ALS Support Group of NW WI

A Gathering of Individuals Touched by ALS
Share Joy, Sorrow, Laughter, Tears, and Hope.

Receiving a diagnosis of ALS is challenging and can be very overwhelming.
The ALS support group provides a safe place where patients, families, friends, and caregivers Gather to share information, support, and resources with others who understand.

Second Thursday of each month, 1:00pm – 3:00pm At Chippewa Valley Bible Church in Chippewa Falls

Support Group March Meeting Notes:

March support group meeting was attended by nine people. We discussed the ALS WALK coming up June 9th for our newcomers. Also discussed the article on ALS, "Hard-nosed reporter faces her final deadline", that was in Leader Telegram March 10th & book coming out Tuesday March 18th , titled "Until I Say Goodbye". One person who has been attending for eleven years gave a brief history of how our group got started.

Upcoming events:

Monsignor Klimek Lecture Series Light a Single Candle Sunday, April 28 6pm Florian Gardens. LeAnn Thieman, nationally acclaimed inspirational speaker, is also the co-author of 12 "Chicken Soup" books, including Chicken Soup for the Nurse's Soul and Chicken Soup for the Caregiver's Soul.

9th Annual ALS Care and Research Symposium

April 20,2013 Sheraton Milwaukee Brookfield Hotel

375 South Moorland Road

Brookfield, WI 53005
(Next to Brookfield Square Mall)
Registration must be received by April 8
Phone Janet Gauger at 262 784 5257 or register online at:
www.alsawi.org

Misc. articles provided by ALS Association Monthly Connections.

Ask the Doc: by Edward Kasarskis, M.D., Ph.D

Edward Kasarskis, M.D., Ph.D. is Director of the multidisciplinary ALS Center at the University of Kentucky Neuroscience Center in Lexington, Kentucky, professor in the Department of Neurology at the University of Kentucky, and Chief of Neurology at the VA Medical Center in Lexington KY.

What You Should Know About Joining a Clinical Trial

Q: I'm thinking about joining a clinical trial testing a possible new medication to treat ALS, but I'm not sure I like the idea of potentially being put on just the placebo drug. What should I know or consider?

A: This is a common question that comes up with every single trial -- why do we need a placebo group? Everyone goes into a clinical trial with high hopes that the drug being tested is going to be better than the placebo, an inactive drug. But that's not always the case. Sometimes after the study is completed, it turns out that the test drug actually made patients worse. So the people who actually do the best can end up being the ones who are taking the placebo. For example, several years ago there was an important clinical trial of Lithium for ALS, but it turned out it was actually harmful to some ALS patients.

So what exactly is a clinical trial? You have to remember that it is simply a scientific experiment. Although clinical trials are based on a great deal of previous research, they are experiments in humans to prove that a test drug either helps, or does not help real people with ALS. Many of the drugs have been tested with success in animals, or in smaller observational studies in people. When you give a test drug into a large number of people with ALS, you don't know for certain if it is safe or if it will effectively treat the disease.

Keep in mind that the people who work to develop effective drugs for ALS do so with great commitment and significant expense. Everyone is highly motivated to figure out a treatment that works. But to determine that a drug is effective, a randomized double-blinded clinical trial remains the "gold standard."

Let me explain what that means. A randomized study means that once you meet the criteria for being included in the clinical trial, you are placed in either the placebo group or a group getting the test drug under evaluation. Typical inclusion criteria might be age, sex, other medical conditions, how long you've had ALS, or your muscle strength and breathing capacity.

The decision of "drug or placebo" is made solely by chance almost like flipping a coin. The research pharmacist is the only person who knows what each research participant is getting. Nothing in the drug's container or packaging will convey whether it is a placebo or the active drug -- they will look identical.

When a study is double blinded, it means neither the patient nor the research team knows what treatment the patient is taking (either placebo or active test drug). The double blind ensures that all people involved in the trial from the patient and the family, to the people administering the drug, to the trial leaders will be completely unbiased as they record drug side effects or potential improvements (in areas such as grip strength, or breathing capacity, for example). Without having a "blinded" system, staff would naturally be rooting for the person taking the active drug, hoping for a big discovery. And that could affect the quality and objectivity of the results.

In a clinical trial, patients are asked to take the medication faithfully according to directions and report any side effects they might have. It's a big responsibility, especially considering patients and their families know that they may be doing all this and still just taking a placebo.

Developing successful new drugs for any condition can be very frustrating. Most studies are based on rock-solid preliminary data and are flawlessly done, but at the end of the day the test drug simply may not work. That was the case with the recent trial of Ceftiraxone.

Researchers know that patients and families typically only have one shot at a drug trial and they understand how frustrating it can be when a trial fails. But it may help to know that just participating in a clinical trial provides a positive psychological benefit. It gives participants a true sense of purpose since they are making a contribution to the global understanding of ALS. We always learn something.

The scientists may gain new insights into which classes of drugs might work for ALS, or could get information that helps them better understand how the disease works.

Although the slow march to new findings can be frustrating, it's important to remember that all our understanding of science has been extremely incremental. I make this point with medical students and residents all the time. The amount of effort it takes to learn one solid fact in clinical medicine is monumental. It's rare that there are big paradigm shifts or major insights from any single study or experiment.

To better understand this, consider how many iterations the concept of a cell phone has gone through. Watch the early James Bond movies and see the "portable" phones they were using. Those clunky machines have now evolved in everyday life into little computer-phones you can put in your pocket.

When it comes to developing new drugs for ALS, the pace of progress is also incremental. So while I am not satisfied with the pace of drug development, I encourage everyone with ALS to participate in any type of research dealing with the disease. We should all pay attention to the lyrics of the song from West Side Story, Something's Coming--"...the air is humming, and something great is coming!" In ALS, this won't happen without you.

RESEARCH

Study Discovers How Gene Mutations Cause ALS and Other Brain, Muscle and Bone Diseases

March 3, 2013

As published in the scientific journal Nature, researchers funded by The ALS Association have discovered how mutations in new genes for ALS cause not only that disease but also other diseases of the brain, muscle and bone. These results also reveal the disease pathways involved in ALS due to other genes and may prepare for the development of new treatments to interrupt these processes.

The researchers found that mutations in genes for certain RNA-binding proteins cause them to switch between alternate shapes and aggregate and to promote the same conformational change and aggregation of the normal protein. This behavior has been seen in other neurodegenerative diseases, collectively called prion diseases, including mad cow disease and Creutzfeldt-Jakob disease. In those diseases, this ability leads to spread of the disease throughout the nervous system.

“This discovery may lead us to think more broadly about how ALS progresses within the brain and to ask whether a similar spreading process is occurring,” said Lucie Bruijn, Ph.D., Chief Scientist for The Association.

The team also found that mutations in proteins called heterogeneous ribonuclear proteins (hnRNPs) caused an inherited disease in a small number of families with symptoms of ALS, the frontotemporal dementia, the muscle disease inclusion body myopathy, and the bone disorder Paget’s disease of bone. This cluster of symptoms has recently been recognized as a unique disorder called multisystem proteinopathy. The mutations increased the tendency of the hnRNP proteins to clump together and to induce non-mutated forms of the protein to do so as well. hnRNP proteins normally link to another ALS-associated protein called TDP-43, and the two were found together in the aggregates.

“While these mutations are themselves a very rare cause of ALS, they may provide an important clue about how other forms of ALS spread over time,” Dr. Bruijn said. “Preventing protein aggregation may be a viable therapeutic approach for many forms of ALS.”

The study was led by J. Paul Taylor, M.D., Ph.D., of the Department of Developmental Neurobiology at St. Jude Children’s Research Hospital in Memphis, Tennessee and colleagues from the United States and Europe. These researchers received funding through The ALS Association’s Translational Research Advancing Therapies (TREAT ALS™) program, which funds a diverse portfolio of research at leading institutions all over the world.

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Take good care.
Julie Chamberlain, LPN
ALSNWWI Patient Services Outreach
715.271.7257
alsnwwi@gmailcom