PKD Leadership Connection

Guest speaker Jean Sommer
Ray Smith
Chief Financial Officer

Financial Report

- Major Donors income was steady (comes from larger donors who donate $50,000 or less)

- Volunteers revenue was down (Comes from Walks. Walk Goal was 2M. 2016 Walks brought in 1.7M)

- Research Grants revenue was down (Dr. Vincent H. gaetone Research Award, Dan Larson Award, and the Eileen Creamer O’Neill Award)

- Misc. revenue was up (Comes from National Convention, sponsorships, AmazonSmiles, Family Endowment)

- 62 Chapters
- $0.77 per dollar goes toward research, awareness, education, and advocacy ($0.45 goes toward research)
- Sponsorship income is used to support the Foundation running cost not Walk income
- AmazonSmiles collected $13,132.55 since the start of the website up to May 2017.
NEW Financial

• 100% of Walk money will go toward research!!!

• 2016 1.7 million Walk money went toward research.

• Monthly giving: The Foundation is asking people to donate $35 or more per month. If you donate $35 per month, you will be sent a copy of the new PKD cookbook, Cooking Well. ($15 dollar value)

• Monthly Giving can be set to a designated walker. The walker has to register for the Walk for the money to show up under the walker’s name. The donor can receive a Walk t-shirt from the team captain, if $100 is donated before the walk.
Dr. David Baron
Chief Scientific Officer

Mission: We give hope. We fund research.
5 year goal

- To increase donations by 300%
- Yearly fellowships starting 2018
- Yearly research grants starting 2019
- Give more money per researcher for lab equipment and supplies
- Develop a PKD Patient registry - Nationally, Worldly, data base for clinical trials
- These goals will allow the Foundation to drive research and therapy development
Family Endowment

- James M and Lucille Kemp Carnes Family Endowment donated $2,973,000 on May 31, 2017 for research

- The PKD Foundation secured $8M which will go toward the 5 year plan of $10 million for operation costs in the bank for sustainability

- They will need 2 million to complete the 5 year plan. Then all other money goes toward research
Research Fellowships

- Fellowships recognizes early career scientist whose achievements identify them as next generation rising stars

- 2 Fellowships are funded every two years

- The Foundation raised enough money to fund 4 fellowships from Feb 2017- Feb 2019

- New: In 2018, the PKD Foundation will start 1 new fellowship every year. Each fellowship will last two years.

- Each fellowship receives $50,000/year
Research Grants

• In February of 2016, the Foundation awarded 5 Research Grants

• Each Research Grant recipient receives $80,000 per year for two years

• Total investment of 2.4 million over 2 years.

• New: Yearly Research grants will start in 2019

• In the future, the Foundation will offer more money per researcher, so they can stock their labs with supplies they need to complete their experiments.
Research and Results
Otsuka - Tolvaptan Reprise Study Results

• Positive effect on GFR (glomerular filtration rate)

• Decreased in growth of TKV (Total kidney volume)

• Safety profile similar to prior studies - liver enzymes a little bit elevated

• Slowed down PKD and preserved kidney function

• Feels confident that FDA will pass Tolvaptan for PKD use sometime in the next couple of months.
Drug/Compounds currently in trials:

- Pioglitazone
- Metformin
- Tesevatinib
- Niacininimide
Drugs being tested

- Pioglitazone gives male rats bladder cancer. Think the cancer is animal specific. This drug is used for Type II diabetes. It reduces cyst in rats.

- Metformin is an even older type II diabetes drug and cheap so drug companies do not want to study it. Slows cyst growth in mice.

- Tesevatinib stops tumor growth. This drug side effect - likes to go to the kidneys. This is being looked at and may use lower dosage.

- Niacinimide study of vitamin B3. Vitamin B3 is not a toxic drug. Vitamin B3 is having great success. It inhibits the growth of cyst. Plan on expanding to more patient trials.
Compound coming soon into trials

- Anti-miR-17 (micro RNA-17)
Anti-miR-17

- Anti-miR-17 is a new area of study
- RNA is scribed from DNA
- Using fragments of RNA has biological activity
- Inactivation of miR17-92 in a mouse model of PKD retards kidney cyst growth, this improves renal function and prolongs survival (Feb 2017 nature.com)
Multiple means of attacking PKD

• Anti-proliferative agents
• Anti-inflammatory agents
• Vasopressin V2 receptor blockers
• hsp90 (heat shock protein) targets
• Many other pathways with potential
• Exosomes, stem cells, genomics
18 Companies interested in PKD research

- Amgen
- Blueprint Medicines
- Clinical Metabolomics
- Cydan
- Endocyte
- Epigen
- Goldfinch
- GSK
- Kadmon
- ManRos
- Novartis
- Otsuka
- Palladio
- Regulus
- Retrophin
- Sanofi
- Vanda
- Vertex
Clinical advances
PKDOC

- The PKD outcomes Consortium (PKDOC) is a collaboration between PKD Foundation, Critical Path Institute, representatives of the pharmaceutical industry, PKD clinicians and U.S. Food and Drug Administration (FDA)

- Total kidney volume

- Foundation wants early screening, to detect if you have PKD at an earlier stage, so treatment can begin and slow the process

- Treat PKD in early stages such as between 1 & 2 instead of stage 4 the transplant stage
Clinical advances
PKDOC cont.

- There is a critical need for biomarkers to enrich study populations
- There is a critical need for biomarkers to show efficacy in two years or less
- There is a critical need for predictive preclinical in vitro and in vivo models
- There is a need for regulatory advancement: A pathway to faster registration for clinical trials
Announcements

- Education webinars will be starting back up
- ARPKD handbook will be out in the fall
- The Foundation is changing the word “conference and convention” to the word “connection”
- 2018 Leadership Connection is June 27-June 29 in Kansas City. I think following will be the 2018 National PKD Connection June 29-July 1??
Andy Betts
Chief Executive Officer
Future Focus
PKD Foundation

• Our vision is that one day no one will suffer the full affects of PKD

• Our mission is to promote programs of research, advocacy, education, support, and awareness in order to discover a treatment and a cure for PKD and improve the lives of all it affects. We give hope.
Next 5 year Vision

• The PKD Foundation 5 year plan is to have $10 million in the bank to cover 5 years of operationally cost

• Getting this money from about 5 larger donors

• All other areas of the money donated goes toward research fellowships and research grants
Focus Future Expenses
PKD Foundation
Future Focus Expenses

• Patient Centered Research $42 million
  • Launch global PKD Patient Registry $5 million
  • Research projects and peer reviewed grants $12 million
  • Usher in a new generation of researchers (fellowships) $10 million
  • Build patient resource center (including patient transplant support) $2 million
  • Facilitate education, resources, meetings, and events on latest research $3 million
  • Secure the future of PKD research - deferred giving program $10 million
Future Focus Expense continued

- Awareness $18 million
  - Ushers in a new generation of supporters (donor acquisition and upgrade) $10 million
  - Deepen understanding of PKD (marketing and advocacy) $8 million
Future Focus Expenses Continued

- **Securing the Viability of the Foundation** $10 million
  - Founder’s Council created so key influencers can underwrite the operational and administrative costs
  - An annual exclusive summit, at which Founders’ Council families and top PKD physicians and researchers meet
Focus Future Revenue
Future Focus Revenue

5 year plan

• Major Gifts $30 million
  - Major gifts $21 million (average $4.2mm/year) $25K + gift cultivation and founders council. Goal is to have 5 years of operational expenses in the bank so all other donations go toward research fellowships and research grants
  
  • Donor upgrade $5 million (average $1mm/year) Goal is to increase donors giving $1K - 5K annually
  
  • Pharma, corporate foundation $4 million (average $800K/year) Pharma marketing fee service and support
Future Focus Revenue
5 year plan

- Planned Gifts $15 million
  - Deferred bequest gifts $10 million (average $2mm/year)
  - Realized bequest gifts $5 million (average $1 mm/year)
Future Focus Revenue
5 year plan

• Mass Market Gifts $25 million

• Walk and tribute gifts $10 million (average $2mm/year), goal is to maintain walks and increase donors

• Monthly recurring gifts $10 million (average 1mm/year, goal is to get 5,000 monthly donors to donate $35/month

• Donor acquisition & reactivation $5 million (average $1mm/year) Tribute and lapsed givers to repeat givers
Where we are going in the next 12 months

- Create Founder’s Council, organize summit and secure 5 year major gift pledge to cover operation sustainability
- Establish a monthly giving program via event, tribute and loyal supporters
- Launch Patient Resource Center
- 90,000 e-mails in the data base. Going to try to reactivate them to have 40,000 donors and have 500,000 sustainers in 5 years.
How you can help

• Encourage your participants to sign up to be monthly givers
• Enlist an army to advocate
• Identify good stories in your area and send them to the Foundation
• Support new initiatives and projects
• Help us get better by giving us feedback
Spreading PKD Awareness Foundation has tables at:

- National Kidney Foundation Convention
- Nephrology Convention
- American Society of Nephrology (ASN) - has a day of training for younger nephrologist
- Dietitian or Nursing Conference
- Partner with education events with NK Foundation
Spread PKD Awareness
Our Chapter can:

- Ask to set up a PKD booth at minor league game
- Ask to stand in front of Sam’s Club or a grocery store and hand out PKD information
The Reluctant Donor

by Suzanne F. Ruff
The Reluctant Donor by Suzanne F. Ruff

- Susanne received 2 calls in June and 1 in May from film producers to turn this book into a movie

- This book is about how PKD affects their family going back multiple generations, family dynamics, Suzanne is reluctant to donate her kidney to her sister-JoAnn, and history of PKD.

- Encourage everyone to buy the book from beaverspondbooks.com or 1-800-901-3480 for $15. Proceeds will be donated to the PKD Foundation.

- Submit a review on amazon.com (If buy book from Amazon, proceeds does not get donated.)
Walk for PKD
Walk for PKD
A cure is our finish line!

Money = Research

• It is an investment into your families future
Walk for PKD

- Sunday, October 8, 2017 at 9:30 AM-12:30 PM
- Location: Tower Grove Park, Sons of Rest Pavilion, St. Louis
- On-line registration is open. Please register your team and gather team members
Sponsorship

• Collected $500 Scott Credit Union Sponsorship

• SSMHealth Transplant Center - freeze on spending - Hoping they will be able to donate $1000 by September 1.

• Barnes Jewish Hospital wants to be the only big sponsor and no competition. They would be okay with WASHU and St. Louis College of Pharmacy. They need 6 months or more notice.

• I contacted local DaVita and Fresenius. They said we need to contact corporate.

• September 1 deadline for sponsorship name on back of T-shirt.
Walk for PKD

• Walk is responsible for nearly 1/3 of our annual operating budget

• This year, 100% of Walk fundraising dollars goes directly to research

• People do not donate to PKD because they have never been asked.

• Ask your walkers to get 10 people to donate $10. This will get them to raise $100 to earn them an official Walk for PKD t-shirt.
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<th>2017 Walk for PKD Sponsorship Packages</th>
<th>Presenting $10,000</th>
<th>Diamond $7,500</th>
<th>Platinum $5,000</th>
<th>Gold $2,500</th>
<th>Silver $1,000</th>
<th>Bronze $500</th>
<th>Friend $300</th>
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<td>Logo placement on print materials (posters, brochures, etc.)*</td>
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<td>Logo placement on local Walk for PKD Banner*</td>
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<td>Hang your company banner at local Walk</td>
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<td>Booth/tabletop display at local Walk</td>
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<td>T-shirts for your registered Walk for PKD team members</td>
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*Print material entitlements are subject to deadlines and will be recognized based on receipt of the sponsorship agreement form.
St. Louis Chapter Calendar
St. Louis Chapter Calendar

- August 14 Allison Hawk Collinger from AHC Consulting/Stryker - Munely Group - St. Louis Strategic Communications - learning different ways to promote our Chapter, fundraisers, and events with social media and media.

- September 11 Perfect Strangers movie - documentary about a perfect stranger donating a kidney to a PKD patient.
Perfect Strangers

- A documentary directed by Jan Krawitz

- This movie raises questions about what motivates an individual to donate a kidney to a perfect stranger, PKD patient

- The documentary tells the story of two unique and engaging characters.

- One is Ellie, who is determined to give away one of her kidneys. She lives 500 miles away from Kathy.

- Kathy endures nightly dialysis and loses hope of receiving a transplant until Ellie reads her profile on an online website.

- Both women face unexpected challenges as their parallel stories unfold over a course of four years.
St. Louis Chapter Calendar

- October 8 St. Louis Walk for PKD Fundraiser 9:30 AM-12:30 PM at Tower Grove Park, Sons of Rest Pavilion (register online, form a team, donate, and ask others to donate)

- October 9 No Meeting

- November 13 Guest speaker Clinical Trials??

- December 11 No Meeting - Winter Break
Karen Thurman
10 years as a Senator
10 years in Congress
Advocacy Expert
Advocacy

- Advocacy is the foundation of democracy.
- It is an attempt to influence public policy, by a person or group, in a way that brings about legislative action or change.
- PKD goal to broaden Government affairs and advocacy
- Have a full time person in DC - Karen Thurman
- Increase interaction with Congress
Make Yourself Known

- Find a Representative at [house.gov](https://house.gov)
- Find your two Senators at [senate.gov](https://senate.gov)
- Send an initial message and/or request a meeting.
- Indicate you’ll be contacting them in the future on other matters of interest to PKD.
- When you have a meeting, you have 10 minutes so the “ask for” is crucial.
How to inform Congress of PKD

• Keep it simple at first - PKD is a genetic kidney disease with no treatment or cure and costs the government a lot of money.

• Make it personal: how it impacts you, your family, your lifestyle, how big this disease is, how important it is to fund research, etc.

• One year of hemodialysis can cost up to $72,000, while a year of peritoneal dialysis costs about $53,000, according to U.S. Renal Data System - Oct. 11, 2014.

• Why certain action is important and how Washington can help.

• PKD Foundation website contains all the information you need for the first contact pkdcure.org/advocacy
The “ASK FOR” is Crucial

• An example: the Living Donor Protection Act (HR 1270). If a cosponsor, thank them.

• If not a cosponsor, explain how the bill would help PKD Patients.

• Request the Member’s or Senator’s position - this requires a response to you.

• Pass on the response to PKD Foundation.
Get Others involved

• As a PKD advocate, you have many contacts: family, neighbors, PTA, work colleagues, etc.

• Translate those contacts into a grassroots advocacy list.

• Let these potential allies know why you need their help.
Create a Contact Tree

- E-mail group
- Social media
- Some way to hit a button and get your message out when immediate action is necessary.
Stay in Touch

• Nearly every Senator has electronic newsletters or updates. Sign up for Health, Appropriations (Funding), and Budget.

• If the Representative is on social media, follow him or her. You may learn about town hall meetings and other events where you can make your case in person.

• Every so often, make another request. Join the Kidney Caucus. Join the Rare Disease Caucus.
When is immediate action required?

• Grassroots or Wildfire?

• Grassroots: Join the Kidney Caucus, support more funds for NIH, or cosponsor the immunosuppressant drug bill (when introduced)

• Wildfire: Action is required immediately. For example, Senate action on Obamacare replacement bill.
The Next few Days are Crucial

- Senate action on health care reform (ACA repeal and/or reform), HR 1628.

- Your contacts should be your two US Senators.

- If your state has several chapters, contact other local chapter leaders to coordinate action (send the same message).
American Care Act or American Health Care Act or What?

- Essential Benefits

- Now: Coverage for hospitals, outpatient care, lab work, etc.

- House bill: If given to the State, the state can waive any or all essentials such as ambulance, ER, Hospital, Pregnancy, new born, mental health, labs, preventative services, pediatric, maternity, prescriptions, and etc.

- Senate bill: ????
ACA or AHCA or What?
cont.

- Preexisting Conditions
  - Now: Plans cannot deny coverage
  - House bill: State may waiver coverage if no insurance for 63 days; then 30% surcharge
  - Senate bill: ???
ACA or AHCA or What? cont.

- **Community rating**
  - Now: No additional premium cost for preexisting conditions
  - House: State waiver could allow higher premiums.
  - Senate: Keeps ACA language????

- **Possible Impact of Waiver on Employer**
  - Move health plan to state with fewer essential health benefits (EHB) etc.
Summer Recess is NOT a vacation

- Congress away from DC July 31-September 4 (as of 2017)
- May have office hours or at town hall meeting - your story is important with staff people.
- Visit their websites to get scheduling information
- Same procedures as initial contact to ask for a meeting
  - What is PKD
  - How it impacts you and your family
  - Not excepting high risk pools, wave out. Why they would be bad for your spouse, children, and living donor.
Summer Recess is NOT a vacation cont.

- Have a definite “ask for”
  - Health care reform
  - National Institute of Health (NIH) funding
  - Cosponsor a specific bill
- Send a thank you and promise to contact in the future on additional items to the local office. They will group them together and send a huge pile to DC. It will be faster.
- Advise PKD Foundation of the results of the meeting.
Once is not enough

• A lot of people vie for attention of elected officials.

• The benefit of action may not be obvious to the officials and their staffs.

• Cost of action may concern them.

• They just do not know you for the issue.

• Your advocacy can overcome these obstacles.
Advocacy Cont.

• Join us on a local level to educate your local elected official how they can help fight PKD

• Success patient policy
Be personal and persistent but be polite

- Stress Impact of PKD: quality of life, financial impact, family impact, etc.
- How PKD differs from other kidney diseases.
- How more research will provide hope to you and your family.
- How health care repeal/reform will impact your family and you.
- Do not get discouraged.