ALS Biomarker Discovery and Validation

Miami, FL (September 25th, 2015) – The CReATe Consortium and the ALS Association are pleased to announce the selection for funding of two new projects that will advance the discovery and validation of biomarkers relevant to ALS therapy development.

The **CReATe** (**C**linical **Re**search in **A**LS and related disorders for **T**h**e**rapeutic development) is a Rare Diseases Clinical Research Consortium (RDCRC) that forms part of the National Institutes of Health (NIH) Rare Diseases Clinical Research Network (RDCRN). A major goal of the CReATe Consortium is to foster the discovery and validation of biomarkers relevant to therapy development for patients with ALS and related disorders. In an exciting partnership with the ALS Association, CReATe is able to advance this goal through the release an annual RFA to fund biomarker pilot projects. CReATe also fosters biomarker development by making its substantial repository of biological samples available to the broader scientific community.

One of the projects funded this year will be lead by Dr. Adrian Isaacs, a Reader (Associate Professor) in the Department of Neurodegenerative Disease at University College London. The goal of this project is to develop a single molecule detection assay for the dipeptide repeat proteins (DPRs) generated by the ALS- and FTD-causing repeat expansion in *C9orf72*. Isaacs and colleagues will employ a methodology that is capable of single molecule detection and has sensitivity up to 1000-fold higher than current ELISA approaches. Using this approach, they hope to detect these DPRs in the blood of patients with C9orf72 ALS/FTD. Successful completion of this project would represent a major advance, as a simple blood test could then be used to inform how well a drug is working, greatly simplifying the assessment of new therapies with this ALS or FTD caused by mutations in the C9orf72 gene.

The principal investigator on the second project is Dr. Benjamin Murdock, a Research Investigator in the Department of Neurology at the University of Michigan. Dr. Murdock's project will build on his laboratory's prior observation that populations of innate lymphoid cells are increased in the blood of ALS patients. He hopes to better define the role of these cells in ALS, by tracking specific innate lymphoid cell populations in individual patients over the course of a year. He and his colleagues will also determine whether these cells express higher levels of pro-inflammatory signals - which may exacerbate disease. Demonstrating that innate lymphoid cells are associated with disease progression will allow these investigators to use these cells to both track and predict disease progression. One hope for the future is that this research may permit alteration of innate lymphoid cell function using novel or existing therapeutics to extend the lifespan of patients with ALS.

"the ALS Association is extremely excited to partner with the CReATe Consortium in supporting these efforts to develop biomarkers that will be so essential to our collective efforts to identify effective therapies for this group of disorders" commented Lucie Bruijn, PhD, the Chief Scientist of the ALS Association.

For more information about the CReATe Consortium, please visit www.RDCRN.org/CReATe or follow us on Twitter @CReATeRDCRN.