New Initiatives Funded by the Ice Bucket Challenge
Push ALS Research Forward

Research Webinar: December 9, 2014
Host/Speaker: ALS Association Chief Scientist Lucie Bruijn, Ph.D., M.B.A.

By Richard Robinson

The extraordinary outpouring of support for ALS research during the Ice Bucket Challenge has provided The ALS Association with an unprecedented opportunity to advance the understanding and treatment of ALS on multiple new fronts. In a recent webinar, ALS Association Chief Scientist Lucie Bruijn, Ph.D., M.B.A., outlined the first four initiatives to be initiated.

Dr. Bruijn provided an overview of The Association’s Translational Research Advancing Therapies (TREAT ALS) program, which encompasses all aspects of research on ALS. “The TREAT ALS program translates laboratory findings into new treatment strategies, as well as providing infrastructure for clinical trials,” she said, as well as helping to build national and international consortia for major projects.

“For some programs that were already in the planning stages or in progress, the new funds will allow us to move forward in an expedited way.” The Association has sponsored a series of workshops and planning meetings, including a recent roundtable meeting with funded investigators, to help shape calls for proposals in 2015.

Genetics is a major focus of ALS research, as genes are believed to contribute to both familial ALS (where more than one family member is affected) and sporadic ALS, where no familial pattern is observed. A new gene discovery (of which there have been gratifyingly many in recent years) “starts to help identify targets, which is the beginning of therapy development,” Dr. Bruijn said. Such discoveries also increase the interest of an ever-larger number of scientists in coming into the field, as more newly-linked cellular mechanisms means overlap with more branches of research.

The four new initiatives include:

The New York Genome Research Center
This new research center is dedicated to applying genomics—the study of the entire set of genes each person has—to understanding neurodegenerative diseases, including ALS. Funding from the national ALS Association, as well as the Greater New York Chapter, will promote not only sequencing of patient DNA but also analysis of new clues that arise regarding the causes of the disease.

Project MinE
Project MinE is an international collaborative effort to sequence the genomes of 15,000 people with ALS and 7500 control subjects. Researchers from more than 15 countries are involved, and funding from The Association is dedicated to bringing United States resources into the coalition. “The higher the number of patients and controls analyzed, the more likely we will be to identify the genes involved in all forms of ALS,” Dr. Bruijn said.

The Neurocollaborative
This new collaboration will combine the efforts of three major labs dedicated to ALS: those of Don Cleveland, Ph.D., Steven Finkbeiner, Ph.D., and Clive Svendsen, Ph.D. Dr. Cleveland will further develop antisense treatments for stopping genetic forms of ALS. Antisense blocks the expression of mutant genes, such as SOD1 or C9orf72. Dr. Finkbeiner’s automated neuron tracking system is a powerful tool for testing the effects of new treatments on individual neurons, offering an unprecedented look at the effects of a drug on these cells. Dr. Svendsen will develop a stem cell facility, or “core,” to create, analyze, test, and distribute stem cells for ALS researchers.

ALS Accelerated Therapeutics (ALS ACT)
ALS ACT is a partnership with Massachusetts General Hospital and GE Health, with a matching $10 million donation from ALS CURE, to speed clinical trials in ALS. Efforts include development of imaging tools that can be used to track biomarkers in trials, funding for pilot trials that use biomarkers, further development of a tissue and biofluid repository for research into biomarkers, and development of the Global Unique Identification (GUID) system to assign a unique patient-specific identifier to every sample and clinical record, in order to allow better integration of data from multiple trials or clinics.

“This is just the beginning,” Dr. Bruijn said. In 2015, efforts will include ramping up the development and distribution of better mouse models of the disease, which remain critically important for research. New studies in the clinical management of ALS are also on tap to improve treatment of people living with the disease. TREAT ALS will also continue to fund the best new ideas coming in from investigators, as well as funding young researchers through the Milton Safenowitz Fellowship program. “Their ideas and enthusiasm are crucial for bringing us into the future of ALS research,” Dr. Bruijn said.