Clinical trials are the most important step in the development of new therapies in ALS. Ideally, a new agent will be brought to trial rapidly once it has been tested in animal models, and the trial will be conducted by clinicians who are experts in the field. Fostering those ideal conditions, in order to maximize the chances of success for today’s ALS patients, is the joint mission of The ALS Association’s TREAT ALS™ Translational Research Advancing Therapies for ALS initiative and the Northeast ALS Consortium (NEALS).

NEALS is an ever-expanding network of ALS clinicians who conduct clinical research in ALS, co-chaired by Merit Cudkowicz, M.D., of Massachusetts General Hospital and Harvard Medical School and Jeremy Shefner, M.D., Ph.D., of Upstate Medical University in Syracuse, New York. Dr. Cudkowicz spoke about the NEALS/TREAT ALS™ partnership in an ALS Association webinar.

“It has been a pleasure to build a partnership with NEALS to develop the clinical trials network,” said ALS Association Chief Scientist Lucie Bruijn, Ph.D., who hosted the webinar. “Dr. Cudkowicz and her team have been critical in advancing trials in ALS.”

From its start in 1995 with only nine centers, all in the northeast, NEALS has grown to encompass more than 100 centers across the United States and Canada and is even developing a partnership with a European ALS network to carry out joint clinical trial efforts.

“Our mission is to have a group of centers with trained personnel to bring therapy forward as quickly as possible,” Dr. Cudkowicz said. “We want to make the time from identification of a potential therapy to a clinical trial not the two years that is standard in medicine, but two to three months. Drug companies tell us they have never seen a field this ready for clinical trials.”

A critical part of the success of NEALS has been the funding support of TREAT ALS™ Dr. Cudkowicz said. The TREAT ALS initiative is a targeted approach to accelerate clinical trials of the most promising new agents in ALS. A major beneficiary of TREAT ALS funding has been the NEALS program to train and support young investigators in becoming ALS clinical trial investigators.

“Using TREAT ALS™ finding, we have been able to attract and train the ‘best and the brightest’ young physicians to study ALS,” Dr. Cudkowicz said. “There is really nothing else like this in neurology.” NEALS currently trains 10 new investigators every year, who then become part of the research network for new drugs. “The number of drugs coming out of the pipeline is quite large, and we need more researchers.”
There are currently 11 active studies being conducted with others recently completed and more in the planning stages. “This shows what a little infrastructure support can do for a group with a good mission,” she said.

Recent positive studies were a trial of the Cytokinetics drug CK-2017357, which improved muscle strength without affecting disease progression, and the Knopp drug dexpramipexole, which appeared to be associated with a slowing of disease progression in a 12-week, blinded Phase II trial in 100 patients. Both drugs are now slated to be tested in larger, Phase III trials, also organized by NEALS.

Results from the dexpramipexole study and from an ongoing trial of ceftriaxone are due in fall of 2012. Other ongoing studies are to examine the potential of turning off the SOD1 gene in patients with SOD1 mutations and to search for biomarkers that may help diagnose and track the disease.

Work by NEALS, supported by TREAT ALS, has “lowered the bar to get drugs into trials,” Dr. Cudkowicz said, and has also improved the ability to generate “high-confidence data” in those trials. “We also share all our results, so there is no redundancy of efforts, and so protocols for new trials can be improved.”

“This really shows the role of collaboration in helping to move things forward,” Dr. Bruijn commented. “The Association is pleased to be able to partner with NEALS to accelerate the pace of clinical trials.”

The ALS Association recognized the important need to better communicate to patients about clinical trials and acknowledged that the current websites describing available trials are complicated to search and often not updated. To address this, the association has partnered with NEALS who is revamping its website (www.alsconsortium.org) which will be directly linked to the ALSA site to be a center for clinical trial information. In addition, the association is supporting a clinical trial expert in partnership with NEALS who is on call to answer questions by phone or email. Contact information:

By phone at 877-458-0631
9 a.m. to 5 p.m. Eastern time, Monday through Friday
Or any time by email at alstrials@partners.org

“Don’t hesitate to call or email us!” Dr. Cudkowicz said.

The full-length presentation is also available by click here or following this link. https://alsa.webex.com/alsa/ldr.php?AT=pb&SP=MC&rID=64036262&rKey=cc519456cecdc877