Children's Hospital of Philadelphia®

Taylor, a patient with CHOPS syndrome, and her parents

CHOPS FAMILY SYMPOSIUM

Friday, July 19 & Saturday, July 20, 2024

Leonard and Madlyn Abramson Pediatric Research Center

AGENDA

**All meetings to be held in conference rooms 123ABC in Abramson Research Center.*

Friday, July 19

8:30 – 9 a.m. Arrival and Registration

9 – 9:15 a.m. Welcome and Introduction
Lainey Moseley and Manuela Mallamaci, PhD

CLINICAL ASPECTS OF CHOPS SYNDROME

9:15 – 9:40 a.m. Genetics
Ian D. Krantz, MD, and Sarah Raible, MS, CGC

9:15 – 9:40 a.m. Child Development
Mary Pipan, MD

10:05 – 10:30 a.m. Cardiology
Catherine M. Avitabile, MD

10:30 – 10:45 a.m. BREAK

10:45 – 11:10 a.m. Speech and Statistical Study Analysis in CHOPS Syndrome
Gemma Levantino, MSc

11:10 – 11:35 a.m. Speech and Alternative Communication Methods
Stephanie A. Zbikowski, MA, CCC-SLP

11:35 a.m. – Noon Immunology and Inhibitor Treatment Therapy
Alix E. Seif, MD, MPH

Noon – 12:25 p.m. Pulmonology
Lisa R. Young, MD

12:25 – 1:30 p.m. LUNCH

1:30 – 5 p.m. Individual Informal Mini Consultations
(to be scheduled)

Saturday, July 20

8:30 – 9 a.m. Arrival and Registration

9 – 9:15 a.m. Rare Disease World and CHOPS Syndrome: What it means to be a family advocate for rare disease
Lainey Moseley

9:15 – 9:30 a.m. Fondazione CHOPS History and Journey
Manuela Mallamaci, PhD

9:30 – 9:45 a.m. Scientific Committee of Fondazione CHOPS: Our tasks and goals
Valentina Massa, PhD

BASIC RESEARCH SESSIONS

9:45 – 10 a.m. Strategies and Roadmap of Clinical Trials for CHOPS Syndrome
Neil Hackett, PhD

10 – 10:15 a.m. BREAK

10:15 – 10:35 a.m. Toward the Identification of Therapeutic Target(s) in CHOPS Syndrome Using Genomic Methodologies
Kosuke Izumi, MD, PhD

10:35 –10:55 a.m. Deciphering AFF4 W Expression Dynamics: Insights Into neurodevelopmental disorders and targeted interventions
Ilaria Parenti, PhD

10:55 – 11:15 a.m. MeCP2 Interacts With the Super Elongation Complex to Regulate Transcription
Junyoung (Jun) Sonn, PhD

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APPLIED RESEARCH SESSIONS

11:15 – 11:35 a.m.	Path to a Cure in Rare Diseases and CHOP *Ethan Perlstein, PhD*
11:35 – 11:55 a.m.	Results From Fondazione CHOPS Drug Screening Project *Richard Novak, PhD*
11:55 a.m. – 12:15 p.m.	Super Elongation Complex and Study of Disruptors/Inhibitors of the Complex and Their Use for Treatment of Cancer and Other Diseases *Ali Shilatifard, PhD*
12:15 – 12:30 p.m.	Closing Remarks
12:30 – 2 p.m.	LUNCH
2 – 4 p.m.	"RARE," A Documentary by Lainey Moseley
4 p.m.	ADJOURN

Ian Krantz, MD, poses with patients who have CHOPS syndrome at the 2019 CHOPS Family Symposium at Children's Hospital of Philadelphia.

SPEAKERS

**Listed alphabetically.*

Catherine M. Avitabile, MD, is an Attending Cardiologist and Pulmonary Hypertension Specialist in the Cardiac Center at the Children's Hospital of Philadelphia, and Assistant Professor of Pediatrics in the Perelman School of Medicine at the University of Pennsylvania. Dr. Avitabile participates in multi-centered clinical trials to advance the care of children with pediatric pulmonary hypertension. Her personal research program studies the impact of musculoskeletal deficits on exercise performance in children with chronic heart and lung diseases. She is an expert in actigraphy and wearable technologies. Dr. Avitabile has cared for and managed cardiac findings in patients with CHOPS syndrome and attended the first CHOPS syndrome family meeting at Children's Hospital of Philadelphia in 2019.

Neil Hackett, PhD, is an independent consultant based in New York City with extensive experience in pre-clinical and translational programs for gene therapy. He currently works with research groups and foundations to navigate stem cell and gene therapy projects through pre-investigational to new drug applications (IND). This includes project management, advising on proof-of-concept and toxicology studies, vector manufacturing and regulatory writing. His background includes professorial appointments at Vanderbilt University and Weill Cornell Medical College resulting in more than 100 academic publications. Dr. Hackett is a genetic consultant for Fondazione CHOPS.

Kosuke Izumi, MD, PhD, has been interested in the molecular mechanisms of pediatric genetic disorders since his time at Keio University School of Medicine in Tokyo. After clinical training in pediatrics and genetics at UH Rainbow Babies & Children's Hospital and Children's Hospital of Philadelphia (CHOP), Dr. Izumi began a fellowship at CHOP where he investigated the molecular mechanisms of Pallister-Killian syndrome and syndromic neurodevelopmental disorders. In 2023, Dr. Izumi joined the Department of Pediatrics at the University of Texas Southwestern. Though the years, Dr. Izumi's research lab has focused on Pallister-Killian syndrome, nuclear speckle disorders, and chromatin disorders. His research group discovered causative genes for many Mendelian disorders, including those that affect AFF4, ARCN1, NKAP, and CBX1. By employing cutting-edge genomic technologies, his research aims to link the clinical findings of patients with genetic disorders to underlying genomic functional alterations. Dr. Izumi founded and named CHOPS syndrome, along with Ian Krantz, MD, in 2015, while both were physicians at CHOP. Dr. Izumi is also on the advisory board of CHOPS Syndrome Global. A grant of $200,000 has been awarded to Dr. Izumi by Fondazione CHOPS.

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Ian D. Krantz, MD, is a Division Chief of Medical Genetics at Cohen Children's Medical Center and System Vice President of Genetics at Northwell Health in New York and the Zucker School of Medicine at Hofstra University. He is also an Emeritus Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania and attends a monthly Cornelia de Lange Syndrome (CdLS) and Related Diagnoses Clinic in the Center for Cornelia de Lange Syndrome and Related Diagnoses at the Children's Hospital of Philadelphia (CHOP). He formerly served as Co-Director of CHOP's Roberts Individualized Medical Genetics Center. Dr. Krantz is the chairman of the medical and advisory board of Fondazione CHOPS. His research interests are focused on identifying and characterizing the molecular etiology of syndromic and non-syndromic developmental disorders and assessing the genomic implications of the genes and pathways perturbed in these diagnoses. His research lab has focused on projects studying the molecular etiologies of CdLS, CHOPS syndrome, Pallister-Killian syndrome (PKS), Alagille syndrome, hearing loss, congenital diaphragmatic hernias and congenital heart defects. Towards this end, his lab has identified many new disease genes and novel human disorders; and implicated many critical molecular pathways, such as Notch Signaling, Cohesin and the Super Elongation Complex, in human developmental disorders for the first time. Clinically, he has been at the forefront of adapting new genomic technologies in the clinical setting (such as rapid genome sequencing into the NICU and CICU) and studying how this evolving, complex and often unclear diagnostic information is understood by — and the impact it has on — the clinicians and families involved.

Gemma Levantino, MSc, is a speech therapist who primarily works with patients suffering from neurodegenerative and oncological diseases. As a member working in SAMOT Onlus, an important Italian nonprofit that is a leader in the field of palliative care, Levantino follows patients from the age of development to the geriatric age, and into the end of their lives, helping them maintain communication, linguistic and swallowing functions or recommending alternative augmentative communication and feeding aids. She performs tutoring activities at the University of Palermo, Italy, by teaching speech therapy in palliative care and supervising students. One of the works she supervised was specifically focused on patients with CHOPS syndrome.

Manuela Mallamaci, PhD, is President and Co-Founder of Fondazione CHOPS Malattie Rare (CHOPS Foundation Rare Diseases), a senior researcher in the Departments of Physics and Chemistry at the University of Palermo, Italy, and a lecturer with the university's Department of Robotic Engineering. She obtained her PhD in Physics, Astrophysics and Applied Physics in 2017 at the University of Milan. She leads the only nonprofit in the world dedicated to driving and accelerating targeted therapies for CHOPS syndrome. Fondazione CHOPS was established in May 2023, just a couple of months after the diagnosis of CHOPS in her son Mario, who is now 3 years old.

See related story on Fondazione CHOPS on page 11.

Valentina Massa, PhD, has focused her scientific research on understanding the cellular and molecular mechanisms underlying malformations of the central nervous system. She has been studying Cornelia de Lange syndrome (CdLS) and other chromatinopathies since 2012 using different and complementary modeling systems. Dr. Massa has identified the canonical WNT pathway as a master regulator of the neurodevelopmental alterations for CdLS. She tested *in vitro* and *in vivo* lithium for defect rescuing. Through solid collaboration with the patient's association and involved physicians, she established a connection among developmental abnormalities and cognitive impairment paving the way for a clinical trial, demonstrating the feasibility of bench-to-bedside approach, even in the most challenging setting of rare disease studies. She designed and conducted several public engagement activities over the past 10 years dedicated to the importance of science and the fundamental bidirectional communication with the general public (pupils, teachers, family association etc.), with special attention to girls and women in STEM. Such activities have been recognized with prizes, including the prestigious "Medaglia d'oro di Benemerenza civica della città di Milano" in December 2021. Dr. Massa is a member of the scientific and medical advisory board of Fondazione CHOPS.

Lainey Moseley is a mom of four and founder/leader of CHOPS Syndrome Global. A journalist by trade, Moseley began her advocacy work involving rare diseases after her daughter Leta's 18-year-diagnostic odyssey that culminated in being diagnosed with an ultra-rare disease, a mutation of the AFF4 gene. The gene was identified by Dr. Izumi and Dr. Krantz at Children's Hospital of Philadelphia, and soon after named CHOPS syndrome. Moseley began her rare disease advocacy that day by starting CHOPS Syndrome Global with the goal of building awareness in the rare disease world and, perhaps more importantly, building a small-but-mighty community of global families affected by the disease. Moseley spent her professional career as a journalist, working in Washington, DC, covering politics for CBS News. Later, she worked in New York City for a variety of TV news programs including *CBS Evening News with Dan Rather, 48 Hours, NBC Nightly News* and *The Today Show.* Moseley is currently an independent filmmaker developing a documentary about her family's story called "RARE: The Story of How Love Drives Change for Rare Disease."

Richard Novak, PhD, is CEO and Co-Founder of Unravel BioScience, Inc. As a bioengineer and entrepreneur, Dr. Novak has dedicated his life-solving unanswerable questions through technology innovation. Prior to Unravel Bio, he was a lead engineer at the Wyss Institute for Biologically Inspired Engineering at Harvard University. There, he managed fast-paced programs in drug discovery, advanced disease models, human

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organ chips, and integrated automation and sensor systems. He has more than 15 years of experience in microfluidic system development and applications in the therapeutic space. Dr. Novak is a founder and president of the nonprofit, Future Scientist, and a founder and director of Rhinostics, Inc., a sample collection automation company. Dr. Novak and his team at Unravel are carrying out a research project on CHOPS syndrome, funded by Fondazione CHOPS.

Ilaria Parenti, PhD, is a medical biotechnologist who is currently working as researcher at the Institute of Human Genetics at the University Hospital Essen in Germany. Her research focuses on the clinical and molecular aspects of Cornelia de Lange syndrome and, more broadly, chromatinopathies. A seed grant was awarded to Dr. Parenti by Fondazione CHOPS.

Ethan Perlstein, PhD, CEO of Perlara PBC and cure Sherpa, is a highly accomplished researcher who's dedicated to disrupting the research and drug development model to discover cures for rare genetic diseases. Over the course of the past decade — first as a graduate student at Harvard University in the Department of Molecular and Cell Biology and later as an independent postdoctoral fellow at the Lewis-Sigler Institute for Integrative Genomics at Princeton University — Dr. Perlstein developed an approach to studying old drugs and discovering new drugs in model organisms called evolutionary pharmacology. Perlstein is the author of 19 peer-reviewed scholarly publications, including the discovery of a novel mechanism of action for the antidepressant Zoloft based on studies in yeast cells. Perlstein's company, Perlara PBC, collaborates with Fondazione CHOPS.

Mary Pipan, MD, is a behavioral pediatrician and Director of the Trisomy 21 Program at Children's Hospital of Philadelphia. She is board-certified in Behavioral Pediatrics and Pediatrics and her research interests include behavioral support for children with special needs and developmental disabilities, Down syndrome and other genetic disorders. Dr. Pipan is on the Board of Directors of the Down Syndrome Medical Interest Group-USA (DSMIG-USA). She has expertise caring for children with Cornelia de Lange syndrome and related diagnoses, including CHOPS syndrome. She also attended the last CHOPS Syndrome Foundation family meeting at CHOP in 2019.

Behavioral Pediatrician Mary Pipan, MD, talks with a patient at the last CHOPS Family Symposium.

Sarah E. Raible, MS, CGC, is a genetic counselor, Clinical Director of the Center for Cornelia de Lange Syndrome (CdLS) and Related Diagnoses, and Rare Disease Core Leader in the Roberts Individualized Medical Genetics Center (IMGC) at Children's Hospital of Philadelphia (CHOP). She received her undergraduate training from Boston College and earned her master's in genetic counseling from the University of Pittsburgh in 2012. Since joining the CdLS center in the summer of 2012, Raible's focus has been on expanding clinical and research programs of the center. She oversees all administrative and patient care operations for the center and is the primary contact for families interested in our monthly multidisciplinary clinics, serving as a resource before, during and after their visit. Raible also assists families interested in enrolling in CHOP's CdLS research studies or participating in the telemedicine/virtual consultation program. Raible is the primary mentor and thesis supervisor for students participating in the Marie Barr Genetic Counseling Award and is spearheading several research projects related to the care and management of children with CdLS. She also has taken on oversight of the National Institutes of Health (NIH) grants that help support the research mission of the center.

Alix E. Seif, MD, MPH, is a pediatric oncologist at Children's Hospital of Philadelphia and has been in practice for more than 20 years. She is an Innovation Faculty member at the Penn Center for Cancer Care Innovation (PC3I), an attending physician in both the Blood and Marrow Transplant Program and the Hematologic Malignancies section at Children's Hospital of Philadelphia (CHOP), an Associate Professor of Pediatrics at the Perelman School of Medicine, and the Richard and Sheila Sanford Endowed Chair in Pediatric Oncology. Her current research focuses on developing and implementing patient-centered interventions to improve outcomes, quality of life, and equity for pediatric patients with leukemia. Dr. Seif is also interested in collaborating with patients and their caregivers to improve the clinical research consent process for children undergoing cancer therapy. Dr. Seif is partnering with patients and caregivers from marginalized or minoritized communities to inform the development of these novel interventions, aiming to mitigate historical inequities.

Ali Shilatifard, PhD, is a biochemist and molecular biologist, Robert Francis Furchgott Professor and Chair of the Department of Biochemistry and Molecular Genetics, and Director of the Simpson Querrey Institute for Epigenetics at Northwestern University Feinberg School of Medicine. He has served as a member of the Senior Editorial Board for the journal *Science*, also serving as the journal's founding Deputy Editor and the first Academic Editor for the publications open access journal, *Science Advances*, between 2014 and 2023. During his tenure as the editor of *Science Advances*, the journal oversaw 400 deputy and associate editors managing more than 22,000 annual submissions and 2,000 annual publications. Dr. Shilatifard has served on the Scientific Advisory Board (SAB) of Keystone Symposia, Max Planck Society, and Genentech and is a member of the jury for the BBVA Foundation Frontiers of Knowledge Awards in Biology

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and Biomedicine. Research in Shilatifard's lab focuses on the cause of childhood leukemia through chromosomal translocations, the role of ELL in this process, and the discovery of the Super Elongation Complex as a central complex linking MLL translocations into a diverse number of genes to leukemic pathogenesis. He is an elected fellow of the American Association for the Advancement of Science (AAAS), and elected member of American Academy of Arts & Sciences (AAA&S).

Junyoung (Jun) Sonn, PhD, is a postdoctoral fellow in Huda Y. Zoghbi's lab at Baylor College of Medicine. He received his PhD in Biological Sciences from Korea Advanced Institute of Science and Technology under the mentorship of Joonho Choe. During his PhD studies, Dr. Sonn researched the genetic mechanisms regulating sleep in Drosophila, a small insect with only four pairs of chromosomes. In the Zoghbi lab, Dr. Sonn is utilizing a cross-species approach to elucidate novel interactors of MeCP2 and studying the mechanistic basis by which their interactions regulate transcription. A seed grant was awarded to Dr. Sonn by Fondazione CHOPS.

Lisa R. Young, MD, is Chief of the Division of Pulmonary and Sleep Medicine at Children's Hospital of Philadelphia (CHOP) and Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania. Before joining CHOP, Dr. Young was an Associate Professor of Pediatrics, Medicine, Cell and Developmental Biology at Vanderbilt University and Director of the Center for Childhood Lung Research and the Pediatric Rare Lung Diseases Clinical Program at Vanderbilt University Medical Center. Dr. Young has been well-funded by the NIH and other funding agencies for laboratory and clinical research on a variety of lung diseases and is currently principal investigator on two NIH R01 awards, an NIH U54 project, and has a K24 Midcareer Award in patient-oriented research. She has a particular interest in interstitial and other rare lung diseases including Hermansky-Pudlak syndrome, neuroendocrine cell hyperplasia of infancy, primary ciliary dyskinesia, bronchopulmonary dysplasia, and lymphangioleiomyomatosis.
Dr. Young has received multiple awards for her research, including the American Thoracic Society Robert B. Mellins Outstanding Achievement Award, the American Thoracic Society Public Advisory Roundtable Excellence Award, the LAM Foundation Scientific Advancement Award, and a Parker B. Francis Foundation Fellowship. She has received recognition for her clinical skills from Best Doctors in America, *Cincinnati Magazine*, and the Hermansky-Pudlak Syndrome Network. Dr. Young is committed to improving child lung health through expanding the outstanding clinical, training and research programs in the Division of Pulmonary Medicine at CHOP. She attended the first CHOPS syndrome meeting in 2015 and has an interest in this patient population.

Stephanie A. Zbikowski, MA, CCC-SLP, is an outpatient speech-language pathologist at the Center for Childhood Communication at Children's Hospital of Philadelphia. She focuses on working with complex communicators and their families. Zbikowski holds active certification with the Neurodevelopmental Treatment Association (NDTA) and American Speech-Language Hearing Association (ASHA). She is also a member of the International Society for Augmentative and Alternative Communication (ISAAC).

SUPPORTERS

CHOPS SYNDROME GLOBAL

Our story

CHOPS Syndrome Global began with three kids and two amazing genetic doctors in search of answers to a genetic anomaly. In 2015, Dr. Ian Krantz and Dr. Kosuke Izumi discovered that Leta, Liam and Nadira all shared a rare "de novo" mutation on the AFF4 gene on chromosome 5.

That day, we became a family and since then our family has grown worldwide. Our organization is comprised of families from Australia to Israel who are all struggling to raise their kids affected by CHOPS Syndrome. But collectively, we are advocating for treatments and answers to what causes this very rare genetic syndrome. Our hope is to find treatments to improve the quality of our kids' lives — but also to someday find a cure.

As parents of CHOPS kids, we understand the confusion, the overwhelming experience of the unknown and the feelings of isolation all too well. That's the reason CHOPS Syndrome Global was born — to connect, empower and inspire our small, but mighty, community.

Our vision

We envision a globally connected community so that families newly diagnosed will not feel so alone. We are here to connect, empower and inspire your family and hopefully work together to someday discover a cure for CHOPS. We also hope to be a resource to help families find doctors and researchers that are working on treatments and answers for our CHOPS kids.

We are here to help you advocate for your child's best quality of life and we are your global support system for whatever else you need to become effective advocates on your own behalf.

Our future

In collaboration with our Italian sister organization, Fondazione CHOPS Malattie Rare **(www.fondazionechopsets.com/en)**, we are raising money, building a scientific board of directors and moving research forward for CHOPS syndrome treatments. Our team is united by our determination to support the CHOPS syndrome community and provide what patients and advocates need to someday find a cure.

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Fondazione CHOPS Malattie Rare is the first and only international non-profit Foundation dedicated to CHOPS syndrome. Its main mission is to drive and accelerate targeted therapies for CHOPS Syndrome.

In one year since the organization's birth:

- More than 340k euros have been raised
- Both medical and scientific advisory boards have been created
- Collaborations with two genetic consultants have begun
- Four research projects have been funded/ approved
- More than 40 fundraising campaigns have been launched across Italy to support Fondazione CHOPS
- More than 2,500 individuals and groups have donated.

Our story

It starts on 26th of January 2023 – Mario is not 2 years old yet when he receives the diagnosis of CHOPS syndrome. Manuela, Mario's mum, leaves little room for bitterness; together with her husband Gianni, Mario's dad, she decided to turn their pain into a path of light, faith and research.

She reads up tirelessly and she finds an international group of families "CHOPS Syndrome Global," who share the same heavy burden. She tries to get in touch with them, but is initially unsuccessful.

A few days later, Mario accidentally starts a video call by touching the phone with his curious little hands. It's the right one: Manuela and Lainey Moseley from Philadelphia are finally in touch!

Mario, 3, and Luce, 16, are the inspiration for Fondazione CHOPS

Thanks to Lainey, Manuela can talk to the other families and to Dr. Krantz. She asks him immediately if there is a hope for a cure. The answer is: "YES, there is."

Manuelaand Gianni cannot but cling to this hope with all their energies, believing in science and progress. They start a fundraising campaign "Un aiuto per Mario e non solo" which has an incredible success.

In the meanwhile another Italian family joins their efforts: Luce's family. Luce is 16 years old and her story is also a story of pain. In her case, it was also a very long wait for a diagnosis — arriving when Luce was 10 years old and after a journey from Italy to Texas Children's Hospital. After meeting Mario's family, Luce's family does not feel "alone" anymore.

Together, these two families developed the idea of establishing a foundation that was established on May 13, 2023.

Fondazione CHOPS is proud to be at CHOP for the 2nd CHOPS Symposium with her sister organization and to support the second day's incredible scientific programme focused on projects funded and/or approved by Fondazione CHOPS.

To find out more information about Fondazione CHOPS, please visit **www.fondazionechopsets.com/en/.**

Our story

Founded in 2016, Cool Cars for Kids (CCfK), is a 501(c)(3) non-profit organization based in Philadelphia, which exists to provide help and hope to children born with rare genetic diagnoses. Through the support of individuals and the classic car community, the mission of CCfK is to improve the lives of children with rare diagnoses and to support research that will identify the best possible treatments.

You may wonder about the connection between classic car enthusiasts and the families of children with birth disorders. These two groups share a common passion for the unique and an appreciation for the one-of-a-kind.

- Both have great respect for individuality and both care for someone or something valuable that is often underappreciated.
- Both give great attention to detail and see the beauty in qualities that are often overlooked.
- Both know that it takes time, patience, dedication and expert care to reach their full potential.

It seems like a destined union to bring the passion of these communities together to allow these beautiful children to benefit from these beautiful cars. Cool Cars for Kids is proud to present the sixth annual Philadelphia Concours d'Elegance.

The Philadelphia Concours d'Elegance is the main fundraising event for this nonprofit organization. Proceeds directly support the Children's Hospital of Philadelphia (CHOP). To date these events have raised more than $300,000 for the Roberts Individualized Medical Genetics Center's Rare Diagnoses Program at CHOP.

We invite you to become a partner in our efforts to make an impact in:

- Accelerating awareness
- Driving research
- Racing towards treatment

Cool Cars for Kids is proud to support and sponsor the 2nd CHOPS Syndrome Foundation meeting at the Children's Hospital of Philadelphia. To find out more information about Cool Cars for Kids and our mission, please visit our website at **www.coolcarsforkids.org.**

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A look back: Participants and staff at CHOPS Family Symposium in 2019.

Children's Hospital of Philadelphia®

About the Division of Human Genetics

The Division of Human Genetics at Children's Hospital of Philadelphia provides comprehensive diagnostic testing, genetic counseling, individualized treatment and ongoing care management for children with a wide variety of genetic diseases and pediatric disorders. We specialize in finding answers for families facing complex and hard-to-diagnose conditions, work with researchers to improve testing, treatment options and outcomes, and remain committed to better understanding the genetic basis for all pediatric disorders.

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