Design and basic characteristics of a national patient 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 (of which ADPKD is the fourth leading cause). Nearly 50 percent of affected individuals reach end stage kidney disease in their 5th decade of life.
- **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 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 outcomes measures.

The ADPKD Registry

- **The ADPKD Registry** is a national, online collection of patient-reported data on ADPKD patients residing in the U.S., 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 is 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.

Core Questionnaire
- Demographics
- Date and method of diagnosis
- Current creatinine and eGFR lab values
- Comorbid conditions
- ESRD status
- Extra-renal manifestations

Family History
Diagnosis, ESRD status, age at ESRD for each of the following:
- Parents
- Siblings
- Grandparents
- Aunts, Uncles on affected parent's side
- Children

Diet and Lifestyle
- Dietary supplements
- History of nicotine, alcohol and caffeine
- Water consumption
- Exercise habits
- Dietary habits
- How these habits have changed over time related to their disease

ADPKD-Impact Scale
Validated patient-reported survey to assess the following impacts of PKD on quality of life:
- Physical
- Emotional
- Fatigue

ADPKD-Pain and Discomfort Scale
Validated patient-reported survey to assess the burden of PKD on quality of life:
- Dull pain
- Sharp pain
- Discomfort

Experience with Liver Cysts
- History of liver cysts
- Issues believed to be due to liver cysts
- Past procedures and current medication

- Coming soon:

Vascular Outcomes **COVID-19 Impact** **Health Care Access**

- 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 15, 2020, 1352 ADPKD patients across the U.S. have enrolled in the ADPKD Registry and completed at least one of the available modules.

	Pre-ESRD	On dialysis	Post-kidney transplant
% of participants*	74.7%	3.5%	19.8%
Median age	46	56	61
Median eGFR	48 mL/min/1.73 m^2	8 mL/min/1.73 m^2	57 mL/min/1.73 m^2

Table 1. Participant cohort ESRD status (N = 1352)
*2% 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 72% female, 94% Caucasian, 4% self-identifying as Hispanic/Latino and 2.4% as African American. 12% had a genetic test for PKD, with a vast majority (94%) reporting diagnosis by imaging (69% by ultrasound, 22% by CT and 13% by MRI). **Below are representative examples of the data we've collected.**

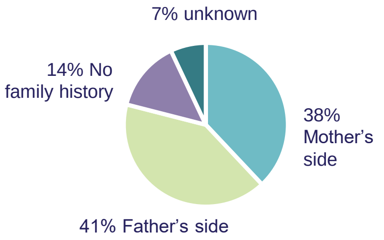
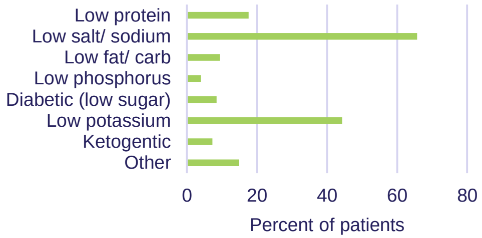
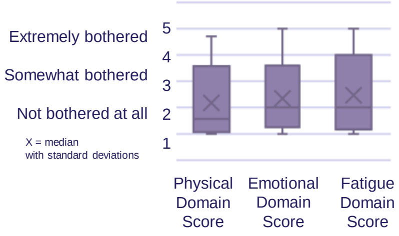


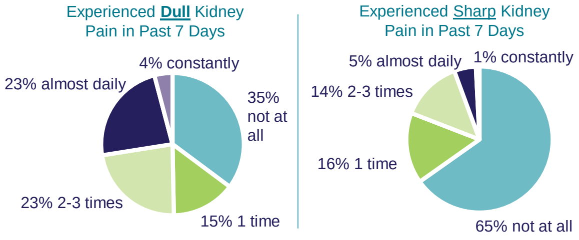
Figure 1: Reported Family History (N= 1,148)



Reported Dietary Restrictions (N= 1,173)



The impact of PKD on three domains of Health-Related Quality of Life (N= 552)



ADPKD-Pain and Discomfort Scale measuring disease-related pain (N= 517)

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 in which to engage with ADPKD patients, and to collect patient-reported outcomes and perspectives.
- Future goals of the project include increasing the diversity of registry participants, creating a process for data access requests, continuation of outcomes module development, and expansion to clinical data sets.
- The Registry began utilizing the self-reported data elements to create cohorts of potentially eligible study participants in August 2020. Thus far, 59% of participants are between the ages of 18-55 with a eGFR above 30 mL/min/1.73m2, which are eligibility criteria for most 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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PKD FOUNDATION
Polycystic Kidney Disease