



Date: May 15, 2025
For more information contact: info@alportsyndrome.org
Web: www.alportsyndrome.org

The Alport Syndrome Research & Regulatory Workshop, organized and sponsored by Alport Syndrome Foundation (ASF), was held May 8-9, 2025 in Boston. The workshop brought together leading experts in this rare genetic kidney disease to review the status of research and clinical studies, and to begin shaping clear regulatory guidelines for drug development.

The 35 workshop participants included members of ASF's [Scientific Advisory Research Network](#) and [Medical Advisory Committee](#), additional researchers exploring novel biomarkers for the disease, representatives from the U.S. Food and Drug Administration (FDA), National Institutes of Health (NIH), and Kidney Health Initiative (KHI), as well as patients and caregivers, including ASF staff and board members.

Dr. Mark Lim, Vice President of Research and Discovery for the American Society of Nephrology noted, "It is exciting to see the Alport Syndrome Foundation, a patient organization providing education and support for patients and invested in Alport syndrome research, taking the leadership to catalyze the development of the first on-label treatment for their community."

The two-day workshop focused on:

- Experiences and needs of individuals living with Alport syndrome
- The importance of genetic testing for diagnosis and for clinical trial recruitment
- Clinical endpoints and potential surrogate endpoints to prove the efficacy of a treatment effect that will meet the needs of patients
- Currently available data supporting clinical and surrogate endpoints that are required to demonstrate that a treatment effect will meet the needs of patients of all ages
- Plans for international collaborative efforts to collect and analyze data to identify clinical and surrogate endpoints
- Investigation of potential novel biomarkers in Alport syndrome
- Updating the 2020 *Kidney International* article, "[Clinical trial recommendations for potential Alport syndrome therapies](#)," resulting from a 2018 ASF-led workshop

To support the current pharmaceutical pipeline and attract other innovators to Alport syndrome drug development, ASF is committed to leading these time-sensitive efforts and to partnering with the FDA, KHI, workshop participants, and other institutions and patient organizations across the world.