

# PKDF Peer Ambassadors November Check-in

November 1, 2022



**PKD FOUNDATION**  
Polycystic Kidney Disease

# Panafest and Trinity Health Systems Event Recap

## Tatiana Mangwi (MD)

- Hosted an awareness booth at Panafest Festival

## Alyssia Gomez (NY)

- Hosted an awareness booth at Trinity Health Systems Hospital



**Celebrate African heritage & culture at Panafest**

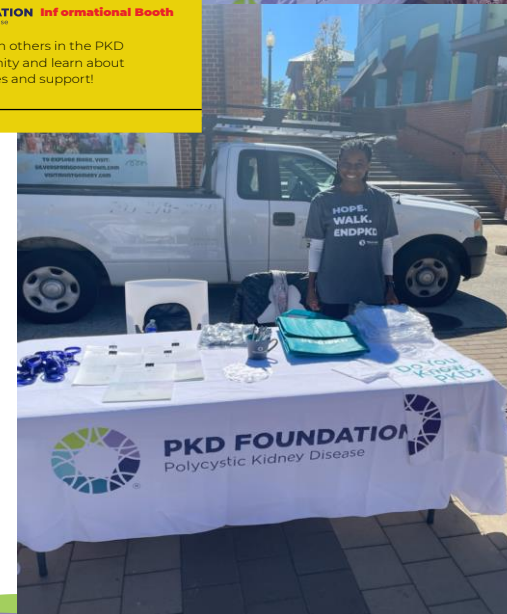
SEPTEMBER 24, 2022  
(12:00 PM – 9:00 PM)

THE SILVER SPRING CIVIC CENTER AT VETERANS PLAZA  
One Veterans Place,  
Silver Spring, MD 20910

FREE EVENT

Stop by the  **PKD FOUNDATION** Informational Booth  
Polycystic Kidney Disease

to make connections with others in the PKD and kidney care community and learn about kidney care resources and support!



# Upcoming/Ongoing Events and Activities

## Cayleigh Sanders

- Social Media Campaign (ongoing)

## Dwelyn Williams & Iris Resto

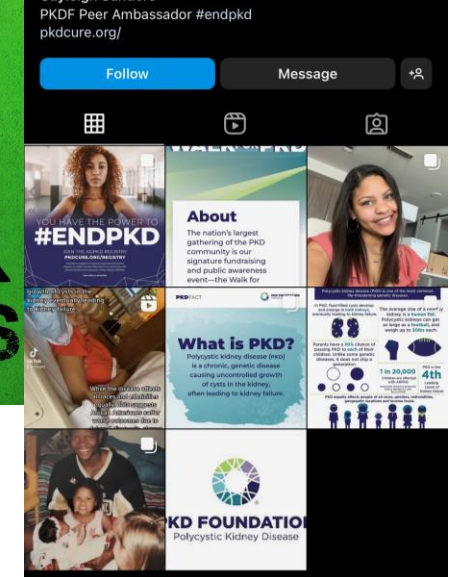
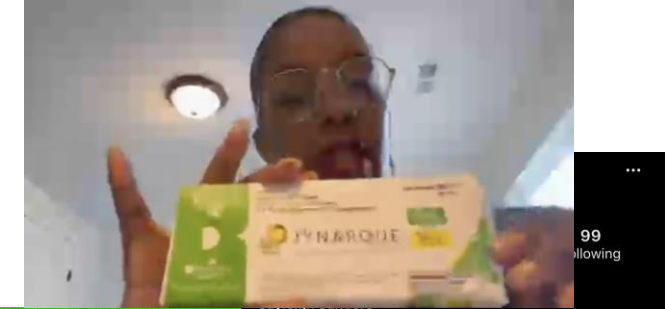
- Kidney Trails Podcast Collab

## Funke Ojuri

- Association of Black Women Physicians Awareness Webinar (2022)

## Patrice Adams

- PKD Awareness YouTube Vlog Launch (ongoing)



*Moving forward, PKDF Peer Ambassadors will be working with Nicole Harr ([nicoleh@pkdcure.org](mailto:nicoleh@pkdcure.org)) to coordinate all events and activities.*





**ADPKD  
REGISTRY**

Powering a Cure

# Peer Ambassadors – Intro to the Program



**PKD FOUNDATION**  
Polycystic Kidney Disease



# ADPKD REGISTRY

Powering a Cure

- Launched September 2019
- Hosted on an online platform
- Goals:
  - Create a tool to better understand how to improve the lives of ADPKD patients
  - Connect patients to clinical trials for which they may be eligible

**Sign in**

**Sign up**

**YOUR INFORMATION IS POWERFUL.**

### What is ADPKD Registry?

The ADPKD Registry is a collection of individuals with autosomal dominant polycystic kidney disease (ADPKD).

<b>Answer survey questions</b> Answer survey questions about your experience with ADPKD	<b>Keep information current</b> Update us on your quality of life and PKD symptoms throughout the year	<b>Participate in research</b> Find out about research studies for which you might qualify
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# About the program



**PKD FOUNDATION**  
Polycystic Kidney Disease

- 2,650 registered participants and growing
- **Participant-powered:** patient journey perspectives and data collected from individuals with ADPKD
- **Participant-centered:** People affected by ADPKD involved in advisory groups, module development, and beta testing
- Remote participation: participate in PKD research from computer, tablet or phone



# Privacy/Confidentiality



Access the Registry using a secure, web-based portal



Portal assigns unique ID and removes patient personal info



E.g., name, contact information, address, date of birth



**ADPKD  
REGISTRY**  
Powering a Cure

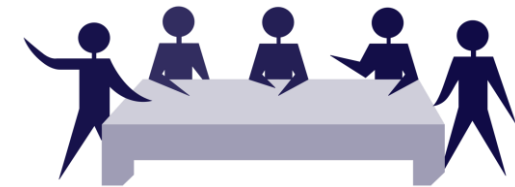
Identifiable data stored in a secure (separate) location



Answers to modules are recorded by participant ID



De-identified data stored in a secure location



Advisory Panels help to decide how to use and share data



# Patient reported outcome data



**Core Questionnaire**



**Vascular Outcomes**



**Diet and Lifestyle**



**ADPKD-Pain and Discomfort Scale**



**Family History**



**ADPKD-Impact Scale**



**Experience with Liver Cysts**



**Healthcare Access and Utilization**





NOVEMBER 2021

Without participants like you, we wouldn't have new insights into the patient journey or what pain looks like in ADPKD. During this month of thanks, we're so grateful for you. Thank you for being a part of this program with us!

what we've learned

## QUALITY OF LIFE



Thanks to the over 800 participants who filled out the ADPKD-Pain and Discomfort Scale or ADPKD-Impact Scale!

How frequently did you experience dull kidney pain in the past seven days?



### Financial assistance resources

The cost of managing chronic kidney disease can be expensive, especially since your disease progresses to kidney function decline and kidney failure. We gathered some financial assistance resources that might help - you offer different aspects of your care.

[Learn more >](#)

How has dull kidney pain interfered with...



## why it matters

We're working on a research abstract for the National Kidney Foundation's Spring Clinical Meeting to talk with clinicians about what pain looks like in ADPKD and how it impacts quality of life.

## CLINICAL STUDIES



The Registry was used to help recruit for four studies this fall!

Once participants completed their modules, we were able to see those that might be a good fit for these studies and sent those patients more information about the research and how to participate.

Participants contacted for each study:

16 Pediatric ADPKD Registry at Children's National	615 FALCON Clinical Trial	745 Study at the University of Colorado Denver	37 Anonym study at the University of Maryland Baltimore
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Quarterly



What we're learning



Impact



Call to action



What's coming up?



what's next

## ENGAGEMENT MODULE



Tell us how we can improve your Registry experience and return more value to you!

We are hoping to gather information to create the best user experience, while also increasing participant engagement in the program.

The module will take 5-10 minutes to complete, is around 15 questions, and asks about the following topics: notification, content, release day preference, and much more.

We are looking to get to know how we can better serve you in your journey.

Coming in December - the Registry will send you an email once available.

Individuals who complete the questionnaire, have the opportunity to opt-in for a PKD Swag drawing!

announcement

## STAY UP TO DATE

We're still missing details of some participant's PKD experience. Let's get closer to 100%

74%  
Core Questionnaire

67%  
Family History

27%  
Vascular Outcomes/  
Aneurysms

act now

1. If you haven't already completed all the modules, please *sign in* today so that our researchers have more data to work with.
2. Keep an eye out for the next modules.
3. Encourage your family or friends with ADPKD to sign up.

Send questions, comments and feedback to [registry@pkdcure.org](mailto:registry@pkdcure.org)

Shared successes



What do participants look like?

ADPKD-Impact Scale

ADPKD-Pain and Discomfort Scale

Family History

Diet and Lifestyle

Liver Cysts

Vascular Outcomes

Health Care Access

Recruiting for clinical studies



# ANNUAL REPORT 2021

### 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.



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



Debbie Patient Advisory Group

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.

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ADPKD REGISTRY ANNUAL REPORT 2021

## PATIENT REPORTING VASCULAR OUTCOMES

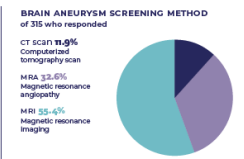
Studies have shown that PKD patients have a 5-10% risk of developing intracranial (brain) aneurysms. This is about five times the risk of the general population. An aneurysm is an outpouching in a blood vessel, which can leak or rupture. They also seem to cluster in certain families — that is, if a member 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](https://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)



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.

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ADPKD REGISTRY ANNUAL REPORT 2021

Participant testimonials



# ADPKD Patient Registry



Thank you for signing up for the Autosomal Dominant Polycystic Kidney Disease (ADPKD) Patient Registry. When you join and update your account, you are strengthening the power of the largest patient-powered PKD database in the US. Your data will be used to advance research to better understand, treat and cure ADPKD, without ever leaving your home.

Email

Password

**Sign in**

[First time to the Registry? Sign up.](#)

[Forgotten your password? Reset it.](#)





**ADPKD  
REGISTRY**  
Powering a Cure



**ADPKD  
REGISTRY**  
Powering a Cure

## Sign up

### Name and password

First name \*

Last name \*

Email \*

Confirm your email

Password \*

Confirm password

\*denotes required field

Next





## Sign up

### Medical information

Gender \*

Please select

Date of birth \*

DD  MM  YYYY

PKD Diagnosis \*

Please select

Your relationship to patient \*

Please select

\*denotes required field

[Previous](#)

[Next](#)





## Sign up

### Contact information

Phone

Address

City \*

State \*

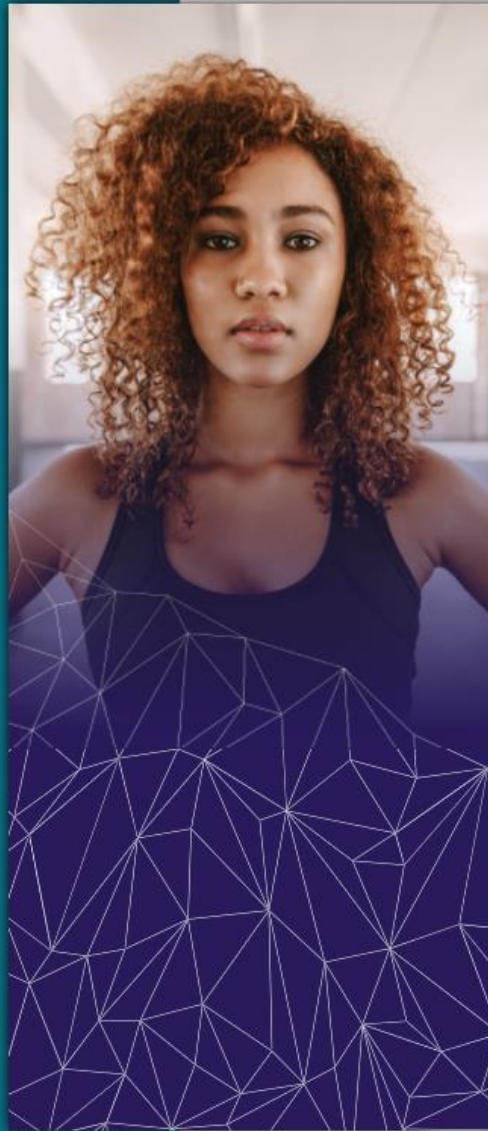
Zip code \*

\*denotes required field

[Previous](#)

[Next](#)





## Sign up

### Consent

#### My Consent.

##### ADPKD PATIENT REGISTRY CONSENT FORM

To complete your registration, please review and agree to the consent form below.

You can also [download/print a copy of this consent form](#).

##### Information about the ADPKD Patient Registry

You are being asked to be in a research Registry run by the PKD Foundation. The PKD Foundation is the only organization in the U.S. solely dedicated to finding treatments and a cure for PKD to improve the lives of those it affects. Our vision is to #endPKD.

Before you agree to take part in the ADPKD Patient Registry, it is important that you understand what is involved, what information you may receive, and what will be done with the information you provide. Please read this form carefully. The Registry staff can provide answers about the ADPKD Patient Registry provide link to FAQs and contact information.

- I understand the purpose and nature of the ADPKD Patient Registry, and I have no additional questions.
- I give permission for the information I share in the Registry to be used for the Registry Goals and provided to researchers, other registries or databases. The information will be shared anonymously unless I give express permission for identifying information to be shared.
- My participation in the Registry is entirely my choice. If I change my mind and wish to withdraw my participation, I will be free to do so at any time without having to provide any explanation, and I will not be penalized in any way for withdrawing my consent.
- I understand the risks and benefits of participation and I agree to participate in the ADPKD Patient Registry.

##### If you are registering a child under 18 years of age:

- The child I am registering has given me his/her assent (agreement) to include his/her information in the ADPKD Patient Registry; OR the child I am registering is not able to understand the idea of participating in a Registry and I am making the choice for him/her.

Please download and review the assent form.

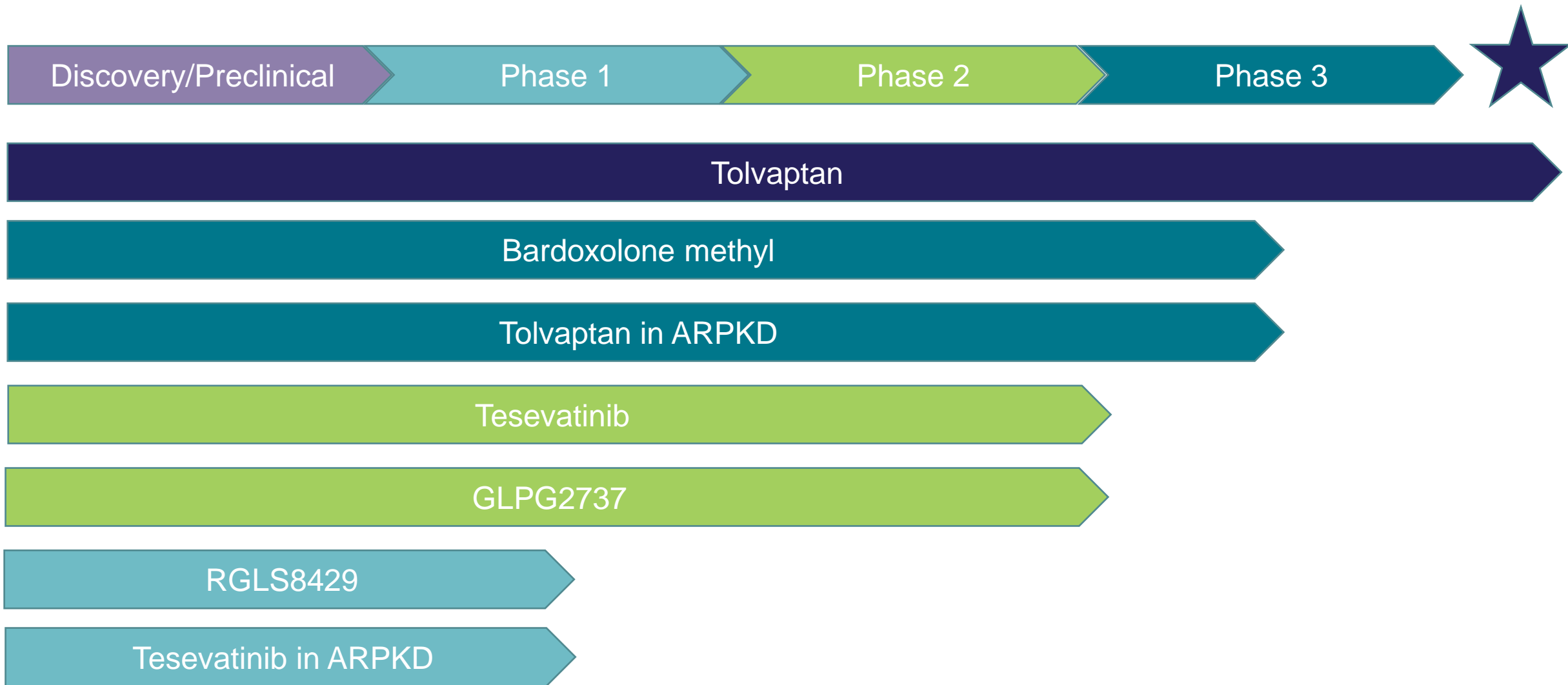
Name of Parent / Legal Guardian

\*denotes required field

[Previous](#)

[Register](#)

# PKD Treatment Pipeline







# Drug Repurposing, Dietary Supplements and Dietary Interventions

Curcumin

Metformin

Caloric Restriction

Empagliflozin

Statin therapy

# Types of clinical research



- **Observational studies:** researchers analyze health data to find links between a diagnosis and disease progression, symptoms or quality of life



- **Interventional studies:** test new ways to prevent, detect, or treat diseases

# Clinical Trials

Clinical trials = human studies of new treatments or therapies for a disease to ensure that it is both safe and effective

**Under enrollment is one of the most significant problems facing PKD drug development**

- Failure to recruit enough volunteers to complete the study
- Failure to recruit patients quickly enough to make study completion feasible

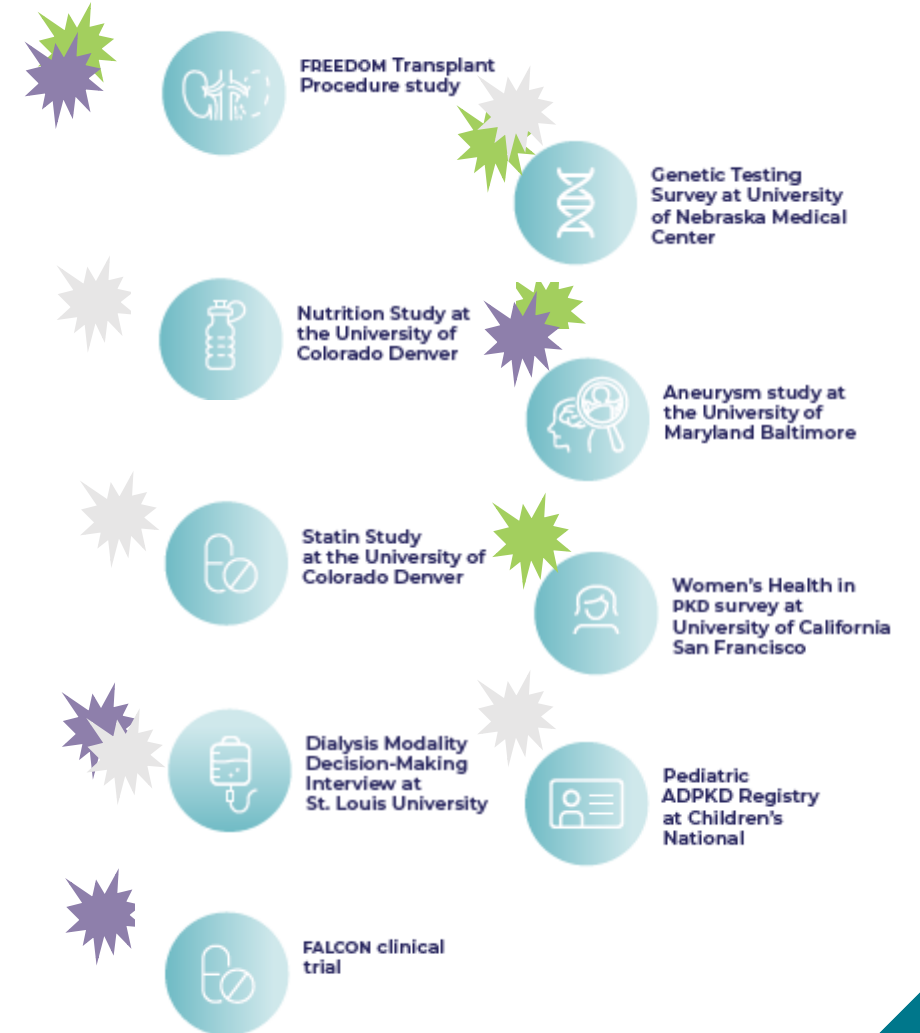


# Research Recruitment




- Kidney function
- Age
- Gender
- Symptoms
- Dialysis or transplant status


*Other eligibility criteria*



# ACT Alerts



Get notified of studies in your area.

 SIGN UP FOR EMAILS



## ACT ALERT

ACCELERATING CLINICAL TRIALS 

Hi Elise,

Researchers have a lot of questions about Autosomal Recessive PKD (ARPKD), including what the disease journey looks like. If your child has been diagnosed with ARPKD, our collaborators at Children's Hospital in Washington, D.C., need you to sign up for the [ARPKD \(and ARPKD-related diseases\) Database](#).

Their study has three parts: the clinical database (medical health information), genetic database (includes research genetic testing), and the educational part. The registry wants to learn more about these diseases and share these discoveries with families, physicians, and genetic counselors via the study website. The database welcomes participation from all over the United States and Canada. *It's not necessary for you (nor your child) to visit the center in D.C.*

### Who can participate in the study?

- Males and females of all ages (including fetuses)
- Diagnosis of ARPKD or ARPKD-like diseases

LEARN MORE

# Clinical Studies tool



**Participate  
in Research**

To develop drugs and therapies for PKD, clinical studies must be conducted. Patients play a key role in the research and development process by volunteering to participate in clinical studies. From observational studies to clinical trials, you can help researchers unlock the secrets of PKD and find a treatment by participating in a study.

**I am a ...**

- PKD Patient
- Patient parent or caregiver
- Researcher or clinician
- Other

Note: If you select "Patient parent or Caregiver" please answer all future questions with the PKD patient in mind.

**What is your diagnosis?**

- ADPKD
- ARPKD
- Not sure

**Have you had a kidney transplant?**

- Have Had Transplant
- Have Not Had Transplant

**Are you currently on dialysis**

- On Dialysis
- Not on Dialysis

**What is your age?**

- Under 18
- 18-65
- Over 65

**Do you know your most recent kidney function (eGFR)?**

- Less than 30 mL/min/1.73m<sup>2</sup>
- Between 30-60
- Between 60-90
- Over 90
- Unknown

?

**Find a Study**

Diagnosis +

Locations -

- Alabama (2)
- Arizona (2)
- California (2)
- Colorado (3)
- Connecticut (2)
- District of Columbia (2)

[Show More +](#)

Age +

eGFR +

Dialysis +

Transplant +

Stage +

### Your Selected

X ADPKD X Have Not Had Transplant X Not on Dialysis X Under 18 X Between 30-60 [Clear all](#)

Showing all 7 results [RSS Feed](#) Sort by: Default ▾

#### ADPKD Registry

**Stage:** Recruiting

**Status:** Active [View Study](#)

**Location:** Online

**Age Range:** All ages

**Tagged as:** [ADPKD](#)

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#### A Study of Genes that Cause Cystic Kidney Disease

**Stage:** Recruiting

**Status:** Active [View Study](#)

**Location:** Mayo Clinic

**Age Range:** All ages

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#### Exploring the perspectives of renal clinicians and patients on the role of diet in chronic kidney disease (non-dialysis dependent) management

**Stage:** Recruiting

**Status:** Active [View Study](#)

**Location:** Online



# **Calls to Action for our Community**

1. Participate in the ADPKD Registry
2. Sign up for ACT Alerts
3. Check out clinical studies happening near you



# Thank you!

[eliseh@pkdcure.org](mailto:eliseh@pkdcure.org)  
[research@pkdcure.org](mailto:research@pkdcure.org)

