Program

# Welcome

We're better together

PKD NATIONAL CONVENTION 2016

# A letter from our CEO

I want to welcome you to the PKD National Convention 2016. The goal of this event is to fulfill the Foundation's promise to inform, educate and connect you with others in the PKD community. We're glad you are here, and we're excited for an amazing weekend, surrounded by the magic of Walt Disney World® Theme Parks.

Whether you are a PKD patient or a caregiver, we're glad to offer you a chance to learn the latest in PKD research. While you are here, I hope you will connect with others affected by PKD, get tips for becoming an advocate for your own health care, and meet the doctors and researchers dedicated to helping PKD patients as they work tirelessly to find treatments and a cure.

Thank you for joining us! I'll be here all weekend and hope to have a chance to visit with you. We all share a common goal: to end PKD. The path to doing so is a journey that requires many hands and many people. We're truly better together. Thank you for all you do to move us closer to ending PKD.

Sincerely,

Jackie Hancock, Jr., CFRE
PKD Foundation CEO

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# General schedule at a glance

## Friday, June 24

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|---|---|
| 10:00 a.m. | **Registration opens** |
| 2:15 p.m. – 2:45 p.m. | **Opening session** |
| 3:00 p.m. – 4:00 p.m. | **Breakout sessions** |
| 4:00 p.m. – 4:15 p.m. | **Break** |
| 4:15 p.m. – 5:15 p.m. | **Breakout sessions** |
| 5:15 p.m. – 7:00 p.m. | **Reception; exhibit hall opening; research poster presentations** |
| 7:00 p.m. – 9:30 p.m. | **Celebration dinner** |

## Saturday, June 25

| | |
|---|---|
| 8:00 a.m. – 9:00 a.m. | **Exhibit hall open; store open** |
| 9:00 a.m. – 9:50 a.m. | **Breakout sessions** |
| 9:50 a.m. – 10:00 a.m. | **Break** |
| 10:00 a.m. – 10:50 a.m. | **Breakout sessions** |
| 10:50 a.m. – 11:10 a.m. | **Exhibit hall open; store open** |
| 11:10 a.m. – 12:00 p.m. | **Breakout sessions** |
| 12:00 p.m. – 1:00 p.m. | **Clinical trials — why *you* should participate (lunch served)** |
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| 3:00 p.m. – 3:20 p.m. | **Exhibit hall open; store open** |
| 3:20 p.m. – 4:10 p.m. | **Breakout sessions** |
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| 4:20 p.m. – 5:15 p.m. | **Breakout sessions** |

## Sunday, June 26

| | |
|---|---|
| 8:30 a.m. – 9:00 a.m. | **Store open** |
| 9:00 a.m. – 10:00 a.m. | **Advocacy and you** |
| 10:00 a.m. – 11:00 a.m. | **Help us change our world: how you can make a difference (brunch served)** |
| 11:00 a.m. – 12:00 p.m. | **Q&A with the docs** |

# Friday, June 24

10:00 a.m. - 9:00 p.m. **Registration**
South Registration Counter

2:15 p.m. - 2:45 p.m. **Opening session: welcome to the Convention**
Fiesta 5

3:00 p.m. - 4:00 p.m. **Learn the facts: the basics of ADPKD and ARPKD**
Fiesta 1-4

**Terry Watnick, M.D.*+** - *University of Maryland*
**John Bissler, M.D.+** - *St. Jude Children's Research Hospital and Le Bonheur Children's Hospital*

Whether you've just been diagnosed or are looking for a refresher on the basics of PKD, attend this session to learn what you need to know about the clinical and genetic aspects of autosomal dominant polycystic kidney disease and autosomal recessive polycystic kidney disease.

3:00 p.m. - 4:00 p.m. **Insurance issues for families affected by PKD: financial security for your loved ones**
Coronado C-E

**Charles J. Newman, CLU**
*The Charles J. Newman Co, LLC*

In this session, you will learn about life, health, disability, long-term care and other insurance options as well as planning strategies for families affected by PKD.

3:00 p.m. - 4:00 p.m. **Transplant A-Z**
Yucatan 1-3

**Patrick Dean, M.D.**
*Mayo Clinic, Rochester*

This session will walk you through the transplantation process, from end-stage renal disease to living with your new kidney.

4:00 p.m. - 4:15 p.m. **Break**

4:15 p.m. - 5:15 p.m. **Pain and PKD**
Coronado C-E

**Theodore Steinman, M.D.**
*Beth Israel Deaconess Medical Center, the Brigham and Women's Hospital, Harvard Medical School*

Attend this session to learn how you can maximize relief from pain with a focus on drug-free approaches to pain management.

4:15 p.m. - 5:15 p.m. **Transplant update: the UNOS perspective**
Yucatan 1-3

**David K. Klassen, M.D.**
*United Network for Organ Sharing (UNOS)*

Learn about trends in national data on kidney transplantation, and get specific information about the kidney allocation system (implemented in December 2014) and its impact on PKD patients.

* Board of Trustees Member + Scientific Advisory Committee Member

## Friday, June 24

5:15 p.m. - 7:00 p.m.

### Reception, exhibit hall opening and research poster presentations

Fiesta 6

Join us to mingle with old and new friends over a drink from the cash bar. Visit with the researchers, view their poster presentations (see page 23) and learn about their work sponsored by the PKD Foundation Research Program. Visit the exhibit hall and check out our exhibitors and sponsors (see page 21).

7:00 p.m. - 9:30 p.m.

### Celebration dinner

Fiesta 5

**Shuvo Roy, Ph.D.** - *University of California, San Franscisco*
**Kriste Lewis** - *NFL New Orleans Saintsations*
**Suzanne Ruff** - *Author of the* Reluctant Donor
**David Baron, Ph.D.** - *PKD Foundation*
**Jackie Hancock, Jr., CFRE** - *PKD Foundation*

Join us for a PKD-friendly dinner to hear from keynote speaker, Shuvo Roy, Ph.D., the co-inventor of the artificial, implantable kidney, and help us celebrate being together at the PKD National Convention 2016 as we honor our Chapters, donors, researchers and doctors dedicated to ending PKD.

Patron Sponsor

# Roger L. Kohn Fund

Roger L. Kohn and Kay M. Gilbert

As long-time attendees, we find these conferences to be invaluable sources of medical information and support from professionals and the PKD community. We welcome all new and returning attendees—you'll be glad you came.

# Saturday, June 25

| | | |
|---|---|---|
| 8:00 a.m. - 9:00 a.m. | **Exhibit hall open; store open** | Fiesta 6 |
| 9:00 a.m. - 9:50 a.m. | **Tesevatinib in ADPKD** (Session sponsored by Kadmon)<br>**Anjay Rastogi, M.D., Ph.D.**<br>*University of California, Los Angeles*<br>Cyst formation in PKD is driven by a protein called epidermal growth factor receptor (EGFR). Kadmon is starting clinical trials on a drug called tesevatinib that is an effective EGFR inhibitor. Join Kadmon in this session to learn more. | Fiesta 1-4 |
| 9:00 a.m. - 9:50 a.m. | **Achieving reproductive success: innovative treatments to help you plan for the future**<br>**Tanmoy Mukherjee, M.D.**<br>*Reproductive Medicine Associates of New York*<br>If you have PKD and are considering your future reproductive goals, you may have many questions and concerns. In this session, you will learn about preimplantation genetic diagnosis (PGD), what it is, its role in the treatment of PKD and what you can expect from this test. | Coronado C-E |
| 9:00 a.m. - 9:50 a.m. | **Nutrition 101: how to optimize your diet through all stages of PKD**<br>**Jacob Taylor, Ph.D., R.D., L.D.**<br>*University Medical Center Groningen*<br>Whether you are living with PKD, have end-stage renal disease or are on dialysis, your diet and nutrition require special care and consideration. Attend this session to learn tips and tricks for making healthy choices in all stages of PKD. | Yucatan 1-3 |
| 10:00 a.m. - 10:50 a.m. | **The importance of self care: caring for yourself as you care for someone with PKD**<br>**Renee Dean, LMFT**<br>*Private Practice, San Francisco*<br>Caring for someone with PKD can take a toll on your own health. In this session, we will discuss self care, signs that may show you need more of it, and how to craft your own self-care plan based on your individual needs. | Fiesta 1-4 |
| 10:00 a.m. - 10:50 a.m. | **Managing your health as PKD progresses**<br>**Benjamin D. Cowley, Jr., M.D.*+**<br>*University of Oklahoma Health Sciences Center*<br>Prepare now for what may come in the future. This session will cover the stages of chronic kidney disease for a PKD patient, what labs you'll need to have and how to take care of yourself in each stage of PKD. | Coronado C-E |
| 10:00 a.m. - 10:50 a.m. | **Stem cells, gene editing and the future of PKD therapy**<br>**Benjamin Freedman, Ph.D.**<br>*University of Washington*<br>Recent years have seen the introduction of powerful new biomedical technologies like stem cells and gene editing. The PKD Foundation is supporting research in these areas, which have the potential to transform how kidney medicine is practiced. In this session, we will discuss how these technologies can be used to understand PKD today and to develop the treatments of tomorrow. | Yucatan 1-3 |

# Saturday, June 25

10:50 a.m. - 11:10 a.m. **Exhibit hall open; store open** Fiesta 6

11:10 a.m. - 12:00 p.m. **Signs of end-stage renal disease** Fiesta 1-4

**Vishal Patel, M.D.**
*University of Texas Southwestern Medical Center*
End-stage renal disease (ESRD) has many signs and symptoms, some that you might not even realize. Attend this session to learn what you need to know to recognize and get relief from the symptoms of ESRD.

11:10 a.m. - 12:00 p.m. **Unusual genetic presentations of PKD - spontaneous mutation, early onset PKD, etc.** Coronado C-E

**Berenice Reed-Gitomer, Ph.D.**
*University of Colorado Anschutz Medical Campus*
Are you the first in your family to have PKD? Or is your PKD different from what others in your family have experienced (more extreme symptoms, early or late onset, etc.)? This session will discuss how the genetics of PKD may vary, causing your experience to be different from others', even in your family.

11:10 a.m. - 12:00 p.m. **Beyond the kidneys: how PKD affects your body** Yucatan 1-3

**Terry Watnick, M.D.*+** - *University of Maryland*
**Neera Dahl, M.D.** - *Yale University School of Medicine*
**Michal Mrug, M.D.+** - *University of Alabama at Birmingham*
**Benjamin D. Cowley, Jr., M.D.*+** - *University of Oklahoma Health Sciences Center*
PKD is not just a disease of the kidneys. In this session, we will discuss some of the non-kidney complications associated with PKD, including aneurysms, effects on the liver (including PLD), gastrointestinal system and heart, and what you can do to stay as healthy as possible while living with PKD.

12:00 p.m. - 1:00 p.m. **Clinical trials - why *you* should participate (lunch served)** Fiesta 5

**Ron Perrone, M.D.+** - *Tufts Medical Center*
**John Bissler, M.D.+** - *St. Jude Children's Research Hospital and Le Bonheur Children's Hospital*
Patient participation in clinical studies and trials is imperative to the therapy development process. Dr. Ron Perrone and Dr. John Bissler will discuss why this is and things to consider when signing up for a study.

1:10 p.m. - 2:00 p.m. **A discussion with a clinical study coordinator** Fiesta 1-4

**Debbra A. Grier RN, BSN, MBA, CCRP**
*University of Pennsylvania*
Interested in participating in a clinical trial but not sure where to start? Attend this session to learn what a clinical trial is, how it works, and the different phases and types of clinical trials. You will also learn about your rights as a patient if you choose to participate in a clinical trial.

* Board of Trustees Member + Scientific Advisory Committee Member

## Saturday, June 25

1:10 p.m. - 2:00 p.m.

**Nuts and bolts of dialysis** — Coronado C-E

**Benjamin D. Cowley, Jr., M.D.*+**
*University of Oklahoma Health Sciences Center*
We all hope to avoid it, but some of us will need dialysis. Learn about your options and how to prepare for dialysis.

1:10 p.m. - 2:00 p.m.

**Managing blood pressure in adults and teens with PKD** — Yucatan 1-3

**Michal Mrug, M.D.+** - *University of Alabama at Birmingham*
**Melissa A. Cadnapaphornchai, M.D.** - *Children's Hospital Colorado on Anschutz Medical Campus*
Recent studies have offered insights into best practices for blood pressure control in teens and adults with PKD. Join this session to learn the latest.

2:10 p.m. - 3:00 p.m.

**Living Kidney Donor Network: having your donor find you!** — Fiesta 1-4

**Harvey Mysel**
*Living Kidney Donor Network*
Learn about living kidney donation and how to effectively communicate your need for a kidney transplant to family members and friends. You will leave this workshop informed, empowered and inspired to pursue living donation.

2:10 p.m. - 3:00 p.m.

**Why don't they call it polycystic liver and kidney disease?** — Coronado C-E

**Neera Dahl, M.D.**
*Yale University School of Medicine*
If you're over the age of 45, living with large liver cysts may be all too familiar to you. Come to this session to review the following issues: factors affecting growth of liver cysts, risks and benefits of various treatment options, and when liver cyst pain is worrisome.

2:10 p.m. - 3:00 p.m.

**Nephrectomy panel: if, when and how** — Yucatan 1-3

**Terry Watnick, M.D.*+** - *University of Maryland (moderator)*
**Stephen T. Bartlett, M.D.** - *University of Maryland School of Medicine*
**Michael Angelis, M.D.** - *Florida Hospital Transplant Institute*
**Ron Perrone, M.D.+** - *Tufts Medical Center*
As a PKD patient, you may be faced with the decision to remove your cystic kidneys. This session will bring together an expert panel to consider and debate this procedure from all perspectives.

3:20 p.m. - 4:10 p.m.

**Patient panel: inspiring stories of empowerment from patients like you** — Fiesta 7&8

**Angela Connelly,** - *PKD Foundation (moderator)*
**Bill Brazell, Kriste Lewis, Jill Riester, Lewis Wolk** *(panelists)*
In this panel discussion with PKD patients, you will meet others like you. Hear their stories, struggles and how they find the support and courage to overcome them.

# Saturday, June 25

3:20 p.m. - 4:10 p.m. **Living a full life while on dialysis** Fiesta 1-4

**Nancy Feeley, CRNP, MS**
*Johns Hopkins Medicine*

Is it possible to maintain a positive attitude and high quality of life while on dialysis? Yes, it is! Learn techniques that you can incorporate at work, while traveling and in your day-to-day life.

3:20 p.m. - 4:10 p.m. **Don't let stress mess with your kidney health** Coronado C-E

**Margot Troutt Keys, LCSW**
*Courageous Growth Counseling, Philadelphia*

Learn why your mental and emotional health are important to how you manage life with PKD.

3:20 p.m. - 4:10 p.m. **Communicating about PKD with patients, providers and families: unique struggles in the PKD experience** Yucatan 1-3

**Katharine J. Head, Ph.D.**
*Indiana University–Purdue University Indianapolis*

PKD is a complex disease with a unique illness experience. From a health communication perspective, we know that the way patients communicate about their disease and the messages they receive from others are important components in their illness experience. In this session, we will present the results of a national survey of ADPKD patients that examined the unique struggles and communication challenges patients face as they deal with PKD.

4:10 p.m. - 4:20 p.m. **Exhibit hall open; store open**

4:20 p.m - 5:15 p.m. **Get the health care you deserve: tips for effective communication with your health care team** Coronado C-E

**Margot Troutt Keys, LCSW** - *Courageous Growth Counseling, Philadelphia*
**Debbra A. Grier RN, BSN, MBA, CCRP** - *University of Pennsylvania*

Are you anxious every time you have a doctor's appointment? Do you leave your appointments with a feeling you did not get your questions answered? In this session, you will learn how to create a partnership with your health care providers so you can get the health care you need and deserve.

4:20 p.m. - 5:15 p.m. **Emerging therapies** Yucatan 1-3

**Ron Perrone, M.D.+**
*Tufts Medical Center*

Learn about PKD therapies in the pipeline, where they are in development and what you need to know about them. Dr. Perrone will cover current clinical trials and the status of emerging therapies including: tolvaptan, pioglitazone, tesevatinib, metformin, niacinamide and more.

# Sunday, June 26

| | | |
|---|---|---|
| 8:30 a.m. - 9:00 a.m. | **Store Open** | Fiesta 6 |
| 9:00 a.m. - 10:00 a.m. | **Advocacy and you**<br>**Hon. Karen L Thurman Member of Congress 1993-2003**<br>*Former U.S. Representative, Florida*<br>Everyone is an advocate! PKD patients, family and friends know better than anyone what it is like to live with PKD. Members of Congress depend on their constituents to bring important information to their attention. One personal story and one relationship can make a significant difference. | Fiesta 5 |
| 10:00 a.m. - 11:00 a.m. | **Help us change our world: how you can make a difference (brunch served)**<br>**Bill Brazell**<br>**PKD Foundation Volunteers**<br>PKD patients, advocates and volunteers share their experiences and discuss how you can get involved in the fight to end PKD. | Fiesta 5 |
| 11:00 a.m. - 12:00 p.m. | **Q&A with the docs**<br>**Terry Watnick, M.D.*+** - *University of Maryland*<br>**Benjamin D. Cowley, Jr., M.D.*+** - *University of Oklahoma Health Sciences Center*<br>**Ron Perrone, M.D.+** - *Tufts Medical Center*<br>Everything you wanted to know about PKD but were (previously) afraid to ask. | Fiesta 5 |

> "The information and the people of the Foundation are such a great resource, and you'll invariably meet someone in similiar circumstances to your own. You don't have to do this alone."
> -Matt Mainolfi

* Board of Trustees Member + Scientific Advisory Committee Member

# Parent schedule at a glance

## Friday, June 24

| | |
|---|---|
| 10:00 a.m. | **Registration opens** |
| 2:15 p.m. – 3:30 p.m. | **Welcome and introductions** |
| 3:30 p.m. – 3:40 p.m. | **Break** |
| 3:40 p.m. – 5:15 p.m. | **Education session** |
| 5:15 p.m. – 7:00 p.m. | **Reception; exhibit hall opening; research poster presentations** |
| 7:00 p.m. – 9:30 p.m. | **Celebration dinner** |

## Saturday, June 25

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|---|---|
| 8:00 a.m. – 9:00 a.m. | **Exhibit hall open; store open** |
| 9:00 a.m. – 10:45 a.m. | **Education session** |
| 10:45 a.m. – 11:00 a.m. | **Exhibit hall open; store open** |
| 11:00 a.m. – 12:00 p.m. | **Education session** |
| 12:00 p.m. – 1:00 p.m. | **Clinical trials — why *you* should participate (lunch served)** |
| 1:00 p.m. – 2:00 p.m. | **Education session** |
| 2:00 p.m. – 2:30 p.m. | **Exhibit hall open; store open** |
| 2:30 p.m. – 5:15 p.m. | **Education sessions** |

## Sunday, June 26

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|---|---|
| 8:30 a.m. – 9:00 a.m. | **Store open** |
| 9:00 a.m. – 10:00 a.m. | **Advocacy and you** |
| 10:00 a.m. – 11:00 a.m. | **Help us change our world: how you can make a difference (brunch served)** |
| 11:00 a.m. – 12:00 p.m. | **Parent to parent Q&A** |

# Friday, June 24

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|---|---|---|
| 10:00 a.m. - 9:00 p.m. | **Registration** | **South Registration Counter** |
| 2:15 p.m. - 3:30 p.m. | **Welcome and introductions**<br>**Julia Roberts* and Michele Karl***<br>*PKD Parents Chapter Co-coordinators* | **Coronado F&G** |
| 3:30 p.m. - 3:40 p.m. | **Break** | |
| 3:40 p.m. - 5:15 p.m. | **Basics and clinical care of ARPKD and ADPKD in children**<br>**Lisa Guay-Woodford, M.D.*** - *Children's National Health System, The George Washington University*<br>**Jeffrey Saland, M.D.** - *Mount Sinai Hospital*<br>Join us for an overview of the clinical and genetic aspects of PKD. We will then dive into the clinical care of children with autosomal recessive polycystic kidney disease and autosomal dominant polycystic kidney disease. | **Coronado F&G** |
| 5:15 p.m. - 7:00 p.m. | **Reception, exhibit hall opening and research poster presentations**<br>Join us to mingle with old and new friends over a drink from the cash bar. Visit with the researchers, view their poster presentations (see page 23) and learn about their work sponsored by the PKD Foundation Research Program. Visit the exhibit hall and check out our exhibitors and sponsors (see page 21). | **Fiesta 6** |
| 7:00 p.m. - 9:30 p.m. | **Celebration dinner**<br>**Shuvo Roy, Ph.D.** - *University of California, San Franscisco*<br>**Kriste Lewis** - *NFL New Orleans Saintsations*<br>**Suzanne Ruff** - *Author of the* Reluctant Donor<br>**David Baron, Ph.D.** - *PKD Foundation*<br>**Jackie Hancock, Jr., CFRE** - *PKD Foundation*<br>Join us for a PKD-friendly dinner to hear from keynote speaker, Shuvo Roy, Ph.D., the co-inventor of the artificial, implantable kidney, and help us celebrate being together at the PKD National Convention 2016 as we honor our Chapters, donors, researchers and doctors dedicated to ending PKD. | **Fiesta 5** |

# Saturday, June 25

| | | |
|---|---|---|
| 8:00 a.m. - 9:00 a.m. | **Exhibit hall open; store open** | **Fiesta 6** |
| 9:00 a.m. - 10:45 a.m. | **Congenital hepatic fibrosis and other non-renal manifestations of ADPKD and ARPKD in children**<br>**Ryan Fischer, M.D.**<br>*Children's Mercy Hospital, Kansas City*<br>What's got you worried, what's got me worried: a look at congenital hepatic fibrosis and other non-renal manifestations of ADPKD and ARPKD in children from the doctor's and the patient's perspective. | **Coronado F&G** |
| 10:45 a.m. - 11:00 a.m. | **Exhibit hall open; store open** | **Fiesta 6** |
| 11:00 a.m. - 12:00 p.m. | **Nutrition and PKD: why diet matters and what parents can do to help their kids**<br>**Jacob Taylor, Ph.D., R.D., L.D.**<br>*University Medical Center Groningen*<br>This session will discuss the suspected link between diet and PKD progression, the published research and practical tips that parents can use to improve their child's diet. | **Coronado F&G** |
| 12:00 p.m. - 1:00 p.m. | **Clinical trials - why *you* should participate** (lunch served)<br>**Ron Perrone, M.D.+** - *Tufts Medical Center*<br>**John Bissler, M.D.+** - *St. Jude Children's Research Hospital and Le Bonheur Children's Hospital*<br>Patient participation in clinical studies and trials is imperative to the therapy development process. Dr. Ron Perrone and Dr. John Bissler will discuss why this is and things to consider when signing up for a study. | **Fiesta 5** |
| 1:00 p.m. - 2:00 p.m. | **Dialysis and transplantation in children**<br>**Jeffrey Saland, M.D.**<br>*Mount Sinai Hospital*<br>Dialysis and transplantation greatly affect children's lives. This session will cover everything you need to know as a parent for when your children reach these stages. | **Coronado F&G** |
| 2:00 p.m. - 2:30 p.m. | **Exhibit hall open; store open** | **Fiesta 6** |
| 2:30 p.m. - 3:30 p.m. | **ARPKD research update**<br>**Lisa Guay-Woodford, M.D.*** - *Children's National Health System, The George Washington University*<br>**Katherine Dell, M.D.** - *Case Western Reserve University*<br>Attend this session to learn the latest in ARPKD research | **Coronado F&G** |
| 3:30 p.m. - 5:15 p.m. | **Q&A with the docs**<br>**Lisa Guay-Woodford, M.D.*** - *Children's National Health System, The George Washington University*<br>**Jeffrey Saland, M.D.** - *Mount Sinai Hospital*<br>**Katherine Dell, M.D.** - *Case Western Reserve University*<br>Everything you wanted to know about PKD in children but were (previously) afraid to ask. | **Coronado F&G** |

# Sunday, June 26

8:30 a.m. - 9:00 a.m. **Store Open** Fiesta 6

9:00 a.m. - 10:00 a.m. **Advocacy and you** Fiesta 5

**Hon. Karen L Thurman Member of Congress 1993-2003**
*Former U.S. Representative, Florida*
Everyone is an advocate! PKD patients, family and friends know better than anyone what it is like to live with PKD. Members of Congress depend on their constituents to bring important information to their attention. One personal story and one relationship can make a significant difference.

10:00 a.m. - 11:00 a.m. **Help us change our world: how you can make a difference (brunch served)** Fiesta 5

**Bill Brazell**
**PKD Foundation Volunteers**
PKD patients, advocates and volunteers share their experiences and discuss how you can get involved in the fight to end PKD

11:00 a.m. - 12:00 p.m. **Parent to parent Q & A** Coronado F&G

**Julia Roberts* and Michele Karl***
*PKD Parents Chapter Co-coordinators*
Having a child with PKD can feel isolating at times. This is your chance to talk with other parents who have gone through it all from financial stresses to dialysis and kidney transplants, growth issues, educational and emotional challenges and more. Hear their experiences and get your questions answered.

> "When I went to Convention, a new feeling crept into me that I had not experienced with PKD before: hope."
> -Ann Wiesman

* Board of Trustees Member + Scientific Advisory Committee Member

# Youth schedule at a glance

## Friday, June 24

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|---|---|
| 10:00 a.m. | **Registration opens** |
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## Saturday, June 25

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|---|---|
| 7:30 a.m. – 9:45 a.m. | **Youth group outing (breakfast served)** |
| 10:00 a.m. – 11:00 a.m. | **Education session** |
| 11:00 a.m. – 11:30 a.m. | **Exhibit hall open; store open** |
| 11:30 a.m. – 12.00 p.m. | **Informal group time** |
| 12:00 p.m. – 1:00 p.m. | **Clinical trials — why *you* should participate (lunch served)** |
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| 8:30 a.m. – 9:00 a.m. | **Store open** |
| 9:00 a.m. – 10:00 a.m. | **Education session** |
| 10:00 a.m. – 11:00 a.m. | **Help us change our world: how you can make a difference** |
| 11:00 a.m. – 12:00 p.m. | **Closing session** |

# Friday, June 24

10:00 a.m. **Registration** South registration counter

2:15 p.m. - 3:00 p.m. **Welcome and introductions** Sierra 1

**Blaire Taylor, LMHC, and Jessica Henderson, LCSW**
*Children's Home Society*

3:00 p.m. - 4:00 p.m. **What does PKD look like for me?** Sierra 1

**Melissa A. Cadnapaphornchai, M.D.**
*Children's Hospital Colorado on Anschutz Medical Campus*

Being a young person is hard enough, having PKD only adds to that. In this session, we will talk about what it means to live with PKD, and learn what we need to know to stay healthy and positive.

4:00 p.m. - 4:15 p.m. **Break**

4:15 p.m. - 5:15 p.m. **Staying positive, even through tough times** Sierra 1

**Blaire Taylor, LMHC, and Jessica Henderson, LCSW**
*Children's Home Society*

Having a chronic disease as a teenager is a game changer, but you are not alone. Attend this session to talk to other teens who know what you are going through and get tips to help you get through it.

5:15 p.m. - 7:00 p.m. **Reception, exhibit hall opening and research poster presentations** Fiesta 6

Join us to mingle with old and new friends. Visit with the researchers, view their poster presentations (see page 23) and learn about their work sponsored by the PKD Foundation Research Program. Visit the exhibit hall and check out our exhibitors and sponsors (see page 21).

7:00 p.m. - 9:30 p.m. **Celebration dinner** Fiesta 5

**Shuvo Roy, Ph.D.** - *University of California, San Franscisco*
**Kriste Lewis** - *NFL New Orleans Saintsations*
**Suzanne Ruff** - *Author of the* Reluctant Donor
**David Baron, Ph.D.** - *PKD Foundation*
**Jackie Hancock, Jr., CFRE** - *PKD Foundation*

Join us for a PKD-friendly dinner to hear from keynote speaker, Shuvo Roy, Ph.D., the co-inventor of the artificial, implantable kidney, and help us celebrate being together at the PKD National Convention 2016 as we honor our Chapters, donors, researchers and doctors dedicated to ending PKD.

# Saturday, June 25

7:30 a.m. - 9:45 a.m. **First ride experience at Disney's Animal Kingdom®** Sierra 1

**Disney's Animal Kingdom®**
Group outing; breakfast to follow

10:00 a.m - 11:00 a.m. **PKD clinical trials: what you should know** Sierra 1

**Melissa A. Cadnapaphornchai, M.D.**
*Children's Hospital Colorado on Anschutz Medical Campus*
Whether you have participated in a clinical trial before or don't even know what it is, attend this session to learn all about it, and the important role you play in finding treatments for PKD.

11:00 a.m. - 11:30 a.m. **Exhibit hall open; store open** Fiesta 6

11:30 a.m. - 12:00 p.m. **Informal group time** Sierra 1

**Blaire Taylor, LMHC, and Jessica Henderson, LCSW**
*Children's Home Society*
Take a break, have a short nap or just hang out.

12:00 p.m. - 1:00 p.m. **Clinical trials - why *you* should participate** (lunch served) Fiesta 5

**Ron Perrone, M.D.+** - *Tufts Medical Center*
**John Bissler, M.D.+** - *St. Jude Children's Research Hospital and Le Bonheur Children's Hospital*
Patient participation in clinical studies and trials is imperative to the therapy development process. Dr. Ron Perrone and Dr. John Bissler will discuss why this is and things to consider when signing up for a study.

1:10 p.m. - 2:00 p.m. **Managing blood pressure in adults and teens with PKD** Yucatan 1-3

**Michal Mrug, M.D.+** - *University of Alabama at Birmingham*
**Melissa A. Cadnapaphornchai, M.D.** - *Children's Hospital Colorado on Anschutz Medical Campus*

Recent studies have offered insights into best practices for blood pressure control in teens and adults with PKD. Join this session to learn the latest.

2:00 p.m. - 3:00 p.m. **Energy drinks, candy and fast food: is it okay to eat like my friends?** Sierra 1

**Jacob Taylor, Ph.D., R.D., L.D.**
*University Medical Center Groningen*

As a teen or young adult, it can seem like such a bore to think about your diet, even if you know it is in your best interest. Learn ways to enjoy every meal, but also stay healthy!

3:00 p.m. - 3:20 p.m. **Exhibit hall open; store open** Fiesta 6

3:20 p.m. - 4:15 p.m. **Connect - Share - Change** Sierra 1

**PKD Foundation Staff**

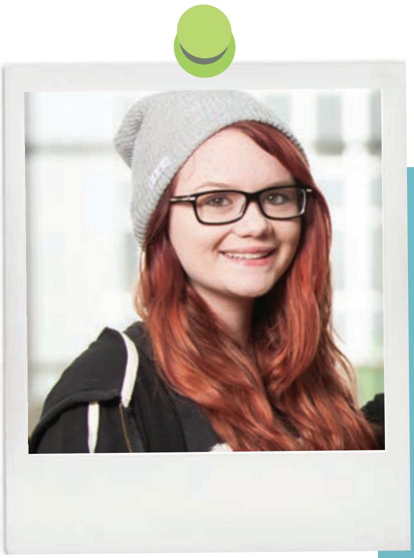
You are the future! Help us make a difference in your community. PKD Foundation staff will discuss ways for teens to get involved, connect with others and join the fight to #endPKD.

4:15 p.m. - 5:15 p.m. **Group discussion** Sierra 1

**Blaire Taylor, LMHC, and Jessica Henderson, LCSW**
*Children's Home Society*
Let's regroup and discuss what we've learned, what makes us nervous and questions we still have.

# Sunday, June 26

| | | |
|---|---|---|
| 8:30 a.m. - 9:00 a.m. | **Store Open** | **Fiesta 6** |
| 9:00 a.m. - 10:00 a.m. | **What is your role in health care transition? How to start managing your own health care**<br>**Laura Castellanos, M.D.**<br>*Icahn School of Medicine at Mount Sinai, New York*<br>Transitioning from pediatric to adult care can be a vulnerable time for you. Attend this session to learn strategies to make this process smoother. Learn how to manage your care independently, feel empowered to make decisions and get the confidence you need to coordinate your own care with multiple health care providers. | **Sierra 1** |
| 10:00 a.m. - 11:00 a.m. | **Help us change our world: how you can make a difference (brunch served)**<br>**Bill Brazell**<br>**PKD Foundation Volunteers**<br>PKD patients, advocates and volunteers share their experiences and discuss how you can get involved in the fight to end PKD | **Fiesta 5** |
| 11:00 a.m. - 12:00 p.m. | **Closing session and goodbye**<br>**Blaire Taylor, LMHC, and Jessica Henderson, LCSW**<br>*Children's Home Society*<br>We had a great time together! Let's share any final thoughts and make sure we know how to stay in touch. | **Sierra 1** |



> "It was cool to hang out with people my age in the same boat. We learned so much about the latest research and taking responsibility for your own health. And it's fun. It's a great place to make new friends."
> -Brittnee Boyd

**#PKDConvention**

# About our speakers

## Kriste Lewis - Emcee

*NFL New Orleans Saintsations*

Kriste Lewis is a 42-year-old NFL Cheerleader for the New Orleans Saints. She resides in Hattiesburg, Miss., with her husband of 18 years and their two boys. She holds a degree in early childhood development and elementary education from the University of West Alabama. Kriste taught reading for several years in the public school system where she also coached cheer and dance. She is currently a group fitness instructor and believes strongly in the importance of a healthy lifestyle. Kriste was diagnosed with polycystic kidney disease (PKD) 15 years ago during a routine pregnancy ultrasound. The diagnosis came as no surprise since her mother had also been diagnosed several years earlier. It wasn't until Kriste witnessed her mother's struggle with dialysis, and eventually a kidney transplant, that she decided to fight the disease head on and not let PKD beat her.

## Friday Celebration Dinner

### Suzanne Ruff

*Author of* The Reluctant Donor

Suzanne Ruff is a freelance writer for the Charlotte Observer. She is the author of the award-winning nonfiction book, *The Reluctant Donor*. Her book was a finalist in the MIPA Book Awards and received the 2015 Illumination book award. Suzanne is an advocate and professional speaker regarding progress made toward the treatment of kidney disease, transplantation and organ donation. She serves on the National Kidney Foundation's Living Donor Council Executive Committee, is a Board Director with American Association of Kidney Patients and a volunteer for the PKD Foundation. This past January, Suzanne became a co-investigator on a Patient-Center Outcomes Research Institute (PCORI) research study granted to Duke Medical School called "Putting Patients at the Center of Kidney Care Transitions."

### Shuvo Roy, Ph.D. - Keynote

*University of California, San Francisco*

Shuvo Roy, Ph.D., is a professor in the Department of Bioengineering and Therapeutic Sciences (BTS), a joint department of the University of California, San Francisco (UCSF) Schools of Pharmacy and Medicine. His work includes development of medical devices to address unmet clinical needs through strong collaboration and a multidisciplinary approach. Dr. Roy is also a faculty affiliate of the California Institute for Quantitative Biosciences (QB3) and the Director of the Biodesign Laboratory located on the Mission Bay campus. In addition, he serves as the Technical Director of The Kidney Project and is a founding member of the UCSF Pediatric Device Consortium. Dr. Roy has developed and currently teaches a course on medical devices, diagnostics and therapeutics, and regularly lectures on the medical device design process to UCSF graduate students and to national and international academic and industry audiences. He is the author of more than 100 publications and co-author of three book chapters, holds multiple patents for device developments and is the recipient of many award titles and nominations.

### David Baron, Ph.D.

*PKD Foundation, Chief Scientific Officer*

David Baron, Ph.D., joined the PKD Foundation after serving as Vice President of Nonclinical Safety Evaluation for the U.S. and European Union for Takeda Pharmaceuticals (Deerfield, Ill.). Dr. Baron, who has PKD and received a living donor transplant in 2009, has a strong personal and biomedical interest in the Foundation's emphasis on research. During his career he has focused on the movement of electrolytes and water across normal and abnormal epithelia, which relates directly to kidney function. Dr. Baron received his B.A. in biology and Ph.D. in anatomy from the University of Chicago. He completed his postdoctoral fellowship in pathology and pharmacology at the Medical University of South Carolina. With a National Institutes of Health grant, Dr. Baron founded the Core Structure-Function Laboratory in the Department of Pharmacology and joined their faculty. He has been a grant reviewer for the National Cancer Institute, given numerous invited seminars and has served on several national scientific boards.

# About our speakers

## Jackie D. Hancock, Jr., CFRE

*PKD Foundation, CEO*

Jackie D. Hancock, Jr., joined the PKD Foundation as CEO in 2013. He has extensive experience in nonprofit executive management, health care delivery systems, advocacy and fundraising, and has led the Foundation's partnership with kidney organizations for advocacy and research issues. He also navigated a partnership with the American Society of Nephrology to honor PKD Foundation co-founder Jared Grantham, M.D., through an endowed nephrology fellowship. He has served as CEO for the National Foundation for Transplants and as a member of its Board of Directors. He has also served on the Patient Advisory Committee of the National Heart Lung Blood Institute at the National Institutes of Health, on several advisory committees with Organ Procurement Organizations, and as a member of Donate Life Tennessee's Advisory Board. Jackie graduated from Park University, Parkville, Mo., with a bachelor's degree in psychology, and earned the designation of Certified Fund Raising Executive from the Association of Fundraising Professionals. He will graduate in 2016 with a Masters Degree in operations management from the University of Arkansas School of Industrial Engineering.

# Saturday Lunch

## Ronald D. Perrone, M.D.

*Tufts Medical Center*
*Member, PKD Foundation Scientific Advisory Committee*

Ronald D. Perrone, M.D., is the Scientific Director of the Clinical and Translational Research Center, the Associate Chief of the Division of Nephrology and the Medical Director of Kidney Transplantation at Tufts Medical Center. He is also a professor at Tufts University School of Medicine. Dr. Perrone is Board certified in nephrology and internal medicine. He has received numerous honors and awards, including the title of "Top Doctor" from the Boston Magazine for several years and the Physician of the Year Award from the PKD Foundation. He is a member of the PKD Foundation's Scientific Advisory Committee and a Region 1 Representative at the OPTN/UNOS Kidney Transplant Committee. Dr. Perrone is also on the Editorial Board of the Clinical Journal of the American Society of Nephrology. Dr. Perrone has a long-standing and wide-ranging interest in ADPKD, including basic investigations addressing mechanisms of ion transport by cystic epithelia, clinical investigations describing causes of mortality in ADPKD end-stage renal disease population, and participation and direction of clinical trials in ADPKD.

## John J. Bissler, M.D.

*FedEx Chair of Excellence*
*Tuberous Sclerosis Center of Excellence*
*Division of Nephrology at St. Jude Children's Research Hospital and LeBonheur Children's Hospital*

John Bissler. M.D., is a pediatric nephrologist in Memphis, Tenn. He is the Director of the Tuberous Sclerosis Center of Excellence and the Director of the Division of Nephrology at St. Jude Children's Research Hospital and LeBonheur Children's Hospital. Dr. Bissler is also a Professor of Pediatrics at the University of Tennessee. He received his medical degree from Northeast Ohio Medical University and has been in practice for more than 20 years. He is Board certified in pediatrics and pediatric nephrology with expertise in angioedema, kidney disease and tuberous sclerosis. Dr. Bissler is a member of the PKD Foundation's Scientific Advisory Committee.

# About our speakers

## Sunday Brunch

### Hon. Karen L Thurman Member of Congress 1993-2003

*TG and C Group Consulting*

Congresswoman Karen Thurman served in the United States House of Representatives from 1993 to 2003, representing Florida's Fifth Congressional District. During her time in Congress, she became a member of the influential Ways and Means Committee, which maintains jurisdiction and oversight of the Medicare and Medicaid program. Her moderate, bipartisan record made her a national leader on health care policy. She also served as a member of the Committees on Agriculture and Government Reform and Oversight. Prior to being elected to Congress in 1992, Thurman served in the Florida State Senate for 10 years. She also served as member of the City Council and mayor of Dunnellon, Fla. Before embarking on her career in elected office, Congresswoman Thurman taught elementary school mathematics in Marion County, Fla. Congresswoman Thurman is the principal partner at the TG and C Group Consulting in Washington, DC. She obtained her associate's degree from Santa Fe Community College and her bachelor's degree in education from the University of Florida. She resides with her family in Dunnellon, Fla.

### Benjamin D. Cowley, Jr., M.D.

*University of Oklahoma Health Sciences Center*
*Board Chair, Executive Committee, PKD Foundation Board of Trustees*

Benjamin D. Cowley Jr., M.D., lives in Oklahoma City. He is the Chief of Nephrology & Hypertension and John Gammill Professor in Polycystic Kidney Disease at the Oklahoma Health Sciences Center. Dr. Cowley has been involved in PKD research for more than 20 years, publishing numerous articles and presenting on PKD at various conferences and events. He trained under Dr. Jared Grantham, co-founder of the PKD Foundation and distinguished physician at the University of Kansas Medical Center. He currently serves as the Board Chair of the PKD Foundation's Board of Trustees. Dr. Cowley completed his post-graduate education in internal medicine, nephrology and biochemistry at the University of Kansas Medical Center. He received his M.D. degree from Baylor College of Medicine and attended undergraduate school at Rice University. Dr. Cowley has been named one of Oklahoma's "Top Doctors" by Castle Connolly Medical Ltd. for the past five years.

### Ronald D. Perrone, M.D.

*Tufts Medical Center*
*Member, PKD Foundation Scientific Advisory Committee*

### Terry Watnick, M.D.

*The University of Maryland*
*Chair, PKD Foundation Scientific Advisory Committee*

Terry Watnick, M.D., is an Associate Professor of Medicine in the Division of Nephrology at the University of Maryland, where she has established an inherited renal disease clinic. Dr. Watnick has been an investigator in several multicenter clinical trials for patients with ADPKD, including TEMPO. She also leads the Baltimore Polycystic Kidney Disease Research and Clinical Core Center at the University of Maryland School of Medicine, and currently serves as the Scientific Advisory Committee Chair for the PKD Foundation's Board of Trustees. Dr. Watnick received her medical degree from the Yale School of Medicine and completed her internal medicine training at Yale-New Haven Hospital. She received clinical training in nephrology at the Johns Hopkins Hospital, where she also completed a research fellowship that was focused on the genetics of ADPKD. Dr. Watnick subsequently joined the faculty at the Johns Hopkins School of Medicine, where she rose through the ranks to Associate Professor.

# Our exhibitors

## American Association of Kidney Patients

For more than 45 years, the American Association of Kidney Patients (AAKP) has been the leader in the fields of patient engagement, patient-centered education and public advocacy efforts. We exist to provide education to patients and their care partners, helping them achieve their best possible outcomes.

## Central Florida Kidney Centers, Inc.

Central Florida Kidney Centers, Inc. (CFKC) is a nonprofit dialysis organization operating nine facilities throughout the Orlando and Melbourne surrounding areas. CFKC is the largest independent dialysis organization in Florida, and the only dialysis facility in Orlando offering dialysis services for pediatrics other than hospital-based clinics. Stop by our booth for more information.

## The Charles J. Newman Co., LLC

Charles J. (Chuck) Newman CLU, Principal of The Charles J. Newman Co., LLC is an independent insurance agent and registered representative. He provides a full range of group and employee benefit programs to more than 2,000 individual clients. Chuck, who has PKD, has assisted a number of families affected by PKD both pre- and post-transplant in obtaining life, health and long-term care insurance.

## Children's Organ Transplant Association

The Children's Organ Transplant Association (COTA) provides fundraising assistance and support to families facing a transplant. Since 1986, COTA has helped patients age 21 and under who require a life-saving organ or tissue transplant. COTA also works with patients of any age who need a transplant due to a single -gene disease like PKD, cystic fibrosis or sickle cell disease.

## HelpHOPELive

HelpHOPELive is a 30-year-old national nonprofit organization that supports organ transplant, catastrophic injury (spinal cord/traumatic brain/ stroke) and catastrophic illnesses (ALS/MS/etc.) to raise funds to offset medical expenses. HelpHOPELive is ranked in the top one percent of all charities nationally. For more information, visit helphopelive.org.

## Kadmon Corporation, LLC

Kadmon is a fully integrated biopharmaceutical company engaged in the discovery, development and commercialization of small molecules and biologics to address disease areas of significant unmet medical need. We are developing product candidates within autoimmune and fibrotic diseases, oncology and genetic diseases. For more information please visit kadmon.com.

## Nebraska Medicine

Nebraska Medicine is the most esteemed academic medical center in the region, offering exceptional patient care combined with innovative research and education. Committed to advancing quality health care, Nebraska Medicine provides patients with access to more than 1,000 physicians and 676 licensed hospital beds, as well as approximately 40 specialty and primary care clinics in Omaha and the surrounding area.

## NxStage Kidney Care

NxStage Kidney Care is dedicated to helping our patients discover, achieve and maintain freedom and flexibility while on a dialysis therapy that meets their needs. Our state-of-the-art dialysis centers are dedicated to providing top quality, compassionate and individualized care. For more information please visit nxstagekidneycare.com.

## NxStage Medical, Inc.

NxStage® is more than a company; we are leading the renal revolution. Our innovative products are helping to shape and transform renal care by making it simpler, portable, and expanding treatment options to enhance patient freedom and fulfillment.

## PKD Foundation

We are the only organization in the United States solely dedicated to finding treatments and a cure for PKD. We do this through research and by promoting education, advocacy, patient support and awareness of PKD. Since our founding in 1982, we have invested more than $40 million in research, clinical and scientific grants, fellowships and meetings, making us the second largest funder of PKD research after the National Institutes of Health. Visit us at the exhibit hall to learn more about how we are leading the way toward a future without PKD.

## PKD Research Group

### University of Colorado Anschutz Medical Campus

We have been involved in PKD research for the past 20 years, conducting clinical and laboratory studies in adult and children. The National Institutes of Health, PKD Foundation and Zell Family Foundation have funded this research since 1985. During the past 13 years, our research has investigated ways to intervene in the progression of PKD and identify factors affecting disease.

## UAB Hepatorenal Fibrocystic Disease Core Center

The University of Alabama at Birmingham Hepatorenal Fibrocystic Disease Core Center, led by Lisa Guay-Woodford, M.D., has developed a unique set of clinical, genetic and educational resources for autosomal recessive polycystic kidney disease (ARPKD) and other recessive forms of renal cystic disease. We are actively seeking participants for our clinical database, tissue repository and genetic studies. For more information, visit arpkdstudies.uab.edu.

## University of Kansas Medical Center Kidney Institute

The Kidney Institute at the University of Kansas Medical Center is a world-class, internationally recognized, multidisciplinary research center comprised of over 35 faculty investigators. A major strength of the Kidney Institute is the basic, translational and clinical investigation of PKD. Learn more about our research, PKD clinical trials or tissue donations on our website: kumc.edu/ki.

# Poster presentations

## Research grants

### Katherine Dell M.D., Case Western Reserve University

*Vincent H. Gattone Research Award for the top-rated grant proposal*

**Magnetic resonance fingerprinting (MRF) to assess ARPKD kidney and liver disease**

(Funded in partnership with friends/family in memory of Sophie St. Aubin and in honor of Sophia Shapiro.)

ARPKD is a disorder that affects both kidneys and the liver and can present life-long challenges to affected patients. Several new therapies have shown promise in ARPKD animal models. However, these therapies have not been studied in ARPKD patients because there are no clinically-available, reliable, non-invasive ways to measure the effects of treatment. We have identified two magnetic resonance imaging (MRI) measures that may provide this key missing piece. Unfortunately, MRI studies are long and require patients to sit still, which is not possible for young children. In the proposed studies, we will investigate a novel MRI method, called MR fingerprinting (MRF). MRF may allow these studies to be performed very rapidly, having the potential to allow ARPKD patients of all ages to be part of clinical trials for new therapies.

### Benjamin Freedman, Ph.D., University of Washington

**Modeling human PKD cystogenesis with pluripotent stem cells**

Human pluripotent stem cells have dual value as personalized laboratory models for human kidney disease and as a potential source of on-demand, immunocompatible kidney replacement tissue. Using these cells, it is now possible to generate human mini-kidney ‘organoids’, which are capable of recreating PKD in lab dishes. The goal of the proposed research is to expand the existing findings to understand how human PKD mutations cause cystic disease, to test drugs that intervene with this process and to generate patient-matched stem cell products for application in future clinical trials.

### Timothy Kline, Ph.D., Mayo Clinic, Rochester

**Development of a computer-aided decision support system for PKD**

ADPKD is one of the most common monogenic disorders, and is a leading cause of end-stage renal disease. Total kidney volume (TKV) has become the main image-based biomarker for following ADPKD progression. This project will develop automated tools to increase measurement throughput, and explore new image-based biomarkers that will significantly add to the assessment of patient prognosis. It will also have the ability to more quickly judge the effectiveness of interventions.

### Stephen Parnell, Ph.D., University of Kansas Medical Center

**Polycystin-1 mediated cyst regression**

Loss of function of the polycystin-1 protein leads to PKD. Re-expression of wild-type polycystin-1 in cystic mice causes visible regression of cystic disease, but the mechanism of regression is not known. This proposal will study the functional consequences of re-expression of polycystin-1 in a mouse model of PKD, thereby facilitating future therapies designed to recapitulate the function of polycystin-1 to halt or reverse cystic disease.

# Poster presentations

**Vishal Patel, M.D., University of Texas Southwestern Medical Center**

**Role of miR-17~92 in the pathogenesis of PKD**

MicroRNAs (miRNAs) are small RNAs that inhibit the function of genes. Abnormal level of miRNA expression is observed in numerous diseases, including PKD. Drugs designed to correct miRNA levels are emerging as promising new ways to treat diseases. We have identified a family of miRNAs called miR-17~92, which promotes kidney cyst growth in mice. In this application, we propose to determine whether miR-17~92 promotes cyst growth in mouse models of autosomal dominant PKD (ADPKD), the most common type of PKD. If successful, our studies will identify miR-17~92 as a new drug target for the treatment of ADPKD.

## Drug development

**Bonnie Blazer-Yost, Ph.D., Indiana University-Purdue University Indianapolis**

**Use of low dose pioglitazone to treat autosomal dominant polycystic kidney disease (PIOPKD)**

Pioglitazone is currently used in clinical practice to treat diabetes and this study will examine the potential use of a low dose of the same drug for the treatment of PKD. The purpose of this study is to determine whether the diabetes drug pioglitazone (Actos) is a safe and effective treatment of ADPKD when treated in its early stages. Pre-clinical models of PKD have shown that low dose treatment with pioglitazone decreases the growth of the cysts. The studies also suggest that effective pioglitazone dosing for PKD may be lower than that used to treat diabetes. The purpose of this study is to see if pioglitazone might slow cyst disease in humans.

## Research fellows

**Marcelo Cassini, Ph.D., Yale School of Medicine**

**Can Mcp1 knock-out and blockage of macrophage receptor CCR2 alter the outcome of polycystic kidney disease (PKD)?**

We have learned that macrophages accumulate in kidneys of patients with PKD and directly surround the cysts in abnormally large numbers. These macrophages secrete factors that promote cyst growth and decrease the normal kidney function. Mouse models of PKD have shown that the polycystin proteins normally prevent the signal that causes macrophages to enter the kidney. The proposed research is designed to identify the mechanism that promotes the increased macrophage homing to the polycystic kidneys, and to develop an approach to interrupt those signals as a new way to slow cyst growth and loss of kidney function.

**Follow the PKD Foundation on social media to stay up to date on the latest Convention news. Use the official event hashtag #PKDConvention to join the conversation.**

# Poster presentations

**Jacqueline Peda, Ph.D., University of Kansas Medical Center**

**The regulation of cardiac remodeling by PC1 and SMYD2 in polycystic kidney disease**

ADPKD-associated cardiovascular complications, the leading cause of premature mortality in ADPKD, have been suggested to be mainly caused by cardiovascular hypertension induced by renal cyst growth and renal failure. However, the fact that cardiac hypertrophy also occurs in young ADPKD patients with normal blood pressure and renal function suggests that cardiac dysfunction in ADPKD patients does not develop solely in response to hypertension and renal failure. This implicates that additional genetic and epigenetic regulators may be required. In this study, we investigate the direct involvement of heart polycystin-1 in regulating the development of cardiac hypertrophy as well as provide a molecular mechanism by which an epigenetic regulator, Smyd2, promotes this process via methylation subsequent activation of NF-κB. Our study will advance pathophysiological understanding of this complex systemic disease.

**Irfana Soomro, MBBS, New York University Langone Medical Center**

**Targeting glutamine metabolism as a potential treatment for ADPKD**

Glucose and glutamine serve as nutrients and provide energy for rapidly growing cells. We found that kidney cells which form cysts require glutamine for cyst formation and blocking glutamine metabolism results in decreased cyst growth in animal models of ADPKD. The main goal of this proposal is to understand the role of glutamine metabolism in cyst formation and to validate the potential to pharmacologically block this pathway as a novel treatment for ADPKD.

## Young scientist

**Greg Mainolfi, St. Mary's College of Maryland**

**Undergraduate research project: the search for mutations that cause the genetic disorder polycystic kidney disease**

Our research has looked into inducing mutations in PKD1, the gene that codes for PC1, and understanding how the modified characteristics of the resulting mutant protein may cause PKD. We hypothesized that mutations that did not impact the natural cleavage of PC1 but altered other physical characteristics of the protein may still bring about cystic development. We therefore prepared mutations in the PKD1 gene through site directed mutagenesis and overlapping extension PCR before inserting our mutant DNA into plasmid vectors. These vectors were then transfected into Human Embryonic Kidney (HEK) cells for subsequent western blotting for the mutant protein to ensure that cleavage had not been affected. Results show the successful generation of the 53054A mutation that still produces a cleavable gene product that can be used for further testing. With the identification of mutations that do not impact cleavage but still lead to cystic development, we can measure the association strength and linker rigidity between the protein fragments when they co-localize and correlate that to the severity of PKD in a corresponding mouse model. By creating a number of mutations like this, we hope to create a spectrum of altered association strength and linker rigidity and relate that to the manifestation of PKD. With this characteristic of the PKD1 protein better understood, we can target this portion of the protein for treatment of PKD.

# 2015 chapter award nominees

## National Fundraising Support

Presented to the Chapter that best communicates national fundraising opportunities to their local members and communities at large. Fundraising opportunities include Run for PKD, DIY for PKD, Vehicle Donation and Workplace Giving.

*Albuquerque Chapter* *Nebraska Chapter* *Northern Indiana Chapter*

## Outstanding Fundraising

Presented to the Chapter that supports an outstanding local fundraising program, including hosting a successful non-Walk fundraising event.

*Delaware Chapter* *New England Chapter* *St. Louis Chapter*

## Outstanding Patient Support

Presented to the Chapter that provides outstanding support for those affected by PKD.

*East Texas Chapter* *Nashville Chapter* *Pittsburgh Chapter*

## Outstanding Education

Presented to the Chapter that supports outstanding local education programs, including advocacy efforts and awareness.

*Cincinnati-Dayton Chapter* *Indianapolis Chapter* *San Francisco Chapter*

## Outstanding Walk

Presented to the Walk that increased fundraising and participation in the event. This includes engaging those touched by PKD, volunteers, the community-at-large, local businesses and the media.

*Charlotte Walk for PKD* *Phoenix Walk for PKD* *South Florida Walk for PKD*

## Outstanding Chapter

Presented to the Chapter that has consistently met objectives related to the Chapter mission areas of education, fundraising and support.

*New Jersey Chapter* *Seattle Chapter* *Tampa Chapter*

## Outstanding Walk Coordinator

Presented to the Walk Coordinator who is outstanding in planning and executing the Walk for PKD event.

*Cynthia Kanazawa and Andy Tang - Los Angeles Walk for PKD*
*Dustin Williams and Bailey Jackson - Nashville Walk for PKD*
*Larianne Austin - Sacramento Walk for PKD*

## Outstanding Chapter Coordinator

Presented to the Chapter Coordinator who is outstanding in initiating, implementing and supporting local Chapter efforts in the areas of education, fundraising and support.

*Darien and Josie DaCosta - Hudson Valley Chapter*
*Judy Ehrlich and Dean Lotito - New England Chapter*
*Jean Sommer - St. Louis Chapter*

# Change the world together: a future without PKD

We are united in the fight to end PKD. Together, we can help advance critical research that will lead us closer to treatments and a cure, and provide much needed education and support to patients and families affected by PKD.

## Learn something new.

- Sign up to receive information about education, research, advocacy and more.
- View or attend one of our education webinars featuring PKD experts.
- Order a PKD patient handbook.

## Raise awareness and fundraise.

- Join a local or virtual Walk for PKD.
- Participate in a Run for PKD event.
- Get creative and find easy ways to raise awareness and funds with a DIY for PKD event.
- Share your story and read the stories of others with PKD on the PKD Connection blog.
- Connect with us on Facebook, Twitter and Instagram, and help spread the word about PKD.

## Get involved.

- Join a local Chapter to give and receive support from others in the PKD community.
- Sign up for our Advocacy Alerts to get the latest information on important legislation to advance research and treatments for PKD, and to learn what you can do to advocate on behalf of all PKD patients.
- Sign up for Accelerating Clinical Trials (ACT) Alerts to hear about clinical trial recruitment and to learn whether you are eligible to participate in a clinical trial.

## Give.

- PKD can affect generations of families. Honor or remember someone you love with a gift to the PKD Foundation.
- Plan ahead to make a lasting contribution to end PKD with a legacy gift.
- Make the PKD Foundation your charity of choice to make a difference in the lives of hundreds of thousands of individuals and families affected by PKD.

Learn more about how you can end PKD: pkdcure.org/get-involved.

# Partner with us through your support!

Foundation Partners
PKD FOUNDATION

We want to partner with you to accelerate PKD research and provide education and support for PKD patients and their families. As a Foundation Partner, you make the PKD Foundation a priority in your philanthropic giving, and your gift is put to work through programs that focus on advancing treatments and finding a cure for PKD. We invite you to join our societies and partner with us in the fight to end PKD.

## Annual Giving Society

These members are generous donors who give $1,000 or more annually to help fund our daily mission-critical work: promoting research, advocacy, education, patient support and awareness.

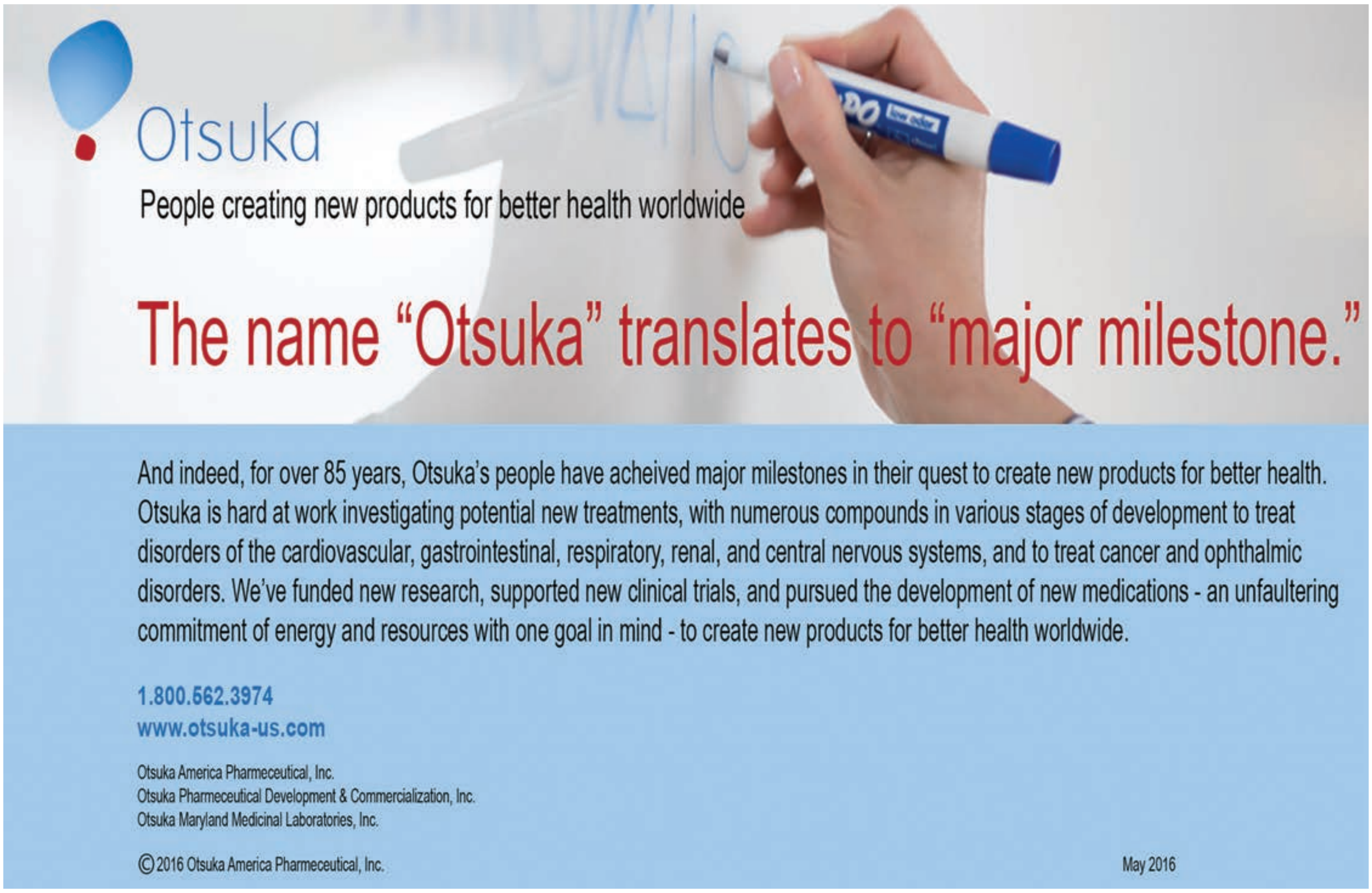
## Lifetime Giving Society

These members have made significant contributions of at least $100,000 over time, and their philanthropy has made a transformational impact on the work of the Foundation.

## Legacy Giving Society

These members have chosen to support the continuing work of the PKD Foundation through their will or other estate plans. By sharing their written intention, these donors have made a strategic investment for generations to come.

Please stop by the PKD Foundation table in the exhibit hall to talk with a member of the development staff about how you can become a Society member or visit pkdcure.org/foundation-partners to sign up today!



# Menus

## Friday

### Dinner

Fresh Bibb lettuce with roasted corn, tomatoes and cucumbers served with serrano vinaigrette

Grilled chicken Veracruz

Spanish rice with pigeon peas

Roasted cumin zucchini and squash

Cornbread muffins and medianoche rolls

Strawberry tres leches

## Saturday

### Lunch Buffet

Vegetarian Italian white bean soup

Hearts of romaine, Parmesan cheese, seasoned croutons and classic Caesar dressing

Bocconcini, tomato and basil salad

Baked seasonal fish with limoncello and herb sauce

Grilled boneless chicken

Marsala with roasted mushrooms

Penne rigate with smoked tomato sauce

Ratatouille

Focaccia bread with pesto

Fresh-cut seasonal fruit
Tiramisù

## Sunday

### Brunch

Croissants with strawberry preserves and butter

Yogurt and fruit parfait

Scrambled eggs with chives and cheese

Chicken and blueberry sausage

Lyonnaise potatoes

Orange juice

Coffee (regular and decaffeinated)

Selection of hot teas

*Please note that menu items are subject to change.*

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**In case of an emergency, local hospitals near *Disney's Coronado Springs Resort* are listed below.**

### Florida Hospital Celebration Health

400 Celebration Pl, Kissimmee, FL 34747 • (407) 764-4000
5.9 miles (11 minutes from resort) • Local Emergency Room

### Florida Hospital Kissimmee

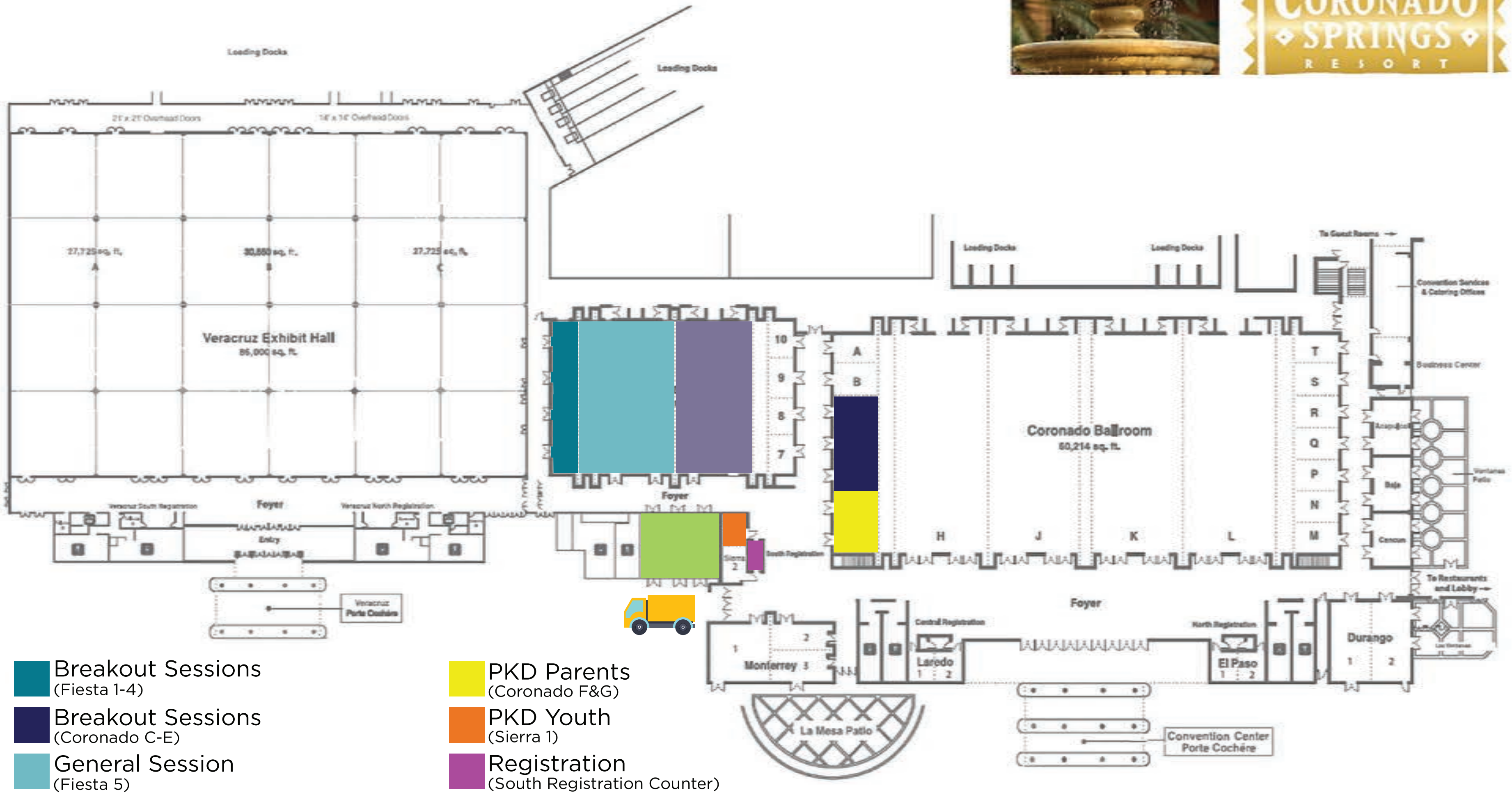
2450 North Orange Blossom Trail, Kissimmee, FL 34744 • (407) 846-4343
11.1 miles (23 minutes from resort) • Local Emergency Room

### Orlando Regional Medical Center

52 W Underwood St, Orlando, FL 32806 • (321) 841-5111
17.6 miles (32 minutes from resort) • Nephrology Department and Services available

**If you need to reach a staff member, call our 24-hour emergency number: 816.268.8481.**

Disney's CORONADO SPRINGS RESORT



🟦 Breakout Sessions (Fiesta 1-4)

⬛ Breakout Sessions (Coronado C-E)

🟦 General Session (Fiesta 5)

🟪 Exhibit Hall (Fiesta 6)

🟩 Breakout Sessions (Yucatan 1-3)

🟨 PKD Parents (Coronado F&G)

🟧 PKD Youth (Sierra 1)

🟪 Registration (South Registration Counter)

🚚 NxStage Freedom RV

# About Orlando

Welcome to Orlando, the home of the world's top 20 theme parks. With nearly 100 attractions, Orlando has something for everyone.

**Enjoy the world of imagination and fantasy** at *Magic Kingdom*® Park and *Epcot*®, or the glamour, action and adventure of the movies at *Disney's Hollywood Studios*®. Be amazed by the magic of Harry Potter at Universal's Islands of Adventure®.

**Get energized** by Orlando's exciting nightlife, with live shows, interactive entertainment, pubs, dance clubs, live music, comedy clubs and more. See live performances at the House of Blues in *Disney Springs*® and Hard Rock Live at Universal CityWalk®. Experience mind-bending entertainment with the Blue Man Group and the La Nouba™ by Cirque du Soleil®. Or discover a modern, big nightlife scene in downtown Orlando.

**Be delighted** by Orlando's diverse and award-winning cuisine and restaurants featuring celebrity chefs that cater to visitors across the globe. 2016 James Beard Foundation Restaurant and Chef Awards' nominees include Orlando chefs Scott Hunnel of Victoria & Albert's at *Walt Disney World*® Resort, James and Julie Petrakis of The Ravenous Pig in Winter Park and Kathleen Blake of The Rusty Spoon in downtown Orlando.

**Enjoy shopping** venues for every budget. From sophisticated malls, such as the Mall at Millenia, to quaint boutiques on Park Avenue in Winter Park or value outlet centers, you will be sure to find something special to take back home.

**Simply relax** in one of Orlando's destination spas, conveniently located in and near *Disney's Coronado Springs Resort*. Or enjoy the outdoors in one of Orlando's 176 golf courses. Play 18 holes on greens designed by golf legends, such as Palmer, Watson, Nicklaus and Norman. Or try the Rees Jones-designed, 18-hole championship golf course shared by the Hilton Orlando Bonnet Creek and the Waldorf Astoria Orlando.

# Notes

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# Notes

# Thanks to our sponsors:

Presenting Sponsor

**PKD FOUNDATION**
Polycystic Kidney Disease

Patron Sponsors

**Roger L. Kohn Fund**
Roger L. Kohn and Kay M. Gilbert

Otsuka