Research Program Impact 2022

Investment in research is central to the mission of Alport Syndrome Foundation (ASF) and has consistently been the largest part of our annual budget for more than a decade. To date, ASF has invested $2,006,600 in seed funding for Alport syndrome genetic, pathologic, modeling, and therapeutic research with laboratories and clinics around the world, including the United States, Canada, Europe, Japan, and Australia.

This seed funding for fundamental research has led to a direct return of 11 times the initial investment. ASF research awardees have collectively secured more than $22 million in additional outside funding from public/private sources to expand their Alport syndrome research.

ASF's Research Program is adding to our understanding of possible pathways for treatments or a cure, shining a light on awareness of Alport syndrome in the research community, attracting innovative minds to study this rare disease, and helped transform the landscape to an active and engaged research community.

We are proud to note that the ASF Research Program has helped build a network of international researchers that have come to know each other and to work collaboratively. The work and activity of the Research Program has brought researchers and patients together in person, proving to be remarkably empowering for both.

In 2021, ASF established a new Scientific Advisory Research Network (SARN) comprised of nine U.S. and international Alport syndrome researchers focused on fundamental research. The role of this group is to provide expert guidance to ASF on how to best achieve targeted research objectives. The group is addressing topics such as:

- Viable genetic curative pathways for Alport syndrome patients. Research guided by the SARN and funded by ASF in 2021 directly led to a new clinical trial exploring a nonsense mutation read-through therapy for Alport syndrome patients with this type of mutation.
- Improving the understanding of the molecular biochemistry of the collagen IV molecule in the glomeruli of the kidney.
- Better understanding of the molecular biochemistry of the collagen IV molecule in less studied comorbidities (hearing, eyes, pregnancy) across all genotypes and phenotypes.

It is a hopeful and productive time for Alport syndrome research. There are therapies being developed now to improve treatment and further postpone the need for dialysis and kidney transplantation. It is our belief that with continued efforts over time, a cure is possible.

In 2022, ASF shifted its available research funding toward the collection of human data, a critical component in supporting translational research from animal models to humans. With fundamental research more well-funded than in the past from other sources, it is important to fill this knowledge gap for the design of clinical trials, and to support the regulatory process for new therapies. ASF is funding the NKF Patient Network – Alport Syndrome, a patient registry to collect patient data over time. We are also funding a new natural history study, including the collection of family history and bio-samples from Alport patients in the U.S., with NEPTUNE at the University of Michigan. These research-focused efforts are our highest priority at this time.