center here at Columbia have many advantages, these kind of of team approach clinics are now launching. A lot of research, both laboratory and clinical, is on-going, but clinical trials are rare.

American patients and family members seem more actively involved in their care and in important decision making. Issues of quality of life are more seriously considered. End-of-life decision making is discussed earlier and more openly. More ALS patients receive permanent mechanical ventilation than those in America. In the same context, most hospice programs in Korea do not yet fully realize the needs of ALS patients.

Definitely, caring for ALS patients is such a big burden on the family. At present, the Korean government's health and welfare program and National Health Insurance give support to ALS patients and families in many ways. Once diagnosed, patients are eligible for disability benefit and benefit from rare diseases. These include medical expenses, visiting nurse service, equipment for transport, ventilator, home aid service, etc.

There is an ALS patients' organization for communicating with each other, exchanging information, and getting support. The community support for ALS patients such as donations, volunteering, and public education is just beginning and not fully established, so more steps need to be taken for more effective care for ALS patients.

Woo-Kyung Kim
Department of Neurology
Kangdong Sacred Heart Hospital
Hallym University College of Medicine
(a research fellow at Columbia's ALS Center)



International MND/ALS Conference in Toronto

In November the ALS center staff went to Toronto, Canada for the 2007 International MND/ALS conference. Our patient Sid and his wife Felicia hosted a cocktail party at their home where we were introduced to the ALS disciplines of Toronto's Sunnybrook Health Sciences Centre. It was a great opportunity to sit and converse about our mutual goals in finding the cure for ALS and listening to their various experiences. We returned to our ALS center with new information to feed our research and patient care. Sid has also been actively fundraising to create better communication among the ALS clinicians, which would increase the efficiency of our research trials and patient education. Thank you Sid, your hope and enthusiasm is an inspiration to all!



(from left to right)
Ronit Gorelik, PT, Winston Cheng, Gabriela Harrington, RN, Felicia, Sid, Darleen Vecchio, Kate Bednarz, RD

Current Clinical Trials

Actively enrolling:

- Modafinil for Fatigue in ALS Exercise and Oxidative Stress in ALS
- Genetic and Epidemiology Study
- Non-Invasive Ventilation

Ongoing (closed to enrollment):

- Clinical Trial of High Dose CoQ10 in ALS-stage II Early Treatment of ALS with Nutrition
- Longitudinal Study in Cognitive Impairment
- Ambispective case-control study of oxidative stress

Enrolling Spring/Summer 2008:

- Clinical Trial of Ceftriaxone in Subjects with ALS
- Study Arimoclomol in ALS
- Dextromethorphan/Quinidine (DMQ) in the Treatment of Pseudobulbar Affect in ALS

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





The Eunice Harris ALS Gift Fund.

Eunice died at home on September 30, 2004 after a five-year battle with ALS. Soon afterward the idea was put together at the Runner's Edge running store in Farmingdale Long Island to see if we could have a fund drive in her memory. The idea quickly caught on. Because Eunice was so well loved in the running community on Long Island, runners gladly came forward to contribute to the drive. The raffle was set up and prizes were given by the Runner's Edge and other stores and individuals, with the raffle taking place at the store just before Christmas. Her two sons regularly attend the raffle drawing and also give generous donations to the fund. While this was going, on I put forward the idea that we should sell raffle tickets at the Massapequa Road Runner's Club and the Long Island Road Runner's Club combined Christmas/Holiday party which has proved to be very successful. Following are two statements which explain that although Eunice is no longer with us, she is the force that drives the fund. From her son Andrew: "As the youngest son of Eunice Harris it gives me great pleasure to see so many people coming out annually to support this cause in my mother's memory. She touched so many lives and I am proud to be her son. I would personally like to thank everyone involved in making this a successful event year after year" From the Long Island Road Runners Club: "Our members loved Eunice as if she was a member of our own families and that is why we give so generously and freely."

- Colin Harris



From the Desk of Dr. Kaufmann

I am very pleased to report the progress of our trial entitled "Clinical Trial of High Dose Coenzyme Q10 (CoQ10) in ALS". Thanks to your help and support, we were able to surpass our goals in terms of patient participation here at the Eleanor and Lou Gehrig MDA/ALS Center as well as at the other sites that are participating throughout the US. Clinical trials are a collaborative effort between researchers, clinicians and patients. Without patient and caregiver support and their efforts in taking the study medication and coming to the center for visits, clinical trials would not be possible.

Clinical trials are an important step in bringing new treatments to patients. Patients and clinicians cannot be sure if a new medication is truly beneficial if it is not tested in a trial. Also, the FDA will not allow the use of a new medication for ALS without controlled trials.

Many patients have asked us why this trial is called a "Phase II" trial. Clinical trials are divided into three phases: Phase I trials typically have less than 20 participants, often healthy volunteers, who first take a new medication at different doses to make sure that it is safe. Phase II trials are the next step, typically have less than 200 participants and are there to find out if there is some evidence that the new medication is beneficial. Phase II trials cannot definitely answer the question if a new drug is truly effective. However, they can tell us if a new drug is

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From Dr. Kaufmann's Desk (from page 5)

worthwhile pursuing in a Phase III trial. A Phase III trial is a definite trial to test a new drug's efficacy. It typically has several hundred participants and takes several years. The FDA usually requires two positive Phase III trials to approve a new drug.

The clinical trial of CoQ10 in ALS was based on encouraging results from a pilot study conducted by Sheila Hays at the Eleanor and Lou Gehrig MDA/ALS Center and Dr. DiMauro, an expert in mitochondrial disease and CoQ10. Dr. Mitsumoto and I, working with statisticians here at Columbia University and ALS centers around the country, have followed up on the pilot study with a Phase II clinical trial, funded by the National Institute for Neurological Disorders and Stroke. CoQ10 is a promising treatment for ALS because it is a mitochondrial co-factor and powerful antioxidant. Mitochondria are the "powerhouses" of the cell, and they are thought to have impaired function in ALS. Free radicals that can be neutralized with antioxidants are also thought to play a role in ALS.

The trial will be completed in late spring of this year. With the results, we will be able to answer the question if it is worthwhile pursuing CoQ10 as a treatment for ALS in Phase III. The trial will not definitely answer the question if CoQ10 is actually beneficial in ALS. Only a Phase III trial can show this. You may ask why we did not start a Phase III trial right away. The reason is that Phase III trials are very large, expensive and take a long time. When there are several new drugs on the horizon, it is an efficient strategy to test some of them in Phase II so that we can have at least preliminary answers more quickly and without using up all the resources.

Some patients who have already completed their follow-up time in the CoQ10 trial have asked us why we cannot tell them right now what type of medication they have been taking. The reason is that in a controlled trial, not only the patients but also the investigators are "blinded", meaning that they don't know if the patients was taking CoQ10 or placebo. We can share the treatment assignment with patients once the analyses are completed which we anticipate to occur in mid-April.

We want to again thank everyone for their support and their contributions to this trial. Most of all, we want to thank patients and caregivers who have participated and generously given their time and effort. Clinical trials can not only benefit those who directly participate, but they can help others with ALS. Clinical trials allow us to rationally test treatments, eliminate harmful or ineffective treatments rapidly, and, hopefully, find an effective and safe treatment for ALS soon.





The Eleanor and Lou Gehrig MDA/ALS Research Center

MDA's Holiday Party

By: Jackie Puerta

The holiday spirit came to the MDA Neuromuscular Center at Columbia, as MDA families gathered to celebrate the holidays. On Saturday, December 15th, the families came together to take part in celebrating the Holidays. It was a very cold day, but that didn't stop the families from attending the event. The day was filled with party games, caroling, dancing and some surprises!

The kids had a wonderful time competing in the caroling contest. Parents enjoyed meeting each other and conversing. To top of the celebration...Santa's helpers dropped off some cool gifts for everyone in attendance, which brought smiles to the children's faces. Michele Galioto, from the MDA Manhattan team states, "Since the holidays are a time for family and friends to connect, it's a wonderful opportunity for everyone in the MDA family to celebrate together."

MDA would like to give thanks to Dr. Mitsumoto and Olena Jennings for making arrangements and providing the space for the Holiday party. In addition, a special mention and gratitude to D'Agastino's Supermarkets for donating the wonderful refreshments and making this occasion a memorable one!





The Eleanor & Lou Gehrig MDA/ALS Research Center
 Division of Neuromuscular Diseases
 Columbia University Medical Center

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

ALS Center Holiday Party

On Wednesday, December 12, the MDA/ALS Research Center hosted its annual holiday party to thank the wonderful people who help them throughout the year. They gave special thanks to respiratory therapists Bill Simonds and Madeleine Ogust, social worker Ruth Anne Rubenstein, volunteer Meredith Pasmantier, the Brace Shop, the Irving Center for Clinical Research, Tech Support, and many others. We wish everyone a Happy New Year!

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MDA/ALS Educational Support Groups:

New York City

All Souls Unitarian Church
 1157 Lexington Avenue
 (between 79th & 80th)
 New York City
 First Floor
 Please join us
 Fourth Monday of each month
 6-8 p.m.
 For more information contact
 Jacqueline Puerta: 212-689-9040

Northern New Jersey

Jewish Community Center
 on the Palisades
 411 E. Clifton Avenue
 Tenafly, NJ 07670
 Please join us
 Third Thursday of each month
 4-6 PM
 For more information contact:
 Maywood Center: 201-843-4452

Westchester

Burke Rehab Center
 (Clock Tower Bldg, Rm 202)
 785 Mamaroneck Ave
 White Plains, NY
 Please join us
 Second Thursday of each month.
 6-8pm
 For more information contact
 Gloria English: 914-345-5062

Long Island

St. Charles Hospital
 200 Belle Terre Road
 Port Jefferson, NY 11777
 Please join us
 First Friday of each month
 4:30- 6:30 PM
 For more information contact
 Deidre: 631-582-7761

Editor

Olena Jennings



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