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***FOR IMMEDIATE RELEASE***

**ANNOUNCING 2017 FUNDING FOR ALPORT SYNDROME RESEARCH**

[August 17, 2017 - Phoenix, Arizona] – The Alport Syndrome Foundation (ASF), Pedersen Family, and The Kidney Foundation of Canada (KFOC) Research Funding Program is pleased to announce that joint funding has been awarded for two new research projects on Alport syndrome, a rare genetic kidney disease.

“We are pleased with the response and quality of research which the program has attracted and the progress which is being made in finding new drugs to further delay the progression of this disease. Previous projects have led to one drug in second year clinical studies and another project in early stage testing showing encouragement,” said Harold Pedersen on behalf of the Pedersen Family. Elisabeth Fowler, National Director of Research for The Kidney Foundation agreed. “We are encouraged by the quality of the research projects received through this research funding opportunity. The Kidney Foundation of Canada is pleased to continue this wonderful partnership with the Alport Syndrome Foundation and the Pedersen Family,” she said. “We hope that the results of these projects will generate new knowledge that will lead to better treatments and an improved quality of life for people living with Alport syndrome.”

Alport syndrome is a rare genetic kidney disease. Without treatment, affected individuals experience a decline in kidney function. The majority of these patients require dialysis and/or a kidney transplant, usually between their early teens and their 40s. In addition, many people with the syndrome develop severe hearing loss that requires hearing aids. The hearing loss often begins in early adolescence. Eye changes are also found in a smaller percentage of those affected. An Alport syndrome diagnosis devastates families because it frequently affects multiple family members across generations. There is currently no treatment proven to prevent the development of kidney failure in people with Alport syndrome; however, early diagnosis is essential as there are medications to delay the progression of the kidney disease. Current research is advancing the knowledge of treatments which could potentially further delay the onset of kidney disease while work continues on a cure.

Research projects awarded this year by the Alport Syndrome Foundation, Pedersen Family and the Kidney Foundation include:

- Dr. Alessia Fornoni of University of Miami (Florida) was awarded \$100,000 for a 15-month study on ***Targeting podocyte lipotoxicity in Alport Syndrome***. In Alport syndrome, mutations in type IV collagen genes lead to abnormal collagens being expressed in kidney basement membranes. These abnormal collagens activate receptors called DDR1 and CD36 that lead to uptake of fatty acids and cholesterol into podocytes, an important cell attached to the basement membrane, and injury to this cell. This research project aims to understand this injury pathway further through studies in cell culture and also aims to prevent the progression of kidney disease in mice with Alport syndrome by blocking the uptake of fatty acids and cholesterol into podocytes by treatment with a currently FDA-approved drug, Ezetimibe.

Dr. Constantinos Deltas of University of Cyprus (Cyprus) was awarded \$100,000 for an 18-month study on *Repurposing of FDA approved chemical chaperones to the rescue of a mouse model of Alport syndrome*. In some patients with Alport syndrome a missense mutation leads to abnormal folding of the type IV collagen protein and breakdown of the protein before it gets to the basement membrane. This research project aims to use chaperone medicines called PBA and TUDCA to help the abnormally folded type IV collagen make it to the basement membrane intact in a mouse model of Alport syndrome. Having any type IV collagen in the basement membrane, even if it has a minor missense mutation, is hypothesized to improve the long-term kidney outcome of the disease.

For full abstracts on these studies, please go to the ASF [website](#).

ASF and its funding partners have provided over \$2 million for Alport syndrome research and have funded researchers at the Washington University (Missouri), Saban Research Institute/Children's Hospital Los Angeles, University of Melbourne (Australia), Monash University (Australia), University Medical Centre Göttingen (Germany), University of Toronto (Canada), and Kumamoto University (Japan) in addition to funding the patient registry (ASTOR) at the University of Minnesota.

“Our vision is to conquer Alport syndrome by preventing kidney failure and hearing loss in all patients,” said Sharon Lagas, Board President of ASF. “We are accomplishing this by working with our funding partners and collaborating with the international community to build a research agenda that will lead to novel treatments for Alport syndrome patients.”

#### **About the Alport Syndrome Foundation**

ASF is the leading independent nonprofit organization in the United States serving and giving a voice to the Alport syndrome community. ASF's mission is to improve the lives of patients through education, empowerment, advocacy and research to realize the vision to conquer Alport syndrome. For more information, please visit [www.alportsyndrome.org](http://www.alportsyndrome.org).

#### **About the Pedersen Family**

The Pedersen Family of Calgary, Alberta is the primary funder of Alport syndrome research in Canada. The Family prefers projects with early impact on the treatment of Alport syndrome such as the repurposing of existing FDA approved drugs which can substantially reduce clinical trial time. Research funding is mainly done through the KFOC to select researchers in collaboration with the Alport Syndrome Foundation. Support is also given to the patient registry program (ASTOR) in Minneapolis.

#### **About The Kidney Foundation of Canada**

*Kidney health and improved lives for all people affected by kidney disease* - for over 50 years, this vision has guided The Kidney Foundation of Canada to be a collaborative, inventive and focused leader in the development of programs, services, research opportunities and awareness campaigns that have had a positive impact on the millions of Canadians living with, or at risk of developing kidney disease. For more information, visit [www.kidney.ca](http://www.kidney.ca).

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