

Autosomal Recessive Polycystic Kidney Disease (ARPKD) Adjunct EL-PFDD Scientific Workshop Report

Executive Summary

The mission of the Polycystic Kidney Disease (PKD) Foundation is to fund research, advocate for patients, and ultimately to end PKD. On August 29, 2023, the PKD Foundation hosted the Autosomal Recessive Polycystic Kidney Disease (ARPKD) Externally Led Patient-Focused Drug Development (ELPFDD) Meeting. As a critical follow up to this meeting, the ARPKD Adjunct EL-PFDD Scientific Workshop was held on January 23, 2024. This workshop gave ARPKD clinical and research experts an opportunity to first reflect on what was shared by the ARPKD community, then to identify important scientific priorities and data needed to move the field forward. ARPKD is a rare genetic disease, characterized by progressive decline in kidney function and progressive changes in liver/bile duct structure and function. ARPKD's rare disease status and clinical variability have limited the development of targeted therapies for this disease. As a result, the burden of ARPKD is profound, not only for those living with the disease but for their entire families.

[Read the publication](#)