2025
RESEARCH
AND
EDUCATION
REPORT

Championing Pediatric Science and Training

Children's℠ Healthcare of Atlanta

EMORY UNIVERSITY

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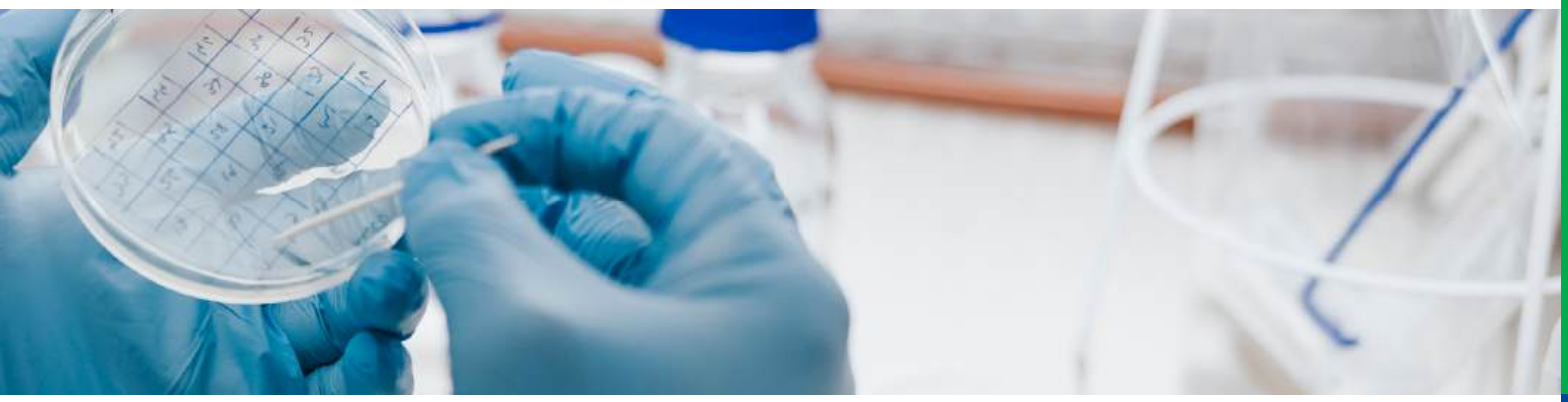


Delivering exceptional care, driving innovation and shaping the future of pediatric medicine together

A Message from Dr. Barkin

Pediatrician in Chief
Children's Healthcare of Atlanta

George W. Brumley Jr. Chair, Department of Pediatrics
Emory University School of Medicine

Dear Colleagues,

As I reflect on 2025, my first year as pediatrician in chief at Children's Healthcare of Atlanta and chair of the Department of Pediatrics at Emory University School of Medicine, I can't help but marvel at all our teams have accomplished during this time of change and uncertainty. It is an honor to be part of the lifesaving and life-changing work conducted at our institutions, where we have faced challenges and exceeded expectations. Faculty and staff at Children's and Emory have:

- **Surpassed research funding goals**

  This achievement reflects the creativity and resilience of our research teams, who let nothing stand in the way of advancing science and improving outcomes for kids in Georgia and around the world. Emory's Department of Pediatrics ranked No. 1 in National Institutes of Health (NIH) funding for both cystic fibrosis and sickle cell disease. Staff supporting this research were able to pivot in the face of uncertainty and examine new ways forward to support and advance discovery, innovation and the training of pediatric scientists.

- **Opened the Marcus Center for Cellular Therapy**

  One of the only pediatric labs of its kind in the country within the walls of a children's hospital, this innovative facility was made possible thanks to a significant grant from The Marcus Foundation. Through cutting-edge clinical trials, it facilitates the manufacturing and delivery of next-generation cellular and gene therapies, offering hope to patients with conditions that are resistant to standard treatments. In addition to the numerous benefits to patients, the Marcus Center for Cellular Therapy will allow Children's and Emory investigators to pursue their own cellular and gene therapy research without having to take manufacturing off-site, enabling them to bring treatments to the bedside more quickly.

- **Recruited the largest Core Pediatrics Residency class to date**

  Working to ensure the next generation is equipped to navigate current healthcare challenges and innovate for the future is key to securing healthier tomorrows for Georgia's kids. This is why, for the first time, we are incorporating educational accomplishments into our annual research report. A testament to our dedication to education and training: 90% of core pediatric residents over the past three years passed the pediatric board certifying exam on their first attempt.

- **Celebrated many 'firsts'**
  These milestones included welcoming the first patient to the Marcus Center for Cellular Therapy, Gabrielle, who received a cell therapy transfusion of newborn donor stem cells to try to combat dilated cardiomyopathy. Gabrielle is part of a safety trial, also funded by The Marcus Foundation, to establish the correct dosage of stem cells. Children's and Emory will enroll 24 patients total for the study.

- **Built upon partnerships and funded innovative work through the Children's Healthcare of Atlanta Pediatric Technology Center (PTC) at Georgia Tech**
  This collaboration brings clinical experts from Children's and scientists and engineers from Georgia Tech together to develop technological solutions to problems we see every day at Children's. Initiatives fall under three pillars that continued to grow in 2025:

  Pillar 1: Data science, machine learning and AI
  New co-lead Lauren Steimle, PhD, of Georgia Tech, joined Naveen Muthu, MD, who has been co-lead for Children's since 2024.

  Pillar 2: Patient-centered care delivery
  The first co-leads for this pillar, John Constantino, MD, of Children's, and Munmun De Choudhury, PhD, of Georgia Tech, focused on improving behavioral outcomes in children.

  Pillar 3: Technology and devices to advance quality and safety
  Omer Inan, PhD, began as co-lead for Georgia Tech, and Jay Shah, MD, began as co-lead for Children's. Research milestones falling under this pillar included funding to test a new bedside pressure injury prevention technology, as well as the publication of study results for a highly accurate anemia detection app (see page 51).

Children's and Emory have so much to be grateful for. Our staff, faculty and trainees are truly exceptional, and I am thrilled to share a snapshot of that work here in the 2025 Research and Education Report. While we celebrate current achievements, we also are looking toward the future. Currently, leadership at our institutions are building out a strategic plan with an eye toward data-driven innovation and sustainability, using the rapidly changing tools at our disposal to advance children's health and healthcare. We will also continue maximizing collaborations like the one with Georgia Tech to advance the PTC. As always, we move forward with a clear commitment to providing care that heals, research that cures, prevention that protects and training that prepares tomorrow's pediatric specialists and researchers.

Best,

Shari Barkin, MD
Pediatrician in Chief
Children's Healthcare of Atlanta

George W. Brumley Jr. Chair, Department of Pediatrics
Emory University School of Medicine

Notable National Awards and Distinctions

Wanda Barfield, MD
Member, National Academy of Medicine

Andres Camacho-Gonzalez, MD
Fellow, Pediatric Infectious Diseases Society

Alexis Carter, MD
- Fellow, American College of Medical Informatics
- Jeffrey A. Kant Leadership Award, Association for Molecular Pathology

Sharon Castellino, MD
Hodgkin Lymphoma Disease Committee Leadership, Children's Oncology Group

Ann Chahroudi, MD, PhD
- Marian W. Ropes, MD, Award, The American Society for Clinical Investigation
- Strategy and Operations Officer, Society for Pediatric Research

Patricia Denning, MD
Founder's Award, 2025 Southern Society for Pediatric Research

Laura Downey, MD
Secretary, Congenital Cardiac Anesthesia Society

Jairo Fonseca, MD
IMG Early Career Development Award, Pediatric Infectious Disease Society

Roshan George, MD
Pediatric Transplant Journal Award, International Pediatric Transplant Association

Grace Gombolay, MD
Fellow, American Academy of Neurology

Laura Hayes, MD
Outstanding Presentation Award/Derek Harwood-Nash Award, American Society of Pediatric Neuroradiology

Kiran Hebbar, MD
Director, INSPIRE Executive Board

Andrew Kirsch, MD
- John W. Duckett, Jr., MD, Pediatric Urology Research Excellence Award, American Urological Association
- Special Recognition Certificate, American Academy of Pediatrics

Wilbur Lam, MD, PhD
- 2024 Fellow, American Association for the Advancement of Science
- Member, Association of American Physicians

Leann Linam, MD
Secretary, Society for Pediatric Radiology

Terri McFadden, MD
President-Elect, American Academy of Pediatrics

Claudia Morris, MD
Global Clinical and Translational Research Award, Department of Pediatrics, University of Abuja, Nigeria

Andrew Muir, MD
2025 Outstanding Mentor Award, Pediatric Endocrine Society

Susan Palasis, MD
2025 President's Award, American Society of Pediatric Neuroradiology

Chris Rees, MD
Jane Knapp Emerging PEM Leader Award, Section on Emergency Medicine, American Academy of Pediatrics

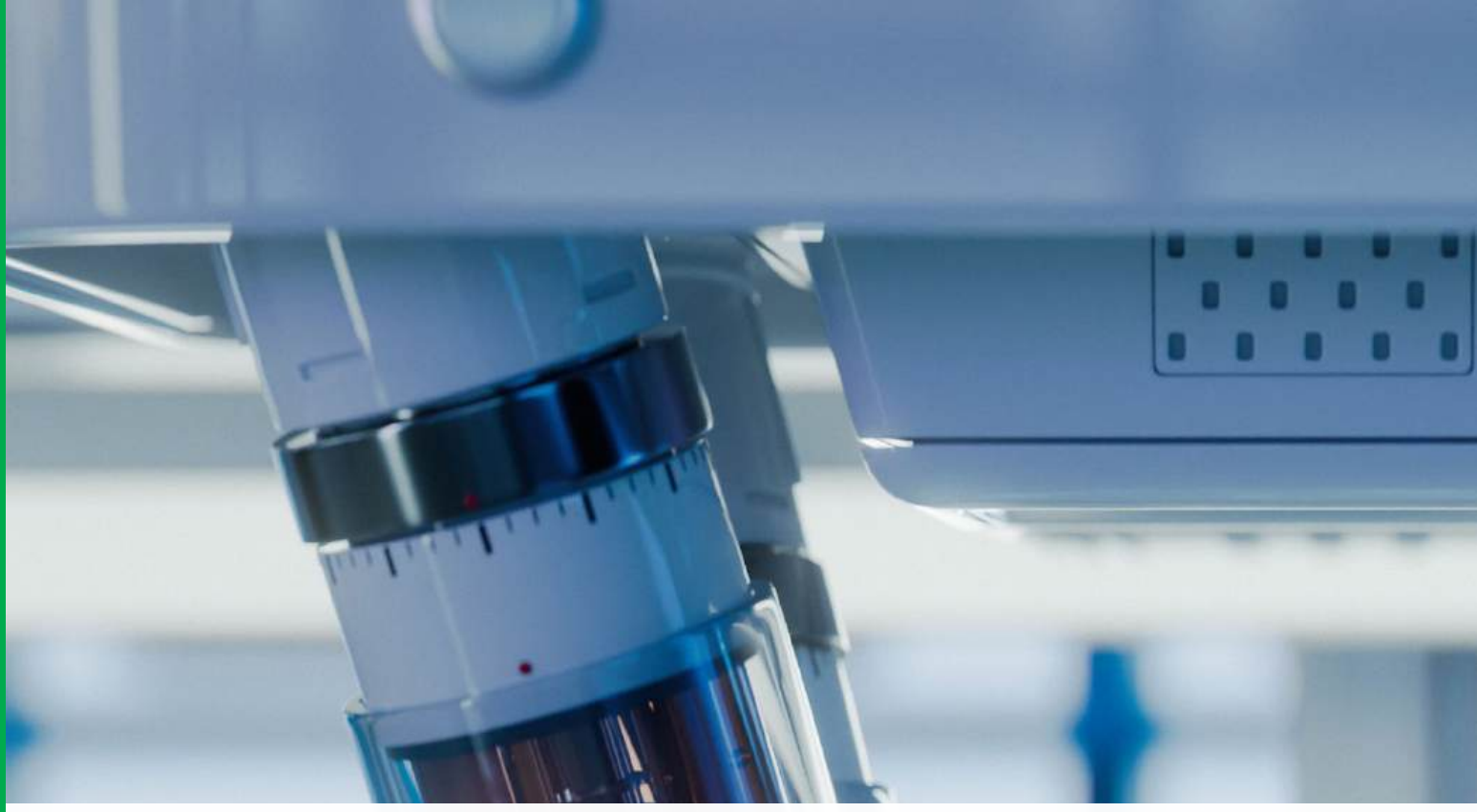
Andrew Reisner, MD
Advisory Board Member, International Society of Pediatric Neurosurgeons

Raymond Schinazi, PhD, DSc
Honorary Doctor, Faculty of Medicine, Uppsala University, Sweden

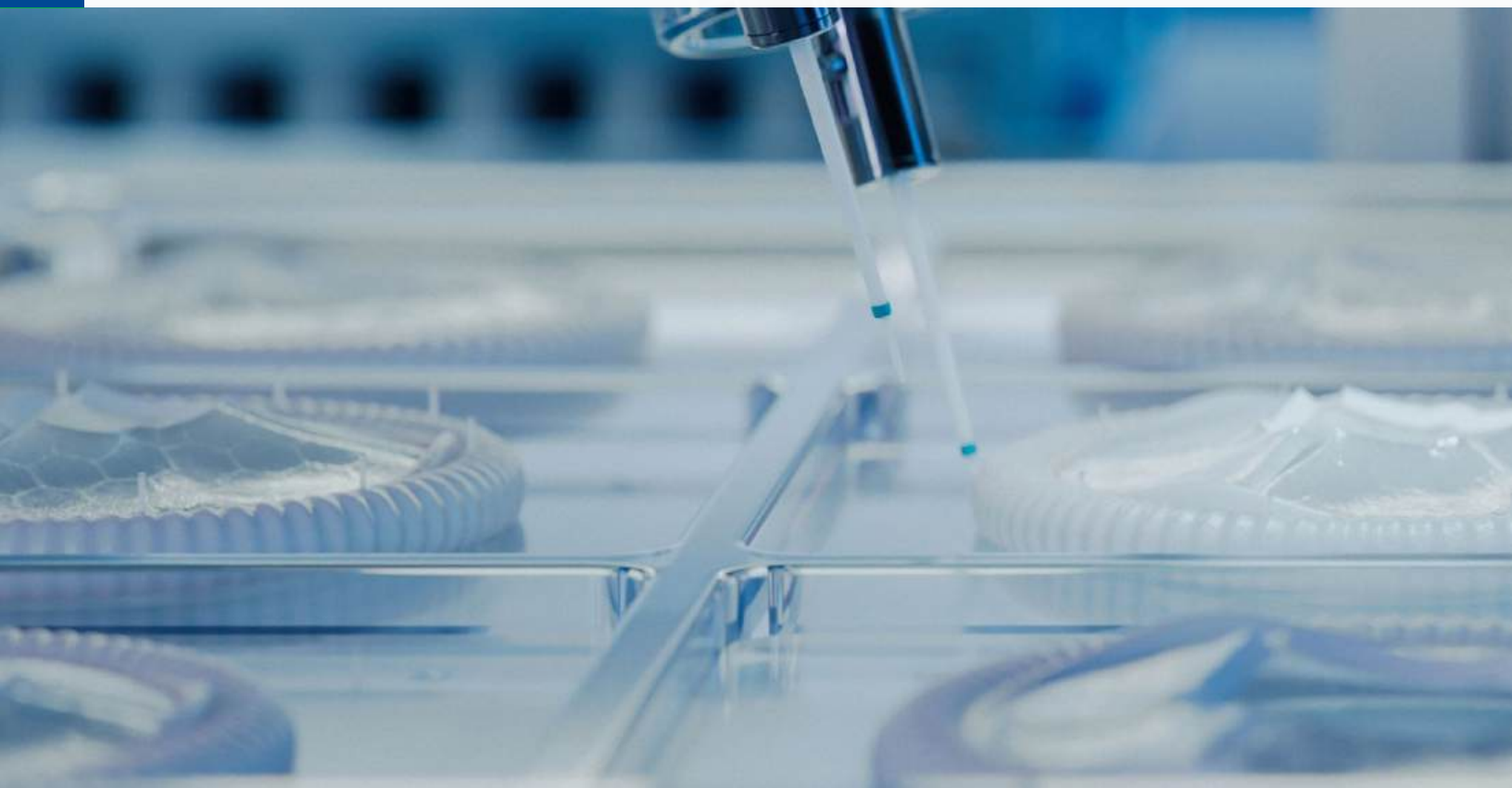
Andi L. Shane, MD
Member, Association of Medical School Pediatric Department Chairs' Pediatric Leadership Development Program

Anna B. Tanner, MD
Fellow, Academy for Eating Disorders

Dan Wechsler, MD, PhD
Vice President, American Society of Pediatric Hematology/Oncology Board of Trustees



RESEARCH



Improving the lives of children through research

A Message from Dr. Murray

Chief Research Officer
Children's Healthcare of Atlanta

Executive Vice Chair of Research
Department of Pediatrics
Emory University School of Medicine

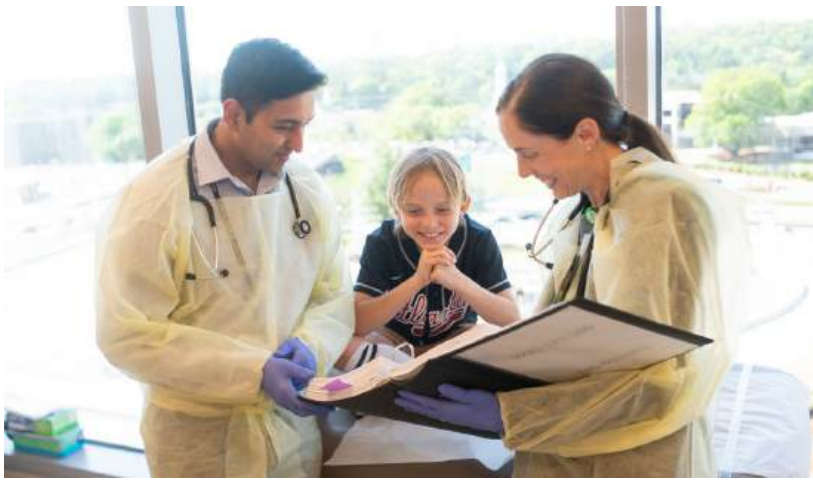
Dear Colleagues,

As you will see in the following pages, this was an exciting year for Children's and Emory. Like many in the research community, we faced challenges, but we drew on our strength and resilience to continue delivering superior results. We remained steadfast because we know this is work that matters.

I'm incredibly proud of all we accomplished in 2025. Our faculty earned 30 notable national awards and contributed to 1,737 publications across 729 journals. We celebrated four years of being in the top three for National Institutes of Health (NIH) research funding. U.S. News & World Report ranked eight of our specialties in the top 20 and one (orthopedics) in the top 10 for 2025-2026. In addition, 11 new researchers joined our team, including experts in basic and translational research who will lay the foundation for tomorrow's cures, as well as luminaries recognized for exploring new frontiers and helping to establish a national framework for using AI in pediatric medicine.

We continued to educate and inspire the next generation of pediatric researchers this past year. In addition to training those in our clinics and laboratories, we embraced opportunities for community building. A highlight for me was the Southeastern Pediatric Research Conference, where we welcomed a record number of attendees from 34 institutions, building new connections, fostering enthusiasm and reinforcing the importance of pediatric research among trainees and early-career scientists.

As I reflect on the incredible work that our researchers do each day, I also want to take a moment to celebrate the biggest heroes in pediatric research: the patients and families who choose to participate. Being willing to test new treatments is an act of great courage and hope. There is nothing better than seeing a patient who has participated in one of our studies experience a better quality of life as a result. Patients like James, who took part



in the Alyftrek™ trial highlighted in this report. He went from taking 60 pills each day for cystic fibrosis to just three pills, enabling him to get involved in more activities and spend more time with friends. We are grateful for all the patients and families who participate in research, and we take the trust they place in us seriously.

We also value the trust that our donors place in us, our programs and our patients. Investing in research is more than an act of altruism. It is the highest form of optimism and a way to leave the world a better place. We are grateful for every gesture of support, especially during this past year of uncertainty.

Throughout my career, I have seen pediatric researchers press forward with unwavering commitment and clarity of purpose, and 2025 was no different. Our researchers demonstrated extraordinary grit, along with profound determination, collaboration and integrity.

I am deeply proud not only of what we accomplished, but of how we accomplished it together. I eagerly anticipate what lies ahead. I'm confident we will continue to reach new heights in improving the health and lives of children in Georgia and beyond, staying true to our Mission to make kids better today and healthier tomorrow.

With gratitude

Kristy Murray, DVM, PhD
Chief Research Officer
Children's Healthcare of Atlanta

Executive Vice Chair of Research
Department of Pediatrics,
Emory University School of Medicine

Research by the Numbers

Ranked **No. 3** nationally in NIH funding for pediatric departments*

$112.9 million
in total extramural funding

$59 million
in funding from NIH

168 new clinical studies opened (including **73 new clinical trials**)

** NIH funding period from Oct. 1, 2024 through Sept. 30, 2025.*

11 new researchers recruited

164,556 square feet dedicated to pediatric research

1,170 hours of CME credits through **248 educational activities**

4,417 Children's patients enrolled in clinical research

1,674 visit hours in the Children's Pediatric Research Unit

3,525 visit hours in the Emory and Children's Center (ECC) Research Unit

313 active clinical trials

1,737 publications in **729 different journals**

Our **Orthopedics Program** is ranked **No. 9** in the nation by U.S. News & World Report with:

- **15** new clinical studies opened
- **17** publications in **7** journals
- **429** orthopedics patients enrolled in clinical research

Large Grants

Gregory B. Melikian, PhD

Inhibition of Viral Membrane Fusion by Interferon-Induced Proteins, NIH, $2.2 million

Philip Lupo, PhD

Down Syndrome Early Childhood Omics, Deep Phenotyping and Epidemiology in Texas: DECODE IT Cohort, NIH, $1.6 million

Outcomes and Health Risks Among Individuals With Genetic Predispositions to Cancer: The ORIGen Cohort, Leidos Biomedical Research, $970,000

Microphthalmia, Anophthalmia and Coloboma Genetic Epidemiology in Children (MAGIC) Study, NIH, $850,000

US-UK Collaborative Study of the Health of Children Born From In Vitro Fertilization: From Conception Through Young Adulthood, NIH, $550,000

Raymond Schinazi, PhD

Anti-Norovirus Protease Inhibitors for Immunocompromised Patients, NIH, $1.5 million

Ann Chahroudi, MD, PhD

Virus and Host Directed Immunotherapies for Cure in SHIV-Infected Infant Macaques, NIH, $1.3 million

Douglas Graham, MD, PhD
Aflac Precision Medicine Program, CURE Childhood Cancer, $1.1 million

Wilbur Lam, MD, PhD
Emory Nurturing Environments for Science Technology (NEST): Research-Based Childcare Center for Influenza and Other Communicable Illnesses Planning Grant, National Philanthropic Trust, $1 million

Benjamin Kopp, MD
Sickle Cell Disease Microbiologic and Immunologic Links to Health Equity (SMILE), NIH, $790,000

Stefanos Sarafianos, PhD
Discovery of Antivirals Targeting Mpox Virus, NIH, $760,000

Nael McCarty, PhD
Mechanisms Linking CFTR to Dysregulated Barrier Function, Insulin Receptor Function and Glucose Transport in the CF Lung, NIH, $730,000

Mindy Scheithauer, PhD
Implementation Pilot Elopement, NIH, $710,000

Anne Fitzpatrick, PhD
Social Determinants of Symptoms in Preschool Children with Recurrent Wheezing, NIH, $690,000

Solomon Ofori-Acquah, PhD
Therapeutic Targets of Acute Chest Syndrome, NIH, $670,000

Gregory Melikian, PhD
Biophysics of Protein-Mediated Membrane Fusion, NIH, $650,000

Robert Dick, PhD
Structural Interrogation of the HIV-1 Virion and the Mechanism of the Capsid Targeting Compound Lenacapavir, NIH, $650,000

Marianne Yee, MD
Donor Red Blood Cell Survival in Recipients with Sickle Cell Disease, NIH, $640,000

Kathryn Oliver, PhD
Translation Efficiency to Overcome Refractory Defects in Cystic Fibrosis Transmembrane Conductance Regulator (CFTR), NIH, $620,000

Stefanos Sarafianos, PhD
Ultrapotent Inhibitors of Wild-Type and Multi-Drug-Resistant HIV, NIH, $620,000

Kelly Goldsmith, MD
Immune Competent 3D Bio-Printed Organoids to Study and Target Barriers to Cellular Therapy for Neuroblastoma, U.S. Department of Defense, $620,000

New Research Recruits

Bhavya Doshi, MD
Hematology

Hananeh Fonoudi, PhD
Cardiology

Chrystian Junqueira-Alves, PhD
Neurosciences

Kathleen Kraus, PhD
Behavioral and Mental Health

Deborah Luessen, PhD
Neurosciences

Philip Lupo, PhD
Genetic Epidemiology

Esther Obeng, MD, PhD
Hematology

Chris Rees, MD
Emergency Medicine

Michael Scheurer, PhD
Oncology

Francesca Vinchi, PhD
Hematology

Satish Viswanath, PhD
Artificial Intelligence

Orthopedics

Nicholas Fletcher, MD

When to Operate: Meta-Analysis of Angulation Guidelines for Pediatric Forearm Fractures

Forearm fractures are one of the most common pediatric injuries treated by orthopedists. Often, these fractures heal effectively through closed reduction and casting without the need for surgical intervention. But when should surgical intervention be considered the proper treatment for both bone forearm fractures (BBFFs)?

Currently, orthopedic surgeons rely on established angulation criteria—at what angulation, or degree of tilt in a fracture, surgery is recommended—to guide treatment decisions. In a study overseen by Children's pediatric orthopedic surgeon Nicholas Fletcher, MD, a team of researchers sought to validate these criteria by performing a systematic review and meta-analysis of all scientific research on the topic going back to 1967.

Their findings, published in the Journal of the Pediatric Orthopaedic Society of North America, determined that the existing guideline of 15% or more angulation is acceptable, as those BBFFs are at a higher risk of loss of motion and unlikely to remodel through casting alone. Therefore, this group should continue to be considered for surgical intervention. However, the report cautioned that further high-quality research is needed to determine if the currently accepted guidelines are indeed best-practice guidelines, as very little research data on the topic has been published over the past half-century.

"There is a tremendous need for a well-done, long-term study on acceptable angulation criteria because of a fair amount of uncertainty about how to treat these fractures and what level of angulation can be tolerated without the need for manipulation or surgery," explained Dr. Fletcher, Medical Director of the Spine Program at Children's and Professor of Orthopedic Surgery at Emory. This study did not receive extramural funding.

Fresh Osteochondral Allograft Transplant Helps Young Athletes Return to Sport Sooner

Crystal Perkins, MD

Young, high-level athletes suffering a debilitating elbow injury typically have one goal in mind: to get back on the playing field at full capacity as soon as possible. To help determine if an innovative treatment is improving the odds of doing so, researchers at Children's conducted a single-center prospective study to gauge the efficacy of a recently adopted surgical procedure to treat osteochondritis dissecans, a condition that impacts the bone and cartilage of an affected joint.

S. Clifton Willimon, MD

The study findings, published in the American Journal of Sports Medicine, revealed that fresh osteochondral allograft transplant or OCAlloT, a new procedure that uses bone and cartilage from a donor to replace diseased bone in the elbow, enabled patients with elbow injury to return to their sport within two years 96% of the time. This is nearly twice the rate of success among high-level upper extremity athletes who underwent conventional surgical treatment for osteochondritis dissecans with arthroscopic debridement.

Michael T. Busch, MD

"We see a number of high-level athletes come through our doors at Children's with promising careers and futures," said Crystal Perkins, MD, pediatric orthopedic surgeon and first author on the study. "Our sports medicine team was committed to finding a solution for these athletes, optimizing their surgical treatment and closely monitoring their outcomes."

Children's sports medicine surgeons, including Dr. Perkins, S. Clifton Willimon, MD, and Michael T. Busch, MD, followed 26 Children's patients ages 11 to 15 who had the OCAlloT surgical procedure and were seen for follow-up evaluation at two years. They analyzed data on how the patient's elbows functioned before and after surgery, if they returned to sports and, if so, at what capacity.

When a severe case of osteochondritis dissecans affects the elbow's capitellum, some high-level athletes respond well to surgery that removes the damaged cartilage and replaces it with a bone and cartilage graft from the patient's knee. However, many of those patients are at risk for injury to the otherwise healthy knee. This risk is eliminated if the OCAlloT method is used.

Dr. Perkins is Medical Director, Orthopedic Quality and Outcomes for the Orthopedics and Sports Medicine Program. Dr. Busch is Surgical Director of the Sports Medicine Program and Director of the Pediatric Orthopedic Fellowship Program, and Dr. Willimon is Chief of Orthopedics and Sports Medicine at Children's. The study did not receive extramural funding.

Neonatology

National Trial Led by Children's Aims to Improve Survival in Extremely Preterm Infants

Ravi Patel, MD, MSc

Researchers at Children's and Emory are spearheading a national trial that aims to improve survival and outcomes for extremely preterm infants. Born at 23 to 26 weeks gestation, these infants have the highest incidence of bleeding and abnormally low platelets in the blood compared to more mature infants, and they have the highest mortality among all babies admitted to the Neonatal Intensive Care Unit (NICU).

The seven-year, multi-site trial will determine the best threshold for administering platelet transfusions to extremely preterm infants. Led by principal investigator Ravi Patel, MD, MSc, a neonatologist and Director of Neonatal Clinical Research at Children's, the trial is underway at 21 centers encompassing several dozen NICUs throughout the U.S., including the the lead NICU at Children's. Researchers intend to enroll more than 2,000 infants across all trial sites, with around 100 patients enrolled at Children's.

"The trial will test the hypothesis that among extremely preterm infants, a low platelet transfusion threshold, compared to a high threshold, will improve survival without major or severe bleeding up to 40 weeks," said Dr. Patel, who is also a Professor of Pediatrics at Emory.

Platelet transfusion timing can be critical as the risk of bleeding is highest in the first week after birth. A platelet transfusion may decrease the risk of bleeding by helping clots form, but neonatal researchers have not yet determined the best threshold for giving these transfusions based on platelet counts.

"We hope knowledge generated from the trial may improve outcomes in our most vulnerable patients," Dr. Patel added.

The trial is funded by an $8.7 million grant from the National Institutes of Health (NIH). Participating NICUs include members of the National Institute of Child Health and Human Development (NICHD) Neonatal Research Network, a consortium of NICUs with established site research infrastructure and extensive experience conducting multicenter clinical trials of high-risk infants, along with other select centers around the U.S.

Nathalie L. Maitre, MD, PhD

Social Media Connections Improve Knowledge, Decrease Stress for Caregivers of Infants With Early Motor Delays

Parents or caregivers of infants with early motor delays and other impairments often experience stress as they wait for specialized early intervention services to begin. However, increasing their knowledge by connecting with each other and medical experts via social media has been shown to decrease their stress levels significantly. That was the key finding from a single-blind randomized controlled trial conducted by researchers at Children's and Emory and published in Pediatric Research.

First author Nathalie L. Maitre, MD, PhD, and her team discovered that caregivers receiving targeted interactions via Facebook private groups had greater knowledge gains, felt more connected and empowered, and experienced fewer instances of parental distress compared to caregivers who did not receive social media-delivered intervention. The types of interventions administered included knowledge questionnaires, parental self-efficacy scales and the Parenting Stress Index. Study participants were caregivers of children ages 3 to 36 months with motor delays, including those also diagnosed with cerebral palsy or other types of complex medical conditions, such as chronic lung disease or a feeding disorder.

The researchers concluded that further studies are needed to determine whether social media-delivered interventions can also improve the motor outcomes of children with motor delays.

Dr. Maitre is a neonatologist at Children's as well as a Professor of Pediatrics and Director of Early Development and Cerebral Palsy at Emory. The study was funded through a private donor to Children's Healthcare of Atlanta Foundation and the Cerebral Palsy Foundation.

Puneet Sharma, MD speaking at TEDxAtlanta.

Research Reveals Potential of AI to Enhance Treatment Outcomes for Critically Ill Preterm Babies

Puneet Sharma, MD, a neonatologist at Children's, is conducting breakthrough research using artificial intelligence (AI) that may one day significantly improve the treatment of a heart condition that can be fatal in preterm infants. His work focuses on developing machine learning models to predict the efficacy of pharmacologic treatment for patent ductus arteriosus (PDA), a heart defect in which a temporary blood vessel connecting the aorta and pulmonary artery remains open after birth, increasing the risk for high blood pressure in the lungs and heart failure.

Results of a study led by Dr. Sharma, published in the Journal of the American Society of Echocardiography, indicate AI is a promising tool in predicting which patients will respond to pharmacological treatment of PDA, and thus may enhance treatment outcomes for critically ill preterm babies. It is the first published application of deep-learning methods to predict the likelihood of PDA closure after pharmacotherapy.

The randomized study followed 174 preterm infants who received pharmacologic treatment for PDA.

Two AI models were developed for comparison: a convolutional neural network (CNN) based on patients' echocardiograms alone, and a multi-modal CNN based on both echocardiograms and perinatal data.

Findings revealed that the multi-modal CNN model showed promise in predicting the success of PDA closure after an initial course of pharmacotherapy. It outperformed the imaging-only model, as well as other statistical analyses that clinicians often use in predicting patient response to medication for PDA closure.

Dr. Sharma spoke about this research during a TEDx Atlanta talk which was selected as Editor's Choice by TEDx. As an Assistant Professor of Pediatrics at Emory, he has devoted much of his research in neonatology to the ethical, equitable and transformative integration of AI into neonatal care. His work, supported by the National Institutes of Health and the American Academy of Pediatrics, aims to transform neonatal care by using AI to analyze vast healthcare data, identify patterns and support clinical decision-making.

Behavioral and Mental Health

Chief of Behavioral and Mental Health Serves on AACAP Presidential Taskforce on Emotional Outbursts in Children

John Constantino, MD

Consistent with his career-long focus on advancing research and innovative practices for long-term mental health outcomes in children and adolescents, John Constantino, MD, a nationally recognized child psychiatrist and Chief of Behavioral and Mental Health at Children's, served on the American Academy of Child and Adolescent Psychiatry (AACAP) Presidential Initiative on Emotion Dysregulation in Youth Taskforce.

The taskforce, comprised of leaders and innovators in pediatric mental health, spent two years addressing the complexities of the aggressive behaviors and emotions associated with outbursts in youth. They developed methods for identifying, characterizing and treating impairing emotional outbursts, and made recommendations for a research agenda to guide future research and funding.

Their key findings, "Research Agenda in Childhood Impairing Emotional Outbursts: A Report of the AACAP Presidential Taskforce on Emotional Dysregulation," were published in the Journal of the American Academy of Child and Adolescent Psychiatry (JAACAP) Open. This report included qualitative findings that addressed the measurement of outbursts, questions linking outbursts to other psychopathologies, and a discussion of how behavior in outbursts is separable from typical behavior. The report did not receive any extramural funding.

Dr. Constantino joined Children's in 2022 as its inaugural Chief of Behavioral and Mental Health and then became the Liz and Frank Blake Chair for Children's Behavioral and Mental Health at Emory, where he also serves as Professor of Pediatrics, Psychiatry and Behavioral Sciences, and Vice Chair of the Department of Pediatrics. He and the behavioral and mental health team are attempting to leverage 30 years of progress in the science of child mental health to help health systems nationwide ensure that proven, medically necessary interventions reach all children whose lives would be improved by them.

Neurology

Experimental Drug CT-179 Shows Potential for Halting Pediatric Brain Cancer Recurrence

Timothy Gershon, MD, PhD

Children with medulloblastoma (MB), the most common form of pediatric brain cancer, may one day benefit from a new treatment that reduces the likelihood of recurrence and improves quality of life, according to the results of an international study co-authored by Children's pediatric neurologist Timothy Gershon, MD, PhD.

Dr. Gershon, a Professor of Pediatrics at Emory, and a team of researchers representing five countries found that a potential new targeted therapy using the experimental small-molecule drug CT-179 in tandem with other proven therapies was effective in infiltrating and killing tumor cells in preclinical models. The researchers found that CT-179 effectively targets the protein OLIG2, a known stem cell marker crucial in the initiation and recurrence of brain cancers.

"Current treatments, including radiation and chemotherapy, often eliminate most of the tumor, but sometimes fail to eliminate cancer stem cells that can cause recurrence," explained Dr. Gershon, a member of the Cell and Molecular Biology Research Program at Winship Cancer Institute of Emory.

"Combining CT-179 with treatments such as radiation therapy treats the whole tumor more effectively and may bring new efficacy to brain tumor therapy."

Researchers hope the promising results in the preclinical study pave the way for the first in-human clinical testing of CT-179 in patients with brain cancer. The study, published in Nature Communications, was supported by donations from several sponsors, including Children's Hospital Foundation, Children's Brain Cancer Centre, Sid Faithfull Group, QIMR Berghofer Medical Research Institute and others. The international collaboration included institutions in Canada, Australia, the U.S., Korea and Sweden.

Marcus Autism Center

Elopement Behavior in Autistic Children Improves With Function-Based Training for Parents

Nathan Call, PhD

Mindy Scheithauer, PhD

Many children with autism inadvertently put their health and wellness at risk by wandering away from their parents or caregivers. This behavior, known as elopement, may include leaving the house in the middle of the night or getting lost from a parent in a crowded location, which can be dangerous for the child and extraordinarily stressful for parents. To better understand why elopement occurs and how it can be deterred, researchers at Marcus Autism Center, a subsidiary of Children's, conducted a 16-week randomized clinical trial of 76 autistic children ages 4 to 12 and their caregivers.

Their findings, published in the journal Autism, found that when caregivers receive function-based elopement training with targeted strategies to improve a child's safety and reduce elopement, the children in their care showed greater improvement in elopement than children whose parents received more general education. Using the Clinical Global Impression-Improvement Scale (CGI-I) to rate elopement revealed that 31.6% of study participants who received function-based elopement treatment improved compared to 2.6% of participants who received general parent education.

Leading the research team was Mindy Scheithauer, PhD, an Associate Professor of Pediatrics at Emory and Complex Behavior Support Program Manager at Marcus Autism Center, where she also serves as Brief Behavioral Intervention Program Manager. Nathan Call, PhD, Clinical Director at Marcus Autism Center and Professor of Pediatrics at Emory, oversaw the study. Funding support for the study was provided by Autism Speaks®.

Cancer and Blood Disorders

World's First Publicly Available Single-Cell Atlas for Pediatric Leukemia

Manoj Bhasin, PhD, MS

Investigators from the Aflac Cancer and Blood Disorders Center and Winship Cancer Institute of Emory are giving pediatric leukemia researchers unprecedented access to an innovative new tool to help better understand and treat the most prevalent childhood cancer. The Pediatric Single-cell Cancer Atlas (PedSCAtlas) is the world's first publicly available online single-cell atlas for pediatric leukemia. It is designed to support the broader research community in identifying new biomarkers and therapeutic targets and gaining insights into leukemia biology at the single-cell level.

As detailed in an in-depth study published in Nature Communications, senior author Manoj Bhasin, PhD, MS, and a large multidisciplinary team analyzed more than 540,000 cells from 159 pediatric leukemia and healthy bone marrow samples obtained from a biorepository at the Aflac Cancer and Blood Disorders Center. The researchers used cutting-edge single-cell RNA sequencing to reveal the diversity of cell types and gene expression patterns within pediatric cancers. By making these resources publicly available, they hope to accelerate discovery in pediatric cancer biology and support the development of more effective treatments.

"The pediatric atlas is not just a data set," said Dr. Bhasin. "It's a tool for the entire cancer research community to understand the contribution of cancer and immune cells in pediatric leukemia outcomes. This study enables the generation of a pan-pediatric leukemia signature that may be associated with the malignant transformation of hematopoietic cells."

Dr. Bhasin is Director of Genomics, Proteomics, Bioinformatics and Systems Biology at Children's, Director of the Single Cell Biology Program at the Aflac Cancer and Blood Disorders Center and Professor of Pediatrics at Emory. The study was funded by the CURE Childhood Cancer Foundation, NIH and Emory.

H. Trent Spencer, PhD

First-in-Human Clinical Study Indicates Promising New Gene Therapy for Hemophilia A

For more than 20 years, researchers have investigated gene therapy for the treatment of hemophilia A with mixed results. But a recent, first-in-human clinical study published in the New England Journal of Medicine and led by senior author H. Trent Spencer, PhD, a specialist in gene therapy at Children's and Emory, has revealed a promising new opportunity for using gene therapy in severe cases of hemophilia A. The therapy may one day be available to patients of all ages, including children.

The single-center study, conducted in India under first author Alok Srivastava, MD, involved five participants with severe hemophilia A, a blood disorder that occurs due to a lack of or deficiency in factor VIII, a protein that is essential for blood clotting. After extensive pre-clinical testing, participants were administered a form of gene therapy that used lentiviral vector–transduced autologous hematopoietic stem cells (HSCs).

Results revealed the gene therapy did not result in any unexpected complications and was effective in stabilizing factor VIII expression to levels that eliminated or vastly reduced spontaneous bleeding into joints and muscles, as well as sporadic life-threatening bleeding. The study authors recommend longer follow-up and additional clinical trials with more participants to further gauge the treatment's efficacy.

Dr. Spencer is Professor of Pediatrics at Emory, Research Director of the Marcus Center for Cellular Therapy at Children's and a scientist in the Aflac Cancer and Blood Disorders Center. During the trial, Dr. Srivastava was a professor in the Department of Clinical Hematology at Christian Medical College Vellore in India. Funding for the study was provided by Hemophilia of Georgia, the National Institutes of Health and India's Ministry of Science and Technology.

Phase I Study Uncovers New Approach to Treating Patients With Relapsed or Refractory Neuroblastoma

Thomas Cash, MD, MSc

Kelly Goldsmith, MD

A phase I clinical study led by pediatric hematology and oncology researchers from Children's and Emory has revealed innovative approaches to treating children with neuroblastoma, one of the most common tumors in infants and young children that initiates in the early nerve cells of the sympathetic nervous system.

The results of the study, published in the Journal of Clinical Oncology, revealed that combining the systemic radiotherapy 131I-metaiodobenzylguanidine with the anti-GD2 antibody dinutuximab (with or without the histone deacetylase inhibitor vorinostat) was effective in inhibiting the growth of tumors in most children with relapsed or refractory neuroblastoma.

The study was overseen by Kelly Goldsmith, MD, who serves as Director of the Neuroblastoma/MIBG Therapy Program and Clinical Director of the Precision Medicine Program at the Aflac Cancer and Blood Disorders Center, Curing Kids Cancer Professor of Pediatric Oncology at Emory and co-leader of the Discovery and Developmental Therapeutics Research Program at Winship Cancer Institute of Emory.

Study chair Thomas Cash, MD, MSc, was first author on the research, which was conducted through the New Approaches to Neuroblastoma Therapy consortium. Dr. Cash is Director of the Solid Tumor Program at the Aflac Cancer and Blood Disorders Center and Associate Professor of Pediatrics at Emory. His research focuses on translating novel therapeutic agents and combinations into clinical trials to improve outcomes for children with high-risk solid tumors.

Funding for the study was provided by United Therapeutics Corporation, CURE Childhood Cancer, Aflac Cancer and Blood Disorders Center, NIH, Band of Parents, St. Baldrick's Foundation, The V Foundation, Catherine Elizabeth Blair Foundation, Vanguard Charitable and Alex's Lemonade Stand Foundation.

Pulmonology

Rachel Linnemann, MD, patient James Cargal and Ajay Kasi, MD

Children's-Led Studies Pave the Way for FDA Approval of New Cystic Fibrosis Treatment

Two research studies led by pediatric pulmonologists at Children's and Emory have shown the effectiveness of the new drug Alyftrek™ in treating certain mutations of cystic fibrosis (CF). The findings from both studies resulted in the drug's approval by the U.S. Food and Drug Administration (FDA).

Ajay Kasi, MD, was co-author for a phase 3 clinical trial conducted at 33 sites globally that determined Alyftrek was effective in 6- to 11-year-old patients with certain mutations of CF. His colleague, Rachel Linnemann, MD, was co-author for a phase 3 clinical trial conducted at more than 100 sites globally that determined once-daily Alyftrek was more effective at lowering sweat chloride levels than twice-daily Trikafta in patients with CF ages 12 and up. Both studies included patients from the Cystic Fibrosis Care Center at Children's. They were funded by Vertex Pharmaceuticals and were published in The Lancet Respiratory Medicine.

Alyftrek is a cystic fibrosis transmembrane conductance regulator (CFTR) modulator for people with CF ages 6 and older who have a mutation eligible for Trikafta, or one of 31 rare mutations that had not been previously approved. "This next-generation medication will be available for even more individuals with CF of various genotypes and may provide an additional option for those that could not tolerate Trikafta," said Dr. Linnemann, Director of the Cystic Fibrosis Care Center at Children's and Associate Professor of Pediatrics at Emory.

"The FDA approval of Alyftrek is a significant milestone for people with cystic fibrosis," added Dr. Kasi, Medical Director of the Technology-Dependent Pulmonary Program at Children's and Associate Professor of Pediatrics at Emory.

Nephrology

E-Phenotypes Produce Useful Incidence Estimates for Five Leading Causes of Pediatric CKD

Chia-shi Wang, MD

Larry Greenbaum, MD, PhD

Edwin Smith, MD

Because pediatric chronic kidney disease (CKD) is rare and often hard to detect, data on how often it occurs is limited, making it more difficult for clinicians and researchers to estimate its rate of incidence.

That prompted a team of researchers, including Children's physicians Chia-shi Wang, MD; Larry Greenbaum, MD, PhD; and Edwin Smith, MD, to develop electronic health record-based algorithms (known as computable e-phenotypes) to identify pediatric CKD cases by underlying diagnosis. They then used that data to produce incidence estimates for five leading causes of pediatric CKD.

The results of the study, published in Kidney360, revealed that the incidences of the five leading causes of pediatric CKD per 100,000 children are higher than previously realized. While the e-phenotypes require validation for use at other institutions, they offer further opportunities to examine determinants of CKD detection, management and outcomes.

The e-phenotypes were tested using a cohort of 1,000 pediatric patients at Children's diagnosed with CKD between 2014 and 2023. The five causes of pediatric CKD included in the study were: autosomal dominant polycystic kidney disease (ADPKD), Alport syndrome (AS), congenital anomalies of the kidney and urinary tract (CAKUT), lupus nephritis (LN) and primary childhood nephrotic syndrome (NS).

Dr. Wang, a pediatric nephrologist at Children's, is Associate Professor of Pediatrics at Emory. Dr. Greenbaum is a pediatric nephrologist at Children's and the Bernard Marcus Professor of Pediatric Nephrology at Emory. Dr. Smith is a pediatric urologist and Surgeon in Chief at Children's. The study was funded by the U.S. Centers for Disease Control and Prevention (CDC) Foundation.

Cardiology

First-Ever Study of Cardiac Stem Cell Treatment in Pediatric Patients With Hypoplastic Left Heart Syndrome

Michael Davis, PhD

William Mahle, MD

Paul Chai, MD

Timothy Slesnick, MD

Subhadra Shashidharan, MD

Babies born with hypoplastic left heart syndrome (HLHS)—a severe congenital heart defect—typically require three sequential surgeries before the age of 3 to reconstruct the heart and restore sufficient blood flow to the body. These surgeries put babies at risk for severe complications affecting the heart's right ventricle, which often leads to a shortened lifespan. Several preclinical studies indicate that a form of stem cell therapy using neonatal cardiac progenitor stem cells (nCPCs) may help treat surgery-related damage to the right ventricle. Yet, nCPC injections had never been attempted on pediatric HLHS patients until recently.

Researchers and surgeons at Children's, Emory University and Georgia Tech were among the co-authors of a Phase I randomized clinical trial that sought to evaluate the effects of autologous nCPCs injected into the right ventricle myocardium during the second HLHS operation.

Although the findings did not show improvement in the functionality of stressed and overloaded right ventricles, it did reveal that nCPC injections showed several other promising clinical benefits, including a reduction in mortality, major adverse cardiac events and the need for heart transplantation, as well as shorter hospitalizations and improvements in quality of life.

The study, which received funding support from The Marcus Foundation and the University of Miami Miller School of Medicine, was published in the Journal of the American College of Cardiology: Heart Failure.

The research was overseen by Michael Davis, PhD, Director of the Children's Heart Research and Outcomes (HeRO) Center and Professor of Biomedical Engineering at Georgia Tech and Emory. Co-authors included William Mahle, MD, Chief of Cardiology and Co-Chief of the Heart Center at Children's and Marcus Professor of Pediatrics at Emory; Paul Chai, MD, Chief of Cardiothoracic Surgery at Children's and Professor of Surgery at Emory; pediatric cardiologist Timothy Slesnick, MD, Director of Cardiac MRI at Children's and Professor of Pediatrics at Emory; and Subhadra Shashidharan, MD, pediatric congenital cardiothoracic surgeon at Children's and Associate Professor of Pediatrics at Emory.

Hunter Wilson, MD

Allen Ligon, MD

Study Confirms 3D Virtual Reality Models Are an Effective Planning Tool for PDA Stenting

Clinicians now have a high-tech tool to help plan and execute a complex procedure called transcatheter patent ductus arteriosus (PDA) stenting to treat infants with ductal-dependent cyanotic congenital heart disease (CHD).

A retrospective study of 42 infants admitted to Children's with ductal-dependent cyanotic CHD found that 3D virtual reality (VR) PDA models allow for reliable measurements of the duct's length. Researchers also examined how these PDA models work with virtual stents, using VR to predict the best stent size and safest vascular access, and then compared those predictions to procedural outcomes.

The findings, published in Pediatric Cardiology, prompted the study's co-authors to conclude that high-fidelity VR models are feasible for PDA stent planning, demonstrate high predictive performance for vascular access and have excellent rater reliability for ductal measurements. As a result, they recommended integrating VR modeling into pre-procedural workflows to improve planning.

The study was led by senior author Hunter Wilson, MD, pediatric cardiologist at Children's Heart Center and Assistant Professor at Emory. Co-author and pediatric cardiologist Allen Ligon, MD, is Director of the Transcatheter Closure of the PDA Program and Assistant Director of Interventional Cardiology at Children's Heart Center and Assistant Professor at Emory. The research did not receive extramural funding.

Infectious Diseases

Ann M. Chahroudi, MD, PhD

Model Predicts Viral Rebound Timing in Pediatric HIV Patients, a Key Determinant for Treatment Strategies

Many adults with human immunodeficiency virus (HIV) volunteer for clinical studies that evaluate the efficacy of strategies to cure the virus. However, since those evaluations require participants to interrupt their HIV treatment for the duration of the trial, pediatric participants are rarely included due to heightened ethical and safety concerns in this vulnerable population.

That prompted a team of infectious disease researchers from Children's and Emory to create a robust preclinical model of pediatric HIV that seeks to predict the time to viral rebound after interruption of antiretroviral therapy (ART). Results revealed potential biomarkers to screen children with HIV being considered for trials that include analytical treatment interruption of ART.

The research may have significant ramifications for the 1.4 million children worldwide living with HIV, and the approximately 120,000 infants who contract the disease annually, half of them through breast feeding from a mother with HIV. While the findings require further validation, this study lays the foundation for future research to assess the viability of pediatric ART interruption.

Ann M. Chahroudi, MD, PhD, pediatric infectious diseases physician at Children's and Professor of Pediatrics at Emory, oversaw the study. The study, published in Science Translational Medicine, received funding support from three NIH grants. Co-authors included 11 faculty and staff from Children's and Emory (listed below):

- Veronica Obregon-Perko, PhD
- Bhrugu Yagnik, PhD
- Tiffany Styles
- Greg Tharp
- Prachi Gupta
- Gloria Mensah
- Margaret Neja
- Maud Mavigner, PhD
- Guido Silvestri, MD
- Rama Amara, PhD
- Steven Bosinger, PhD

Surveillance Network Enhances Detection and Treatment of Acute Febrile Illnesses in Belize

Kristy Murray, DVM, PhD

Kristy Murray, DVM, PhD, Chief Research Officer, Children's, spearheaded the development of a surveillance network in the Central American country of Belize that is helping physicians identify and treat children and adults who are carriers of potentially life-threatening, disease-causing organisms.

Since its founding in early 2020, the Belize Acute Febrile Illness (AFI) Surveillance Network has enrolled thousands of Belize residents and tourists who are patients at 11 participating public hospitals. Researchers test patients to detect and diagnose 54 vector-borne, respiratory and gastrointestinal pathogens.

At least one pathogen has been detected in 51% of participants, including the first known case of acute Chagas disease in a 7-year-old child, as well as a small outbreak of non-epidemic Vibrio cholerae. Results from the Belize AFI Surveillance Network are communicated in real time through automated data analytics. The information is then used to assist the country's medical practitioners, researchers and health officials in responding to outbreaks more rapidly and to more effectively monitor disease activity.

As reported in The American Journal of Tropical Medicine and Hygiene, the network was expanded to include detection of SARS-CoV-2 infections after the outbreak of COVID-19. Emerging and re-emerging pathogens are at high risk for transmission in Central America, which has large population centers, areas of extreme poverty, limited resources for surveillance and diagnostics, and a tropical climate.

For more than two decades, Dr. Murray's research has focused on both laboratory and clinically based studies related to emerging infectious diseases. She leads the Children's and Emory pediatric research enterprise and plays an important role in the integration of clinical, research and teaching endeavors for both institutions. She also helps facilitate research for the Children's Healthcare of Atlanta Pediatric Technology Center at Georgia Tech and Morehouse School of Medicine. The current study was supported by Baylor College of Medicine and the U.S. Centers for Disease Control and Prevention (CDC).

Endocrinology

Study Reveals Race and Ethnicity Factor Into the Development of Cystic Fibrosis-Related Diabetes

Tanicia Daley Jean-Pierre, MD

Do race or ethnicity play a role in the prevalence and incidence of cystic fibrosis-related diabetes (CFRD) in children? The answer is "yes" based on findings from a retrospective cohort study of nearly 15,000 patients with CF overseen by Tanicia Daley Jean-Pierre, MD, pediatric endocrinologist at Children's.

Results from the study, published in the Journal of Cystic Fibrosis, revealed that non-Hispanic Black patients with CF had a higher prevalence of CFRD across all age groups compared to Hispanic and non-Hispanic white patients. In addition, non-Hispanic Black and Hispanic individuals in the youngest age group showed a higher incidence of CFRD. Consequently, these groups of individuals are more likely to face an accelerated decline in pulmonary function and a death rate six times higher than CF patients of all races and ethnicities.

The study is one of the first to document racial and ethnic differences in CFRD diagnoses. The investigators recommended that multicenter studies performed in diverse CF populations are needed to identify modifiable factors influencing earlier CFRD development in minority groups and their potential contribution to disparities in diabetes complications.

To conduct the research, the investigators reviewed the CF Foundation Patient Registry from 2010 to 2019. The following organizations provided funding support for the study: CF Foundation, National Institute of Diabetes and Digestive and Kidney Diseases, Seattle Children's Research Institute Summer Scholars Program and Mentored Research Career Development. Dr. Daley Jean-Pierre is an Assistant Professor of Pediatrics at Emory. Her research centers on understanding the pathophysiology, diagnosis and treatment of CFRD.

Gastroenterology

Research Review Sheds Light on Renal Bile Acid Transport Inhibitors' Impact on Liver and Kidney Diseases

Paul Dawson, PhD

An Emory professor of pediatrics spearheaded a basic science study that furthers the potential for novel strategies that may better protect both the liver and kidney in patients with forms of liver disease.

Paul Dawson, PhD, a scientist in the Division of Pediatric Gastroenterology, Hepatology and Nutrition, oversaw a research review describing biological mechanisms for renal bile acid transport. This is the process by which bile acids are filtered from the blood into the kidney tubules and then returned to the enterohepatic circulation, the system that moves substances from the liver into bile and then into the small intestine.

The review highlights very recent discoveries that challenge current thinking on the pathogenesis of cholemic nephropathy—a type of kidney dysfunction that occurs in patients with severe liver disease and jaundice—and the formation of renal tubule casts or solid, rod-shaped structures that are formed inside the kidney's renal tubes when proteins trap other components.

In children with liver disease, changes in bile acid levels and how they're filtered can disrupt how kidney transporters function. When these transporters can't manage bile acids as part of enterohepatic circulation, it can contribute to the development of cholemic nephropathy.

This review, published in the Journal of Hepatology, was part of Dr. Dawson's career-long research focus on developing new therapies and preventive measures for diseases associated with the gut-liver axis.

The study concluded that a deeper understanding of the enteronephrophepatic bile acid axis will provide further insights into the formation and treatment of liver and related diseases.

Funding support for the study was provided by the German Research Foundation, the National Institutes of Health and the Austrian Science Fund.

Urology

Review of Magnetic Resonance Urogram Showcases its Efficacy in Pediatric Urology

Andrew Kirsch, MD

Joo Choo, MD

Since its development in the early 2000s, magnetic resonance urography (MRU) has proven to be a highly effective, advanced imaging technique that provides detailed anatomical and superior functional information about the kidneys, ureters and bladder. While it has primarily been used in adults, MRU holds promise for pediatric urology patients because, unlike computed tomography (CT) scans and nuclear medicine studies, it does not require the use of ionizing radiation, too much of which can be harmful to children.

To provide a detailed explanation of how MRU is used in the pediatric population and how effectively it performs compared to standard imaging modalities, two Children's physicians published a review of this emerging technology in the International Brazilian Journal of Urology.

Andrew Kirsch, MD, Division Chief of Urology, and pediatric radiologist Joo Choo, MD, described how MRU demonstrated faster imaging and superior accuracy compared to conventional imaging. Their review also revealed that MRU offers one of the most comprehensive assessments of the urinary tract in children, enabling detailed evaluation of the renal parenchyma, collecting systems, ureters and bladder, while also providing both static and dynamic functional information.

The researchers concluded that by reducing the need for multiple diagnostic studies, MRU use in pediatric urology is expected to expand and potentially replace traditional imaging modalities in select cases due to its efficiency and diagnostic utility.

In addition to their roles at Children's, Dr. Kirsch is Adjunct Professor of Urology at Emory, and Dr. Choo is Assistant Professor of Pediatrics at Emory. There was no extramural funding support for the study.

Children's Healthcare of Atlanta Pediatric Technology Center at Georgia Tech

"Fingernail Selfie" App Shown to be Highly Effective and Globally Accessible for Anemia Testing

Wilbur Lam, MD, PhD

A "fingernail selfie" may help save lives. This is thanks to breakthrough biomedical technology originally developed under the direction of Wilbur Lam, MD, PhD, pediatric hematologist/oncologist at the Aflac Cancer and Blood Disorders Center and Co-Director of the Children's Pediatric Technology Center (PTC) at Georgia Tech, and with support from an Imlay Innovation Fund award.

Individuals with anemia, a potentially serious blood disorder affecting 1 in 3 children and adults worldwide, often require frequent blood tests to monitor the severity of the condition. But testing can be problematic for those living in medically underserved or poorer socioeconomic communities.

As detailed in a research study published in Proceedings of the National Academy of Sciences, the non-invasive smartphone app enables an anemia patient to snap a photo of their fingernails, which is then uploaded and assessed for anemia based on fingernail coloration. The app then calibrates the results with the patient's existing data. Doing so creates a personalized algorithm, thereby increasing the accuracy and precision of the information for that specific patient.

The "fingernail selfie" app was used 1.4 million times during the study and demonstrated strong performance when compared to conventional blood tests—achieving 89% sensitivity and 93% specificity for anemia detection.

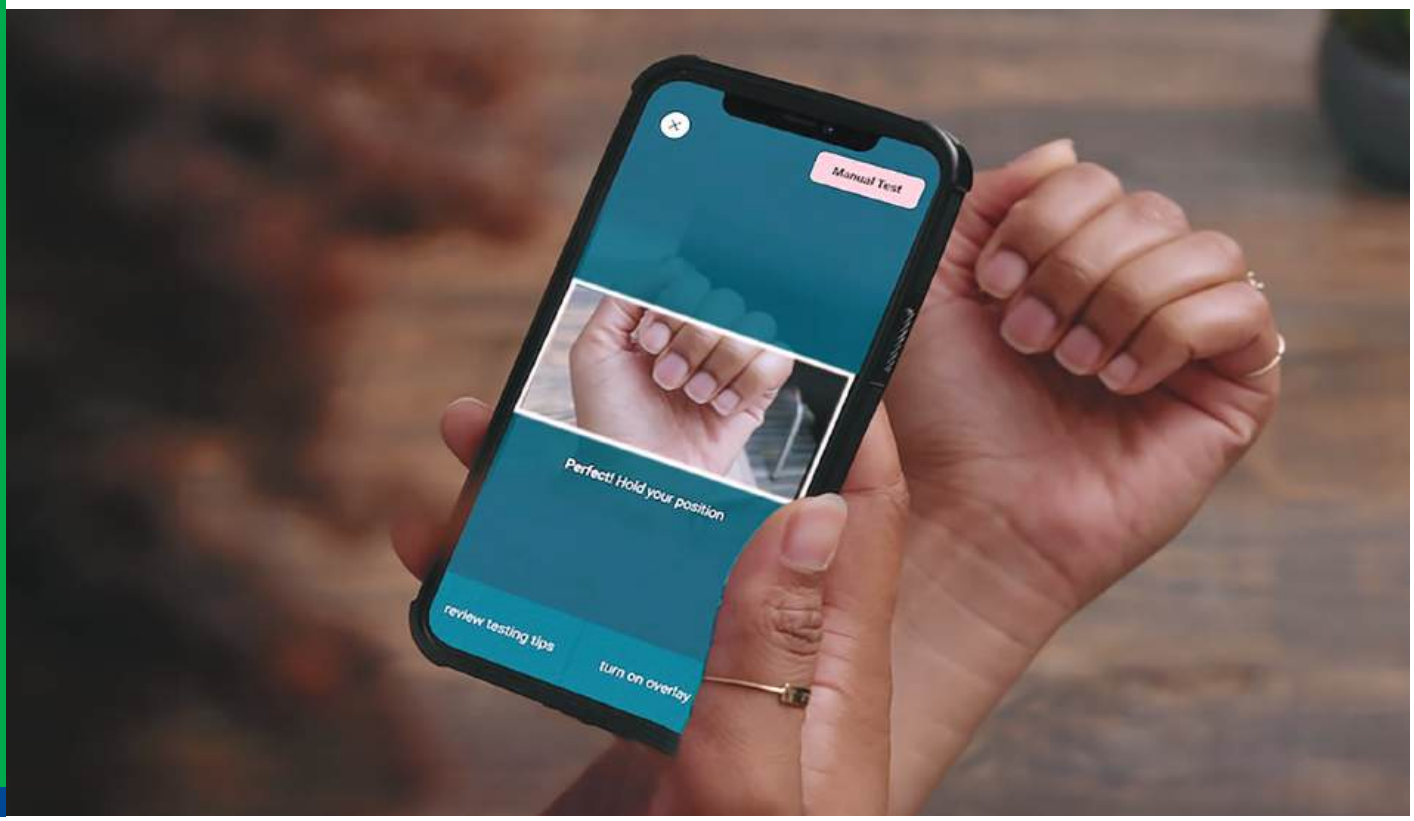
"For a disease that affects 2 billion people worldwide, this is really a game changer, as anyone can screen or monitor their anemia anytime, anywhere and as frequently as they want," said Dr. Lam, Associate Dean of Innovation for Emory, whose research interests involve developing and applying novel bioengineering technologies for hematologic disorders.

Another important aspect of the technology is its potential public health benefits. By geotagging app data, public health officials can map hemoglobin levels across the U.S., providing critical demographic and socioeconomic information.

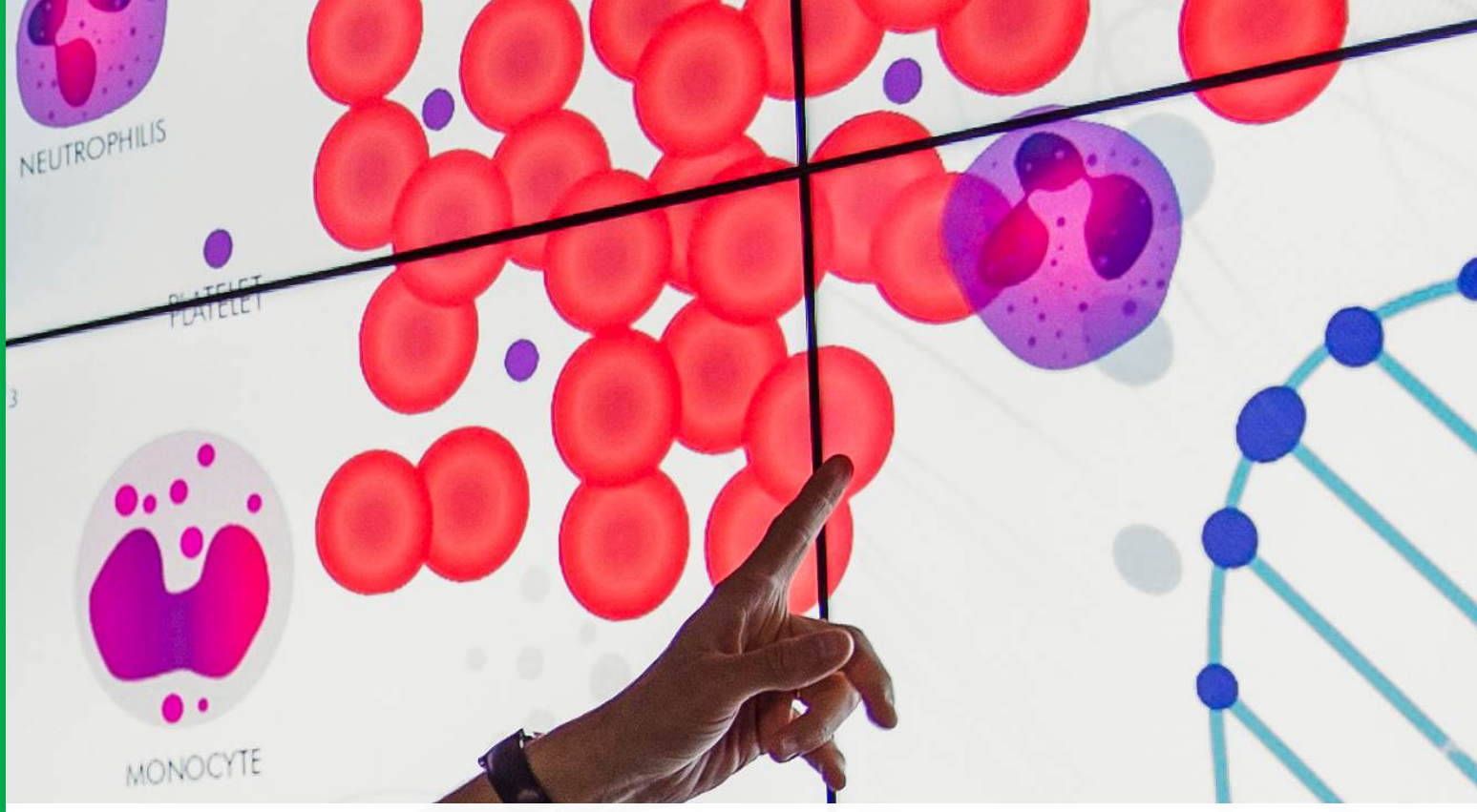
The app is part of the doctoral work of former biomedical engineering graduate student Rob Mannino, PhD, first author on the study and a former Aflac Cancer and Blood Disorders Center patient, who was motivated to conduct the research by his own experience living with beta-thalassemia, an inherited blood disorder caused by a mutation in the beta-globin gene.

Corresponding author, Erika Tyburski, and Dr. Mannino met in 2011 in Dr. Lam's bioengineering hematology laboratory while she was a Georgia Tech biomedical engineering undergraduate student and intern at the PTC. The pair worked together with Dr. Lam to launch their first anemia diagnostic technologies and to test the first prototype for this app technology. Today, Tyburski is CEO of Sanguina Inc.*, which is disseminating the app. Dr. Mannino is the company's CTO.

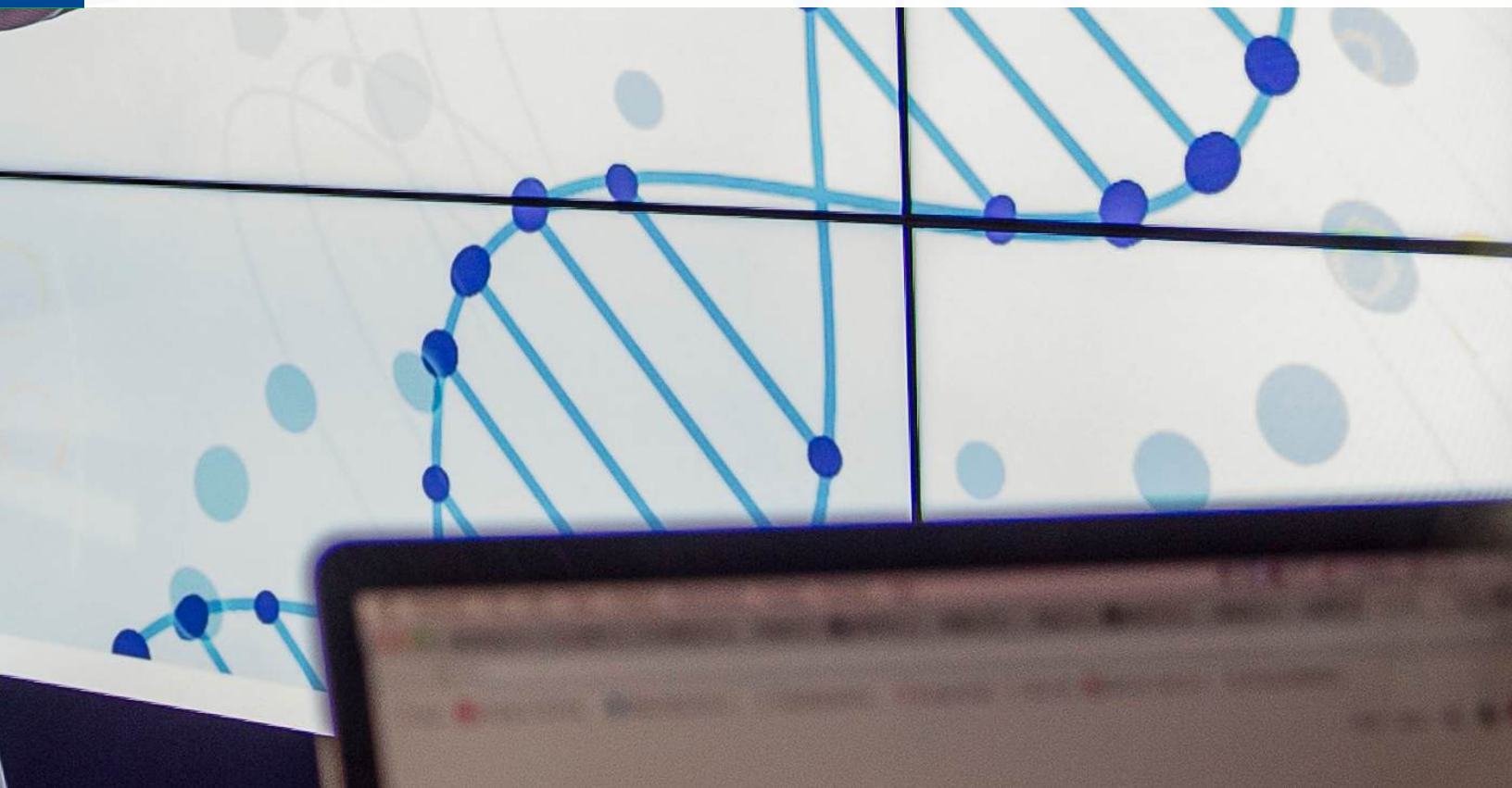
Funding for the current study was provided in part by the National Heart, Lung and Blood Institute of the National Institutes of Health and by AstraZeneca. Paul George, MD, a hematology/oncology fellow at the Aflac Cancer and Blood Disorders Center and Emory, was co-author. Dr. Lam is a tenured professor of pediatrics at Emory and a tenured professor of biomedical engineering in the Wallace H. Coulter Department of Biomedical Engineering at Emory and Georgia Tech. He also serves as inaugural vice provost for entrepreneurship for Emory University.



** Children's Healthcare of Atlanta Inc. has a license agreement in the app under evaluation and equity interests in Sanguina Inc., the company sponsoring the research. As a result of these interests, Children's Healthcare of Atlanta could potentially benefit financially from the outcomes of this research.*



EDUCATION



Graduate Medical Education by the Numbers*

806 Residents and fellows trained at Children's and Emory, with 325 dedicated exclusively to pediatrics

90% Core pediatric residents in last three years passed the pediatric board certifying exam on their first attempt

52 Pediatric residency and fellowship programs were offered

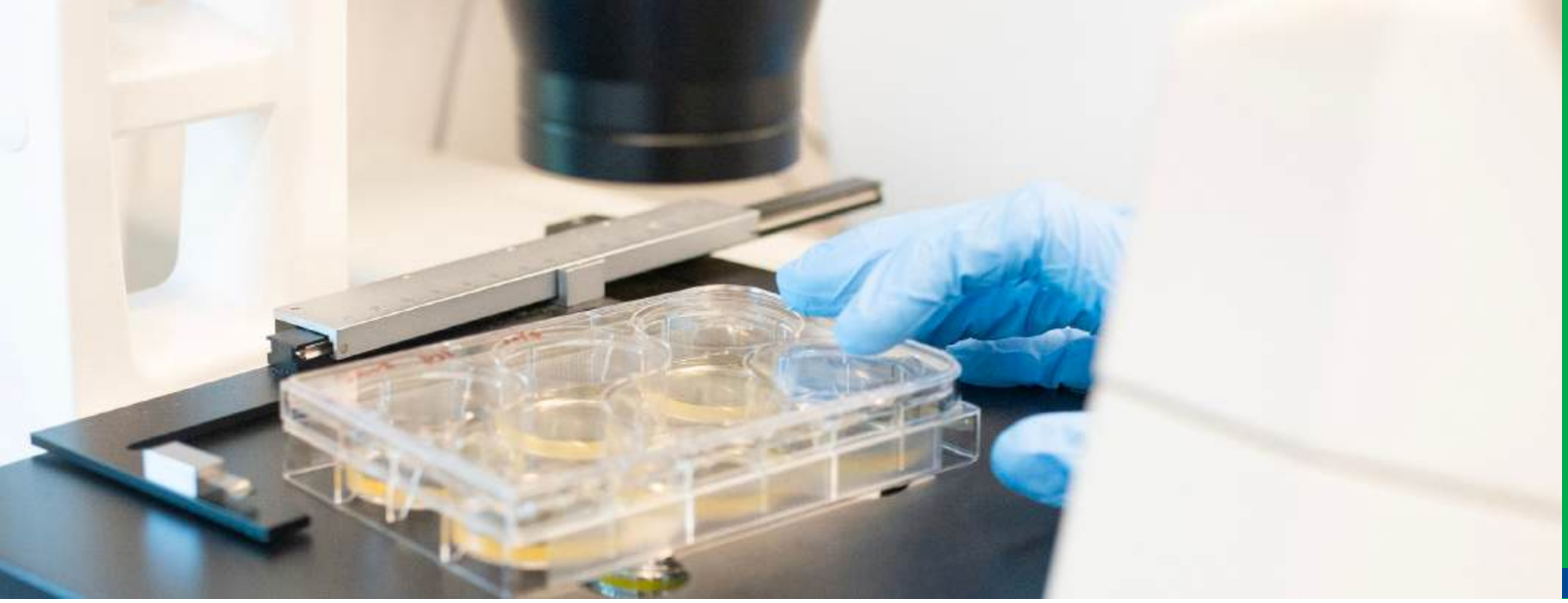
100% Slots recruited were filled for the largest Core Pediatrics Residency class to date

100% Slots recruited were filled for the inaugural Combined Internal Medicine and Pediatrics Residency class

54% Terminal graduates remained in Georgia to complete subspecialty training at Children's and Emory or to practice as employed physicians

594 Medical and physician assistant student rotations occurred at Children's

**Numbers represent the 2024-2025 academic year*



Warshaw Fellow and Buchter Resident Research Grant Awardees

Receiving funding in 2025 through the Warshaw Fellow and Buchter Resident Research Awards were seven research projects led by residents and fellows. Named in honor of Barry Warshaw, MD, former Vice Chair of Education at Emory and Chief Teaching Officer at Children's, and Susie Buchter, MD, former Pediatric Core Residency Program Director, these awards honor each educator's pioneering and tireless efforts to cultivate fellows and residents in pediatric research endeavors at Children's and Emory.

The awards were created in 2017 to encourage pediatric trainees to not only engage in high-quality research, but also to provide a mechanism to fund trainee projects and enhance research at Children's. Funds support a variety of child health projects, including basic, clinical, translational and outcomes research. Fellows receive a maximum of $5,000 and residents receive a maximum of $2,500. Applications are reviewed and selected based on the National Institutes of Health grant application scoring system.

Fellow Research Grant Awardees

- **Demy Alfonso (Neuropsychology)**, Advancing Bilingual Pediatric Neuropsychological Evaluations: The Impact of Assessment Structure
- **Akshaya Arjunan (Hematology/Oncology)**, Complement Dysregulation and Hemolytic Phenotype in Pediatric Sickle Cell Disease
- **Zuri Hudson (Hematology/Oncology)**, Defining and Targeting Gamma Delta (γδ) T Cell-Induced Apoptosis in Neuroblastoma
- **Kaveeta Kaw (Cardiology)**, Investigating ER Stress-Driven Vascular Remodeling in a Novel 3D-Bioprinted Pulmonary Arterial Hypertension Model
- **Abigail Schnaith (Nephrology)**, Caregiver Mental Health and Stress in Pediatric Chronic Kidney Disease

Pediatric Core Resident Research Grant Awardees

- **Tyler Vajdic**, Antifungal Azole-Exacerbated Neurotoxicity in Pediatric Patients With Acute Lymphoblastic Leukemia and CYP3A5 Polymorphisms Receiving Vincristine
- **Heather Xiao**, Outpatient Procedural Sedation in Pediatric Hematopoietic Stem Cell Transplantation

Dr. Warshaw holding his lifetime achievement award at the 2025 Georgia AAP Annual Awards.

Dr. Barry Warshaw

Dedicated Physician and Educator Recognized With Highest AAP Honor

After a distinguished career of more than 46 years, Barry Warshaw, MD, Chief Teaching Officer for Children's and Vice Chair of Education for the Emory Department of Pediatrics, retired in February 2026 after helping inaugurate the nephrology training program and overseeing more than 50 other physician training programs. In recognition of his unwavering commitment to education and mentorship, and his sustained contributions to the field of pediatrics, Dr. Warshaw received the Leila D. Denmark Lifetime Achievement Award from the Georgia Chapter of the American Academy of Pediatrics (AAP), the highest honor bestowed by the chapter.

An Atlanta native, Dr. Warshaw's passion for medicine began early in childhood, but it was during a pediatric rotation in his third year of medical school that Dr. Warshaw found his calling: "I was inspired by the resilience of children, and their ability to transition from tears to laughter in an instant." He completed his medical degree and pediatric residency at Children's and Emory, quickly distinguishing himself by becoming chief resident in 1975. Following his residency, he completed a fellowship in pediatric nephrology in Los Angeles, a decision that set the stage for his long and impactful career.

In 1979, Dr. Warshaw was welcomed back to Children's, where he would go on to start and grow the nephrology division. Dr. Warshaw's vision was clear from the outset: to establish a world-class nephrology division capable of offering pediatric dialysis and transplants. Through his leadership as founding division chief from 1979 to 2006, the program grew exponentially, offering these services and more. Today, it is recognized as one of the leading nephrology programs in the country, ranked in the top 20 by U.S. News and World Report.

A Career Dedicated to Teaching, Guiding and Inspiring

A staunch advocate for lifelong learning, Dr. Warshaw has worked closely with fellows, residents and medical students throughout his tenure. Dr. Warshaw believes in the importance of formalized mentorship, emphasizing that even experienced staff need guidance and should, in turn, mentor others. Among his most influential mentors was Dr. Joe Patterson, a chief physician in the 1970s at the former Egleston Hospital, who Dr. Warshaw remembers as a positive force who celebrated accomplishments and instilled confidence.

One of Dr. Warshaw's favorite contributions to graduate medical education was his involvement in the annual residency and fellowship match process, which he found both important and exciting as it evolved into the more standardized system it is today. A career highlight was the establishment of the Children's and Emory Nephrology Fellowship in 2007, a testament to his enduring legacy.

Reflecting on the evolution of medical education at Children's and Emory over the past 45 years, Dr. Warshaw describes the transformation as night and day. The Core Residency Program has grown to be one of the largest in the country with cutting-edge training in both subspecialty and primary care pediatrics. Fellowship training has evolved to educating more than 100 subspecialty fellows annually. Formal simulation centers are available, and programs now include curricula in global health, ethics, medical education, health equity and advocacy, basic and clinical research, medical innovation, quality improvement and patient safety. There is also a greater focus on personal wellness among residents and fellows. Noting the elite status of the training programs at Children's and Emory, now overseen by more than 500 faculty members employed by both institutions, Dr. Warshaw takes great pride in his city and institutions.

Dr. Warshaw's retirement marks the end of an era, but his influence will continue to be felt through the countless lives he has touched and the robust programs he has built. His legacy as a mentor, educator and innovator inspires future generations of medical professionals.

Children's and Emory Offer New Combined Internal Medicine and Pediatrics Residency Program

During summer 2025, Children's and Emory welcomed four residents into the inaugural class of the Combined Internal Medicine and Pediatrics Residency Program, a four-year physician training program offering education in both pediatric and adult general medicine. As members of one of only 80 programs of its kind in the nation, the residents represent the start of a new chapter in the Children's and Emory tradition of clinical excellence, community service and innovation in medical education.

The idea behind the program was to bridge the best of adult and pediatric medicine while reflecting Atlanta's spirit of diversity and both organizations' deep roots in community service. With this goal in mind, the program launched a continuity clinic for residents to train at Mercy Care Chamblee, which provides comprehensive primary care for patients across the lifespan, and strengthened clinical partnerships with Grady Health System and Emory Healthcare, where residents will also rotate.

Katie Gielissen, MD

The new program's director, Katie Gielissen, MD, is an Assistant Professor of Medicine in the Departments of Medicine and Pediatrics at Emory who came from Yale University where she served as an assistant professor. Dr. Gielissen earned her medical degree at the University of Chicago-Pritzker School of Medicine before completing her internal medicine-pediatrics training at Yale New Haven Hospital.

The Combined Internal Medicine and Pediatrics Residency Program is further strengthened by an exceptional team of faculty who bring a wide range of expertise, experience and commitment to resident growth:

- **Emily Pinto Taylor, MD**, Associate Program Director and Assistant Professor, Department of Medicine, Emory
- **Ronnye Rutledge, MD**, Longitudinal Advocacy Curriculum Director, Pediatrics Hospitalist, Children's; and Assistant Professor, Department of Medicine, Emory
- **Stan Sonu, MD, MPH**, Medical Director, Child Advocacy, Children's; and Associate Professor, Department of Medicine, Emory
- **Gerald Lee, MD**, Pediatric Allergist/Immunologist, Children's; and Associate Professor, Department of Pediatrics, Emory
- **Alexandra Arges, MD**, Pediatrics Hospitalist, Children's; and Assistant Professor, Department of Medicine, Emory

Together, this group exemplifies the program's focus on bridging disciplines, championing health equity and fostering the development of professional identity across internal medicine and pediatrics.

"As we celebrate the arrival of the first class, the mission remains clear: to cultivate physicians who see the full spectrum of human health—from childhood through adulthood—and who will lead in how care is delivered, taught and imagined," said Dr. Geilissen. "Strengthened by its partnerships, the new program will shape healthier tomorrows for generations to come."

Trainee Research Highlight: Facilitators and Barriers to Safe Sleep Practices in Atlanta Refugee Communities

Andrew Potter, MD

During the 2025 American Academy of Pediatrics (AAP) annual meeting, Andrew Potter, MD, a core pediatrics resident, received the best abstract award and was recognized for the best advocacy initiative in the Council on Immigrant and Child Health category for a poster presentation titled: "Facilitators and Barries to Safe Sleep Practice in Atlanta Refugee Communities." The qualitative research project underscores the importance of tailoring educational materials to meet the unique needs of vulnerable communities, recognizing that a one-size-fits-all approach is insufficient.

"Access to safe sleep education in preferred languages, with a deep understanding of cultural context, is vital to empowering immigrant families to make informed decisions that protect their infants," said Dr. Potter.

The project started with focus groups conducted in collaboration with the Center for Pan-Asian Community Services, a community outreach group and healthcare center dedicated to serving immigrants and refugees in Atlanta. The focus groups included Swahili-speaking women from Tanzania and Congo, and Amharic-speaking women from Ethiopia, who provided insight into their unique challenges and perspectives.

This insight was instrumental in the design of educational videos tailored to each community's specific needs. These videos, produced in Swahili and Amharic, aim to bridge the gap between traditional practices and evidence-based safe sleep guidelines. Dr. Potter and his team plan to have residents and nurses share the videos with families during patient discharge at Arthur M. Blank Hospital and Hughes Spalding Hospital, as well as during well-child visits at Hughes Spalding Primary Care Clinic.

As part of an AAP Community Access to Child Health (CATCH) grant supporting the creation of culturally relevant safe sleep materials for immigrant and refugee communities, a residency cohort known as "PEACHES," or Pediatric Equity, Advocacy, Community Health, Environmental and Social Justice, established mentorship and community partnerships that provided the infrastructure for the research.

Training at Children's has enhanced Dr. Potter's advocacy experience, allowing him to engage with immigrant and refugee families and, as a result, he will remain at Children's and Emory to complete a pediatric hospital medicine fellowship. With support from fellowship program directors Sarah Varghese, MD, and Gargi Mukherjee, MD, Dr. Potter is involved in ongoing efforts to improve communication and discharge processes and reduce readmission rates for immigrant and refugee families in Atlanta.

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