Pioneering New Ways to Deliver Healthier Tomorrows

Children's Healthcare of Atlanta and Emory University | **2024 Research Report**

Children's℠ Healthcare of Atlanta

EMORY UNIVERSITY

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Message From Dr. Lucky Jain

Realizing the impact of pediatric research, clinical care and medical education

Dear Colleagues,

This has been a truly transformative year for all of us at Children's Healthcare of Atlanta and the Emory University School of Medicine Department of Pediatrics. We celebrated the opening of the state-of-the-art Arthur M. Blank Hospital. We welcomed Kristy Murray, DVM, PhD, as the new Chief Research Officer for Children's and Executive Vice Chair for Research for the Emory Department of Pediatrics. We also had the honor of bringing Shari Barkin, MD, on board in December 2024 as the new Pediatrician-in-Chief for Children's and Chair of the Emory Department of Pediatrics. It's been a year of incredible growth, made even more memorable for me as I announced my own retirement.

Dedicating the last three decades to Children's and Emory has been one of the biggest joys of my life. From an early age, I learned from my mother and through my education to find joy in everything I do, and I feel incredibly lucky to have found it in my work here. Through pediatric research, clinical care and medical education, I've had the privilege of helping shape healthier futures for children and their families. When my wife, Shabnam Jain, MD, MPH, and I first joined Emory and Children's Egleston Hospital in 1994, we were embraced with warmth and open arms, and since then, I've been blessed with many enriching opportunities.

One unforgettable moment in my career was receiving a call from the director of the Eunice Kennedy Shriver National Institute of Child Health and Human Development. Diana Bianchi, MD, shared that Congress had approved funding for a stillbirth prevention task force—and I was invited to co-chair it. Working with a dedicated team, we were given the opportunity to directly address this tragedy that takes away 24,000 precious lives each year in the U.S. and countless more worldwide. This work was enshrined into federal law with the passage of the Maternal and Child Health Stillbirth Prevention Act of 2024. Experiences like this truly underscore the privilege and joy of this work and demonstrate the reach and impact of the work we do together.

Over the years, I've watched the Children's and Emory Pediatrics teams grow and thrive. Back in 2004, we were ranked 49th in the nation for National Institutes of Health (NIH) funding. For the past eight years, we've held a place in the top five. U.S. News & World Report continues to recognize Children's as one of the Best Children's Hospitals in the country.

This year, our research endeavors have continued to shine. The Department of Pediatrics at Emory received $108.7 million in extramural funding for the 2024 fiscal year, supporting groundbreaking projects, such as:

- EarliPoint™ Evaluation, led by Warren Jones, PhD, Director of Research at Marcus Autism Center, and Ami Klin, PhD, Director of Marcus Autism Center, which was recognized by Time magazine as one of the top medical innovations of 2024. This tool provides an objective way for experts to assess and diagnose autism in young children, ages 16 to 30 months.
- A clinical trial at Children's and Emory led by Brian Vickery, MD, Division Chief of Allergy and Immunology, examining omalizumab, an injectable medication, which shows promise in reducing allergic reactions for children accidentally exposed to allergens like peanuts, tree nuts, eggs, milk and wheat. It is the first drug for multiple food allergies now approved by the U.S. Food and Drug Administration (FDA).

The relationships I've built with so many over the years have been deeply meaningful. Serving as the pediatrician-in-chief at Children's and chair of pediatrics at Emory has been one of the greatest privileges of my life. As I step into retirement, please know this is not goodbye—Children's and Emory will forever hold a special place in my heart.

Best wishes,

Lucky Jain, MD
Former Pediatrician-in-Chief, Children's Healthcare of Atlanta
Former Chair, Department of Pediatrics, Emory University School of Medicine

A New Vision for Pediatric Research

Kristy Murray, DVM, PhD

The Children's and Emory pediatric research enterprise has experienced tremendous growth over the last year. Kristy Murray, DVM, PhD, joined as the new Chief Research Officer at Children's and Professor and Executive Vice Chair for Research in the Department of Pediatrics at Emory. She aims for the pediatric research enterprise to be at the forefront of scientific discovery and innovation, revolutionizing pediatric healthcare globally through interdisciplinary collaboration, visionary leadership and a commitment to excellence. Dr. Murray restructured the Emory and Children's research leadership team to be a cohesive unit

that aligns on the vision and priorities for pediatric research. With an initial charge to develop a strategic roadmap to advance the joint research priorities for the next three to five years, this team includes newly named vice chairs for research:

- Ann Chahroudi, MD, PhD, Vice Chair for Basic Science
- Miriam Vos, MD, Vice Chair for Clinical Research
- Stacy Heilman, PhD, Vice Chair for Research Operations

Ann Chahroudi, MD, PhD

Miriam Vos, MD

Stacy Heilman, PhD

Additionally, Children's and Emory have initiated plans for an innovative new research center called the Center for Research in Equity and Epidemiology for Adolescent and Child Health (REACH). It will focus on health equity, epidemiology, environmental health, global health and outcomes-based research. With game changers recruited jointly by Children's and Emory, this center will help take child and population health research to new levels for Georgia and beyond.

In December 2024, Shari Barkin, MD, MSHS, began her role as the Pediatrician-in-Chief at Children's and Chair of the Department of Pediatrics at Emory. Dr. Barkin is trained as an academic general pediatrician, health outcomes and health services researcher. She is an international expert in the field of behavioral interventions and community engaged, pragmatic, randomized controlled trials to address issues like pediatric obesity. She is the former president of the Society for Pediatric Research and an elected member of the National Academy of Medicine. She will lead strategy and oversight for all parts of the Children's vision, including research, with Dr. Kristy Murray reporting to her, as well as teaching, covering more than 50 pediatric physician training programs.

Shari Barkin, MD, MSHS

Dr. Barkin will play a pivotal role in furthering our vision of becoming a unified pediatric system that does everything possible to make anything possible for kids.

$54.9 million
in funding from the NIH

7
new researchers recruited

$108 million
in total extramural funding

RESEARCH BY THE NUMBERS*

956
hours of continuing medical education (CME) credits through **86** educational activities

146
new clinical studies opened

153,263
square feet dedicated to pediatric research

240
new grant and contract awards totaling
$43 million

1,838
publications in **844** journals

771
residents and fellows in training

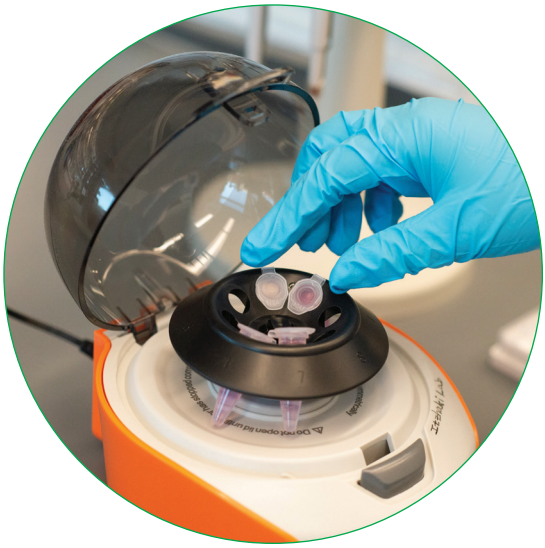
Ranked
No. 5
nationally in NIH funding for pediatric departments**

1,747
visit hours in the Children's Pediatric Research Unit

2,284
Children's patients enrolled in clinical research

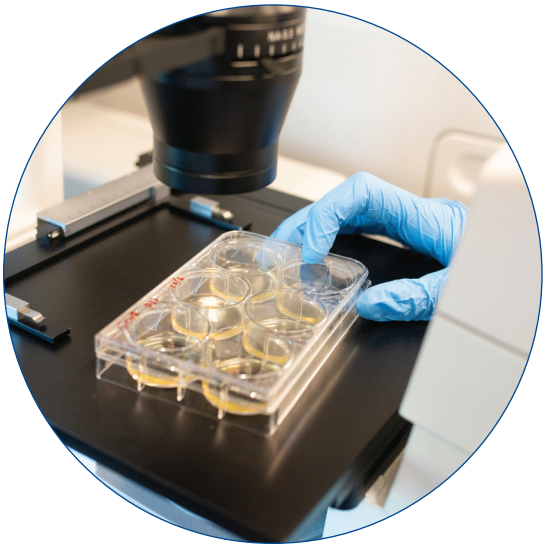
** This data represents the 2024 calendar year.*

*** NIH funding period from Oct. 1, 2023 through Sept. 30, 2024.*



New Recruits

- **Shari Barkin, MD, MSHS** (General Pediatrics)
- **Biplab Dasgupta, PhD** (Hematology/Oncology)
- **Neal Laxpati, MD, PhD** (Neurosurgery)
- **Veneet Menachery, PhD** (Infectious Diseases)
- **Kristy Murray, DVM, PhD** (Infectious Diseases)
- **Solomon Ofori-Acquah, PhD** (Hematology/Oncology)
  Georgia Research Alliance (GRA) Scholar
- **Jennifer Wong, PhD** (Neurosciences)



Large Grants

- **Wilbur Lam, MD, PhD**, Rapid Acceleration of Diagnostics (RADx) research projects, NIH, $5.5 million
- **Soumitri Sil, PhD**, Integrative Training Program for Pediatric Sickle Cell Pain, NIH, $4 million
- **Jason Yustein, MD, PhD**, Targeting TBL1/Beta-Catenin Signaling Axis for High-Risk Osteosarcoma, U.S. Department of Defense, $1.5 million
- **Lisa Ingerski, PhD**, A Biopsychosocial Approach to Advancing Juvenile Pilocytic Astrocytoma Survivorship, U.S. Department of Defense, $1.3 million
- **Latania Kisa Logan, MD**, Community Reservoirs of Extended-Spectrum Beta-Lactamase-Producing and Multi-Drug Resistant Enterobacterales, NIH, $800,000+
- **Arlene Stecenko, MD**, The Georgia Cystic Fibrosis Data Warehouse, NIH, $780,000+
- **Satoshi Kamidani, MD**, CISA Project: Simultaneous RSV Prevention Antibody (Nirsevimab) and Other Childhood Vaccines in Infants' Study, CDC, $770,000+
- **Nathalie Maitre, MD, PhD**, Cerebral Palsy Program of Excellence in Ireland, Cerebral Palsy Foundation, $750,000
- **Arlene Stecenko, MD**, Pulmonary and Pancreatic Response to Cystic Fibrosis Modulator Therapy in Young Children, NIH, $747,000

New Marcus Center for Cellular Therapy Will Give Patients Quicker Access to Novel Cell and Gene Therapies

Thanks to a generous grant from the late Bernie Marcus and The Marcus Foundation, the Marcus Center for Cellular Therapy will open at Arthur M. Blank Hospital in 2025. Through cutting-edge clinical trials, the center will facilitate the manufacturing and delivery of cellular and gene therapies, which are some of the most novel treatments available to patients with diseases resistant to standard care protocols. The state-of-the-art, good manufacturing practices (GMP) compliant lab will significantly shorten wait times for cellular and gene therapy by allowing patients to receive these experimental therapies in-house, and it is anticipated to help hasten Children's-led research examining new uses for cellular and gene therapy.

Cellular and gene therapy uses modified cells derived from a patient or healthy donor to treat various diseases and conditions when patients elect to participate in trials examining their effectiveness. Cells may be used as regenerative medicine to help repair diseased or damaged tissues, or they may be retrained to help fight disease. In gene therapy, genes are modified to address the underlying genetic cause of a disease. Although some forms of cellular therapy have received FDA approval, because the treatments are so new, most are still under investigation.

"[This] is one of the only labs for pediatrics in the country offering a full range of cellular therapy treatments for a broad spectrum of diseases—not just cancer," said Douglas Graham, MD, PhD, Chief of the Aflac Cancer and Blood Disorders Center.

"The lab will manufacture and deliver cellular therapies directly to patients at Arthur M. Blank Hospital," said Ana Marie Landin, PhD, Director, the Marcus Center for Cellular Therapy GMP Lab. "With the manufacturing in-house, patient wait times will be reduced, as the cells will not need to be transported from other centers around the country. This may provide children with quicker access to the therapies."

Shortening the Wait for Effective Treatment

Diagnosed as an infant with a severe form of sickle cell disease that causes intense pain crises and reduces life expectancy to about 50 years, 23-year-old Kyle has benefited from gene therapy. He participated in a study led at Children's by his physician,

hematologist/oncologist Suhag Parikh, MD, of the Aflac Cancer and Blood Disorders Center. The study was evaluating a one-time treatment that involved taking patients' stem cells, modifying them by adding a normal beta globin gene called LentiGlobin and then giving them back to the patient.

Kyle received this gene therapy six years ago at age 18. His stem cells had to be sent off-site to a processing facility in Massachusetts, which meant he had to wait two months to complete his treatment. Two months can feel like a lifetime when you are suffering from a debilitating disease and your dreams are at stake. For Kyle, a cure for his sickle cell disease meant that he could pursue a career as a pilot, which he'd been dreaming of since he was a preschooler.

Fortunately, Kyle's gene therapy was a success. Since receiving the therapy four years ago, he has been free of pain crises and other severe sickle cell disease symptoms. He has had zero hospital admissions, and he's now training to become a pilot.

For future Children's patients, similar therapies are anticipated to be more readily accessible. In many cases, thanks to the grant provided by Bernie Marcus and The Marcus Foundation, the Marcus Center for Cellular Therapy will allow Children's researchers to pursue their cellular and gene therapy ideas without having to take materials off-site for manufacturing, allowing them to bring the investigational therapies to the bedside more quickly.

"Because we will have the facility right here in our hospital, this will enable some of the ideas to be homegrown," said Dr. Graham. "Our own researchers will be able to develop ideas that we'll be able to deliver directly to our patients. And in some cases, it might be the only place in the country that you can get this type of therapy."

Dr. Graham added that the next phase for the center is to determine how to partner with other centers across the country to be able to deliver the treatment options not only in Atlanta, but also through a variety of sites around the nation. "We're really excited about the care for the patients we serve here in Atlanta, but also the potential to deliver these treatments across the country," he said. "Bernie Marcus has given us the ability to build a national center of excellence in cellular therapy, which affords us the opportunity to conduct world-class research. He leaves a lasting legacy within Children's."

Making Innovation Possible

The Marcus Center for Cellular Therapy is registered for accreditation with the Foundation for the Accreditation of Cellular Therapy and will receive this designation after one year of operations. The center meets the Current Good Manufacturing Practice (CGMP) regulations for human pharmaceuticals established by the FDA, which ensure proper design, monitoring and control of manufacturing processes and facilities. It includes four ISO 7 manufacturing clean rooms, a GMP analytical laboratory for quality control testing, a cryogenic storage facility, quarantined good storage area, sample processing area and a released goods storage area, as well as closed bioreactor systems. It also includes flexible configurations to support client requirements, high-volume manufacturing and dual production lines for early and late-stage clinical trials.

BEHAVIORAL AND MENTAL HEALTH

John Constantino, MD, Awarded the Nation's Highest Prize in Academic Child Psychiatry

John Constantino, MD

John Constantino, MD, Chief, Behavioral and Mental Health at Children's, received the Ruane Prize for Outstanding Achievement in Child and Adolescent Psychiatric Research. Deemed the highest prize in academic child psychiatry in the United States, the recognition honors scientists for their achievements in brain and behavior research. Dr. Constantino has made many significant contributions to the field over the course of his career, including critical advances in understanding and improving disorders of social development, such as autism and long-term consequences of adverse early life experience.

"Being selected to join such an extraordinary group of academic behavioral scientists who have received the Ruane Prize is a highlight of my career," said Dr. Constantino. "I look forward to a next wave of discovery efforts that will occur here in Georgia and at Children's thanks to the unprecedented investment of our health system in pediatric behavioral and mental health."

Since his leadership appointment in 2022 as the first Chief of Behavioral and Mental Health at Children's, Dr. Constantino has led efforts to change the landscape of behavioral and mental health care amid a growing pediatric mental health crisis. The October 2024 announcement of the Ruane Prize coincided with an editorial authored by Dr. Constantino that was published in JAMA Psychiatry. Titled, "An Architecture for Transformation in Child Mental Health," the article outlined his roadmap for improving long-term mental health outcomes for children and adolescents throughout Georgia.

The plan focuses on two main phases: The first establishes a system of care designed to reach tens of thousands of children in Georgia to help ensure those at risk for serious adverse mental health outcomes are receiving comprehensive, evidence-based intervention. The second is rigorous data collection to assess the cost and impact of this transformed model of care compared to the existing system of care.

Dr. Constantino, who is also the Liz and Frank Blake Chair for Children's Behavioral and Mental Health and a Professor in the Departments of Psychiatry and Behavioral Sciences, Pediatrics, and Human Genetics at Emory University School of Medicine, accepted the Ruane Prize which included an award of $50,000, at the annual International Awards Dinner for the Brain & Behavior Research Foundation. This is the nation's top nongovernmental funder of mental health research grants, supporting the most innovative ideas in neuroscience and psychiatry.

NEUROLOGY

Research Explores Potential of Artificial Intelligence Systems to Support Neurologists

Grace Gombolay, MD

Artificial intelligence (AI)-based decision support systems (DSS) are making inroads throughout society, including within the field of medicine. But are these tools being optimized for the benefit of the clinicians they serve?

To seek answers to that question, researchers at Children's and Georgia Tech launched a study of the effects of explainable AI (xAI) techniques on neurologists as compared to the general population. Their findings, published in the Annals of Clinical and Translational Neurology, revealed that xAI methods have different impacts on medical practitioners, that those impacts are not uniformly beneficial and that there is no one-size-fits-all approach to using xAI. Consequently, the researchers recommended further user-centered xAI research specifically targeting clinicians.

"AI is being used to help make medical decisions, but people are often less trusting if they do not understand how the AI algorithm works," explained study co-author Grace Gombolay, MD, pediatric neurologist at Children's and Associate Professor of Pediatrics at Emory. "Neurologists preferred decision trees more than the lay population, but AI was less helpful for more experienced clinicians."

Among the study's key takeaway, which the authors say should be considered when designing future tools, is that xAI has a person-specific, subjective and objective impact on clinicians. As such, DSS design must be adaptable to the needs of each end user, a critical step for improving xAI outcomes.

The randomized, blind study, which surveyed more than 300 neurologists and members of the general population, was supported by the National Center for Advancing Translational Sciences (NCATS) of the NIH and by a gift from Konica Minolta to the Georgia Tech Research Corporation.

CANCER AND BLOOD DISORDERS

Children's Co-Leads Hodgkin Lymphoma Trial Revealing New Standard of Care Option

Sharon Castellino, MD, MSc

A breakthrough multicenter clinical trial led by the Aflac Cancer and Blood Disorders Center revealed positive results that may change the standard for how advanced-stage classic Hodgkin lymphoma is treated in newly diagnosed adolescents and adults.

Based on the phase 3 trial results published in The New England Journal of Medicine, patients receiving nivolumab (brand name Opdivo®) experienced fewer side effects and had a 50% lower risk of disease progression after treatment than those receiving the standard treatment, brentuximab vedotin (brand name Adcetris®). All patients participating in the study also received AVD, a chemotherapy cocktail.

This was the largest classic Hodgkin lymphoma clinical trial ever conducted in the National Clinical Trials Network, enrolling 970 patients at 736 centers in the U.S. and Canada, including 237 between the ages of 12 and 17. The trial results are particularly significant because classic Hodgkin lymphoma is one of the most common cancers in people ages 12 to 39.

According to Sharon Castellino, MD, MSc, the study's Pediatric Chair and Director of the Leukemia and Lymphoma Program at the Aflac Cancer and Blood Disorders Center, "The high rate of progression-free survival represents another paradigm shift incorporating immunotherapy into the frontline treatment of advanced-stage classic Hodgkin lymphoma for adolescents 12 and older, with a dramatic reduction and near elimination of the need for radiation therapy."

Dr. Castellino collaborated with Kara Kelly, MD, Chair of the Department of Pediatric Oncology at Roswell Park Comprehensive Cancer Center, and a team of other national lymphoma experts during the study. Dr. Castellino also serves as Professor of Pediatrics at Emory and a research member of Winship Cancer Institute of Emory University.

Ancestral Genetic Sequence Reconstruction Identifies Factor IX Variants That May Lead to New Treatments for Hemophilia

Christopher B. Doering, PhD

Potential new treatments for hemophilia B, an inherited blood disorder caused by a lack of factor IX (a protein that helps blood clot properly to control bleeding), may one day be available thanks to the work of an Emory and Children's researcher. Christopher B. Doering, PhD, discovered an ancestral gene sequence and mapping approach that represents a promising platform for developing a wider pool of drug and gene therapies.

Dr. Doering was senior author on a study published in the Journal of Thrombosis and Haemostasis that sought to identify the amino acid substitution responsible for enhanced activity of an ancient coagulation factor IX variant in creating a transgene for gene therapy application. Using adeno-associated viral (AAV) vectors, the study successfully identified the activity and translated it into an AAV-FIX gene therapy cassette that demonstrated high potency.

"This ancestral sequence reconstruction discovery and sequence mapping refinement approach represents a promising platform for broader protein drug and gene therapy candidate optimization," said Dr. Doering, Professor of Pediatrics at Emory and part of the cell and gene therapy research team at the Aflac Cancer and Blood Disorders Center. His research laboratory focuses on the development of modified blood coagulation factors and their implementation in gene transfer-based therapies for hemophilia.

Prophylaxis Provides Safe, Effective Treatment for Patients With von Willebrand Disease

Robert F. Sidonio Jr., MD, MSc

A study investigating the efficacy and safety of a long-term prophylaxis in patients with von Willebrand disease (VWD), the most common inherited bleeding disorder, found that it reduces the length and severity of bleeding, lowers the risk of joint damage and improves quality of life.

Robert F. Sidonio Jr., MD, MSc, pediatric hematologist/oncologist and Medical Director of Hemophilia at the Aflac Cancer and Blood Disorders Center, led the phase 3, international WIL-31 study. It is the largest prospective study to date specifically investigating the prophylaxis called Wilate®, a plasma-derived factor concentrate with von Willebrand factor (VWF) and Factor VIII (FVIII), as an on-demand treatment for patients with VWD. The prophylaxis has previously been demonstrated to be effective in acute bleeding and surgeries.

Study findings, published in Blood Advances, revealed that Wilate was safe and well tolerated, and that it reduced bleed rates in more than 84% of patients.

"This trial suggests that the VWF prophylaxis should be the standard of care for VWD patients with a history of severe and frequent bleeds," said Dr. Sidonio, Associate Professor of Pediatrics at Emory.

HEART

Children's Conducts One of the First Pediatric Heart Studies Evaluating Blood Oxygen Levels and Race

A team of Children's cardiologists conducted one of the first research studies to determine whether race is a factor in blood oxygen level discrepancies in infants who have undergone heart surgery.

Alaa Aljiffry, MD

Children's pediatric cardiologists Alaa Aljiffry, MD, senior author, and Marcos Mills, MD, first author, led a study of 123 Black and white infants with single ventricle anatomy who underwent stage 1 palliation at Children's over a seven-year period. As published in JAMA Network Open, study results showed that the discrepancy between pulse oximetry and blood gas analysis was worse in the preoperative period compared to prior reports. Particularly for preoperative oxygen saturation levels below 75%, Black patients exhibited a greater discrepancy than white patients. However, this racial discrepancy was not significant postoperatively.

The authors hypothesized several reasons for the results, including changes in the proportion of fetal hemoglobin (lowered after cardiopulmonary bypass), lower oxygen saturation levels in the postoperative period or changes in overall cardiac output, which can affect device accuracy.

"The findings highlighted the need for additional studies investigating the efficacy of pulse oximetry and blood gas analysis devices in multiple phases of care, which should use multiple measured sample comparisons over time, a quantifiable skin tone scale and an outcomes-based analysis," said Dr. Aljiffry.

Genomics Play Key Role in Understanding Vein Graft Failure After Heart Bypass Surgery

Manoj Bhasin, PhD, MS

A research team from Children's and Emory, led by Manoj Bhasin, PhD, MS, played a key role in the first vascular field study to successfully use single-nuclei RNA sequencing and spatial transcriptomics to understand the causes of vein graft failure following pediatric cardiovascular bypass surgery, which is life-threatening.

By employing this novel approach to investigating vascular pathologies, Dr. Bhasin and team collaborated with Harvard Medical School to profile the genomic effects of vein grafts after harvest and distension, a process that uses high pressure to eliminate spasms and create space for grafting during surgery. They compared these findings to vein grafts obtained 24 hours after carotid-carotid vein bypass implantation, a surgical procedure that treats blockages in carotid arteries (the pair of large blood vessels in the neck that supply blood to the brain) by placing a graft between the two arteries to improve blood flow. Results, which were published in Circulation Research, suggested that distension initiates change in pathological pathways that may ultimately contribute to bypass graft failure.

"These findings help reveal early objectives for investigation of targeted therapies," said Dr. Bhasin, Director of Genomics, Proteomics, Bioinformatics and Systems Biology at the Aflac Cancer and Blood Disorders Center and Professor in the Department of Pediatrics and the Department of Biomedical Informatics at Emory.

Study Reveals the Potential of Myocardial Matrix Hydrogel in Treating Right Ventricle Heart Failure

Michael Davis, PhD, Director of the Emory and Children's Heart Research and Outcomes Center (HeRO) and Professor of Biomedical Engineering at Georgia Tech and Emory, was senior author of the first study to explore the use of an injectable biomaterial for treating right ventricular heart failure (RVHF) in infants.

The preclinical study, published in the Journal of the American College of Cardiology: Basic to Translational Science, showed that injecting a myocardial matrix (MM) hydrogel can mitigate damage to the right ventricle. While MM, a hydrated polymer material, and other injectable biomaterials have previously been evaluated for treating left ventricle heart failure (LVHF), this study revealed the potential of a pro-reparative injectable biomaterial in treating RVHF.

"Because of these findings, the U.S. Food and Drug Administration has approved Children's to conduct a clinical trial of the hydrogel in newborns with hypoplastic left heart syndrome, which is a factor in 40% of heart defect-related deaths in newborns," said Dr. Davis. The study authors note that the results suggest that the material should also be explored in phase 1 clinical trials for RVHF.

Michael Davis, PhD

Study Finds Link Between Gut Microbiome and Growth in Infants With Congenital Heart Disease

Michael P. Fundora, MD

A study published in the Journal of Pediatrics found that the state of the gut microbiome, an ecosystem in the intestines made up of micro-organisms including bacteria, viruses, fungi and parasites, may be a factor affecting the growth of children with congenital heart disease (CHD).

The study, conducted in the Cardiac Intensive Care Unit (CICU) at Children's and in newborn nurseries within Emory Healthcare, demonstrated that children with CHD develop more proinflammatory and pathogenic bacteria in the gut microbiome by the time they are discharged from the hospital than healthy infants. Further, the results revealed an association between a dysbiotic gut microbiome profile—when there is a disrupted balance of micro-organisms causing too many harmful bacteria or a lack of beneficial bacteria—and underweight infants. This could contribute to growth failure and related comorbidities, including delays in neurocognitive development.

"This study has identified the gut microbiome as a potential biological target for future interventions to reduce growth failure among children with CHD," said Michael P. Fundora, MD, pediatric cardiologist at Children's and Assistant Professor of Pediatrics at Emory, who led a team of 13 researchers in conducting the trial and authoring the study.

INFECTIOUS DISEASES

Research Study Reveals Unexpected Insights Into Primary Dengue Infections

Murali Krishna Kaja, PhD

Dengue is a rapidly spreading mosquito-borne viral infection that poses a significant threat worldwide, including in the U.S. Symptoms of the illness can range from a mild fever to severe dengue hemorrhagic fever or dengue shock syndrome, especially among children. The prevailing belief among the infectious diseases community that severe dengue primarily arises from secondary dengue infections is now being challenged.

Emory Department of Pediatrics researcher Murali Krishna Kaja, PhD, was senior author on a study examining data from more than 600 children with febrile dengue-confirmed infection at three leading hospitals in different regions of India. These hospitals have the highest incidence of dengue cases in the world.

The study results, which were published in Nature Medicine, showed that primary dengue infections accounted for more than half of total clinical cases (344 out of 619), severe cases (112 out of 202) and fatalities from dengue (5 out of 7), as compared to secondary dengue infections.

"These findings underscore the urgent need to recognize the rising risk of primary infections in populations not yet exposed as dengue viruses continue to expand globally," said Dr. Kaja, an Associate Professor in the Department of Pediatrics and the Emory Vaccine Center, and an investigator with the Center for AIDS Research. Dr. Kaja led multiple authors from Emory on the study.

ORTHOPEDICS

Outcomes of Surgical Treatment of Kneecap Instability Examined in Children's Orthopedic Study

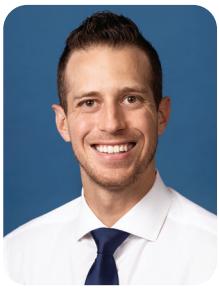
Kneecap, or patellar, instability is a common pediatric orthopedic condition. There are many surgical treatments available, and careful assessment of each individual patient's anatomy and risk factors is important for selection of the most successful surgical techniques.

To determine the outcomes of two specific types of patellar stabilization surgeries, sports medicine surgeons at Children's conducted a single-center study of 74 patients ages 12 to 19 years old who were treated with medial patellofemoral ligament (MPFL) reconstruction with or without tibial tubercle osteotomy (TTO). The results, published in the Journal of Pediatric Orthopaedics in February 2024, revealed that 95% of patients experienced successful surgical outcomes with no further instability episodes an average of three years following surgery. Among the 5% of patients who experienced recurrent patellar instability, there were no characteristics that predicted failure.

Among the researchers involved in the study were the four orthopedic sports medicine surgeons from Children's: Crystal A. Perkins, MD, first author and Medical Director of Orthopedic Quality and Outcomes; Anthony C. Egger, MD; Michael T. Busch, MD, Surgical Director of the Sports Medicine Program; and S. Clifton Willimon, MD, senior author and Medical Director of the Atlanta United Academy team.

"Based on our experience and clinical decision-making, excellent patient outcomes were obtained following both isolated MPFL reconstruction as well as TTO in addition to MPFL reconstruction for those with more severe instability," said Dr. Busch, who developed the TTO technique.

Crystal A. Perkins, MD



Anthony C. Egger, MD

Michael T. Busch, MD

S. Clifton Willimon, MD

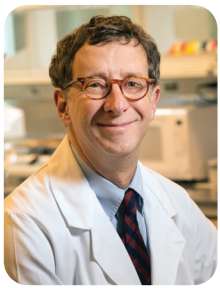
PULMONOLOGY

A Promising New Treatment Option for Rare Forms of Cystic Fibrosis

Individuals with rare genetic causes of cystic fibrosis (CF) may benefit from a new treatment option: a medication called elexacaftor-tezacaftor-ivacator, also known by the brand name Trikafta®. This finding is based in part on the results of a clinical trial conducted at Children's by pediatric pulmonologists and acclaimed CF researchers Rachel Linnemann, MD, and Eric Sorscher, MD.

Rachel Linnemann, MD

The multisite national trial found clinically meaningful improvements in lung function, respiratory symptom scores and nutritional outcomes among adolescents and adults with CF treated with Trikafta who have an N1303K CF transmembrane conductance regulator (CFTR) gene variant and a second minimal function variant. The medication is not currently approved by the FDA for this subset of patients, but this study may help amend eligibility requirements.



Eric Sorscher, MD

"We are excited to share this compelling clinical trial evidence," said Dr. Linnemann, who was co-first author of the study. "These data may help expand drug eligibility to include a new and important CF patient population."

The findings from the study, funded by the CF Foundation and NIH, were published in August 2024 in The Lancet Respiratory Medicine, a leading respiratory medicine and critical care journal. Dr. Linnemann also co-presented the results at the CF Foundation's Therapeutics Development Network during an April 2024 plenary session. Experiments to test leading-edge pluripotent stem cells as predictors of patient responsiveness are also being evaluated.

Dr. Linnemann serves as the Director of the Cystic Fibrosis Center at Children's. She is also the Hertz Professor of Cystic Fibrosis Care and Associate Professor of Pediatrics at Emory. Dr. Sorscher, senior author of the study, is a Georgia Research Alliance Eminent Scholar, the Hertz Endowed Professor in Cystic Fibrosis Research and Professor of Pediatrics at Emory.

GASTRO-ENTEROLOGY

Study Reveals Early aTNF Therapy May Slow Progression of Complicated Pediatric Crohn's Disease

Subra Kugathasan, MD

A nationwide, multicenter study led by Children's and Emory, and sponsored by the National Cancer Institute, revealed promising results for treating certain newly diagnosed patients with complicated pediatric Crohn's disease (CD), a type of inflammatory bowel disease (IBD) that causes inflammation in the digestive tract.

The study, published in Clinical Gastroenterology and Hepatology, found that patients undergoing antitumor necrosis factor (aTNF) therapy, a drug therapy that reduces inflammation by blocking the activity of an inflammation-causing protein called TNF, within the first three months of diagnosis may reduce the progression of stricturing. This occurs when the intestines narrow, leading to surgery or other complications. Study results also showed that a lack of body mass index z-score (BMIz) normalization within six months of diagnosis is associated with increased risk of surgery and inflammation.

"Because CD progresses from inflammatory to stricturing and penetrating disease behaviors in a subset of patients, we aimed to understand the risk of developing complicated disease behavior or undergoing surgery in relation to aTNF timing and BMIz normalization," said Children's pediatric gastroenterologist Subra Kugathasan, MD, senior author of the study. "We hope the results will shed light on the benefits of early aTNF therapy."

A specialist in caring for children and adolescents with IBD, Dr. Kugathasan also serves as the Marcus Professor of Pediatric Gastroenterology and Professor of Pediatrics and Human Genetics at Emory. His research focuses on further extending novel genetic discoveries, including common and rare susceptibility variants that are the cause of very early onset IBD.

Trainee Research Highlight

Early Peanut Introduction Is Improving Thanks to CDS Tool Designed for Pediatricians

A team of Children's allergists, informatics experts and pediatric residents developed and tested a clinical decision support (CDS) tool that is helping pediatricians better understand and discuss the benefits of early introduction of peanuts in infant diets.

Tricia Lee, MD

Evan Orenstein, MD

Annabelle Rowland, MD

A study published in the Journal of Allergy and Clinical Immunology showed that the use of the point-of-care CDS tool raised providers' awareness of early peanut introduction guidelines from 18% to 67%, with 79% saying they were comfortable using the tool. The CDS tool also increased the number of conversations occurring among providers and families about early peanut introduction, rising from 3% or less to as high as 84% at four-month, six-month and 12-month well-child checkups (WCCs). Importantly, those conversations were found to have a direct impact on caregiver decision-making, as 63% of families reported they had introduced peanuts without concerns by the time of the child's 12-month WCC.

While research has shown that early introduction and regular consumption of peanuts decreases the risk of developing a peanut allergy, guidelines regarding the practice have changed often since 2000. As a result, many pediatricians have not been conveying these benefits to parents during patient visits.

The study's senior author, Tricia Lee, MD, a Children's pediatric allergist and immunologist and Assistant Professor of Pediatrics at Emory, collaborated with Evan Orenstein, MD, Children's Chief Medical Informatics Officer and Assistant Professor of Pediatrics at Emory, as well as first author Annabelle Rowland, MD, General Pediatrics Resident at Children's and Emory, to establish clear recommendations for caregivers to follow.

"Early introduction of peanuts has been shown to reduce the likelihood of developing this allergy," said Dr. Rowland. "I was thrilled to be a part of implementing an effective tool to help facilitate these important conversations in our Hughes Spalding Primary Care Clinic."

Notable National Awards and Distinctions

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Subra Kugathasan, MD (Gastroenterology)
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