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PRESS RELEASE

Amyloidosis Foundation

Awards 2014 Research Grant Winners

November 25, 2014: The Amyloidosis Foundation is pleased to introduce three researchers who were recently awarded grants by our Board of Directors. A monetary award of $50,000 is provided to each of the junior awardees and $100,000 to our senior research grant winner. Our foundation supports patients, family members, physicians and caregivers who are battling this rare disease.

Amyloidosis occurs when amyloid proteins deposit and accumulate in the body's organs and tissue, ultimately causing organ failure if the deposition is not stopped. Treatment includes stem cell transplant, chemotherapy and possible organ transplants. If diagnosed early and treatment is swift, many patients live long, happy lives.

The Amyloidosis Foundation is a nonprofit organization in Clarkston, MI that was started over ten years ago by Don Brockman and his wife Mary O'Donnell when Don was first diagnosed with Amyloidosis. He passed away ten years ago this summer and every day Mary and her team continue to raise awareness to fund research and support those affected by this disease. The primary purpose of the foundation in funding scientific research is to advance our mission and support research for all types of amyloidosis. Our goal is to increase education and awareness of amyloidosis and support research towards a cure.

Guillermo A. Herrera, MD—Senior Research Grant Kidney involvement and eventual kidney failure are common in amyloidosis. If the kidneys fail, the only options are dialysis and transplantation, both of which can cause significant complications. Today, amyloidosis is diagnosed earlier resulting in prolonged survival. The need for repairing damaged organs is obvious. This grant investigates the use of stem cells administered intravenously to repair/heal damaged kidneys in an animal model. Stem cells are undifferentiated and can transform into specialized cell types. Results from this study will pave the way to use stem cells in human patients with amyloidosis to improve survival and quality of life. Dr. Herrera works at LSU in Shreveport, LA.

Jennifer Kollmer, MD —Junior Research Grant A severe impairment of the peripheral nerves is one of the main manifestations in hereditary amyloidosis (TTR-FAP). Our grant will develop a new and highly sensitive diagnostic tool **1)** for the detection of very early nerve damage in gene-carriers without symptoms (family members of patients with already symptomatic disease), and **2)** for the monitoring of nerve lesions in patients with symptomatic amyloid polyneuropathy under treatment. Our study of participants will be done in different groups of disease severity. We will then perform a MRI of the lower limb peripheral nerves to find out which have the highest sensitivity in detecting peripheral nerve damage and determining a certain stage of polyneuropathy. This will hopefully lead to earlier treatment after initial diagnosis and successful therapies for patients in advanced disease. Dr. Kollmer works at the University of Heidelberg in Germany.

Rockland L. Wiseman, PhD—Junior Research Grant The systemic amyloid diseases are a group of diseases caused by the build-up of unstable proteins that form toxic tangles in the blood. These tangles accumulate on organs such as the heart, gut, and kidney, leading to organ failure and ultimately death. Currently, no treatments besides invasive surgery exist to treat the majority of these diseases, making systemic amyloid diseases a large, unmet medical need. We are developing new strategies to reduce the lethal accumulation of unstable proteins associated with systemic amyloid diseases by enhancing the natural, protective pathways that regulate the levels of unstable proteins in the blood. Our establishment of this strategy will demonstrate that a single therapeutic approach can be used to treat many different systemic amyloid diseases, and therefore that it may be possible to use a single drug to broadly treat these devastating disorders. Dr. Wiseman works at The Scripps Research Institute in La Jolla, CA.