Researchers at the University of South Florida have discovered novel biomarkers associated with Amyotrophic Lateral Sclerosis.

Amyotrophic Lateral Sclerosis (ALS) is a debilitating neuromuscular disorder. Given the prevalence of ALS worldwide, and the severity and rapidity of the disease, development of therapies for ALS is of paramount importance. The sporadic form of the disease is most prevalent and has no clear risk factors associated with it. Presently, only one compound, riluzole, is approved for treatment of ALS. While an understanding of the mechanism of the disease will allow the identification of novel drug targets, the discovery of biomarkers, which may predict the risk of developing ALS, would be beneficial in early detection. Identification and validation of biomarkers for ALS will facilitate early detection of the disease and make it more amenable to pharmaceutical intervention.

Our inventors have discovered novel biomarkers for ALS detection. A SILAC-based study using ALS fibroblast cells and age-matched control fibroblast cells was performed. SILAC stands for Stable Isotope Labeling by Amino Acids in Cell Culture, and is an innovative technique for studying mass spectrometry-based proteomics. Upon data analysis we found 36 proteins that were differentially regulated in ALS fibroblasts compared to normal control cells. Among these proteins, 19 proteins were up-regulated and 17 were down-regulated in ALS compared to control. Based on literature search 26 proteins were found to be novel. We have validated 3 novel proteins: apolipoprotein B48 (Apo B48), fibulin-1, and Hsp20 based on the availability of reliable antibodies for Western blotting. Validation data confirms that Apo B48 and Hsp20 are down-regulated, and fibulin-1 was found to be up-regulated in ALS fibroblasts in comparison to the normal cells. To our knowledge, there are no current reports of these validated proteins in relation to ALS biomarker use. Overall, our data revealed several novel biomarkers that can be used for development of unique screening kits for ALS detection and also can be used as novel drug targets.

Technology Advantages:
- Early Detection of ALS
- Identify Targets for Drug Therapy
- Screening kit for ALS

Figure: Comparative analysis of ALS Biomarkers using human ALS and control fibroblasts. Expression of Apo B48, fibulin-1 and Hsp20 in normal control and ALS patient fibroblasts. Western blot analysis shows a decrease in Apo B48 (left) and Hsp20 (middle), and an increase in fibulin-1 (right) in ALS patient fibroblasts.

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