Two years in: the development and basic characteristics of a national, patient-powered registry in ADPKD

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ADPKD REGISTRY
Powering a Cure

For more information, visit
pkdcure.org/registry

Background

- **Autosomal dominant polycystic kidney disease (ADPKD)** is one of the most common, life-threatening genetic diseases. Fluid-filled cysts develop and enlarge in both kidneys, eventually leading to kidney failure. Nearly 50 percent of affected individuals reach end stage kidney disease (ESKD) in their 6th decade of life, and ADPKD is the 4th leading cause of ESKD in the U.S..
- **The PKD Foundation** is the only organization in the U.S. solely dedicated to finding treatments and a cure for polycystic kidney disease (PKD) and to improving the lives of those whom it affects.
- With multiple new therapeutics in development for ADPKD and clinical trials enrolling participants, there is a call for increased ADPKD patient participation in research to support these efforts. In addition, there remains a large need for an improved understanding of the disease's impact on quality of life through longitudinal collection of outcome measures.

The ADPKD Registry

- **The ADPKD Registry** is a national, online collection of U.S. patient-reported data related to ADPKD, launched in September of 2019.
- The purpose of the Registry is to **simultaneously facilitate research discoveries while addressing patient needs**:
  - To support patient-centered outcomes research to learn more about the patient journey and discover unmet needs.
  - To aid in the development of new therapies by connecting likely eligible patients with enrolling clinical trials.
  - To capture quality of life and other patient-reported outcomes on a standardized platform.
- The significance and relevance of the Registry design, implementation, and management are maintained through the **activities of Advisory Committees and Working Groups** consisting of clinician scientists, researchers and patient stakeholders.
- The Registry is hosted on a secure, online platform; participants are registered and consented through the online system.

IQVIA

ADPKD-IS and ADPKD-PDS contact information and permission to use: Mapi Research Trust, Lyon, France, https://eprovide.mapi-trust.org

Methods

Participants are asked to fill out a series of longitudinal modules about their ADPKD. In the past year, we've added three new modules.

Core Questionnaire
Demographics and current disease status

Family History
Diagnosis and ESKD status for all family members

Diet and Lifestyle
Current diet and exercise

ADPKD-Impact Scale
Physical, emotional and fatigue disease-related impact

ADPKD-Pain and Discomfort Scale
Burden of dull pain, sharp pain and discomfort related to PKD

Experience with Liver Cysts
Symptoms and complications attributed to liver cysts

NEW
Vascular Outcomes
History of brain, chest or abdominal aneurysms
- Method of screening and diagnosis
- Size and treatment methods
- Family history

NEW
COVID-19 Impact
- Latest COVID-19 test and case details if diagnosed
- How medical care changed due to challenges/concerns surrounding the pandemic

NEW
Health Care Access
- Types of clinicians managing their disease
- Challenges affording or accessing medications, procedures, dialysis and transplant services, and other medical costs

Modules are released either at three-month, six-month, or annual time points starting at enrollment. New patient-reported modules are designed and implemented utilizing Advisory Committee-directed working groups and patient beta testing panels.

Participants at Enrollment

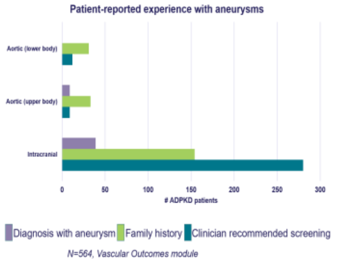
As of September 27, 2021, 1545 ADPKD patients across the U.S. have enrolled in the ADPKD Registry and completed at least one of the available modules.

	Pre-ESKD	On dialysis	Post-kidney transplant
% of participants*	77.1%	3.1%	19.8%
Median age	48	59	62
Median eGFR	48 mL/min/1.73 m^2	<10 mL/min/1.73 m^2	57 mL/min/1.73 m^2

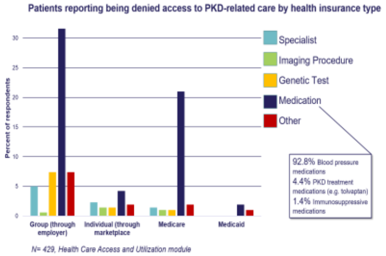
Table 1. Participant cohort ESKD status (N = 1,545)
*2.6% suspect ADPKD but have no formal diagnosis; not included in the above

Features of Enrollees

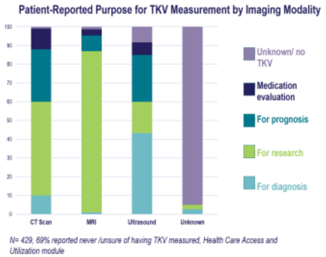
Participants have a median age of 52 years, and are 71% female, 93% Caucasian, with 5.2% self-identifying as Hispanic/Latino and 2.4% as African American. 13.5% had a genetic test for PKD, with a vast majority (94%) reported diagnosis by imaging (70% by ultrasound, 23% by CT and 14% by MRI). **Below are representative examples of the data we've collected.**



N=564, Vascular Outcomes module



N= 429, Health Care Access and Utilization module

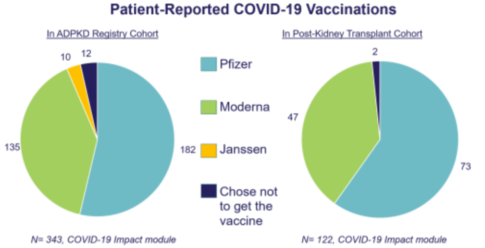


N= 429, 69% reported never /unsure of having TKV measured, Health Care Access and Utilization module

COVID-19 Infection Outcomes by Age

Age Group	20-29 (n=8)	30-39 (n=5)	40-49 (n=16)	50-59 (n=18)	60-69 (n=19)	70-79 (n=2)	80+ (n=2)
Experienced Symptoms, N (%)	5 (63)	5 (100)	16 (100)	17 (94.4)	19 (100)	2 (100)	2 (100)
Treatment method, N (%)							
- Admitted to the hospital	1 (12.5)	0 (0)	0 (0)	**7 (38.9)**	1 (5.3)	0 (0)	1 (50)
- Treated from home	0 (0)	1 (20)	1 (6.3)	1 (5.6)	5 (26.3)	0 (0)	1 (50)
- Recovered at home without outpatient care	4 (50)	4 (80)	15 (93.7)	9 (50)	13 (68)	2 (100)	0 (0)
- n/a no symptoms	3 (37.5)	0 (0)	0 (0)	1 (5.6)	1 (5.3)	0 (0)	0 (0)
Breathing Treatment, N (%)							
- Put on a ventilator	1 (12.5)	0 (0)	0 (0)	2 (11)	0 (0)	0 (0)	0 (0)
- Oxygen through tube under nose	0 (0)	0 (0)	0 (0)	**3 (16.7)**	**3 (15.8)**	0 (0)	1 (50)
- None	7 (87.5)	5 (100)	16 (100)	13 (72)	16 (84)	2 (100)	1 (50)
Acute Kidney Injury, N (%)	0 (0)	0 (0)	**2 (12.5)**	**2 (11)**	**2 (10.6)**	0 (0)	0 (0)
Recovered at time of survey, N (%) *Potential for reporting bias – those who did not recover or died did not complete this module*	8 (100)	5 (100)	15 (93.7)	15 (83)	18 (95)	2 (100)	2 (100)

N= 70; COVID-19 Impact module, all cases reported March 2021 and prior (before access to vaccinations by these individuals)



N= 343, COVID-19 Impact module

N= 122, COVID-19 Impact module

Participant Engagement

Participants are recruited and engaged through PKDF social media and other communications.

A personalized Dashboard is provided on the Registry Portal to show participants select aggregated data.

A quarterly newsletter is released, highlighting what the Registry is learning and what is coming next.

Conclusions

- The ADPKD Registry is a valuable resource through which to engage with ADPKD patients, and to collect patient-reported outcomes and perspectives.
- Underrepresented groups include Black, Asian and Hispanic patients, as well as males. Recruitment efforts over the next year will include strategies to increate representativeness of the cohort
- The Registry began utilizing the self-reported data elements to create cohorts of potentially eligible study participants in August 2020. Thus far, 64% of participants are between the ages of 18-55 with a eGFR above 30 mL/min/1.73m2, which are eligibility criteria for many ADPKD clinical trials. Researchers and industry partners are encouraged to contact the Registry team to aid in recruitment for clinical trials and other outcomes research.

Contact

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Polycystic Kidney Disease