ADPKD REGISTRY
Powering a Cure

ANNUAL REPORT

2020

Since the PKD Foundation was founded, we show up to work each day thinking about the best ways to impact the lives of patients.

> TOGETHER, THOSE WHO SIGNED UP *for the* REGISTRY ARE CREATING *a* **NEW BODY** *of* **KNOWLEDGE,** *a* NEW SOURCE *of* DATA *for* RESEARCHERS *and* CLINICIANS.

This community is at the heart of our work. Whether that's raising money to fund research, creating educational resources, coordinating support communities, or focusing on finding new ways to speak directly with patients. And now we've created our most patient-centric program yet: a registry that collects important data directly from ADPKD patients. The value of the ADPKD Registry to the work we do is indescribable.

When published research talks about ADPKD, the information is drawn from medical records, insurance claims, and longitudinal studies in the clinic. Together, those who signed up for the Registry are creating a new body of knowledge, a new source of data for researchers and clinicians. The collection of data presented in the following pages highlights the need to collaborate with experts of the disease experience: those living with PKD, watching their family members live with PKD, and those experiencing the changes in quality of life as the disease progresses.

Through the Registry, we can now ask our questions directly to patients on a large scale. This not only better informs our research, education, and advocacy programs, but it allows for patient-powered data to answer the questions of PKD researchers. As we connect the patients in our Registry to clinical studies, we accelerate the work that drives our mission to find treatments and a cure.

One concern we've heard from the PKD community is that of confidentiality and privacy. The Registry asks very personal questions about diagnosis, family history, kidney pain, and other symptoms. We know that this information is confidential, and so the first thing we did when building this program was to find a platform with an excellent reputation for storing data in a way that protects patient information. We've established policies to ensure any data we share with researchers does not include information that could be used to identify an individual, such as name, contact information, or date of birth.

We hope that you feel safe sharing your information with us—the trust of the PKD patient community is so vital to the work we can do together.

In the past, arguably two of the most impactful PKD research projects were the Consortium for Radiologic Imaging Studies of PKD (CRISP) and the HALT Progression of PKD (HALT-PKD) studies. Together, they consisted of almost 1,300 ADPKD patients. We've already surpassed this number with almost 1,400 participants having answered at least one of our modules. And as our program expands, our potential impact on the body of knowledge of this disease will multiply.

Thank you so much to everyone who signed up in our first year! Let's continue to grow and work together to #endPKD.

Chris Rusconi, PhD
CHIEF RESEARCH OFFICER

Elise Hoover, MPH
DIRECTOR OF RESEARCH

About the PROGRAM

On September 4, 2019 (PKD Awareness Day), we launched the ADPKD Registry, a collection of information about individuals with autosomal dominant polycystic kidney disease (ADPKD).

Participation is entirely online: through a phone, tablet or computer. All those diagnosed with ADPKD in the United States are invited to participate.

ANSWER SURVEY QUESTIONS

Answer survey questions about your experience with ADPKD.

KEEP INFORMATION CURRENT

Update us on your quality of life and PKD symptoms throughout the year.

PARTICIPATE IN RESEARCH

Find out about research studies for which you might qualify.

The ADPKD Registry was built by the PKD Foundation with the help of patients, researchers, clinicians, and other members of the PKD community. We are so grateful for their help and dedication to our mission: to find treatments and a cure for PKD.

Learn more

pkdcure.org/registry

> THROUGH *the* WORK *of* THESE INDIVIDUALS, WE'RE PROUD *to* CALL THIS PROGRAM **PATIENT-POWERED.**

ABOUT THE MODULES

When we think about what questions we want to ask in the Registry, we bring together working groups made up of clinicians, researchers, patients, and caregivers. These groups discuss the gaps in knowledge in the clinic, overlooked parts of the disease experience, and research questions that need data to answer. We agree on how to ask these questions and then bring in real Registry participants to test the modules for us and ensure it all makes sense.

Core questionnaire

Pain and Discomfort Scale

Experience with Liver Cysts

Diet and lifestyle

ADPKD Impact Scale

Family history

COVID-19 Impact

> The ADPKD Registry will be the largest collection of patient experiences with their disease that has ever existed. No one knows ADPKD like those of us who live with it and have lived with it through generations in our families. If we all share our information and experience through the Registry, we'll provide direction on what needs to be studied, where the greatest clinical challenges lie, and how to most effectively move forward with vital research into the treatments we all need and deserve.

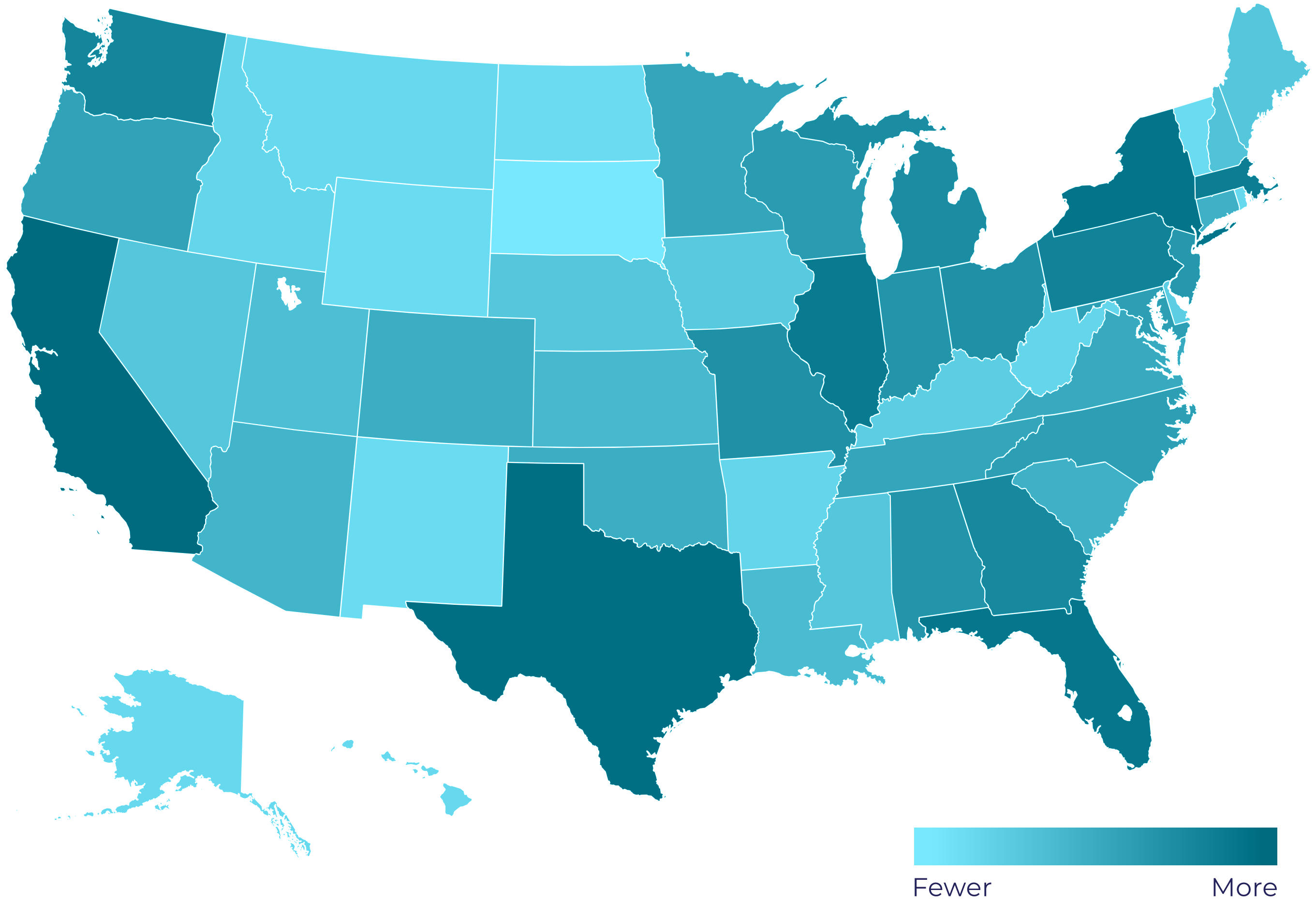
Judy
PATIENT ADVISORY GROUP

About RECRUITMENT

As *of* **DEC. 2020,** THERE WERE **1,807 PARTICIPANTS** ENROLLED *in the* **ADPKD REGISTRY** *from* **ALL 50 STATES** *and the* **DISTRICT** *of* **COLUMBIA.**

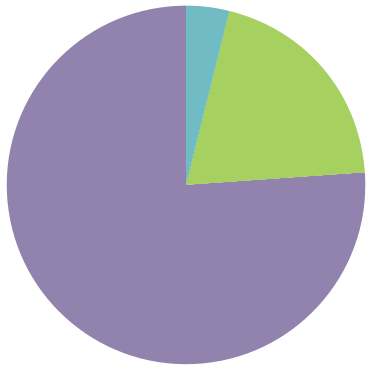
Signing up is easy.

1. Create an online account.
2. Agree to an informed consent.
3. Start completing the available modules!



In 2021, OUR GOAL IS *to* **INCREASE** BOTH RACIAL *and* GENDER **DIVERSITY** *in the* PROGRAM.

PKD STATUS

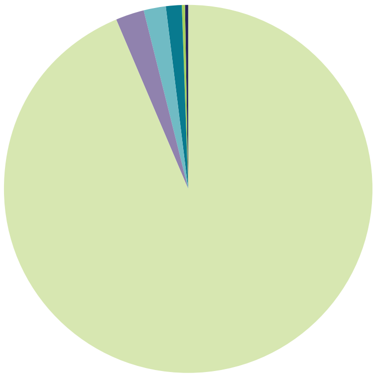


Post-kidney transplant **20%**

On dialysis **4%**

PKD kidneys still functioning **76%**

RACE & ETHNICITY



White **93.8%**

Black or African American **2.4%**

Asian **1.9%**

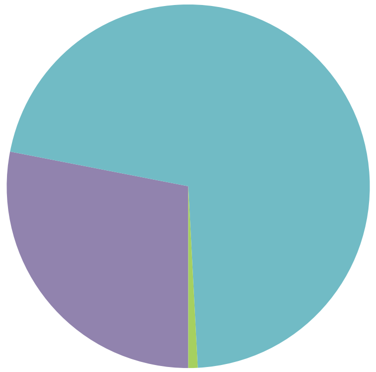
Multiple races **1.4%**

American Indian or Alaska Native **0.3%**

Native Hawaiian or Other Pacific Islander **0.2%**

4% also self-identified as Hispanic or LatinX

GENDER



Female **71%**

Male **28%**

Non binary, transgender, or other **1%**

Julianne
REGISTRY PARTICIPANT

> Having PKD can seem like an awful inheritance. Multiple generations of my family have had to live with fatigue, pain, high blood pressure, and dialysis, and for so long, they did so without hope of a treatment for cure. With this program, my "family" has expanded to a registry of thousands. And by uniting us, we're able to test the research being done and arrive at treatment options faster than my grandfather and mother would ever have imagined possible. Without patient participation, the brilliant minds in labs only have theories. It's when PKD patients become active in trials and help to directly develop better patient care that we all move forward and get closer to a cure.

REGISTRY PARTICIPANT

> When I first heard about the Registry, I didn't think it was for me. I haven't told any of my friends about my diagnosis and don't have time to go back to the hospital for a study. When I read more in a blog about how it's anonymous and online, I finally registered. It's easy to answer most of the questions and they remind you when it's time to answer more.

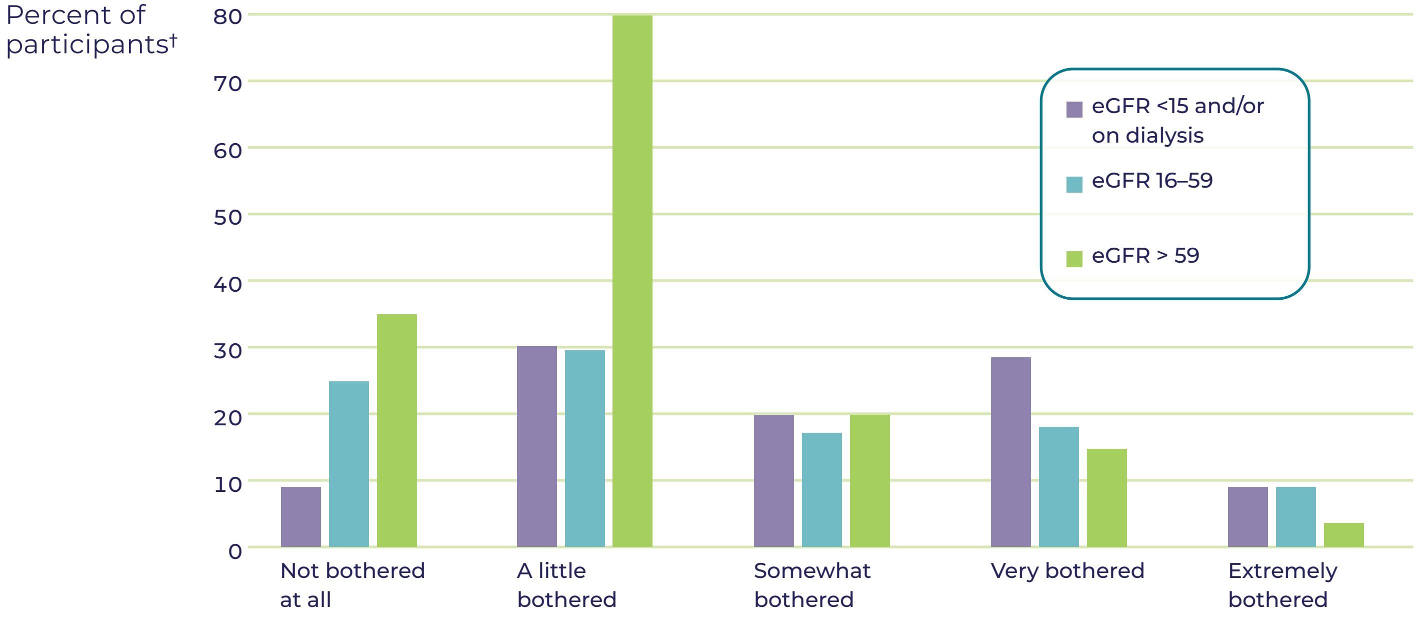
PATIENT REPORTING

QUALITY *of* LIFE

It's difficult to measure the impact of declining kidney function on fatigue without asking patients directly. We used the ADPKD-Impact Scale,* developed specifically for PKD patients, to understand what this looks like.

Learn more
pkdcure.org/living-with-pkd/chronic-pain-management

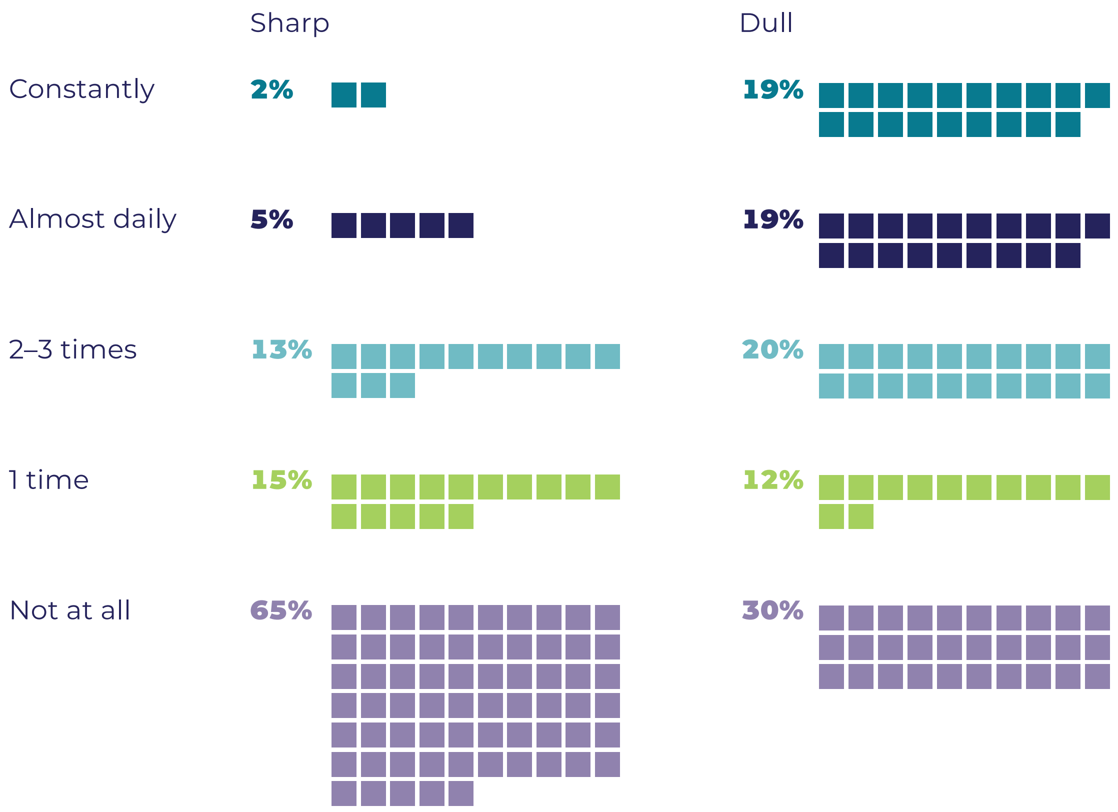
PARTICIPANTS REPORTING FEELING EXHAUSTED OR FATIGUED OVER THE PAST 7 DAYS



† Only those with no kidney transplant

Knowing more about kidney pain will help inform clinicians about the symptoms that impact their patients' daily lives. We used the ADPKD-Pain and Discomfort Scale,* designed specifically for PKD patients, to understand what this looks like. Since these modules only ask about the past seven days, we ask them multiple times to measure pain throughout the year.

FREQUENCY OF KIDNEY PAIN



> Over the last year, my physical condition seems to have worsened, with more abdominal and back pain. I am also not able to eat as much as I used to so I have to work on maintaining a healthy weight.
>
> REGISTRY PARTICIPANT

> I have not been able to exercise anymore due to chronic back pain, excessive fluid, enlarged abdomen, and severe fatigue due to having cystic kidneys and liver.
>
> REGISTRY PARTICIPANT

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

PATIENT REPORTING

FAMILY HISTORY

We know your PKD progression may look different from that of your other family members. We're collecting data to better understand what that means.

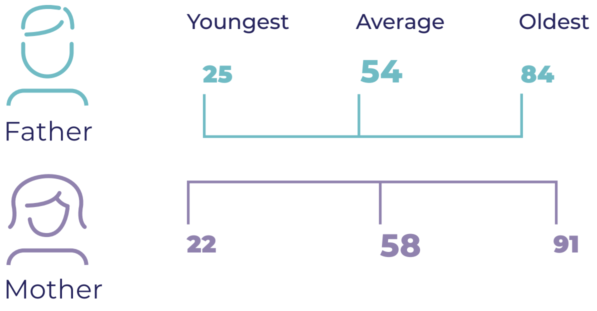
Learn more

pkdcure.org/what-is-adpkd/what-causes-adpkd

PKD INHERITANCE

Unknown **7%**
No family history **13%**
Mother's side **39%**
Father's side **41%**

AGE AT KIDNEY FAILURE



Louise
REGISTRY
PARTICIPANT

> Four generations have navigated unique PKD journeys. My grandfather was never diagnosed, passing away when my mom was a teenager. My mom didn't know she had the disease until her mid-50s when her kidneys failed. She lived 30 extra years with dialysis and a living donor transplant from her sister. I advocated for myself and received a living kidney transplant from my best friend 12 years ago and avoided dialysis, while my sister received a deceased donor kidney three years ago after struggling with dialysis. My niece will need a treatment option for her PKD in the future. We've all navigated our own PKD journeys and continue hoping there will be a cure one day.

PATIENT REPORTING

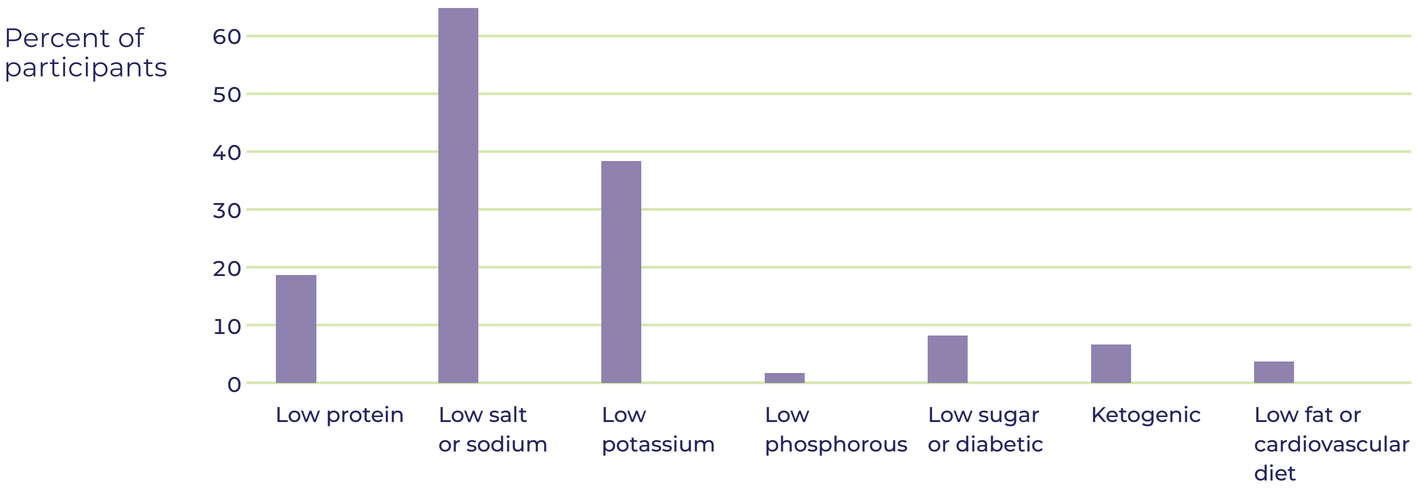
DIETARY CHOICES

There is no specific diet proven to make polycystic kidneys better or keep them from getting worse. However, as patients progress, clinicians may recommend limiting sodium (salt) in the diet to help lower blood pressure or eating foods lower in phosphorus or potassium as kidney function declines. More research is needed to understand the impact of these diets on disease progression and quality of life. *That's where the Registry comes in.*

Learn more

pkdcure.org/living-with-pkd/nutrition

DO YOU FOLLOW ANY OF THE FOLLOWING TYPE OF DIETS?



REGISTRY
PARTICIPANT

> I made very drastic changes to my diet once I reached stage five, such as eliminating meat and dairy and also reducing or eliminating high potassium and high phosphorous foods. I restrict protein and take phosphorous binders with meals containing phosphorous.

PATIENT REPORTING

LIVER CYSTS

This disease isn't just about the kidneys. Other parts of the body can be affected, such as the liver.

Learn more
pkdcure.org/what-is-adpkd/what-are-the-related-health-complications

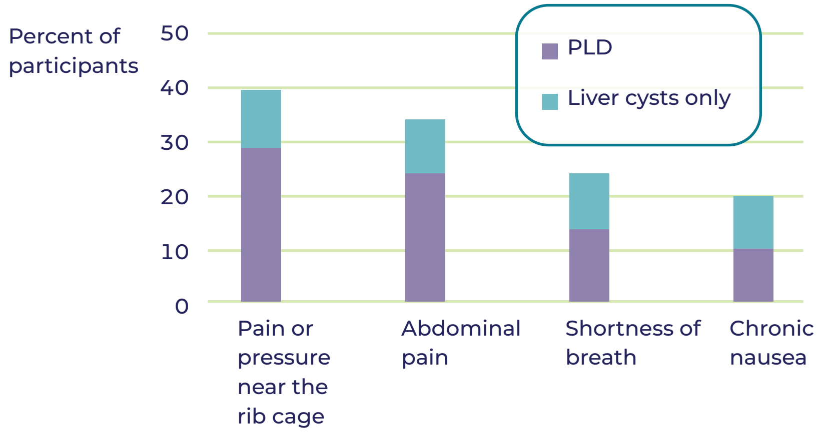
31%

had received a blood **diagnosis** of polycystic liver disease (PLD)

69%

of participants reported liver cysts

HAVE YOU EVER BEEN TOLD BY A MEDICAL PROVIDER THAT THE FOLLOWING SYMPTOMS COULD BE DUE TO LIVER CYSTS?



> I got diagnosed with PLD about 17 years ago after an evening in the ER. I was in excruciating pain, but my doctors pretty much dismissed it because, although enormous, my liver was functioning and there was little research on treatment. At one point, after my kidney transplant, my liver was noted to have grown 10% in less than a year, and I ultimately needed a liver transplant. Earlier attention to my PLD by my doctors would have been life changing, and PKD researchers *must* pay more attention to this aspect of disease and its symptoms.

Debra
REGISTRY PARTICIPANT

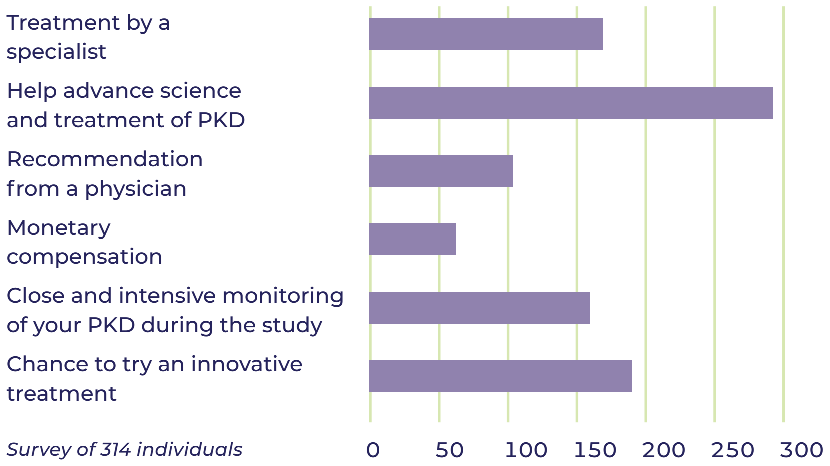
PATIENT REPORTING

RESEARCH STUDY RECRUITMENT

Based on their answers in the Registry modules, the Registry staff can match individuals to studies for which they may qualify.* We then provide information about that study in an email and leave the power in the patient's hands for whether they want to reach out to the study team. After sending information about the study, we asked those same individuals to tell us if they decided to sign up to participate as well as what motivated them.

Learn more
clinicalstudies.pkdcure.org

WHAT WOULD OR DOES MOTIVATE YOU TO PARTICIPATE IN PKD RESEARCH?



Survey of 314 individuals

FREEDOM Transplant Procedure study

Nutrition Study at the University of Colorado Denver

Dialysis Modality Decision-Making Interview at St. Louis University

> The PKD Registry sent me an email about a transplantation trial that I wasn't aware of. This was perfect timing, since I was researching centers at the time. After looking into the trial, I have enrolled and am very excited about the possible life-changing benefits that I may receive. I am very glad that the organization running the trial worked with the Registry to find people like me who might be eligible.

REGISTRY PARTICIPANT

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* Individual participant names and contact information are never shared with the study sponsor.

MEET OUR TEAM

Members of our team live throughout the United States, so we're no strangers to meeting remotely. Despite the challenges of the COVID-19 pandemic, we got a lot done.

PATIENT REGISTRY ADVISORY COMMITTEE (PRAC)

Ron Perrone, MD (Chair)
Terry Watnick, MD (Chair)
Bev Benson, PhD
Neera Dahl, MD
Berenice Gitomer, PhD
Klee Kleber
Amy Manelli
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Amy Manelli
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Debbie Plunkett

PKD FOUNDATION (PKDF)

Chris Rusconi, PhD
Elise Hoover, MPH
Nicole Harr
Glenn McMillan

About the FOUNDATION

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.

Since 1982, we've proudly funded more than $50 million in PKD research and leveraged $1.5 billion in government funding, all while serving our local communities across the country.

We're inspired by our mission. And driven by our vision.

ADPKD PATIENTS

JOIN OUR MISSION *to* ADVANCE PKD RESEARCH *by* SIGNING UP *for the* ADPKD REGISTRY TODAY. WE INVITE YOU *to* **SHARE THIS REPORT** *with* ANYONE YOU THINK WOULD BE INTERESTED IN LEARNING MORE *about* PKDF'S MISSION *and* IMPACT.

PKD CLINICIANS

Tell your patients about this research program and encourage them to sign up.

PKD RESEARCHERS

The ADPKD Registry is a powerful recruitment tool. Do you have upcoming clinical studies? Let us help with your enrollment by spreading the word about your research and the potential impact on the patient community.

PKD FOUNDATION
Polycystic Kidney Disease

FIGURE PERMISSIONS

To request use of charts and data provided in this report, contact the ADPKD Registry team by emailing registry@pkdcure.org.

SUGGESTED CITATION
PKD Foundation ADPKD Patient Registry
2020 Annual Data Report
Kansas City, Missouri

registry@pkdcure.org

800.PKD.CURE

pkdcure.org/registry