



National **Kidney** Foundation®



**NEPHCURE**®  
Kidney International

# Externally Led Patient-Focused Drug Development Meeting on FSGS

August 28, 2020

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## **AGENDA: EXTERNALLY LED PATIENT-FOCUSED DRUG DEVELOPMENT MEETING ON PRIMARY FSGS**

- 10:00 – 10:05am**      **Welcoming Remarks**  
*David Feldman, PhD, National Kidney Foundation*
- 10:05 – 10:15**      **Opening Remarks**  
*Aliza Thompson, MD, US Food and Drug Administration*
- 10:15 – 10:25**      **FSGS: Natural History of Disease and Treatment**  
*Laura Mariani, MD, Univ. Michigan*
- 10:25 – 10:35**      **Overview of Discussion Format and Demographic Polling Questions**  
*James Valentine, JD, MHS, Moderator*
- 10:35 – 10:55**      **Panel Presentations on Topic 1**  
Living with FSGS: Disease Symptoms and Daily Impacts
- 10:55 – 11:55**      **Polling Questions and Virtual Audience Discussion on Topic 1**  
Living with FSGS: Disease Symptoms and Daily Impacts  
*James Valentine, Moderator*
- 11:55 – 12:05pm**      **Challenges to FSGS Clinical Trial Design**  
*Suneel Udani, MD, Nephrology Associates of Northern Illinois*
- 12:05 – 12:35**      **Polling Questions and Virtual Facilitated Audience Discussion on Topic 2**  
Clinical Trials in FSGS Using Traditional Approval Pathway  
*James Valentine, Moderator*
- 12:35 – 1:05**      **Lunch Break**
- 1:05 – 1:15**      **Introduction to Accelerated Approval Pathway for New Drugs**  
*Kimberly Smith, MD, US Food and Drug Administration*
- 1:15 – 1:45**      **Polling Questions and Virtual Facilitated Audience Discussion on Topic 3**  
Clinical Trials in FSGS Using Accelerated Approval Pathway  
*James Valentine, Moderator*
- 1:45 – 2:05**      **Panel Presentations on Topic 4**  
Current Challenges to Treating FSGS
- 2:05 – 3:05**      **Polling Questions and Virtual Facilitated Audience Discussion on Topic 4**  
Current Challenges to Treating FSGS  
*James Valentine, Moderator*
- 3:05 – 3:15pm**      **Closing Remarks and Next Steps**  
*Lauren Lee and Joshua Tarnoff, NephCure Kidney International*
- Adjourn**

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## MESSAGE FROM OUR LEADERSHIP

Hello FSGS EL-PFDD Participants,

It is our great pleasure to welcome you to this virtual Externally led Patient-Focused Drug Development Meeting. The National Kidney Foundation (NKF) and NephCure Kidney International are proud to partner in presenting this meeting. In recent years, we have been fortunate to see a significant increase in the number of late phase clinical (human) trials in FSGS, so this meeting takes on special importance. We thank you for your commitment to this effort by preparing for the meeting and participating in today's proceedings.

This gathering is an important milestone in the development of FSGS treatments because it is an opportunity for you, FSGS patients and caregivers, to speak directly to the FDA and voice your experience with this disease and what you need from new therapeutics. The FDA recognizes you as experts in facing FSGS, and they want to hear your experiences and preferences. You *are* the "voices of patients."

We thank the representatives from the FDA for taking time to attend today's meeting to learn from the patients whose lives, every day, they strive to improve. In particular, we thank Drs. Aliza Thompson and Kimberly Smith for the informative presentations they will make today.

EL-PFDD meetings are particularly significant to both of our organizations because of our dedication to accelerating change through research, support, and education so patients like you, can live healthier and longer lives. The patient testimonies and experiences shared through today's meeting landmark a pivotal time of change. Today is truly one of the most important days in the history of FSGS. Today, we work together to accelerate and improve future treatment options for all who live with FSGS.

We want to especially acknowledge the panelists for today's meeting; we are grateful for their preparation and commitment to the meeting.

We are grateful to our Co-chairs, Drs. Laura Mariani and Suneel Udani, whose collective expertise and guidance will be evident here today.

Finally, we are very grateful to Boehringer Ingelheim, Cycleron, Mallinckrodt, Retrophin, and Vertex for sponsoring this meeting.

We know that all of you will make this an extraordinary meeting. Thank you very much for your significant contributions today.

Sincerely,



A stylized, handwritten signature in black ink, consisting of a large 'K' followed by a few loops.

Kevin Longino, CEO  
National Kidney Foundation



A handwritten signature in black ink that reads "Joshua Tarnoff" in a cursive script.

Joshua Tarnoff, CEO  
NephCure Kidney International

## ABOUT THIS MEETING



Externally Led Patient-Focused Drug Development (EL-PFDD) meetings are the Food and Drug Administration's (FDA) response to the need for more systematic and detailed documentation of the burden imposed on patients by particular diseases, and to patient experiences with currently available treatments.

EL-PFDD meetings bring together patients and their care-partners, representatives from the FDA, pharmaceutical companies, and other stakeholders who focus on a particular disease. In these meetings, patients' voices come to the forefront to inform the FDA on what matters most to patients as they live with their disease and what patients feel is needed from future clinical trials and therapeutics. The FDA is particularly interested in learning about the personal and family burdens imposed by the disease, as well as the efficacy, side effects, strengths, and limitations of available treatments for the disease in question.

During today's EL-PFDD meeting on FSGS, the FDA will hear specific, real-life examples of how FSGS affects patients, their care-partners, and families. In addition, participants will inform the FDA of their successes and frustrations in treating their FSGS, as well as what they are looking for in new drugs. The FDA will use this information in their deliberations when they evaluate new potential drugs for FSGS.

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## BIOS OF TESTIMONY PANELISTS

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**Jacqueline Botta**

*Danbury, CT*

Jacqueline is a kidney healthcare advocate and rising sophomore at Western Connecticut State University in Danbury, Connecticut. She was diagnosed with idiopathic FSGS at the age of 14. Her journey with CKD has been bumpy but hopeful. Jacqueline and her family are committed to sitting at the table to discuss options to enhance the life of patients with CKD, especially kids with FSGS. Jacqueline is one of the founding members of Kidney Fight Club, an Instagram group for teens suffering with glomerular disease and their side effects. She and her family are working towards finding effective therapies and ways to connect young people with these diseases.



**Taylor Faulkner**

*Fort Walton Beach, FL*

Taylor is 24 years old and lives with her family in Fort Walton Beach, Florida. She has always had a deep love for musical theater, baking and traveling. After her diagnosis of FSGS at age 18, Taylor's focus shifted toward wanting to give back to the Kidney Community. Taylor will be returning to school to complete a degree in Public Relations and pursuing a career geared toward helping others with FSGS.



**Christine Gwinn**

*Battle Creek, MI*

Christine was diagnosed with FSGS in 2012. She is 46 years old and lives in Battle Creek, MI with her fiancé. Christine graduated with a B.S. in Biology from Grand Valley State University. Her career has involved molecular assay development for nutritional supplement companies and food safety microbiology. Christine is an avid skydiver, backpacker, scuba diver and she is currently working on getting her private pilot license.



**Nicki Kaplan**

*Detroit, MI*

Nicki was diagnosed last year with nephrotic syndrome just before her graduation from Northwestern University. She currently resides in the Detroit area, where she works in Product Management. Outside of work, Nicki is a self-published author and volunteers for an organization that re-homes retired racehorses. In her free time, she enjoys fitness, healthy cooking, horseback riding, writing, and cuddling with her cats. Recently, Nicki has made it her mission to advocate for the FSGS community.



**Alyse and Melissa Naquin**  
*Lafayette, LA*

Alyse is a 14-year-old incoming freshman to high school. She enjoys reading, baking, playing piano, shopping, and hanging out with friends and family. Alyse's dream is to become a pediatric nephrologist where she can truly empathize with her patients and help find a cure for this disease! Melissa is the proud mother of Alyse.



**Becky Oag**  
*Marshfield, MA*

Becky is 40 years old and lives in Marshfield, MA with her husband and two children. She first discovered proteinuria when she was pregnant with her second child and was officially diagnosed in 2014 with FSGS Stage 1. Becky has been treatment-resistant and had a second biopsy in 2018 where it was discovered she had a recessive genetic mutation. She has been actively involved with NephCure since her diagnosis and sits on their Board of Directors.



**Bernadine Watson**  
*Washington, D.C.*

Bernadine ("Dine") is a writer and poet who lives in Washington, DC with her husband, Joe. She was diagnosed with FSGS in 1984. Dine has had two kidney transplants and spent five years on dialysis. Her social policy writing has been published in the Washington Post and her poetry has appeared in the Beltway Poetry Quarterly. Dine reads her poetry throughout the Washington, DC region. She is working on a memoir about her experiences living with FSGS.



**Christopher Windisch**  
*Orange County, CA*

Christopher is 47 years old and lives in Orange County, California with his wife of 17 years and 2 sons, Christopher Jr. and Alexander. Christopher has worked for 25 years in technology sales and leadership roles and is currently the founder of several tech startups. He also serves as the SoCal Regional Lead for NephCure and sits on their Board of Directors. Christopher was diagnosed with FSGS in January 2010 and is currently in partial remission.

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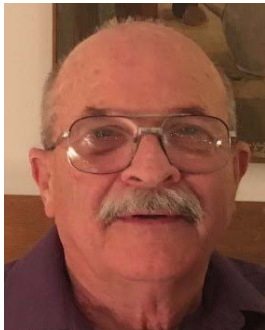
## BIOS OF DISCUSSION PANELISTS

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**Valerie Bailey**  
*Birmingham, AL*

Valerie is from Birmingham, Alabama and has two sons. She was a registered nurse for over 30 years, first working as a staff dialysis nurse. She retired recently. Valerie received a kidney from her son in 2007. In 2012 she addressed Congress about the importance of federal funding for the expenses following a kidney transplant. Valerie actively educated her patients on preventing kidney disease and living kidney donation. She hopes to continue to educate her community in Alabama and Congress on kidney disease.



**Kent Bressler**  
*Kerrville, TX*

In 1982, Kent was diagnosed with FSGS and in 1987 he received a kidney transplant from his brother Kip. Kent is a US Army Veteran and a retired RN. He worked in the VA and the private sector for over 40 years. Kent is a Peer Reviewer for the Patient-Centered Outcomes Research Institute and the Department of Defense and has written articles on preemptive kidney transplant and immunosuppressive drug coverage. Kent co-founded Kidney Solutions, a 501c that helps patients find a kidney donor without fees. He is an active advocate, committed to assisting kidney disease patients. Kent and his wife Catherine celebrated their 50th wedding anniversary in 2019.



**Karlene and Zane Cabinson**  
*Irvine, CA*

Karlene is from Irvine (California) and the mother of 4-year-old Zane. Zane was diagnosed with Nephrotic Syndrome/FSGS at 15 months old. In 2018, he had over 8 hospital admissions and his nephrologists struggled to bring him into remission with multiple strong medications (prednisone, CellCept, tacrolimus). Fortunately, Zane has been in remission and much more stable for about 1.5 years now on tacrolimus and enalapril. Karlene is dedicated to helping find a cure and creating more awareness around nephrotic syndrome/FSGS to help other families and kids.



**Kayla Coon**  
*Clarksville, TN*

Kayla Coon is 29 years old and a wife, mother of 3, and emergency roadside assistance dispatcher. She was diagnosed with FSGS two weeks before her 16<sup>th</sup> birthday and learned this year that a genetic mutation, PAX2, is the cause. When she is not researching, advocating, or studying about FSGS and treatment options, Kayla spends time with family, singing, or honing her photography and pastel drawing skills. Kayla's mother suffered from kidney disease as well, so advocating for kidney disease awareness and the effects on those living with it is very important to her. Kayla feels honored to serve on the Discussion Panel and hopes that revealing the struggles patients face every day will help to develop new treatment options.



**Erich Ditschman**  
*East Lansing, MI*

Erich inspires dialysis patients and others living with chronic illness to get outdoors everywhere he goes – and online through Facebook page, and Paddling on Dialysis for Kidney Health. Erich learned in high school that he had lost fifty percent of his kidney function from an unknown cause. In 2001, he received a kidney transplant from his wife, Andria, which started to fail due to FSGS soon after the surgery. In 2005, a second kidney transplant failed soon after surgery, again from FSGS. After undergoing in center hemodialysis and peritoneal dialysis, in 2006 Erich started home hemodialysis. To celebrate his new vigor for life, he canoed Michigan’s longest river, the Grand River, over ten days to raise money for kidney education and research. Each night he performed nocturnal “home” hemodialysis. Erich lives with his family in the East Lansing, Michigan.



**Katey and Reed Dominick**  
*Grand Rapids, MI*

Katey is 42 years old and lives in Grand Rapids, Michigan, with her husband and two kids. Her son Reed was diagnosed with minimal change disease at the age of 3 after weeks of several pediatrician trips to determine why he was so sick. A few months later, after a kidney biopsy, Reed was diagnosed with FSGS and has tried several treatments to help control this disease. Now 10, Reed has experienced periods of remission, but the family lives with the unknown of when his disease will strike again. Katey's primary goal is to advocate for her son and share her family's experience to help others dealing with this disease.



**Janice Earle**  
*Tucson, AZ*

Janice is 68 years old and lives with her husband in sunny Tucson, Arizona. She retired seven years ago from the University of Arizona Campus Health Service where she supervised the medical records department. Janice is an avid animal lover and has spent some of her spare time volunteering with a local greyhound rescue and animal shelter. Travel and cooking are also interests of hers and she loves to experiment with kidney-friendly ingredients to create tasty new recipes. She enjoys keeping active with exercise and gardening. Janice was diagnosed with CKD in 2010 and FSGS via biopsy in 2017. She completed a six-month clinical study in February 2020 which evaluated the safety and efficacy of the investigational drug CCX140-B in subjects with FSGS. Janice is hoping that the recent Executive Order on advancing kidney health will ultimately open a pathway to experiencing a better quality of life for kidney patients in all stages of the disease.





**Cheryl Gitter**  
*Pahrump, NV*

Cheryl is 68 years old and lives with her husband of 44 years and their dog. They have one son and two grandchildren who live in Wisconsin, where they originally lived. Cheryl is a retired RN/Clinical Research Monitor, after working over 23 years in critical care areas of hospitals and 19 years in clinical pharmaceutical research trials. After undergoing a lot of testing to rule out other diseases, she was finally diagnosed in 2009 with primary idiopathic FSGS. Since being diagnosed, she has spent time learning as much as possible about the disease and available treatments and participating in patient forums. She enjoys spending time with family and friends. Cheryl is looking to advocate in any way to help those with FSGS find a voice. She has a strong belief in patients/caregivers having a major role as part of their treatment team.



**Ely Grau**  
*Mesa, AZ*

Ely is 57 years young. She was diagnosed with FSGS at 41 years old. In her doctor's office, she heard only “you have blood in your urine and you’re spilling 3,000 mg of protein; you are in stage 4 CKD and close to going on dialysis.” Ely thought it was a bad dream. In 2003/2004, Ely started steroid therapy which brought her to remission, where she currently is.

Ely took control of her body and outlook on life as she did not want FSGS to control her destiny. Even after battling thyroid cancer and neuropathy on a daily basis, life is great for Ely. She has a beautiful daughter and a great daughter-in-law. Ely’s loves are her two dogs. Her passion is cooking, and her daughter and niece have convinced her to write a book on her journey with FSGS by using cooking to better her kidney health.



**Jill Hammonds**  
*Columbus, OH*

Jill is 43 years old and resides in Columbus, Ohio with her husband and 5-year old son. She is a Licensed Independent Social Worker who has lived with FSGS for over 29 years. Jill’s journey has included three kidney transplants, and a wild journey of various treatments and medications. Jill strives to always learn more about FSGS and ways in which she can live a healthier, fuller life. In her free time Jill enjoys creating stories with her son, playing with her puppy, and helping her husband with their photography business. Jill is participating as a panelist to be proactive, help find a cure, and educate others about her personal experience as a patient living with FSGS.



**Dan Keaveney**  
*Baltimore, MD*

Dan noticed his energy and endurance decreasing while his belly was starting to swell over the last several years. During a holiday party in December 2018 his leg alarmingly swelled over the course of minutes. He was diagnosed with nephrotic syndrome on December 31<sup>st</sup> and with FSGS in early February. He spent that entire month in the hospital trying to control the disease that was stealing his joy. Dan used to live a healthy and continually active lifestyle – whether physically at work or helping friends and family. Now he is disabled and must plan two days ahead to have energy for planned activities. He must accommodate FSGS with his daily decisions about balancing energy with the activities of the day. Also, with immunosuppressant treatment, Dan must be vigilant against everyday diseases as he will be affected worse than in the past.



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**Fred J. Kuo, Cristina Dieguez-Kuo, and Ani Kuo**  
*Wethersfield, CT*

Fred (48) and Cristina (43) reside in Wethersfield, CT and have three children, Lucas (11), Javier (8), and Anabel (6). Their daughter Anabel (Ani) was diagnosed with FSGS at 13 months old and is currently in remission. Ani is entering 1<sup>st</sup> grade and loves art, skating, soccer, and playing the piano.

Cristina is the Campaign Director at Miss Porter's School and has held various development roles at the University of New Haven and Fairfield University. Fred is a Senior Associate Director of the Annual Fund at the Loomis Chaffee School. Prior to Loomis, Fred spent nearly two decades in higher education.



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**Eftihia and Olivia Neupauer**  
*Lancaster, PA*

Eftihia is a 37-year-old patient parent who lives in Lancaster, PA with her husband, Andy, their three daughters, and son. Her 5<sup>th</sup> baby (girl) is due in January 2021. Eftihia has been an Autistic Support Teacher for 15 years.

Eftihia's daughter, Olivia, was diagnosed with minimal change disease in 2018 soon after her 4<sup>th</sup> birthday. She is steroid-resistant and has been treated as though diagnosed with FSGS. Olivia has experienced a seizure,

blood clots and thrombotic microangiopathy. She participated in the Liposorber Clinical Trial 2019 and while not achieving remission, Liposorber "reset" her kidneys and she avoided the transplant discussion. Since her diagnosis, Olivia has experienced depression, not understanding why her life changed so abruptly. However, Olivia is currently her delightful self, despite still leaking protein.



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**Diane Osgood**  
*Oswego, IL*

Diane helps companies build profits with purpose, innovate for environmental and social solutions, and connect with key communities on difficult issues. She has worked with Fortune 100 and 500 companies as well as private equity around the world for over 30 years.

Diane was a Senior Advisor to former President Clinton at the Clinton Global Initiative (CGI). She was a founding board member of the Vatican Arts and Technology Council, a cross-sector effort to apply technology to spread tenderness across the world. She is a long-term advocate and thought leader in developing solutions to end slavery in supply chains. Diane holds a PhD in Environmental Economics and Development Studies from the London School of Economics. She consults and writes.



**Kimberly Queen**  
*Douglasville, GA*

Kimberly is 33 and lives in Douglasville, GA. She was diagnosed with FSGS at 25 in 2012. Her FSGS progressed quickly because of strep that turned into septic shock. She was diagnosed in January and on dialysis by September. Kimberly did dialysis for two years and received a kidney transplant from her brother in 2014. Unfortunately, FSGS reoccurred immediately. She is five-year kidney strong, but she still fights to put the FSGS into remission. Kimberly has tried numerous treatment options with no results. She currently does plasmapheresis and has completed over 400 treatments. Kimberly has advocated in Washington D.C. for about four years, because she knows one day there will be better treatments and a cure for FSGS.



**Jen Trunk**  
*Chaska, MN*

Jenn lives in Minnesota with her husband, daughter, dogs, and cat. She was diagnosed with FSGS in 2005 and was able to stave off the disease until 2014 when she had to start emergency dialysis. For the next year Jenn did peritoneal dialysis at home. In 2015 she received a living donor transplant from a friend, but FSGS returned two days after surgery and Jenn started dialysis again in 2017, first in-center hemodialysis and eventually transitioning to nocturnal hemodialysis at home. Jenn enjoys photography, cooking, hot yoga, and volunteering with a local animal rescue. She is involved in advocacy with the National Kidney Foundation and has volunteered for NephCure. Jenn is currently listed for another transplant at the University of Minnesota.



**Acacia Walter-Rooks**  
*Grand Rapids, MI*

Acacia is 18 years old and recently graduated from East Grand Rapids High School. She will be a freshman at Calvin University this fall. Acacia enjoys playing tennis, photography, being with her friends, and traveling. She has lived with FSGS for 8 years. Acacia was diagnosed at 11 years old after coming home from a camping trip. Since FSGS attacked both of her kidneys she underwent a double nephrectomy and dialysis for several months, along with a strict diet and water restrictions. Thankfully, Acacia received a living donor transplant at the age of 14. Living with FSGS has totally changed her life. Now, every day Acacia must take medicine, drink at least three liters of water, and in general be careful not to harm her body. But overall, she has overcome these challenges and has become a stronger person.



**Curtis Warfield**  
*Indianapolis, IN*

Curtis is a Senior Quality Analyst from Indianapolis, IN. In 2012 he was diagnosed with FSGS and started peritoneal dialysis by December 2014. When his daughter was deemed unsuitable to be his living donor, her college sorority sister donated a kidney “just because” she wanted to help.

Curtis’s passion is advocating and educating about CKD, dialysis, organ donation and living donors. He advocates on Capitol Hill and provides peer counseling for CKD and post-transplant patients and their families. He has spoken at schools and various groups. Curtis has written articles for newsletters, newspapers and was featured in annual reports of kidney and organ donation organizations. Curtis serves on Technical Expert Panels and study groups for CKD and dialysis and is on the Board of Directors of Home Dialyzors United and the National Kidney Foundation – Indiana. Curtis has been married for 34 years and has 4 adult children and one grandson.



**Michelle, Paizley, and Bentley Welch**  
*Grande Prairie, Alberta , Canada*

Michelle is the mother of four children, two of whom have FSGS congenital nephrotic syndrome. Michelle's 13- and 11-year-old children are healthy.

Bentley is 3 years old and was diagnosed at 10 months of age. He immediately began maintenance therapy and once a week albumin infusions. Paizley is 6 years old and was diagnosed at 2 years of age, just ten days before Bentley was born. Paizley's journey has been difficult. In

addition to maintenance medication and albumin infusions three times weekly, she was treated over eight months with prednisone, cyclosporine, tacrolimus, and rituximab. None of the drugs were effective. This was eventually explained by genetics testing, which showed NPHS2 and SMARCAL1. Michelle sees no options left other than a kidney transplant when Paizley's kidneys fail.

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## SPEAKER BIOS

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**David Feldman**

*National Kidney Foundation*

David received a PhD in Biology from the State University of New York at Binghamton and was a post-doctoral fellow at the Cleveland Clinic Foundation. He joined the National Kidney Foundation in 2015 as a Medical Project Director after enjoying a career of more than 30 years conducting preclinical research in cardiovascular and kidney disease in the pharmaceutical industry. David is passionate about making a difference in the lives of patients with kidney disease through patient education and engagement. He has a daughter and lives with his wife in Teaneck, NJ.



**Lauren Lee**

*NephCure Kidney International*

Lauren is the Executive Vice President, Stakeholder Engagement at NephCure. She leads strategy and programming around the organization's research-focused mission of expediting treatments for glomerular diseases. This involves the development of content and messaging designed to empower the patient community to make informed decisions around their healthcare. Lauren also focuses on stakeholder collaborations to connect patients and physicians to clinical trial opportunities.

Lauren holds a Master's degree in History Museum Studies from the State University of New York at Oneonta. Throughout her career, Lauren has worked in nonprofit settings, including the University of Pennsylvania Health System. Prior to joining NephCure, Lauren worked in investor relations at Formula Pharmaceuticals, an oncology biopharmaceutical start-up.



**Laura Mariani**

*Co-chair*

Laura Mariani, MD MS, is a nephrologist and clinical researcher at the University of Michigan. She is an Assistant Professor of Medicine in the Division of Nephrology at the University of Michigan. Laura completed her undergraduate education at Harvard University, medical training at the University of Michigan, and residency, nephrology fellowship, and Master's of Science in Clinical Epidemiology at the University of Pennsylvania. She sees patients with glomerular disease and teaches medical students. Her primary interest is glomerular disease and predicting its

progression.

Lauren lives in Ann Arbor, MI with her husband and their three boys. Her passion is to improve the lives of patients with nephrotic syndrome by working with scientists to find more effective and less toxic therapies.



**Kimberly A. Smith, MD, MS**  
*US Food and Drug Administration*

Dr. Kimberly A. Smith, MD MS, is a nephrologist and clinical team leader for products being developed to treat kidney diseases in the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research (CDER), at the U.S. Food and Drug Administration (FDA). Prior to joining the FDA, she was with the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services (CMS). Dr. Smith is a graduate of the University of Michigan Medical School, and she completed her residency and chief residency in Internal Medicine and fellowship in Nephrology at Vanderbilt University Medical Center. She then returned to the University of Michigan as faculty and obtained a Master of Science in Health and Healthcare Research through the Robert Wood Johnson Foundation Clinical Scholars Program.



**Joshua M. Tarnoff**  
*NephCure Kidney International*

Josh came to NephCure in early 2018 when he fulfilled a long-held desire to more closely serve the rare disease patient community. Josh spent over 30 years working in the pharmaceutical and biotechnology community moving new therapies closer to reality. Personal contact with NephCure families drives his passion to achieve the goals of our mission and he always appreciates directly speaking with our community. Likely due to a snake bite, he also has personal insights into renal disease, having been a patient.



**Aliza Thompson, MD**  
*US Food and Drug Administration*

Dr. Thompson is Deputy Director of the Division of Cardiovascular and Renal Products, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Division of Cardiovascular and Renal Products regulates and reviews Investigational New Drug Applications and marketing applications for drug and biologic products for the treatment of cardiovascular and kidney diseases. Dr. Thompson joined the FDA in 2007. Prior to her current position, Dr. Thompson served as a clinical team leader for products being developed to treat kidney diseases. She received her medical degree from Johns Hopkins Medical School and completed her Internal Medicine and Nephrology training at Columbia University/New York-Presbyterian Hospital. She holds a Master of Science in Biostatistics/Patient Oriented Research Track from the Columbia University Mailman School of Public Health.



**Suneel Udani**

*Co-chair*

Suneel Udani, MD MPH, is a clinical nephrologist in the community (private) practice Nephrology Associates of Northern Illinois (NANI) in the Chicago metropolitan area. Also, as an Associate Member of the University of Chicago Section of Nephrology, he teaches glomerular disease to Nephrology fellows.

Dr. Udani completed his undergraduate and medical school training at Northwestern University and his residency and fellowship at the University of Chicago, with one year at the University of Pittsburgh. He focuses on patient care, but also serves as the local principal investigator for a clinical trial on FSGS. Dr. Udani's primary interests are glomerular disease, diabetic kidney disease, and cardiorenal (heart-kidney) syndrome. He concentrates on optimizing the delivery of care to patients with kidney disease, with the goal of preventing the progression of their disease. Dr. Udani lives in Chicago—the city in which he was raised and continues to love—with his wife, daughter, and son.



**James Valentine, JD, MHS**

*Moderator*

James Valentine is an associate at Hyman, Phelps & McNamara where he assists clients in the medical product industry and patient advocacy organizations in a wide range of regulatory matters, including new drug and biologic development and approval issues. Mr. Valentine has been central to the transition of the FDA Patient-Focused Drug Development (PFDD) program to externally-led meetings, having helped plan and moderated the majority of those, and is also working on novel methodologies for capturing patient experience data. Mr. Valentine has also

authored an analysis cataloguing FDA's flexibility in assessing efficacy in the approval of drugs for rare, or orphan, conditions.

Before joining the firm in 2014, Mr. Valentine worked in FDA's Office of Health and Constituent Affairs (previously Office of Special Health Issues) where he facilitated patient input in benefit-risk decision-making and served as a liaison to stakeholders on a wide range of regulatory policy issues. Mr. Valentine administered the FDA Patient Representative Program, facilitated stakeholder consultations during the reauthorization of PDUFA and MDUFA, helped launch the PFDD program, and developed the FDA Patient Network.

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## MEETING ORGANIZERS

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**Kelly Helm**

*NephCure Kidney International*

Kelly is the Associate Director of Patient Advocacy for NephCure Kidney International, where she advocates for patients across the globe, supporting, educating, and connecting them with each other. Kelly started with NephCure as a volunteer walk leader 10 years ago. After working as a regional coordinator, she was promoted to a full-time employee in 2014. Kelly's favorite thing is to help patients and their families feel less isolated and alone in their journey and to provide hope. She is also known as "Macy's mom." Macy is a 14-year-old recurrent FSGS patient who was diagnosed at the age of 3, in 2009. Macy had a living donor kidney transplant at the age of 5 and she recently celebrated her 9-year kidneyversary this June.



**Kristen Hood**

*NephCure Kidney International*

Kristen, MSA BA RN, serves as the Director of Clinical Outreach for NephCure Kidney International. She is a Registered Nurse with over 16 years of experience and has a Master's degree in Nursing Education. Kristen first became involved with NephCure in 2016 as a volunteer leader after her son, Logan, was diagnosed with Nephrotic Syndrome. She joined the NephCure team professionally in 2019 where she educates the patient community through multiple patient education programs and advocates for them in clinical research and clinical trials. Kristen also works closely with the nephrology physician community and other key stakeholders to ensure the advancement of research and better treatment options for protein-spilling kidney disease patients. Kristen resides in San Antonio, Texas with her husband Randy, children, Ben, Logan, and Emma, and their beloved Vizsla, Copper Sky.



**Sarah Kim**

*National Kidney Foundation*

Sarah is the Corporate Relations and Development Assistant at the National Kidney Foundation. She graduated from Rutgers University in 2014 with a BA in English. Over the last two years, she has become passionate about patient advocacy and further progressing NKF's mission to improve the lives of patients with kidney disease. Sarah lives in Ridgewood, NJ with her husband.



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## DISCUSSION QUESTIONS

### Topic 1: Living with FSGS: Disease Symptoms and Daily Impacts

1. Of all the symptoms that you experience because of your condition, which 1-3 symptoms have the most significant impact on your life?
2. Are there specific activities that are important to you but that you cannot do at all, or as fully as you would like, because of your condition?
3. How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days?
4. How has your condition and its symptoms changed over time?
5. What worries you most about your condition?

### Topic 2: Clinical Trials in FSGS Using Traditional Approval Pathway

1. What information is important for you to understand when considering participation in a clinical trial?
2. In which type of clinical trial would you be more likely to enroll:
  - a. A trial that studies how well a new drug manages the underlying cause of kidney damage in FSGS but does not study symptoms or progression
  - b. A trial to study if and how well a new drug reduces symptoms of FSGS
  - c. A trial to study if and how well a new drug reduces progression of FSGS
3. What would be most important in deciding whether to participate in a clinical trial (e.g., frequency of 24-hour urine, travel to study site, number of biopsies, frequency of clinic visits)?
4. Does the type of medication (e.g. pill, IV infusion, injection) and/or the frequency of taking the medication influence your decision to participate in a clinical trial?

### Topic 3: Clinical Trials in FSGS Using Accelerated Approval Pathway

You are considering whether to enroll in a clinical trial where you have a chance of being given either a potential medication for FSGS or standard of care treatment (e.g., prednisone, ACE inhibitor) and you won't know which you are getting.

- The trial will evaluate whether the medication lowers protein in your urine (proteinuria) in the first phase.
  - If the trial shows that proteinuria is lowered enough, the medication will be approved under the accelerated approval pathway.
  - To verify that the medication slows the loss of kidney function, patients who enrolled in the trial must remain in the trial in their assigned treatment arm for 1 to 2 more years.
1. What factors would motivate you to participate in an accelerated approval clinical trial?
  2. If a medication is approved for reducing proteinuria, and has a 50% chance of slowing disease progression or improving how patients feel, how would that impact your decision to use that drug?

**Topic 4: Current Challenges to Treating FSGS**

1. What are you currently doing to help treat your condition or its symptoms?
    - a. How has your treatment regimen changed over time, and why?
  2. How well does your current treatment regimen treat the most significant symptoms of your disease?
    - a. How well do your treatments address specific symptoms?
    - b. Which symptoms are not addressed as well?
  3. What are the most significant downsides to your current treatments and how do they affect your daily life?
  4. Assuming there is no complete cure for your condition, what specific things would you look for in an ideal treatment for your condition?
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