From Data to Hope: C-Path's Polycystic Kidney Disease Outcomes Consortium and the Power of Data Sharing

Every year on September 4, families, patients, advocates, and researchers come together to recognize **Polycystic Kidney Disease (PKD) Awareness Day**. While Critical Path Institute (C-Path) and its Polycystic Kidney Disease Outcomes Consortium (PKDOC) strive for advocacy and awareness every day, it's still an important time to recognize those living with PKD and the unique health concerns this inherited disease can cause.

PKD is a genetic disorder that causes fluid-filled cysts to grow in the kidneys. Over time, these cysts multiply and enlarge, often leading to kidney failure. It's one of the most common genetic diseases, bringing life-threatening conditions to millions worldwide. Yet behind these numbers are stories—parents balancing care with daily life, young adults managing their health while building their futures, and families determined to create a better tomorrow.

How PKDOC Supports the PKD Journey

At C-Path we uphold the principle that regulatory science must serve the people behind the data. That's why in 2010, we launched the Polycystic Kidney Disease Outcomes Consortium (PKDOC). Our mission has always been simple, but urgent: to lead academic research centers, pharmaceutical companies, patient advocacy organizations, and international regulatory agencies, such as the FDA and EMA, to accelerate drug development for those living with PKD.

What makes PKDOC unique is its collaborative spirit and distinct status as the ecosystem's neutral convener. Instead of working in isolation, this public-private partnership brings together patient voices, researchers, and regulators to build tools that make clinical trials faster, more efficient, and more meaningful for patients. By

pooling data from thousands of individuals with PKD through the PKDOC Database, the consortium has been able to create a clearer picture of how the disease progresses, helping regulatory scientists design better studies and give families hope that breakthroughs are within reach.

Real Impact for Real Lives

The positive impact of PKDOC has been felt by the PKD community for the last 15 years. Together with our valued partners, we have brought valuable initiatives and actions forward, including:

- **Using Modern Tools to Predict the Future**: By advancing total kidney volume (TKV) as a way to track disease progression, PKDOC gave researchers and clinicians a powerful tool. For patients, this means shorter, smarter clinical trials and faster access to potential new treatments.
- **Powering a Global Collaboration**: PKDOC creates a space where everyone—patients, doctors, scientists, and regulators—work collaboratively. This ensures that the challenges patients face every day are at the heart of every decision.
- **Being the Neutral Convener that Shares Progress**: Perhaps most importantly, PKDOC's work doesn't stop at one project or one drug. Every tool and standard developed is shared with the entire research community. That means every patient who participates in a study is contributing to something bigger than themselves—helping to move the whole field forward.

Looking Toward Tomorrow

For many people living with PKD, the journey is filled with uncertainty. But thanks to the dedication of patients who share their stories, families who advocate tirelessly, and researchers who innovate boldly, the path forward is brighter than ever. At C-Path and within PKDOC, we are honored to walk alongside this community, transforming personal stories of struggle into collective momentum for change.

On **Polycystic Kidney Disease Awareness Day**, we celebrate resilience, honor those we've lost, and commit ourselves once again to the fight. Together, we can turn awareness into action—and action into a future where PKD no longer defines lives, but instead becomes part of history.