



# SPEAKER BIOS





## WENDY WHITE

Founder and CEO, Siren Interactive

Wendy founded Siren Interactive in 1999. For more than 14 years across more than 30 disease states, Siren Interactive has had one focus: finding rare disease patients and connecting them to appropriate therapies. Wendy approaches the business from her unique perspective of being an entrepreneur and digital marketing expert, as well as being the mother of a daughter with a rare disorder that was diagnosed as a direct result of Wendy becoming an empowered caregiver.

Wendy's personal journey with her daughter, which began in 2001, was the impetus for Siren to focus solely on marketing rare disorder therapies. In 2011, Wendy led the collaboration with 12 fellow rare disease caregivers to co-author *Uncommon Challenges; Shared Journeys: Stories of Love, Hope, and Community by Rare Disease Caregivers*. In 2012 she was recognized as one of the 100 most inspiring people in pharma.

THURSDAY,  
OPENING REMARKS

@sirenwendy

## JANE MEIER HAMILTON, RN, MSN

Founder, CEO, Partners on the Path

A nurse for 40 years and family caregiver for 20, Jane Meier Hamilton is the Founder of Partners on the Path ([www.PartnersonthePath.com](http://www.PartnersonthePath.com)), which provides caregiver support programs to businesses and non-profits, healthcare and faith-based organizations. Jane is a content expert on managing caregiver stress, building resilience, and preventing compassion fatigue.

She has created original, research-based resources that help professional and family caregivers remain healthy, effective, and compassionate. Her book, *The Caregiver's Guide to Self-Care* is available in print, 1 hour audio, and e-book formats at your favorite online book distributor.

THURSDAY, SESSION 1

@PartnersonPath



## JOHN SCHALL

CEO, Caregiver Action Network

John Schall is a public policy and communications professional with noted expertise in a wide range of policy fields, including healthcare, labor, education, economic development, taxation, and budget policy. He became Chief Executive Officer of the Caregiver Action Network [formerly the National Family Caregivers Association (NFCA)] in June 2012.

Prior to CAN, Mr. Schall was Deputy CEO of the Parkinson's Action Network. John also brings to CAN a wealth of experience in senior federal government positions, including serving as former Sen. Bob Dole's Chief Budget Advisor; Chief of Staff of the U.S. Department of Labor in the early 1990s; and as deputy of the White House Domestic Policy Council under President George H.W. Bush.

He holds a Master of Public Policy degree from Harvard University's John F. Kennedy School of Government, and he is a graduate of the University of Michigan.

THURSDAY, SESSION 1

@CaregiverAction

## ROBERT M. MILLER

Human Service Consultant,  
Robert Miller Human Service Consulting

For the past four decades, Robert has been working with families who have a child or children with special needs, along with the professionals who work with those children and families. He is particularly interested in how families learn about, access, and receive meaningful information and services from competent professionals, organizations, and institutions.

From 1999 through 2013, he served as Executive Director of the National Fragile X Foundation, where he played a leading role in the formation of a nationwide network of evaluation, treatment, and research clinics. He has also made multiple presentations and assisted with the development of parent support organizations throughout the world.

THURSDAY, SESSION 1  
DEEP DIVE





## LISA RAMAN

Director of the National Resource Center,  
Spina Bifida Association

A registered nurse with extensive experience in nonprofit health-care and education for vulnerable groups, Lisa worked as a National Medical Adviser in the UK at the Association for Spina Bifida and Hydrocephalus (ASBAH). Lisa is also the co-founder and executive director of the Klippel Feil Syndrome Alliance, a nonprofit patient organization whose mission is to help people with KFS, one of several rare conditions that her daughter lives with.

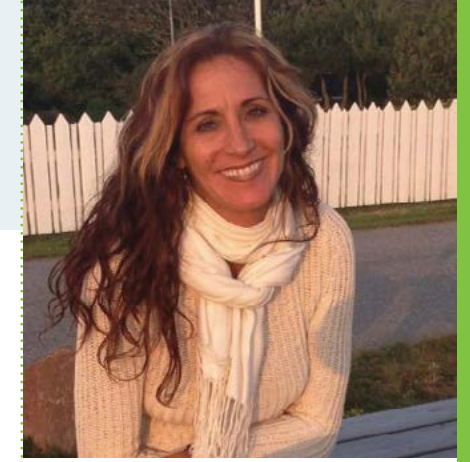
Lisa's work gives her the unique opportunity to help people across all ages and geographic locations in the United States and abroad. She feels very honored also to be a parent advocate for Global Genes and co-chair of the HCP Outreach Committee.

## CYNTHIA J. FRANK

Director of Patient Advocacy and Meetings,  
National Gaucher Foundation

In addition to being a strong patient advocate with type 1 Gaucher disease, Cynthia is a long-standing member of the Gaucher community, participating in many clinical trials to help get treatments to market. She is an NGF Mentor and advocate for Gaucher patients and raising awareness through speaking at conferences, meetings and events, including NGF and other rare and genetic disease organization meetings, LSD symposiums, and industry and pharmaceutical conferences and educational events.

She serves on multiple boards and committees for many organizations, including the Association of Fundraising Professionals Wine Country Chapter, Global Genes Advocacy Leaders Group and Corporate Alliance Patient Education Committee, and the NGF's Gaucher Advisory Group.



THURSDAY, SESSION 1  
DEEP DIVE

@KFSalliance

@NGF\_Gaucher

THURSDAY, SESSION 1  
DEEP DIVE



## C. GRACE WHITING, JD

Director of Communications and Coalitions,  
National Alliance of Caregiving

Grace is a strategic communications professional and an attorney member of the District of Columbia Bar. She is passionate about supporting family caregivers who are navigating the long-term care system for their loved ones and who are managing their own financial, health, and wellness needs. Grace initially served as the National Alliance for Caregiving's Manager of Programs and Communications, before being promoted to Director of Communications and Coalitions in March 2014. Prior to joining the Alliance, Grace served as the Director of Strategic Initiatives & Communications and Special Assistant to the Executive Director at the Alliance for Home Health Quality and Innovation in Washington, D.C. Grace has also worked as a Communications Manager for the Louisiana Family Recovery Corps in the aftermath of Hurricanes Katrina and Rita. Named an "Outstanding Law Student" in 2011 by the National Association of Women Lawyers, Grace earned her law degree from the University of Memphis Cecil C. Humphreys School of Law and her undergraduate degree from Louisiana State University in Communication Studies.

## TANYA JOHNSON

Special Education Resource Teacher,  
Halton Catholic District School Board

Tanya Johnson is a passionate Special Education Resource Teacher who has been teaching for 15 years. She currently works with students of various abilities and rare disorders. Tanya has a Specialist in Special Education and is the co-founder of FPWR Canada along with her husband Keegan.

She is a proud mother to Denzel (8yrs) and Dante (9yrs). Since receiving Dante's diagnosis of Prader-Willi Syndrome (PWS), Tanya has made it her mission to unite and empower families to eliminate the challenges of Prader-Willi Syndrome and to help families of all rare disorders to advocate for their children in the school system. She is currently launching a new project called "IEP Champion" to help children maximize their learning potential. You can connect with Tanya on facebook by joining her group, "Educational Advocacy: Maximizing Learning Potential."



THURSDAY, SESSION 1  
DEEP DIVE

@NA4Caregiving

THURSDAY, SESSION 1  
DEEP DIVE



## INDU SUBAIYA

CEO and Co-Chairman, Health 2.0

Indu Subaiya, MD, MBA is Co-Chairman and CEO of Health 2.0, where she drives strategic technology innovation and co-curates a worldwide series of conferences, code-a-thons, prize challenges, and more. Prior to co-founding Health 2.0, she was Entrepreneur-in-Residence at Physic Ventures, a consumer health and wellness investment fund.

She has also served as President of Etude Scientific, a consulting firm in the biotechnology and consumer healthcare space, Vice President of Healthcare and Biomedical Research at Gerson Lehrman Group, and Director of Outcomes Research at Quorum Consulting. Indu received her MD from Stony Brook University School of Medicine at the State University of New York, an MBA from the Haas School of Business at the University of California, Berkeley, and a BS in Science and Technology Studies from Cornell University.

THURSDAY, SESSION 2

@health2con

## GILLES FRYDMAN

Advocate and Co-Founder, Smart Patients

Gilles Frydman is the founder of the Association of Cancer Online Resources (ACOR), the largest online social network for cancer patients, which is composed of nearly 200 communities for individuals with cancer. ACOR has served over 1 million cancer patients and their caregivers to optimize patient care, for which Frydman was named by CNN in 2008 as one of six Empowered Patient's Health Heroes. In 2012, he co-founded Smart Patients to bring the newest in collaboration technology to networks of cancer patients and caregivers. Gilles blogs regularly at epatients.net and on his own blog, patientdriven.org. He is a frequent speaker to healthcare audiences in the US and France, and has appeared in many publications ranging from the British Medical Journal to the Wall Street Journal. He serves on a number of advocacy and advisory committees in support of patient-centered computing, consults for Internet-based corporations and start-up initiatives, and has received funding from the Robert Wood Johnson Foundation to study and disseminate information about online patient resources.

@smart\_patients



THURSDAY, SESSION 2



## RONI ZEIGER, MD

Co-Founder, Smart Patients

Roni Zeiger is the former Chief Health Strategist at Google, where he led efforts ranging from Google Flu Trends to Symptom Search. In 2012, he co-founded Smart Patients in order to amplify the knowledge created by networks of engaged patients. Roni serves as CEO of Smart Patients and continues to see urgent care patients part time at Santa Clara Valley Medical Center, where he is a Community Staff Physician.

Roni earned his M.D. at Stanford University and completed his internal medicine residency at the University of California, San Francisco. He has served as a clinical instructor of medicine at Stanford University School of Medicine and received a master's degree in biomedical informatics from Stanford.

THURSDAY, SESSION 2

@smart\_patients

## JULIA HALLISY, DDS

Advocate, Founder, and President, The Empowered Patient Coalition

Dr. Julia Hallisy is a practicing dentist in San Francisco, California. In 1989, Dr. Hallisy's late daughter, Katherine, was diagnosed at five months of age with bilateral retinoblastoma. Dealing with Kate's life-threatening diagnosis marked the beginning of a 25-year involvement in our healthcare system.

The combination of her scientific training, her work as a healthcare provider, and guiding a child through a chronic illness has afforded her invaluable insight as an advocate for patients. Dr. Hallisy founded The Empowered Patient Coalition nonprofit organization and has worked with the IOM Initiative on the Future of Nursing, NQF, AHRQ, and the Partnership for Patients.

@EPCoalition



THURSDAY, SESSION 2



## RONNIE SHARPE

Founder and Chief Community Servant, CysticLife

Ronnie Sharpe is the founder and Chief Community Servant at CysticLife, an organization that works to positively impact the lives of the cystic fibrosis (CF) community via educational materials, grants, and its leading social network, CysticLife.org, where over 7500 members share tips, questions, ideas, experiences and most of all encouragement.

As a CF patient and leader, he is a true believer in the power of social media and patient driven research and speaks nationally at health-related conferences about the impact of technology on rare diseases.

THURSDAY, SESSION 2  
DEEP DIVE

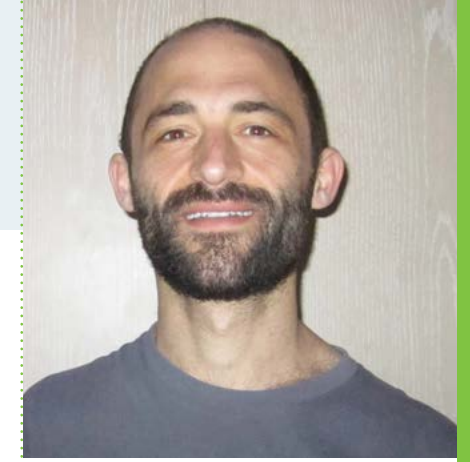


## JOHN STAMLER

Executive Director, Ben's Friends

John Stamler is the Executive Director of Ben's Friends, a 501(c)3 nonprofit, where he is focused on sponsorships, member services, fundraising, partnerships with corporations, hospitals, physicians, and the financial side. John got involved with Ben's Friends in June 2010, a month after a successful open heart surgery to fix one of his two rare diseases, Atrial Septal Defect.

In November 2010, John ran the New York City Marathon less than six months after open heart surgery to raise awareness for folks with rare diseases and money for Ben's Friends. He's a graduate of Brown University and lives in London. As of July 2014, Ben's Friends had over 37 rare disease support communities with more than 40,000 members. The organization's mission is to ensure everyone in the world with a rare disease has a safe place to go and connect with others like them.



THURSDAY, SESSION 2  
DEEP DIVE



## MANU KODIYAN

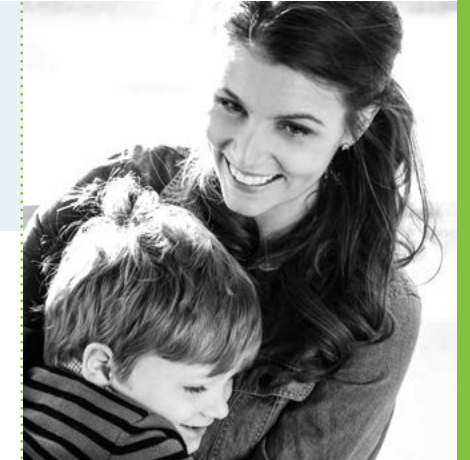
Founder, Althea Health

Althea Health, a health technology company with a novel platform to conduct, crowdfund and manage health research for rare and neglected diseases, was borne from Manu's decade long struggle with a complex health issue and his belief that the confluence of new technologies and legislation will create a major opportunity for rare disease patients to engage and have a voice in health research and driving better clinical outcomes.

## COURTNEY LARNED

VP of Marketing, CareSync

Although she lends a hand in sales, design, and overall corporate strategy, Courtney's passion is sharing the CareSync story, whether it's at events, writing articles, designing graphics, interviewing users, or positioning the company's thought leadership in healthcare. An advocate for patients and their families, Courtney frequently speaks at events regarding the importance of having access to understandable health information.



THURSDAY, SESSION 2  
DEEP DIVE

THURSDAY, SESSION 2  
DEEP DIVE



## MARK KRUEGER, MPH

President, Mark Krueger & Associates, Inc.

Mark Krueger has 25 years of public health and advocacy experience in building equitable and responsible partnerships between patient consumer and professional groups; industry; and government. Working with colleagues in offices in New York and London, Mark has helped organizations like Parent Project Muscular Dystrophy shape effective scientific, clinical, and regulatory policies and practices.

The firm also supports disease awareness campaigns, clinical trial awareness and enrollment efforts, and more. Mark and his colleagues draw on their experience as former chief executives and senior staff of voluntary health organizations to create respectful, compliant collaborations that lead to better lives for patients and their caregivers. Mark holds a BA from the College of Columbia University and an MPH from Columbia's Joseph A. Mailman Graduate School of Public Health.

THURSDAY, SESSION 3

## KIMBERLY MCCLEARY

Director of Strategic Initiatives, FasterCures

FasterCures is a center of the Milken Institute determined to remove barriers to medical progress. Kim leads key programmatic areas, including expanding patient engagement in FDA's assessment of benefits and risks for medical products, the valuation of medical innovations by various stakeholders, and coverage for medical products that affects reimbursement and access. She also works closely with FasterCures' network of patient-focused venture philanthropy organizations, The Research Acceleration and Innovation Network (TRAIN).

Prior to joining FasterCures' staff, Kim was President & CEO of the CFIDS Association of America from 1991 until June 2013. There, Kim earned a reputation as an articulate patient advocate, a keen policy strategist, a diplomatic bridge builder, and a dedicated servant leader.

@fastercures



FRIDAY, SESSION 4



## PAT FURLONG

Founding President and CEO,  
Parent Project Muscular Dystrophy (PPMD)

PPMD is the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy (Duchenne). When doctors diagnosed Pat's two sons, Christopher and Patrick with Duchenne in 1984, she refused to accept "there's no hope, and little help" as an answer. Instead, she immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment, and the mechanisms for optimal care.

Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne. In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure. Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world.

@ParentProjectMD

## ROY ZEIGHAMI

Founder and Board President, Sanfilippo Foundation for Children

Roy Zeighami is the father of a child with MPS III Sanfilippo Syndrome and founder of the Sanfilippo Foundation for Children. Roy has been served as a strong advocate on Capitol Hill and with the FDA for accelerated approval of treatments for rare disease.

He has long advanced his opinion that parents and patients suffering from catastrophic illnesses with no treatment have a fundamental right to accept higher risk as a trade-off for a faster route to the clinic.

@zeighami



FRIDAY, SESSION 4



## ERIC GASCHO

Assistant VP of Government Affairs, National Health Council

Eric Gascho is the Assistant Vice President of Government Affairs at the National Health Council. The NHC, a Washington-based non-profit, is the only organization that brings together all segments of the health care community to provide a united voice for the more than 133 million people with chronic diseases and disabilities and their family caregivers. Its core membership includes the nation's leading patient advocacy groups. Other members include professional and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, and biotechnology companies. In his role with the NHC, Eric helps develop policies that benefit people with chronic conditions and advocates for these policies on Capitol Hill and within the administration. Prior to joining the NHC in 2009, Eric worked in the government relations department of the Society of Teachers of Family Medicine. He earned BA degrees in Government and Business Management from the University of Redlands in California.

FRIDAY, SESSION 4  
DEEP DIVE



## KEEGAN JOHNSON

Founder and CEO, Zenzaga

As both an entrepreneur and non-profit executive, Keegan Johnson has seen the power of collective action up close and has been obsessed with the questions of what sets teams on fire and how powerful massive action can be.

Keegan is embarking on a new challenge as the founder and CEO of Zenzaga ([www.zenzaga.com](http://www.zenzaga.com)), a company focussed on bringing the power of social movements to companies, communities, and causes around the world. Whether through speaking or consulting, Zenzaga can help you finally solve the question, how do you get your community to take action now.

At the same time, Keegan found out his first born son had a rare genetic disorder, called Prader-Willi Syndrome (PWS). He co-founded a Canadian charity ([www.fpwr.ca](http://www.fpwr.ca)) and became the Executive Director of the affiliated US charity ([www.fpwr.org](http://www.fpwr.org)) with a mission to eliminate the challenges of PWS through research.

@keeganj

FRIDAY, SESSION 5

## TIM J. O'CONNOR

Partner, O'Connor, Schmeltzer & O'Connor

Timothy O'Connor obtained a Juris Doctor Degree from Loyola University School of Law in 1990. He is admitted to practice law before all of the courts of the State of California and is a partner in the Orange County law firm of O'Connor, Schmeltzer & O'Connor, which was founded in 1984. Tim provides a full range of legal services including nonprofit consultation and formation, estate planning, probate/trust administration, and civil litigation.

FRIDAY, SESSION 5  
DEEP DIVE



## LISA SCHILL

Vice President, RASopathies Network USA

Lisa Schill is a parent advocate, dedicated to improving the lives of those living with rare disease. Lisa specializes in connecting caregivers, researchers, support organizations, and families to help support patients in the pursuit of advancing treatment options and patient outcomes. She currently serves as Vice President of the RASopathies Network USA, a nonprofit dedicated to improving outcomes for families living with a RASopathy.



FRIDAY, SESSION 5  
DEEP DIVE





## DEBRA MILLER

President and CEO, CureDuchenne

Debra Miller co-founded CureDuchenne in 2003 with her husband, Paul, after their only son was diagnosed with Duchenne muscular dystrophy. Miller has led CureDuchenne in its mission to raise awareness and fund research to find a cure for Duchenne.

Miller has created a successful venture philanthropy model that helps to accelerate drug development for the treatment of Duchenne. Under her leadership, CureDuchenne has funded seven research projects that have advanced to human clinical trials.

## JEAN F. CAMPBELL

Principal, JF Campbell Consultants, LLC

Jean Campbell is nationally recognized as one of the leading rare disease patient advocates with an impressive record of success in fundraising and corporate relations. After serving nearly two decades with the National Organization for Rare Disorders (NORD) as Vice President of Development, Jean created her consulting firm, JF Campbell Consultants LLC, in 2010. A well-respected and extremely connected resource in the rare disease health community, Jean created her consulting firm, JF Campbell Consultants LLC, in 2010. Her expertise in patient advocacy, non-profit strategic planning, organizational governance, corporate relations, patient assistance programs and program management is extremely valuable to the non-profit health community, pharmaceutical corporations, and biotech industries. Jean also provides valuable mentorship to start-up and growing patient organizations helping to strengthen their infrastructure in order to better serve their mission, members and communities.



FRIDAY, SESSION 5  
DEEP DIVE

@CureDuchenne

FRIDAY, SESSION 5  
DEEP DIVE



## DAVID M. LAGRECA

Executive Director, VCG Governance Matters

David LaGreca is the Executive Director of VCG Governance Matters. He works with clients on issues of strategy, board recruitment and leadership. David has extensive experience assisting corporate and nonprofit executives with strategy, performance coaching and refining business operations. He is one of the founders and former Board Chair of JOBSFIRSTNYC, an intermediary organization focused on out-of-school, out-of-work young adults. A graduate of the Katholieke Universiteit te Leuven, Belgium, and a former priest, Mr. LaGreca received his MBA in Management and Marketing from the Columbia University School of Business. David has contributed chapters in the following books: *Secrets of Successful Boards: The Best from the Nonprofit Pros* (Chairing a Board); *Secrets of Successful Fund Raising* (Fundraising in the Corporate Community); and *Secrets of Successful Retreats* (Implementation Issues Surrounding Strategic Planning).

## LESLIE PEROVICH, CFRE

Senior Director of Advancement, Pretend City Children's Museum

Leslie Perovich is the Senior Director of Advancement for Pretend City Children's Museum in Irvine, California. She has over 25 years in the nonprofit arena with extensive experience in marketing, grant writing, membership, special events, and individual giving.

Leslie has consulted with and worked for a variety of non-profit organizations throughout her career including Friends of Santa Ana Zoo, Discovery Science Center, Planned Parenthood and Pretend City Children's Museum.



FRIDAY, SESSION 5  
DEEP DIVE

@pretendcity

FRIDAY, SESSION 5  
DEEP DIVE





## STEPHANIE FISCHER

Senior Director of Communications,  
Pharmaceutical Research and Manufacturers of America

Stephanie Fischer is a rare disease patient and stroke survivor based in Washington, DC. She is a senior director of communications at the Pharmaceutical Research and Manufacturers of America (PhRMA).

## DEANA BOWDEN, CPA

Partner, White Nelson Diehl Evans LLP

Deana Bowden has nearly 20 years of public accounting experience, more than 10 of which have been with White Nelson Diehl Evans LLP. Deana's area of expertise includes the performance of audits, reviews, and compilations. The industries she specializes in are manufacturing and distribution, nonprofits, construction, educational institutions, and professional services.

Deana also provides business consulting services to various entity types as well as perform employee benefit plan audits.



FRIDAY, SESSION 5  
DEEP DIVE

@SDFatPHRMA

FRIDAY, SESSION 5  
DEEP DIVE



## SUSAN HOOVER MILLER, CFRE

Fundraising Consultant, Susan Hoover & Associates

Susan Hoover Miller is a fundraising consultant with more than 26 years of professional experience in Orange County. She has managed all aspects of development programs including strategic planning, major gifts and capital campaigns, annual fundraising, grant writing, events, marketing and budgeting.

Susan works closely with Board members and other community leaders, engaging them in the fund development process through leadership training and mentorship.

## CANDACE HUIE

Partner, White Nelson Diehl Evans LLP

Candace Huie has over 20 years of public accounting experience, all with White Nelson Diehl Evans LLP. Candace's area of expertise is in tax planning and compliance, with a focus on real estate partnerships, small businesses, and nonprofits. She serves large, middle-market companies located in Orange County and the Inland Empire.



FRIDAY, SESSION 5  
DEEP DIVE

@CFRE\_INTL

FRIDAY, SESSION 5  
DEEP DIVE



## MAYA DOYLE, PhD

Senior Pediatric Social Worker, Children's Hospital at Montefiore

Dr. Maya Doyle is an Assistant Professor of Social Work at Quinnipiac University in Connecticut. In 2013, she completed her doctoral work, a qualitative study of the experience of emerging adults and their families living with cystinosis, a rare metabolic disorder.

She is currently undertaking research regarding the transition of pediatric patients to adult-oriented care. Since 1999, she has been the senior social worker in Pediatric Nephrology at the Children's Hospital at Montefiore in New York, and continues to coordinate the Ruth Gottscho Dialysis and Children's Kidney program at Frost Valley YMCA, a mainstream summer camp program for children with kidney disease.

## JAMES ROMANO

Director of Government Relations and Advocacy, Patient Services Incorporated (PSI)

For close to 20 years, James has advocated on behalf of the rare diseases community beginning with his advocacy for the passage of the Ricky Ray Hemophilia Relief Fund Act. James works to remove barriers to access to care for patients with rare diseases.

James spent 4 years as the Health Care Policy Advisor to Congresswoman Cynthia McKinney (D-GA). After his service on Capitol Hill, he served as a contract lobbyist with the Health and Medicine Counsel of Washington where he represented rare disease communities.



FRIDAY, SESSION 6

@UneedPSI

FRIDAY, SESSION 7



## JULIA JENKINS

Director at Rare Disease Legislative Advocates (RDLA) & Director of Public and Government Relations at EveryLife Foundation for Rare Diseases

Julia Jenkins' background in grassroots organizing, political strategy, and legislative advocacy helped unite more than 180 patient organizations under the CureTheProcess Campaign. She worked to ensure ULTRA/FAST was included in the 2012 FDA Safety and Innovation Act (FDASIA).

Julia initiated RDLA to serve as a communication platform for rare disease stakeholders to work on legislative issues and to help elevate the voice of rare disease patients on Capitol Hill.

## JENNIFER BERNSTEIN

Executive Vice President, Horizon Government Affairs

Jennifer Bernstein joined Horizon Government Affairs as Vice President, focusing on legislative and regulatory developments within the pharmaceutical, biotechnology, and medical device sectors.

Prior to joining Horizon, Jennifer was Vice President of Healthcare Research at a healthcare advisory and financial services firm and was responsible for anticipating and translating legislative and regulatory catalysts within all sectors of the healthcare marketplace.



FRIDAY, SESSION 7

@RareAdvocates

FRIDAY, SESSION 7 & 8



## BARBARA H. WUEBBELS, RN, MS

Vice President of Patient Advocacy and Medical Affairs,  
Audentes Therapeutics

Barbara has been involved in patient advocacy activities for patients with rare diseases for the past 15 years at Ucyclid Pharma and BioMarin. Recently, she joined Audentes Therapeutics as Vice President of Patient Advocacy and Medical Affairs. She has been actively involved with the National Organization of Rare Diseases, International Conference of Rare Disorders, and Global Genes.

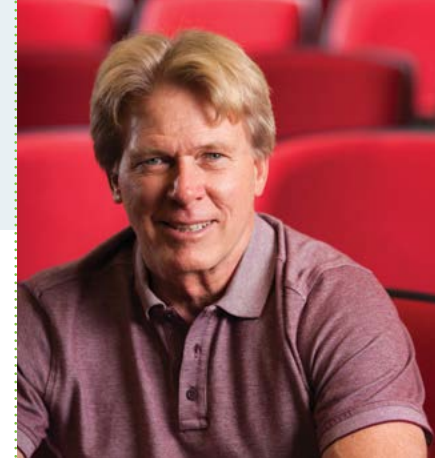
Currently she serves on the Boards of the International Conference on Rare Disorders and the Batten's Disease Support and Research Association. She holds Bachelor's and Master's degrees in nursing.

## HUDSON FREEZE, PhD

Professor and Director, Human Genetics Program at Sanford Children's Health Research Center, Sanford-Burnham Medical Research Institute

Dr. Freeze has worked on rare diseases for over 35 years, specializing in the discovery of Congenital Disorders of Glycosylation (CDG) and their treatments. He is Professor of Glycobiology and the Director of the Human Genetics Program at Sanford-Burnham Medical Research Institute.

His current interests are in defining new types of CDG and in NGLY1, the first Congenital Disorder of Deglycosylation. He is the Vice President for Science Policy at FASEB, a 120,000 member alliance of biomedical researchers.



FRIDAY, SESSION 9

@AudentesTx

FRIDAY, SESSION 9



## STEVE C. GROFT, PharmD

Senior Advisor to the Director, NCATS at the National Institutes of Health (NIH)

His current major emphasis at NCATS/NIH is on patient and community engagement in rare diseases research and medical devices as orphan products. Steve served as Director of the Office of Rare Diseases Research (ORDR) at the NIH prior to his retirement in 2014 after 44 years in government service.

His major focus for the previous 32 years was on stimulating research with rare diseases and developing information about rare diseases and conditions for researchers, biopharmaceutical industry, healthcare providers, and the public.

## MATT WILSEY

President, Grace Wilsey Foundation

Matt Wilsey is a Silicon Valley entrepreneur, investor, and advisor. Beyond consumer products and services, Matt advocates for and invests in biomedical research, drug development, and genetic sequencing technologies.

After starting his career at the White House and the Department of Defense, Matt spent numerous years as a frontline operator at Zazzle, KKR, Howcast Media, and CardSpring before becoming a "rare disease hunter" and moving to the investment side. Matt holds a BA from Stanford University and a MBA from Stanford's Graduate School of Business.



FRIDAY, SESSION 9

@NIH

@gracewilseyorg

FRIDAY, SESSION 9



## JONATHAN JACOBY

Founder, Hide & Seek Foundation

Jonathan Jacoby chairs the Hide & Seek Foundation for Lysosomal Disease Research. He is a principal organizer of SOAR-NPC (Support Of Accelerated Research for Niemann-Pick Type C Disease), a collaborative research & development model that he helped develop with a group of scientists and parents. Designed to help bridge the gap between basic science and patient treatments, the SOAR-NPC project aims to develop a “virtual cocktail” that will delay or lessen the severity of symptoms for people affected by Niemann-Pick Type C, a neurodegenerative disease that is generally fatal in children. Jonathan also has worked with numerous not-for-profit organizations to launch new ventures and programs, develop strategic plans, and guide agencies through periods of transition.

Jonathan holds a Master of Education degree from Harvard University, a Bachelor of Arts from UCLA, and a Bachelors of Literature from the University of Judaism.

Jonathan has two sons and lives in Los Angeles.

## KATHERINE A. RAUEN, MD, PhD

Professor, Department of Pediatrics; Chief, Division of Genomic Medicine; Albert Holmes Rowe Endowed Chair in Human Genetics II; Director, NF/Ras Pathway Clinic; UC Davis MIND Institute



Katherine (Kate) Rauen, MD, PhD is a Professor in the Department of Pediatrics, Division of Genomic Medicine at the UC Davis where she currently serves as the Chief of Genomic Medicine. She received a MS in Human Physiology and a PhD in Genetics from UC Davis doing research on gene dosage compensation and genetic evolution. She obtained her MD at UC Irvine where she also did research in cancer genetics. Dr. Rauen did her residency training in Pediatrics and fellowship in Medical Genetics at UC San Francisco.

Dr. Rauen is internationally known for her pioneering work in the application of array CGH in clinical genetics and as a leader and major contributor to the understanding of the “RASopathies”, the Ras/MAPK pathway genetics syndromes. Her research program involves the clinical and basic science study of cancer syndromes with effort to identify underlying genetic abnormalities affecting common developmental and cancer pathways. Dr. Rauen led the research team, including the CFC International Family Support Group that discovered the genetic cause of cardio-facio-cutaneous syndrome.

Dr. Rauen is committed to academic medicine, medical education, and advancing best practices for patients with RASopathies. She has successfully obtained both intramural and extramural funding for her research activities, and currently holds a 5-year NIH grant studying skeletal myogenesis in Costello syndrome and CFC. She is the innovator of the world-renowned NF/Ras Pathway Clinic which she initiated in 2007 and this clinic has now been emulated around the globe. She serves on the medical advisory board of CFC International and is a Co-Director for the Costello Syndrome Family Network.

Dr. Rauen was recently awarded the Presidential Early Career Award for Scientists and Engineers (PECASE), the highest honor bestowed by the United States Government on science and engineering professionals in the early stages of their independent research careers.

