Proteostasis Therapeutics Announces Scientific Publication in Cell Reports

CAMBRIDGE, MA--(Marketwired - November 06, 2014) - Proteostasis Therapeutics, Inc., a company developing novel therapeutics that regulate protein homeostasis to improve outcomes for patients with orphan and neurodegenerative diseases, today announced the publication of its pioneering research study in the field of aging.

This study entitled "A Chaperome Sub-Network Safeguards Proteostasis in Aging and Neurodegenerative Disease" was published in the Nov 6 issue of Cell Reports.

Aging is the most significant and universal risk factor for developing neurodegenerative diseases, such as amyotrophic lateral sclerosis (ALS) and Alzheimer's, Parkinson's and Huntington's diseases. This risk increases disproportionately with age, but no one really knows why.

Now a team of scientists from Proteostasis Therapeutics, Inc., Northwestern University and Harvard University has uncovered some clues. The researchers are the first to find that the quality of protective genes called molecular chaperones declines dramatically in the brains of older humans, both healthy and not, and that the decline is further accelerated in humans with neurodegenerative disease.

Chaperones are a class of proteins encoded by a special set of highly conserved genes that watch over cells, keeping them, and the entire organism, healthy by preventing protein damage. If this critical system declines it leads to misfolded and damaged proteins, eventually leading to tissue damage and death. In keeping the chaperones healthy, we may be able to keep a person healthier, even in old age.

The researchers specifically found a decline in 100 genes, corresponding to approximately one-third of all human molecular chaperone genes. Then, with additional studies, they winnowed that number down to 28 human genes specifically involved in age-associated neurodegeneration. These critical genes provide a basis for a biomarker, an early indicator of disease and a target for new therapeutics.

To zero in on the subnetwork of 28 key genes, the scientists combined genomic analysis of human brain tissue, from both healthy individuals and those with neurodegenerative diseases (Alzheimer's, Parkinson's and Huntington's), with functional studies of C. elegans, a transparent roundworm. (The worm is a popular research tool in neurobiology and has provided many insights into human disease.)

Meenu Chhabra, President and CEO of Proteostasis Therapeutics commented that "this pioneering work is particularly notable because it has been able to distill out only 28 key genes from the vast human genome of 25,000 genes and elegantly demonstrate their connection with the aging process. Using our expertise in protein biology we will now be able to delve into the molecular basis for the decline in the encoded chaperones proteins, with the long term goal of developing chaperone targeted therapies to prevent their age associated decline. Preventing or reversing age related neurodegenerative processes is one of the highest unmet medical needs and this research brings us one step closer to the solution and we thank all the scientists involved in this study for their dedication and groundbreaking work."

The primary co-authors of this study are Drs. Marc Brehme and Cindy Voisine. The senior and corresponding authors are Dr. Marc Vidal from Harvard medical School, Dr. Hui Ge, formerly at Proteostasis Therapeutics, and Dr. Richard Morimoto from Northwestern University, who is also a co-founder of and scientific advisory board member at Proteostasis Therapeutics. No funds from the company were used in Dr. Morimoto's laboratory at Northwestern University.

About Proteostasis Therapeutics
Proteostasis Therapeutics is developing disease-modifying therapeutics for cystic fibrosis, genetic diseases and neurodegenerative diseases. The Company's technology combines both phenotypic and target based drug discovery to develop therapeutics that modulate protein homeostasis pathways and correct for imbalances in protein folding, trafficking, and clearance. For more information, please visit www.proteostasis.com.

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