A magazine for PKD patients, Foundation supporters, health professionals and researchers.

LETTER FROM THE CEO

15 RESEARCH GRANTS AWARDED

PKD NATIONAL CONVENTION 2014 JUNE 20-22 IN KANSAS CITY

GRATITUDE BENEFIT: HONORING PKD SUPPORTERS

HELP RAISE AWARENESS FOR PKD
On the cover and throughout the magazine you’ll notice the new PKD Foundation logo. A logo leaves an immediate impression on people and conveys what an organization is about. For us to be seen as contemporary and forward looking, we needed an updated logo that was fresh and vibrant. Foundation leadership, along with feedback and guidance from PKD groups and branding experts, put a lot of thought into this design, including what concepts to show. **These included continuous energy, forward movement, interconnectedness and wholeness.** The Foundation’s revitalized brand strengthens the mission and visibility of the organization as we work to elevate awareness and stand out from the crowd.

A logo also represents an organization’s promise. The PKD Foundation promises to:

- Fund research to find treatments and a cure
- Provide education and information to empower people in managing their health
- Connect people to support each other
- Increase awareness, and support advocacy to elevate the patients’ voice
- **Improve the lives of everyone affected by PKD**

I am pleased to share that we are making bold steps toward fulfilling that promise. In this issue you’ll read about how we’re supporting discovery research and therapy development by providing grants for 15 research projects (pages 4-11). Without this money – which has been provided by generous donors – these projects could not move forward. You’ll learn how important this funding is to researchers from Dr. Greg Germino, who received a grant from the Foundation early in his career. Dr. Germino is now Deputy Director of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), part of the National Institutes of Health.

You’ll have a special chance to meet Dr. Germino, along with other preeminent PKD researchers and physicians, at the **PKD National Convention, June 20-22.** To fulfill the promise to inform, educate and connect people, we are bringing back this important event. This Convention is your chance to learn about the latest in PKD science, as well as connect with others impacted by PKD, and directly meet the researchers who have dedicated their work to treating this disease. I hope to see you in June in Kansas City, Mo., home of Foundation headquarters, for a weekend of learning and connecting. We’ll even have some fun! **Read more about it on page 13,** and go to pkdcure.org/convention to register and learn more.

These are just a few of the things we’re doing. All of our efforts work in conjunction to “Improve the lives of everyone affected by PKD.” Please keep reading to learn more about progress we’re making and how you can help support this work. Thank you for all you do to help move us closer to ending PKD.

Warm regards,

Jackie D. Hancock, Jr.
CEO
Germino started his first independent laboratory with a PKD Foundation grant in 1992 at The Johns Hopkins University. Germino is now widely recognized as one of nephrology's premier scientists. His research has helped to explain the genetic and cellular mechanisms of PKD, as well as generated numerous experimental tools and systems for its study. In the process, Germino has mentored more than 20 researchers, many of whom remain actively engaged in PKD research.

As a young investigator, Germino greatly depended on PKD Foundation funds for his lab, even when he had substantial NIH funding. With NIH funding and other sources of support so highly competitive, it is difficult for newly independent investigators to build their program, and even established researchers can struggle to maintain their operations.

Germino also notes that while the NIH does what it can to support PKD research, it has a broad mission that includes many different topics at a time while there are significant budgetary pressures. Grants from a private foundation like the PKD Foundation can provide important support through targeted funding of a specific disease, such as PKD, where every grant goes to support a project or individual working on PKD.

Private foundations also can have faster turn-around times and provide funds to meet unmet needs, like support for pilot projects and for non-permanent resident or non-U.S. citizen trainees, an important part of PKD research teams. The Foundation provided Germino support multiple times to help fund trainees and new pilot projects.

In commenting on the impact of his first PKD grant as new investigator, Germino noted, "Funding from the PKD Foundation was incredibly important for three reasons: 1) the funds doubled my annual supply budget for that year, greatly increasing my ability to build my program and take on additional staff; 2) it provided an important psychological boost: it was the first time I landed funds completely on my own; 3) it helped me to build credibility within The Johns Hopkins University."

There has been an amazing explosion of knowledge since I joined the field," Germino said. “We have terrific research tools, a vastly increased basic and clinical knowledge base, recent experience performing large clinical trials in PKD patients, ongoing dialogue with the FDA, and a PKD advocacy community that is engaged, informed and dedicated. All of this progress has been on a background of incredible growth and innovation in the general biomedical science community, which directly and indirectly benefits PKD science. And this isn’t

continued page 11…
THE REVIEWERS

The Review Committee was comprised of ten PKD Foundation Scientific Advisory Committee (SAC) members and seven additional PKD scientists. The PKD Foundation SAC oversees PKD Foundation research and medical programs aimed at developing treatments for PKD.

SAC Members – Review Committee
Terry Watnick, M.D., SAC Chair
University of Maryland School of Medicine
Stefan Somlo, M.D., SAC Vice Chair
Yale University School of Medicine
Benjamin Cowley, Jr., M.D.
University of Oklahoma Health Sciences Center
Richard Sandford, Ph.D., FRCP
University of Cambridge
Cambridge Institute of Medical Research
John Bissler, M.D.
University of Tennessee Health Sciences Center
Angela Wandinger-Ness, Ph.D.
University of New Mexico Health Sciences Center

Iain Drummond, Ph.D.
Massachusetts General Hospital
Harvard Medical School
Ronald Perrone, M.D.
Tufts Medical Center
Darren Wallace, Ph.D.
University of Kansas Medical Center
York Pei, M.D.
Toronto General Hospital
University Health Network

Additional PKD Experts – Review Committee
Michael Köttgen, M.D., Ph.D.
University of Freiburg
Michal Mrug, M.D.
University of Alabama at Birmingham
School of Medicine

Greg Pazour, Ph.D.
University of Massachusetts Medical School
Jen Pluznick, M.D.
The Johns Hopkins University School of Medicine
Norann Zahgloul, Ph.D.
University of Maryland School of Medicine
Maureen Barr, Ph.D.
Rutgers University
Peter Igarashi, M.D.
University of Texas Southwestern Health Sciences Center

The Review Committee was comprised of ten PKD Foundation Scientific Advisory Committee (SAC) members and seven additional PKD scientists. The PKD Foundation SAC oversees PKD Foundation research and medical programs aimed at developing treatments for PKD.

THE PROCESS

Each grant had two reviewers.
Each reviewer received six grants to review.

Grants ranked from 1-9 (one being strongest)

Rankings based on:
- Significance: Request for Applications specifically solicited proposals with obvious or direct potential to accelerate the development of potential therapies. Proposals focusing on childhood PKD were also encouraged.
- Innovation
- Investigator
- Environment
- Approach

The PKD Foundation grant review process is modeled on the NIH peer-reviewed paradigm.
Advancing PKD Research: PKD Foundation Awards 15 Research Grants

To facilitate therapy development and ultimately find a cure, the PKD Foundation awarded research grants to 15 PKD researchers. The Foundation will spend nearly $2.4 million over the next two years on these grants. Read project details on pages 6-11

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*related to polycystic liver disease  
**related to ARPKD and ADPKD
Research Grant Project Summaries

Dr. Vincent H. Gattone Research Award for the Top-Rated Grant Proposal
Funded by The Carl and Micol Schejola Foundation

Advancing HSP90 Inhibitors Towards Clinical Trials for ADPKD
Erica Golemis, Ph.D.
The Research Institute of Fox Chase Cancer Center
(Therapy Development)

Heat shock protein 90 (HSP90) has emerged as an important target for drug development for cancer, and HSP90 inhibitors are being tested in clinical trials for cancer. In preliminary studies, an HSP90 inhibitor was very effective in stabilizing kidney function and limiting cyst growth in a mouse model for ADPKD with a mutated PKD1 gene. In this project, mouse models will be used to extend this work in three ways: (1) evaluating whether HSP90 inhibition blocks the growth of ADPKD with a mutated PKD2 gene, (2) determining the effect of long-term dosing of an HSP90 inhibitor on kidney function and (3) determining whether the combination of an HSP90 inhibitor with another promising inhibitor, 2-deoxy-D-Glucose (2DG), further improves effectiveness in stabilizing kidney function and limiting cyst growth.

Grant Named to Honor Life of PKD Research Pioneer

Vincent H. Gattone, Ph.D., anatomist and PKD researcher, died on January 26. His research focused on renal cystic diseases, including polycystic kidney disease. He conducted initial testing of a tolvaptan precursor in rodent models of cystic disease in early 2002, which eventually led to the tolvaptan clinical trial.

Dr. Gattone was closely associated with the PKD Foundation for a number of years, serving on the Scientific Advisory Committee for the last six years. He was also the Director of the Electron Microscopy Core at Indiana University School of Medicine, one of the Foundation’s Core Labs, and a professor of anatomy and cell biology at Indiana University School of Medicine. He previously held faculty positions at Penn State University and University of Kansas Medical School. Dr. Gattone loved teaching anatomy and research ethics. He received numerous awards and distinctions, most recently the Lillian Jean Kaplan International Prize for the Advancement in the Understanding of PKD, from the PKD Foundation and International Society of Nephrology.

He is survived by his wife of 36 years, Diane, and their five children: Jennifer, Christina, Anthony, Nicholas and Catherine.

To make a tribute gift in honor of Dr. Gattone, visit pkdcure.org/donate and select the tribute option at the bottom of the page.

Key facts and definitions:
- In PKD, fluid-filled cysts develop and enlarge in both kidneys, eventually leading to kidney failure.
- ADPKD can be inherited in a dominant way from mutations in the PKD1 or PKD2 genes.
- ARPKD can be inherited in a recessive way from mutations in the PKDH1 gene.
- A channel is a way for charged particles to move across the membranes of cells.
- A cell pathway is a biochemical pathway through which cells signal each other.
- Rodent models include rats and mice with PKD.
- A drug target is a naturally existing compound in the cell the drug is meant to act on.
- It is important to note that discovery research that focuses on understanding the way in which cysts develop or enlarge in PKD must be conducted in order to identify cell compounds and pathways before therapy development can be done.
ADPKD cells are highly dependent on glucose, a simple sugar, for their energy production. 2-deoxy-D-Glucose (2DG) is a simple molecule resembling glucose that was found to slow down cyst growth in rodent models of PKD. This project will test whether or not 2DG improves the outcome in mouse models of late-onset and slowly progressive PKD that more closely resemble the human version of the disease. Furthermore, the project will look for additional changes in metabolism in the body of PKD patients.

Recent advances have led to the identification of multiple classes of drugs that have the potential to modify kidney disease progression in ADPKD. Looking forward, multiple classes of drugs have the potential to become available for treatment of ADPKD in the coming decade. However, long-term use of these medications is expected to be expensive, which poses a challenge for health care policy decision makers. This project will define the health care use and cost of ADPKD using several unique research databases that track health care use and costs in Ontario, Canada. It will also perform health economic analyses to identify the circumstances where new disease-modifying treatments offset the health care costs associated with kidney disease progression in ADPKD. This knowledge will be useful to inform health care policymakers how best to provide coverage and reimbursement for a treatment for ADPKD.

Senicapoc, a non-toxic inhibitor of the potassium channel KCNN4, will be tested as a new oral drug treatment for ADPKD. Experiments in cells show that KCNN4 function drives the renal cyst expansion believed to cause the progression of ADPKD towards renal failure. Preliminary results indicate two genetically engineered mouse models of ADPKD lacking KCNN4 show slower disease progression, and one model resembles the natural history of human PKD. The project will therefore test senicapoc’s ability to slow progression of PKD in both mouse models with intact KCNN4 genes. If senicapoc slows disease progression in these mice, then senicapoc tests in ADPKD patients can be initiated promptly, since senicapoc is known to have a lasting effect and to be non-toxic in human subjects without kidney disease.

*related to polycystic liver disease
The cyst-filling fluid secretion process, which is critical to the cyst growth of ADPKD, is dependent on two channels, CFTR and NKCC1. This project will determine how essential these channels are for cyst growth and overall kidney enlargement in mouse models of ADPKD. This project will also test (1) the effectiveness of a potential new treatment for PKD that targets cyst-filling fluid secretion processes and (2) whether decreased calcium in kidney cells results in increased CFTR and NKCC1 protein levels.

White blood cells called macrophages promote cyst growth and disease progression in PKD mouse models. Cyst cells attract macrophages primarily by making a protein called monocyte chemotactic protein -1 (MCP-1). Compelling preliminary results suggest that deleting the MCP-1 gene or blocking the macrophage infiltration with a drug (CCR2A) can slow PKD progression. This project will extend these studies in mice. Treatment to block the infiltration with CCR2A alone or in combination with tolvaptan, a drug that has shown clinical promise for PKD, will be tested. A successful outcome in these studies could facilitate the repurposing of CCR2As, alone or in combination with tolvaptan, for PKD treatment. The goal of this work is to establish new therapies for PKD that slow disease progression and relieve associated suffering.
Investigation of the Biological Basis and Therapeutic Effect of Anti-tumor Drug 11beta-dichloro on PKD**
Anna Rachel Gallagher, Ph.D.
Yale University
(Therapy Development)

Preliminary evidence that suggests the cells without the PKD1 gene can be treated with an anti-tumor drug, 11beta-dichloro, to cause cell death which would result in slower-growing cysts. This project will study the effect of this drug on (1) cysts from a mouse model of PKD1 and (2) on cysts derived from cells that lack PKDH1 (ARPKD). The goal is providing a new treatment for PKD.

Role of Tulp3 and the Hedgehog Pathway in PKD
Karel Liem, Jr., M.D., Ph.D.
Yale University
(Discovery Research)

A new mouse model has been created that develops polycystic kidney disease at birth. Studies have shown that a particular pathway is overactive in these mice. When the overactivity of the pathway is decreased, this lessens the cystic kidney disease. The project will test the role of the pathway in the new mouse model and an ADPKD-1 animal model system with both young and adult animals. Identifying the pathway and a target for drugs to treat PKD will be greatly informative to treat PKD in children and adults.

The PKD Foundation will spend nearly $2.4 million over the next two years.

The Role of Beta-catenin as Signal Integrator in PKD
Jordan Kreidberg, M.D., Ph.D.
Boston Children’s Hospital
(Discovery Research)

The exact cause of the cysts in ADPKD is not completely understood. Over the past five years, the Kreidberg laboratory at Boston Children’s Hospital has studied kidneys of mice with mutations in the same PKD genes that cause ADPKD in humans and found abnormalities in several cell pathways. Many pharmaceutical companies are developing drugs that target these same pathways, but drugs will not be developed for use in ADPKD in humans without more clarity of the abnormalities. This project will further study these pathways to more clearly define and understand the cause of the cysts.

The Effects of Genetic Reactivation of Functional Polycystins on Progression of PKD**
Ming Ma, Ph.D.
Yale University
(Discovery Research)

Recently, polycystins, important proteins in ADPKD, were shown to block signals in kidney cells that cause cyst growth. The next step is to think about reintroducing normal PKD1 or PKD2 genes back to cystic tissue and, therefore, block cyst growth. ADPKD mouse models have been made that allow us to do exactly that—re-activate normal, silenced PKD1 or PKD2 genes. This project will use these models to determine whether cyst growth can be slowed or perhaps even reversed and when is the best stage in the disease to start this intervention. It will also examine the effects of altering PKD1 expression in ARPKD models. This study serves as a proof of principle (an early stage of clinical drug development when a compound has shown potential in animal models and early safety testing) for what the expectations and goals of therapy in ADPKD may be.

**related to ARPKD and ADPKD

Meet the Researchers
Several grant awardees will be at the PKD National Convention June 20-22. Register today at pkdcure.org/convention.
The grants support projects that will inform therapy development in the future and move PKD science forward to accelerate treatments to patients.

Role of miR-17–92 in the Pathogenesis of PKD
Vishal Patel, M.D.
University of Texas Southwestern Medical Center
(Discovery Research)

MicroRNAs (miRNAs) inhibit the function of genes. Abnormal levels of miRNA expression are observed in numerous diseases, including PKD. Drugs designed to correct miRNA levels are emerging as promising new ways to treat diseases. A family of miRNAs called miR-17–92 have been identified, which promotes kidney cyst growth in mice. In this application, the aim is to determine whether miR-17–92 promotes cyst growth in mouse models of ADPKD. If successful, the study will identify miR-17–92 as a new drug target for the treatment of ADPKD.

Unifying Cystogenic Mechanism of ADPKD and ARPKD**
Feng Qian, Ph.D.
University of Maryland Medical Center: Division of Nephrology
(Discovery Research)

This project tackles the fundamental question as to how the two genes, PKD1 and PKDH1 (ARPKD), work together to ensure normal kidney development and to protect the kidney from cyst formation. Recently, it was discovered that PKDH1 mutation causes kidney disease likely by damaging the PKD1 gene function. This project will investigate how the PKDH1 gene controls PKD1 gene function during development using a mouse model. The hypothesis is that the two forms of PKD have the same disease-causing process and it is expected that this work will open new ways to identify new drug targets for therapies for both ADPKD and ARPKD.

HDAC Inhibitor Mediated Kidney Cyst Reduction
Zhaoxia Sun, Ph.D.
Yale University
(Discovery Research)

In preliminary studies, histone deacetylase (HDAC) inhibitors have been found to reduce the defects seen in ADPKD in cystic animal models. Interestingly, HDAC inhibitors (HDACIs) are already being used to treat epilepsy and cancer. Although well tolerated, they do have side effects. Tissue-specific HDACIs will be more potent, have fewer side effects and are in active development for cancer treatment, providing a rich source of candidate drugs for repurposing for PKD. This project will determine which HDACIs are most relevant to ADPKD, critical information for selecting and testing type-specific HDACIs as candidate drugs for ADPKD. It will also investigate the impact of HDACIs on other forms of PKD.

The Rosemary M. Peppet Award Phosphodiesterase/cAMP Signaling in Renal Cystogenesis (Cyst Formation) in Zebrafish
Caroline Sussman, Ph.D.
Mayo Clinic
(Discovery Research)

NOTE: This proposal uses zebrafish to screen compounds that might slow cyst development and enlargement in PKD. The zebrafish is a simple model for studying vertebrate development and genetics and is a means of

Donate PKD Kidneys to Research
PKD kidneys are needed for scientific research. Anyone wishing to donate their kidneys during a nephrectomy should contact 1.800.PKD.CURE. (Note: The kidneys need to be from a living donor to be accepted.) Please contact the PKD Foundation at least two weeks prior to the scheduled surgery, so arrangements can be made.

**related to ARPKD and ADPKD
Research has shown that increased levels of a compound called cAMP in kidney cells are an important change that occurs in ADPKD. For example, clinical trials have shown that treatments that decrease the formation of cAMP slow disease progression. Therefore, therapies decreasing cAMP formation are promising, but they do not offer a cure, nor are they well-tolerated by many patients. Phosphodiesterases are proteins that lower cAMP levels by breaking-down cAMP, so they may also affect PKD progression. This study is testing whether altering phosphodiesterase activity affects renal cyst formation using zebrafish as a model. Information gained from this research will indicate whether drugs targeting phosphodiesterases might be effective in treating PKD.

In Vivo Analysis of Cilia Mechanosensation in the Kidney
Bradley Yoder, Ph.D.
University of Alabama at Birmingham (Discovery Research)

The objective of this proposal is to analyze the role of cilia (short, hairlike organelles on the surface of cells) and the cystic kidney disease protein, polycystin-2, based on a mouse model. For this, a mouse model was generated in which green fluorescent cilia can be seen in a living animal. This study will determine whether bending of the cilia by urine flow through a small tube of the kidney in normal and cystic conditions results in events involving PKD2. It will also assess whether bending of the cilia results in changes in gene expression. The current models in which the cilia is studied with its relation to cystic kidney disease are based on cells grown on petri dishes. This study is one of the initial works to explore what cilia are doing while in a living kidney.

World–Renowned Expert continued from page 3...

all just theoretical progress: this work led to the report of one intervention that potentially has modest benefit in humans, and a host of others that delay progression of disease in relevant rodent models.”

Germino finished with, “So while we haven’t yet found the cure we all have hoped for, we have made important progress toward reaching that goal. The moral of the story is that we are not great at predicting where the next breakthrough will come from, which is why it is so important that we maintain a vibrant research community with a broad research portfolio.”

Participants Needed for Clinical Studies

Research studies give us important information to detect and treat disease – you can help by getting involved.

ADPKD Study in Atlanta Region
Eligibility: Participants must be diagnosed with ADPKD. The study is currently seeking Caucasian participants who live near the Atlanta area.
Study Purpose: To identify genetic factors that may influence the severity of ADPKD.
Contact: Sarah Nicholls at 404.712.9209 or sarah.nicholls@emory.edu.

Identifying Genetic Modifiers in PKD Clinical Research Study in Colorado
Eligibility: Men and women may be eligible for this study if they are between 18 and 70 years of age, have a diagnosis of PKD and are in Colorado or Arizona.
Study Purpose: To understand which genetic factors influence how severely an individual is affected by PKD.
Contact: Berenice Gitomer at 303.724.1685 or PKDResearch@ucdenver.edu.

Study of KD019 in ADPKD
Eligibility: Patients diagnosed with ADPKD between the ages of 18 and 55, have multiple cysts measuring at least 1 cm in size and acceptable kidney function with eGFR > 50 mL/min/1.73 m2. The study is open to anyone who can participate at one of eight clinics around the country: Boston, Los Angeles, St. Louis, New York, Cleveland, Milwaukee, Rochester, Minn. and Charlottesville, Va.
Study Purpose: To test the safety and effectiveness of the investigational drug KD019 when given to patients with ADPKD.
Contact: Maria Roche at 724.778.6125 or maria.roche@kadmon.com.

In the U.S., there are more than 40 clinical studies related to PKD. For more information, visit pkdcure.org/clinicalstudies.

To receive emails about trials (called ACT Alerts) visit pkdcure.org/email-preferences, enter your information and check the ‘Clinical Trials and Research’ button.
Organizations and Scientific Meetings Work Toward Answers for the PKD Community

**International Conference Addresses Need for Practice Guidelines for PKD**

While ADPKD is the most common genetic kidney disease, approaches to diagnosing, evaluating, preventing and treating ADPKD vary widely and there are no accepted practice guidelines for this disease. To address this and determine if guidelines in disease management can be drafted, the global not-for-profit organization Kidney Disease Improving Global Outcomes (KDIGO) held a special conference in January.

Approximately 40 clinical experts attended the meeting in Edinburgh, Scotland. This was the first Controversies Conference on PKD and the first that included a patient support group.

Co-chairs were Dr. Vicente Torres (Mayo Clinic) and Dr. Olivier Devuyst (University of Zurich, Switzerland). Dr. Torres has a long association with the PKD Foundation. In addition, PKD Foundation Scientific Advisory Committee Chair Dr. Terry Watnick attended along with other key experts associated with the PKD Foundation. PKD Foundation Board Member and Los Angeles Chapter Coordinator Dwight Odland, and Kansas City Chapter Coordinator Nicole Harr, also a PKD Foundation staff member, attended the event as patient representatives.

KDIGO Co-chair Dr. David Wheeler, University College, London said: “ADPKD is such a widespread and prevalent hereditary kidney disease that it is important for KDIGO to add to the current knowledge base. Thus we are responsible for translating the evidence we have examined into basic recommendations for care of this disease all around the world.”

The mission of KDIGO is to improve the care and outcomes of kidney disease patients worldwide through the development and implementation of global clinical practice guidelines. For more information, visit kdigo.org.

**PKD Foundation Co-founder Honored at the Grantham Symposium on “Future Directions of PKD Research”**

The Kidney Institute at the University of Kansas Medical Center will host the first annual Dr. Jared J. Grantham Symposium on “Future Directions of PKD Research” in Kansas City on May 7-9. The symposium will feature panel discussions by 13 recipients of the Lillian Jean Kaplan International Prize for Advancement in the Understanding of Polycystic Kidney Disease. The Kaplan award was established by the PKD Foundation and International Society of Nephrology through the generosity of Thomas Kaplan, in memory of his mother Lillian Jean Kaplan who was a PKD sufferer and died in 2002.

The symposium honors Dr. Jared J. Grantham, who along with Joseph H. Bruening started the PKD Foundation in 1982 with a vision to find treatments and a cure for PKD. The PKD Foundation is a sponsor of the event, which is open to all researchers interested in attending. For more information, visit kumc.edu/ki.
This June, the PKD community will converge in Kansas City for three days of relationship building, education and fun. Join us as we hear from experts in the field, including Gregory G. Germino, M.D., of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), part of the National Institutes of Health (NIH), and leading ARPKD expert Lisa Guay-Woodford, M.D.

**Top Reasons to Attend the PKD National Convention**

- Learn the latest developments in PKD from the leading clinicians, researchers and nephrology experts in the country.
- Make new friends. Connect, support and learn from others affected by PKD.
- Walk away with practical tips to manage your health, or support a loved one.
- Choose from more than 30 sessions on a variety of PKD-related topics including living with PKD, dialysis, pre- and post-transplantation, research and clinical trials and more.
- Spend time in beautiful Kansas City, with world-class shopping at the Country Club Plaza, Crown Center, historic Westport and more within minutes from the Convention hotel.

**What Past Attendees Say About the Convention**

“The Convention gives you a built-in network of resources, so if you experience something down the road, you have people to call. The wide range of information you receive is extremely helpful, much more than you could get from a single nephrologist, and on all aspects of PKD. I came away from the first National Convention with a feeling of empowerment at being armed to tackle some of the issues with PKD.”

– Heidi Cambareri

“Within six months of being diagnosed with PKD, my wife and I attended our first Convention. While I was not thrilled at having PKD, I found the meeting extremely informative and supportive. Meeting so many other people with PKD and hearing their stories was comforting. I came away knowing that the PKD Foundation was on my side and I needed to do what I could to support it.”

– Frank Condella, PKD Foundation Board of Trustees Chair

“Twelve years ago, my mother was in a coma on life support when a nurse mentioned the PKD Foundation and the Convention as a resource. That was the first sign of hope for me. With only two weeks’ notice I made arrangements to go, and attending the Convention was life-changing. I met several key doctors and one helped me to move my mother to Boston for further treatment. It is a miracle, but my mom is still alive today, and that is thanks to the PKD Foundation and the PKD National Convention.”

– Melissa McCutcheon

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Thanks to Otsuka America Pharmaceutical, Inc. and Kadmon Corporation for their sponsorship of the PKD National Convention.
Join us to Walk for PKD in 2014
Towards a Future Without PKD

The Walk for PKD is the PKD Foundation’s signature fundraising and public awareness event. The Walks are responsible for one fourth of the PKD Foundation’s budget, a budget that funds research grants, provides new education materials and elevates patients’ voices through advocacy and awareness. In 2013, more than 11,000 walkers raised nearly $1.9 million towards the fight to end PKD, with $1.4 million of the total coming from team fundraising.

Spring Walks Debut in 2014
For the first time, 2014 will include several Walks in the spring. In working with our volunteer leaders, we discovered that spring would be a better fit for events in some cities. Austin, National Capital and ShreveportWalks took place in early April. New Orleans is May 18. Visit walkforpkd.org for more information.

Penny Kids Dash
The Penny Kids Dash is a great way for children to fundraise during the Walk for PKD ... and have a lot of fun. On Walk day, kids can participate in the Penny Kids Dash—a short, fun run included as part of the day’s events. Participation in the Penny Kids Dash is open to all registered children 12 and under.

For more information on the Penny Kids Dash and fundraising activities for kids, visit walkforpkd.org.

Virtual Walk for PKD
If there is not a Walk for PKD in your area, or you are not able to attend your local Walk, the Virtual Walk for PKD is a great way to join the fight to end PKD. You can walk anytime, anywhere and with anyone you want! Pick a day that works for you, grab family or friends and hit a local park or even a treadmill at the gym. You can start a team and use the online fundraising tools on walkforpkd.org. To join the Virtual Walk, visit walkforpkd.org in May.

In 2013, walkers raised nearly $1.9 million towards ending PKD.

View video testimonies about why people and teams have united to fight PKD with the Walk for PKD at walkforpkd.org/why-i-walk.
### 2013 TeamFirst and Visionary Awards Honor Highest Fundraisers

We’re proud to recognize the extraordinary efforts of our Walk for PKD fundraisers. The more money our walkers raise, the more we can do in the fight to end PKD! We couldn’t do it without their dedication.

#### 2013 TeamFirst
The TeamFirst awards go to team captains whose teams have raised $10,000 or more.

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<thead>
<tr>
<th>City Walk</th>
<th>Team Name</th>
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<tbody>
<tr>
<td>Chicago Walk</td>
<td>The Goodmans</td>
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<td>Atlanta Walk</td>
<td>Friends of Marie Q-J</td>
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<td>Pittsburgh Walk</td>
<td>Bost Bunch</td>
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<td>New York City Walk</td>
<td>Team Kringstein</td>
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<td>Tampa Bay Walk</td>
<td>Ivan’s Investor’s for a PKD Cure</td>
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<td>New York City Walk</td>
<td>Mike &amp; Poppy’s All-Stars</td>
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<td>Hudson Valley Walk</td>
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<td>Los Angeles Walk</td>
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<td>New York City Walk</td>
<td>Team Odyssey</td>
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<td>San Diego Walk</td>
<td>Adam’s Apples</td>
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<td>New York City Walk</td>
<td>Brooke’s Bunch</td>
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<td>Helen’s Team</td>
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<td>Team Pinnacle</td>
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<td>Team AAs</td>
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<td>Houston Walk</td>
<td>Please Recycle!</td>
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<td>Boston Walk</td>
<td>Your Kidneys</td>
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<td>Rhode Island Walk</td>
<td>Levenaide</td>
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<td>Houston Walk</td>
<td>Hunting for a Cure</td>
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<td>San Francisco Walk</td>
<td>Schoch Crew</td>
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<td>Oak Island</td>
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#### 2013 Visionary
Visionaries are top fundraisers who individually raise $6,000 or more.

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<th>City Walk</th>
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<td>Chicago Walk</td>
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<td>Jean Bost</td>
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<td>Atlanta Walk</td>
<td>Jim Murphy</td>
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<td>Hudson Valley Walk</td>
<td>Michele Karl</td>
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<td>Houston Walk</td>
<td>Lisa Waxman</td>
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<td>Tampa Bay Walk</td>
<td>Harold Saul</td>
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<td>New York City Walk</td>
<td>Bill Brazel</td>
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<td>New York City Walk</td>
<td>Andrea Kringstein</td>
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<td>Houston Walk</td>
<td>Betsy Cook</td>
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<td>San Francisco Walk</td>
<td>Anne Ryan</td>
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<td>Rhode Island Walk</td>
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<td>Brian Ridingson</td>
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<td>Houston Walk</td>
<td>Pamela Cagle</td>
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#### TeamFirst Profile: Team Kringstein
For Andrea Kringstein, PKD is rampant in her family. She has PKD, her father and grandmother died from PKD, and now one of her two children, her daughter Jamie, has it. “This is why the PKD Foundation is the main charity my family is involved in,” Andrea said. “I want to prevent my kids and future generations from suffering from PKD, and the PKD Foundation is working towards finding treatments and a cure.” To read more about Andrea, visit pkdcure.org/gratitudehonorees.

#### Visionary Profile: Jim Murphy, Friends of Marie Q-J Team
With 87 team members, made up of family, friends and co-workers, Team Friends of Marie Q-J raised more than $53,000 for the Atlanta Walk for PKD. Jim Murphy personally raised more than $23,000. “Our co-worker Marie has PKD and needs a kidney,” Jim said. “I can’t donate a kidney to Marie, but I can raise awareness for PKD and organ donation. When I heard about the Atlanta Walk for PKD, I decided to mobilize the troops. Marie has a lot to live for.”
On March 6, members of the PKD community gathered at the Harvard Club in New York City to honor several individuals and a corporation for their many years of support and service. The Gratitude Benefit brought together volunteers, donors, friends, current and past board members, clinicians and researchers for an evening of celebration.

Thanks to the generous support of our sponsors and an overwhelming response from attendees to a $10,000 match offered by PKD Foundation Board of Trustees Chair Frank Condella and his wife Mary, in total the Gratitude Benefit raised **more than $100,000**.

The honorees included:
- **Physician:** Ron Perrone, M.D.
- **Corporate Philanthropist:** Select Equity Group Foundation
- **Fundraisers:** The Kringstein Family
- **Volunteers:** Heidi Cambareri
  - Robin Rothman
  - Michele Karl

**Honorary Chairs:** Retired New York Giants Offensive Coordinator, Kevin Gilbride, and Deborah Gilbride

**Chairs:** Victoria and Bill Brazell

If you would like to make a gift to the PKD Foundation in support of these honorees, please visit pkdcure.org/gratitudehonorees.

Thank you to the chairs, sponsors, guests and honorees, who like so many of you, have made the PKD Foundation what it is today.

A special thanks to our Platinum Sponsor Otsuka America Pharmaceuticals, Inc.

PKD Foundation CEO Jackie Hancock, Jr., Honoree Dr. Ron Perrone and Judy Ehrlich

Retired New York Giants Offensive Coordinator Kevin Gilbride with New York Giants Quarterback Coach Danny Langsdorf. Langsdorf donated his kidney to Gilbride’s youngest sister, who has PKD.

(L to R) Adam Schwartzfarb, Honoree Robin Rothman, Louis Collier, Jerry and Karyn Waxman

Honoree Abby Schumer (center) with her mother, Amy Epstein (right), and Guest

Honorees Andrea Kringstein and her son, Jason Kringstein
The Transplant Games of America is a multi-sport festival event for individuals who have undergone life-saving transplant surgeries. Competition events are open to living donors, organ transplant recipients, bone marrow recipients, and a limited number of corneal and tissue transplant recipients. More than an athletic event, the Transplant Games of America highlight the critical importance of organ, eye, and tissue donation, while celebrating the lives of organ donors and recipients.

The Transplant Games of America take place July 11-15 in Houston. The PKD Foundation will be there to celebrate with PKD families, transplant recipients, donors and donor families. If you are planning to participate or need more information, visit pkdcure.org/aga. You can also visit transplantgamesofamerica.org for more details and to register.

The Transplant Games of America are special to sisters JoAnn Villanueva and Suzanne Ruff, and their entire family. JoAnn has PKD, and Suzanne does not. In 2004, Suzanne donated her kidney to JoAnn. “The Games started as a way to show the world that transplants work,” Suzanne said. “It is a celebration of life as donors and recipients come together.”

Their first experience with the Games came in 1994 when their mother, Joan Gill, participated and won the bronze medal in golf in her age group. Joan received a transplant in 1988 after ten years on dialysis from PKD.

Then, in 1996, Suzanne and JoAnn’s sister, Janice Gill, competed in the Transplant Games along with their mother Joan. Janice also has PKD and received a transplant in 1995 the day before she was scheduled to start dialysis. She won a gold medal in the long jump, even though she had a cast on her leg from a stress fracture.

In 2000, Joan carried the flag for the State of Illinois team into the arena at Epcot where the Games were held, as the team member with the oldest transplanted organ.

“As a kidney recipient, I would encourage people to participate to show donor families and living donors that their gift of life has given us the opportunity to do what we dream about,” JoAnn said. “It is a way to honor the donor family. My mother and sister received kidneys from deceased donors. It is a great way to show our gratitude and say thank you.”

As a thank you to Suzanne, JoAnn swam in the 2006 games. She was nervous, but she did it. “It was the only reason I got in the pool,” she said. “She went into surgery for me, I can swim for her. It was easier to appear in a swimsuit than to honor someone who saved your life. But I had to show Suzanne what she did for me. It is another way to say thank you.”

In 2010, living donors were allowed to compete and Suzanne ran in the 5k.

JoAnn and Suzanne strongly encourage people to participate in the Transplant Games. “It doesn’t matter if you place first or last,” Suzanne said. “The cheers around you are as loud for last as they are for first. You don’t have to be an incredible athlete to participate. Being in front of the fans cheering you on is the best way to show gratitude and that organ donation works.”

Suzanne writes about her family’s battle with PKD and specifically, the 2006 Transplant Games, in her book, The Reluctant Donor. To purchase, visit pkdcure.org/reluctantdonor and 20 percent of sales will go to the PKD Foundation.
Help Raise Awareness for PKD

By sharing your story, you can help put a face and a voice to this widely unknown disease. The more people who know about PKD, the closer we can get to treatments and a cure. Every day is an opportunity to create awareness about PKD, and there are countless ways to spread the word.

Share Your Story on Voices of PKD
Share your personal journey with PKD, read about others’ experiences or download a flyer to share with family and friends.

pkdcure.org/voicesofpkd

Be social
Follow us on Facebook and Twitter. Help us increase our reach by inviting friends, co-workers and family members to follow us too. Then post, tweet and repost content from the Foundation to inform people who don’t know about PKD.

Connect online through PKD Discussion Forums
The PKD Online Discussion Forums allow you to connect with others in the PKD community. This feature on our website lets you ask questions and share stories and experiences with others. It is a place to connect with others who understand.

Some discussions have already started, including:
- living with PKD
- PKD parents
- caregivers
- dialysis and transplantation

Join the conversation today.

pkdcure.org/pkdconnection

Wear it with Pride

Back by popular demand, the PKD Foundation Online Store is now open for business. Nearly 20 items are available for purchase and can be shipped to your home.

Products include:
- t-shirts
- hooded sweatshirts
- hats
- pens
- tote bags
- tumblers
- car clings
- pill cases
- bracelets
- buttons
- lapel pins

Items are available with the PKD Foundation logo and some have the END PKD logo.

Proceeds will go to support the Foundation’s mission. Personal checks and credit cards (Visa® and MasterCard®) are accepted. Orders placed standard delivery are shipped via UPS Ground, and items usually ship one to two days after the order is placed.

pkdcure.org/store
The new logo positions the PKD Foundation as a contemporary, forward-looking organization. The new brand will strengthen the mission and visibility of our organization as we work to elevate awareness. More importantly, it will distinguish us as the leader in the fight to end polycystic kidney disease.

The logo design was inspired by the circular cells in our body, the sun’s radiating strength, and the wholeness, comfort and forward movement of a circular form. It also conveys a sense of community – individual segments intersecting and interlocking.
Kansas City Here I Come!
Join us at the PKD National Convention
June 20-22, 2014

Learn from leaders in the fight against PKD, including doctors, researchers and nephrologists from Mayo Clinic, George Washington University, Children’s Mercy Hospital and the University of Oklahoma.

Connect and re-energize in Kansas City, the home of the PKD Foundation. Whether you are a PKD patient, caregiver or family member, you will walk away from the Convention with new connections, friends and practical tips for managing your health or supporting a loved one.

See page 13 for more information or go to PKDCURE.ORG/CONVENTION and register today.