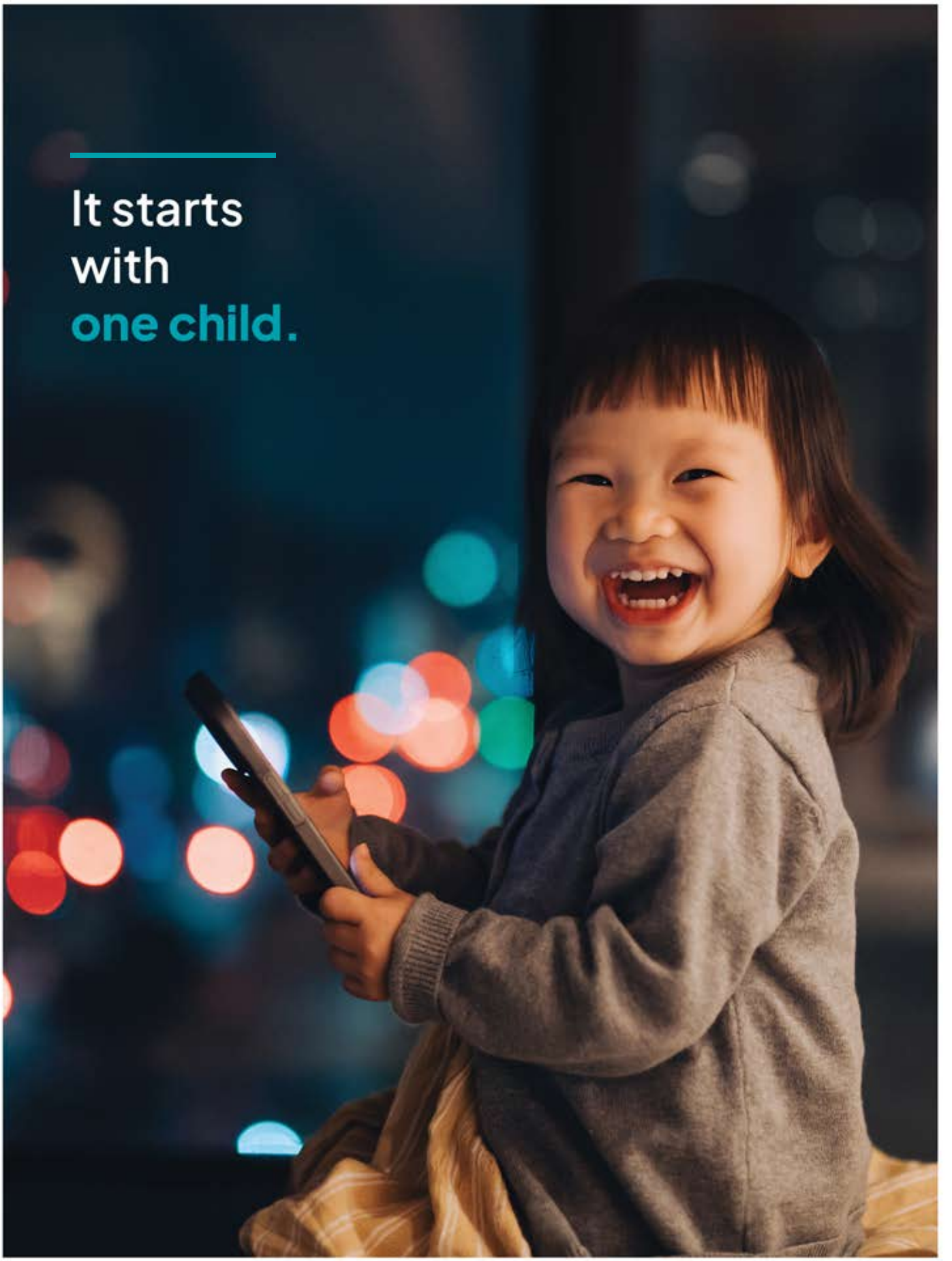
RESEARCH DAY 2025

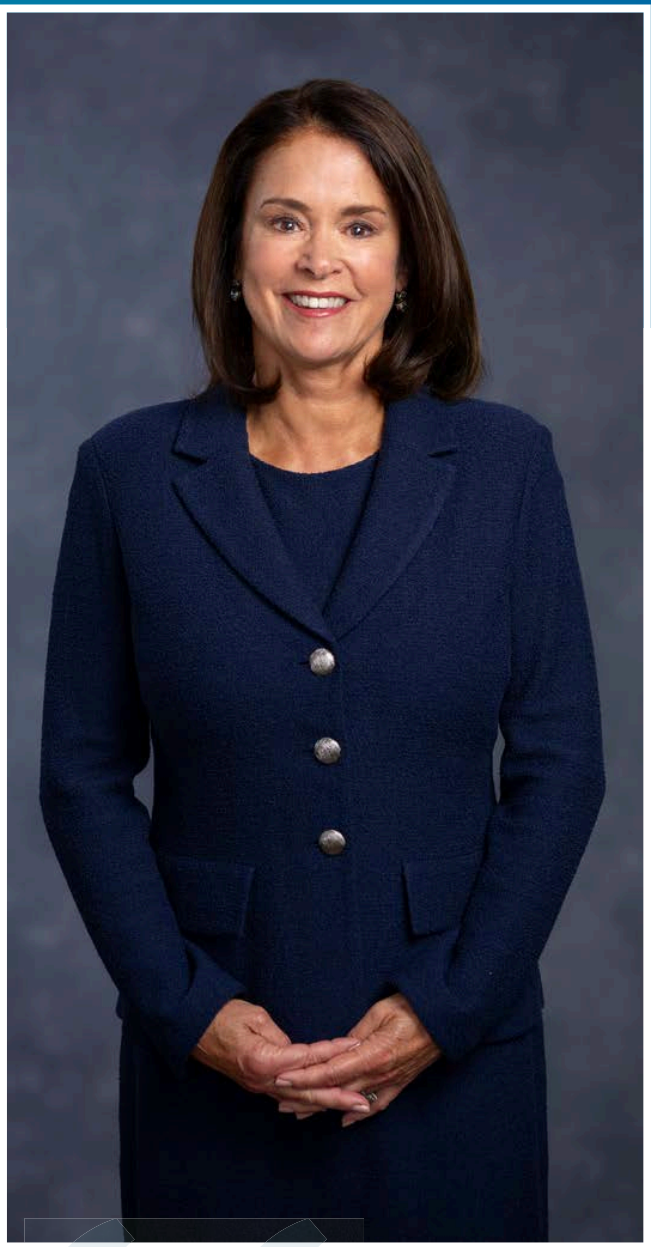
WED, NOV. 19



CHOC Research
GO BEYOND.

RESEARCH DAY 2025

from *the* CEO



Kimberly Chavalas Cripe

Co-President & Chief Executive Officer
Rady Children's Health

2025 has been a year of remarkable transformation and growth at Rady Children's Health. In January, the parent organizations of Children's Hospital of Orange County and Rady Children's Hospital, San Diego came together to form a combined health system, creating new opportunities to advance care, discovery, and hope for the children we serve. In June, we celebrated another milestone with the opening of the nine-story, 330,000-square-foot Southwest Tower on the Rady Children's Orange County campus. This state-of-the-art facility dedicates an entire 29,000-square-foot floor to the Research Institute, dramatically expanding our capacity to conduct clinical, basic, and translational research.

At the heart of all we do is a simple, powerful idea: it starts with one child. Every question we ask, every study we undertake, and every breakthrough we pursue begins with a single patient—the child whose story sparks curiosity, inspires discovery, and drives progress. Today, as you explore the outstanding posters and presentations, we invite you to remember that each discovery reflects the courage, resilience, and hope of one child and one family.

It is that child—the one who challenges us, teaches us, and motivates us to ask "why"—who reminds us of our purpose: to turn questions into answers, challenges into solutions, and hope into reality. Thank you for joining us to celebrate a year of discovery, dedication, and research that begins with one child, yet has the power to touch the lives of many.

> *"Every question we ask, every study we undertake, and every breakthrough we pursue begins with a single patient– the child whose story sparks curiosity, inspires discovery, and drives progress."*

At the
CHOC Research Institute

166 Employees 

24 Affiliated Staff 

44 Visiting Scientists 

142 Research Interns 

102 CHOC Staff & Physicians w/UCI Faculty Appointments 

3,440 CHOC Patients Enrolled in Research 

655 Active Studies 

278 Active Principal Investigators 

FY2025

A vibrant community of dedicated scientists, physicians, and trainees is pushing the boundaries of pediatric discovery to improve care for children everywhere. Through hundreds of active studies and thousands of published findings, our work is translating into real-world impact for patients and families. By nurturing the next generation of innovators and collaborating across institutions, we're building a healthier future for every child.

CHOC Research
GO BEYOND

from *the* CSO

Terence Sanger, MD, PhD

Vice President, Chief Scientific Officer, *CHOC*
Professor of Electrical Engineering & Computer Science, *UCI*
Dept. Pediatrics Vice Chair for Research, *UCI School of Medicine*
Child Neurology & Movement Disorders, *CHOC*

This year's theme, "It Starts with One Child," reminds us that every question, discovery, and collaboration begins with a single patient. At Rady Children's Health in Orange County, the CHOC Research Institute is guided by patient-centered discovery, where we are committed to turning questions into answers—and answers into hope—for one child today and countless children tomorrow.

The Institute stands at a pivotal moment in its evolution, building on five years of infrastructure development to expand its vision and establish world leadership in pediatric research. Our new FY26–FY30 Strategic Plan, "The Path Forward," sets a bold course for the next five years. It outlines a tactical approach that integrates recruitment excellence, education and mentorship, innovative partnerships, operational efficiency, and sustainable growth to create transformative impact. Each priority amplifies our capacity to conduct groundbreaking research while maintaining operational excellence, regulatory compliance, and the highest ethical standards.

Hospitals like ours have a unique responsibility in shaping the future of pediatric healthcare. The most important questions and ideas come from the children we serve, their families, and the clinicians who care for them. Basic, translational, and clinical science are essential to turn these questions into answers, feeding directly into clinical care and offering new treatments and cures. Research is a service to our patients. We MUST innovate—our patients, now and in the future, depend on our research.

Today, Research Day is an opportunity to celebrate the innovation, dedication, and discoveries of our researchers. As you explore presentations, posters, and discussions, let this day spark new ideas, foster collaboration, and inspire us to Go Beyond in our pursuit of answers for every child.

> *"The most important questions and ideas come from the children we serve, their families, and the clinicians who care for them."*

AGENDA

Morning Sessions	**Wade Education Center, CHOC West**
08:00 am	Opening Remarks & Keynote Address
09:15 am	Panel 1 | **The Spark of One: How a Single Patient Inspires Innovation**
10:45 am	Panel 2 | **From Genes to Care: Interventional Genomics in Pediatric Medicine**
Afternoon Sessions	**9th floor, Southwest Tower**
12:00 pm	Poster Session
02:00 pm	Welcome Address
02:25 pm	Podium Poster Presentations
03:50 pm	Awards Ceremony & Closing Remarks
04:30 pm	Networking Reception

Kids Activities

In addition to the above programming, we have a number of kids activities planned throughout the day to help growing and inquisitive young minds better understand research and science! In the morning, mini lectures will presented through the Seacrest Studios; kids who are able will be invited to join and engage with our presenters in studio. In the afternoon, kids will be given the opportunity to learn some new skills that our researchers and scientists use each day, including pipetting skills, observation, and measuring and mixing items to create something new.

Table of Contents

Morning Sessions

Opening Remarks | **Welcome and Introduction**

Coleen Cunningham, MD
Senior Vice President & Pediatrician-in-Chief, Rady Children's Health in Orange County
Professor & Chair of Pediatrics, University of California, Irvine

Keynote Address | **Seeing the Unseen: Unraveling Blind Spots in Clinical Genetic Testing**

Seth I. Berger, MD, PhD
Translational Genomics Director for Rare Disease,
Research & Development, Ambry Genetics

Seth I. Berger, MD PhD is the Translational Genomics Director for Rare Diseases in Research and Development at Ambry Genetics. Dr. Berger is board certified in Pediatrics, Medical Genetics, and Medical Biochemical Genetics.

Prior to joining Ambry in 2025, he was a principal investigator in the Children's National Center for Genetic Medicine Research, an attending physician in the Children's National Rare Disease Institute, and a tenured Associate Professor in the George Washington University Departments of Genomics and Precision Medicine, Pediatrics, and Biochemistry and Molecular Medicine. In his current role, he works to assess new technologies and informatic pipelines for molecular diagnostics of Rare Disease.

He currently focuses on integration of long read genomic data with transcriptomic datasets with novel bioinformatic approaches. He also works closely with the University of California, Irvine - Genomics Research to Elucidate the Genetics of Rare Disease (GREGoR) research center.

Panel Descriptions

Panel 1 | The Spark of One: How a Single Patient Inspires Innovation

Moderated by: **Mustafa Kabeer, MD**
Pediatric General Surgeon, Rady Children's Health in Orange County
Clinical Professor of Surgery, University of California, Irvine

Every research breakthrough begins with a single question—and often, a single patient. In this panel, leading scientists and innovators will share the stories of the children who inspired their work, illustrating how one child's journey can spark discoveries that transform pediatric medicine. Attendees will hear firsthand how curiosity, compassion, and collaboration turn bedside experiences into life-changing research and innovation.

Panel 2 | From Genes to Care: Interventional Genomics in Pediatric Medicine

Moderated by: **Raymond Wang, MD**
Director, Campbell Foundation Multidisciplinary Lysosomal Disorder Program
Division of Metabolic Disorders, Rady Children's Health in Orange County
Associate Clinical Professor of Pediatrics, University of California, Irvine

This panel explores the cutting-edge field of interventional genomics, highlighting how scientists and clinicians are translating discoveries about the human genome into targeted treatments for children. Panelists will discuss innovative therapies, precision medicine approaches, and the challenges and opportunities of bringing genomic insights from the lab to the bedside. Attendees will gain insight into how genomics is transforming pediatric care and shaping the future of personalized medicine.

Shaping Tomorrow's Researchers

Pediatric Fellowship Research Core Curriculum

Pediatric Fellows Hasmik Verdyan, Channing Greenburg & Sean Dornbush

Modern medicine does not always have the clear-cut answers needed by our patients. When this happens, research becomes an alternative avenue. However, to find the answers each child needs to thrive, clinicians must first be able to launch their inquiry so the resulting answers are both meaningful and timely.

In mid-2025, the Research Institute, in partnership with Academic Affairs, redesigned research training for University of California Irvine-affiliated pediatric fellowship programs. to ensure that all fellows received consistent, foundational research training during their advanced clinical education at CHOC, now part of Rady Children's Health.

The resulting curriculum, developed with input from clinical fellows and a committee of fellowship program leaders, was designed with three guiding goals in mind: (1) Provide structured research training to all clinical fellows in two-and-three-year pediatric fellowship training programs; (2) Integrate Research Institute resources, infrastructure, and mentorship into fellowship education; and (3) Align with the American Board of Pediatrics (ABP) scholarly activity requirements.

While not every fellow plans to be a physician-scientist, each is expected to develop skills to conduct hypothesis-driven scientific inquiry that contributes to medical literature and patient care. Many ABP subspecialty certifications also require a written scholarly product by the end of fellowship.

To meet the priorities of standardization and efficiency, Dr. Virginia (Ginny) Muscatello, Manager of Research Education, supported by Keri Zabokrtsky, Manager of Research Programs, developed a fellowship research core curriculum featuring a two-day "Research Bootcamp" (five hours per day). Held early in fellowship, this bootcamp gives fellows the opportunity to identify their area of inquiry or interest, formulate a hypothesis or specific aim, and outline a research protocol. They also begin developing a project management plan in collaboration with subject matter experts, ensuring they are well prepared to carry their projects forward successfully.

The first day of research bootcamp, held in September, was themed around Laying the Foundation, where core concepts in research design and infrastructure were the focus. Topics included an overview of Research Institute resources, crafting a clear and testable research question, research design and methodology, and research ethics and compliance.

The second day of research bootcamp was themed around Applying the Tools, building on knowledge gained previously and focused on the practical skills needed to support project execution. Topics included biostatistics, data science and study feasibility, research tools and technology, scientific writing and case reports, study timeline, feasibility, and Scholarly Oversight Committee expectations.

By introducing fellows to research concepts and tools early in their training, the curriculum is designed to spark curiosity, build confidence, and lay the groundwork for projects that can grow over the course of fellowship.

One person alone may not change the course of pediatric and adolescent health care. However, one curious clinician equipped with the tools to execute high-quality research in partnership with their clinical program team and the Research Institute can absolutely Go Beyond and profoundly impact the care we provide to each child who comes through our doors.

> *"One curious clinician equipped with the right tools can profoundly impact the care we provide to each child who comes through our doors."*

A Message from A Mother

My Son, Sebastian Guerra

Sebastian was born on January 17, 2014, and from the beginning, he had a spark that lit up every room. A natural conversationalist who loved connecting with people. His laugh was contagious, his smile genuine, and his curiosity about the world endless.

He loved life's little joys. He enjoyed learning about U.S. Presidents, a hobby most 10-year-olds wouldn't think twice about. He loved breakfast, dancing, taekwondo, playing Fortnite and Minecraft with friends, telling silly jokes, and dreaming big —one dream was to become a famous YouTuber. He even made his own business cards, because when Sebastian believed in something, he went all in.

His favorite color was green, which now feels like his signature — a symbol of his life, energy, and the love we carry for him.

On January 17, 2024 — his 10th birthday our world changed when Sebastian was diagnosed with diffuse midline glioma, a rare and aggressive pediatric brain tumor. There is no cure, and treatments are limited. From that moment, our mission became clear: fight alongside Sebastian, make memories, and share his story so one day no family will hear the words, "there's nothing we can do."

Sebastian showed extraordinary courage. He faced treatments, hospital visits, and uncertainty with strength that inspired everyone around him. He donated his tumors to research to help find a cure for the children who come next. That was Sebastian — thinking of others, always. Because what families like mine need isn't just comfort, we need breakthroughs.

He joined Dr. Ashley Plant-Fox's DIPG vaccine trial through the CHOC Research Institute, now part of Rady Children's Health. That trial gave us something every family in this fight hopes for: time. Time to live out his dreams. He traveled to France and saw the Eiffel Tower and the Mona Lisa. Thanks to Make-A-Wish, he visited Washington, D.C., where he toured the White House. He loved history, and those trips meant the world to him — and to us.

Rady Children's Health in Orange County became more than a medical center — it became a place of compassion, hope, and dedication. The doctors, nurses, and research teams treated Sebastian not just as a patient, but as the vibrant boy he was. They saw his humor, dreams, and his determination, and they fought for him with the same devotion we did. Sebastian even appears in a Research Institute video, proudly lending his voice and face to raise awareness for work that could save children's lives.

Sebastian Guerra & Katie Mosier

Pediatric brain tumor research is severely underfunded yet urgently needed. Research offers hope — for better treatments, improved quality of life, and cures. Rady Children's Health commitment to advancing pediatric cancer research matters because it gives families like ours something to hold on to. Every clinical trial, every breakthrough, and every step forward changes the future for children diagnosed with brain tumors.

On February 6, 2025, after a brave and deeply fought battle, Sebastian passed away. He was only 11, but in those years, he touched more lives than some do in a lifetime. His absence is felt every day, yet so is his presence — in everything green, in the songs that remind us of him. We believe he sends us signs to let us know he's near, encouraging us to keep going, to have unshakable faith, and fight for change.

Being honored as this year's Research Institute Mission Moment is more than recognition — it's a promise to carry his legacy forward. His story is a call to action. A reminder that every child deserves the chance to grow up, to dream, to chase their passions.

We are grateful to Rady Children's Health and the CHOC Research Institute for honoring our son, for fighting alongside us, and for their relentless pursuit of breakthroughs in pediatric brain tumor research. Sebastian's life may have been short, but it was powerful. And his impact will continue —in every donation to research, in every family supported, and in every step toward a cure.

We love you, Sebastian. Always and forever, you are our light, our inspiration, and our mission.

Raymond Wang, MD., & Harriet Chang, Clinical Research Coordinator II, PhD

Brianna Leyden, MD

Virginia Allhusen, Manager, Health Sciences Administration, PhD
& Aaron Yengo-Kahn, MD

Jennifer Hayakawa, Director of Nursing Research & Innovation, DNP, CNS, CNRN, CCRN-K, Paulina Schuhler, Clinical Research Coordinator I, MSN, RN, CCRN,
& Rebecca DeAnda, Coordinator, Special Projects

Celina Padilla, Research Intern & Lia Galut, Clinical Research Coordinator Assistant

RESEARCH DAY 2024

Brent Dethlefs, Former Executive Director, Research Institute, John Crawford, MD, MS, Ashish Chogle, MD, & Phuong Dao, Executive Director, Research Institute

Breaking New Ground

The expansion of the Research Institute into a state of the art space on the ninth floor of the Southwest Tower marks a defining moment for pediatric research at Rady Children's Health. Spanning nearly 29,000 square feet—about half a football field—the facility is more than an expansion of space. It is a catalyst for discovery, designed to translate bold ideas into better outcomes for children.

Formed in 2003, the Institute has grown into a dynamic engine of innovation, advancing studies across nearly every pediatric subspecialty. The new space builds on that momentum, providing state-of-the-art infrastructure to support breakthroughs in human performance, translational science, biomedical engineering, and patient-centered clinical trials.

To help share this vision, the Institute has launched the Research Institute brand video, which offers a closer look at the mission driving our work. You can scan the QR code on this page to watch and learn how research is shaping the future of children's health.

With this milestone, the Institute is poised to tackle the next generation of questions—answering them with the rigor, creativity, and compassion that define our mission.

Brent Dethlefs, Former Executive Director, Research Institute,
Phuong Dao, Executive Director, Research Institute, Dr. Terence Sanger, CSO

Center for Clinical Research

The Center for Clinical Research (CCR) is the first facility of its kind in Orange County, offering a dedicated, licensed multispecialty space that fully integrates pediatric research into the hospital's clinical environment. By combining research excellence with the hospital's highest standards of clinical care, the CCR provides investigators with the infrastructure to conduct complex and diverse studies while ensuring every interaction meets rigorous standards of quality and compliance. For children and families, this seamless integration makes participating in research more convenient and accessible than ever, creating a professional, family-centered setting where innovation, safety, and compassion come together to advance discoveries that directly improve child health.

The center features multiple exam and consultation rooms, as well as a nearly 200-square-foot observation room uniquely designed to support clinical research. Equipped for both real-time monitoring through a one-way mirror and asynchronous review via secure video recording, this space allows researchers to carefully observe participants without disrupting the clinical setting. This versatility makes it ideal for a wide range of studies—from neurological and behavioral assessments to emerging therapeutics—where precise observation and accurate data collection are essential.

By expanding both capacity and capability, the CCR strengthens CHOC's position as a regional leader in pediatric research—offering the resources to serve more families, support more studies, and generate discoveries that can shape the future of care.

Human Performance Lab

The Human Performance Lab is one of the most advanced resources in the new tower, uniquely combining a gait and motion lab with a pediatric exercise lab to study how children move, recover, and adapt after illness or injury. The lab allows researchers to measure strength, endurance, balance, coordination and cardiopulmonary function, providing a comprehensive understanding of how treatments and therapies affect a child's daily life.

In the lab, children may walk or run on treadmills, climb stairs, or engage in targeted balance and strength exercises, all while advanced equipment monitors muscles, joints, heart, and lung activity in real time. A specialized safety harness prevents falls, while a sensor-lined walkway maps each step with precision, capturing detailed gait and motion patterns. For a child with cerebral palsy relearning how to walk or a teenager recovering from orthopedic surgery, these safe tools provide detailed insights that help researcher refine and personalize therapy.

What makes the lab unique is not just its technology but its adaptability. The space can shift to allow investigators to study a single child's rehab journey and also support large-scale clinical trials on new devices or therapies. This versatility ensures that discoveries aren't confined to theory -- they are evaluated, refined, and applied in real-world conditions.

Ultimately, the Human Performance Lab is about more than data. It's a place of innovation and promise, pioneering therapies that give children and their families renewed hope and the tools to thrive.

Basic & Translational Science

Two-thirds of the ninth floor is dedicated to the Basic and Translational Science Lab, where discoveries move from ideas at the lab bench to therapies that can change children's lives. With 46 wet benches and shared resources for cell analysis, imaging, and molecular testing, the lab gives scientists the tools they need to tackle some of the toughest questions in pediatric health.

Of particular importance are two pharmaceutical-grade clean rooms, which make it possible to safely develop emerging treatments such as cell and gene therapies. These therapies hold promise for conditions once thought untreatable—rare genetic diseases, immune deficiencies, and certain cancers. Having this capacity in house positions the Research Institute as a leader in bringing cutting-edge trials directly to children.

Shih-hsin Kan, PhD, Scientist II

But perhaps the most powerful feature is the lab's collaborative design. Instead of closed-off cubicles, the space is open and interconnected, encouraging scientists from oncology, neuroscience, cardiology, immunology, and more to work side by side. High-demand equipment is centralized, ensuring efficient use and sparking collaboration across specialties.

This design mirrors the reality of children's health: no disease exists in isolation. A child with a heart condition may also face immune complications; a young cancer patient may develop neurological side effects. By encouraging scientists to share ideas across disciplines, the lab ensures discoveries move more quickly from the "what if" stage to therapies that reach children's bedsides.

For families, this means hope. It means that a diagnosis once considered a dead end may now have a new pathway forward, fueled by collaboration and relentless pursuit of solutions. By emphasizing translational work, it ensures that discoveries move rapidly from bench to bedside, impacting patient care in real time.

Maker Space

The Maker Space is a distinctive addition to the Institute, offering a hub where clinicians, engineers, and scientists can transform ideas into tangible solutions. Equipped with 3D printers, rapid prototyping tools, and collaborative workstations, it enables the development of devices and technologies tailored specifically to pediatric needs—from adaptive rehabilitation tools to custom surgical instruments.

Beyond innovation, the Maker Space serves as a training ground for students and early career researchers. By engaging directly in device design and prototype development, they gain valuable experience that prepares them to navigate the complex pathways of biomedical engineering and medical innovation. In this way, the Maker Space not only advances discovery but also nurtures the next generation of problem solvers.

Looking Forward

The new research floor puts children at the center of everything we do, transforming discovery into care. Here, guided by the Research Institute's Strategic Plan, every study and innovation serves our patients and healthcare teams, advancing pediatric research with focus, collaboration, and impact. This space is not just breaking new ground—**it's transforming the future of care for children everywhere.**

PODIUM PRESENTATIONS

2:25-3:45PM

Moderator: Carol Davis-Dao PhD

Title	Speaker
Opening	Carol Davis-Dao
Reduction of Post-Operative Antibiotic Utilization in the NICU	Sonia A Mehta
Day-to-day cognitive variability in executive function but not hippocampal dependent memory in youth with obstructive sleep apnea compared to control participants	Katharine C. Simon
Increasing Severity of Pediatric Patellar Fractures from E-Bike/E-Scooter Injuries: A Shift Toward More Advanced Fixation Techniques	Steven Halvorson
Impaired bioenergetics in FBXL4-deficient fibroblasts is ameliorated by aminolevulinate plus iron	Uyen Vo
Population Pharmacokinetics and Safety of Continuous Oxacillin in Preterm and Term Neonates and Infants	Adam Lee
Investigating choroid plexus organogenesis using an organoid model	Celine Thao-Quyen Tran
Utilization of Electroacupuncture to Manage Neuropsychiatric Symptoms Among Adolescent and Young Adult Cancer Patients and Survivors	Matthew Heshmatipour
Retrospective Application of Genome-Based Newborn Screening Platforms Identifies Large Number of Diagnoses in Critically Ill Children	Erica Sanford Kobayashi
Closing Remark	Carol Davis-Dao

SPEAKER PROFILE

Sonia Mehta, MD, is a third-year Neonatal-Perinatal Medicine fellow at UC Irvine/CHOC. Her clinical and research interests focus on optimizing perioperative care and outcomes for surgical neonates.

Katharine Simon, PhD, is an Assistant Professor in the Department of Pediatrics, School of Medicine, at UC Irvine and the Assistant Director of Research in Pediatric Sleep at the Children's Hospital of Orange County. She is a licensed clinical psychologist specializing in pediatric health and behavioral sleep medicine. Dr. Simon's current research investigates the mechanisms underlying sleep, memory, and mental health across development and in pediatric patient populations. Her work is supported by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) and the Children's Hospital of Orange County.

Steven Halvorson, DO*,* is a third-year orthopedic surgery resident at Riverside University Health System with a strong interest in pediatrics. He intends to pursue a fellowship in pediatric orthopedic surgery.

Uyen Vo, MS, is a Laboratory Technician in the Laboratory of Energy Metabolism (E-Lab) at Rady Children's Health. She holds a master's degree in Biochemistry and has a strong interest in cellular metabolism research. In her current role, she conducts and optimizes biochemical analyses to investigate mitochondrial function and metabolic pathways associated with rare mitochondrial disorders, aiming to identify potential therapeutic strategies.

Adam Lee, MD, is a recent fellowship graduate who is now a full-time physician at CHOC. His research interests include pharmacokinetics and pharmacodynamics in pediatric patients, particularly to determine appropriate dosing of antimicrobial agents in children.

Celine Tran is a Laboratory Technician in CHOC's Hydrocephalus Lab. Her work involves organoid culture, characterization of choroid plexus organogenesis, and investigation of inflammatory mechanisms underlying the etiology of acquired hydrocephalus.

Matthew Hemshmatipour, MPH (candidate), is a Clinical Research Coordinator and Master of Public Health student at the University of California, Irvine. Since 2022, he has worked in the Department of Clinical Pharmacy Practice, managing clinical studies aimed at improving health outcomes for cancer populations, including adolescent and young adult (AYA) cancer patients and survivors. To further his skills in developing effective interventions for the AYA cancer population, he began pursuing his MPH degree in 2024, concurrently with his research coordination work.

Dr. Sanford-Kobayashi is an Assistant Professor and Pediatric Intensivist at CHOC, as well as a Clinical Research Physician at Rady Children's Institute for Genomic Medicine.

ABSTRACTS FOR PODIUM PRESENTATIONS

PODIUM PRESENTATION #1

Reduction of Post-Operative Antibiotic Utilization in the NICU

Sonia A Mehta MD[1,2]; Kaley Haymond MSN, RNC NIC[1]; Tina Lee PharmD[1], Ayan Rajgarhia MD[1,2]

[1]Neonatology Division, Children's Hospital of Orange County, Orange, CA
[2]Neonatology Division, Department of Pediatrics, University of California Irvine, Irvine, CA

Background: Antibiotics are the most frequently used drugs in the NICU, but exposure carries risks including drug toxicities, prolonged intravenous access, altered microbiota, and antimicrobial resistance. Significant variation exists in perioperative antibiotic use and prophylaxis, creating opportunities for stewardship.

Aim: Prior recommendations were fragmented across procedure-specific guidelines and often lacked clarity. A comprehensive literature review and evaluation of clinical guidelines from other leading children's hospitals, as well as Enhanced Recovery After Surgery (ERAS) recommendations, informed revisions.

Methods: The team reviewed existing post-operative antibiotic guidelines in the CHOC NICU, a level IV unit with 500 neonatal surgeries per year. Multidisciplinary discussions with neonatology, pediatric surgery, infectious disease, and pharmacology resulted in a single, cohesive document specifying antibiotic choice and duration for each procedure (Image 1). A fishbone diagram (Image 2) was also created to identify drivers of inappropriate use and guide project aims.

Results: A retrospective review of 149 infants who received post-operative antibiotics for abdominal surgeries (January 2022–September 2024) showed overutilization in 60% (90/149) of cases. Most instances (73%, 66/90) were due to one to two unnecessary additional doses, often related to absent automatic stop dates in order sets. Seventeen cases involved prolonged antibiotic duration, ranging from 3–9 days.

Conclusions: This ongoing quality improvement initiative aims to reduce antibiotic overutilization by implementing revised guidelines. Planned PDSA cycles target a 15% increase in compliance per cycle, with an ultimate goal of >85% adherence. Key interventions include: (1) education of essential personnel, (2) integration of revised recommendations into perioperative checklists, and (3) addition of automatic stop dates to order sets in the electronic medical record. Our global aim is to optimize antimicrobial stewardship in the NICU and minimize unnecessary antibiotic exposure.

PODIUM PRESENTATION #2

Day-to-day cognitive variability in executive function but not hippocampal dependent memory in youth with obstructive sleep apnea compared to control participants

Katharine C. Simon, Tricia Morphew, Shun Iwata, Lia Galut, Spencer Polk, Lois Sayrs, & Neal Nakra
University of California, Irvine & Children's Hospital of Orange County

Introduction: Obstructive sleep apnea affects 9.5% of youth leads to behavior dysregulation, memory deficits, and emotional instability. Adenotonsillectomy is a common surgical treatment that improves sleep-disordered-breathing symptoms, health, and cognition. However, most studies exploring OSA's impact on cognition are obtained with cross-sectional studies. How surgery impacts day-to-day cognition in youth is unknown at short and long-term intervals. Currently, we are repeatedly and remotely tracking pediatric patients before and after surgery and healthy age/sex matched controls.

Methods: Using a measurement burst design, we tracked patients over nine months: before and twice post-surgery (controls are time matched). Eight pediatric patients (Mage=11.375, SD=1.4. F=4) and eight age/sex matched controls are enrolled. Our mobile health platform, HowRU, administers twice-daily cognitive tasks (hippocampal-dependent word pair associates and executive function operation span at easy and difficult levels), along with sleep diaries and mood assessments. Participants also use Muse and Garmin wearables to track sleep neurophysiology and physical activity. Baseline data are analyzed using a linear regression within the GLMM framework, accounting for the multilevel structure of the data, to examine daily variability in cognitive performance.

Results: Cognitive performance patterns in the baseline period did not significantly differ between patients and controls. For executive function, significant learning effects were observed, improving across difficulty levels after the first day. We found an interaction between easy performance and sleep, with greater sleep duration negatively impacting performance. Neither group showed a circadian-effect on performance. For hippocampal-dependent memory, neither group exhibited learning effects or day-to-day performance variability. However, both groups demonstrated sleep-dependent consolidation, with no significant overnight decline from encoding.

Conclusions: Day-to-day analyses reveals distinct performance patterns in executive function but not hippocampal-dependent tasks. Executive function learning effects were observed. This discrepancy in performance may stem from the heightened sensitivity of executive function to fragmented sleep, whereas hippocampal-dependent memory appears less affected, provided sufficient sleep is obtained. Our preliminary findings are promising, suggesting a nuanced understanding of the impact of fragmented sleep in youth. These findings suggest that youth with OSA experience subtle cognitive disruptions linked to sleep fragmentation, underscoring the need for targeted interventions.

Funding: Children's Hospital of Orange County, KS funded by NIH K08 XXX

PODIUM PRESENTATION #3

Increasing Severity of Pediatric Patellar Fractures from E-Bike/E-Scooter Injuries: A Shift Toward More Advanced Fixation Techniques

Steven Halvorson, DO[1]; Sallie Canumay, DO[1]; Tiffany Lubrino MS[2]; Katrina Belilovets[2]; Blake Han, BA[1]; John Schlechter, DO[1,2,3]

[1]Department of Orthopedic Surgery, Riverside University Health System, Moreno Valley, CA
[2]Orthopaedic Institute, Children's Hospital of Orange County, Orange, CA
[3]Pediatric Orthopaedic Specialists of Orange County, Orange, CA

Background: Electric motorized bicycles and scooters (EB/ES) are increasingly used by children and adolescents, contributing to a rise in high-energy pediatric trauma. Compared to traditional bicycles, e-bike injuries are associated with greater severity, including complex orthopedic trauma.

Purpose/Aim(s): This study aims to portray the increasing severity of fractures resulting from EB/ES injuries in the pediatric population.

Methods: A retrospective chart review was conducted on patients aged ≤17 years who underwent open reduction and internal fixation (ORIF) for patellar fractures from January 2015 to March 2025. Data collection included demographics, mechanism of injury, fracture severity, open vs closed injuries, type of fixation, and length of surgery. Imaging was reviewed and fracture classification applied. EB/ES injuries were identified from clinical documentation and compared to all other mechanisms to assess variation in fracture pattern and treatment.

Results: Twenty children underwent ORIF for patellar fractures, with 15 (75%) classified as AO/OTA 34-A1 and 5 (25%) as comminuted (34-C1 or 34-C3). Three injuries (15%) were EB/ES related; all resulted in severely comminuted fractures. Two of these required locking plate fixation, compared to none in the non-e-bike group. One of the three (33%) EB/ES injuries resulted in an open fracture, compared to one of the 17 (5.8%) non-EB/ES injuries. EB/ES patients were older (mean 13.7 vs. 11.3 years) and had longer operative times, indicating increased injury severity and surgical complexity.

Conclusions: Pediatric EB/ES-related patellar fractures presented as more severe and comminuted, and underwent more complex fixation with locking plates and longer operative times compared to lower-energy injuries. These findings highlight evolving injury patterns related to the pediatric patella linked to EB/ES devices and underscore the need for heightened clinical awareness, implant availability and targeted injury prevention strategies.

PODIUM PRESENTATION #4

Impaired bioenergetics in *FBXL4*-deficient fibroblasts is ameliorated by aminolevulinate plus iron

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Background: F-box and leucine-rich repeat protein 4 (*FBXL4*) deficiency is a rare mitochondrial disorder characterized by decreased mitochondrial DNA (mtDNA) content and increased BNIP3/BNIP3L receptor-mediated mitophagy. Clinical features include severe neurodevelopmental delay, encephalopathy, hypotonia, and lactic acidosis. Only supportive treatments are available, and most affected individuals die in infancy. We previously demonstrated that aminolevulinate plus iron (ALA/Fe) restore mtDNA levels, improve bioenergetics, and rescue the antioxidant system in DARS2-deficient fibroblasts [1]. Here, we investigated whether a similar approach could benefit *FBXL4* deficiency**.**

Aims: (1) To characterize the bioenergetics of fibroblasts obtained from three individuals harboring different *FBXL4* pathogenic variants; (2) To assess the therapeutic effects of ALA/Fe.

Methods: The bioenergetic status was investigated before and after 14-day ALA/Fe (100/50 μM) exposure. Oxygen consumption rate (OCR) was measured using Resipher (Lucid Scientific, USA) and Seahorse XFe96 real-time bioanalyzers (Agilent, USA). Lactate production was measured by colorimetry (Pointe, USA), mtDNA content by qPCR, and the activities of mitochondrial complexes I and IV by spectrophotometry. Cellular antioxidant system was assessed by measuring heme oxygenase-1 (HO-1) expression by qPCR, and glutathione levels by spectrophotometry. Studies were performed under CHOC IRB protocol #130990.

Results: *FBXL4*-deficient fibroblasts showed significantly reduced basal, maximal OCR, and ATP synthesis efficiency in basal conditions. These cell lines exhibited impaired activities of complexes I and IV, increased lactate production, increased BNIP3 and BNIP3L, and reduced aconitase levels. A 14-day ALA/Fe exposure significantly improved OCR, mtDNA content, and mitochondrial protein levels. Concomitantly, there was an increase in HO-1 levels and glutathione levels denoting enhanced cytoprotection.

Conclusions: Fibroblasts derived from individuals with *FBXL4* deficiency presented impaired bioenergetics. ALA/Fe exposure significantly improved the deficient energy metabolism observed in *FBXL4* deficiency and enhanced cytoprotective pathways. Thus, repurposing already FDA-approved drugs can help address the urgent need for treatments in this devasting disorder.

[1] Huang et al. Aminolevulinate/iron exposure elicited Nrf-2-mediated cytoprotection in DARS2-deficient fibroblasts with impaired energy and antioxidant metabolisms. *BBA - Molecular Basis of Disease* (2025). doi.org/10.1016/j.bbadis.2025.167824

PODIUM PRESENTATION #5

Population Pharmacokinetics and Safety of Continuous Oxacillin in Preterm and Term Neonates and Infants

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Background and Objectives: *Staphylococcus aureus* increasingly causes late onset neonatal sepsis. Prompt effective therapy is crucial, as mortality approaches 25%. Oxacillin is standard of care for methicillin-susceptible *Staphylococcus aureus* (MSSA) infections. However, no previous studies exist to inform on dosing for neonates and infants under 90 days of age. Currently, 100% free drug time > minimum inhibitory concentration (*f*T > MIC) is recommended for treatment of sepsis in immunocompromised hosts with beta-lactam antibiotics. Our objective was to characterize the pharmacokinetics (PK) and pharmacodynamics (PD) of oxacillin in this age group.

Methods: We conducted a prospective, open label, single-center PK/PD study of oxacillin in infants ≤ 90 days old. Patients were divided into 4 cohorts per gestational (GA < 32 or ≥32 weeks) and postnatal age (PNA < 14 or ≥14 days). Patients received a loading dose of 25 mg/kg followed immediately by continuous infusion of 160 mg/kg/day (120 mg/kg/day for GA < 32 weeks and PNA < 14 days). Oxacillin plasma levels were obtained at specified times on continuous infusion. Population PK parameters and variances were estimated using NONMEM. Multiple dosing regimens were evaluated using Monte Carlo simulations to estimate probability of target attainment (PTA) of the PD target *f*T 100% > MIC using CLSI breakpoint MIC 2 mcg/ml. Outcomes were collected.

Results: We analyzed 89 plasma samples from 22 infants, median (IQR) GA: 38 weeks (30 to 39), PNA 32 days (12 to 46). PNA and weight were covariates for oxacillin clearance. Assuming 90% protein binding, unbound oxacillin concentrations at steady state remained > MIC 0.5 mcg/ml throughout the dosing interval for >90% of modeled patients on continuous oxacillin infusions of 160 mg/kg/day. This dosing also achieved PD targets up to MIC 1 mcg/ml for infants up to 28 days of life. Modeled infants > 7 days old regardless of GA receiving oxacillin up to 200 mg/kg/day divided every 4h or 6h failed to consistently meet PD targets for any MIC ranging 0.25 to 2 mcg/ml. All patients survived to discharge. Few serious adverse events were noted but none related to oxacillin administration; oxacillin in general was found to be safe.

Conclusions: Oxacillin 160 mg/kg/day as a continuous infusion was well tolerated, which reliably achieved PD targets for MIC ≤ 0.5 mcg/ml (MIC 1 for infants 28 days and younger). Modeled intermittent dosing ≤ 200 mg/kg/day divided q4h or q6h, up to eight times the Food and Drug Administration (FDA) labeled dose of 25 mg/kg once daily, failed to achieve PD targets. This study demonstrates that the dose of oxacillin on the FDA label is woefully inadequate and may help explain the high mortality of serious MSSA infections in this age group.

PODIUM PRESENTATION #6

Investigating choroid plexus organogenesis using an organoid model

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BACKGROUND: Organoids serve as a powerful tool for *in vitro* modeling of human organ development and disease, offering three-dimensional, tractable systems. In 2022, the first commercialized choroid plexus organoid (ChPO) kit was released, providing a novel platform for hydrocephalus research. However, the developmental trajectory and maturation of ChPOs remain poorly understood.

PURPOSE/AIM(S): This study aims to characterize ChPO formation and maturation over time and compare it with human choroid plexus (ChP) development.

METHODS: ChPOs were generated using a commercial kit, following the manufacturer's protocol. Organoids were harvested on days 22, 32, 42, 52, and 62 for immunofluorescence staining. Markers of interest included transthyretin, aquaporin-1 (AQP1), claudin-1, forkhead box protein J1 (FOXJ1), paired box protein 6, t-box brain protein 2, and glucose transporter type 1 (GLUT1).

RESULTS: Immunofluorescence analysis revealed that early-stage ChPOs exhibited pseudostratified epithelial architecture at the embryoid periphery, with bipolar AQP1 expression. As cysts grew larger, AQP1 became restricted to the basal surface of the cyst wall not in contact with the media and both AQP1 and GLUT1 expressions diminished from the media-tissue interface. FOXJ1 expression was predominantly localized to the media-facing surface of the cysts.

CONCLUSIONS: Initial bipolar AQP1 expression suggests early bidirectional cerebrospinal fluid transport. The subsequent loss of AQP1 and GLUT1 from the media-tissue interface combined with FOXJ1 enrichment, indicates a transition from ChP identity towards a simple, ependymal-like epithelium. Ongoing investigation seeks to explore the differences found between ChPOs and human ChP. With further functional validation and cellular characterization, ChPOs may serve as a valuable model for investigating hydrocephalus pathophysiology.

PODIUM PRESENTATION #7

Utilization of Electroacupuncture to Manage Neuropsychiatric Symptoms Among Adolescent and Young Adult Cancer Patients and Survivors

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Background: While electroacupuncture (EA) shows promise in managing neuropsychiatric symptoms in adolescent and young adult cancer (AYAC) patients, it remains unclear whether EA administration is feasible among AYAC patients.

Purpose/Aim: (1) To evaluate the completion rate of EA among AYAC patients, and (2) compare outcomes of two EA regimens on managing neuropsychiatric symptoms.

Methods: This is a randomized, controlled, patient- and assessor-blinded pilot trial. (Clinicaltrials.gov: NCT05283577). AYAC patients (16-39 years old) who self-reported cognitive impairment, fatigue, insomnia, and/or distress were randomized (1:1) to receive ten weekly EA sessions to target either neuropsychiatric-specific (verum EA, vEA) or non-neuropsychiatric-specific (sham EA, sEA) acupoints. Outcomes were assessed using validated self-reported measures (EORTC QLQ-C30, FACT-Cog, MFSI-SF, RSCL, EQ-5D) and neurocognitive tests (CANTAB®). Between-group differences were determined using Cohen's d (*d*) from linear mixed models adjusted for baseline variability. Adverse events (AEs) were graded with CTCAE v5.

Results: Thirty-four participants were recruited, with 11 (64.7%) participants from the vEA group (n=17) and 12 (70.6%) from the sEA group (n=17) having completed all treatment sessions. The mean age was 20.9 (±3.5) with 59% being Hispanic/Latino, 59% with hematologic cancers, and 82% reporting ≥2 neuropsychiatric symptoms. The vEA group experienced greater attention improvements (*d*=1.480) upon treatment cessation, whereas larger improvements favored the sEA group in response speed at the end-of-treatment (*d*=-1.091) and at the 4-week post-treatment follow-up (*d*=-1.046). Self-perceived cognition (*d*=-1.017) and fatigue (*d*=-0.940) improvement favored the sEA group at post-treatment follow up. Most commonly reported all grades AEs were pain (vEA, n=1; sEA, n=1) and tremors (vEA, n=1; sEA, n=2).

Conclusions: EA was feasible for managing neuropsychiatric symptoms in AYAC patients; however, strategies to improve completion rates are needed. Biomarker analysis will help elucidate the underlying mechanisms associated with the improvements observed in this trial.

Funding: University of California, Irvine Anti-Cancer Challenge

PODIUM PRESENTATION #8

Retrospective Application of Genome-Based Newborn Screening Platforms Identifies Large Number of Diagnoses in Critically Ill Children

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Background: Large prospective trials are underway that examine the clinical utility and cost effectiveness of genome-based newborn screening (gNBS). The three most well-described gNBS platforms are BeginNGS, GUARDIAN, and Generation Study (GenomeEngland), with each screening for between 400 and 500 severe childhood genetic diseases. gNBS was conceptualized as a supplement to traditional newborn screening in an effort to identify childhood-onset genetic disease prior to manifestation of associated morbidity and mortality. Over the last decade, rapid diagnostic genomic sequencing (RDGS) has been increasingly performed in children with diseases of uncertain etiology at ICU admission, but mortality in some of these studies has exceeded 20%, behooving genome sequencing advocates to identify these children prior to their sentinel admission.

Purpose: With the promise of gNBS programs to identify at-risk infants and children prior to clinical decompensation, we wanted to know what proportion would have been potentially identified at birth with gNBS.

Methods: We retrospectively evaluated a cohort of children admitted to the NICU, PICU, or CVICU at one of two tertiary children's hospitals (CHOC or Rady Children's) who received rapid phenotype-driven (diagnostic) WGS during ICU admission between 2017 and 2025.

Results: One hundred and five patients had likely pathogenic or pathogenic variants in genes that are present on at least one of the three gNBS platforms, out of a total of 426 patients that received a molecular diagnosis from RDGS during their ICU admission (24.6%). Associated change in management and clinical outcomes are in the process of being evaluated. There was minimal overlap between the three gNBS platforms: only 18 genes were present on all three gene lists.

Conclusion: These findings lend support for genome-based newborn screening initiatives and their potential promise to decrease infant/childhood morbidity/mortality. Prospective studies are needed. More concordance is needed between different gNBS platforms to avoid future geographically-dependent discrepancies.

Poster Presentation Abstract Index

#	Presenting Author	Poster Title
		CLINICAL RESEARCH
1	Grace Yip	Pain Catastrophizing in a Diverse Sample of Youth and Caregivers in the Pediatric Emergency Department
2	Aubrey Gibson	Multicenter Analysis Confirms that Ultrasounds in the First Two Days of Life Underestimate Severity of Hydronephrosis
3	Jonathan Nguyen	Single-Layer Cytal Urinary Bladder Matrix as a Novel Graft in Corporoplasty for Ventral Curvature Repair in Pediatric Hypospadias
4	Vivian Nguyen	How Do Coping Styles Predict Future Optimism During COVID-19?
5	Arya Sherafat	Patient and Guardian Perspectives on Tissue Engineering in Microtia Reconstruction
6	Arcadia Lee	Brain MRI at Term-Equivalent Age and Neurodevelopmental Outcomes at School Age in Children Born Very Preterm: A Systematic Review and Meta-Analysis
7	Tiffany Huang	Cost Analysis of Managing Low-Grade Hydronephrosis During the First Two Years of Life
8	Jenny Zhu	Return to the Emergency Department: Utilization and Risk Profiles in Pediatric Patients With Primary or Unspecified Headache
9	Jacqueline Raetz-Vigon	Characterizing Distress and Referral Source in the Emergency Department Waiting Room
10	Ruchira Puri	Gross Hematuria (GH) and Urinary Tract Infections (UTI) Among Young Male Patients in the Emergency Department (ED)
11	Fernando Gomes	Cervicomedullary CSF space obliteration as a predictor of reoperation in Chiari malformation type I
12	Ignacio Mesina	Clinical Patterns Following Intracranial Hemorrhage in Pediatric Mild Traumatic Brain Injury
13	Jennifer Morgan	Cardiopulmonary characteristics of young pectus excavatum patients with lower versus higher exercise performance
14	Candela Romano	Valosin-Containing Protein Associated Disease in Children: Emerging Clinical Features
15	Naman Shah	Mesenchymal Chondrosarcoma Presenting as Atypical Brown-Sequard Syndrome in a Healthy Teenager
16	Alyssa Joachim	Beyond the Pull-Through: A Multi-Institutional Study of Long-Term Surgical and Functional Outcomes in Total Colonic Hirschsprung Disease: A Pediatric Colorectal and Pelvic Learning Consortium (PCPLC) study
17	Alice Martino	Percutaneous Pigtail Catheter versus Chest Tube for the Treatment of Pediatric Traumatic Hemothorax: An EAST Multicenter Study
18	Blake Han	One and Done: A Retrospective Analysis Supporting Streamlined Follow-Up in Pediatric Clavicle Fractures
19	April Carlson	Association of Maximum CPAM Volume Ratio in Postnatal Surgical Outcomes in Congenital Lung Malformations
20	Christopher Baker	One up, One down: Analyzing Patient Preference of Ice Machine Tubing Placement
21	Aubree Goodman	Outcomes of Slipped Capital Femoral Epiphysis Utilizing a Broad Federated Network of Global Real-World Data
22	Michelle Hough	Comparative Outcomes of Proximal Stoma Versus Primary Anastomosis in Pediatric Colorectal Trauma
23	Lauren Burnett	Comparative Outcomes of Haploidentical versus Matched Sibling Donor Hematopoietic Stem Cell Transplantation in Pediatric Acute Lymphoblastic Leukemia
24	Sean Dornbush	The Differences in Outcomes at Pediatric vs. Adult Hospital for 18-26 years old with Complex Chronic Conditions
25	Zoe Flyer	Optimizing Timing for Asymptomatic CPAM Resection: A PHIS Database Analysis (Phase 1)
26	Jennifer Barrows	Systematic Review of Electronic Monitoring Devices to Increase Medication Adherence in Children with Asthma
27	Amirhossein Shahriari	Reducing Cellular Adhesion on Shunt Catheters Using Zwitterionic Surface Modification: A Bioreactor-Based Study
28	Shayan Ahmad	Association between Brain Volumes at Term-Equivalent Age and Neurodevelopmental Outcomes in Very Preterm Infants: a Systematic Review and Meta-Analysis
29	Emily Garavatti	Retrospective review of sleep in children with a history of HIE
30	Van Huynh	The Utility of Antifungal Prophylaxis to Prevent Invasive Fungal Disease in Pediatric and Adolescent Young Adults with Hematologic Malignancies
31	Sumiko Abe	Serial Brain MRI Volumetrics and DTI Analysis in Atypical CLN2 Patients Treated with ICV Cerliponase Alfa
32	Anahita Darabpour	Pain Score Discordance Among Pediatric Patients, Parents, and EMS Providers
33	Kimberly Maldonado	EMS Use of the Pediatric Assessment Triangle in the Prehospital Environment
34	Raymond Nguyen	Climate Anxiety among Adolescents in a Pediatric Emergency Department

35	Catherine Roman-Estrada	Health Care Transition for Adult-Aged Patients with Medical Complexity: Caregiver Perspectives and Experiences in the Pediatric Emergency Department
36	Madison Palarca-Wong	An Adolescent-Caregiver Dyad Approach to Climate Anxiety in the Pediatric Emergency Department
37	Trinidad Alcala-Arcos	Assessing the Financial Burden of Congenital Diaphragmatic Hernias from Diagnosis through Childhood
38	Andreina Urrutia-Gonzales	Timing of Colectomy and Surgical Outcomes in Pediatric Patients with Ulcerative Colitis
39	Peter Yu	Typology of Legal Intervention Trauma Among Pediatric and Young Adult Decedents: A Latent Class Analysis
40	Heather Speer	Recent Advancements in Treatment in Pediatric Diabetes and Endocrinology
41	Makenzie Ferguson	Child Restraint System Use and Injury Severity in Pediatric Motor Vehicle Collisions: Insights from the NTDB
42	Justin Shen	Comparison of the Auditory Environment in Premature Infants by Room Type and Postmenstrual Age in a Dedicated Small Baby Unit
43	Aaron Yengo-Kahn	Dance of the dura: Why does neuromonitoring trigger contraction during spinal lipoma excision?
44	Shelby Shelton	Bridging Gaps in Emergency Care: A Clinical Pathway to Improve Healthcare Transition for Medically Complex Adults
45	Diana Magana Martinez	Redefining Patient-Centered Care in Pediatric Endocrinology
46	Heidi Joyce	Comparative Efficacy of Proton Pump Inhibitors in Pediatric Eosinophilic Esophagitis: A Retrospective Review
47	Sonia Morales	Predicting Asparaginase Activity in Pediatric Leukemia: The Role of Fibrinogen and Antithrombin III
48	Katherine Rodriguez	Intersecting Inequities: Neighborhood Deprivation, Psychological Distress, and Financial Burden Among Pediatric and AYA Patients with a Solid Tumor Diagnosis and Their Caregivers
49	John Schomberg	Validation of Key Genetic Pathways in Compensatory Lung Growth via Meta-Analysis of Mouse Model Data in the Gene Expression Omnibus (GEO)
BASIC & TRANSLATIONAL RESEARCH		
50	Kalysa Bui	How does sleep quality affect emotional regulation and daily mood in pediatric patients with and without Obstructive Sleep Apnea?
51	Sarah Randall	Regulatory Gene Analysis in Enteric Neural Crest Cells Stratified by GDNF Activation Status
52	Pallabi Pal	MicroRNAs targeting Valosin-containing protein improve molecular defects in cell model of multisystem proteinopathy
53	Alyaa Shmara	Translational studies in iPSC-derived skeletal muscle progenitor cells and Hspb8 mouse model for HSPB8- associated myopathy by upregulating autophagy with trehalose.
54	Hafiz Muhammad Umer Farooqi	Developmental and Behavioral Characterization of Ndufs4 Mutant Mice
55	Gio Jison	A stereotactic injection method to establish a clinically relevant germinal matrix hemorrhage murine model
56	Chloe Christensen	In vitro assessment of cytosine base editing and prime editing for correction of the common p.N409S and p.L483P GBA1 pathogenic variants
57	Allen Murray	Oligosaccharide Storage in Pompe Mouse Tissues and History of Oligosaccharide Research
58	Uyen Vo	Impairment of energy metabolism in THG1L-deficient fibroblasts
59	Derek Chan	Organoid-Based Modeling of NURDopathies: Insights from GATAD2B-Associated Neurodevelopmental Disorder
60	Valentina Muti	Machine Learning-Based Prediction of Sagittal Alignment in Adolescent Idiopathic Scoliosis
61	Seunghyun Lee	Dual-Approach Surface Engineering of Ventricular Catheters to Reduce Cellular Occlusion in the Treatment of Hydrocephalus
62	John Schomberg	Placental transcriptome analysis of co-occurrent increased maternal BMI and preeclampsia demonstrates distinct gene expression profiles
63	Calvin Li	Genetically Modified Neuroblastoma–Dendritic Cell Fusion Cells as Autologous Vaccines for Childhood Cancer Immunotherapy
64	Habiba Aziz	Amniotic Fluid and Plasma microRNA Performance in the Prenatal Diagnosis of Congenital Diaphragmatic Hernia
65	Karen Appel	The Role of Regulatory T-Cells in Predicting Outcomes in Inflammatory Bowel Disease
COMMUNITY-BASED, SOCIAL SCIENCE or EDUCATIONAL RESEARCH		
66	Anna Le	Comprehensive Assessment of T-cell exhaustion in allogeneic HSCT and CAR T-cell immunotherapy
67	Cathy Duong	A comparison of clinical features in two patients with Mucopolysaccharidosis VI treated with ERT versus HSCT
68	Charissa Lau	Improving Screening for Social Drivers of Health in Hospitalized Pediatric Patients
69	Sheila Modir	Feeling Seen and Heard at School: Innovation with Mental Health Wellness Centers
70	Reny Partain	Quantifying Success: Evaluating Transfer Metrics in a Specialty Transition Clinic

POSTER #1

Pain Catastrophizing in a Diverse Sample of Youth and Caregivers in the Pediatric Emergency Department

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Background: Pain catastrophizing (PC) is well-documented in pediatric chronic pain but remains understudied in the pediatric emergency department (PED), particularly among ethnically diverse patients with varying pain presentations. While prior literature has studied contributing factors, their correlation remains unknown.

Purpose: To examine factors (demographics, pain duration, pain intensity, and caregiver and youth PC) that contribute to PC in youth presenting to a PED with abdominal pain and their caregivers.

Methods: English and Spanish-speaking youth 8-17 years old presenting to a PED (N=680, 76.7% Latinx/Hispanic) and their caregivers were included. Youth reported pain intensity and pain duration (≥3 months indicated chronic pain). Youth and caregivers completed the Pain Catastrophizing Scale (PCS-C & PCS-P). PCS-C scores >26 indicated high youth PC.

Results: In this sample, 54.3% endorsed high levels of PC. High PC was associated with female gender (X^2 =11.15, p =.004), chronic pain (X^2 =10.58, p =.001), and higher pain intensity (Z =-7.06, p <.001), which were all independent predictors in regression analyses (OR=1.63; OR=2.21; OR=1.23, respectively, *p's* <.01). Caregiver PC was significantly associated with youth PC (r =0.25, p <.001). Spanish-speaking caregivers and caregivers of youth with chronic pain reported significantly higher PC (Z =-6.22; Z =-2.68, *p's* <.01). Regression analyses indicated a significant effect of caregiver language on PC (b =8.49, p <.001), which wasn't moderated by pain duration.

Conclusion: Over half of youth presenting to a PED with abdominal pain endorsed high PC. Chronic pain, pain intensity, and caregiver PC were significant contributors to youth PC. Spanish-speaking caregivers reported higher PC regardless of pain duration. Findings underscore a need to examine youth and caregiver PC in the ED setting, particularly in culturally diverse populations.

POSTER #2

Multicenter Analysis Confirms that Ultrasounds in the First Two Days of Life Underestimate Severity of Hydronephrosis

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Background: Current guidelines for postnatal management of patients with prenatal hydronephrosis caution that the first ultrasound be performed after two days of life. However, many renal ultrasounds are performed during the newborn hospitalization period shortly after birth. Our objective was to determine if ultrasounds performed at 0-2 days of life underestimate hydronephrosis severity.

Methods: Eligible patients were reviewed from a prospectively maintained multi-institutional registry. Inclusion criteria were history of prenatal hydronephrosis, first postnatal ultrasound in the first month of life, and second ultrasound within 6 months of life. Patients were divided into comparative groups: initial postnatal ultrasound during 0-2 or 3-30 days of life. The main outcome was an increase in Society for Fetal Urology (SFU) hydronephrosis grade from the first to the second ultrasound. Analyses were conducted using Chi-square or Wilcoxon tests, and multivariable logistic regression.

Results: Of 2,770 patients, 845 met inclusion criteria. 42% (351/845) of patients had initial ultrasound on days of life 0-2. Patients with first ultrasound during days 0-2 were significantly more likely to show an increase in SFU grade (19% versus 11%, $p = 0.001$) and to have an increase from low grade to high grade (13% versus 5%, $p = 0.002$) on their next ultrasound compared to those with first ultrasound during days 3-30. Adjusting for other anomalies and sex, patients with first ultrasound on 0-2 days of life were 2.7 times more likely to show an increase from low to high SFU grade at the second ultrasound as patients with first ultrasound on days 3-30 (OR=2.7, 95% 1.6-4.4).

Conclusions: Our results confirm that the postnatal ultrasound during the first two days of life may underestimate hydronephrosis severity. Patients with low grade hydronephrosis on ultrasound in the first 48 hours require close follow-up with urology and at least one additional confirmatory ultrasound.

POSTER #3

Single-Layer Cytal® Urinary Bladder Matrix as a Novel Graft in Corporoplasty for Ventral Curvature Repair in Pediatric Hypospadias

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Introduction: Single-layer Cytal® matrix (ACell Inc, Columbia, MD) is a single-layer acellular scaffold matrix derived from porcine bladder epithelial basement membrane. Like small intestinal submucosa, it provides a scaffold stimulating neovascularization and new tissue proliferation. We present the use of ACell in corporoplasty for correction of ventral curvature in hypospadias repair by four surgeons at a single institution.

Methods: Data was collected prospectively on hypospadias surgeries performed between 2020 and 2024. All patients undergoing an initial surgery for hypospadias repair with ventral curvature that utilized ACell with at least 3 months of follow-up were included. Analysis included recurrent ventral curvature and any adverse events.

Results: Twenty-four patients had ACell used in their first stage surgery as part of corporoplasty and met inclusion criteria. Median age was 16.0 months (IQR 14.8-20.2). Median curvature at time of surgery was 85 degrees (52-90). Median follow up was 13.4 months (9.3-17.1). Twenty-one patients (87.5%) had either observed erections or erection tests performed in the operating room, all of which were reported as straight. The remaining three patients have not had observed erections. For the 16 patients (67%) who have completed a second stage repair at time of last follow up, median age at second surgery was 27.0 months (25.3-28.8). Six of these 16 second stage surgeries utilized Acell. Six other patients are pending a second stage surgery. Three patients developed urethrocutaneous fistulas that were or will be repaired at a subsequent surgery.

Conclusions: ACell is a readily available, relatively inexpensive material that can be used as a graft during corporoplasty, thus reducing morbidity associated with flaps or autografts. This study shows its efficacy in the repair of ventral curvature in the short term. Long term follow up, as well as direct comparisons to other commonly used grafts, will help confirm durability of these results.

POSTER #4

How Do Coping Styles Predict Future Optimism During COVID-19?

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Background: How one deals with stressors may be a key element behind optimism, which has been linked to greater resilience development. Resilience is correlated with better psychological and physiological well-being, including faster healing and recovery, which is crucial for pediatric patients. As such, it may be worth examining potential predictors of optimism, such as stress coping. Coping strategies can be classified into three overarching styles, including avoidant, emotion-focused, and problem-solving coping.

Purpose/Aims: Our research sought to explore the link between coping styles and state optimism.

Methods: Data was collected from 292 participants during the height of the COVID-19 pandemic via a questionnaire assessing coping mechanisms in May, 2020 and a second questionnaire assessing optimism levels in September, 2020. Linear regression analysis was performed to analyze whether each coping style predicted state optimism, controlling for race, age, and sex.

Results: Results indicated that participants with higher avoidant coping levels were less optimistic, b = -0.442, p = 0.012. Participants with greater problem-solving coping were more optimistic, b = 0.318, p = 0.016. Those with greater emotion-focused coping also showed greater optimism, b = 0.327, p = 0.020. When including all three coping styles in one model, results revealed avoidant coping as a significant negative predictor of optimism, with greater avoidant coping linked to less optimism, b = -0.623, p < .001.

Conclusion: This study empirically highlighted how avoidant coping may be maladaptive and negatively predicts optimism during adversity. Results underscored the importance of targeting avoidant coping to foster optimism, which may help build resilience in pediatric hospital patients. Resilience enhancement may have positive influences on patient recovery and health outcomes.

POSTER #5

Patient and Guardian Perspectives on Tissue Engineering in Microtia Reconstruction

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Background: Tissue Engineering (TE) is a newer technology with ongoing development across various areas of healthcare. For microtia patients, TE holds promise as a viable option for ear reconstruction, making it essential to understand the perspectives of patients and guardians. This study aimed to evaluate the perspectives of microtia patients and guardians, specifically investigating levels of awareness, comprehension, and interest in TE.

Methods: A survey and educational material were distributed to microtia patients and guardians. Surveyors were assessed on a Likert scale, and comparative analyses, including two-sample t-testing and multivariate analysis with logistic regression, were used to observe trends in responses.

Results: A total of 40 surveys were recorded. White patients reported greater familiarity with TE (3.00 vs. 2.13, $p < 0.05$). More than half of the respondents ($n = 26/40$) expressed an interest in TE as a reconstructive option. Having a doctor provide TE information positively correlated with willingness to adopt TE reconstruction ($p = 0.014$). Understanding of TE positively correlated with willingness to pursue TE for reconstruction ($p<0.001$). Patients with more severe grade microtia and white patients were more likely to travel to a hospital that offered TE ($p = 0.029$, $p = 0.030$).

Conclusions: We found that TE is an unfamiliar concept for most patients. Demographic, socioeconomic, cost, and trust in a doctor played an essential role in patients' and guardians' willingness to choose TE for microtia reconstruction. As TE for reconstruction approaches clinical use, these key considerations from a patient and family-centered lens about TE-based reconstruction is critical for application.

POSTER #6

Brain MRI at Term-Equivalent Age and Neurodevelopmental Outcomes at School Age in Children Born Very Preterm: A Systematic Review and Meta-Analysis

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Background: Qualitative brain MRI at term equivalent age for very preterm (VP) or very low birth weight (VLBW) infants may predict later neurodevelopmental outcomes.

Purpose/Aims: To better understand the association between qualitative brain MRI at term-equivalent age and school-age cognitive, language, behavioral, and motor outcomes among VP/VLBW children.

Methods: We searched Web of Science, Scopus, and PubMed from inception to March 2025 for articles exploring the relationship between term-equivalent age brain MRI and neurodevelopmental outcomes in VP/VLBW cohorts. Longitudinal cohort studies with infants born ≤ 32 weeks or ≤ 1500g with a term equivalent age MRI and follow-up at or beyond 3 years of age were included. Eligibility and risk of bias was performed by two reviewers and data extraction by one reviewer. A meta-analysis was conducted for each outcome of interest if four or more eligible studies were identified.

Results: The meta-analysis revealed that VP/VLBW children with a normal brain MRI at term-equivalent age have a significantly higher IQ at school-age than those with an abnormal brain MRI (pooled mean difference = 6.6, 95% CI: 4.3–8.9). Due to a suboptimal number of eligible papers, meta-analyses were not conducted for behavioral, motor, and language outcomes. However, a review of the eligible papers suggests abnormal brain MRI findings are also associated with motor and language outcomes, with behavior demonstrating more variable findings.

Conclusions: Abnormal findings on qualitative brain MRI at term-equivalent age is related to poorer neurodevelopmental outcomes at school age. These results suggest that term-equivalent brain MRI may serve as a useful prognostic marker for neurodevelopment, though further large-scale, longitudinal studies are needed to establish its broader clinical utility.

POSTER #7

Cost Analysis of Managing Low-Grade Hydronephrosis During the First Two Years of Life

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Background: Prenatal hydronephrosis is the most common fetal anomaly detected on prenatal ultrasound. Although prenatal diagnosis allows for early postnatal intervention for urinary obstruction, most cases are mild and resolve spontaneously. Serial outpatient visits with ultrasound imaging can impose a financial burden on caretakers.

Aim: To quantify costs associated with management of isolated low-grade hydronephrosis with data sourced from the Societies for Pediatric Urology Hydronephrosis Task Force.

Methods: Patients with postnatally confirmed prenatal hydronephrosis were enrolled in a multi-institutional database. Inclusion criteria were initial renal and bladder ultrasound (RBUS) with Society for Fetal Urology (SFU) grade 1–2 hydronephrosis and ≥2 years of follow-up. Patients with dilated ureters, duplex collecting systems, or other anomalies were excluded. Total surveillance costs included an initial level IV urology consult, follow-up visits, RBUS (Medicare/Medicaid Fee Schedule and CHOC Fee Schedule), and family time costs for a half-day clinic visit (based on June 2025 Orange County median household income).

Results: Of 2,770 patients in the Task Force database, 1,672 had isolated hydronephrosis (21% SFU grade 1, 40% SFU grade 2). A total of 294 patients with ≥24 months of follow-up met criteria. Median number of RBUS over two years was 5.0 (IQR 4.0–7.0). With Medicare-indexed costs, imaging totaled $812.25, initial visit was $178.34, and follow-up visits were $137.58. Per CHOC pricing, RBUS, facility fees, and physician services were $2024, $450, $514, and $332, respectively. Family time costs for five half-day visits were $514.40. The composite two-year cost per patient was $2055.31 with Medicare indexing and $14,476.50 with CHOC pricing.

Conclusions: Although management of low-grade hydronephrosis is conservative and typically limited to serial RBUS, the costs are substantial ranging from $2055 or nearly $14500 per patient over two years. Given most cases resolve without intervention, guidelines should weigh both outcomes and financial burden to optimize care.

POSTER #8

Return to the Emergency Department: Utilization and Risk Profiles in Pediatric Patients with Primary or Unspecified Headache

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Background: Primary or unspecified headache is a common pediatric emergency department (ED) presentation. Diagnostic uncertainty and limited access to specialty care may drive repeat ED use; however, longer-term utilization data are limited.

Purpose/ Aims: The purpose is to characterize ED utilization among pediatric patients presenting with headache and assess whether utilization is associated with risk factors like prior ED visits, neurology care, and reported pain and symptom duration.

Methods: This prospective cohort study included 93 children (ages 8–17 years) diagnosed with primary or unspecified headache in a pediatric ED. Demographics and headache characteristics were self-reported. ED and neurology visits 12 months before and 6 months after the ED index visit were extracted from hospital medical records. Chi-square and Mann Whitney U tests were conducted.

Results: Overall, 16.1% (n=15) of children with primary or unspecified headache had a previous headache-related ED visit and 12.9% (n=12) returned for headache within six months. Neurology utilization significantly increased after ED index visit (14%-28%, p=.011). Prior headache-related ED and neurology visits were significantly associated with headache-related ED returns within one (p's<.001) and three months (p's<.015). Post-index neurology visits were not associated with ED returns (p>.05). Children returning within one month were more likely to report chronic headache (p=.048). Those with $\geq$2 headache-related ED encounters were more likely to report >1 month headache duration (p=.019).

Conclusions: Nearly 13% of children with primary or unspecified headache returned to the ED for headache, exceeding prior reports with shorter follow-up windows. ED return visits were linked to prior headache-related ED and neurology encounters, and recurrent or chronic headache, suggesting a need to screen for these risk factors. Although neurology utilization increased after the ED index visit, post-neurology care did not influence ED returns, highlighting gaps in longitudinal headache treatment and a need for improved management.

POSTER #9

CHARACTERIZING DISTRESS AND REFERRAL SOURCE IN THE EMERGENCY DEPARTMENT WAITING ROOM

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Background: Patient satisfaction in the emergency department (ED) is a multifactorial issue facing hospital leadership with wait periods being a primary factor influencing satisfaction in the pediatric ED. The use of the ED for non-urgent medical issues may exacerbate wait times and burden hospitals globally. Parental anxiety and perceived severity as well as access to quality care appear to influence this decision; however, further understanding of these factors is required to ensure patients have a satisfactory ED experience.

Purpose: To identify factors associated with choosing the ED over other levels of care by determining the source of referral, reasons for ED visit, and anxiety levels.

Methods: This is a prospective cross-sectional electronic survey of English- and Spanish-speaking parents/caregivers waiting in the Children's Hospital of Orange County (CHOC) ED waiting room. Associations were determined using Wilcoxon rank sum test and Pearson's Chi-square test.

Results: We recruited 1,537 participants from 1/7/25-7/14/25. A majority of patients (54%) were male gender, white race (58%), and of Hispanic or Latino ethnicity (69%). The mean length of stay was 4.5±2.8 hours, and the mean Emergency Severity Index (ESI) was 3.3±0.7. One-third (n=458) of participants were referred to the ED from other clinicians. Of families who had not been referred to the ED (N=968), 55% were male, 74% of Hispanic ethnicity ($p<0.001$), and 34% in lower income households ($p<0.001$). Further, the non-referral cohort reported lower anxiety (6.6 vs 7.5, $p<0.001$) and rated seriousness of illness lower than participants in the referred cohort (5.9 vs 6.3, $p<0.001$).

Conclusions: The results demonstrated that referral to the ED may increase anxiety and perceived severity of illness. Next steps may be to educate families who are referred to the ED on reasons why acute care might be needed and supportive behaviors from health care providers to lower anxiety for these families.

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POSTER #10

Gross Hematuria (GH) and Urinary Tract Infections (UTI) Among Young Male Patients in the Emergency Department (ED)

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Background: Hemorrhagic UTIs are rare among healthy young males and lack epidemiological data in the ED.

Purpose/Aim: The purpose of this study is to investigate the clinical presentation and lab features of UTIs in healthy young males presenting to the ED with GH.

Methods: Retrospective chart review of male patients aged 1–9 years presenting to a single urban pediatric ED between January 2018 and December 2023 with ICD-10 codes related to hematuria, UTI, and other genitourinary complaints. Inclusion criteria were urinary symptoms including GH during the ED visit. Patients were excluded if they had pre-existing exclusionary diagnoses. Demographic, clinical, laboratory, and imaging data were extracted. UTI was defined as positive LE and/or 5+ WBC/hpf and 100,000 colony forming units (CFU)/mL for mid-stream urine specimens or >10,000 CFU/mL for catheterization. GH was defined as blood in urine/hematuria as an acute symptom.

Results: 763 of 1175 patients were included in our study pool. Fever (38%), dysuria (35%), and hematuria (23%) were the most common symptoms and 28% of patients with documented hematuria status had defined GH. GH was associated with older age (median 5.2 vs 3.9 years, $p<0.001$) and a lower proportion of confirmed UTI (10% vs 25%, $p<0.001$). After adjusting for confounders, GH was associated with a reduced risk of UTI ($p<0.001$). No significant differences in urologic complications, ED antibiotic administration, or 6-month outcomes were observed between patients. *E. coli* (13%) and *Proteus mirabilis* (10%) were the most commonly isolated organisms.

Conclusion: Our study shows that GH at the time of ED presentation was not a reliable predictor of confirmed UTI in this pediatric male cohort. Our findings suggest that isolated GH should not prompt antibiotic treatment in the absence of other supportive clinical or laboratory findings.

POSTER #11

Cervicomedullary CSF Obliteration in Chiari I Malformation: Clinical Relevance and Interrater Reliability

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Background: Various radiographic schemes quantify tonsillar and brainstem ectopia in Chiari malformation type I (CM1), yet few have direct clinical utility. Cervicomedullary CSF obliteration may represent a simple, dichotomous measure with prognostic value.

Purpose/Aims: This study examined whether cervicomedullary space obliteration predicts surgical complications and reoperation in CM1 and assessed the interrater reliability (IRR) of this measure among practicing pediatric neurosurgeons.

Methods: We retrospectively reviewed 272 surgically treated pediatric CM1 patients, comparing those with and without preoperative cervicomedullary CSF obliteration. Multivariable logistic regression estimated adjusted odds ratios (AORs) for postoperative complications and reoperation. Separately, 68 attending neurosurgeons at the 2024 AANS/CNS Joint Pediatric Section and ASPN meetings rated 15 MRIs for obliteration. Respondents' practice focus, annual CM1 case volume, and years of experience were recorded. IRR was assessed with Fleiss' kappa.

Results: Patients with obliterated spaces more frequently presented with ataxia, weakness, swallowing difficulty, sleep apnea, and cranial neuropathies, and had lower tonsillar and obex positions (all $p \leq 0.02$). Obliteration predicted postoperative hydrocephalus (21.4% vs. 8.5%; AOR 2.93; 95% CI 1.50–6.34; $p=0.003$) and reoperation (7.1% vs. 1.7%; AOR 4.23; 95% CI 1.18–27.17; $p=0.03$). Among survey participants, 79.4% had pediatric-focused practices, 30.9% treated >10 CM1 patients annually, and 44.1% had >10 years' experience. IRR for obliteration was substantial ($\kappa=0.789$) and consistent across practice groups ($\kappa=0.746$–0.835).

Conclusions: A dichotomous assessment of cervicomedullary CSF obliteration provides clinically meaningful prognostic information, identifying CM1 patients at increased risk for postoperative hydrocephalus and reoperation. Its high reproducibility across diverse neurosurgeons makes it a practical tool for both surgical planning and incorporation into risk prediction models.

POSTER #12

Clinical Patterns Following Intracranial Hemorrhage in Pediatric Mild Traumatic Brain Injury

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Background: Mild traumatic brain injury (mTBI) is a common cause of emergency department visits among school-aged children. Although considered the least severe form of traumatic brain injury, intracranial hemorrhage (ICH) can still occur. Clinical tools such as the Glasgow Coma Scale and PECARN aid in initial decision-making, but the clinical relevance of imaging findings in mTBI remains uncertain. We evaluated whether ICH on baseline imaging is associated with follow-up outcomes and resource utilization.

Methods: We conducted a retrospective cohort study of patients seen in the CHOC Concussion Clinic following mTBI. Consecutive patients aged 8 to 21 years who underwent brain imaging at the index encounter were included. Patients were categorized as imaging-positive if any ICH was present. Outcomes included: (1) occurrence of post-concussion syndrome, (2) prescription of medications for persistent symptoms, and (3) number of follow-up imaging studies. Unadjusted comparisons were performed using Fisher's exact test for categorical variables and Wilcoxon rank-sum or Student's t-test for continuous variables, as appropriate. Adjusted estimates were obtained using multivariable regression, with effect sizes reported as odds ratios (ORs) and 95% confidence intervals (CIs).

Results: We analyzed 226 patients: 50 with ICH and 176 without. Subdural hematoma was the most common hemorrhage subtype. Age and sex were balanced between groups. Mechanism of injury differed, with falls more common in the ICH group (38% vs 18%, $p = 0.041$). Time to concussion clinic was shorter in the ICH group (median 30 days [IQR 22–44] vs 66 days [IQR 31–137], $p < 0.001$). Post-concussion syndrome occurred at similar rates (28% vs 27%, $p = 0.99$). Medication prescription rates were also comparable (46% vs 61%, $p = 0.075$). Patients with ICH underwent more imaging at follow-up (median 2 studies [IQR 1–3] vs 1 [IQR 1–2], $p < 0.001$). Adjusted analyses yielded consistent effect estimates across outcomes.

Conclusion: Intracranial hemorrhage on index imaging was associated with earlier specialty follow-up and greater imaging utilization, but not with differences in short-term clinical outcomes. These findings suggest that ICH in the context of mTBI may influence care pathways more than clinical trajectory.

POSTER #13

Cardiopulmonary Characteristics of Young Pectus Excavatum Patients with Higher versus Lower Exercise Performance

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Background and Aim: Pectus excavatum (PE) is a common chest wall abnormality often characterized by right heart compression, possibly affecting cardiopulmonary function and exercise tolerance. This study sought to evaluate associations between cardiac MRI (CMR) parameters and exercise performance on cardiopulmonary exercise testing (CPET).

Methods: A single-center, IRB-approved, retrospective review of 62 young PE patients was performed. CPET measurements included O2 pulse and VO2max. Two subgroups were selected, with 14 patients (64%) in the lower performance subgroup and 8 (36%) in the higher. Percent predicted VO2max and O2 pulse of >80% defined higher performance; ≤ 60% defined lower performance. Haller Index (HI), Correction Index (CI), Cardiac Compression Index (CCI), Sternal Torsion Angle (STA), cardiac axis (CA), IVC/RA junction compression, and biventricular volumes/function were obtained by CMR.

Results: The cohort was 11 Hispanic (50%), 6 White (27%), 2 Asian (9%), and 3 Other (14%); majority were male (86%). There were no differences in expiratory HI (8.1 vs. 6.1,p=0.29), inspiratory HI (5.3 vs. 4.4,p=0.30), CI (45.3% vs. 32.5%,p=0.11), CCI (3.68 vs. 3.34,p=0.50), CA (22.7 vs. 26.7 degrees, p=0.43), Sternal Torsion Angle (17.2 vs. 17.8 degrees, p=0.89), or sternal tilt direction between the groups. The lower performance group tended to have more IVC/RA junction compression on CMR [7 (50%) vs. 1 (13%), p=0.079)]. Indexed RV end-systolic volume was greater in the higher performance group (45.3 vs. 35.9 mL/m2,p=0.04), with a trend towards lower RV ejection fraction (51% vs. 55%,p=0.08).

Conclusions: Widely-used and novel CMR-derived chest wall and cardiac measurements did not discriminate between level of exercise performance in our cohort. IVC/RA junction compression may be associated with worse exercise capacity. Higher exercise performance may mask subtle RV dysfunction. Larger studies are needed.

POSTER #14

Valosin-Containing Protein Associated Disease in Children: Emerging Clinical Features

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Background: Gain-of-function pathogenic variants in the valosin-containing protein (*VCP*) gene typically cause late-onset multisystem proteinopathy (MSP1) with progressive muscle weakness, Paget disease of bone, frontotemporal dementia, or ALS. Recently, a few pediatric cases have been described by Mah-Som et al. (2024), with a distinct presentation including developmental delay, autism, and macrocrania, and absent classic adult features associated with de novo loss-of-function VCP variants. We now report children presenting with gain-of-function MSP VCP variants associated with muscle weakness. These reports in children of adult-onset disease lead to complex ethical considerations.

Objective: Describe clinical and genetic findings of pediatric patients with VCP pathogenic variants associated with MSP1.

Methods: Medical records of pediatric patients with confirmed VCP pathogenic variants were reviewed, including developmental, neuromuscular, systemic, and genetic data**.**

Results: Five patients (3 females, 2 males) were identified, with symptom onset between 3 and 15 years. All presented initially with muscle weakness or myopathy. One child with the c.464G>A variant developed weakness at age 3 and died at 10 years. A patient with p. Leu198Trp presented with myopathy and early Paget's disease at 12 years. Two siblings with p.Arg159His developed symptoms at 12 and 14 years, including myopathy, hyperlaxity, and fatigue; the older sibling also experienced cognitive decline at 16 years. Another patient developed proximal weakness at 14 years, accompanied by elevated creatine kinase levels and skeletal pain, in the context of a family history of Paget's disease.

Conclusion: Pediatric VCP-associated disease is rare but heterogeneous, ranging from weakness in early childhood to adolescent-onset myopathy with systemic or cognitive features. Awareness is essential for timely diagnosis, genetic testing, and counseling. Longitudinal studies are needed to better define prognosis and management**.**

POSTER #15

Mesenchymal Chondrosarcoma Presenting as Atypical Brown-Sequard Syndrome in a Healthy Teenager

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Objective: Brown Sequard syndrome is typically associated with traumatic injuries, however other structural etiologies have also been reported. We report a case of a teenager with Brown-Sequard syndrome and bilateral vibration deficits, later diagnosed with mesenchymal chondrosarcoma (MCS).

Methods: Case report.

Results: A 14-year-old healthy, neurotypical teenager presented with decreased cold sensation in their right hand for 1 week followed by progressive left-arm weakness for 3 days prompting emergency care.
Neurologic examination showed low muscle bulk, absent right-sided pain and temperature sensation, rapidly progressing left-sided weakness, absent vibration bilaterally (except face and neck), hyperreflexia (worse on right side) with intact cranial nerves. MRI brain was not immediately possible due to braces, so he had CT head with contrast which showed significant spinal cord compression from an enhancing mass at the craniocervical junction. After braces were removed, MRI neuro-axis was completed and demonstrated a homogenously enhancing, calcified, lobulated lesion centered at the anterior foramen magnum with mass effect on the medulla, craniocervical junction, and proximal cervical cord.
The patient underwent gross-total resection. Pathology showed malignant mesenchymal neoplasm. RNA analysis identified fusion of HEY1:NCOA2, confirming diagnosis of MCS.

Conclusion: MCS is an extremely rare form of bone tumor, and diagnosis is often delayed as presenting symptoms can be highly variable. This case underscores the importance of thorough neurologic exam which found a Brown-Sequard-like syndrome, albeit with bilateral vibration loss, prompting emergent workup. It also highlights the importance of CT scans with contrast when MRI is not immediately an option. Atypical Brown-Sequard has not been reported in the pediatric population as the initial presentation for MCS. We aim to highlight a unique presentation of this highly malignant tumor.

POSTER #16

Beyond the Pull-Through: A Multi-Institutional Study of Long-Term Surgical and Functional Outcomes in Total Colonic Hirschsprung Disease: A Pediatric Colorectal and Pelvic Learning Consortium (PCPLC) study

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Purpose: Total colonic Hirschsprung disease (TCHD), the most severe HD variant (~5–10% of cases), is defined by aganglionosis of the entire colon. This study evaluates short-term surgical outcomes and long-term functional results in children with TCHD.

Methods: A retrospective cohort study was conducted on patients enrolled at PCPLC institutions from 2017 to July 2024. Patients were excluded if their primary pull-through was performed elsewhere or if follow-up was <4 years. Demographics, surgical details, and functional outcomes—including bowel management, toilet training, and stooling accidents—were collected. Fisher's exact test compared demographics between patients who did or did not undergo pull-through.

Results: Of 76 patients meeting inclusion criteria, 34 underwent primary pull-through at a PCPLC site and 29 at non-PCPLC sites; demographics and associated diagnoses did not influence whether patients received pull-through. Among PCPLC patients, median age at diagnosis was 11.5 days [IQR 5–112.5] and median age at pull-through was 13 months [IQR 10–21]. The most common procedures were Duhamel (n=12, 35.3%), Yancey-Soave (n=9, 26.5%), and Swenson (n=8, 23.5%). Four patients required re-diversion after ostomy closure, four required redo pull-through, and 10 (29.4%) underwent colon resection after ostomy creation but before pull-through. Among non-diverted patients, 17 were toilet trained at 4–7-year follow-up, while 8 reported stooling accidents, including 6 with more than one accident per week.

Conclusions: Primary pull-through of patients with TCHD yielded generally favorable early and mid-term outcomes, with most patients achieving toilet training by 4–7 years. However, a subset required re-diversion, redo pull-through, or colon resection, and some continued to experience stooling accidents, highlighting ongoing functional challenges despite standardized care.

POSTER #17

Percutaneous Pigtail Catheter versus Chest Tube for the Treatment of Pediatric Traumatic Hemothorax: An EAST Multicenter Study

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Background/Aim: Small percutaneously placed pigtail catheters (PCs) for traumatic hemothorax (HTX) are safe and effective in adults but have not been evaluated in children. We hypothesized PC would have similar efficacy and complication rates compared to chest tubes (CTs).

Methods: A retrospective study of hemodynamically stable pediatric trauma patients (<18 years) with HTX or hemopneumothorax (HPTX) was conducted at 41 trauma centers (01/2010-12/2022). Catheter failure was defined as a requirement for surgery, additional tube placement, or thrombolytics. Multivariable logistic regression analysis adjusting for age, sex, mechanism of injury, and injury severity score (ISS) was used to evaluate the associated risk of failure.

Results: Of 548 patients, 477 had CT and 71 PC. Median age (CT: 15.7 vs PC: 15.6, p=0.49) and ISS (CT: 17 vs PC: 16, p=0.17) were similar between cohorts. Penetrating trauma patients more often received CTs (62.6 vs 35.2%, p<0.0001). Failure rate was similar between CT vs PC (17.6 vs 12.6%, p=0.38). While the overall complication rate (respiratory distress, effusion, empyema, pneumonia, infection, deep venous thrombosis) was higher in the PC group on univariate analysis, (19.7% vs 11.9% in CT p=0.02), the risk of complications was not increased on multivariable analysis (OR 1.05, 95% CI 0.95-1.15, p=0.3). Length of stay (LOS), and intensive care unit LOS were similar between cohorts (all p>0.05). Logistic regression analysis revealed PC was not associated with the risk of failure (OR 0.95, CI 0.87-1.04, p=0.31). There was an increased risk of complications with ISS>15 (OR 1.17, CI 1.10-1.26, p<0.0001) and lower risk with penetrating injury (OR 0.86, CI 0.80-0.92, p=0.0001).

Conclusion: There was no difference in risk of failure between PC and CT for pediatric HTX/HPTX, and no difference in risk of complications after adjustment for confounders. PCs had similar safety and efficacy compared to larger-bore CTs in this large multi-institutional study.

POSTER #18

One and Done: A Retrospective Analysis Supporting Streamlined Follow-Up in Pediatric Clavicle Fractures

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Background: Clavicle fractures in children are typically managed nonoperatively due to immense bony remodeling potential. Therefore, repeat radiographs may not be necessary.

Purpose/Aim(s): This study aims to quantify follow-up visits in which repeat radiographs were performed to evaluate pediatric nonoperative clavicle fractures.

Methods: A retrospective study was performed by reviewing charts of children 8 years of age or younger with clavicle fractures between January 2018 and December 2022. Children were excluded if the fracture was managed operatively, if initial radiographs were not available, or if repeat imaging was indicated due to concomitant shoulder girdle fracture or history of ipsilateral shoulder surgery.

Results: Charts of 244 children with a mean age of 3.5 years were reviewed. The majority of fractures were mid-clavicular (96.7%), of which 56.1% were nondisplaced and 40.6% were minimally displaced. Of 244 children, 52.9% had only initial radiographs (mean age 2.9 years), 29.5% had up to two sets of radiographs (mean age 3.6 years), 13.5% up to three (mean age 4.8 years), and 4.1% had four sets (mean age 4.9 years). The mean time to follow-up was 4.2 days (initial), 23.2 days (second), 50.4 days (third), and 72.8 days (fourth). Routine healing was appreciated in all third and fourth sets of radiographs. The majority of initial radiographs were ordered by the emergency department (59.6%), with orthopedic surgery ordering 89.4% of second-visit radiographs, 97.6% of third-visit radiographs, and 100% of fourth-visit radiographs.

Conclusions: Repeat clavicle radiographs needlessly expose pediatric patients to radiation and their families to inessential costs. Eliminating third and fourth visit radiographs would have prevented 86 radiographs in 43 children, saving over $20,000 and mitigating over 40 mSv of radiation.

POSTER #19

Association of Maximum CPAM Volume Ratio in Postnatal Surgical Outcomes in Congenital Lung Malformations

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Introduction: Congenital lung malformations (CLMs) range from asymptomatic to those causing significant neonatal morbidity. The maximum CPAM volume ratio (mCVR) is an established prenatal risk stratification tool, but its postnatal predictive value remains less defined.

Aim: This study aimed to evaluate the relationship between mCVR and postnatal clinical outcomes.

Methods: We conducted a retrospective cohort study of neonates with prenatally diagnosed CLM who underwent resection at two fetal centers (2013–2023). Patients were stratified into three groups based on mCVR: <1, 1–2, and >2. Descriptive statistics, Kruskal-Wallis, and Chi-squared tests compared demographic, clinical, and outcome variables across groups.

Results: Among 129 neonates with reported mCVR who underwent postnatal resection, median mCVR was 0.8. Neonates with mCVR >2 were significantly more likely to require in-center delivery (95.4% vs. 84.3% vs. 48%, $p<0.001$), present with symptoms at birth (86.3% vs. 28.1% vs. 20%, $p<0.001$), and require NICU admission (68% vs. 31% vs. 17%, $p<0.001$). They also underwent earlier surgery, had longer hospital stays, more thoracotomies, and higher complication rates (all $p<0.001$).

Conclusion: mCVR >2 is a meaningful predictor of neonatal morbidity and can serve as a valuable tool for prenatal counseling and delivery planning.

POSTER #20

One up, One down: Analyzing Patient Preference of Ice Machine Tubing Placement

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Background: Cryotherapy is used in the postoperative period following orthopedic procedures. The recommended orientation of tubing from knee cuffs is variable across units.

Purpose/Aim(s): This study aims to investigate the preference and satisfaction based on whether the tubing was oriented Up / proximally or Down /distally following knee surgery.

Methods: Children who underwent an Anterior Cruciate Ligament (ACL) reconstruction or Medial Patellofemoral Ligament Reconstruction (MPFLR) and received a cryotherapy unit were included. Block randomization was utilized to assign patients to proximal or distal tubing directions. A survey was administered at one-week postoperative which included questions to assess overall satisfaction, comfort, and ease of use related to the unit. The primary outcome was the reported satisfaction level with secondary outcomes including pain scores in the six days following surgery.

Results: 42 children were enrolled in the study (Up group: n=16; Down group: n=26). The distribution of handedness and laterality of surgery between groups did not differ significantly (Table 1). There were no significant differences between groups regarding satisfaction, preference, ease of use, or comfort (Table 2). There was a significantly greater number of patients in the down group who reported needing assistance in connecting/disconnecting the tube ($p=0.027$). There were no differences in pain scores for post-operative days one through six between groups ($p>0.05$).

Conclusions: In children who utilize cryotherapy units following ACL reconstruction or MPFLR, there appears to be no difference in satisfaction, or comfort, based upon the orientation of the cryotherapy unit tubing. However, ease of use may be affected as those with the tubing facing their foot reported needing help in connecting/disconnecting the tubing more frequently. Therefore, consider orienting the tube directed proximally towards the thigh, particularly in those who might not have assistance readily available in the early post-operative period.

POSTER #21

Outcomes of Slipped Capital Femoral Epiphysis Utilizing a Broad Federated Network of Global Real-World Data

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Background: Slipped capital femoral epiphysis (SCFE) is a common childhood disorder associated with osteoarthritis (OA) and total hip arthroplasty (THA). To date, five large non-U.S. studies report THA outcomes after SCFE, yet none quantify short-term (adolescent/young-adult) THA risk. In two U.S. published TriNetX studies involving SCFE, neither evaluated OA or THA endpoints.

Purpose/Aim(s): To analyze the one, five, and ten year association of SCFE with OA and THA compared to other pediatric hip disorders.

Methods: We conducted a retrospective cohort study using the TriNetX U.S. Network. Children under 18 years diagnosed with SCFE were compared to congenital hip deformities, Legg-Calvé-Perthes (LCP), and unspecified hip pain/injury (control). Propensity score matching balanced covariates among cohorts.

Results: Among 9,150 children with SCFE, THA incidence rose from 0.16% at one year to 0.35% at five years and 0.42% at ten years. OA risk rose progressively to 2.2% at ten years. Compared with congenital hip deformities, SCFE demonstrated no difference in five-year risk of OA or THA. Relative to LCP, SCFE showed lower five-year risks of THA (0.46% vs 1.20%) and OA (2.2% vs 3.2%). SCFE also carried significantly higher risks for both outcomes compared with unspecified hip pain/injury.

Conclusions: The outcomes of THA and osteoarthritis rose significantly between one and five years after SCFE diagnosis, but only osteoarthritis continued to increase between five and ten years. Compared to other pediatric hip disorders, no difference was observed, but SCFE carried lower risks of both outcomes compared to LCP disease. These findings highlight the need for vigilant follow-up after SCFE, as well as tailored counseling regarding both early and progressive complications.

POSTER #22

Comparative Outcomes of Proximal Stoma Versus Primary Anastomosis in Pediatric Colorectal Trauma

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Introduction: Historically, fecal diversion via proximal stoma (PS) was an accepted option for pediatric colorectal trauma, but recent studies have suggested primary anastomosis (PA) to be safe.

Purpose: This study aimed to compare outcomes between PS and PA, hypothesizing PS will be associated with increased complications but lower mortality compared to PA.

Methods: A retrospective study using the National Trauma Data Bank (2018–2022) was conducted, including ≤18 years old with colon and/or rectal injury who underwent either PS or PA. PS was defined as fecal diversion using a stoma proximal to the colorectal injury; PA was defined as any colon and/or rectal anastomosis without proximal fecal diversion. Descriptive statistics, Kaplan-Meier curves and logistic regression were performed.

Results: A total of 3,511 pediatric patients met inclusion criteria; 548 (15.6%) underwent PS and 2,963 (84.4%) underwent PA. Demographics were similar between groups, though PS patients had higher median ISS (17 vs. 16, p=0.0003) and more rectal injuries (46.7% vs. 8.2%, p<0.0001). PS was associated with longer ICU (5 vs. 4 days, p<0.0001) total hospital stay (12 vs. 8 days, p<0.0001), higher rates of deep space surgical site infection (2.4% vs. 1.6%, p=0.05), return to OR (8.8% vs. 4.7%, p=0.003), and mechanical ventilation use (41.9% vs. 35.7%, p=0.006). Unadjusted mortality was lower with PS (2.6% vs. 5.4%, p=0.0007), but multivariable analysis showed no mortality difference by diversion type; instead, colonic resection/excision independently increased mortality risk (OR 1.02, 95% CI 1.00–1.05, p=0.04).

Conclusions: Despite longer hospitalizations and higher complication rates, PS patients demonstrated lower unadjusted mortality compared to PA. However, after adjustment, diversion type was not independently associated with mortality, and the need for colonic resection/excision emerged as a more important predictor of death.

POSTER #23

Comparative Outcomes of Haploidentical versus Matched Sibling Donor Hematopoietic Stem Cell Transplantation in Pediatric Acute Lymphoblastic Leukemia

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Background: Hematopoietic stem cell transplantation (HSCT) is a potentially curative therapy for pediatric acute lymphoblastic leukemia (ALL). Matched sibling donor (MSD) transplantation has long been the preferred option due to favorable outcomes, but only ~30% of patients have an eligible MSD. Haploidentical (haplo) donors greatly expand access but remain underutilized in pediatrics due to concerns regarding graft-versus-host disease (GVHD) and survival outcomes.

Aim: To compare overall survival and GVHD incidence in pediatric patients with ALL undergoing haploidentical versus matched sibling donor HSCT at a single institution.

Methods: We retrospectively reviewed pediatric patients with B- or T-cell ALL who underwent HSCT at CHOC Children's Hospital between January 2014 and December 2023. Clinical variables included donor type, remission status, and GVHD incidence. A total of 53 transplants were analyzed: 38 haplo and 15 MSD (excluding one MSD due to incomplete data). Statistical analyses included Kaplan–Meier survival estimates, log-rank testing for group comparisons, and chi-square or Fisher's exact tests for categorical variables.

Results: Overall survival at 2 years was 62% for haplo-HSCT and 67% for MSD-HSCT (p=0.74). Event-free survival was similar between groups (58% haplo vs 64% MSD, p=0.69). Incidence of grade II–IV acute GVHD was higher in haplo recipients (32% vs 20%), though not statistically significant (p=0.28). Chronic GVHD rates were comparable (16% haplo vs 13% MSD, p=0.81). Improved survival was observed in the later era (2019–2023: 70% haplo, 75% MSD) compared to earlier years (2014–2018: 55% haplo, 57% MSD).

Conclusions: Our findings suggest haplo-HSCT provides survival outcomes comparable to MSD-HSCT while expanding donor availability. Although MSD remains the gold standard, haplo donors represent a feasible alternative, reducing barriers to timely transplantation. However, interpretation is limited by small sample size, single-center design, and retrospective methodology. Larger multicenter studies are needed to validate these results.

POSTER #25

Optimizing Timing for Asymptomatic CPAM Resection: A PHIS Database Analysis (Phase 1)

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Background: Congenital Pulmonary Airway Malformation (CPAM) is the most common congenital lung malformation. While most patients with CPAM are asymptomatic at birth, an unknown number will later experience CPAM-related complications. There is disagreement regarding its management. Citing the risk of leaving CPAM *in situ*, many advocate for "proactive resection" (PR). Conversely, others favor surveillance with intervention performed only after the development of issues ("reactive resection" or RR).
Using a large, national database that allows longitudinal analysis of CPAM patients, we aim to better understand clinical outcomes that inform the decision-making process of CPAM management. This analysis is the first of a series of 5 planned studies to address the question of whether PR is superior to RR. In phase 1 we will establish a comprehensive CPAM cohort and define short-term outcomes.

Methods: A retrospective cohort study was conducted using the Pediatric Health Information System (PHIS) from 2013-2023. The timing of resection was defined as the patient's age in months at the encounter for resection and was categorized into 5 groups based on clinical rationale: "neonatal" (0–<3 months), "early" (3–<6 months), "standard" (6–<12 months), "later" (12–<24 months), and "delayed" (>24 months). Mortality risk and disease severity at the time of resection were classified using the All Patient Refined Diagnosis-Related Groups (APR-DRG) algorithm, as defined in PHIS. Group demographics and key outcome variables were compared using descriptive and inferential statistics.

Results: A total of 3,329 CPAM patients were captured. Median age at CPAM resection within "neonatal," "early," "standard," "later," and "delayed" groups was 1, 4, 7, 15, and 79 months, respectively ($p<0.0001$). Open operative approach was most common in the neonatal group compared to all others ($p<0.0001$). The presence of high severity level and high mortality risk were greatest in the neonatal group compared to all others ($p<0.0001$) and decreased with age, though a notable increase was observed in the delayed group ($p<0.0001$). This trend was also observed for outcome variables: total length of stay, post-operative length of stay, blood transfusions, post-operative ventilator, and mortality ($p<0.0001$) (see table 1). Median adjusted total cost for resection was highest in the neonatal group ($24,000) followed by the delayed group ($24,000), with a decrease in the early ($19,000), standard ($18,000), and later ($19,000) groups, respectively ($p<0.0001$).

Conclusion: We observe meaningful differences in clinical outcomes after CPAM resection when stratified by age. The youngest (ages 0 to <3 months) and second youngest patients (ages 3 to <6 months) are most likely to have high severity and mortality risk, and are also more likely to require longer hospitalization, blood transfusions, and mechanical ventilation. Their hospital costs and mortality rate are also higher. Interestingly, however, all of these variables decrease with advancing age before a notable spike in the oldest, delayed cohort. The planned next phases of this project will provide additional insight into the optimal management of CPAM patients.

POSTER #26

Systematic Review of Electronic Monitoring Devices to Increase Medication Adherence in Children with Asthma

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Introduction. While asthma can be well-controlled with the use of inhaled corticosteroids (ICS), most children do not adhere to treatment, leading to exacerbations, emergency department visits, hospitalizations, and deaths. Electronic monitoring devices (EMDs) have been shown to increase ICS adherence and improve asthma outcomes, but have not been well-studied in young people. The aim of this systematic review is to determine the feasibility and efficacy of EMDs in children with asthma.

Methods. This review was conducted according to PRISMA guidelines. PubMed and CINAHL were searched using keywords such as asthma, wearable electronic devices, and medication adherence and screened and appraised using Covidence software. Articles were included if they were in English, peer-reviewed, published since 2000, used EMDs for asthma adherence, and included participants under 21.

Results. Fourteen studies met inclusion criteria, with durations ranging from 2 to 12 months. Recruitment rates varied from 28% to 100%, and retention rates ranged from 51% to 100%. Participants found EMDs acceptable in four of six studies and feasible in five of six studies, highlighting ease of use and perceived benefits. Six of the 12 studies measuring ICS adherence reported improvements using EMDs with feedback compared to monitoring alone. Seven out of nine studies found improvements in asthma control, and four out of seven found reduced emergency department visits and hospitalizations. These findings highlight the overall efficacy of EMDs.

Conclusion. EMDs can improve ICS adherence in children with asthma, especially with sustained feedback. However, inconsistent results highlight the need for comprehensive approaches addressing behavioral and systemic factors.

Keywords: Asthma, Pediatrics, Treatment Adherence and Compliance, Medication Adherence, Wearable Electronic Devices, Digital Technology, Medical Electronics

POSTER #27

Reducing Cellular Adhesion on Shunt Catheters Using Zwitterionic Surface Modification: A Bioreactor-Based Study

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INTRODUCTION: Given that the dominant cause of shunt malfunction is catheter obstruction by cells, numerous studies have explored the potential to reduce cellular attachment through the modification of catheter surface chemistry. Zwitterionic coatings, given strong hydrophilic properties and resistance to protein and cell attachment, are excellent candidates for further study.

METHODS: Commercial shunt catheters were compared with and without surface treatment using a zwitterionic coating. Polysulfobetaine (PSB), a zwitterionic polymer known for its antifouling properties, was used as a surface treatment for shunt catheters made from polydimethylsiloxane (PDMS). To covalently attach PSB to the PDMS surface, it was combined with perfluorophenylazide (PFPA), a photoreactive crosslinker that enables stable bonding upon UV activation. Experiments were conducted using a bioreactor developed in our lab, designed for testing shunt catheters. It uses a micropump to circulate media containing cells through a catheter in a closed loop system. Astrocytes were exposed to a flow rate of approximately 300 microliters per minute for 16 hours. After this period, the catheters were fixed, stained with 4',6-diamidino-2-phenylindole (DAPI), and immunolabeled for glial fibrillary acidic protein (GFAP) to visualize the cells attached to catheters.

RESULTS: The experiments revealed a clear difference in cell attachment between the commercial catheters and those surface-treated with a zwitterionic coating. The surface-treated catheters exhibited markedly reduced astrocyte adhesion compared to the untreated commercial catheters. Ongoing experiments are currently assessing the attachment of additional cell types, including microglia.

CONCLUSIONS: These results suggest that zwitterionic surface-treated catheters may offer a promising strategy to reduce cell attachment. Longer term studies with additional cell types, along with further testing to assess durability and performance, are needed to fully evaluate the coating's biological interactions and long-term performance under conditions that better mimic the *in vivo* environment.

POSTER #28

Association between Brain Volumes at Term-Equivalent Age and Neurodevelopmental Outcomes in Very Preterm Infants: A Systematic Review and Meta-Analysis

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Background: Very preterm birth (VP; less than 32 weeks gestational age [GA]) and very low birth weight (VLBW; less than 1500 grams) are associated with high rates of neurodevelopmental delays in childhood (1, 2). Magnetic resonance imaging (MRI) taken at term-equivalent age (TEA; between 36 and 42 weeks) has emerged as a promising tool for identifying brain dysmaturation and neurodevelopmental outcomes in infants born VP (3, 4).

Aim: This review aims to assess whether MRI brain volumes at TEA are associated with neurodevelopmental outcomes (cognition, language, behavior, and motor) in children born VP or VLBW aged between 2 and 18 years.

Methods: Studies are searched for using the following inclusion criteria: [1] VP and/or VLBW infants who [2] had brain volumes assessed at TEA using structural brain MRI and [3] had a follow-up between 2 and 18 years of age with [4] age-standardized neurodevelopment testing assessing cognition, behavior, language, and/or motor function. The following databases will be searched: PubMed, Scopus, and Web of Science. Meta-analyses are planned if data from five or more studies can be pulled.

Results: 1426 studies were identified through abstract and title search. 147 moved to full-text review, and 33 studies were eligible for data extraction. Analysis of the pooled data will be completed by the end of October.

Conclusion: This study addresses the relationship between early brain development and later neurodevelopmental outcomes in children born VP or VLBW, which may have implications on our understanding of the neurological mechanism for later challenges observed in this population.

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POSTER #29

Retrospective review of sleep in children with a history of HIE

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Sleep disturbances have only recently been identified as common sequela of hypoxic ischemic encephalopathy (HIE). The pathophysiology of sleep disturbance in survivors of HIE may relate to the known vulnerability of several brain regions that have been shown to be highly sensitive to hypoxic injury, such as the pineal gland and hypothalamus. As impaired sleep is associated with memory and attention difficulties, sleep could be a target for intervention to improve neurodevelopmental outcomes in children with HIE. A gap in this knowledge limits such rational approaches.

Sleep ontogenesis is the development of sleep patterns, which is an active process in the first few months of life. Most children undergo sleep consolidation in the first few months of life, which involves increasing duration of night time sleep and decreasing day time naps. Children who experienced a perinatal brain injury, may have difficulty in developing maturation of sleep behaviors. We hypothesize that HIE leads to altered sleep ontogenesis. We anticipate that behavioral differences in sleep in those with history of HIE will have immature sleep consolidation with more frequent night time awakenings, more day time naps, and shorter longest sleep duration at night, compared to normative data for infants in the first 12 months of life measured through actigraphy.

POSTER #30

The Utility of Antifungal Prophylaxis to Prevent Invasive Fungal Disease in Pediatric and Adolescent Young Adults with Hematologic Malignancies

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Background: Invasive fungal disease (IFD) causes significant morbidity and mortality in pediatric and adolescent/young adult cancer patients. Guidelines recommend antifungal prophylaxis in certain high-risk groups.

Methods: A retrospective study was performed at CHOC Children's Hospital and Lucile Packard Children's Hospital to evaluate the utility of antifungal prophylaxis in preventing IFD. This included patients with acute lymphoblastic leukemia (ALL), acute myeloid leukemia (AML), and recipients of hematopoietic stem cell transplant aged 0 to 26 years who received antifungal prophylaxis January 2014-December 2021 and developed an IFD.

Results: We identified 81 cases of IFD in 70 patients, which included 34 B-ALL/lymphoblastic lymphoma (LLy), 21 AML, 9 T-ALL/Lly, 3 infant ALL, and 1 mixed phenotype ALL. Mean age was 13 years, 57% were male, 59% were Hispanic, and 29% were overweight/obese. 64.2% with IFD received antifungal prophylaxis. The incidence of organisms included *Candida* spp 29/70 (41%), Mucorales spp 15/70 (21%) and *Aspergillus* spp 13/70 (19%). Overall survival (OS) at 6 months after IFD was 75.7%. There was a trend toward improved OS in patients receiving anti-mold versus those receiving anti-yeast or no prophylaxis. Mortality at 6 months after IFD was 46.2% for *Aspergillus*, 33.3% for Mucorales, and 13.8% for *Candida*. In the univariate analyses, body mass index (BMI) and *Aspergillus* infections were associated with increased mortality (hazard ratio 1.05 per1-unit increase in BMI, p=0.04; hazard ratio 4.11, p=0.03, respectively). In the multivariate analyses, BMI and Mucorales infections were associated with increased risk of death (hazard ratio 1.10, p=0.02; and hazard ratio 7.49, p=0.02 respectively). Receiving anti-mold prophylaxis was associated with decreased risk of all-cause mortality (hazard ratio 0.08, p=0.04).

Conclusion: We did not find significant impact of anti-fungal prophylaxis on OS in patients with IFD. However, there was improved survival in patients with IFD who received anti-mold prophylaxis versus those who received anti-yeast or no prophylaxis.

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POSTER #31

Serial Brain MRI Volumetrics and DTI Analysis in Atypical CLN2 Patients Treated with ICV Cerliponase Alfa

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Ceroid Lipofuscinosis Type 2 (CLN2) is caused by inherited tripeptidyl peptidase 1 (TPP1) enzyme deficiency. The main treatment is a biweekly intracerebroventricular enzyme replacement therapy (ICV-ERT) of recombinant human TPP1 (cerliponase alfa). Classical CLN2 patients show consistent symptom onset at ages 3-4, while atypical patients (aCLN2) have later and more varied symptoms. This study utilized longitudinal brain imaging in ICV-ERT treated aCLN2 subjects to determine how central nervous system (CNS) gray and white matter responds to cerliponase alfa.

Five aCLN2 subjects (ages 14-21) were enrolled, undergoing longitudinal brain magnetic resonance imaging (MRI) scans which included T1-weighted imaging and diffusion tensor imaging (DTI) sequences. MRI scans from twelve age- and sex-matched healthy controls from the Human Connectome Project image database were utilized as comparators.

MRI volumetric analysis revealed aCLN2 subjects had significantly smaller mean volumes of the whole brain (-22±8%), cerebral white matter (WM) (-25±10%), cerebellar WM (-23±11%), and corpus callosum (-26±15%), as well as -35±10% smaller cerebellar cortex, compared to controls. Conversely, mean aCLN2 subject ventricular volume was 50±36% larger compared to controls.
DTI fractional anisotropy (FA) group analysis was significantly lower in the aCLN2 subjects compared to the controls in 37 out of 48 WM regions (-10±4%), indicating reduced WM integrity. DTI radial diffusivity (RD) group analysis was significantly higher in the aCLN2 subjects than in the controls in 44 of 48 WM regions (39±1%), which is concordant with the abnormal FA analysis and demonstrates progressive WM abnormalities.

The longitudinal MRI/DTI findings from this cohort of 5 aCLN2 subjects suggest that cerebral and cerebellar atrophy progresses in conjunction with global deterioration of WM tracts throughout the CNS despite treatment with cerliponase alfa. These findings must be replicated in larger longitudinal cohorts of aCLN2 subjects and should also be assessed in younger, classical CLN2 patients.

POSTER #32

Pain Score Discordance Among Pediatric Patients, Parents, and EMS Providers

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Background: Over a third of patients transported via Emergency Medical Services (EMS) report acute pain and over half of these endorse severe pain. However, only a small fraction of patients receive pain medication. Pain assessment is critical to pain management thus it is crucial to evaluate pain assessment accuracy.

Purpose: In this study we sought to compare pediatric pain with caregiver and EMS pain assessments.

Methods: Patients aged 0-17 years who were transported by EMS to an Emergency Department (ED) at a free-standing children's hospital were prospectively enrolled. Pain scales (FLACC, FACES, or numeric) were completed based on patient age within five minutes of arrival. Simultaneously, the patient's caregiver and EMS clinician estimated the patient's pain using a visual analogue scale. The intraclass correlation coefficient (ICC) was used to assess agreement between the raters. Chi-square and one-way ANOVA were used to evaluate correlations between predictor variables and pain rating agreement.

Results: We recruited 250 patient and EMS clinician dyads; of these, 114 patient-caregiver dyads were also enrolled. The ICC for EMS-child pain ratings was 0.426 ($p<0.001$); the ICC for child-caregiver pain ratings was slightly higher in agreement with an ICC of 0.429 ($p<0.001$). Nearly half (49.6%) of EMS clinicians were accurate in their rating, 18.0% overestimated patient pain, and 32.4% underestimated. A similar proportion of caregivers were accurate, though more overestimate patient pain (36.8%), while fewer underestimated pain (17.5%). Patient age, sex, race, ethnicity, language, insurance type, ED length of stay, ED emergency severity index were not associated with accurate, under, or over-estimation of child pain.

Conclusion: Overall, we found a low level of agreement between patient reported pain and EMS and caregiver pain assessments. EMS clinicians more frequently underestimated pain, while caregivers generally overestimated pain. These findings suggest that working towards improving prehospital pediatric pain assessment may offer opportunities for improved prehospital pain management.

POSTER #33

EMS Use of the Pediatric Assessment Triangle in the Prehospital Environment

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Background: EMS clinicians often report a lack of training and experience with children, leading to discomfort regarding assessment and treatment. The Pediatric Assessment Triangle (PAT) was designed to provide a rapid and standardized approach. Despite widespread adoption, previous literature examining EMS implementation of the PAT remains limited.

Purpose: To further explore PAT use in the real-world prehospital setting.

Methods: This was a retrospective cohort study of pediatric patients aged <15 years who were transported to a quaternary care pediatric Emergency Department (ED) via EMS between October 2022 and November 2023. Data were abstracted from EMS and ED charts and analyzed using counts and percentages, logistic regression, chi-square, and McNemar's test.

Results: A total of 2,929 patients were included. The majority (65.9%) had a PAT score of 0. Among those with non-zero PATs, abnormalities in appearance were most prevalent (50.7%). A PAT ≥1 had marked higher odds of transport via Advanced Life Support (OR 67.93; 95% CI 32.02, 144.13) compared to a PAT of 0. Most patients (62.2%) received an EMS intervention; the most common was diagnostics (blood glucose or EKG) and transported patients were administered medications (22%). PAT scores of ≥2 were associated with double the odds of admission to the floor (OR 2.09; 95% CI 1.42, 3.07) and quadruple the odds of admission to ICU level of care/direct to surgery/expired (OR 4.92; 95% CI 2.90, 8.35); PAT's only abnormal for work of breathing were associated with increased odds of admission to floor level of care (OR 2.57; 95% CI 1.8, 3.66).

Conclusions: This study suggests that EMS PAT assessment in the field appropriately reflects patient stability and may be associated with EMS intervention en-route. EMS PAT scores also demonstrate promise as an adjunct to ED assessment, alerting providers to the increased likelihood of admission and possibly assisting in earlier mobilization of resources.

POSTER #34

Climate Anxiety among Adolescents in a Pediatric Emergency Department

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Background: A changing climate indirectly threatens health by acting as a stressor from perceived threats to well-being. Few studies assess youth-centered climate anxiety in clinical settings. The emergency department (ED) is a prominent source for adolescent mental health triage.

Purpose: The primary objective was to understand demographic and clinical predictors of climate anxiety among ED pediatric patients. Secondarily, to externally validate an abbreviated version of the climate change anxiety scale (CCAS).

Methods: In this cross-sectional study, data was collected from 2023 to 2024 in a single-center pediatric ED, with 860 patients ages 12-17 years approached via convenience sampling; 116 did not complete study surveys. Exposures included demographic factors (age, gender, race, ethnicity, insurance payor), chief complaint type (mental health or medical), temporal season, and general anxiety disorder-7 (GAD-7) score. The main outcome was climate anxiety, measured with the CCAS.

Results: We recruited 744 participants, 79.8% (n=594) presented with medical complaints and 28.1% (n=150) reported climate anxiety. Females had 1.49 times higher odds of experiencing greater climate anxiety compared to males (95% CI 1.05, 2.12), p=.027. Having public health insurance increased the odds of experiencing climate anxiety (OR=1.82, 95% CI 1.21, 2.73), p=.004, as well as warm seasonality (OR=1.51, 95% CI (1.07, 2.13), p=.018. Participants with moderate or severe GAD-7 had five times higher odds of elevated climate anxiety than those with minimal generalized anxiety (p<.001). The CCAS-short form (CCAS-S) demonstrated high sensitivity (91.4-96.8%), specificity (85-95.5%) in detecting mild to severe levels of distress compared to the full CCAS. A four-tiered threshold of CCAS-S severity showed excellent reliability (quadratic weighted kappa = 0.838).

Conclusions: We identified clinical predictors of climate anxiety and externally validated the CCAS-S in a large pediatric cohort. Given the increasing impact of the changing climate on human health, we advocate utilizing the tool alongside routine pediatric mental health assessments.

POSTER #35

Health Care Transition for Adult-Aged Patients with Medical Complexity: Caregiver Perspectives and Experiences in the Pediatric Emergency Department

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Background: Less than 20% of children with medical complexities receive services to facilitate adult health care transition. Limited time, resources, and emergency planning are barriers to providing transition care within the pediatric emergency department (PED). Yet, adult-aged patients with medical complexity (PMC) frequently return to PEDs for acute care presenting with case management challenges.

Purpose: We sought to understand perspectives and barriers to transition of care in adult-aged PMC presenting to a PED.

Methods: English- and Spanish-speaking caregivers of PMC (18+ years) who presented to a PED completed a survey examining their perspectives of seeking care in a general ED (GED) compared to PEDs. Participants rated agreement with statements about the PED vs. GED using a 7-point Likert scale. They also indicated their experience with transition planning. Patient demographics, chief complaint, arrival method, emergency severity index (ESI), and ED disposition were extracted from the medical record.

Results: Sixty-six caregivers completed the survey from 10/08/24- 03/27/25. Average patient age was 19.8 years, 53% were female, 63.6% utilized public insurance, and 42.4% were admitted to the hospital. Half reported they had never taken their child to a GED before. Participants rated "Trust in all staff members" as the highest endorsement for motivations to seek care at the PED. Participants rated the PED higher in cleanliness, nursing skill level, clinician knowledge and communication, diversity of staff members, clinician inclusion of caregiver in medical decisions, and treatment of the patient's pain. Most caregivers reported that medical providers had spoken with them about transition to adult care but had not initiated the transition.

Conclusions: Adult-aged PMCs in the PED tend to have high resource care requirements, yet many have not initiated transfer to adult care. Caregivers reported greater levels of trust in the pediatric care team. These findings may help optimize transition of care programs in pediatric settings.

POSTER #36

An Adolescent-Caregiver Dyad Approach to Climate Anxiety in the Pediatric Emergency Department

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Background: Climate change is a recognized social driver of health with potential detrimental effects on the mental health and wellbeing on adolescents and adult caregivers alike. Household-level perceptions of environmental challenges may amplify individual climate-related anxieties, yet little research explores climate change anxiety in adolescents nor the potential for transmission within caregiver–adolescent dyads.

Purpose: This study aims to identify community-level and global issues contributing to climate anxiety while examining associations between caregivers' community-level concerns and climate anxiety and adolescent patient climate anxiety.

Methods: We conducted a single-center, cross-sectional study of adolescent (12–17 years) and caregiver dyads presenting to a pediatric emergency department with mental health concerns between January and December 2024. Both pediatric patients and adult caregivers completed the Generalized Anxiety Disorder-7 (GAD-7) and Climate Change Anxiety Scale (CCAS) to assess general anxiety and climate anxiety respectively. Caregivers were additionally asked to report their perceptions of domestic and global issues and community-level environmental concerns.

Results: A total of 557 dyads completed CCAS surveys, with low within-dyad agreement (ICC=0.224, 95% CI 0.142–0.274). Moderate-to-severe GAD scores strongly predicted climate anxiety for both adolescents and caregivers (ORadj 6.59, 95% CI 3.52–12.30, p<0.001) and caregivers who rated climate change as "very important" had higher odds of moderate-to-severe CCAS scores (ORadj 4.50, 95% CI 2.03–9.98, p<0.001). However, caregiver evaluation of climate change were not significantly associated with adolescent CCAS scores (p=0.172). ICC=0.224, 95% CI 0.142–0.274).

Conclusion: Moderate-to-severe generalized anxiety is a strong predictor of climate anxiety in both caregivers and adolescents, despite lack of association within dyad attitudes regarding climate change. Targeting generalized anxiety through clinical interventions may help mitigate climate-related anxiety among adolescents.

POSTER #37

Assessing the Financial Burden of Congenital Diaphragmatic Hernias from Diagnosis through Childhood

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Objective: Research on Congenital Diaphragmatic Hernia (CDH) has emphasized clinical outcomes, with limited data on family financial burden. This study examines economic impact from diagnosis through childhood.

Methods: A REDCap survey was distributed via a national CDH Facebook group to assess family-reported costs, insurance coverage, debt, work disruption, and psychosocial impact.

Results: Nineteen parents completed the survey. Most were married (84%) and had other children (58%). All children were alive, underwent CDH repair, and 58% received ECMO. Left-sided defects were most common (79%). Prolonged NICU stays were reported, with 39% hospitalized >3 months. Total medical bills exceeded $1M for 63%. Despite 84% having private insurance, 42% were still paying off NICU debt, 58% reported ongoing costs (e.g., therapies, specialists), and 79% experienced moderate to extreme financial strain during hospitalization; 47% continued to report strain. Employment was affected in 63% of households, and 90% reported lost income. Facebook influenced care decisions in a minority—16% chose a different surgeon based on posts; median influence rating was 0/5. Still, 93% who traveled >1 hour for care would do so again.

Conclusion: Families of children with CDH face substantial financial and employment burdens despite insurance, highlighting unmet support needs beyond clinical care.

POSTER #38

TIMING OF COLECTOMY AND SURGICAL OUTCOMES IN PEDIATRIC PATIENTS WITH ULCERATIVE COLITIS

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Background: Ulcerative colitis (UC) is a chronic inflammatory condition of the colon that presents more aggressively in pediatric patients compared to adults, often necessitating earlier and more frequent surgical intervention. This study evaluates the impact of colectomy timing on outcomes, hypothesizing that earlier surgery leads to improved clinical outcomes.

Methods: A retrospective study was conducted using the 2004–2022 Pediatric Health Information System (PHIS). A total of 1,147 patients ≤18 years old with UC who underwent colectomy were included. Timing of colectomy was defined by the interval between UC diagnosis and surgery, and patients were grouped into quartiles: A (<1 month), B (1–6 months), C (6–24 months), and D (>24 months).

Results: Median age increased progressively from group A (13.8 years) to D (16.7 years) ($p<0.0001$). Disease severity was highest in group A (47.5%) and declined across groups ($p<0.0001$). Biologic use was lowest in group A (32%, $p<0.0001$). Steroid use was highest in groups C and D (>98%) and lowest in group A (76%) ($p<0.0001$). Elective surgery was most frequent in group D (25.2%) and rare in group A (2.5%) ($p<0.0001$). Median length of stay and return to the OR were highest in group A (9 days, 35.8%, respectively; $p<0.0001$). Mortality risk was highest in group A (11.7%) and decreased with delayed colectomy ($p<0.0001$).

Conclusions: Colectomy within one month of diagnosis is associated with worse outcomes, likely reflecting higher disease severity. Further analysis is needed to guide optimal timing of surgical intervention in pediatric UC.

POSTER #39

Typology of Legal Intervention Trauma Among Pediatric and Young Adult Decedents: A Latent Class Analysis

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Background: Legal intervention trauma (LIT), referring to injuries sustained during encounters with law enforcement (LE), is an understudied public health concern in pediatric populations. We aimed to better understand the circumstances surrounding LIT-related deaths among children and young adults. Using latent class analysis (LCA), we classified cases into typologies and evaluated disparities by age, race, mental health, incident characteristics, and LE use of force.

Methods: The National Violent Death Reporting System (NVDRS) is a CDC database collecting information on violent deaths from 50 U.S. states, the District of Columbia, and Puerto Rico. We analyzed LIT cases among decedents aged ≤26 years from 2003–2022. Thirteen LIT-specific variables were abstracted from coroner/medical examiner and LE narratives by two independent reviewers, with discrepancies resolved by a third. LCA was applied to classify cases by incident features and LE actions. A four-class model was selected based on lowest Bayesian Information Criterion (BIC = 20,349).

Results: Among 1,528 decedents, four typologies emerged. Class 1 (6.9%) involved decedents with mental health crises and substance use, with minimal aggression toward LE. Class 2 (14.2%) comprised domestic disturbances with mostly unarmed decedents. Class 3 (54.9%) included armed, aggressive individuals. Class 4 (20.0%) involved nonviolent, often unarmed individuals, disproportionately Black (44.0%) and frequently stopped for traffic violations. Hispanic decedents were overrepresented in this class (21.4%).

Conclusions: Distinct typologies of LIT-related deaths exist among youth, with racial disparities evident, particularly in low-threat encounters. Findings support reforms including de-escalation training, mental health response alternatives, and bias mitigation.

POSTER #40

Recent Advancements in Treatment in Pediatric Diabetes and Endocrinology

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Background: Pediatric Endocrinology encompasses many lifelong chronic conditions. We celebrate a number of approved treatments that have improved the care for individuals with T1DM, Stage 2 T1DM, CAH and hypothyroidism from 2022 to 2025 here at Rady Children's Health Orange County.

Purpose/Aims: CHOC Endocrine Research Team and CHOC subjects contributed to groundbreaking research that resulted in pediatric FDA approved treatments, devices and medication for these conditions. In some cases, this represents new treatments for pediatric conditions for which few advancements were available in the last 20 years.

Methods: Our poster shows completed trials with FDA approved treatments in pediatrics and a validated Child Health survey for Diabetes.

Results: Child Health Rating Inventory CHRIS - Diabetes specific **FDA Approved treatments:**

- Tzield teplizumab disease-modifying therapy in type 1 diabetes
- Afrezza ultra rapid acting inhaled insulin in T1DM and T2DM
- T1DM-insulin lispro-aabc rapid acting insulin in Tandem IIS Closed Loop
- Tildacerfont Children with CAH
- Tirosint-Sol in pediatric congenital Hypothyroidism

Conclusions: The results of these treatments will become apparent as standards of care are updated and efficacy is reported. We expect these innovations will result in overall improvement in endocrine pediatric health, relieving the burden of endocrine related chronic illness, reducing complications and increasing quality of life while simultaneously saving healthcare resources.

POSTER #41

Child Restraint System Use and Injury Severity in Pediatric Motor Vehicle Collisions: Insights from the NTDB

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Background: Motor vehicle collisions (MVC) are the second leading cause of childhood mortality in the US. Child restraint systems (CRS) remain underutilized, often due to lack of access and education. However, the full extent of underutilization has not been clearly delineated in the trauma population.

Aims: We sought to examine differences in demographics and clinical characteristics in pediatric patients who presented to the emergency department (ED) after MVC with or without CRS.

Methods: We identified 4666 MVC trauma patients reported within the National Trauma Data Bank (NTDB) <8 years of age and ≤ 40 lbs. Patients were stratified by reported use of CRS. Bivariate descriptive analyses, chi-square tests of proportions, and a Wilcoxon rank-sum test were used to perform our analyses.

Results: In all patients, median age for those without CRS was older (6 years, IQR 4-7) than those with CRS (4 years, IQR 2-5, $p<0.0001$; Table 1). A lower proportion of Black patients had CRS when compared to White patients (24.0% vs. 32.6%, $p<0.0001$). Injury severity score (ISS) was higher in the non-CRS group (8, IQR 4-14) than CRS group (5, IQR 2-11, $p<0.0001$). A larger proportion of patients with CRS were discharged home from the ED without services when compared to those without CRS (26.8% vs. 18.5%). Racial disparities persisted in older patients, with fewer Black children who were booster-eligible in a CRS (30.0% vs 17.9%, $p<0.001$) when compared to all other races.

Conclusions: This study demonstrates that older and black children were less likely to be in a CRS, and that those who were not in CRS were more severely injured. Parents may not be adequately informed about CRS use for older and larger children, which elucidates a need for trauma system educational tools to address these disparities to follow national guidelines.

POSTER #42

Comparison of the Auditory Environment in Premature Infants by Room Type and Postmenstrual Age in a Dedicated Small Baby Unit

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Background: The environment within the NICU is characterized by noxious exposure (harsh electronic noise of monitors, alarms, respiratory equipment etc.), and a deficiency of meaningful language often replaced by excessive silence. The auditory environment in the NICU appears to impact brain development of the auditory cortex with implications for future language development.

Purpose/Aim: Analyze auditory environment in a dedicated hybrid Small Baby Unit (SBU) and compare our findings to those from the Brigham and Women's Hospital (BWH-unpublished), Carle Foundation Hospital (UIUC), and St. Louis Children's Hospital (WUSTL).

Design/Methods: Prospective observational study recruited infants with gestational age (GA) <30 weeks at birth and admitted within 7 days of life. Perinatal demographics and environmental factors (crib vs isolette, open bay vs private room, parental presence) were recorded. Biweekly 16-hour auditory recordings were collected using the LENA Device (LENA Research Foundation, Boulder, Colorado) for 8 weeks with recordings staggered to reflect a 7-day week. Data was analyzed via LENA Advanced Data Extractor (ADEX) to determine meaningful language, TV/electronic noise, non-biological noise and silence.

Results: To date, 17 infants (GA=26 weeks, SD=2.03) have been recruited, totaling over 2500 hours of auditory data. Our cohort was comparable to previous studies (Figure 1), documenting a deficiency in meaningful language (29 min/16 hrs) and an excess of silence and non-biological noise. Preliminary analysis of our cohort reveals a trend towards increasing meaningful language with postmenstrual age (PMA)—less than 16 minutes at <34 weeks PMA versus greater than 45 minutes at >34 weeks PMA.

Conclusion: Our study continues to reinforce that the auditory environment of the preterm infant is detrimental in its deficiency of meaningful language and excessive silence/noise. Although these findings have been known for over a decade, little change has occurred. We aim to complete recordings of additional preterm infants to further confirm results and compare across PMA and room type.

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POSTER #43

Dance of the dura: Why does neuromonitoring trigger contraction during spinal lipoma excision?

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Background: Intradural spinal lipomas are a form of closed dysraphism and a frequent cause of tethered cord. Although usually adipocyte-predominant, rare lesions may contain additional histologic elements that can yield unexpected intraoperative behavior.

Observations: A 13-year-old boy presented with progressive right foot deformity, distal weakness, gait limitation, and a low conus at S1–S2 with a large terminal lipoma on MRI. He underwent detethering with debulking and L5 osteoplastic laminoplasty under intraoperative neuromonitoring (IONM). During microsurgical dissection, a flesh-colored band along the right aspect of the lipoma appeared unlike a nerve root. Targeted stimulation produced visible contraction within the thecal sac. The segment was isolated and divided under IONM guidance. Histopathology confirmed benign skeletal muscle fibers intertwined with lobulated adipose tissue and fibrovascular elements of the filum terminale, consistent with a myolipoma. The patient had no postoperative sensory changes and progressed satisfactorily.

Lessons: Electrically evoked contraction is a physiologic signature suggestive of myolipoma and can refine the extent of safe untethering. IONM adds value beyond evoked-potential mapping by eliciting myogenic contraction and helping discriminate resectable, non-neural elements from neural structures.

POSTER #44

Bridging Gaps in Emergency Care: A Clinical Pathway to Improve Healthcare Transition for Medically Complex Adults

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Background: Transition from pediatric to adult healthcare remains a challenge for medically complex adults. Pediatric emergency departments (PEDs) often serve as points of re-entry into pediatric systems, perpetuating fragmented care and delaying transition to adult services. Integrating transition-focused interventions within the PED is complex due to competing demands but represents a critical opportunity to improve care continuity.

Purpose: This quality improvement project evaluated the feasibility and utility of implementing a clinical pathway (CPW) within the PED to support transition services for medically complex adults.

Methods: This project included adult patients (≥18 years) who presented to a PED at a stand-alone pediatric hospital. A structured CPW was developed to standardize screening, identify medically complex patients eligible for the transition clinic, offer transition education, or connect patients with external adult providers when specialty consultation was needed.

Results: A total of 118 adults were screened over 10 weeks, identifying 115 unique patients. CPW utilization reached 100% by Week 9 and was maintained through project completion. Of the 115 patients screened, 85 (73.9%) were excluded due to absence of a chronic condition (50.4%) or presence of a single chronic condition (16.5%). Patients meeting criteria for medical complexity (n=27, 23.5%) were referred to a transition clinic. Among those referred, 100% had ongoing pediatric subspecialty oversight, 96.3% lacked a documented transition readiness assessment, and 40.7% required inpatient admission. Nine patients (33%) completed a transition clinic visit by project conclusion.

Conclusion: Medically complex adults frequently return to the PED due to continued dependence on pediatric specialists and insufficient transition planning. While the PED is designed for emergency management, it also represents a critical gateway to comprehensive transition planning. By optimizing clinical pathways and intentionally prioritizing transition services, healthcare systems can reduce the burden on PEDs, improve care continuity, and lower healthcare utilization costs.

POSTER #45

Redefining Patient-Centered-Care in Pediatric Endocrinology

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Background: Through PI-initiated studies our Endocrinology team study critical gaps in research that address the unique challenges of pediatric populations. These studies assess the efficacy of standard of care AND ***go beyond*** by studying factors that influence patient outcomes such as social-determinants-of-health, health-related quality of life and individual risk factors to personalize patient care.

Purpose/Aims:

- ***Review of Hormone Replacement Treatment in patients with Turner Syndrome (TS)***
  - Compares outcomes of treatments in patients with TS with Premature Ovarian Insufficiency through the lens of menstrual regulation and metabolic changes related to HRT in adolescents.
- ***Metabolic Profile of Early-Onset Obesity***
  - Children with obesity have increased risk of cardiovascular and metabolic disorders. This study aims to improve early detection of individual genetic risk factors and tailor treatment accordingly.
- ***Health-Related-Quality-of-Life in Pediatric Thyroid Cancer Patients***
  - Pediatric thyroid cancer (TC) is the most common pediatric endocrine neoplasm. This study adapts the adult EORTC-Thy34 to improve assessment of physical functioning, emotional well-being, and social functioning of pediatric patients.
- ***Demographic Analysis of the Relationship Between Polycystic-Ovary-Syndrome and T2DM***
  - Long-term complications of PCOS disproportionately affect adolescent girls and women of ethnic minorities. This study looks at genetic, environmental, cultural, and socioeconomic factors that influence disease development.

Methods: These ambispective chart review studies used CHOC's patient pool. Variables analyzed included treatment history, laboratory data, genetic testing, family history, and patient-reported outcomes.

Results:

1. Optimization of treatment adherence for overall health.
2. Advance knowledge in genetic pathways to tailor treatment.
3. Further understanding of disease's influence on patients' wellbeing.

Conclusions: The studies in these endocrinopathies embody **the future of health care, one which is individualized, culturally humble, and holistic, which not only advances science but redefines what patient-centered care looks like.**

POSTER #46

Comparative Efficacy of Proton Pump Inhibitors in Pediatric Eosinophilic Esophagitis: A Retrospective Review

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Background: Eosinophilic esophagitis (EoE) is a chronic, immune-mediated disease characterized by esophageal dysfunction and eosinophilic inflammation. In the US the prevalence of pediatric EoE is 29.5 per 100,000, with higher incidence among Caucasian males. Diagnosis requires esophagogastroduodenoscopy (EGD) demonstrating ≥15 eosinophils per high-power field (eos/hpf). Proton pump inhibitors (PPIs) are the first line of therapy, with dietary elimination and topical corticosteroids as alternatives. Among the commonly used PPIs (Esomeprazole, Lansoprazole, and Omeprazole), a registry study reported histologic remission in 75.5%, 59.1%, and 64.9% of patients, respectively. However, standardized data on comparative efficacy remains limited. This study aimed to compare histologic response rates among these three PPIs in pediatric EoE.

Methods: We conducted a retrospective review of children seen in CHOC Children's EoE Multidisciplinary Clinic between 2014 and 2023. Eligible patients had ≥15 (eos/hpf) on initial EGD and received ≥8 weeks of PPI monotherapy (Lansoprazole, Esomeprazole, or Omeprazole). Histologic response was defined as <15 eos/hpf on follow-up EGD. Chi-square tests were used to compare response rates among Lansoprazole, Esomeprazole, and Omeprazole ($p < 0.05$). Bayesian analysis was used to estimate the probability of treatment differences.

Results: Of the 482 charts reviewed, 183 patients met inclusion criteria (Lansoprazole: n = 16, Esomeprazole: n = 66, Omeprazole: n = 72). Histologic response rates were 35.6% for Lansoprazole, 43.9% for Esomeprazole, and 27.8% for Omeprazole. Pairwise chi-square test revealed no significant differences between Lansoprazole and Esomeprazole ($p = 0.43$) or Lansoprazole and Omeprazole ($p = 0.41$). A borderline significant difference was observed between Esomeprazole and Omeprazole ($p = 0.05$). Bayesian analysis estimated a 97.5% probability that Esomeprazole has a higher histologic response rate than Omeprazole.

Conclusion: Among the three PPIs, Esomeprazole demonstrated the highest response rate suggesting it may be the most effective PPI for inducing histologic remission in pediatric EoE.

POSTER #47

Predicting Asparaginase Activity in Pediatric Leukemia: The Role of Fibrinogen and Antithrombin III

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Background: The addition of asparaginase has significantly improved survival in pediatric acute lymphoblastic leukemia (ALL). However, the development of silent inactivation, the formation of neutralizing antibodies without overt allergic reactions, can lead to subtherapeutic asparaginase activity and treatment failure. While direct measurement of serum asparaginase activity is the gold standard, it is not widely available in clinical practice. This study aimed to evaluate whether fibrinogen and antithrombin III (ATIII) levels could serve as surrogate markers for detecting silent inactivation.

Methods: Fibrinogen and ATIII levels were measured prior to and 7 days after asparaginase administration. Asparaginase activity levels were obtained on day seven post-infusion. Fisher's exact test was used to assess the relationship between asparaginase activity and surrogate marker levels. ROC curve analyses were performed to evaluate the diagnostic performance (AUC) of Δ and % change in fibrinogen and ATIII in detecting silent inactivation. Statistical significance was set at $p < 0.05$.

Results: Silent inactivation occurred in 5.9% (4/68) of treatments. It was significantly more likely when biomarker changes from Day 0 to 7 were below key thresholds. Fibrinogen Δ≤30 mg/dL: 17.6% vs. 2.0% (p=0.46); fibrinogen % change <25%: 17.4% vs. 0% (p=0.011), AT3 Δ≤50%: 13.8% vs. 0% (p=0.029); ATIII % change <38%: 16.7% vs. 2.0% (p=.054). ROC curves demonstrated good discriminatory ability of fibrinogen and ATIII changes in identifying silent inactivation: AUC=0.848 and AUC=0.840, respectively. Fibrinogen % change <25% and ATIII% change <38% each achieved 100% sensitivity and 100% negative predictive value (NPV).

Conclusion: Decreased fibrinogen and ATIII levels correlate with therapeutic asparaginase activity and may help identify patients at risk of silent inactivation. These markers may serve as accessible, cost-effective tools to flag subtherapeutic responses and guide further evaluation.

POSTER #48

Intersecting Inequities: Neighborhood Deprivation, Psychological Distress, and Financial Burden Among Pediatric and AYA Patients with a Solid Tumor Diagnosis and Their Caregivers

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Background: A cancer diagnosis places substantial stress on both patients and caregivers. While caregiver burden in pediatric leukemia is well-documented, less is known about families facing pediatric and adolescent/young adult (AYA) solid tumors, which often require intensive, multidisciplinary care. This study aims to further characterize the associations between area deprivation, perceived financial burden, and psychological distress in a diverse cohort of pediatric and AYA solid tumor patients and their caregivers.

Methods: A cross-sectional analysis was performed using baseline data from 7 pediatric/AYA patients and 8 caregivers enrolled in a prospective longitudinal study (target N=40, including 20 dyads and 20 additional individuals). Psychological distress was assessed via the Distress Thermometer and Problem List (DT&PL), with clinically significant distress defined as a score ≥4. Financial burden was measured using the ENRICh Financial Toxicity instrument, with higher scores indicating greater burden. Neighborhood-level socioeconomic deprivation was quantified using the Area Deprivation Index (ADI), which ranks census block groups on a 1–10 scale (1 = least disadvantaged, 10 = most disadvantaged).

Results: Majority of our patients were diagnosed with a sarcoma, with a median age =16.5 years [IQR: 13.5–19.25] and 66.7% required multimodal therapy (radiation, surgery and chemotherapy). Caregiver median age was 44.5 years [IQR: 40.00-46.00]; 66.7% female; 66.7% Hispanic/Latino. Median ADI among the group was 5 [range: 3-8]. Caregivers reported high levels of distress (median=8[IQR: 6.00-9.00]) and stress (median=10 [IQR: 8.5-10.00]), characterized by sleep difficulties (83.3%); persistent anxiety (83.3%); sadness/depression (66.7%); and financial burden (median=6 [IQR: 2.00-7.00]). Patients reported a median distress score of 4 [IQR: 0–5], indicating moderate to severe distress. Common physical concerns included sleep, pain, and fatigue (40%). When reporting emotional concerns changes in appearance and worry/anxiety were highly reported (80%, 60% respectively). Social concerns were prevalent with all participants being concerned with how the cancer diagnosis may impact peer relationships and 66.7% expressing concern about future fertility. Financial concerns were reported by 40%, while 60% had concerns regarding their ability to care for themselves and 30% concerned with continuing school.

Conclusions: Early findings reveal high psychological and financial distress among pediatric/AYA solid tumor patients and their caregivers, particularly in socioeconomically disadvantaged families. Caregivers reported high levels of distress, largely driven by emotional, financial, and caregiving-related stressors, while patients expressed concerns spanning physical symptoms, emotional well-being, and social functioning. These results highlight the need for early, targeted psychosocial and financial support. Ongoing data collection will clarify how these burdens change over time.

POSTER #49

Validation of Key Genetic Pathways in Compensatory Lung Growth via Meta-Analysis of Mouse Model Data in the Gene Expression Omnibus

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Background: Compensatory lung growth (CLG) is defined as an absolute increase in the quantity of functional lung tissue after partial lung resection. CLG has been observed in mice, rats, cats, dogs, rabbits, and ferrets, occurring rapidly in the contralateral lung after pneumonectomy. This phenomenon is also observed in humans but appears largely relegated to infants and young children.
The mechanism for CLG remains incompletely understood. The molecular basis for CLG carried out over the past decade has broadened knowledge of the genetic pathways driving this phenomenon. However, results have not been replicated across all studies, possibly due to differences in design or sample size. We conducted a meta-analysis of gene expression in CLG across 6 different experiments within the Gene Expression Omnibus (GEO) and report upon those genes and pathways that are consistently up- and downregulated across studies.

Methods: GEO is an international public functional repository that archives and freely distributes genomics data submitted by the research community. Six studies containing control and experimental pneumonectomy data using mouse models were extracted from the GEO repository. Each study had a minimum of 2 samples in both the experimental and control groups. Meta-analysis was conducted using random effects model; vote counting; and a third process that used mean gene fold change and p-values from all six studies. Significantly up- and downregulated genes were evaluated using Gene Set Enrichment Analysis (GSEA). Network analysis (NA) was then used to identify genes with high topology.

Results: The observed transcriptomic patterns demonstrated regulation in mesenchymal cell signaling (VEGF-C, IGFBP-3, ECM2, TGFBI), epithelial organogenesis (FAM83B, SPINT1, BARX2, CLDN23), and filament-based pathway (SHH, KRT16, PKP1, KRT14). The data also revealed early downregulation of pro-inflammatory cytokine transcripts although this gene set only met nominal significance thresholds. High topology genes and/or genes that met significance criteria for up- or downregulation were SPINT1 in the epithelial organogenesis pathway, SHH in the filament-based pathway, and VEGF-C in the mesenchymal transition pathway.

Conclusion: These data suggest that mesenchymal cell expression patterns, and specifically epithelial cell transition for organogenesis, in conjunction with genes within the filament-based pathway, are associated with the mechanism of CLG post-pneumonectomy. These findings validate the results of the individual studies that first reported them, but have not yet been replicated. Identification of SPINT1, SHH, and VEGF-C as influential genes in CLG provides targets for future study.

POSTER #50

How does sleep quality affect emotional regulation and daily mood in pediatric patients with and without Obstructive Sleep Apnea?

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Background: Obstructive sleep apnea (OSA) is a sleep-related breathing disorder characterized by recurrent episodes of airway obstruction during sleep, causing awakenings and disrupting restorative sleep. Sleep is crucial for healthy development as it affects physical, neurocognitive, emotional, and behavioral outcomes (Liu et al., 2022). Children with or at high-risk for OSA have a higher prevalence of depression, emotional dysregulation, and behavioral issues (Pereira et al., 2025; Trosman & Trosman, 2017). These studies did not examine day-to-day changes. Here, we examined whether sleep quality and OSA status predicted daily mood and baseline emotion regulation.

Methods: Youth participants aged 9 to 16 (n=59, Patients = 31) completed a baseline emotion regulation questionnaire (Difficulties in Emotional Regulation) and reported sleep quality and morning mood for four-consecutive days. Morning mood ratings were analyzed with linear mixed-effects models to account for repeated measurements within participants, with sleep quality, OSA status, age, sex, and day as predictors. Baseline emotion regulation was analyzed using linear regression with average sleep quality, OSA status, age, and sex as predictors. For both analyses, we tested whether there was an interaction between sleep quality and OSA status.

Results: Sleep quality interacted with OSA status to predict happiness (b=7.59, p=.036) and confidence (b=10.42, p=.005), with better sleep quality linked to greater positive mood in both groups, yet stronger benefits in youth with OSA. There was a main effect of sleep quality on emotion regulation (b=-3.33, p=.016), suggesting that better sleep quality predicts fewer difficulties in emotion regulation.

Conclusion: Sleep quality is crucial for youths' psychosocial functioning. Better perceived sleep quality predicted greater positive affect and emotion regulation, with positive mood benefits even more pronounced in youth with OSA. These results suggest that improving sleep quality may broadly enhance emotional well-being in youth with and without OSA.

Funding: PSF Award, CHOC Pulmonology

POSTER #51

Regulatory Gene Analysis in Enteric Neural Crest Cells Stratified by GDNF Activation Status

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Purpose: Incomplete migration of enteric neural crest cells (ENCCs) into the colon underlies the pathogenesis of Hirschsprung disease, resulting in aganglionosis and disordered intestinal motility. Although activation of the glial cell line-derived neurotrophic factor (GDNF) pathway has been implicated in neurosphere differentiation, the specific regulatory genes governing ENCC differentiation and proliferation remain poorly characterized. This study seeks to elucidate regulatory genes and principal molecular pathways involved in neural crest cell differentiation, thereby advancing our understanding of GDNF's role in enteric nervous system development.

Methods: *Mus musculus* (mice) ENCC gene expression data were obtained from GEO, a public functional genomics repository, to compare GDNF (n=13) and non-GDNF (n=12) samples in dataset GSE34208. We calculated repressive tendency scores to rank genes by their typical epigenetic repression and identify likely context-specific regulatory genes. Unique regulatory genes in GDNF-activated and non-activated neurospheres were identified based on mean repressive tendency scores for each group. Regulatory pathways were then characterized using Gene Ontology database.

Results: Using 50 top-scoring regulatory genes in GDNF activated samples, 13 gene pathways with marked gene enrichment levels were identified as being involved in anatomical development, cell differentiation, neurogenesis, and RNA Polymerase II regulation. Of the 50 regulatory genes, seven homeobox protein genes were found consistently across these pathways: DLX1, NKX2-2, LHX3, VAX2, ARX, HOXC10 and GSX1. Differential gene expression analysis on regulatory genes also showed the olfactomedin gene OLFM1 had the highest log fold change (logFC=1.85).

Conclusion: Our analysis clarifies the regulatory mechanisms governing enteric neural crest cell differentiation and proliferation, identifies novel targets for small molecule intervention, and establishes a foundation for future research into the molecular regulation of enteric neural crest cell development.

POSTER #52

MicroRNAs targeting Valosin-containing protein improve molecular defects in cell model of multisystem proteinopathy

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Background: Valosin-containing protein (VCP) related disease, also known as multisystem proteinopathy 1 (MSP1), is an autosomal dominant disease caused by gain-of-function pathogenic variants of the *VCP* gene. The disease is associated with inclusion body myopathy, early-onset Paget's disease of bone, frontotemporal dementia, and familial amyotrophic lateral sclerosis. There is currently no treatment for this progressive disease associated with early demise resulting from proximal limb girdle and respiratory muscle weakness. We hypothesize that regulating VCP hyperactivity to normal levels can reduce the disease pathology. Recently, the use of microRNA-dependent post-transcriptional suppression of transgene expression has emerged as an effective method to knock down or silence gene expression.

Aim: We hypothesize that by knocking down the VCP with microRNA, we will reduce the gain of function to normal values.

Method: We designed and tested the knockdown efficiency of three microRNA (miR-VCP #2, #6, and #8) constructs targeting the human VCP (*hVCP*) gene. Human embryonic kidney 293 (HEK293T) cells were used due to their human-like posttranslational modification of protein molecules.

Results: The western blot results show mirVCP#2 was the most efficacious in knocking down the WT VCP up to 22%. Next, we evaluated the effect of mirVCP#2 on patient (R155H) iPSC-derived skeletal muscle progenitor cells (SMPCs). MirVCP#2 significantly decreased VCP protein levels by 30% in SMPCs. Additionally, we observed improvements in the autophagy proteins LC3 and p62, as well as a reduction in TDP-43 expression through western blot, which are hallmarks of VCP disease.

Conclusion: Success in these preclinical studies offers promising therapeutic potential for patients with VCP disease.

POSTER #53

Translational studies in iPSC-derived skeletal muscle progenitor cells and Hspb8 mouse model for HSPB8- associated myopathy by upregulating autophagy with trehalose

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Background: Heat shock protein family B member 8 (HSPB8) is a chaperone involved in the Chaperone Assisted Selective Autophagy (CASA) complex. HSPB8 in conjunction with BAG3 and HSP70 promotes removal of misfolded proteins in amyotrophic lateral sclerosis and spinal and bulbar muscular atrophy. Mutations in *HSPB8*, which have been linked to the axonal type of Charcot Marie Tooth, have recently been associated with autosomal dominant adult-onset limb girdle myopathy. Muscle pathology displays TDP-43 aggregates, arrested autophagy, and rimmed vacuoles, leading to muscle atrophy and early demise.

Purpose: We have demonstrated reduced expression of HSPB8, disrupted autophagy and TDP-43 in patient fibroblasts and iPSC-derived skeletal muscle progenitor cells (SMPC). There is no treatment for this disease, an unmet need which we tried to address by upregulation of HSPB8 and enhancement of autophagy. High throughput screening identified trehalose, a naturally occurring disaccharide, as a potent HSPB8 inducer and autophagy facilitator.

Methods: We assessed the effect of trehalose at 100 mM concentration in the HSPB8 patient iPSC-derived SMPCs. The knock-in Hspb8 mouse model with the c.515dupC fs variant shows muscle weakness by 15 months of age. Muscle pathology reveals increased TDP-43, and autophagy pathology recapitulating disease phenotype. We treated Hspb8 mutant mice with 2% trehalose in drinking water for 4 months and compared motor performance to untreated Hspb8 mutant and WT mice.

Results: The qPCR and western blot showed that trehalose induced gradual increase of HSPB8, autophagic proteins LC3B and p62, and decrease of TDP-43 expression in SMPCs. In vivo results demonstrate that trehalose moderately improves motor performance of the HSPB8$^{c515dupC/+}$ mouse. Additionally, trehalose restored HSPB8 protein expression, and trends to improve autophagic markers and TDP-43 pathology.

Conclusion: Our preliminary studies suggest that trehalose may rescue impaired CASA pathway and may have therapeutic potential for HSPB8- myopathy and related neuromuscular disorders.
This work is supported by a grant R21AR080407 from NIH.

POSTER #54

Developmental and Behavioral Characterization of *Ndufs4* Mutant Mice

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Background: Pathogenic variants in the *NDUFS4*, encoding an essential accessory subunit of mitochondrial complex I, are a well-established cause of a mitochondrial disease, most commonly manifesting as Leigh syndrome in infancy. Although the available mouse model of complex I deficiency due to *Ndufs4* mutations have provided important insights into the pathophysiology of adult animals, the early developmental trajectory of affected mice remains inadequately characterized.

Aims: To characterize the developmental and behavioral phenotypes of *Ndufs4* knockout (KO) mice from postnatal day 3 (P3) to P30, in comparison to wild-type (WT) and heterozygous (Het) littermates.

Methods: B6.129S4-*Ndufs4*$^{tm1.1Rpa}$/J Het mice (Jackson Laboratory, USA) were interbred to generate KO (*Ndufs4*$^{-/-}$), Het (*Ndufs4*$^{+/-}$), and WT (*Ndufs4*$^{+/+}$) offspring. Mice were monitored longitudinally from P3 to P30 to assess developmental milestones and behavior. A battery of physical, anthropometric, and reflex characteristics, and behavioral performances assessed by open-field, light-dark box, wire hang, and grip strength tests were performed. All housing and experimental procedures were approved by Institutional Animal Care and Use Committee.

Results: *Ndufs4*$^{-/-}$ mice exhibited reduced breeding efficiency and elevated mortality. Mice also displayed significantly reduced body weight, along with shortened body and tail lengths and fur loss. No overt macroscopic ocular or visual impairments were detected. Significant impairments were observed across multiple domains, including reduced spontaneous locomotor activity, exploratory and grooming behavior, increased anxiety-like behavior, decreased muscle strength and endurance, and impaired motor coordination.

Conclusions: This study is the first to characterize the distinct developmental and sensorimotor deficits in *Ndufs4* mice, revealing early phenotypic signatures that inform disease mechanisms, which may enable earlier detection of the deficiency in humans. Moreover, detailed behavioral assessment will help establish the model for pharmacological testing of FDA-approved compounds, potentially identifying interventions that decelerate disease progression and contribute to the development of effective treatments, or even cures.

POSTER #55

A stereotactic injection method to establish a clinically relevant germinal matrix hemorrhage murine model

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INTRODUCTION: Germinal matrix hemorrhage (GMH) and acquired posthemorrhagic hydrocephalus (PHH) remain the leading causes of premature infant morbidity and mortality. A GMH model that recapitulates both GMH-like tissue injury and hemorrhage via injection of clostridial collagenase into the ganglionic eminence (germinal matrix) has been previously established in rats. Given the goal of exploring pharmacologic interventions to mitigate the risk of acquired hydrocephalus using transgenic mouse lines, we sought to scale down this established rat model to mice.

METHODS: Wild type mouse neonates received stereotactic microinjections of clostridial collagenase into the germinal matrix to produce tissue injury and hemorrhage. Serial dilutions of clostridial collagenase and vehicle alone were delivered across four study groups to develop a collagenase dose-response curve. A fifth study group received intraventricular injections of whole blood to recapitulate intraventricular hemorrhage (IVH). Animals underwent 9.4T magnetic resonance imaging 14- and 28- days post-injection to analyze ventricular index and volumetric ventricular measurements. Animals were euthanized 28 days post-injection, and their brains were harvested for histologic analysis.

RESULTS: Our GMH/PHH model produced greater rates of ventriculomegaly in IVH and medium-dose (0.024 collagenase digestion units (CDU)) study groups compared to vehicle. Half-dose (0.012 CDU) collagenase groups did not produce greater rates of ventriculomegaly compared to vehicle. Double-dose (0.048 CDU) collagenase groups produced slightly greater rates of ventriculomegaly compared to vehicle but posed risk for ischemic stroke and cystic encephalomalacia. Histologic analyses confirmed tissue injury in the ganglionic eminence leading to GMH/PHH in our neonatal mouse model.

CONCLUSIONS: Our group developed a GMH/PHH neonatal mouse model with easily reproducible stereotactic microinjection coordinates. This model will serve as the foundation for future studies with transgenic mouse lines aimed at developing a more comprehensive understanding of the inflammatory response underlying acquired hydrocephalus development as well as support the preclinical study of potential therapeutic interventions.

POSTER #56

In vitro assessment of cytosine base editing and prime editing for correction of the common p.N409S and p.L483P GBA1 pathogenic variants

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Gaucher disease (GD) is the most common lysosomal storage disorder and is caused by biallelic pathogenic *GBA1* variants with resultant insufficiency of the lysosomal glycoside hydrolase, glucocerebrosidase (GCase). GD manifests in hepatosplenomegaly, pulmonary disease, and neurological involvement in the most severe cases. In heterozygosity, *GBA1* variants confer an elevated risk for Parkinson's disease (PD), which may be partially due to aggregation of mutant GCase and other proteins leading to the formation of Lewy bodies. The current standard of care is life–long enzyme replacement therapy (ERT), however, insufficient pharmacodistribution to the brain limits the treatment of neurological symptoms. Alternative therapeutic strategies include gene therapy, which may eliminate the need for ERT and reduce Lewy body formation. Since the majority of GD patients harbor common *GBA1* variants, the gene editing therapy described here would have widespread impact. Additionally, an estimated 1% of people carry at least one pathogenic *GBA1* variant and are at a fivefold increased risk for PD. Developing a gene editing therapy is critical for patients with neuronopathic GD and could also mitigate PD risk. By evaluating gene editing strategies in vitro, we aim to identify an approach that could offer therapeutic benefit.

Here, we report base editing of the most common *GBA1* variant, p.N409S (c.1226A>G), using several cytosine base editors (CBEs). BE4max, which utilizes SpCas9 and APOBEC1 (specifying a base editing window (BEW) C4-7), results in >50% editing efficiency, but is hindered by bystander editing of c.1224G, resulting in a (likely intolerable) splice site alteration. A CBE SpRY approach, which allows for flexibility of BEW positioning, showed low on-target editing efficiency. To isolate the target cytosine, we then evaluated two modified SpCas9 CBEs (YEE and TargetAID2S) that shift the BEW. The TargetAID2S strategy greatly reduced bystander editing but also resulted in reduced editing at c.1226A>G (~20%). We are further evaluating TargetAID2S for therapeutic restoration of enzyme activity in patient–derived cell models. To overcome limitations of bystander editing associated with CBE, we also report the preliminary results of our iterative optimization of prime editing strategies for the two most common GD-causing variants, c.1226A>G and p.L483P (c.1448T>C).

POSTER #57

Oligosaccharide Storage in Pompe Mouse Tissues and History of Oligosaccharide Research

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Background: Pompe disease is a glycogen storage disease caused by a deficiency of the lysosomal a-glucosidase (GAA). The treatment by enzyme replacement therapy (ERT) is helpful but it is not as successful as anticipated.

Aim: The original aim was to assess the actions of enzyme replacement therapy on stored glycogen in tissue. However, in the course of the investigation we have discovered that the storage product is not only glycogen but includes a major fraction of soluble oligosaccharides that contain glucose.

Methods: Glycogen determination involved KOH/HCl extraction, ethanol precipitation of the glycogen, reduction of the ethanol fraction and amyloglucosidase degradation followed by enzymatic glucose determination. Carbohydrate determination by the Phenol Sulfuric Acid method was also employed.

Results: In Pompe and WT mice the KOH/EtOH method detected significantly less glycogen than the direct amyloglucosidase method. Amyloglucosidase is not specific for glycogen and will cleave any a-1,6- and a-1,4- linkages. The ethanol fraction was reduced in a Speed Vac and the material was first analyzed by the Phenol Sulfuric Acid method for carbohydrate followed by amyloglucosidase degradation and enzymatic glucose determination. A review of the literature revealed several reports of maltooligosaccharides being synthesized concomitantly with glycogen. Research in this area was halted due to comments by Mordoh, Krisman and Leloir (1966) based on their severely negative comments of a paper by Sie and Fishman (1958). A review of our results and the other papers between 1955 and 1966 led to the belief that there is a problem with the Sie and Fishman (1958) paper. **This is the first report of the confusion in the Sie and Fishman paper which influenced other investigators.**

Conclusion: Synthesis of maltooligosaccharides concomitant with glycogen is a well-known metabolic event and Glycogen determination should be done specifically and oligosaccharides which should be determined separately.

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POSTER #58

Impairment of energy metabolism in *THG1L*-deficient fibroblasts

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Background: tRNA-histidine guanylyltransferase 1-like *(THG1L*) encodes a protein primarily involved in mitochondrial tRNA maturation, and therefore mitochondrial protein synthesis. Individuals affected by *THG1L* pathogenic variants exhibit developmental delay, cerebellar ataxia, and epileptic encephalopathy, with a strong genotype-phenotype correlation. There is evidence that *THG1L* deficiency is associated with altered increased mitochondrial fission, suggesting an underlying mitochondrial dysfunction [1].

Aims: Characterize the bioenergetics of *THG1L*-deficient fibroblasts derived from a 4-month-old individual carrying compound heterozygous c.164T>C (p. Cys51Trp) and c.153C>G (p. Val55Ala) variants and presenting with intractable epilepsy and hypotonia; and the potential therapeutic effects of aminolevulinate plus iron (ALA/Fe) and omaveloxolone (Omav).

Methods: Oxygen consumption rate (OCR) was assessed using Resipher (Lucid Scientific, USA) and Seahorse XFe96 real-time bioanalyzers (Agilent, USA). Lactate production was measured by colorimetry (Pointe, USA), mitochondrial DNA (mtDNA) copy number by qPCR, and the activities of the respiratory chain complexes I and IV by spectrophotometry. Cells were exposed to ALA/Fe (100/50 µM) and Omav (100, 200, 400, 500 nM) for 7 days. Experiments were conducted under CHOC IRB protocol #130990.

Results: *THG1L*-deficient fibroblasts showed markedly reduced basal and maximal OCR, spare respiratory capacity, and ATP synthesis efficiency, accompanied by increased extracellular acidification. No significant changes in lactate production and mtDNA content were observed. Respiratory chain analysis revealed preserved complex I but impaired complex IV activity. *THG1L* mRNA level and protein content were markedly reduced. Exposure to ALA/Fe and Omav showed an improvement in energy metabolism parameters.

Conclusions: *THG1L*-deficient fibroblasts showed key bioenergetic defects that allow to suggest that the deficiency is associate with hypometabolism. Future studies will focus on elucidating the mechanisms by which pathogenic variants in *THG1L* affect mitochondrial function and optimizing therapeutic dosages of ALA/Fe, Omav, or other FDA-approved drugs in *THG1L* – deficient fibroblasts and mouse models of mitochondrial diseases.

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POSTER #59

Organoid-Based Modeling of NURDopathies: Insights from GATAD2B-Associated Neurodevelopmental Disorder

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Background: The Nucleosome Remodeling and Deacetylase (NuRD) complex is a chromatin remodeler essential for gene regulation during neurodevelopment. NuRD was initially shown to be involved in SATB2's negative regulation of CTIP2 during laminar corticogenesis. NuRD functions as a chromatin remodeler through the combined processes of histone deacetylation and ATP-dependent nucleosome remodeling. Disruption of NuRD function has been linked to a spectrum of neurodevelopmental disorders (NDDs) termed NuRDopathies, often presenting with cortical dysfunction that includes intellectual disability, hypotonia, and apraxia of speech, while also exhibiting features that include congenital heart defects, macrocephaly, and distinct facies. Pathogenic variants in *GATAD2B*, a key subunit of the NuRD complex, cause *GATAD2B*-associated neurodevelopmental disorder (GAND). In NuRD, *GATAD2B* serves as a molecular keystone between the histone deacetylase (HDAC) core and chromatin remodeling subcomplex (CRS). As a result, GAND exhibits a cumulative combination of the clinical features seen in the other NuRD-related conditions, supporting its classification as a "pan-NuRDopathy."

Purpose: To investigate GAND and NuRD pathogenesis, we generated dorsal forebrain organoids (DFOs) from patient-derived GAND and control IPSCs.

Methods: Immunocytochemistry was used to assess cortical laminar structural organization and marker expression in sectioned DFOs.

Results: Consistent with NuRD's role in regulating CTIP2 expression, preliminary data shows GAND DFOs an increased frequency of neurons co-expressing CTIP2 and SATB2, which are laminar markers typically associated with distinct cortical layers (V and II-IV, respectively). Similar findings were seen in GAND mice. These findings suggest defects in cortical patterning in GAND DFOs.
Conclusions: This human organoid model provides a platform to dissect NuRD-dependent mechanisms during neurodevelopment and complements prior clinical and animal studies. Our results highlight the essential role of NuRD in establishing neuronal identity, as well as the micro-architecture of the cerebral cortex. This work offers insights into the molecular basis of GAND and related NuRDopathies.

POSTER #60

Machine Learning-Based Prediction of Sagittal Alignment in Adolescent Idiopathic Scoliosis

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Background: Adolescent idiopathic scoliosis (AIS) is a three-dimensional spinal deformity that requires precise analysis of spinal imbalance to guide surgical correction. Traditional classification methods, such as Lenke, rely predominantly on coronal imaging and overlook sagittal alignment, leading to reduced accuracy in surgical planning[1]. Emerging sagittal-focused models attempt to address this gap but remain constrained by small sample sizes and subjective interpretation[1,2,3].

Purpose/Aims: We propose a machine learning (ML)-based approach to objectively assess sagittal spine curvature in AIS by extracting precise geometric and mathematical features from sagittal imaging. By replacing subjective visual interpretation with data-driven analysis, our method enables more consistent, reproducible, and accurate evaluation of disease severity, reducing ambiguity and improving classification accuracy and surgical planning.

Methods: We will develop a multi-modal deep learning framework that integrates whole-spine x-rays, sagittal superposition images, geometric curvature data, and tabular EHR data. Superposition data will be sourced from the UNiD Adaptative Spine Intelligence platform (Medicrea-Medtronic), which provides sagittal curve information at pre-operative, proposed correction, and post-operative stages. Our methodology is guided by two specific aims: (1) predict post-operative outcome, and (2) predict sagittal spine classification.

Results: Preliminary modeling demonstrates improved accuracy in measuring sagittal alignment, supporting the feasibility of our approach.

Conclusions: This ML-based framework offers a scalable, objective alternative to traditional sagittal assessment methods, addressing variability in surgical planning and enabling more personalized correction strategies. Through multi-institutional collaboration, we aim to develop a comprehensive tool for AIS evaluation and surgical optimization.

Funding: CSO Grant 354

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POSTER #61

Dual-Approach Surface Engineering of Ventricular Catheters to Reduce Cellular Occlusion in the Treatment of Hydrocephalus

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INTRODUCTION: Proximal catheter occlusion remains a major cause of shunt failure in hydrocephalus treatment. To address this, we evaluated two catheter surface modification strategies: a zwitterionic anti-fouling coating, and a combination of zwitterionic coating with an additional web-spun fiber layer designed to physically block cellular infiltration into catheter drainage holes.

METHODS: Silicone catheters were modified using a zwitterionic silane-based surface treatment involving plasma activation, silanization, and zwitterion grafting. A subset of catheters was further layered with a web-spun polymer mesh fabricated by electrospinning. Human astrocytes were cultured on both modified and unmodified catheters. We analyzed 4 zwitterionic-coated catheters, 4 zwitterionic-coated catheters with web-spun mesh, and 4 unmodified control catheters. Cellular attachment was assessed by immunostaining for glial fibrillary acidic protein (GFAP) and 4′,6-diamidino-2-phenylindole (DAPI), followed by quantitative imaging analysis. Cerebrospinal fluid (CSF) flow performance was also evaluated to ensure functional compatibility.

RESULTS: Preliminary data shows that the zwitterionic coating significantly reduced astrocyte attachment compared to unmodified catheters (mean cell density: ~ 0 vs. 8.5 ± 2.7 cells/mm²). The modified catheters with the additional web-spun layer also demonstrated reduced overall cellular attachment relative to controls (3.7 ± 1.5 cells/mm²) and effectively prevented astrocyte penetration into drainage holes. Flow testing confirmed that both modified catheter types maintained fluid dynamics comparable to unmodified catheters.

CONCLUSIONS: Zwitterionic surface modification effectively minimized astrocyte adhesion on ventricular catheters. The addition of a web-spun layer protected the drainage holes by physically limiting cellular access. Notably, the web structure appeared to block cellular penetration, and pore sizes smaller than 5 µm are expected to be critical for maintaining the barrier function. These surface engineering strategies show strong potential to improve the long-term durability of ventricular shunt systems in the treatment of hydrocephalus.

POSTER #62

Placental transcriptome analysis of co-occurrent increased maternal BMI and preeclampsia demonstrates distinct gene expression profiles.

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Objectives: We investigated how increased maternal BMI modifies the placental molecular profile of preeclampsia (PE) by analyzing transcriptomic data to characterize gene expression changes in PE among overweight and obese women relative to those with a healthy weight.

Methods: Placental microarray data from the Gene Expression Omnibus were analyzed using a multivariate model with three pairwise contrasts, adjusted for confounders. Contrasts compared women with and without PE within CDC-defined BMI categories: heathy weight, overweight, and obesity. Differential gene expression was assessed and Gene Set Enrichment Analysis (GSEA) used to identify enriched pathways. Leading-edge genes were further examined to characterize network differences.

Results: Core PE-associated genes were differentially expressed across all contrasts. Notably, *SERPINA3*, a protease inhibitor linked to inflammation and previously associated with PE, was significantly upregulated only in the obesity contrast. GSEA revealed distinct molecular profiles between contrasts: immune-related pathways were uniquely upregulated in the healthy-weight contrast, while cell cycle and stress-response pathways were downregulated in higher BMI contrasts. Network analysis indicated progressive dysregulation of proliferation-related genes with increasing BMI.

Conclusion: These findings support the existence of BMI-associated PE subtypes and highlight the importance of accounting for phenotypic heterogeneity in PE research and treatment strategies.

POSTER #63

Genetically Modified Neuroblastoma–Dendritic Cell Fusion Cells as Autologous Vaccines for Childhood Cancer Immunotherapy

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Introduction: Childhood neuroblastoma is among the most common malignant tumors. In patients older than 18 months with advanced disease, long-term survival remains poor despite intensive multimodal therapy. The cytokines interleukin-12 (IL-12) and interleukin-18 (also known as interferon-inducing factor) are pleiotropic activators of immune responses with potent anti-tumor effects. However, achieving localized and sustained delivery of these immunostimulatory interleukins within the tumor microenvironment remains a major challenge.

Project Aims: 1) Construct recombinant plasmids individually expressing IL-12, IL-18, and B7, as well as a dual-expression plasmid encoding IL-12 and IL-18.2) Generate Neuro-2A mouse neuroblastoma cell lines stably expressing IL-12, IL-18, or B7, designated as Neuro-2A (IL-12), Neuro-2A (IL-18), and Neuro-2A (B7).3) Establish mouse dendritic cell lines stably expressing B7, termed DC (B7).4) Create chimeric fusion cells by fusing Neuroblastoma cells with DC (B7). 5) Evaluate the therapeutic efficacy of these genetically engineered fusion cells as tumor vaccines in eliminating established tumor burden in a murine model. 6) Assess the translational relevance of these strategies for potential application in human neuroblastoma immunotherapy.

Results/Conclusion: We successfully constructed retroviral vectors expressing IL-12, IL-18, and B7 individually, as well as a dual-expression vector encoding IL-12/IL-18. Using the Invitrogen Neon™ electroporation system, we established Neuro-2A and dendritic cell lines with stable expression of these immunostimulatory molecules. Specifically, we generated 29 Neuro-2A clones expressing IL-18, 12 clones expressing IL-12, 4 clones expressing B7, 9 dendritic cell clones expressing IL-18, 6 dendritic cell clones expressing B7, and 5 Neuro-2A clones co-expressing IL-18 and IL-12. In vivo studies showed that Neuro-2A cells induced tumor formation in immunodeficient NSG mice but not in immune-competent C57BL/6 or C3H/HeJ strains, while tumorigenicity was observed in A/J mice. Importantly, Neuro-2A (IL-18) and Neuro-2A (IL-18/IL-12) did not form tumors in immune-competent mice (C57BL/6J, C3H/HeJ, A/J), whereas Neuro-2A (B7) unexpectedly retained tumorigenicity. Survival analyses, defined by endpoint tumor growth ≥1000 mm^3 or unrecoverable condition within 29 days, demonstrated significant differences between groups (N = 30 mice; 23 controls vs. 7 experimental vaccine-treated). Kaplan–Meier analysis revealed a Chi-square of 3.929 with $p = 0.047$ (Log-Rank [Mantel–Cox]) using SPSS v18.0. These findings highlight the potential of IL-18 and IL-18/IL-12–engineered Neuro-2A vaccines in suppressing tumorigenicity and improving survival in immune-competent hosts. Future efforts will focus on refining this strategy to develop more effective neuroblastoma vaccines with translational relevance to human patient pathology.

POSTER #64

Amniotic Fluid and Plasma microRNA Performance in the Prenatal Diagnosis of Congenital Diaphragmatic Hernia

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Objective: This study evaluates the performance of microRNA in amniotic fluid and plasma samples in normal versus congenital diaphragmatic hernia (CDH) pregnancies.

Methods: Using Gene Expression Omnibus (GEO) data (GSE242364), miRNA profiles were compared between two sample types: amniotic fluid (N=41, 18 CDH, 23 healthy euploid) and plasma (N=20, 8 CDH, 12 healthy euploid) to distinguish CDH from healthy euploid pregnancies. Both sample types were evaluated using the Random Forest classification model and then cross-validated to assess the model's performance for the top 10 performing miRNAs.

Results: Plasma samples showed perfect classification (0% error, AUC: 1, sensitivity: 1, specificity: 1), while amniotic fluid had a 2.44% error rate (AUC: 0.9952, sensitivity: 0.944, specificity: 1). Five of the top ten amniotic fluid miRNAs show AUCs equal to 1.00 (miR-122-5p, miR-451a, miR-590-5p, miR-612, miR-644a). Eight of the top ten plasma miRNAs demonstrate strong predictive power, each with an AUC above 0.9 (miR-99b-5p, miR-323a-3p, miR-151a-5p, miR-409-3p, miR-148a-5p). Annotation of top plasma and amniotic fluid miRNAs provided evidence of association with other congenital diseases and metabolic dysregulation, including steatotic liver disease and pancreatitis.

Conclusion: Amniotic fluid and plasma sample miRNAs could serve as potential biomarkers for prenatal CDH detection, though further validation is needed.

POSTER #65

The Role of Regulatory T-Cells in Predicting Outcomes in Inflammatory Bowel Disease

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Background: Inflammatory bowel disease (IBD), (Crohn's disease, CD; ulcerative colitis, UC), is a chronic, immune-mediated condition causing gastrointestinal inflammation. The heterogeneity in disease phenotype and treatment response remains poorly understood. Regulatory T cells (Tregs) are essential for maintaining intestinal immune tolerance and preserving mucosal homeostasis. Their dysregulation is implicated in IBD pathogenesis, resulting in uncontrolled immune responses and chronic intestinal inflammation.

Aims: To investigate the relative proportion of Tregs in pediatric IBD during flare and remission as potential predictive biomarkers of therapeutic response, potentially enabling less invasive monitoring and enhanced understanding of immunomodulatory pathways.

Methods: A single-center pilot study was conducted in pediatric IBD patients (<21 years) experiencing a disease flare, defined by increased clinical symptoms and supported by objective findings necessitating therapy escalation. Peripheral blood was collected at flare or diagnosis and within six months of treatment initiation or modification. Treg expression was analyzed by flow cytometry to compare immune profiles by disease severity, subtype, and at flare and remission. Statistical analyses included paired t-tests and Wilcoxon, as appropriate**.**

Preliminary Results: Among 19 patients (11 UC, 8 CD), UC showed a trend toward higher Treg proportions than CD at initial flare (6.82 ± 3.07 vs. 4.26 ± 3.06; p = 0.06). CD127hi-Tregs were significantly higher in inactive/mild vs. moderate/severe disease (6.39 ± 3.45 vs. 3.31 ± 2.24; p = 0.018), with a similar pattern for $CD3^{+}CD25^{+}CD127hi$ cells (7.63 ± 4.00 vs. 3.97 ± 1.72; p = 0.029). Treg levels did not significantly change between flare and remission.

Conclusion: These differences in Treg subset levels by disease severity and subtype (UC vs. CD) suggest elevated peripheral CD127hi-Tregs may be associated with reduced IBD disease activity and support their potential role as immune biomarkers. Although limited by sample size, the observed patterns merit validation in larger cohorts to determine clinical significance.

POSTER #66

Comprehensive Assessment of T-cell exhaustion in allogeneic HSCT and CAR T-cell immunotherapy

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Background: T-cell exhaustion impacts survival outcomes following CAR T-cell therapy and Allogeneic stem cell transplantation (Allo-SCT). T-cell exhaustion also has a distinct differentiation trajectory arising from chronic antigen stimulation, characterized by progressive functional decline, sustained inhibitory receptor expression, and stable epigenetic reprogramming.

Purpose/Aims: Our goal was to produce a comprehensive review synthesizing current knowledge of T cell exhaustion mechanisms, differentiation states, and functional consequences of T-cell exhaustion in CAR T-cell therapy and Allo-SCT; evaluate factors and markers differentiating exhausted T-cells; and explore emerging techniques to measure T cell exhaustion; and discusses future research directions with an emphasis on relapse prevention and improving therapeutic interventions.

Methods: We conducted a literature review of studies from 2000 to 2025, focusing on experimental and clinical literature addressing T-cell exhaustion in allo-SCT and CAR T-Cell therapy. Included studies examined exhaustion differentiation, molecular regulations, and profiling techniques. Papers on T-cell exhaustion in viral infection and autoimmune disease were excluded.

Results: T cell exhaustion represents a parallel developmental pathway distinct from effector and memory lineages, progressing from precursor to terminally exhausted subsets. It exists as a dysfunction spectrum caused by mechanistic drivers, including persistent antigen exposure, inadequate CD4+ help, inhibitory signaling, and extrinsic modulators that influence exhaustion severity. In Allo-SCT, partial exhaustion may attenuate graft-versus-host disease (GvHD) but can compromise graft-versus-leukemia (GvL) activity; therefore, maintaining a balance to minimize relapse is crucial. In CAR T-cell therapy, exhaustion limits persistence and antitumor efficacy, contributing to relapse.

Conclusions: Measuring T-cell exhaustion has the potential to serve as a predictive biomarker for relapses following CAR T-cell therapy and allo-HSCT; however, standardized approaches to assess immune function remain lacking. Developing a method to quantify antigen load alongside pre T cell exhaustion markers by integrating multi-omics, single-cell transcriptomics, and epigenetic profiling may provide an early signal of relapse risk.

POSTER #67

A comparison of clinical features in two patients with Mucopolysaccharidosis VI treated with ERT versus HSCT

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Background: Mucopolysaccharidosis Type VI (MPS VI, Maroteaux-Lamy Syndrome) is a rare lysosomal storage disorder with an incidence of 0.04 per 100,000 livebirths in the US. The condition is caused by mutations in the *ARSB* gene, resulting in arylsulfatase B deficiency. Glycosaminoglycan (GAG) accumulation causes widespread tissue damage and eventual organ failure. Treatment by enzyme replacement therapy (ERT) is standard-of-care since 2005, while hematopoietic stem cell transplantation (HSCT) is optional and has been available for decades.

Purpose/Aim(s) and **Methods:** We highlight the clinical features of two females diagnosed with MPS VI at an early age: one treated with HSCT at age 2, the other with ERT at age 13. We discuss advantages and disadvantages of these two treatments by comparing outcomes and financial burden.

Results: In our cohort, Patient 1 was diagnosed as an infant and received HSCT at 2y. She began ERT at 23y due to elevated urinary GAGs and is currently living at 35y. Patient 2 was diagnosed at 2y, began ERT in 2003 at 13y, and required mitral valve replacement at 16y. She passed away at 24y due to cardiac arrhythmia. Autopsy showed cardiomegaly with myocyte hypertrophy suggestive of chronic ischemic heart disease, and valvular heart disease with dystrophic calcification of the aortic valve leaflets and thickening of tricuspid leaflets.
Clinical features of both patients include short stature, dysostosis multiplex, macrocrania, glaucoma, corneal clouding, and Chiari malformation. Discordant features include hypertension, left ventricular hypertrophy, stenosis in the aortic and tricuspid valves, seizure disorder, and sudden death in Patient 2. Initial treatment by HSCT notably reduced Patient 1's total cost of ERT by billions of dollars over two decades.

Conclusions: It is possible that Patient 2's outcome would have been more favorable if treatment were available at an earlier age than 13 years. Patient 1, treated with HSCT early and ERT, had a better outcome. ERT and HSCT mitigate many symptoms, but tissues including cardiac valves continue to accumulate GAGs, necessitating surgical replacement. HSCT may be more cost-effective for patients in medically underserved communities when compared to the annual financial burden of weekly ERT.

POSTER #68

Improving Screening for Social Drivers of Health in Hospitalized Pediatric Patients

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Background: Social drivers of health (SDOH) are associated with poor health outcomes including longer hospital stays, greater hospital readmission rates, and higher morbidity and mortality. Hospitalization provides an opportunity to identify and intervene on social needs to improve patient health.

Objective: We applied improvement science methodology to increase SDOH screening of pediatric patients hospitalized in acute care units from 0% to 20% within eighteen months

Methods: A multidisciplinary team of physicians, residents, nurses, and social workers was formed. A process map, key driver diagram, and Ishikawa diagram were created. The primary measure was the percentage of admitted patients screened for SDOH (process). Additional measures included the percentage of positive SDOH screenings (process) and percentage of urgent SW assessments completed (outcome). Balancing measures included staff satisfaction and perceived impact on other duties. Plan-Do-Study-Act Cycles (PDSA) began in June 2024. Interventions included the introduction of an electronic SDOH screening tool, display of QR code in patient rooms, and a screening process that alternated both the personnel introducing the screening tool (case managers, medical students, bedside nurses, or unit assistants) and the device used to access it (personal device or hospital tablet). Staff surveys and patient/family interviews were conducted throughout. Control and run charts were analyzed for impact.

Results: SDOH screening increased to 0.5% of admitted patients (Figure 1). An astronomical point was identified when unit assistants brought tablets to patients to complete screenings (41%, Figure 2). Staff reported decreased motivation to conduct screening, citing redundancy with the nursing admission history and workflow disruptions for unit assistants, case managers, and medical students.

Conclusions: We have not yet observed significant improvement in SDOH screening of pediatric patients hospitalized in our acute care units. PDSA's are ongoing; possible interventions to increase distribution of the screening tool include televised advertisements in patient rooms and printed flyers on meal trays.

POSTER #69

Feeling Seen and Heard at School: Innovation with Mental Health Wellness Centers

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The rising incidence of mental health issues among students demands creative solutions to enhance well-being, such as "WellSpaces," dedicated areas within schools where students, families, and staff can participate in mental health activities to foster a supportive community. WellSpaces, though rarely explored in research (Moya et al., 2022), offer diverse benefits, including improved self-regulation, mood enhancement, and reduced mental health stigma (Clayton, 2024). While schools provide a critical platform for mental health support, the large-scale implementation and utilization of permanent WellSpaces remain largely uninvestigated (Guerra & Williams, 2003; Moya et al., 2022). Methods: WellSpaces offers a range of programming including coping tools readily available for student use, wellness activities, and parenting workshops. Results: Since its inception, there have been over 50 installed WellSpaces, where they have served over 30,256 students, facilitated 18 mental health presentations, and hosted 65 wellness activities. Data examining the responses of 31 high and middle school students, who participated in the WellSpaces this past year, found that 93% reported that they will use something they learned from the WellSpace for their mental health. Quotes from qualitative data demonstrates how students have found the space to be "eye-opening, reflective" and helping them "feel more aware of my feelings." Out of 100 parent responses regarding the parenting workshops, 71% strongly agreed that the workshops contained useful information that could be applied to parenting. Conclusion: By establishing a dedicated space on school campuses that provides students with resources and opportunities to explore mental health topics, cultivate wellness practices, and develop coping skills, we can significantly improve student mental health outcomes and create a supportive environment on campus.

POSTER #70

Quantifying Success: Evaluating Transfer Metrics in a Specialty Transition Clinic

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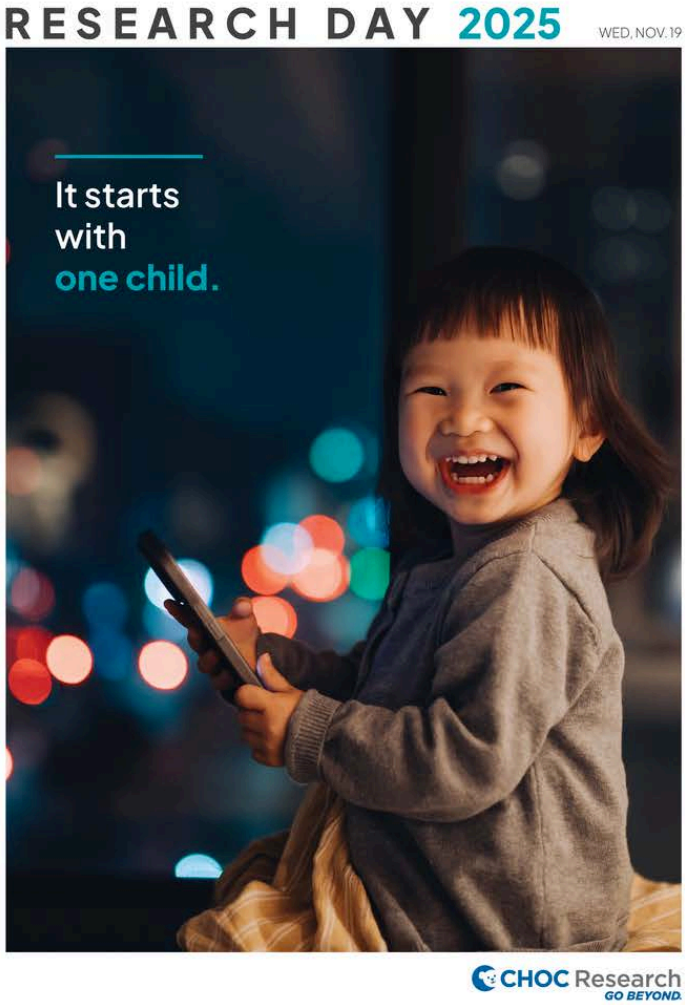
Background: Attending an adult provider appointment is one measure of transition success, but it only marks the endpoint of a broader process. Little has been published on transfer metrics in medically complex youth. We present data on transfer metrics from our specialty transition clinic.

Methods: Our clinic, a referral-based consultation service for medically complex youth, provides customized transition care guided by the Six Core Elements. To evaluate transfer success, we reviewed the literature to identify appropriate metrics. We identified and tracked metrics related to clinic accessibility, transition readiness, care coordination, and continuity of care.

Results: From mid-2023 to April 2025, our clinic transferred 46 medically complex patients to adult care. Sixty-six patients are currently "in process" and 17 were lost to follow-up. Time from referral to first outreach and first appointment was 71 and 96 days, respectively (clinic accessibility). All patients had their transition readiness assessed (transition readiness), but only 10 had multiple assessments (M =13 percentage point improvement). All patients had a transfer checklist, and 98% had an adult PCP (care coordination). All attended an adult provider appointment, with 61% seeing their new provider within six months of their last pediatric visit (continuity of care).

Conclusions: Our clinic effectively transfers medically complex patients to adult care. However, few patients had multiple transition readiness assessments, suggesting many providers are not discussing transition earlier, relying on our clinic as the "first and final stop" for transition services. Delays in linkage reflect internal referral processing challenges rather than clinic availability. With an established tracking system, we aim to improve timely linkage to care. Transfer success extends beyond adult provider appointment confirmation. Clinic accessibility, transition readiness, care coordination, and continuity of care offer a broader understanding of successful transfer and where transition programs can focus future efforts.



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from the executive director

Research Day 2025 would not have been possible without the extraordinary dedication and expertise of the many colleagues who brought it to life. Each member of this team played a vital role in ensuring that the day reflected both the scientific excellence and the human heart of our mission.

Behind the scenes, months of thoughtful planning went into every detail. The children's activities—often the highlight of the day—required imagination, care, and coordination to create experiences that both delighted and inspired our youngest guests. The creative design team brought a cohesive look and feel to the event, ensuring that every program and display reflected the energy and identity of our institute. Abstract submissions and podium presentations were carefully managed so that the groundbreaking science presented by our researchers was highlighted with clarity and respect.

Equally important was the logistical planning. From scheduling and registration, to catering, technology, and pre-event workshops and other mentoring and support for early career investigators presenting their work, each detail was handled with precision. This behind-the-scenes work may not always be visible, but it is what allows the event to run seamlessly for attendees.

Together, these efforts created more than just a conference—they created an experience that honored our researchers, engaged our community, and celebrated discovery and innovation. This year's theme, "It Starts with One Child," reminded us that every breakthrough begins with a single story, a spark of curiosity, and a reason to keep striving for better care for children everywhere.

Research Day goes beyond a single day of celebrating pediatric research at Rady Children's Health in Orange County. It serves as a catalyst for forming connections across disciplines, encouraging dialogue, and fostering collaboration within the greater healthcare system. These conversations spark future projects and partnerships that extend well beyond the event itself, strengthening not only our research community, but the entire network dedicated to improving children's health.

To all who contributed: thank you. Your hard work, creativity, and commitment made this day possible, and your efforts help bring us closer to transforming care for every child.

Phuong Dao, JD
Executive Director,
Research Institute