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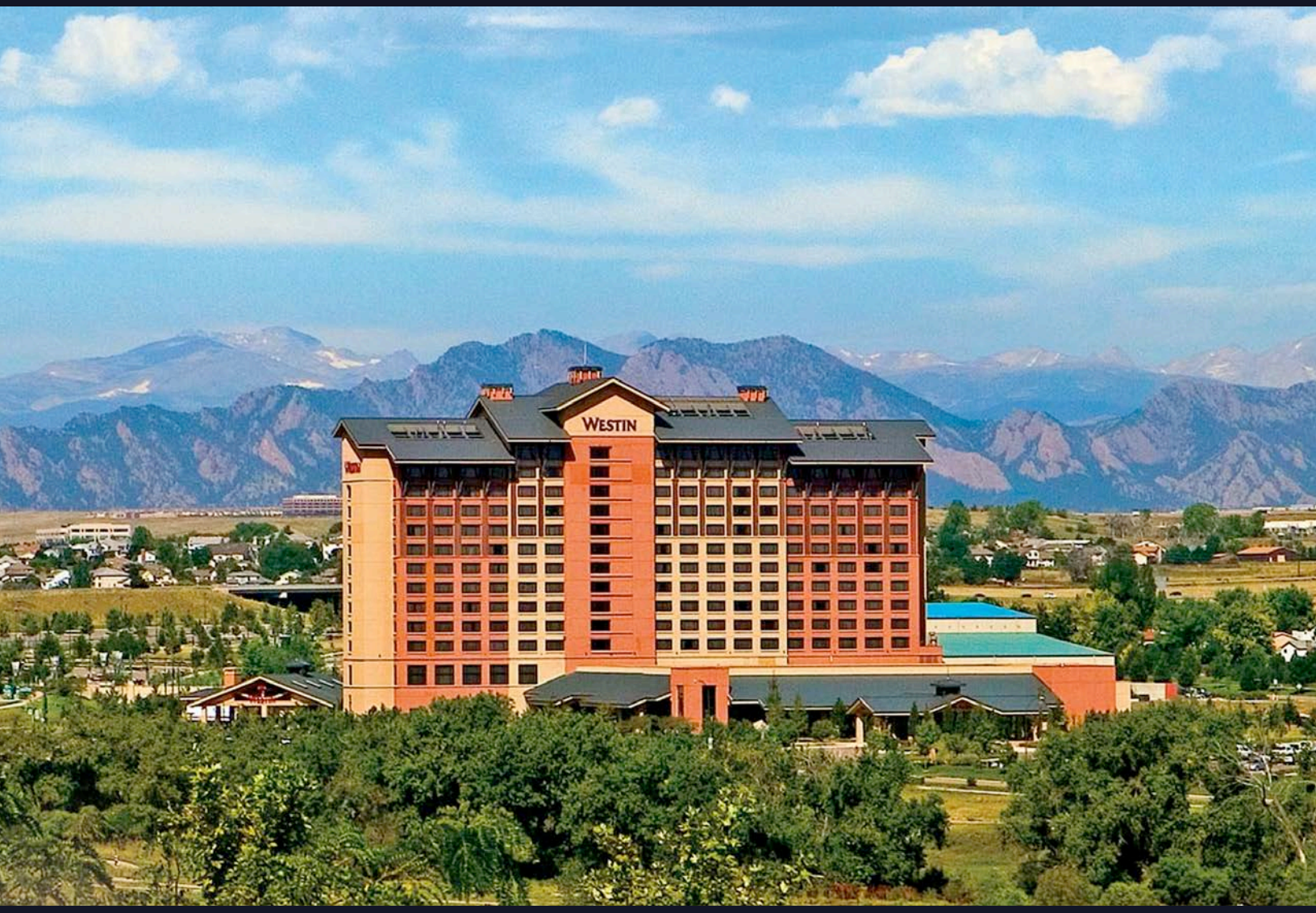

JULY 8 – 9 2016
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FRIDAY AGENDA

FATTY OXIDATION DISORDERS GROUP

ORGANIC ACIDEMIA ASSOCIATION GROUP

7:00 - 8:00 am Continental Breakfast for Conference Registrants - Westminster 3 & 4, food in hallway

8:00- 8:30 am Introductions by Deb & Kathy - Westminster 1 (and then OAA moves to Westminster 2)
Keynote (Janet Thomas, MD, Children's Hospital Colorado) - [Kids room open-Cotton Creek]

TIME & LOCATION	SPEAKER
8:30 - 9:30 am Westminster 1	**Kathryn Chatfield, MD, PhD** University of Colorado School of Medicine **TOPIC:** Cardiac 101 and FODs
9:30 - 10:30 am Westminster 1	**Johan Van Hove, MD, PhD** University of Colorado School of Medicine **TOPIC:** Anabolism: What happens when fasting?

TIME & LOCATION	SPEAKER
8:30 - 9:00 am Westminster 2	**Kim Chapman, MD, PhD, FACMG** Children's National Medical Center **Brian Wamhoff, Ph.D,** Head of Innovation at HemoShear Therapeutics **TOPIC:** OAA/NORD Natural History Registry
9:00 - 10:15 am Westminster 2	**Chuck Venditti, MD, PhD** NHGRI/NIH **TOPIC:** MMA Update **Emily McCourt, MD** University of Colorado School of Medicine **TOPIC:** Ocular disease in Cobalamin C

10:30 am Meadowbrook **TOPIC:** Low Protein Cooking Demo for Teens/Young Adults **– Laurie Bernstein**

10:30 - 10:45 am	Break
10:45 - 11:30 am Westminster 1	**Mark Korson, MD** Genetic Metabolic Center for Education **TOPIC:** FAOD Management Strategies at Home and in the Hospital: Keeping your child safe
11:30 - 12:00 pm Westminster 1	**Laurie Bernstein, MS,RD,FADA,FAND** Children's Hospital Colorado **TOPIC:** Facts about Fats (Benefits of Micronutrients and Essential Fats)

10:15 - 10:30 am	Break
10:30 - 11:15 am Westminster 2	**Jerry Vockley, MD, PhD** Children's Hospital of Pittsburgh **TOPIC:** Novel therapies for Organic Acidemias
11:15- 12:00 pm Westminster 2	**Nicola Longo, MD, PhD** University of Utah School of Medicine **TOPIC:** Liver Transplant in MMA-PA

12:00 - 1:00 pm LUNCH BUFFET for Conference Registrants - Westminster 3 & 4, Foyer

1:15 - 2:15 pm Westminster 1	**Jerry Vockley, MD, PhD** Children's Hospital of Pittsburgh **TOPIC:** New treatments for FAODs (MCAD, long chain disorders and GA2
2:00 pm Meadowbrook	**TOPIC:** DNA Talk with Teens/Young Adults **– Cindy Freehauf and/or Shannon Scrivner**

1:15 - 2:00 pm Westminster 2	**Kathryn Chatfield, MD, PhD** University of Colorado School of Medicine **TOPIC:** Cardiac 101 and OAs

2:15 - 3:15 pm Westminster 1	**Nicola Longo, MD, PhD** University of Utah School of Medicine **TOPIC:** Clinical Aspects of Carnitine transporter deficiency as one ages (and similarities to other FODs)	2:00 - 3:00 pm Westminster 2	**Irini Manoli, M.D., Ph.D.** NHGRI/NIH **TOPIC:** Update on MMA/PA Diets **Jennifer Sloan PhD**, **MS** NHGRI/NIH **TOPIC:** cblC Mice and Gene Therapy cblC
3:00 pm Meadowbrook	**TOPIC:** DNA Talk with children under age 10 – Genes, not Jeans! (for ages 4-10) **Cindy Freehauf and or Shannon Scrivner** (max 42)		
3:15 - 3:30 pm	Break	3:00 - 3:15 pm	Break
3:30 - 4:15 pm Westminster 1	**Stephen Kahler, MD** Arkansas Children's Hospital **TOPIC:** Autism and mitochondrial disorders	3:15 - 4:15 pm Westminster 2	**Jan Kraus, MD PhD** University of Colorado School of Medicine **TOPIC:** Enzyme replacement therapy for propionic acidemia-early attempts **Oleg Shchelochkov, MD** NHGRI/NIH **TOPIC:** PA Protocol
4:15 - 5:00 pm Westminster 1	**Professional Panel:** Q&A with several of our speakers	4:15 - 5:00 pm Westminster 2	**Stephen Goodman, MD**, University of Colorado School of Medicine **TOPIC:** GA1
5:00 pm	Summary and THANK YOU's!		
6 - 9 pm	RECEPTION and Cash Bar for Conference Registrants - South Court (weather backup – Westminster 3 & 4)		

SATURDAY AGENDA

FATTY OXIDATION DISORDERS AND ORGANIC ACIDEMIA ASSOCIATION JOINT SESSION

Wear your FOD and OAA t-shirts

7:00 - 8:00 am	Continental Breakfast for Conference Registrants – Westminster 3 & 4, food in hallway
8:00 - 8:15 am	Introductions by Kathy & Deb in each room | **Remember PICTURES at 9:15am!!**
8:15 - 9:15 am Westminster 1	**FOD Breakout sessions/networking** by Disorder
8:15- 9:15 am Westminster 2	**OA Breakout sessions/networking** by Disorder and Young Adult Panel
9:15 - 9:45 am Open Room Divider	Break - **PICTURE OF EACH Individual GROUP WITH YOUR T-SHIRTS!** Meet by the FOD and OAA wall Banners
9:45 - 10:45 am Westminster 1 & 2	JOINT Presentation – **Mark Korson , MD** **TOPIC:** Genetic Metabolic Center for Education
10:45 - 11:45 am Westminster 1 & 2	JOINT Panel **TOPIC:** Open discussion with many of our speakers
11:45 - 12 noon	**Ending Ceremony**, Thank you's and slide show

See You in 2018!

CONFERENCE **SPEAKERS**

The **FOD Family Support Group** and the **Organic Acidemia Association** would like to extend a very sincere appreciation to all of our honored Speakers. Our Families will benefit greatly from having them share their expertise and experiences in the various fields related to metabolic disorders. **Thank you!**

+ Laurie **BERNSTEIN**, MS, RD, FADA, FAND

Laurie E. Bernstein, MS, RD, FADA ,FAND , is an Associate Professor, Department of Pediatrics, and Director of the IMD Nutrition Clinic, Section of Clinical Genetics and Metabolism at the University of Colorado School of Medicine, Denver; and Children's Hospital Colorado in Aurora, Colorado..

A Fellow of the American Dietetic Association and a Fellow of The Academy of Nutrition and Dietetics, Ms. Bernstein is also a five-time recipient of the ADA's Creative Nutrition Education Award (1997, 2001, 2005, 2008, and 2013) for her extensive contributions. She was presented the Children's Choice Award for Innovation in 2000, and the Health and Science Communication Association's Silver Award in 2004.

Ms. Bernstein also has a patent for low-protein bacon which is now distributed by Taste Connections. She is a co-founder of Metabolic University, a training forum for registered dietitians, nurses, genetic counselors, and medical doctors and a founding member of Genetic Metabolic Dietitians International (GMDI). She is the co- editor and contributing author of Nutrition Management of Inherited Metabolic Diseases: Lessons from Metabolic University and co-author of Penny the Penguin Has PKU (English, Norwegian, and Spanish), Setting Up Your Low Protein Kitchen, and Max The Monkey has MCADD. She has developed numerous educational modules for PKU and Galactosemia.

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+ Kimberly A **CHAPMAN** MD, PhD, FACMG

Dr. Kim Chapman is an assistant professor of Pediatrics at the George Washington Medical School and Health Sciences Center. She is also an attending geneticist at the Children's National Medical Center in Washington, D.C.. Dr. Chapman co-hosted the 2011 Consensus conference of PA diagnosis and management at Children's National Medical Center. She received a B.S. in chemistry and a B.A. in biology from Saint Louis University (St. Louis, MO), a Ph.D. in molecular biology and biochemistry from the University of Nebraska, and a M.D. from the University of Nebraska, College of Medicine. She subsequently trained in internal medicine and pediatrics at the University of Pittsburgh Health Sciences Center in Pittsburgh, PA, clinical genetics and clinical biochemical genetics at Children's Hospital of Philadelphia, Philadelphia PA. She is board certified in internal medicine, pediatrics, clinical genetics and clinical biochemical genetics. She divides her time between clinical activities in genetics and metabolism at Children's National Medical Center and basic science research exploring energy metabolism in patients and their cells with Propionic Acidemia with the aim of improving therapies and morbidity in organic acidemias and other energy deficiency inborn errors.

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+ Kathryn **CHATFIELD**, MD, PhD

Dr. Chatfield is currently an Assistant Professor of Pediatrics in the Division of Cardiology at the University of Colorado and Children's Hospital Colorado. She completed her clinical pediatric cardiology fellowship after training in clinical genetics and metabolism. With dual specialization in pediatric cardiology and genetics/metabolic disease, she sees children and adolescents with genetic forms of heart disease, including inborn errors of metabolism, mitochondrial disease, chanelopathies, and syndromic forms of structural cardiovascular disease. She also cares for patients with heart failure and those who are waiting for, or have received a heart transplant. She continues to work on a project begun during her cardiology fellowship studying normal and abnormal metabolism in the heart, and the differences seen between children and adults, and between "iodiopathic" and metabolic causes of cardiomyopathy. In collaboration with Dr. Brian Stauffer (University of Colorado, Denver, Division of Cardiology) who studies age-specific aspects of myocardial remodeling in response to heart failure in children, Dr Genevieve Sparagna (University of Colorado, Denver) an expert in cardiolipin (CL) and other lipid analysis using electrospray ionization mass spectrometry and Dr. Johan Van Hove (University of Colorado, Denver), an international expert in mitochondrial energetics, the lab is focused on better understanding of how CL and other lipid metabolic changes in mitochondria affect myocardial energy production in dilated cardiomyopathy. Dr. Chatfield is currently funded by an NIH KO8 award to study mitochondrial function in pediatric dilated cardiomyopathy, and by the Barth Syndrome Foundation to study how CL alterations lead to energy failure in the Barth syndrome heart. The work thus far has validated that mitochondrial dysfunction is a common feature of familial and sporadic dilated cardiomyopathies in children. Ultimately, Dr. Chatfield and her collaborators are working towards a goal of generating therapies directed toward improving mitochondrial energetics in metabolic and idiopathic forms of cardiomyopathy. Dr. Chatfield's scientific interest in cardiac metabolism and cardiac genetics are well suited to improve our general understanding and treatment approach to inherited and idiopathic cardiomyopathies, as well as in primary disorders of mitochondria. The lab aims to generate novel clinical data relevant to a devastating and poorly understood diseases and, if the outcome is as we hypothesize, can realistically lead to a dietary and medical intervention to reverse the metabolic abnormalities they have identified in pediatric cardiomyopathies of known (metabolic) and unknown etiology.

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+ Stephen **GOODMAN**, MD

Dr Goodman received his medical and pediatric training at McGill University in Montreal, and came to Colorado in 1965 for a two year fellowship in what was then called "pediatric metabolism" - then somehow never got around to returning to Canada. He joined the faculty of the University of Colorado School of Medicine in 1967, and has since that time been deeply involved in clinical and lab investigation of inborn errors, and particularly on organic acidemias - some of which were first described in his laboratory.

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CONTINUED NEXT PAGE

CONFERENCE **SPEAKERS**

+ Stephen **KAHLER** MD

Dr. Kahler received his MD degree from Duke, trained in Pediatrics at UCSD (where he learned about inborn errors from Dr. Bill Nyhan), then trained in clinical and biochemical genetics at UNC-Chapel Hill. Throughout his career he has been a clinician-educator, seeing patients and teaching, but he has always had strong affiliations with research physicians and scientists. He worked at Duke from 1983-1997, in the division of Genetics and Metabolism directed by Dr. Roe. He was part of the team there that developed what is now called expanded newborn screening, with funding obtained from the state of North Carolina. He moved to Melbourne, Australia to direct the Victorian Clinical Genetic Services in 1998, returned to the US (Johns Hopkins) in 2003, and moved to Arkansas in 2005, where he succeeded Dr. Gibson. He has served on newborn screening advisory committees in five states. He has been interested in autism for many years, particularly the children who are responsive to diet changes, as they are similar in this way to children with defined inborn metabolic errors. There are also many children with these inborn errors who have features of autism. In Arkansas he helped found the Autism Specialty Clinic, devoted to metabolic, intestinal, and neurologic aspects of autism, where he works with Dr. Richard Frye (neurology). Investigations in that clinic include studying the mitochondrial dysfunction and oxidative stress that many of these children have. Dr. Kahler and Dr. Frye work closely with Jill James, PhD, on biochemical aspects of autism, particularly involving vitamin B12, folate, and glutathione. Dr. Kahler has written many articles and book chapters on genetic and metabolic disorders, and newborn screening. He has served as a medical advisor to many support organizations, including the FOD and OAA groups.

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+ Mark **KORSON** MD

Mark Korson graduated from the University of Toronto School of Medicine and completed his pediatric residency nearby at The Hospital for Sick Children. He came to Boston to do a fellowship in genetics and metabolism at Children's Hospital. Following that, he directed the Metabolism Clinic at Children's until 2000, transferring then to Tufts Medical Center's Floating Hospital for Children where he remained the Director of the Metabolism Service and an Associate Professor of Pediatrics at Tufts University School of Medicine until 2014.

Dr. Korson promotes an educational approach to address the growing crisis in metabolic health care due to the shortage of clinicians available to treat this community. Between 2007 and 2011, Dr. Korson directed the Metabolic Outreach Service, for which he traveled on a regular basis to five teaching hospitals in the northeastern US where there is no on-site metabolic service. The goal was to provide educational and consultative support so that non-metabolic clinicians could learn how to participate more in the diagnosis and management of patients with metabolic disease. That same year, Dr. Korson also co-founded the North American Metabolic Academy, an annual one-week intensive course about metabolic disease for genetic and metabolic trainees; to date, more than half of all American genetic trainees have enrolled in this course. NAMA is sponsored by the Society for Inherited Metabolic Disorders.

In 2015, Dr. Korson co-founded the Genetic Metabolic Center for Education, a comprehensive, multi-modal approach for improving the level of care for children and adults with metabolic disease. The components of the GMCE include a consultation and clinical support service, subspecialty-specific conferences, an interactive training/reference web site, a live patient speaker program and library of patient presentations, and development of symptom-based, practical brochures for distribution to clinicians.

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+ Jan **KRAUS** PhD

Dr. Jan P. Kraus completed his masters at the Charles University in Prague and his Ph.D. at the Basel University in Switzerland and is currently a Professor of Pediatrics at the University of Colorado School of Medicine. Since his postdoctoral years at Yale University he has been involved in the study of several inherited metabolic diseases including homocystinuria (HCU) and propionic acidemia (PA). His work included biochemical and enzymological studies of the enzymes involved, CBS and PCC respectively, cloning of the corresponding cDNAs and genes, chromosomal mapping studies, X-ray crystallography, and mutation determinations in patients with HCU and PA.

His laboratory is currently expressing human CBS and PCC in bacteria, and purifying the enzymes to homogeneity. The lab is using several different mouse models to develop enzyme replacement therapies (ERTs) for HCU and PA, the HCU ERT is destined to enter clinical trials in 2017. Dr. Kraus is also maintaining databases of HCU and PA patient alleles.

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+ Nicola **LONGO** MD, PhD

Dr. Longo received his M.D. and Ph.D. in molecular biology and pathology from the University of Parma, School of Medicine in Italy. He trained in Pediatrics, Medical and Biochemical Genetics at Emory University in Atlanta, Georgia, USA. Dr. Longo is board certified in medical genetics and clinical biochemical genetics. Currently, Dr. Longo is Professor of Pediatrics and Pathology at the University of Utah, Chief of the Division of Medical Genetics, Director of the Metabolic Service in the Department of Pediatrics, Director of the Training Program in Clinical Biochemical Genetics and Medical co-Director of the Biochemical Genetics and Newborn Screening Laboratories at ARUP Laboratories in Salt Lake City. His research concerns the molecular bases of metabolic disorders and their identification through newborn screening. His focus is on disorders of the carnitine cycle and of fatty acid oxidation. He has an active clinical research program directed toward the development of new therapies for patients with metabolic disorders.

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CONFERENCE **SPEAKERS**

+ Irina **MANOLI** PhD

Dr Manoli is a physician scientist who works as a staff clinician in the Organic Acid Research Section of the National Human Genome Research Institute (NHGRI), National Institutes of Health (NIH), in Bethesda, MD. She received her M.D. from the University of Athens, Greece and subsequently pursued residency training in pediatrics and neonatology at the John Radcliffe hospital, University of Oxford, UK. She went on to do postgraduate training including a M.Sc. in pediatric endocrinology and a Ph.D. in genetics, both at the University of Athens, Greece. She moved to the US and worked as a postdoctoral fellow on mitochondrial genomics at the National Center for Complementary and Alternative Medicine, NIH, Bethesda, MD. Subsequently she trained in genetics and clinical biochemical genetics, at the Medical Genetics Branch, NHGRI, NIH, Bethesda, MD and was board certified in 2009.

Her primary interest is in combining work on animal models and clinical studies with the aim to develop new therapies for methylmalonic acidemias (MMA) and defects of intracellular cobalamin metabolism. She has worked with a number of mouse models of methylmalonic acidemia, studying the pathophysiology underlying different disease manifestations, discovering new disease biomarkers and testing small molecule therapies. Along with the work in the lab, she takes care of the patients enrolled in the NIH clinical protocol on MMA and cobalamin disorders and works on developing new metabolic formulas and dietary guidelines for the management of MMA patients. Her ultimate aim is to translate preclinical therapies from animal models of MMA into the clinic.

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+ Emily **MCCOURT**, MD

Assistant Professor of Ophthalmology, University of Colorado School of Medicine

Dr. McCourt received her undergraduate degree from University of Pennsylvania and medical degree from SUNY Buffalo. She completed ophthalmology residency and a pediatric ophthalmology fellowship at the University of Colorado School of Medicine in Aurora Colorado. She is director of research for her section and holds numerous leadership roles in the residency program.

Dr. McCourt treats a wide range of pediatric eye disease. Her clinical interests include pediatric cataract surgery, retinal disease, and ocular surface disease. Her main research focuses are understanding the clinical eye disease in patients with cobalamin C defect, treatments for pediatric corneal anesthesia, epidemiology of Retinopathy of Prematurity, and alternative medications for pediatric ocular surface disease.

Emily McCourt, MD
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+ Oleg **Shchelochkov** MD

Dr. Shchelochkov received his medical degree from Tashkent Pediatric Medical Institute, completed Pediatric residency at the University of Iowa, Clinical Genetics Fellowship and Medical Biochemical Fellowship at Baylor College of Medicine. Prior to joining NIH as a Staff Clinician, Dr. Shchelochkov was an Assistant Professor at the University of Iowa Hospitals and Clinics. Dr Shchelochkov studies natural history of organic acidemias and works on developing new treatments for patients with metabolic disorders.

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+ Jennifer **SLOAN** PhD, MS, CGC

Dr Sloan is currently working at the National Human Genome Research Institute (NHGRI) as the genetic counselor and protocol coordinator for the methylmalonic acidemia (MMA) and cobalamin disorders study with Dr. Venditti. She received her bachelor of science in Biology from Penn State University and went on to complete a PhD in Neurobiology. Dr. Sloan was interested in pursuing more clinically relevant work, which led her to pursue a Master's program in genetic counseling shortly after finishing her doctoral dissertation. She received a M.S. in Genetic Counseling from Northwestern University in2004 and has been at the NHGRI since that time. Dr. Sloan became a board-certified genetic counselor in 2005. She is interested in genetic counseling issues in metabolic disorders and identifying new disease genes.

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+ Janet **THOMAS** MD

Associate Professor, University of Colorado Health Sciences Center, Department of Pediatrics, Division of Clinical Genetics and Metabolism. Board certified in Pediatrics, Clinical Genetics, and Clinical Biochemical Genetics.

Dr. Thomas is the Director of the Inherited Metabolic Diseases Clinic at Children's Hospital Colorado. She has been on the faculty of the University of Colorado School of Medicine since 1996 and devotes her time to caring for individuals of all ages with inborn errors of metabolism in the Rocky Mountain region. She has a particular interest in lysosomal storage disorders, newborn screening, and regional care. Dr. Thomas is a faculty member of the UCDHSC Human Medical Genetics Program, a member of the State of Colorado Newborn Screening Program Advisory Committee, and former Director of the University of Colorado Medical Genetics Residency Program. She recently became the Associate Program Director for the Mountain States Genetics Regional Collaborative, an organization focused on regional and national delivery of genetic services including underserved populations, telegenetics, consumer advocacy and resources, and health care policy. Teaching medical students, pediatric and genetic residents, and graduate genetic counseling students is also a part of her duties. She is actively involved in industry sponsored therapeutic research for PKU and several lysosomal storage disorders.

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+ Johan **VAN HOVE** MD, PhD

I had my medical and pediatric training at the Catholic University of Leuven. I then had training in Genetics and Metabolism at Duke University 1990-93, followed research training 1993-1995. I was faculty at Duke from 1995 to 1997, at Royal Children's Hospital Melbourne and the Victorian Clinical Genetics Services in Melbourne, Australia in 1997; then was faculty at the University of Leuven from 1998 to 2003. I have been faculty at the University of Colorado and practicing at Children's Hospital of Colorado from 2003 to now.

My research interest is focused on translational research, taking problems from patients into the laboratory, finding solutions and then applying these to patients. I particularly like to work on the development of new treatments for disorders that have no good solution. My primary research focus is twofold: disorders of mitochondrial bioenergetics (includes mitochondrial respiratory chain diseases and fatty acid oxidation disorders), and disorders of neonatal epileptic encephalopathy caused by metabolic diseases particularly nonketotic hyperglycinemia and pyridoxine dependent epilepsy.

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CONFERENCE **SPEAKERS**

+ Chuck **VENDITTI** MD, PhD

Dr. Charles P. Venditti is an Investigator in the National Human Genome Research Institute and the Director of the Organic Acid Research Section at the National Institutes of Health in Bethesda, MD. He received his BS from and was an MD, PhD scholarship recipient at Penn State University. After graduation, he completed a pediatrics residency at Massachusetts General Hospital/ Harvard Medical School and a combined clinical and biochemical genetics fellowship at the Children's Hospital of Philadelphia/University of Pennsylvania School of Medicine. He is board-certified in pediatrics, clinical and biochemical genetics and is an Attending Physician at the Mark O. Hatfield Clinical Center at the NIH, where he has initiated a translational research program to study the natural history and clinical phenotype(s) of the hereditary methylmalonic acidemias (MMA) and cobalamin metabolic disorders. The clinical research studies are complemented by laboratory investigations that have focused on the development of experimental systems to study genetic, genomic and biochemical aspects of organic acid metabolism in model organisms, including roundworms, mice and zebrafish. Using a translational research approach, Dr Venditti and his colleagues have published a number of papers that connect disease pathophysiology in MMA to mitochondrial dysfunction and prove the efficacy of gene therapy as a treatment for both methylmalonic acidemia and propionic acidemia. Dr Venditti is a member the American Society of Clinical Investigation (ASCI) as well as numerous professional organizations in the fields of inborn errors of metabolism, genetics and gene therapy. In 2009, he was the recipient of a Presidential Early Career Award for Scientists and Engineers (PECASE), the US Government's highest honor for early-career scientists. He serves on the medical advisory board of the Organic Acid Association (OAA) and as a member of the Scientific Review Committee for the NHGRI IRB. He has authored and co-authored more than 70 research articles, clinical reports, patent applications and textbook chapters.

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+ Jerry **VOCKLEY** MD, PhD

Cleveland Family Professor of Pediatric Research, School of Medicine

Professor of Human Genetics, Graduate School of Public Health

Chief of Medical Genetics, Children's Hospital of Pittsburgh

Director of the Center for Rare Disease Therapy, Children's Hospital of Pittsburgh

Dr. Vockley received his undergraduate degree at Carnegie-Mellon University in Pittsburgh, Pennsylvania, and received his degree in Medicine and Genetics from the University of Pennsylvania School of Medicine in Philadelphia, Pennsylvania. He completed his pediatric residency at the University of Colorado Health Science Center, and his postdoctoral fellowship in Human Genetic and Pediatrics at Yale University School of Medicine in New Haven, Connecticut. Before assuming his current position in Pittsburgh, Dr. Vockley was Chair of Medical Genetics in the Mayo Clinic School of Medicine.

Dr. Vockley is internationally recognized as a leader in the field of inborn errors of metabolism. His lab has been responsible for identifying multiple new genetic disorders, many of them defects in mitochondrial energy metabolism, and he has published over 180 scientific articles in peer review journals. His current research focuses on the molecular architecture of mitochondrial energy metabolism, in which he is breaking new ground in describing the role of dysfunction of mitochondrial energy metabolism in such common conditions as diabetes, obesity, and Alzheimer disease. He also is a leader in the development and testing of novel therapeutic agents for treating inborn errors of metabolism.

Dr. Vockley has served on numerous national and international scientific boards including the

Advisory Committee (to the Secretary of Health and Human Services) on Heritable Disorders in Newborns and Children where he was chair of the technology committee. He is co-chair of the International Network on Fatty Acid Oxidation Research and Therapy (INFORM). He also serves as chair of the Pennsylvania State Newborn Screening Advisory Committee and the American College of Medical Genetics Therapeutics Committee. He is a past president of the International Organizing Committee for the International Congress on Inborn Errors of Metabolism and the Society for the Inherited Metabolic Disorders (SIMD). He is also a volunteer medical advisor for several parent and family support groups including the Fatty Acid Oxidation Family Support Group, Save Babies through Screening, United Mitochondrial Disease Foundation, and the Organic Acidemia Support Group. He speaks at multiple family support functions throughout the year for CanPKU and the NPKU Alliance.

Dr. Vockley is the co-founder and editor of the North American Metabolic Academy established by the SIMD to help educate the next generation of metabolic physicians in the United States, and serves as associate editor for the journal Molecular Genetics and Metabolism. He is founder of the International Network on Fatty Acid Oxidation Research and Management (INFORM). Dr. Vockley was recognized in 2002 as the Research Educator of the Year while at the Mayo Clinic. At the University of Pittsburgh, Dr. Vockley teaches in the both the Medical School and Graduate School of Public Health. Dr. Vockley has mentored numerous Ph.D. candidates, post-doctoral fellows, and undergraduate in their research.

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+ Brian **WAMHOFF** PhD

Dr. Wamhoff is a founder of HemoShear Therapeutics' and serves as Head of Innovation, leading efforts to develop preclinical models to better understand therapeutic options for the treatment of organic acidemias. Dr. Wamhoff was faculty at the University of Virginia in the Departments of Medicine and Biomedical Engineering. His research was part of several patents that provided foundational intellectual property for four medical device and therapeutics companies. Dr. Wamhoff has over 60 publications that have been cited over 4,350 times. Dr. Wamhoff has 8 issued US patents for his work in human disease models, medical devices and therapeutics. Dr. Wamhoff obtained a BS in biology with a minor in business administration from Rhodes College in 1996, where he was the 2011 Distinguished Alumnus, and received his Ph.D. in medical physiology from the University of Missouri in 2001. HemoShear's current drug discovery efforts are focused on organic acidemias, a group of rare, often lethal genetic metabolic disorders in children, and nonalcoholic steatohepatitis (NASH), a global, epidemic disease in adults.

TEDx "Saving a child with a rare disease"
www.youtube.com/watch?v=DrwmAOrFS9o&feature=youtu.be

MedScape "The Future is Now: Rare Diseses in a Dish
www.medscape.com/viewarticle/854374

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Once again ~ a big THANK YOU to all that helped make this conference possible!

CONFERENCE **NOTES**

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