ADPKD REGISTRY
Powering a Cure

ANNUAL REPORT 2021

Since we launched the ADPKD Registry program in 2019, we've seen an incredible response from the patient community.

You signed up and answered research questions about your diagnosis and kidney function, aspects of the disease outside of the kidney, and the ways that pain impacts your quality of life. By sharing these intimate details of your ADPKD experience, you're providing researchers and clinicians with new information to help them understand the disease. Thank you!

So far, we've used your data to talk with clinicians at two nephrology conferences and to inform our educational program around barriers to care. This summer, we'll start sharing your data (your names and other identifying information will be removed) with researchers through a brand new data-sharing portal. They'll be able to explore how elements such as quality of life, liver cysts, and dietary habits change as disease progresses. The value of your answers to our questions will grow exponentially.

The collection of data presented in the following pages highlights the need to collaborate with those who are experts of the disease experience: those who live with PKD. And as we connect the patients in our Registry to clinical studies, we accelerate the work that drives our mission—work toward accelerating treatments and finding a cure.

Before growing any further, we want you to know that confidentiality and privacy of your information is incredibly important to our team. The Registry asks very personal questions about diagnosis, family history, symptoms, and your relationship with your health care team. We know that this information is confidential, and we're proud to work with a platform which has an excellent reputation for storing this data in a way that protects patient information. We've put policies in place ensuring that 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.

One of the last things we did before the end of 2021 was send you an engagement module to understand your preferences for interacting with the Registry and how to better return value to you. We learned about the kind of topics that interest you, the incentives that would motivate you, and how we can improve our Dashboard and Quarterly Newsletters. And in the next year, we'll be acting on it!

Thank you so much to all those who have signed up in our first two years! Let's continue to grow and work together to #endPKD.

Chris Rusconi, PhD
CHIEF RESEARCH OFFICER

Elise Hoover, MPH
SENIOR DIRECTOR OF RESEARCH

Vanessa Holliday, MPH
RESEARCH SPECIALIST

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4 *About the Program*

Learn about what it means to participate in the ADPKD Registry, launched in 2019, as well as see the list of modules containing our research questions.

6 *About Recruitment*

As of December 2021, over 2,300 ADPKD patients have joined the Registry from all over the United States.

8 *Quality of Life*

We used two quality of life tools to understand the impact of ADPKD on the mental health of participants. We also asked about sharp pain, dull pain, and fullness/discomfort, and looked at how results vary by chronic kidney disease stage.

10 *Family History*

Over 1,500 participants told us about their family history of ADPKD, and we looked at what diagnosis looked like between siblings.

12 *Dietary Choices*

Participants told us about the kind of supplements they take and how much water they drink on an average day.

13 *COVID-19 Impact*

95% of participants are vaccinated against COVID-19. We also heard from 70 individuals who were diagnosed with the disease.

14 *Health Care Access and Utilization*

We were surprised to hear that 69% of participants have never had a total kidney volume measurement; this is important for understanding your risk for rapid progression.

15 *Vascular Outcomes*

Over 50% were told that they should be screened for brain aneurysms, largely as a result of family history. ADPKD patients are at higher risk for this event than the general population, especially if they have a family history.

16 *Liver Cysts*

73% of participants reported having cysts on their liver. This is one of the most common aspects of the disease outside of the kidney for patients, and 26 reported needing a liver resection.

18 *Research Study Recruitment*

The Registry has helped to recruit for nine clinical studies — participating in research is key to finding new treatments and a cure for ADPKD.

20 *Meet our Team*

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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.**

Debbie
PATIENT ADVISORY GROUP

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.

"For at least two generations, my family has suffered from PKD. It has touched the lives of many and resulted in the untimely death of others. For many of these years, there was little hope or concerted effort toward treatment for PKD. We now live at a time where there is sincere interest in solving PKD, great news for those of us who suffer from this disease. Now, the deficit is in gaining deep insights into PKD so that viable solutions can be conceived, tested and ultimately made available to the millions of PKD patients. This is where the ADPKD Registry comes into play. The information derived from surveys and engagements with PKD patients and families is invaluable in illuminating this disease. History has shown that, through similar data-gathering initiatives, energy and commitment emerges to solving tough medical challenges such as PKD. For this reason, the ADPKD Registry is a worthy investment of time and a vital contributor to solving PKD.

ADPKD Impact Scale

Core questionnaire

COVID-19 Impact

Diet and lifestyle

Engagement

Experience with Liver Cysts

Family history

Health Care Access and Utilization

Pain and Discomfort Scale

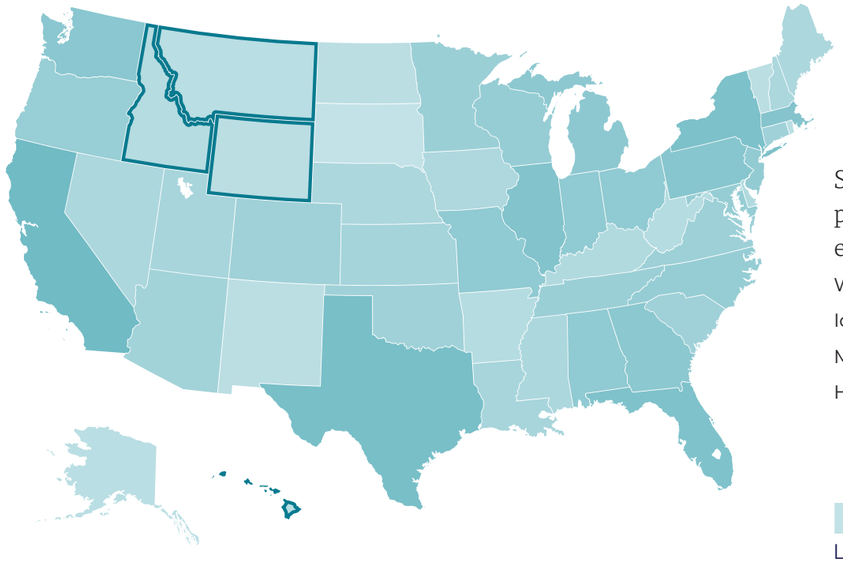
Vascular Outcomes

About RECRUITMENT

As *of* DECEMBER 2021, THERE WERE **2,348 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!



States with the greatest percent increase in enrollment in 2021:

Wyoming · 75%

Idaho · 50%

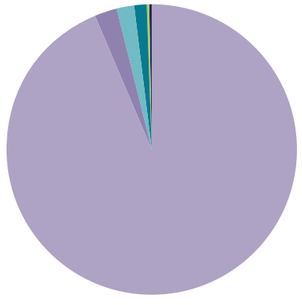
Montana · 50%

Hawaii · 43%

Less — More

IN 2022, OUR GOAL IS *to* **INCREASE** BOTH RACIAL *and* GENDER **DIVERSITY** *in the* PROGRAM. WE'RE ALSO **TRANSLATING** REGISTRY MATERIALS *into* SPANISH.

RACE & ETHNICITY



White **89.4%**

Black or African American **2.4%**

Asian **2.13%**

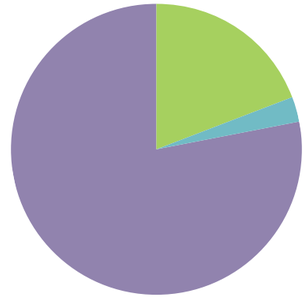
Multiple races **1.58%**

American Indian or Alaska Native **0.55%**

Native Hawaiian or Other Pacific Islander **0.06%**

4% also self-identified as Hispanic or LatinX

PKD STATUS

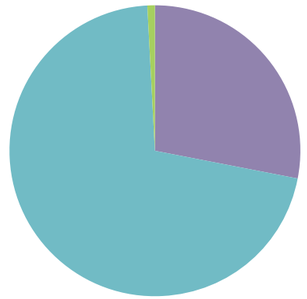


Post-kidney transplant **19%**

On dialysis **3%**

PKD kidneys still functioning **78%**

GENDER



Male **28%**

Female **72%**

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

I feel motivated to participate in this Registry because I truly believe that the only "constant" is change and I appreciate the researchers' intention to follow patients longitudinally for a more complete understanding of the disease.

REGISTRY PARTICIPANT
Texas

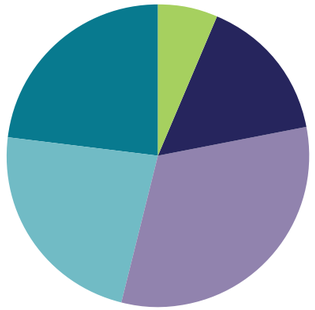
PATIENT REPORTING

QUALITY *of* LIFE

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

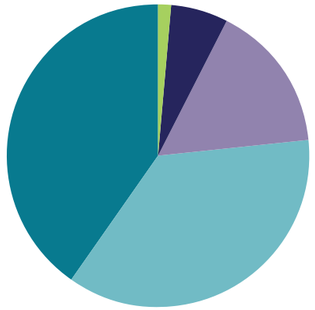
Learn more
pkdcure.org/living-with-pkd/chronic-pain-management

ANXIOUS ABOUT YOUR PKD GETTING WORSE IN THE PAST TWO WEEKS
of 856 who responded



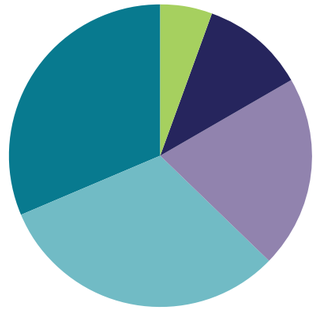
Extremely bothered **8.5%**
Very bothered **15.4%**
A little bothered **31.9%**
Somewhat bothered **23.1%**
Not bothered at all **22.9%**

ACCEPTING YOUR PKD AS PART OF LIFE
of 856 who responded



Extremely difficult **1.6%**
Very difficult **6%**
A little difficult **15.8%**
Somewhat difficult **36.3%**
Not difficult at all **40.2%**

FEELING GUILTY ABOUT THE IMPACT OF YOUR PKD ON YOUR CHILDREN OR FAMILY
of 856 who responded

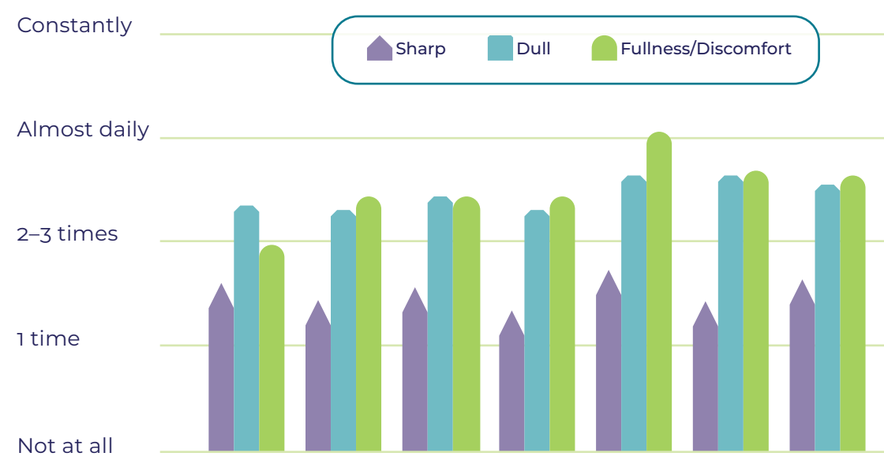


Extremely bothered **5.7%**
Very bothered **11.1%**
A little bothered **20.6%**
Somewhat bothered **31.3%**
Not bothered at all **31.2%**

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

Knowing more about kidney pain as the disease progresses will help inform clinicians about the symptoms that impact their patients' daily lives. Since these modules only ask about the past seven days, we ask them multiple times to measure pain throughout the year. There are slight differences between patients at different stages of chronic kidney disease, but overall we can see that pain is a constant feature of PKD.

FREQUENCY AND TYPE OF KIDNEY PAIN OVER THE PAST WEEK
average answers of 856 who responded



CKD STAGE	1	2	3A	3B	4	5	Unknown
People with stage	*59*	*165*	*136*	*117*	*108*	*36*	*117*

We need more people in CKD Stages 1 and 5 to fill out the module. Add your answers so we can better see how pain might change as the disease progresses.

> I am the fourth generation of my family to have ADPKD. My son may be the fifth. I hesitated for a long time to tell him about my diagnosis because that meant I had to tell him I might have passed it on.

REGISTRY PARTICIPANT
Pennsylvania

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
of 1,544 who responded

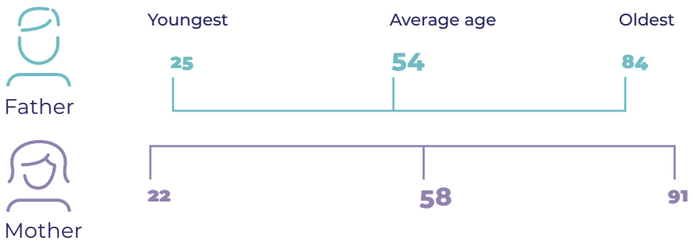
Unknown **7%**

No family history **13%**

Mother's side **39%**

Father's side **41%**

AGE OF PARENTS WITH ADPKD DIAGNOSIS AT KIDNEY FAILURE
of 1,544 who responded



SIBLINGS WITH PKD
of 842 who responded

How many siblings?	Particpant was only one with ADPKD	At least one sibling had ADPKD	All children in family had ADPKD
I'm an only child 10.21%	100%	——	——
One sibling 34.68%	63%	——	37%
Two siblings 30.88%	45%	34.6%	20.4%
Three siblings 17.58%	17.6%	82.4%	8.8%
Four siblings 7.72%	13.8%	83.1%	4.6%

"This disease goes back seven generations in my family without skipping any. My grandma was one of 12 children. All except two had the disease. None of them lived to see 50."

REGISTRY PARTICIPANT
Wyoming

"I find it interesting that all of my father's four children have ADPKD and that all of my children (and none of my sisters') have ADPKD."

REGISTRY PARTICIPANT
Vermont

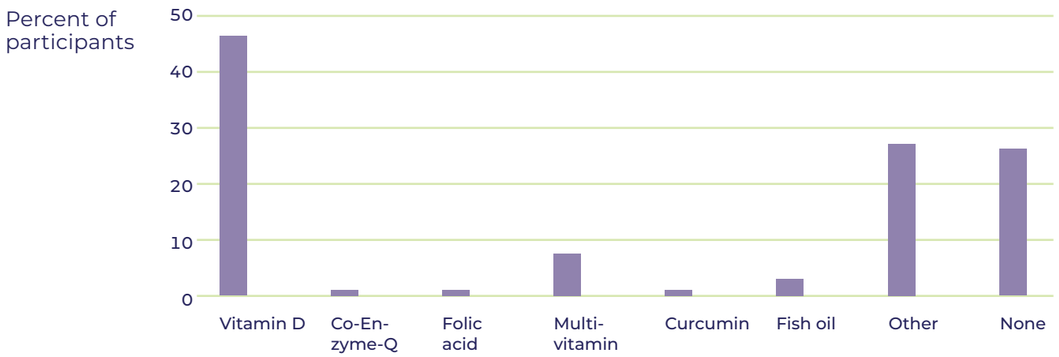
PATIENT REPORTING

DIETARY CHOICES

There is no specific diet proven to make polycystic kidneys better or keep them from getting worse. However, for most patients, clinicians recommend limiting sodium (salt) in the diet to help lower blood pressure and slow progression, 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

PATIENTS TAKING DIETARY SUPPLEMENTS
of 891 who responded



"This year, I have dramatically increased my daily water intake to about 64 oz/day and transitioned to a plant-based diet — but am not 100% compliant. I am underweight due to feeling full consistently, and have not exercised in two years due to chronic pain.

REGISTRY PARTICIPANT
Florida

53.7%
of participants report drinking at least eight cups of water per day

PATIENT REPORTING

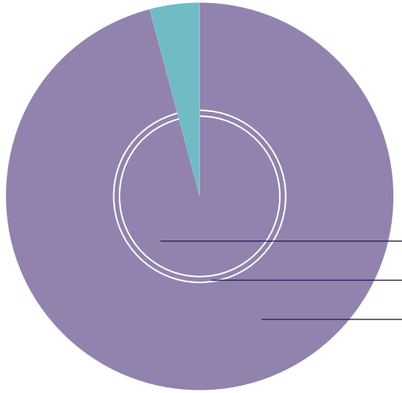
COVID-19 IMPACT

In October 2020, we asked about how the pandemic has impacted the PKD community. Luckily, only 70 reported a positive COVID-19 diagnosis, and all have since recovered. However, this is likely an underestimate since those who may have died or were very sick with the disease likely did not complete the module.

Those living with chronic diseases, like PKD, are at increased risk for more severe COVID-19 illness. Keep up the good work of protecting yourselves!

Learn more
pkdcure.org/coronavirus

PATIENTS WHO RECEIVED A COVID-19 VACCINE
of 659 who responded



Not vaccinated **4%**

Top two reasons for not getting vaccine:
— couldn't get an appointment
— are choosing not to get the vaccine

Vaccinated **96%**
Moderna 41%
Johnson & Johnson 3%
Pfizer-BioNTech 56%

"I pretty much isolated myself from the public for the first year. Once I was vaccinated, I felt safer until transplant recipients found out we are getting little to no protection from the vaccine. However, I just wear a mask when in public and look forward to the research being done to find out what can be done to make sure we are protected.

REGISTRY PARTICIPANT
California

70
individuals reported a diagnosis with COVID-19

10 of those were post-transplant
— 50% were admitted to the hospital
— 40% needed oxygen through a tube under the nose
— 10% experienced acute kidney injury

The other groups who were most admitted to the hospital were those 50–59 years old (39%) or 80+ (50%)

6 other cases of acute kidney injury were reported in those between 40–69 years of age

PATIENT REPORTING

HEALTH CARE ACCESS *and* UTILIZATION

We know that total kidney volume (TKV) is important for understanding risk of rapid progression of PKD. However, 69% said they've either never had their TKV measured or were unsure. Of those who reported getting a measurement, especially by CT or MRI, a large majority was for research purposes, not even for prognosis or medication evaluation.

Learn more

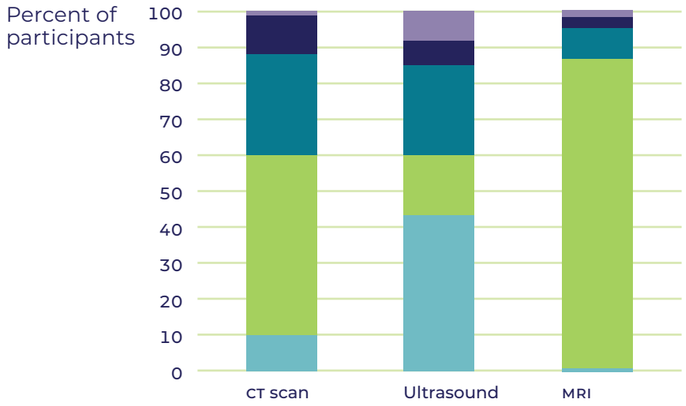
resources.pkdcure.org/resources/understanding-your-risk-for-adpkd-progression/

69%

never had their TKV measured or were unsure

PATIENT-REPORTED PURPOSE FOR TKV MEASUREMENT BY IMAGING MODALITY

of 429 who participated



MEYEON PARK, M.D.
University of California San Francisco

"The TKV is a critical piece of the puzzle for me when helping to advise patients about risk of rapid progression and the role of various treatment strategies in PKD. Many imaging studies can be used to approximate the TKV, and although MRI is certainly the gold standard, finding ways to measure TKV—even from other types of scans not necessarily obtained for kidney purposes—can be very helpful.

PATIENT REPORTING

VASCULAR OUTCOMES

Studies have shown that PKD patients have a 5–10% risk of developing intracranial (brain) aneurysms. This is about five times the risk of the general population.

An aneurysm is an outpouching in a blood vessel, which can leak or rupture. They also seem to cluster in certain families — that is, if a member of your family has an aneurysm or has ruptured an aneurysm, you may be at a higher risk of having an aneurysm yourself.

Learn more

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

50.4%

were told by their doctor they should be screened for brain aneurysms

DOCTOR'S REASON FOR RECOMMENDATION TO SCREEN FOR BRAIN ANEURYSM

of 315 screened participants

36%	Someone in your family had a brain aneurysm or had sudden death (presumed to be due to brain bleed)
34%	Doctor screens everyone with ADPKD
14%	Symptoms concerning for brain bleeding
6%	Before a major surgery
6%	Your family history is unknown
4%	Reassurance (personal preference)

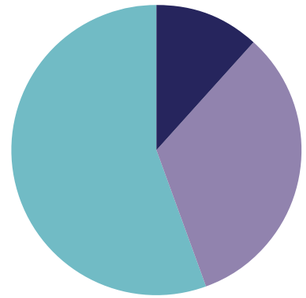
BRAIN ANEURYSM SCREENING METHOD

of 315 who responded

CT scan **11.9%**
Computerized tomography scan

MRA **32.6%**
Magnetic resonance angiopathy

MRI **55.4%**
Magnetic resonance imaging



REGISTRY PARTICIPANT
Washington

"All of my family members have had aneurysms: one aunt died at 55 from a rupture and another aunt had surgery after an aneurysm ruptured. My father also has an aneurysm that they're watching.

REGISTRY PARTICIPANT
Kansas

"Regularly screened every five years since being diagnosed. Very small aneurysm behind right eye but not inside the brain. This has not changed in the last 15 years.

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

resources.pkdcure.org/resources/understanding-pld/

34%

of 697 received a **diagnosis** of polycystic liver disease (PLD)

73%

of 697 participants reported liver cysts

26

patients reported having had a liver resection

100%

of 26 were female

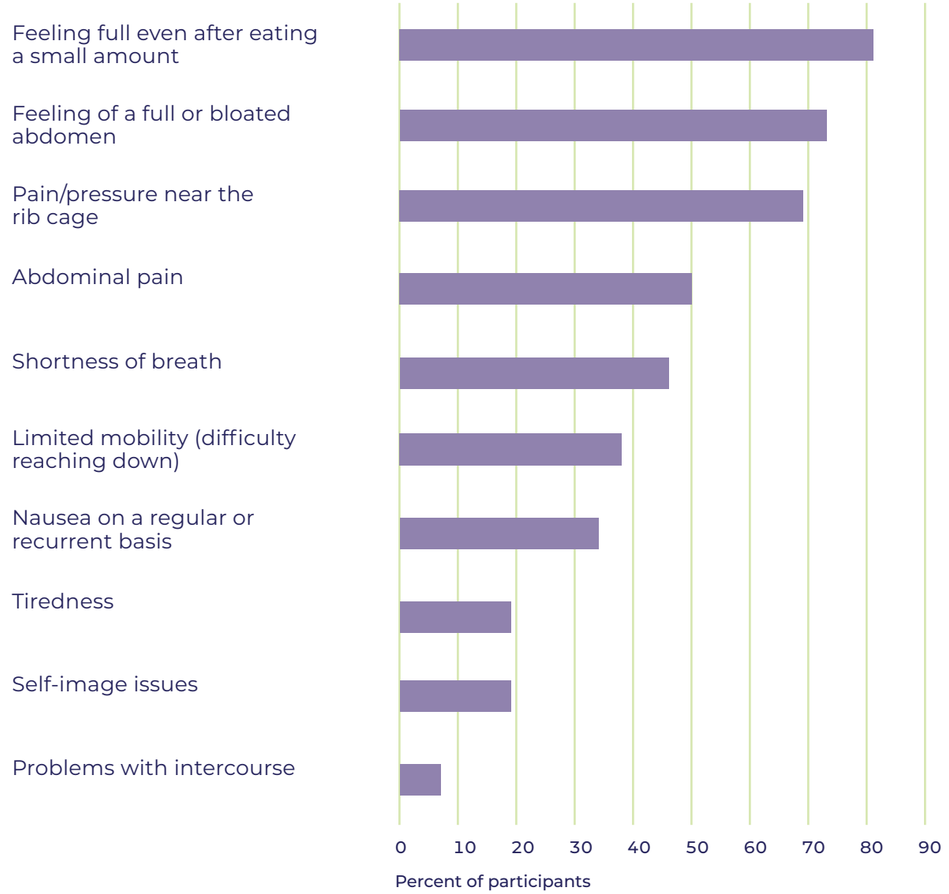
96%

of 26 had their doctor tell them their liver is larger than average

5%

of 26 had a suspected liver cyst infection

HAVE BEEN TOLD BY A MEDICAL PROVIDER THAT THE FOLLOWING SYMPTOMS COULD BE DUE TO LIVER CYSTS
of 26 with a liver resection



"As the polycystic kidneys and liver grow, they cause the rib cage to expand (mine is now totally "open" with large liver taking up room. Had resection of left lobe of liver at time of my kidney transplant and had excellent relief for about five years — liver portion has since regenerated. I have episodes of discomfort from time to time but not worth any procedures at this time. I also believe it is important to minimize procedures to ensure a healthy (normal function) liver even if it is huge.

REGISTRY PARTICIPANT
Texas

PATIENT REPORTING

RESEARCH STUDY RECRUITMENT

Since we launched the Registry, we've helped to recruit for nine clinical studies, and contacted nearly 3,400 of those who may be eligible (some were contacted more than once).

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?
of 314 who responded

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

FREEDOM Transplant Procedure study

SINCE WE LAUNCHED *the* REGISTRY, WE'VE HELPED *to* RECRUIT *for* NINE CLINICAL STUDIES.

Nutrition Study at the University of Colorado Denver

Statin Study at the University of Colorado Denver

Dialysis Modality Decision-Making Interview at St. Louis University

Aneurysm study at the University of Maryland Baltimore

FALCON clinical trial

Women's Health in PKD survey at University of California San Francisco

Genetic Testing Survey at University of Nebraska Medical Center

Pediatric ADPKD Registry at Children's National

"I would join any study that doesn't require me to travel more than 50 miles from my home."

REGISTRY PARTICIPANT
Georgia

"I am in stage 4 now and most treatments are too late for me but I want to advance treatments for my two children who have PKD. I hope there will be more available to them than there was for me and my mother."

REGISTRY PARTICIPANT
Massachussetts

"What is learned through a study I am enrolled in may help my PKD diagnosed daughter. I was very glad to be enrolled in TAME study for two years. The constant medical evaluations were reassuring at a time when I was worried about my new diagnosis."

REGISTRY PARTICIPANT
Maryland

MEET OUR TEAM

We're lucky to have a great group of collaborators from all over the United States who advise us on how to make the Registry the best research tool it can be. This group includes researchers and clinicians, but most importantly, patient stakeholders.

PATIENT ADVISORY GROUP


Amy Manelli


Cari Maxwell


Judy Ehrlich


Debbie Plunkett


Dwight Odland


MaryKatherine Michiels-Kibler

PATIENT REGISTRY ADVISORY COMMITTEE

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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 the* **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
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2021 Annual Data Report
Kansas City, Missouri

registry@pkdcure.org

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