January 2013

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 January Meeting Notes:

Our first meeting of 2013 was attended by eight people. Some members had traveled over the Holidays & some are gone for the winter. We discussed traveling without your spouse for the first time & the extra effort of traveling with a person who has ALS. We exchanged photos & greetings. We also discussed the pros & cons of the ' Sit to Stand' & the 'Hoyer Lift'. Also how difficult it can be to function with equipment in the bathroom since they are almost always too small.
We talked about losing a loved one to ALS. Whether the loss was recent or years ago missing that special person remains.
We had a new person join the group who has Ataxia, joining an existing Ataxia patient so they had much in common & a lot to talk about and were both glad to meet!

Upcoming events:

Next support group meeting: February14, 2013 at Chippewa Valley Bible Church

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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

***Ask the Doc:* Advice About Caregiving**

Monthly "Question and Answer" with Dr. Kasarskis -- The topic this month is, "Advice About Caregiving" Read the full story.
http://web.alsa.org/site/PageServer?pagename=ALSA_Ask_January2013

Q: I'm taking Rilutek now but am wondering what else might be on the horizon in terms of potential drugs for ALS?

A: That's a great question. There are several high-potential drugs being tested right now through clinical trials.

By the way, the drug you're taking, Rilutek (riluzole) has been around for about 20 years now. You should know that the American Academy of Neurology's 2009 Practice Parameters say that the drug is effective in slowing the rate of progression of ALS. Some physicians may downplay the benefits and potential impact of Rilutek, but it's important to know that it does play a role in treating ALS. It is not a perfect drug, and so the quest for additional drug treatment continues.

When thinking about the future of drug therapy for ALS, most experts agree that the most effective treatment will likely be a "drug cocktail," combining two or more medications, rather than taking just one. This may ultimately be our best strategy in treating the disease.

There are a large number of clinical trials in progress right now testing drugs, stem cells, and gene therapy that could change the course of the disease and preserve or improve muscle strength. Here's a quick update:

- **Dexpramipexole by Biogen Idec:** This drug looks promising. It is believed to work by increasing the efficiency of mitochondria the power houses of cells in motor neurons.

  The drug is a three-dimensional mirror image of a current treatment for the symptoms of Parkinson's disease (Pramipexole). The chemical difference in structure may make "Dex" less likely to have unwanted side effects that some people with Parkinson's experience with the parent drug. A small Phase 2 trial of the drug in 102 newly-diagnosed people with ALS showed improvement in functional capability and survival.

  A Phase 3 trial with almost 1,000 people enrolled should be completed very soon.

  If, and this is always a BIG IF, this drug proves successful and is approved, it's likely that it would be taken along with Rilutek.

- **Ceftiraxone:** We just learned a few of months ago that a clinical trial was halted because the drug proved to be ineffective in a pivotal Phase 3 trial. This was a complicated and very long study, and was particularly disappointing since there was a lot of good scientific support for this approach. I know I speak for all the members of the study teams in thanking the patients and their families for their devotion to getting an answer. It was truly an amazing effort lead by Drs. Cudkowicz and Shefner, and supported by the National Institutes of Health.

- **CK2017357 (Tirasemtiv) by Cytokinetics:** This drug increases a muscle's strength when it contracts and might decrease fatigue. It has the greatest impact on what is described as mid-level exertion, such as walking, reaching and talking. CK2017357 appears to reduce fatigue and improve function in people with ALS. This drug, now in a Phase 2 trial, would also be used with other medications. It looks pretty promising.

- **NP001, by Neuraltus Pharmaceuticals:** Results from a Phase 2 clinical study of NP001 were released this fall (on the eve of Superstorm Sandy, as it turns out, so you probably didn't read about it) and just reported at the International ALS/MND Symposium in Chicago last week. The study showed that 27% of patients who received this drug had no progression of their disease during the six-month period they were taking the medication. That means that twice as many people experienced no disease progression as compared to those who were taking a placebo (an inactive drug). The trials were conducted at 17 sites across the country, involving a total of 136 patients. People were randomly assigned to get a low dose, a high dose or a placebo. We participated in both the Phase 1 and Phase 2 trials of NP001 at the University of Kentucky.

Dr. Bob Miller, the principal investigator of the study, described the study results as "encouraging" in a Neuraltus press release: "Halting the rate of disease progression, in a subset of patients, as this study suggests, would translate into a clear clinical benefit for these patients," he said. I agree completely. Unfortunately, rather than being an oral drug, the medication is given intravenously over several days at a time, for a period of months. The next step: a more definitive Phase 3 trial is likely to be started in 2013.

RESEARCH

Updates on Two Clinical Trials: NP001 Phase III and Tirasemtiv Phase IIb

On October 30, Neuraltus Pharmaceuticals announced that it is planning a Phase III clinical program to continue to study of its drug, NP001, in people with ALS. According to the company, its Phase II clinical study demonstrated positive trends in slowing the rate of disease progression, and NP001 was found to be safe and well-tolerated. The data will be presented at the 23rd International Symposium on ALS/MND in Chicago this December.

On October 29, Cytokinetics, Incorporated, announced a Phase IIb clinical trial to continue studying it drug, tirasemtiv, formerly known as CK-2017357, in people with ALS. It has opened BENEFIT-ALS (Blinded Evaluation of Neuromusclar Effects and Functional Improvement with Tirasemtiv in ALS), formerly known as CY 4026, to enrollment.

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Take good care.

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