



FOR IMMEDIATE RELEASE:
June 25, 2019

FOR MORE INFORMATION:
Cadie Connors
Parris Communications
(816) 352-8504
cconnors@parriscommunications.com

The PKD Foundation to Invest Nearly \$5 Million to Fund Treatment Studies and Research for a Cure Over the Next Two Years

The PKD Foundation funds over 30 researchers this year

KANSAS CITY, Mo. – The [PKD Foundation](#) announces today its investment of nearly **\$5 million on research** over the next two years **to further understand Polycystic Kidney Disease (PKD) and to find more treatments and eventually a cure.**

Since the Foundation's establishment in 1982, **it has invested almost \$50 million in more than 1,300 research, clinical and scientific grants, awards, fellowships and scientific meetings.** The Foundation's financial commitment over the years has seen results on a local, national and even global level, including initiating funding for young investigators from around the world; supporting innovative research ideas; and providing bridge funding to researchers to allow researchers world-wide to apply for larger National Institutes of Health (NIH) grants.

“For the past 36 years, our goal has been to support PKD patients from care to cure. With the support of generous donations, we have the ability to stand beside PKD patients until there is a cure, supporting research for new treatments and to support researchers,” said Andy Betts, CEO of the PKD Foundation. “We are excited to announce that for the first time since 2009, due to rapidly expanding interest from the PKD research community, the Foundation has begun funding grants and fellowships on an annual cycle, rather than biennial, to better identify and support promising research.”

Current Research Portfolio

With the transition to annual awards, the total number of funded researchers in 2019 is over 30. This marks 2019/2020 being the largest number of awards in one year in the Foundation history. In 2019, the PKD Foundation awarded new research funding to eleven outstanding PKD researchers. Eight research grants were awarded to outstanding PKD scientists through the Foundation's Research Grant Program, and three recipients were awarded PKD Foundation Fellowships, which recognize early-career scientists who are rising starts in the field. The current research funding portfolio includes:

- **Grants.** One of the PKD Foundation's principal goals is the development of treatments for PKD. This program funds basic research aimed at increasing the understanding of

the genetic and pathological processes involved in PKD as well as research with an obvious or direct potential to accelerate the development of new therapies.

- **Young Investigator Award.** In order to support the transition of a junior scientist to an independent investigator, the PKD Foundation offers an additional year of research support for one grantee per year. The Foundation hopes that this award will not only create stronger, more established junior researchers, but will encourage scientists to find value in a long-term career in PKD research and treatment.
- **Fellowships.** The PKD Foundation's fellowship grants are designed to allow trainees to obtain significant research experience as they initiate and spend long, productive careers in PKD research. The program recognizes early-career scientists' achievements and potentially identify the next generation of scientific leaders in PKD research. The fellowship program also encourages and facilitates mentorship from an established PKD scientist.
- **Scientific Meetings.** The PKD Foundation is focused on funding relevant scientific and clinical meetings that bring together PKD scientists from around the world to discuss current findings and encourage research collaborations. The Foundation also funds medical education programs at national clinical meetings to educate medical professionals in the basic science, diagnosis, disease management and potential treatments for PKD.

Past Research Achievements

A direct result of our investment in research, valuable collaborations and achievements over the years include:

- The most significant is the **FDA approval of tolvaptan in the spring of 2018**, making it the first treatment in the United States for Autosomal Dominant Polycystic Disease (ADPKD). Funding from the Foundation supported early research of the vasopressin/cAMP hypothesis that resulted in the development of tolvaptan. The Foundation was also involved in spreading awareness of the clinical trials to the patient community.
- In 2007, PKD Foundation began long-term funding of the **Mayo Clinic ADPKD Mutation Database**, which contains information on all published mutations and other variants to the ADPKD genes, including PKD1 and PKD2. The research made possible by this resource is valuable in improving interpretation of diagnostic and prognostic tests, informing the development of targeted therapies and advancing technologies to simplify and reduce the costs of these tests.
- The Foundation also funded a pilot project in 2016 at Mayo Clinic to **develop automated tools** to accurately and reproducibly measure total kidney volume (TKV), the only FDA-approved prognostic biomarker for ADPKD.

- In 2016, The PKD Foundation, in collaboration with the Critical Path Institute (C-Path), sponsored the **ADPKD Biomarker Summit Meeting**, which brought together representatives from the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), Health Canada, the NIH and PKD experts. The Summit has been followed up with ongoing monthly PKD Outcomes Consortium (PKDOC) meetings.
- In 2018, The Foundation established a **PKD in Children Council**. As the PKD Foundation seeks to give hope and serve those affected by PKD, this Council was created to better understand what the community wants and needs, and to raise awareness through elevating the voices of families with young children affected by ARPKD and ADPKD. The Council hosts annual meetings to bring these voices together to discuss research, awareness and advocacy.

About the PKD Foundation

The PKD Foundation has been dedicated since its founding in 1982 to supporting and improving the lives of patients affected by polycystic kidney disease. These efforts are accomplished through promoting research to find treatments and a cure, as well as providing education, advocacy and awareness on a national level. The Foundation provides direct services to local communities nationwide and is the largest private funder of PKD research.

The PKD Foundation is the only organization in the United States solely dedicated to finding treatments and a cure for PKD. **Our mission:** We give hope. We fund research, advocate for patients and build a community for all affected by polycystic kidney disease (PKD). **Our Vision:** #endPKD

###

****For more information about PKD, please see the following page.***

Polycystic Kidney Disease (PKD) Fact Sheet

- There are two types of PKD, both are genetic: **autosomal dominant (ADPKD)** and **autosomal recessive (ARPKD)**.
 - ADPKD is the more common type and affects more than 600,000 Americans and 12.4 million people worldwide.
 - ARPKD is a rare form of the disease that occurs in 1 in 20,000 children worldwide.
- Autosomal dominant polycystic kidney disease (ADPKD) is a chronic, genetic disease causing uncontrolled growth of fluid-filled cysts in the kidneys often leading to kidney failure in many patients. It affects all gender, racial and ethnic groups equally.
- As the cysts accumulate more fluid, they get bigger and bigger, destroying healthy tissue, which can lead to high blood pressure, other complications, and often kidney failure.
- Prior to the FDA approval of tolvaptan, there was no treatment for PKD in the U.S. There is no cure. But there is hope.

Note: Tolvaptan is only approved to treat adult patients with ADPKD.

ADPKD Facts

- ADPKD is the fourth leading cause of kidney failure.
- More than 50% of people with PKD will develop kidney failure by age 50. Once a person has kidney failure, dialysis or a transplant are the only treatment options.
- A typical kidney is the size of a human fist and weighs about a third of a pound. Polycystic kidneys can be much larger, some growing as large as a football. Kidneys can develop cysts ranging in size from as small as a pinhead to as large as a grapefruit.
- Affected parents have a 50% chance of passing the disease to their children, so it often affects many people in one family.
- Approximately 10% of patients with PKD have no family history as PKD may develop as a spontaneous (new) mutation.