CReATing New Opportunities for Clinical Research in ALS
Research Webinar: July 22, 2015
Speaker: Michael Benatar, M.D., Ph.D., of the University of Miami
Host: ALS Association Chief Scientist Lucie Bruijn, Ph.D., M.B.A.

By Richard Robinson

A new initiative to advance understanding of ALS and accelerate clinical trials is underway, as outlined in a recent webinar. The initiative, called CReATe (Clinical Research in ALS and related disorders for Therapeutic development), is being led by Michael Benatar, M.D., Ph.D., of the University of Miami, and Richard Bedlack, M.D., of Duke University. Dr. Benatar outlined the structure, goals, and current efforts of CReATe during the webinar, hosted by ALS Association Chief Scientist Lucie Bruijn, Ph.D., M.B.A.

The consortium is funded by the National Institutes of Health, with additional funding from The ALS Association. It stands out for its focus on ALS and “related disorders,” including frontotemporal dementia (FTD), primary lateral sclerosis (PLS), progressive muscular atrophy (PMA), and hereditary spastic paraplegia (HSP). Each of these disorders differs in some respects, but all involve degeneration of motor neurons, the hallmark of ALS. Bringing together scientists and clinicians who are studying these diseases is a goal of CReATe.

The current major thrust of CReATe is to study “genotype-phenotype” relationships in ALS and the related disorders. “Phenotype,” Dr. Benatar explained, “refers to the clinical manifestations of the disease,” such as age of onset, site of onset, and rate of progression. “Genotype is the set of genes possessed by the person,” including genes that may directly cause the disease (such as the C9orf72 gene that causes ALS and FTD), or those that influence some aspect of the phenotype.

By studying the relationship of genotype and phenotype in people with ALS or related conditions, Dr. Benatar and colleagues hope to better understand differences between various types of ALS, and to use this information to design and test better therapies.

For instance, if their research shows that people with a specific genotype are likely to progress more slowly than others, it may make sense to design a clinical trial around a group people expressing slow disease progression. This would allow any disease-slowing effects of treatment to be distinguished from the influence of the genotype.

Biomarker discovery is the second major thrust of the CReATe consortium. “A biomarker is something we can measure that tells us about the disease,” Dr. Benatar said. “Biomarkers can be important for diagnosis, prognosis, and monitoring treatment.” For instance, a blood test that determined how quickly the disease is progressing could be more responsive to a disease-modifying therapy than the current “gold standard,” the ALS Functional Rating Scale. That could allow clinical trials to be smaller and faster, speeding the testing of new treatments.
CReATe includes a growing network of clinical sites across the country, a “genetics core” that handles the genotyping portion of the project, and patient advocacy groups that help spread the word and facilitate study enrollment. The CReATe website http://www.rarediseasesnetwork.org/cms/create contains up-to-date information on trials and other studies, as well as useful articles about basic concepts and new research. There are also resources for researchers.

A simple but powerful way to help advance ALS research is to sign up at the CReATe site on the contact registry. The sign-up process, which asks basic questions about your health, takes less than 30 minutes. By signing up, you can contribute directly to increasing the understanding about ALS. But more importantly, by providing your contact information, you can learn about research opportunities appropriate for you as they arise. Your privacy is protected, and you may choose to withdraw at any time. The contact registry is distinct from the National ALS Registry, Dr. Benatar noted, although they are complementary.

Getting people with ALS involved in clinical research is central to the mission of CReATe, Dr. Benatar said. “Despite remarkable participation by people with ALS in research studies, enrollment is still lower than expected. Only through research can we learn more about these diseases and make progress toward effective therapies.”

Unfortunately, due to a technical issue, Dr. Benatar’s presentation did not record properly, and thus it is unavailable for replay.