



DATE: March 18, 2021

TO: All Media Outlets

RE: Grant Award, Proof-of-Concept Gene Therapy

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With gratitude for the patients, families, and friends that support our Research Program, Alport Syndrome Foundation is pleased to announce a 2021 funding award of \$132,000 to Washington University's Division of Nephrology in the Department of Medicine. These funds were awarded to Dr. Jeffrey Miner's laboratory to perform proof of concept gene therapy studies in Alport mice carrying transgenes that allow induction of collagen 345(IV) expression at any age. These studies will determine the ages and stages of kidney disease at which promising gene therapy approaches such as CRISPR would be effective in patients with Alport syndrome. In light of his innovative and impactful research in nephrology, earlier this month, Dr. Miner was named the inaugural Eduardo and Judith Slatopolsky Professor of Medicine in Nephrology at Washington University.

According to Dr. André Weinstock, Volunteer Chair of ASF's Research Program, *"The recent discovery of the highly efficient gene-editing mechanism CRISPR has prompted significant attention and investment toward its potential use as a curative therapy for monogenic diseases. Alport syndrome is the second most prevalent monogenic disease of the kidney. Therefore, it is important for the Alport community to understand, as soon as possible, if this pathway should be further explored."*

Although CRISPR may offer an efficient way to edit a person's DNA, several challenges must be overcome to achieve a viable CRISPR-based therapy for any monogenic disease patient. Alport Syndrome Foundation assembled a team of eight scientific researchers, the core of an inaugural Scientific Advisory Research Network, to align on a strategic roadmap to investigate whether CRISPR is a viable curative pathway.

The researchers were selected based on their history of productive collaboration, expertise in Alport syndrome pathology, being principal investigators of laboratories with diverse Alport syndrome research capabilities, and dedication to the Alport syndrome community. These include: Dr. Billy Hudson, Vanderbilt University (TN, USA); Dr. Hirofumi Kai, Kumamoto University (Japan); Dr. Ron Korstanje, The Jackson Laboratory (ME, USA); Dr. Rachel Lennon, University of Manchester (England); Dr. Jeff Miner, Washington University in St. Louis (MO, USA); Dr. Mary Nabity, Texas A&M (TX, USA); Dr. Kandai Nozu, Kobe University (Japan), and Dr. Laura Perin, University of Southern California (CA, USA). Additionally, the group is joined by Dr. André Weinstock of the ASF Board of Directors as a coordinator of the group's efforts.

Based on consensus of this highly qualified network of researchers, Alport Syndrome Foundation will begin this research with the work of Dr. Jeffrey Miner's laboratory, building on his initial exploration of this question in 2014. Alport Syndrome Foundation is strategically investing in this research to determine the feasibility of harnessing new technologies used in other diseases to potentially unlock a cure for Alport syndrome.

For questions, contact: info@alportsyndrome.org.