A magazine to inform and educate PKD patients and families,
Foundation supporters, health professionals and researchers.

PKD Progress

Fall 2016

ANNUAL REPORT ISSUE

INSIDE:

3 Dr. Perrone on PKD research progress

8 What total kidney volume means and why it is important

12 Annual report special section

20 Fall recipe from new PKD cookbook, *Cooking Well: Delicious, everyday recipes for the PKD family*

PKD thought leaders join forces

Scientific meetings engage PKD thought leaders for collaboration and breakthroughs

Page 4

PKD FOUNDATION
Polycystic Kidney Disease

READ MORE ONLINE!
pkdcure.org/pkd-progress

Leadership Letter

I've been a part of the PKD Foundation for more than 15 years, supporting the organization in numerous ways. My current dual role, as a Board of Trustees Member and Scientific Advisory Committee (SAC) Chair, has given me a special perspective that I treasure. The progress I've seen from my front row seat is invigorating.

This special issue of *PKD Progress* focuses on the important role the Foundation plays in supporting and engaging in scientific meetings. These meetings foster collaboration and set the stage for scientific breakthroughs. **This is critical for inspiring change and moving us closer to treatments.**

I would like to highlight the ADPKD Summit, which was attended by U.S. and international regulatory agencies, PKD academicians and eight companies interested in developing therapies for ADPKD. This conference focused on establishing clinical endpoints for PKD, in particular total kidney volume (TKV), in order to facilitate clinical studies. This is essential to providing clarity on the regulatory path to drug approval.

This special issue also showcases the Foundation's accomplishments during the last fiscal year through the generous support of donors and volunteers. From July 1, 2015 through June 30, 2016, we invested $5,763,021 in research, education, advocacy, awareness and support in fulfillment of our mission.

Highlights include:

- Fifteen research grants were selected for the PKD Foundation to support at $2.4 million over two years.
- The Foundation committed $500,000 in seed money to establish the Jared J. Grantham Research Fellowship in perpetuity. This investment was matched with $1.5 million by the American Society of Nephrology.
- A successful PKD National Convention was held, with more than 400 patients, caregivers, researchers and physicians in attendance.
- The 21st Century Cures Act, which passed in the House in July 2015 is helping accelerate the development of new treatments.

These strides and breakthroughs lay the groundwork for an even stronger fiscal year in 2016-17. In fact, five potential treatments are currently in clinical trials for PKD, and more than 30 active PKD Foundation-funded research projects are being conducted.

We appreciate all you do to keep the momentum going—this work could not be done without the dedicated support of donors and volunteers like you.

Terry Watnick

Terry Watnick, M.D.
PKD Foundation SAC Chair

READ MORE
See the timeline of our progress and how far we have come at pkdcure.org/timeline

COVER PHOTO:
Benjamin Cowley Jr., M.D., PKD Foundation Board Chair, and Ronald Perrone, M.D., SAC Member facilitate and represent the Foundation at many PKD meetings.

Leading PKD scientist Dr. Ron Perrone reflects on progress in PKD research

When Ronald Perrone, M.D., became involved with the Foundation in the mid-1990s, the genes for PKD had just been discovered. His role as an ad-hoc grant reviewer led him to become a member of the Foundation's Scientific Advisory Committee (SAC) in 1999, which he would chair from 2006 to 2010. Since then, he has witnessed significant progress. **"The Foundation's work has been integral to every significant development in PKD.** It has had an important role in advancing treatments and clinical research."

Dr. Perrone is pleased to see the Foundation's focus evolve beyond basic science to investigating potential treatments. "There has been a lot going on in PKD science since the genes were discovered. What's different now is the intense interest in translating basic mechanisms into development of therapies, a more immediate translation of research results into drugs that can actually be tested in humans."

Dr. Perrone's role in increasing the understanding behind the drug development process has influenced this shift. "I'm proud of helping the Foundation's leadership fully understand the process, which has led them to focus on therapies in a way directly related to drug development." Patient education has also played a role. "The Foundation's educational efforts are phenomenal. They've done a great job educating patients and families on what it takes to discover therapies, and helping them understand they need to participate in clinical trials. There is no other way to develop drugs."

Dr. Perrone's involvement with the Polycystic Kidney Disease Consortium (PKDOC) initiative has significantly impacted the drug development process. This work has moved us closer to establishing total kidney volume (TKV) as an endpoint for human clinical trials. (Read more about PKDOC and TKV on pages 6 through 9). "The fundamental problem for PKD drug development is that kidney function deteriorates slowly, which makes it very hard to study. We're looking to establish endpoints that can be used to judge the effect of an intervention earlier rather than later."

Finding interventions for PKD is Dr. Perrone's goal. What really drives him are the patients who need chronic care over the long term, like those with PKD. "I find that patients with PKD are some of the most highly motivated and interested in helping themselves. Working with people who are trying to do the best for themselves and their families is very rewarding." ■

> "My involvement with the SAC and PKD research has expanded my ability to improve life for patients, not just one at a time but in a global way."

EDUCATION AND CAREER HIGHLIGHTS

- **Tufts Medical Center**
  - **Scientific Director Clinical and Translational Research Center**
  - **Associate Chief Division of Nephrology**
  - **Medical Director Kidney Transplantation**
- **BS, Zoology, Pennsylvania State University**
- **MD, Hahnemann Medical College (Drexel University College of Medicine)**
- **Postdoctoral: Boston University Medical Center**
- **"Top Doctor" from the Boston Magazine (multiple years)**
- **"Top Doctor" U.S. News and World Report**
- **Donald W. Seldin Distinguished Award, National Kidney Foundation**
- **Physician of the Year–PKD Foundation**
- **Outstanding Physician in Nephrology, National Kidney Foundation of MA, RI, NH and VT**
- **Distinguished Faculty Award, Tufts University School of Medicine**
- **PKD Foundation Scientific Advisory Committee (SAC) Member**
- **Region 1 Representative OPTN/UNOS Kidney Transplant Committee**
- **Reviewer: Clinical Journal of the American Society of Nephrology, Journal of the American Society of Nephrology; American Journal of Kidney Disease**

RESEARCH

Scientific meetings

engage PKD thought leaders for collaboration and breakthroughs

Each year, the PKD Foundation sponsors and participates in meetings that bring scientists and clinicians from all over the world together to exchange ideas, collaborate and move us closer to therapies for PKD.

Last fiscal year we invested more than $83,000 in scientific meetings, and we'll continue to support these meetings as part of our key research efforts.

These meetings often lead to research collaborations that make a significant impact on advancing PKD science. Discussions with early-career investigators at these meetings have often led them to become PKD fellows, increasing the pipeline of researchers and nephrologists focused on PKD. Conversations with investigators who have attended these meetings have also led many of them to successfully apply for our research grants. Additionally, according to our Chief Scientific Officer David A. Baron, Ph.D., "in these meetings, we, as an organization, have the opportunity to meet and gauge the caliber of researchers we look to fund."

FY 15-16 MEETINGS

Sponsored by the Foundation

ADPKD Summit: **$51,540**

Jared J. Grantham Symposium: **$10,000**

Cell Volume Regulation Congress-PKD Symposium: **$6,000**

Cilia 2016: From Fundamental Biology to Human Disease: **$5,582**

American Society of Pediatric Nephrologists Genomic Gymnastics: **$5,000**

Brigham and Women's Hospital PKD Symposium: **$5,000**

$83,122 TOTAL INVESTMENT

We have an ongoing presence at these annual meetings. Some are initiated and planned by the Foundation, and representatives often facilitate and present. **Here is a look at some of them.**

KIDNEY WEEK

Hosted by American Society of Nephrology (ASN). Held in Chicago, Ill. (2016)

At the annual ASN Kidney Week, more than 13,000 kidney health care professionals from all over the world meet and present the latest advancements in nephrology and renal research. Our participation in this meeting raises awareness of PKD, establishes us as thought leaders, and lets us share and learn about the latest in PKD research, science and patient care. Our booth is visited by a variety of nephrologists, scientists and care providers.

To support physicians who treat PKD patients, every other year, we host a two-day course. Last year's *Polycystic Kidney Disease: Translating Mechanisms Into Therapy* was chaired by PKD Foundation Scientific Advisory Committee (SAC) members Benjamin Cowley Jr., M.D., (also Chairman of the PKD Foundation Board of Trustees) and John Bissler, M.D. Among the leading PKD expert presenters were SAC members York Pei, M.D., and Terry Watnick, M.D., (SAC Chair).

The PKD Foundation was inducted into the ASN Foundation for Kidney Research's Founders Circle at Kidney Week 2015. Left to right: ASN President Jonathan Himmelfarb, M.D., Jared J. Grantham, M.D., and Benjamin Cowley Jr. M.D.

JARED J. GRANTHAM SYMPOSIUM

Hosted by the Kansas University Medical Center (KUMC) Kidney Institute. Held in Kansas City, Kan.

We are the lead sponsor of the annual Jared J. Grantham Symposium, which brings together nearly 100 PKD researchers and clinicians. The meeting was first held in 2014 to celebrate the retirement of PKD Foundation and KUMC Kidney Institute Co-founder, Jared J. Grantham, M.D. Today, the Grantham Symposium continues to honor Dr. Grantham's many contributions to PKD research, while providing scientists deeply involved in PKD research with an interactive forum to discuss innovations and the latest in PKD science.

This year's Symposium included a presentation by Ronald D. Perrone, M.D., (see profile on page 3). Dr. Perrone discussed predicting disease management and progression of ADPKD using TKV and his clinical study of metformin, an FDA-approved type 2 diabetes drug that could potentially slow cyst growth and be repurposed to treat PKD.

Other presenters included Stephen Parnell, Ph.D., and Xia Julie Zhou, Ph.D., both from the University of Kansas Medical Center and 2016 PKD Foundation research grant recipients, along with Erica Golemis, Ph.D., from Fox Chase Cancer Center – Temple University, also a PKD Foundation 2014 research grant recipient.

Representing the Foundation at national meetings

David Baron P.h.D, Chief Scientific Officer (CSO)

Recently I have been invited to speak at several scientific meetings. At the One Health Innovations Symposium in Kansas City, Mo., I spoke on a panel about what PKD is and how PKD in Persian cats might be a relevant model of human ADPKD that could benefit cats and humans alike. I also spoke on the renal panel during a forum on regenerative medicine sponsored by the National Academy of Sciences in Washington, D.C.

These opportunities, especially in D.C., **put PKD on the radar as an important genetic disease that urgently needs funding.** If we can bring effective therapies to patients that might delay or avoid the need for renal replacement therapy, the quality of life of PKD patients would be improved and Medicare costs would be reduced by billions of dollars. By speaking at these meetings, I bring my perspective as a PKD scientist and patient, helping move PKD to the forefront and establishing the Foundation as a thought leader.

About PKDOC

The precursor of the ADPKD Summit was work done from 2009 to 2015 through the Polycystic Kidney Disease Consortium (PKDOC) initiative. PKDOC is a collaboration between the Foundation, C-Path (a non-profit focused on improving the drug development process), the pharmaceutical industry, PKD researchers and clinicians from four leading academic medical centers (Tufts University, University of Colorado Denver, Emory University and Mayo Clinic) and the FDA.

PKDOC was primarily funded through the PKD Foundation, an investment of more than $3 million. The goal of PKDOC was to aid clinical trial development for PKD therapies by establishing a means to monitor the progression of the disease and provide a clear regulatory pathway for the pharmaceutical industry to evaluate the effectiveness of potential treatments. In 2015, efforts of PKDOC resulted in the FDA and EMA approval of TKV as a prognostic enrichment biomarker for use in clinical trial design, which in turn led to the first ADPKD Summit.

Addressing the need for clinical endpoints* in ADPKD

Hosted by the Critical Path Institute (C-Path) and the PKD Foundation. Held in Bethesda, Md.

This summer the Foundation initiated, sponsored and helped facilitate the first ADPKD Summit, where we met with representatives from the U.S. Food and Drug Administration (FDA), the European Medicines Agency (EMA), Health Canada, the National Institutes of Health (NIH) and PKD academicians. Eight companies including biotech start-ups and large pharmaceutical corporations interested in developing therapeutic solutions for PKD also attended.

The purpose of this important meeting was to promote conversations to help set the criteria for advancing a drug through development and approval by regulatory agencies including the FDA, Health Canada and EMA. According to Dr. Baron, **"It was particularly encouraging to have multiple drug companies engaged in this process."**

For these companies, discussions like this are essential to providing clarity on the regulatory path to approval of novel treatments for PKD. The Summit also provided a forum for three major regulatory agencies to clarify their requirements for PKD drug approvals.

One of the most critical topics discussed in this Summit was the need for new and better use of existing **biomarkers*** for use in clinical trials for PKD therapies. In particular, **surrogate endpoints*** early in the course of the disease are urgently needed. The gold standard for any clinical trial of PKD is the rate of decline in estimated Glomerular Filtration Rate (eGFR) or progression to end-stage renal disease (ESRD). Because eGFR declines slowly, while kidneys are enlarging, eGFR is not a useful endpoint in the early stages of ADPKD. Unfortunately, by the time patients have shown a considerable decline in eGFR or are close to ESRD, the kidneys are already irreversibly damaged and therapies are not likely to be effective at forestalling the need for renal replacement therapy (dialysis or transplant). Research suggests that growth of total kidney volume (TKV) may be useful as a surrogate endpoint to predict the benefit of a potential therapy. See pages 8 and 9 to learn about TKV.

This is why the focus of this Summit was to evaluate and discuss TKV as a potential surrogate endpoint for PKD. TKV has already been established as a prognostic enrichment biomarker, indicating that someone who has a large TKV is more likely to go on to exhibit declining eGFR or ESRD and therefore more likely to show a drug effect. Establishing TKV or another biomarker as a surrogate endpoint would allow drug companies and regulatory agencies to use it as a marker to define whether a potential drug is effective by determining whether it is slowing or stopping cyst growth, and therefore halting or slowing TKV increase much earlier in the course of the disease. This would become an important tool for getting therapies approved for PKD that would actually prolong the time to renal failure **or actually prevent renal failure.**

WHERE WE ARE NOW

*WHAT IS A BIOMARKER?

Biomarker (Biological marker): A characteristic that is objectively measured and evaluated as an indicator of normal biologic processes, pathogenic processes or pharmacologic responses to a therapeutic intervention. Currently TKV is an approved prognostic biomarker that may be used to enrich clinical trials with patients (those with large TKV) most likely to show a response to an experimental therapeutic.

(Control Clin Trials. 2001 Oct;22(5):485-502.)

> "We still have a long way to go, but what's important is that regulators from the FDA, EMA and Health Canada were represented. We are encouraged by the interest of the regulatory agencies and their willingness to engage in dialogue." *– Dr. Perrone*

Dr. Perrone, who was instrumental in the PKDOC initiative (see sidebar), and was a member of the planning committee of the ADPKD Summit, knows how important this meeting was. **"This Summit initiated a process by which scientists, regulators, and the pharmaceutical industry can have ongoing dialogue about the regulatory path to approval for drugs for PKD."**

A manuscript with proceedings of the Summit will be written and published followed by an FDA and EMA perspective. We anticipate that this publication will encourage drug companies interested in developing therapies for PKD.

According to Dr. Baron, "The goal is to interest companies in the PKD 'space,' which is already evident by participation of the eight companies at the Summit. I'm confident that this meeting along with follow-up discussions will accelerate the development and approval of novel therapeutics for the treatment of PKD that will significantly delay or prevent the onset of renal failure."

Courtesy of KDIGO

Common elements in uncommon kidney diseases

Hosted by Kidney Disease Improving Global Outcomes (KDIGO). Held in Amsterdam, The Netherlands (2016)

KDIGO regularly holds global conferences. This year, we participated in this conference that brought together clinicians, researchers and patients to discuss common clinical and patient issues across rare kidney diseases, such as autosomal recessive polycystic kidney disease (ARPKD).

Pediatric nephrologist and Foundation Board Member Lisa Guay-Woodford, M.D., from Children's National Health System, Washington D.C., co-chaired the meeting. **"Rare kidney diseases like ARPKD, while clinically distinct, share common challenges in diagnosis and treatment.** These include small populations of affected patients, limited insights into disease mechanisms and a lack of biomarkers for monitoring disease progression (essential for designing clinical trials with new therapeutics). This conference brought together clinicians, scientists, and patient advocates to address five central issues for rare kidney diseases: diagnostic challenges, managing renal function decline and progression of chronic kidney disease, challenges in clinical study design, translating advances in research to clinical care, and providing practical and integrated patient support." From these discussions, consensus recommendations for addressing these challenges were developed and will be published.

One important aspect was the patient perspective. According to PKD Foundation Advocacy and Patient Services Manager and Kansas City Chapter Coordinator Nicole Harr, KDIGO conferences are unique because they include patients, forming a true partnership with clinicians and researchers to achieve health care that considers their choices and lifestyle. **"Conferences like these help health care professionals incorporate the patient perspective into the care they provide.** KDIGO was one of the most fascinating and impactful conferences I've been a part of," says Nicole. ■

*WHAT IS A CLINICAL ENDPOINT?

Clinical endpoint: A characteristic or variable that reflects how a patient feels or functions, or how long a patient survives. (For example: eGFR or ESRD).

*WHAT IS A SURROGATE ENDPOINT?

WHERE WE WANT TO BE

Surrogate endpoint: A biomarker intended to substitute for a clinical endpoint. A clinical investigator uses epidemiologic, therapeutic, pathophysiologic or other scientific evidence to select a surrogate endpoint that is expected to predict clinical benefit, harm, or lack of benefit or harm. (For example: Does a drug effect on TKV predict an effect to protect eGFR or to delay ESRD?)

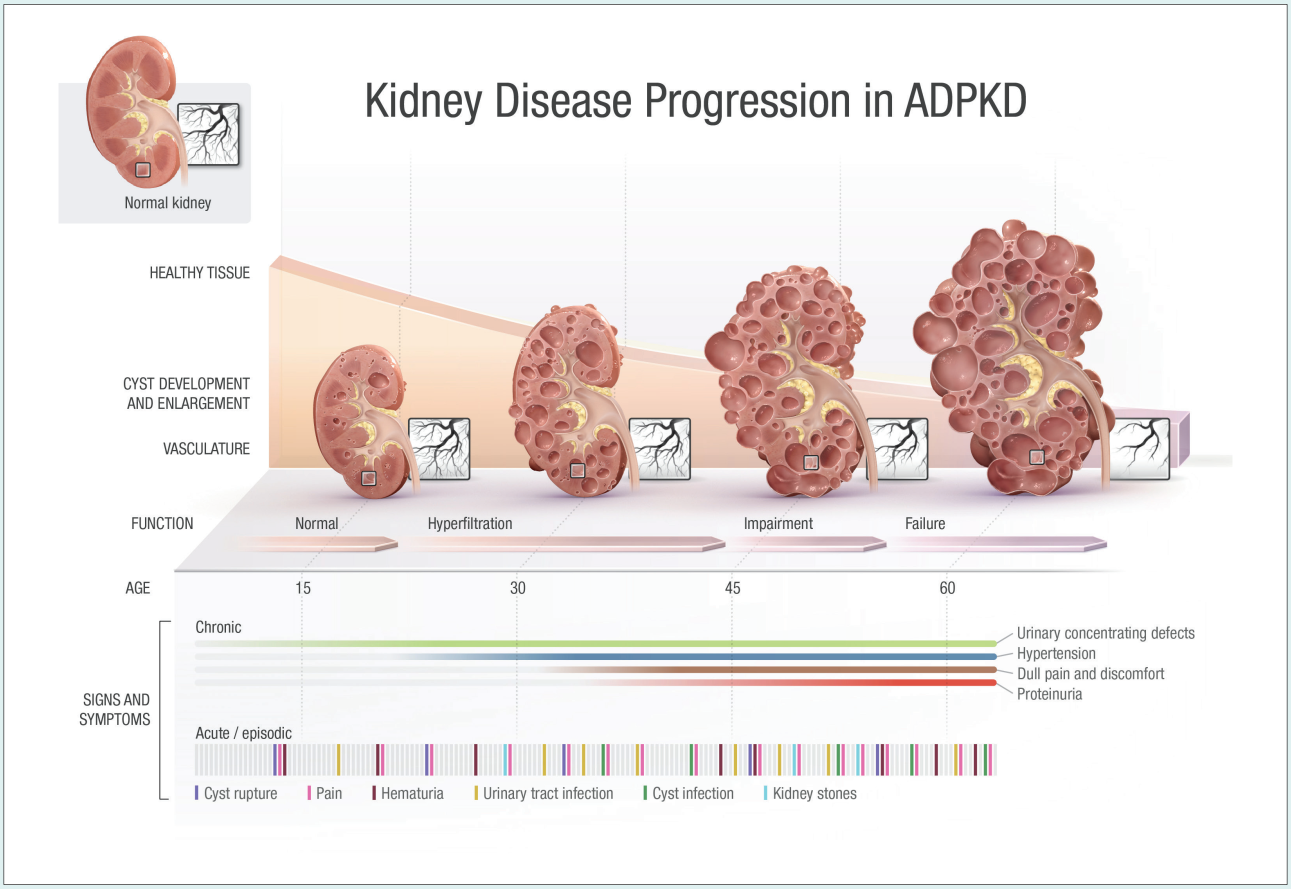
What is **total kidney volume** (TKV) and why is it important?

The total kidney volume (size) of both kidneys, or TKV, is an estimated measurement of both kidneys. TKV can be measured with imaging techniques. The most commonly used technique is ultrasound, but magnetic resonance imaging (MRI) is a far more accurate test that provides more information.

TKV is a key biomarker for PKD. According to Jared J. Grantham, M.D., Co-founder of the PKD Foundation, "the cysts are the disease." Without cysts, there would be no increasing harm to the kidneys. The importance of TKV is its relationship to kidney function, commonly measured by estimated **glomerular filtration rate (eGFR)** and progression to end stage renal disease (ESRD). Although cysts continue to grow throughout life, kidney

The relationship of TKV to kidney function and age

Kidney function declines with age and cyst growth happens over time. Around age 60 is when many ADPKD patients suffer kidney failure.



Illustrations adapted from Jared Grantham, M.D., and Vicente Torres, M.D.; etal. NEJM, 2006; 354 (20):2122-30 function generally stays in the normal range until you reach your 40s or 50s (see illustration). This means that, although your TKV increases over many years, you may still maintain good kidney function for a long time. Many experts believe that the best time to treat PKD is when kidney function is normal or near normal (despite growth in TKV) rather than when eGFR is clearly declining due to increasing damage to normal tissue from encroaching cysts. **This is why TKV could be a valuable potential surrogate endpoint for a therapy started earlier in the course of the disease.**

The FDA currently requires that PKD clinical studies use kidney function to measure the efficacy of a potential therapy (tolvaptan is one example). If the therapy slows decline in kidney function, it would probably gain FDA approval. We have been working for many years to convince the FDA that, as an alternative to eGFR, **they should consider TKV as a surrogate endpoint in early stages of PKD,** possibly together with other potential biomarkers such as urinary tract infections and pain. This would allow drug companies to target both kidney function and/or TKV as endpoints, to maintain kidney function by slowing the growth of cysts before renal function is in decline. This type of therapy would begin early in the disease, when TKV is relatively small and kidney function is still fairly normal or good, and inhibition of cyst growth could prevent or delay ESRD later in life.

> Many experts believe that the best time to treat PKD is when kidney function is normal or near normal (despite growth in TKV) rather than when eGFR is clearly declining due to increasing damage to normal tissue from encroaching cysts.

To learn more about TKV from CSO Dr. Baron, visit **pkdcure.org/Webinar-Wednesdays/tkv**

Teaming up to end PKD

Teams are the lifeblood of the Walk for PKD. Each year, teams raise approximately $1.4 million, which accounts for about 80 percent of total funds raised from the Walks. The Walk is our signature fundraising event, drawing more than 10,000 people in the PKD community and accounting for nearly one-third of the budget that funds PKD research, education, and amplifies the voices of hundreds of thousands of patients in the U.S.

Among this year's 900 Walk teams* is the Renal Avengers, formed by researchers, physicians, nurses and staff from the Kidney Institute at the University of Kansas Medical Center (KUMC). The Kidney Institute has strong ties to the PKD Foundation. Jared J. Grantham, M.D., PKD Foundation Co-founder, also founded the Institute, and is now one of its Distinguished Emeritus Professors. The Renal Avengers understand that the Walk raises critical funds for PKD research.

In their fifth year walking, Team Captain Robin Maser, Ph.D., Assistant Professor, Department of Clinical Laboratory Science, says the team looks forward to the Kansas City Walk. She and 24 of her colleagues, their families and friends joined the Renal Avengers to raise money for a cause they work for every day. "Having a Kidney Institute Walk team and raising money to support the mission of the PKD Foundation has been a great way to unite all of us. It reminds us of why we do the work that we do," says Robin.

The Walk also gives researchers and clinicians a chance to interact with patients, volunteers and others in the PKD community. "We also enjoy visiting with and updating PKD patients and their families on research at the Kidney Institute. It's invigorating to explain what we do and answer questions about research at our 'Ask the Scientist' booth," says Robin. ■

TOP 2016 TEAMS **(As of Oct. 18)*

There is still time to donate to the Walk for PKD to support PKD research, and provide education and support to those living with PKD. The Walk fundraiser is open until December 31. Visit walkforpdk.org.

WALK	TEAM NAME	AMOUNT RAISED
Pittsburgh Walk	Bost Bunch	$31,930
Tampa Walk	Ivan's Investors for a PKD Cure	$23,441
New York City Walk	Team Odyssey	$20,500
Chicago Walk	Going for Geigers	$20,150
Twin Cities Walk	Team OC	$14,550

Philanthropists of the year:

The Klingbeils are recognized for a lifetime of giving to the Foundation

For Jim and Sally Klingbeil, helping the PKD Foundation has been a longtime family passion. In recognition of the great impact of their contributions, Sally and Jim will be presented with the Philanthropists Award at the 2017 Gratitude: A Celebratory Benefit on April 22, in San Francisco. They hope this will encourage others to support the Foundation as they have for so many years. “Being honored with this award is a way to enhance this important cause.”

Sally’s mother passed from complications of PKD when Sally was 16, and each of her three deceased siblings suffered from the disease as well. Sally was diagnosed with PKD in 1983, though she considers herself fortunate that she did not have symptoms for most of her life. A kidney from an altruistic living donor in 2008, before she needed dialysis, renewed her drive to help. “Supporting the PKD Foundation became a major focus for our family. This is particularly important for Jim and me as two of our four children have PKD.” And even several years after her transplant, PKD-related heart issues still affect Sally. “My PKD story did not end with my transplant.”

When Jim learned about the disease that ran in Sally’s family, he became determined to make a difference. A successful businessperson, Jim served on the Foundation’s Board from 2000 to 2010. “With Sally and our children having PKD, we needed to do all we could to find treatments. There have been tremendous strides in research. I feel Sally and I have been a part of supporting that.”

Sally and Jim believe that helping raise awareness and giving time is as important as giving money. Their daughter Anne Ryan, who also has PKD, has been a PKD Foundation Board member since 2012.

Jim knows their support is making a difference, “There are promising treatments on the horizon, and I feel very good about the clinical studies taking place.” They both believe that “It doesn’t get any more personal than your family. If you want to make a difference for future generations, donating to the Foundation should be a priority. **We encourage people with PKD in their family to stretch as far as they can and make the Foundation their number one charity.”**

For opportunities to sponsor the Gratitude Benefit, contact Pat Smithson, Regional Development Director at 1.800. 753.2873, ext. 131. Sponsors who sign up by Jan. 13, 2017, will be listed on the invitation. Individual ticket sales begin Feb. 1, 2017.

Gratitude

A CELEBRATORY BENEFIT FOR THE PKD FOUNDATION

Fourth annual Gratitude: A Celebratory Benefit

April 22, 2017 | San Francisco

In just a few months, we will gather again at the fourth annual Gratitude: A Celebratory Benefit, this time in beautiful San Francisco. Gratitude honors philanthropists, fundraisers, volunteer leaders, researchers and physicians who have significantly contributed to the mission of the PKD Foundation and patients, helping us move toward a future without PKD.

This special event will bring together PKD community supporters from the West Coast and across the country to celebrate our 2017 honorees, strengthen relationships among our PKD community and raise critical funds to help end PKD.

Join Honorary Chairs Greg and Anne Swart and Benefit Chairs Drs. Beverly Benson and Pamela Farmer in congratulating our 2017 Gratitude honorees.

Philanthropists:
Jim and Sally Klingbeil, Sr.
Volunteers:
Katherine Michiels, MaryKatherine Michiels-Kibler
Fundraiser:
Linda Rose Koehler
Physician:
Meyeon Park, M.D., MAS, University of California, San Francisco
Researcher:
Benjamin Freedman, Ph.D., University of Washington, Seattle
Corporate partner:
Otsuka America Pharmaceutical, Inc.

To learn more about Gratitude and our honorees, visit **pkdcure.org/gratitude**

ANNUAL REPORT 2016

Together, we are making significant strides toward finding treatments and a cure, as well as improving the lives of those living with PKD. Your generous donations and volunteerism make this work possible – read about the impact you have made during fiscal year 2015-2016 (July 1, 2015-June 30, 2016).

Advancing research toward treatments

Last fiscal year we selected 15 outstanding research projects to support. The PKD Foundation Scientific Advisory Committee (SAC) reviewed 51 research grant applications to determine which ones would merit funding:

a total of **$2.4 million** to be awarded over two years.

Visit pkdcure.org/grants to learn about the researchers and their projects.

Awarded
15
research grants
2 related to PLD & **3** Related to ARPKD
1 in Canada
1 in Europe
13 in the U.S.

At the PKD National Convention 2016, Katherine M. Dell, M.D. presented her grant project, named in memory of Sophie St. Aubin and in honor of Sophia Shapiro. Dr. Dell's project, Magnetic Resonance Fingerprinting (MRF) to Assess ARPKD Kidney and Liver Disease Progression, *received the Dr. Vincent H. Gattone Research Award for the Top-rated 2016 Grant Proposal.*

Eileen Creamer O'Neill leaves a legacy for future generations by funding PKD research

Eileen Creamer O'Neill knew how devastating PKD can be. She saw her father and uncle succumb to PKD when they were in their 30's, and she was diagnosed in her 40s. In addition to PKD, she also experienced severe pain from polycystic liver disease (PLD), a common complication of PKD. She required several surgical procedures to remove liver cysts. Eileen eventually had a kidney transplant in her 50s and passed away at the age of 65 in 2010.

Eileen left behind memories of a private but caring, loyal and determined woman. She cared for her ailing mother, who had Alzheimer's disease, for 11 years. Eileen loved animals, especially cats, and often helped support animal shelters. She was a talented designer who worked for the Direct Selling Association in Washington, D.C. her whole life.

Eileen was determined to help find treatments and a cure for future generations. She started supporting the Foundation in 2002, and regularly gave generously to advance PKD research. Her legacy gift, a bequest of nearly $1.8 million to the PKD Foundation, funded several research projects related to PLD. One of our recent grants titled *Deregulated cholangiocyte autophagy: a new target for polycystic liver disease,*

Supporting research through clinical studies and tissue donation

Clinical studies are critical to the development of drugs and treatments for PKD. We have supported five potential treatments currently in clinical trials through our Accelerating Clinical Trials (ACT) program. The program alerts you to current and upcoming PKD clinical studies so you can make informed decisions about participating. Last fiscal year, we aided in recruitment by emailing and mailing information about trials for tolvaptan and niacinamide, as well as several observational studies aimed at better understanding PKD and its impact on daily life. To learn about participating in clinical studies visit pkdcure.org/clinicalstudies.

Another important piece to studying PKD is our tissue donation program. The program provides patients with an avenue to contribute to the advancement of the understanding of PKD by donating their cystic kidneys that have been removed via nephrectomy. Last fiscal year, we worked with seven patients to donate their cystic kidneys to PKD researchers and to contribute to the advancement of research.

Research Opportunity Fund: a legacy for future generations

The Foundation's Research Opportunity Fund was established by two benefactors who made generous bequests, as a way for them and others to leave their legacy for future generations. The fund is intended to provide growth-oriented, long-term investment of funds that are not needed to meet the day-to-day financial obligations of the Foundation, but to advance PKD research and therapy development, and to ensure funding to find treatments and a cure is never at risk.

Lucille and James Carnes were active in supporting the PKD Foundation since 1999. Lucille helped to start the San Antonio Chapter. She spent 13 years of her life on dialysis and died in 1994. Her husband James continued his support of the Foundation as both of his daughters were diagnosed with PKD. Before his death in 2014, James Carnes prepared a codicil to his will, and with a $1 million gift established the James M. and Lucille Kemp Carnes Family Endowment.

PKD is a disease that often devastates many members in one family. This was the case with the Duvall sisters from St. Louis, Mary Ann, Laverne and Ruth. Laverne was determined to change the legacy of future generations of Duvalls, as well as countless others suffering from PKD. She made her wishes clear with a $1.7 million bequest to the PKD Foundation, which the Board of Trustees set aside to establish the Laverne H. Duvall Endowment.

To contribute to this special fund, please email Reids@pkdcure.org.

in honor

Researchers and patients are grateful to the following who provided support for these research grants: **Estate of Eileen Creamer O'Neill, Friends and family in memory of Sophie St. Aubin and in honor of Sophia Shapiro, Karyn Waxman and family.**

led by Tetyana Masyuk, Ph.D., from Mayo Clinic, was named the Eileen Creamer O'Neill Award because of her support. This project aims to study the role of specific biological processes on the progression of PLD and to test new strategies for its treatment.

Thanks to Eileen's desire to help others avoid the struggles she faced with PKD, her legacy gift has allowed us to continue to do critical work to move us toward treatments and a cure for PKD and its associated complications.

PKD Foundation helps fund ASN Foundation for Kidney Research Fellowship Program and Jared J. Grantham Fellowship

We made a significant commitment to attract early-career scientists to the PKD field by investing in a groundbreaking fellowship program of the American Society of Nephrology (ASN) Foundation for Kidney Research. The PKD Foundation's contribution of $500,000 was generously matched with $1.5 million from the ASN to fund the Jared J. Grantham Research Fellowship in perpetuity, for **a total of $2 million.**

Visit pkdcure.org/news/jared-grantham-fellowship to learn more.

We’re making strides to the finish line through the Walk for PKD*

The Walk is our signature fundraising and public awareness event, and the largest gathering of PKD patients and supporters. **Since 2000, the Walk for PKD has raised more than $26 million.** This family-oriented event brings together people of all ages, and the Penny Kids dash is a fun way for the little ones to get excited about fundraising. The Walk for PKD creates a sense of community and provides a unique opportunity to support and honor loved ones.

**Numbers include Walks that took place in the fall of 2015 and spring 2016.*

54 WALKS

TOTAL RAISED
$1,767,169

More than
10,000
participants

1,500
Walk volunteers

Local and virtual Chapters: support when and where you need it

With more than 60 volunteer-led local Chapters and two virtual Chapters, we are there to offer patients the support they need when they need it most. Our Chapters serve their PKD communities with valuable education, and patient and fundraising support to help us continue to fulfill our mission. Visit pkdcure.org/chapters to find your local Chapter.

> *“Volunteering as Co-coordinator of the Phoenix Chapter is one of the most gratifying things I have done. The Chapter is so important to the patients in this community. It lets them know they are not alone in the journey of dealing with PKD every day. Knowing that I may be able to give some support or assistance to someone else means the world to me.”*
>
> — Terri Simon, Phoenix Chapter Coordinator

34,366
volunteer hours

a value of
$809,663

62 Chapters

Chapter events **212**

1,933
volunteers

2016 Gratitude: A Celebratory Benefit

The third annual event held in Chicago last year **raised over $320,000** and was **attended by more than 300 people.**

Honorary Chair: **Wendy Brown, M.D.**
Benefit Chairs: **Amy and Mike Manelli**
Philanthropist: **Scott Goodman**
Volunteers: **Tracee Hicks, Dave Kappas**
Fundraisers: **Jen and Pete St. Aubin**
Physician and researcher: **Arlene Chapman, M.D., Vicente Torres, M.D., Ph.D.**

Honorees Scott Goodman, Vicente Torres, Dave Kappas, Arlene Chapman, M.D., Tracee Hicks, Jen St. Aubin and Pete St. Aubin

Raised Over $378,000

Fundraising for a future without PKD

Chapter and DIY fundraising events are critical to raising the money that allows us to continue to fulfill our mission to find treatments and a cure for PKD. Last fiscal year, a total of 89 individuals organized fundraising activities that raised $378,014.14.

To learn how you can host a DIY event, visit
pkdcure.org/DIY

Chapter and DIY fundraising events

Our sincere appreciation to all who have fundraised on behalf of those living with PKD. The top five fundraisers last fiscal year were:

89
organizers

Memphis Driving Home a Cure - $98,296

Miami Kidney Casino - $68,355

Turpin Sisters Golf Tournament - $43,887

Celtic Marketing Golf Tournament - $33,060

Sacramento Corks for a Cure - $19,963

A full session at the PKD National Convention

Andrea and Walter Butler attended their first National PKD Convention

Benjamin Freedman, M.D., talks about his research.

PKD National Convention: better together at our biggest education event

The PKD National Convention 2016 gathered more than 400 PKD patients, families, volunteers and researchers to share the latest in PKD research and science, to learn together and connect with others in the PKD community. This year, Convention attendees had the opportunity to choose from 45 education sessions on topics including diet and nutrition, pain and PKD, potential new therapies, transplantation and more. Teen and young adult PKD patients, as well as parents of children with ARPKD and ADPKD, attended sessions designed for their specific needs, and had the opportunity to connect with others going through similar challenges.

> "This was one of the most wonderfully inspiring events I have ever been to, this being my first time at a PKD Convention. I learned so much and was able to meet so many awesome people. I left with hope and tons of knowledge"

291 patients

53 Chapter Coordinators

37 researchers, clinicians & fellows

20 exhibit staff

Most popular sessions at the PKD National Convention 2016:

Pain and PKD | Transplant A-Z | Managing your health as PKD progresses
Stem cells, gene editing and the future of PKD research

13 webinars
with over **2,500** attendees

90%
attendees say
easy to understand

Website visits
from more than
180 countries

*Visit **pkdcure.org/WebinarWednesdays** for past and upcoming webinars.*

Improving lives through webinars and the PKD Patient Handbook

The monthly Webinar Wednesdays (held the third Wednesday of every month at noon central) combined with evening national webinars featured 13 topics with over 2,500 attendees and more than 30,000 web views.

More than 90 percent of attendees report that the webinars are easy to understand and have helped increase their knowledge of specific topics related to PKD. The most popular topics included CRISPR gene-editing technology, potential therapies for PKD, the emotional toll of PKD and the genetics of PKD.

The PKD Patient Handbook was sent to 3,306 people to help them navigate PKD. We're grateful for the education grant from **Otsuka America Pharmaceutical, Inc.** for making this handbook possible.

21st Century Cures Act passed by the House: Making strides toward health innovation

PKD advocates continue to support the 21st Century Cures Act (H.R. 6). This legislation will spur biomedical innovation, change the way the federal health officials approve drugs and medical devices, and increase research funding for the National Institutes of Health (NIH).

Advocates shared their voice on Capitol Hill by meeting with members of Congress, using social media and sending over 600 emails in support of 21st Century Cures. The House passed the 21st Century Cures Act in July 2015. Sens. Lamar Alexander (R-TN) and Patty Murray (D-WA) are the driving forces behind the Senate health innovation package, the Senate version of 21st Century Cures. Each continues work to get final approval from Congress for this vital legislation this year.

To learn how you can advocate and to receive advocacy email alerts, visit pkdcure.org/advocacy.

Putting PKD in the spotlight

Raising awareness of PKD is an important part of the work we do. It brings attention to the urgent need for treatments and a cure for PKD and helps us raise funds to continue to advance our mission. Last fiscal year, **Chef Michael Psilakis** helped us expand our reach to bring PKD awareness to national TV. Psilakis competed on the *Food Network's Guy's Grocery Games -- All-Stars in the Aisles*, where he won $16,000 for the Foundation, his charity of choice. He also competed in *Chopped All-Stars,* coming very close to winning $75,000 for the Foundation in May 2015.

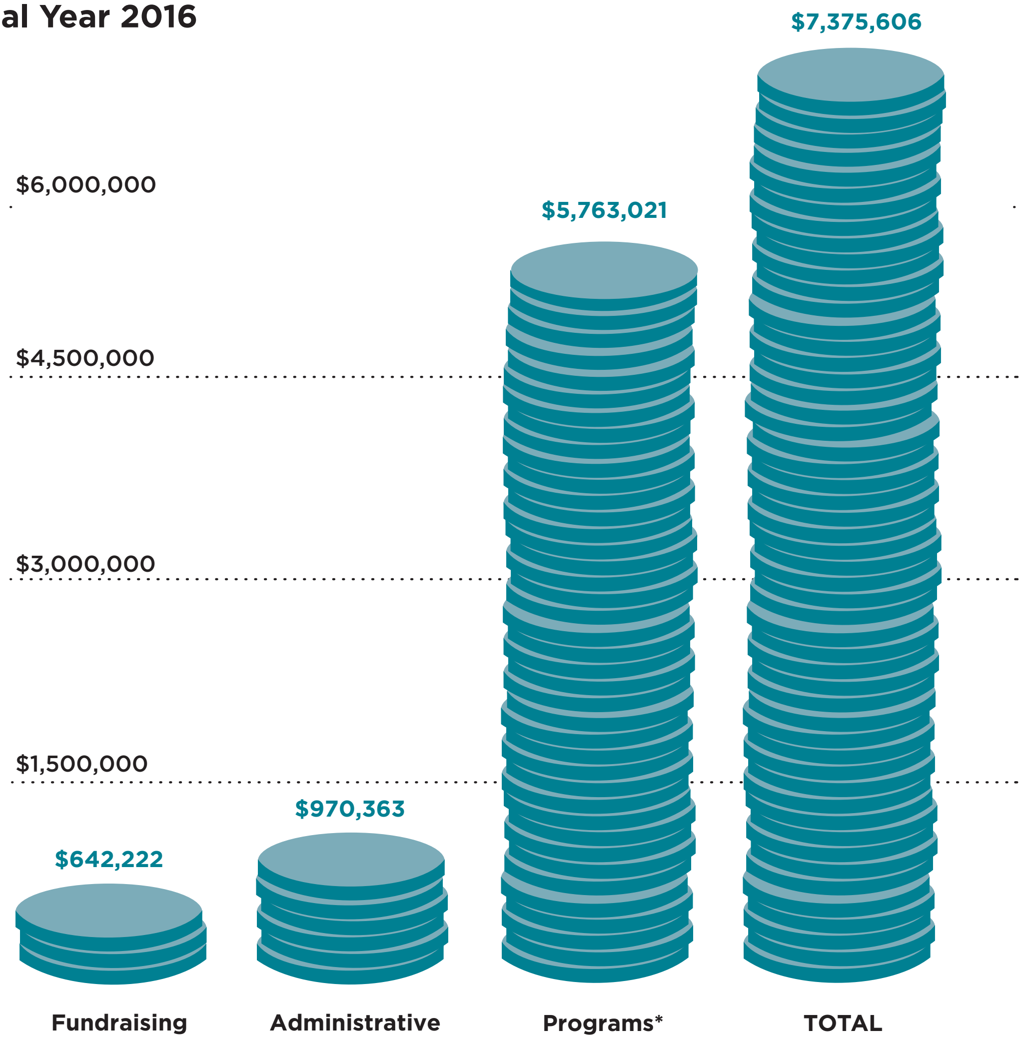
Psilakis' father died of complications of PKD, which is why the celebrity chef decided to raise awareness of PKD on a national level. "If I can raise awareness of PKD on television, I will do it. I want to engage the viewers and reach those affected by PKD." As the executive chef and owner of seven New York restaurants, Michael holds many of the culinary world's highest honors, including Food & Wine's Best New Chef and Bon Appetit's Chef of the Year distinctions.

NFL cheerleader **Kriste Lewis** also uses her spotlight to bring attention to the disease. Kriste, who has PKD, is a Saintsation for the New Orleans Saints for the third year in a row. Because of her, the Saintsations named the PKD Foundation one of their charities of choice. Kriste attends PKD fundraising events and was the emcee for the PKD National Convention 2016. She was a big force behind a successful #PKDAwarenessDay 2015 by starring in the "Do You Know PKD?" video. We reached nearly 800,000 people on Facebook and Twitter for the campaign.

To learn how you can help spread awareness,
visit **pkdcure.org/aware**

Where Your Money Went

Fiscal Year 2016

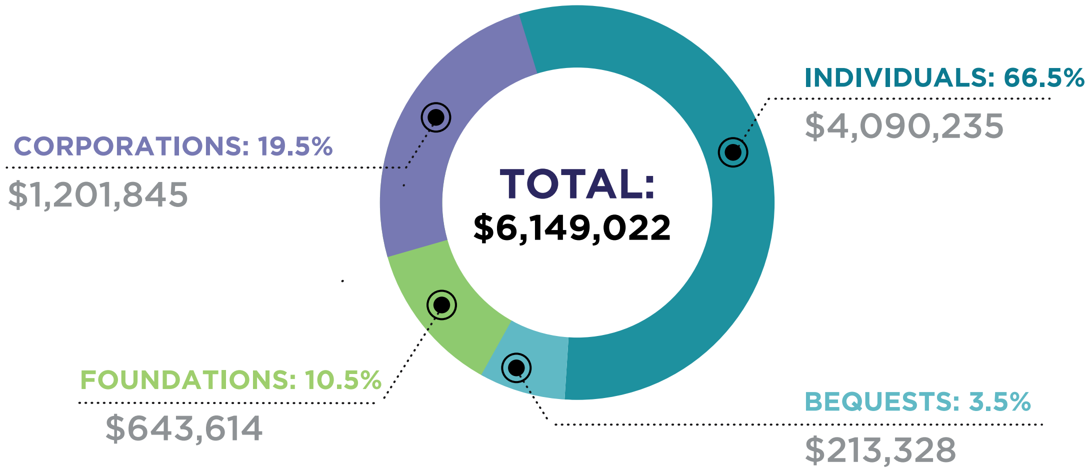


of every dollar goes to research, education, advocacy, support or awareness.

WHERE YOUR MONEY WENT

	FISCAL YEAR 2015	FISCAL YEAR 2016
Research*	$2,502,278	$2,528,864
Education & Support*	$1,657,058	$1,923,803
Awareness & Advocacy*	$1,177,939	$1,310,354
Administrative	$895,403	$970,363
Fundraising	$681,504	$642,222
TOTAL	**$6,914,182**	**$7,375,606**

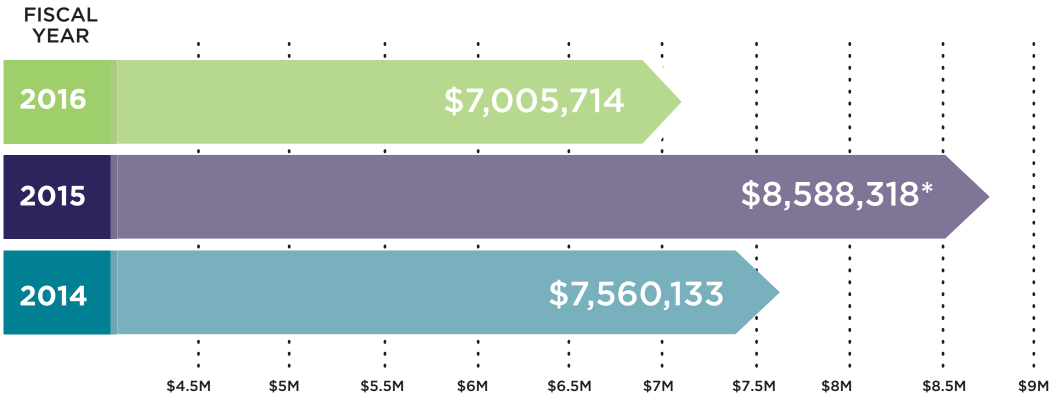
Where Our Money Came From



Please note: The above revenue figures do not include in-kind gifts.

Annual Revenues and Financial Position

Annual Revenues



Please note: Annual revenues are presented on a cash basis and include in-kind gifts.
*Total revenue in fiscal year 2015 includes a single bequest of $1 million

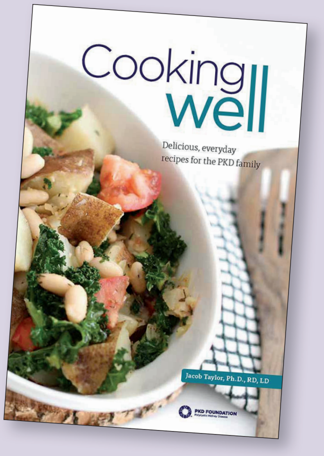
Financial Position: June 30, 2016

ASSETS		LIABILITIES		NET ASSETS
$5,695,290	–	$855,835	=	$4,839,455

View our audited financial statements at pkdcure.org/audited-financial-statements

New!
PKD cookbook now available

Get your copy for the holidays!



The holiday—and eating—season is here! Make healthy choices and find simple, delicious PKD-friendly recipes without sacrificing taste with our new cookbook, *Cooking Well: Delicious, everyday recipes for the PKD family.* Visit our online store and get your copy today. Perfect for holiday gifts!
pkdcure.org/cookbook

Bonus: *Listen to author and renal dietitian Jacob Taylor, Ph.D, discuss nutrition tips for patients and families.*
pkdcure.org/Webinar-Wednesdays/nutrition

PKD FOUNDATION
Polycystic Kidney Disease
1001 E. 101st Terrace, Suite 220
Kansas City, Missouri, 64131
pkdcure.org | pkdcure@pkdcure.org | 1.800.753.2873

NONPROFIT ORG
U S POSTAGE
PAID
KANSAS CITY, MO
PERMIT NO. 2344

facebook.com/pkdfoundation
@pkdfoundation

Please send your contact information updates or notify us of duplicate mailings of this publication by contacting donate@pkdcure.org or 1.800.PKD.CURE (753.2873), ext. 187.

Eggplant Vegetable Soup

LOW SODIUM HIGH FLUID

Servings: 12 *Serving size: 1 Cup*

INGREDIENTS

1 pound 93% lean ground turkey
1/2 cup onion, chopped
1/2 cup celery, chopped
1/2 cup carrots, chopped
1 can (28 ounces) low-sodium or no-salt-added crushed tomatoes with liquid
28 ounces low-sodium or no-salt-added beef broth
1 medium eggplant, peeled and cubed
1 clove garlic, minced
1/2 teaspoon salt
1 1/2 teaspoon ground nutmeg
1/2 cup dry macaroni
2 teaspoons dried parsley
Pepper to taste
3/4 cup grated Parmesan cheese for garnish

SERVING SUGGESTION

This hearty soup is loaded with healthy vegetables. Want it as a side dish instead? Consider making it without the meat.

NUTRITION INFORMATION

(per serving)
Calories 145
Fat 5.6 g
Carbohydrates 12 g
Protein 13 g
Dietary fiber 2.2 g
Calcium 106 mg
Phosphorous 156 mg
Sodium 252 mg
Potassium 312 mg

COOKING INSTRUCTIONS

In a pot, brown turkey. Add onions, celery, carrots, tomatoes and broth. Stir well. Add eggplant and spices; simmer for 30 minutes. Add macaroni, cook 10 minutes longer, until macaroni is done. Add parsley. Serve sprinkled with cheese.

Visit **pkdcure.org/cookbook** to get your copy of *Cooking Well: Delicious, everyday recipes for the PKD family.*

The mission of the PKD Foundation is to promote programs of research, advocacy, education, support and awareness in order to discover treatments and a cure for polycystic kidney disease and improve the lives of all it affects.