TOGETHER
we can give children a *brighter future*

Cook Children's
Research Report
2018

CookChildren's

Our Promise

Knowing that every child's life is sacred, it is the Promise of Cook Children's to improve the health of every child in our region through the prevention and treatment of illness, disease and injury.

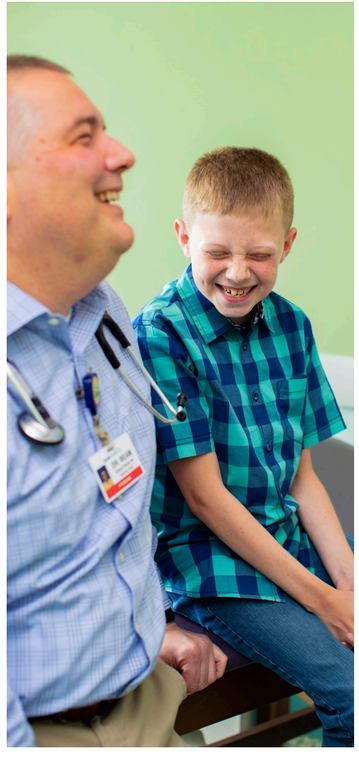
Knowing that every child's life is sacred

The first words of Cook Children's Promise resonate deeply with each and every person in our health care system. We know that when a family brings their child to us for treatment, we have to look beyond what we do today. We must remember that the help we give them now can make a lifetime impact.

That's why innovation and research are so important to us here at Cook Children's. Kids are more than just small adults. They have special needs because their bodies are constantly growing and they are affected by different diseases. The treatment for an adult illness may not work for kids, when you consider the possible side effects or secondary conditions later in life.

Our pediatric experts are naturally driven to study the problems of today and develop even better treatments for tomorrow. We believe that all children deserve the best, evidence-based treatment strategies available to manage their illnesses and to help them stay healthy. By supporting and encouraging research, Cook Children's contributes to the quality, effectiveness and safety of pediatric health care.

With our Endowed Chair program, our collaboration with national consortiums and our participation in investigator-initiated and sponsored clinical trials, Cook Children's is committed to expanding our national research initiatives, growing our reputation and bringing the world's leading-edge therapy home to North Texas.



Types of research

There are two types of research at Cook Children's: investigator-initiated research and externally sponsored research.

Investigator-initiated research is conceived and designed by a Cook Children's staff member and is conducted solely at Cook Children's. With these projects, the investigators are responsible for coordinating and facilitating the entire project life-cycle. This includes the research design, initiation and conduction of the study, as well as analysis and publication of the clinical research. These are most often smaller-scale studies aimed at evaluating drugs already on the market or providing open access to drugs in development that are not otherwise available.

Externally sponsored research is conceived, designed, initiated and managed by a non-Cook Children's investigator or company. Sponsors contract Cook Children's to recruit individuals to participate in their trials. These are most often large pharmaceutical trials or collaborative group trials studying drugs, procedures or medical devices in development for the purpose of bringing new therapies to the market. Also, clinical research involves the study of the biology of many diseases via tests on blood and tissue so that we can learn more about the specific disease and how best to treat our patients.

Creating a research culture

By Paul Thornton, M.D., Medical Director, Cook Children's Endocrinology and Diabetes program and Hyperinsulinism Center

Clinical research has become a large part of the care for patients with endocrinology and diabetes. Through clinical research, we can provide our patients access to medications that would otherwise not be available to them. There is an extensive range of medications (proven to progress care) available to treat Type 2 diabetes in adults, very few of which are approved for use by children. Through the support of research at Cook Children's, we can provide leading-edge therapy for pediatric patients.

By participating in research,

Cook Children's Congenital Hyperinsulinism Center has become one of the top centers in the world. For example, we hold an investigational new drug license to use 18F DOPA to take functional images of the pancreas that allow us to see where the insulin is being over-produced in tiny babies. As part of this research study, we scanned 50 babies from January 2014 through November 2018; 22 of which had lesions that were carefully and surgically removed, resulting in a cure for the patient. Prior to 2014, we would have had to remove 98% of the pancreas giving the children a 30% chance of having diabetes immediately after the surgery and a 95% chance of having diabetes by 15 years of age.

Now, as a result of our groundbreaking research, 21 of 22 patients have been cured and no longer are at risk of brain damage from hypoglycemia. In addition, our congenital hyperinsulinism team has partnered with pharmaceutical companies to develop new drugs for this rare disease (there is currently only one FDA-approved drug for treatment at this time).

Our group also participates in investigator-initiated clinical studies. Many of these can be carried out using existing resources available to us in the medical center. There is a constant need for funding of smaller research projects that provide our medical teams an opportunity to lead the way in providing better, more economical and safer medical care.

Empowering physicians to pursue their passions

Cook Children's Endowed Chair program

In 2013, Cook Children's implemented a special program to recognize the visionaries on our medical staff. The Endowed Chair program was established by Cook Children's Health Foundation with a $10 million endowment. This program recognizes the great work of our innovative physicians who are advancing pediatric care along with Cook Children's research reputation.

This program is our commitment to supporting research and teaching. Through their endowed chair, physicians develop programs, participate in clinical research, write for national publications, make presentations on a national level and further elevate the regional, national and global prominence of their work.

The following physicians have been given an endowed chair in support of their work in their program areas:

- **Paul Thornton, M.D.**
  Hyperinsulinism Center: Develop and manage the Hyperinsulinism Center, a subspecialty of Endocrinology and Diabetes Services, with a dedicated multidisciplinary team focused on the diagnosis and management of patients with hypoglycemic disorders caused by inappropriate insulin secretion.

- **Don Wilson, M.D.**
  Risk Evaluation to Achieve Cardiovascular Health (REACH) Clinic: Create and manage the pediatric REACH program, a subspecialty of Endocrinology and Diabetes Services, with a dedicated multidisciplinary team designed to identify, manage, treat and educate children at risk for developing cardiovascular disease.

- **Warren Marks, M.D.**
  Movement Disorders Clinic: Develop and manage the Cook Children's Center for Pediatric Neuromotor Disorders, which includes the Deep Brain Stimulation program, Motion Analysis Laboratory, Transcutaneous Magnetic Stimulation, Spinal Cord Rehabilitation program, Therapeutic Sports and Recreation program and Transitional lifespan "Bridge to Adulthood" program for children with complex neurological disorders.

- **Steve Muyskens, M.D.**
  3D aPPROaCH Lab - Heart Center: Continued advancement of the Cardiovascular MRI (cMR) for Congenital Heart Disease program at Cook Children's, including the development of the Adult Congenital cMR program and incorporating state-of-the-art imaging techniques including a 3D printing program.

- **Marcela Torres, M.D.**
  Pediatric Stroke and Thrombosis Program: The development of a Pediatric Stroke and Thrombosis Center with a highly specialized and trained multidisciplinary team able to provide the best medical care to children who suffer an acute stroke or suffer from chronic neurological deficits due to the stroke.

- **Artee Gandhi, M.D.**
  Center for Pain Management and Integrative Health: The development of the Center for Pain Management and Integrative Health focusing on expanding complementary medicine services, developing patient and family support groups to increase community awareness and providing a network for patient encouragement and inspiration, increasing community and staff education on the implications of untreated pain and the associated disability that ensues and creating a transitional care program for patients admitted to the rehabilitation unit.

- **Ariel Brautbar, M.D.**
  Personalized Genomic Medicine Program: Genomics is the study of the structure, function, evolution and mapping of DNA (which makes up our genes). Learning about a patient's genomics can help physicians to personalize medical care. This program aims to provide individual patient and provider education regarding drug therapy with regard to genomics. Additionally, a Clinical Decision Support (CDS) committee will be created to discuss and develop an approach to prescribe medications that require individualization according to a patient's genetics and disorder.

- **Scott Perry, M.D.**
  Comprehensive Epilepsy Program: A three-tiered program designed within the Comprehensive Epilepsy program of the Jane and John Justin Neurosciences program to expand the use of new and existing technologies in a minimally invasive epilepsy program, further local and collaborative research, continue the education of the next generation of neurologists to guide the program at Cook Children's and serve as a method to introduce Cook Children's to the broader neuroscience community and help foster continued collaborative relationships.

Santino's story

Before starting kindergarten, Santino (Tino) endured more than most people do in a lifetime. In July 2015, he was diagnosed with neuroblastoma just two weeks before his third birthday. Throughout his long treatment journey, Tino underwent a variety of treatments, including experimental I-131 metaiodobenzylguanidine (MIBG) therapy, a targeted therapy that delivers radiation directly to cancer cells.

There are only about a couple dozen facilities in the United Stares that offer MIBG therapy. Because Cook Children's has one of the four MIBG facilities in Texas, patients like Tino don't have to leave the state to receive the treatment they need.

"We had just moved to Fort Worth before his diagnosis, I truly believe we were brought here to be treated at Cook Children's. Tino is able to fight surrounded by our friends and family. We never once thought to look anywhere else for treatment. We love our oncology team deeply," said Sassy, Tino's mother.

Following his diagnosis, Tino received 16 rounds of chemotherapy, two MIBG infusions, 39 transfusions and two stem cell transplants. Altogether, Tino's treatment lasted more than two years. Currently, Tino has no evidence of disease in his body and has embarked on a new journey, kindergarten. Although the treatment Tino received at Cook Children's successfully removed the

cancer cells from his body, he has experienced latent effects from his treatment.

“Tino knows that he was sick with neuroblastoma and remembers being in the hospital for treatments. Right now, we just take it day by day. As he gets older, we will try to help him better understand why he has some of these side effects,” said Sassy.

Cook Children’s groundbreaking clinical research has led to higher cure rates and more children surviving neuroblastoma. But with that comes higher responsibility to look beyond just curative measures and to explore therapies that will give a child a greater quality of life after cancer. Tino and his family are thankful for the care they have received at Cook Children’s and look to the future with positivity.

“I feel hopeful because Tino has shown signs of improvement. He has always been a shy kid, but has now become Mr. Social, according to his kindergarten teacher,” said Sassy.

Moving forward, neuroblastoma research will help uncover better treatments for kids with minimized lasting effects. At Cook Children’s, our Promise continues beyond treatment of each patient’s initial disease.

Study sponsors for the last *five years*

Abbott Laboratories
AbbVie
Alexion Pharma International
Allergan
American Society of Parenteral and Enteral Nutrition
American Thrombosis and Hemostasis Network
Amgen Inc.
Anthera
Ascendis Pharma
Astellas Pharma Europe B.V. (APEB)
Baxter Healthcare Corporation
Bayer
Baylor College of Medicine
Baylor University Medical Center
Bioverativ Therapeutics Inc.
Bone Marrow Transplant Clinical Trials Network
Brigham and Women’s Hospital
Bristol-Myers Squibb
Cancer Prevention and Research Institute of Texas
Cancer Research Foundation of North Texas
Cangene Corporation
Celtaxsys
Center for Disease Control and Prevention
Center for International Blood and Marrow Transplant Research
Center for Medicare and Medicaid Innovation
Center for Oncology Education and Research
Cerexa, a subsidiary of Forest Laboratories Inc.
Child Health Corporation of America
Children’s Hospital of Atlanta
Children’s Hospital of Michigan
Children’s Hospital of Philadelphia
Children’s Mercy Hospitals & Clinics
Children’s of Alabama
Children’s Oncology Group
Chimerix
Cincinnati Children’s Hospital Medical Center
City of Hope
Cochlear Americas

Study sponsors for the last 5 years, continued

Collaborative Antiviral Study Group
Columbia University
Congenital Cardiovascular Interventional Study Consortium
Corbus
Cubist Pharmaceuticals
Cystic Fibrosis Foundation
Dana-Farber Cancer Institute/Harvard Cancer Center
Diamond Blackfan Anemia Foundation
DiaSorin Molecular
Discovery Labs
Duke University
Eisai Inc.
Eli Lily
Emory University School of Medicine
Endo Pharmaceuticals
Eunice Kennedy Shriver National Institute of Child Health and Human Development
Fern Kyba Dissertation Fellowship
FH Foundation
Fondazione Angelo Bianchi Bonomi
Genentech Pharmaceuticals
Gentium S.P.A.
Genzyme
Gilead Sciences Inc.
GlaxoSmithKline
Global Blood Therapeutics Inc.
Greehey Children's Cancer Research Institute at the University of Texas Health Science Center San Antonio
GW Research Ltd.
Helsinn Healthcare S.A.
Hemophilia & Thrombosis Research Society
Hospital for Sick Children
Ignyta Inc.
Improve Care Now
INO Therapeutics/Ikaria
Instituto Grifols S.A.
inVentiv Health
Syneos Health
Ipsen Biopharmaceuticals Inc.
Janssen Research & Development LLC
Jazz Pharmaceuticals Inc.
JMI Laboratories
Jubilant DraxImage Inc.
K2M Inc.
KAI Research Inc., an Altarum Company
Kedrion BioPharma Inc.
Luipold
Lundbeck Inc.
Mallinckrodt Inc.
MAST Therapeutics Inc.
Medical College of Wisconsin
MedImmune
Medical College of Wisconsin Blood Center
MEDNAX Center for Research, Education and Quality
Medtronic
Merck Sharp & Dohme Corp.
Mercy Children's Hospital
Meridian Bioscience
Millennium/Takeda
Miller Children's & Women's Hospital Long Beach
Miltenyi Biotec
National Association of Children's Hospitals and Related Institutions
National Cord Blood Program
National Institute of Health
National Institute of Occupational Safety and Health
National Marrow Donor Program
National Pediatric Cardiology Quality Improvement
New Approaches to Neuroblastoma Therapy Consortium
New York Medical College
NIH/National Cancer Institute
NIH/National Heart, Lung and Blood Institute
NIH/National Institute of Allergy and Infectious Diseases
Nivalis Therapeutics Inc.
Novartis Pharmaceutical
Novo Nordisk
OPKO Biologics Ltd.
Parexel International
Pediatric Epilepsy Research Consortium
Pediatrix
Pfizer
PPM Services SA
Prolacta Bioscience Inc.
ProPath Services LLP
Quintiles Inc.
Sage Therapeutics
Santhera Pharmaceuticals
Savara Inc.
Schering-Plough
Seattle Children's Hospital

Selexys Pharmaceuticals Corporation
Seton Healthcare
Severe Chronic Neutropenia International Registry
South Plains Oncology Consortium at Texas Tech University Health Sciences Center
St. Jude Children's Research Hospital
Sucampo AG
Synthes Spine
Takeda Development Center Americans Inc.
Texas A&M University
Texas Children's Cancer Center
Texas Children's Hospital
Texas Woman's University
Texas-Oklahoma Pediatric Neuro-Oncology Consortium
The National Institute for Neurological Disorders and Stroke (NINDS)
The University of Texas Health Science Center-Houston Medical School
Therapeutic Advances in Childhood Leukemia and Lymphoma (TACL)
Ultragenyx Pharmaceutical Inc.
United Therapeutics Corporation
University of California San Diego Medical Center
University of California San Francisco
University of Colorado
University of North Texas Health Science Center
University of Rochester Batten Center (URBC)
University of Texas Southwestern Medical Center

Upsher-Smith Laboratories Inc.
Vanderbilt University Medical Center
Vermont Oxford Network
Vertex Pharmaceuticals Incorporated
Washington University School of Medicine
Weill Cornell Medical College
Wills Eye Institute in Philadelphia
Wisconsin Children's Hospital
Xeris Pharmaceuticals
Zogenix International Limited

Consortiums

American Thrombosis and Hemostasis Network (ATHN)
Bone Marrow Transplant Clinical Trials Network (BMTCTN)
Center for International Bone Marrow Transplant Registry (CIBMTR)
Child Health Corporation of America
Children's Oncology Group (COG)
Clinical Trials Network (CTN) for Blood/Bone Marrow Transplantation
Congenital Cardiovascular Interventional Study Consortium (CCISC)
Cystic Fibrosis Foundation Therapeutic Development Network (TDN)
Hemophilia and Thrombosis Research Society
International Pediatric Stroke Society (IPSS)
Muscular Dystrophy Association Care Center
National Institute for Neurological Disorders and Stroke (NINDS)
National Institute of Health (NIH)
National Marrow Donor Program (NMDP)
National Pediatric Cardiology Quality Improvement Consortium (NPCQIC)
New Approaches to Neuroblastoma Therapy Consortium (NANT)
Pediatric Blood and Marrow Transplant Consortium (PBMTC)
Pediatric Epilepsy Research Consortium (PERC)

Severe Chronic Neutropenia International Registry (SCNIR)
South Plains Oncology Consortium (SPOC)
Southern Pediatric Neuro-Oncology Consortium (SOPNOC)
Therapeutic Advances in Childhood Leukemia & Lymphoma (TACL)

Collaborative efforts

Regional institutions

Children's Health Dallas/UT Southwestern (UTSW)
Dell Children's Medical Center of Central Texas
Medical City Dallas
University of North Texas Health Science Center (UNTHSC)
Texas Children's Hospital and Baylor College of Medicine
Texas Christian University
Texas Tech University-Lubbock, Amarillo, El Paso
MD Anderson Cancer Center
University of Texas at Arlington (UTA)
University of Oklahoma/Oklahoma Children's Medical Center

Collaborative projects

I. UT Southwestern (UTSW)

- Multiple clinical trials: Oncology, sickle cell disease, hemophilia.
- Regular referrals between institutions for open therapeutic trials.
- Regular teleconferences.
- Collaboration with UTSW genetic counselor for our cancer predisposition clinic.
- Joint educational efforts for comprehensive symposiums including sickle cell, bleeding disorders and cancer.
- Co-member of Southern Pediatric Neuro-Oncology consortium.

II. University of North Texas Health Science Center (UNTHSC)

- Basic lab and translational research efforts in leukemia, medulloblastoma, neuroblastoma and sarcomas, as well as late effects of cancer therapy, including cardiotoxicity.
- Co-development of an adolescent young adult (AYA) cancer registry database for North Texas regional medical institutions.
- Paul Bowman, M.D., Cook Children's oncology physician, is chair of pediatrics at UNTHSC.
- Medical education of third and fourth year medical student in the field of pediatric hematology/oncology.
- Mentorship for summer medical student clinical research projects (several subsequent presentations and publications in peer-reviewed journals).

III. Texas Children's Hospital (TCH)

- Participant/collaborations in multiple TCH trials-both clinical and biologic.
- Evaluating Utility and Improving Implementation of Genomic Sequencing for Pediatric Cancer Patients in the Diverse Population and Healthcare Settings of Texas: The KidsCanSeq Study.
- Co-submission of multiple Cancer Prevention and Research Institute of Texas research grants for clinical trials.
- Co-investigator on an R01 grant in patients with hemophilia.
- Reducing Ethnic Disparities in Acute Leukemia Consortium for tissue banking and cancer genomic studies.
- Invited participant in multitude of educational symposiums for both oncology and hematology topics.

IV. Texas pediatric/adult hospital consortiums

- Fort Worth AYA Consortium (first research project in development).
- Southern Pediatric Neuro-Oncology Consortium (SOPNOC): clinical trials, tumor tissue sharing, medical manuscript authorship.
- Cancer Prevention and Research Institute of Texas grant submission and awards.
- Oncofertility programs.

V. St. Jude Children's Research Hospital

- Invited participant for largest pediatric leukemia/lymphoma trial to date: Total Therapy XVII.

- Acute myeloid leukemia (AML) trials: AML 16, PANAML: A Phase I and Dose Expansion Cohort Study of Panobinostat in Combination with Fludarabine and Cytarabine in Pediatric Patients with Refractory or Relapsed Acute Myeloid Leukemia or Myelodysplastic Syndrome, SELHEM: Selinexor with Fludarabine and Cytarabine for Treatment of Refractory or Relapsed Leukemia or Myelodysplastic Syndrome.
- Relapsed acute lymphoblastic leukemia (ALL) trial: ALLR18: Therapy for Pediatric Relapsed or Refractory Precursor B-Cell Acute Lymphoblastic Leukemia and Lymphoma.
- Invited participant on large medulloblastoma clinical trial (third largest contributor of 20 institutions nationally).
- Submission of multiple tumor tissue samples for biomedical research and cancer genomic studies.

VI. MD Anderson Cancer Center

- Collaboration on numerous clinical trials.
- Cancer epidemiology research.

VII. University of Texas at Arlington/North Texas Genome Center

- New efforts for many potential biologic studies and therapy development for targeted "personalized" cancer therapy and immunotherapy.

VIII. Pediatric Eye Specialists and Texas Retina

- Collaborative clinic and research projects for retinoblastoma.

IX. Texas Department of Health and Human Services: Sickle Cell Advisory Committee

X. Other prominent national/international pediatric institutions

- Large contributor to our national/international clinical trials consortium: Children's Oncology Group (COG).
    - Annual large number of enrollments.
    - Meaghan Granger, M.D., and Kelly Vallance, M.D., with leading roles on disease specific committees and clinical trial principal investigators.
- Dana Farber Cancer Institute/Harvard University: Neuro-oncology research trial.
- New Approaches to Neuroblastoma Therapy (NANT) Consortium: Cook Children's is one of 13 institutions nationally participating in new approaches to neuroblastoma therapy.
    - One of the leading enrolling institutions.
    - Audit committee for NANT.
- Duke University: Co-submission of a large National Institute of Health grant proposal investigating childhood brain tumor metabolism.
- Invited as initial elite group of institutions for participation in a gene therapy study for individuals with severe hemophilia.
- Multiple national pharmaceutical trials for bleeding disorders, thrombophilia disorders, stem cell transplant, leukemia and sarcomas.
- Participant in international phase I/phase II therapeutic advances for childhood leukemia (TACL) clinical trials.

Areas of research

Anesthesia/pain

Pain control in pediatric patients

Audiology

Evaluation of assisted hearing devices

Cardiology

Antegrade pulmonary blood flow
Aortic translocation
Arrhythmia therapeutics
Cardiac catheterization procedures
Cardiac high acuity monitoring
Cardiac MRI and 3-D modeling/printing
Cardiac MRI diagnostics
Cardiac surgical site infection prophylaxis
Cardio-respiratory support with extracorporeal membrane oxygenation
Clinical experience with Ebstein's anomaly
Congenital heart defect or disease
Donation after cardiac death
Hypoplastic left heart syndrome
Vascular stents and valve replacement devices

Child Advocacy Resources and Evaluation (CARE) Team

Evaluation of drug-endangered children
Victims of childhood exploitation

The Center for Children's Health

Children with medical complexity

The Center for Prevention of Child Abuse and Neglect

Statewide child and fetal deaths

Child Life

Narrative language skills of chronically ill children
Parental cognition in chronically and acutely ill children

Child Study Center

Management for children with autism spectrum disorder and developmental coordination disorder

Emergency Medicine

Gun injuries in children

Endocrinology

Cardio-metabolic risk and weight management
Cholesterol disorders
Congenital hyperinsulinism diagnosis and treatment
Congenital hypothyroidism
Diabetes mellitus types I and II therapeutics, life quality and complications
Hypoglycemia
Hypophosphatasia (HPP) disorders
Lysosomal acid lipase deficiency
Nonalcoholic fatty liver disease in children
Short stature and growth hormone therapeutics
Vitamin D and insulin resistance

Gastroenterology

Acid-related diseases
Crohn's disease genetics and therapeutics

Functional constipation
Irritable bowel syndrome and therapeutics
Nutrition care
Parenteral nutrition-induced liver injury
Pediatric lymphocytic colitis
Ulcerative colitis and inflammatory bowel disease diagnosis and therapeutics

Genitourinary

Bladder exstrophy program
Intervention for caregivers of children with difference of sex development or congenital adrenal hyperplasia
Neurogenic detrusor overactivity therapeutic
Reproductive dysfunction
Urinary tract infection therapeutics

Hematology

Bleeding disorders surveillance
Chronic neutropenia registry
Congenital thrombocytopenia purpura therapeutics
Diamond–Blackfan anemia
Hemophilia genetics, diagnosis and therapeutics
Immune thrombocytopenia (ITP) therapeutics
Immune tolerance induction (ITI) therapeutics
Iron deficiency anemia therapeutics
Paroxysmal nocturnal hemoglobinuria (PNH) therapeutics
Pediatric stroke and thromboembolism diagnosis and therapeutics
Sickle cell anemia crisis therapeutics
Stem cell transplantation and complications
Thrombosis diagnosis and therapeutics

Infectious Disease

Acute flaccid myelitis
Acute hematogenous osteomyelitis therapeutics
Adenovirus therapeutics
Antibiotic utilization and resistance
Central venous catheter infection
Chronic hepatitis therapeutics
Clostridial infection therapeutics

Complicated skin and skin structure infections therapeutics
Congenital cytomegalovirus infection
Encephalitis diagnosis and therapeutics
Enteroviral sepsis syndrome therapeutics
Fungal infection therapeutics
HIV-infected children and youth
Influenza therapeutics
Staphylococcal infection therapeutics

Laboratory/ microbiology

Antibody resistance testing
Antimicrobial surveillance
Bordetella direct
Mycoplasma testing

Neonatology (NICU)

Developmental outcomes of prematurity
Evaluation of neonates with critical congenital heart disease
Exposure to isocyanate and medications of pre-term infants
Human milk fortifier and milk cream therapeutics
Hypothermia therapeutics
Mortality in neonates
Necrotizing enterocolitis
Neonatal encephalopathy therapeutics
Neonatal hypoxic-ischemic encephalopathy (HIE) therapeutics
Parental and staff stress
Parental decision making
Prevention of bronchopulmonary dysplasia (BPD)
Prevention of infection
Retinopathy of prematurity therapeutics
Surfactant therapy for respiratory distress syndrome

Nephrology

Atypical hemolytic uremic syndrome (aHUS) therapeutics
Pediatric dialysis

- Primary focal segmental glomerulosclerosis
- Rare disease
- Referral patterns for evaluation of hypertension

Neurosciences

- Complex movement disorders diagnosis and therapeutics
- Deep brain stimulation therapeutics
- Dravet syndrome therapeutics
- Duchenne muscular dystrophy diagnosis and therapeutics
- Epilepsy diagnosis and therapeutics
- Glucose transporter Type I deficiency syndrome therapeutics
- iMRI and advanced neuroimaging
- Laser ablation in epilepsy surgery
- Lennox-Gastaut syndrome therapeutics
- Migraine and headache therapeutics
- Motion analysis
- Neuropsychological outcomes
- Pompe disease therapeutics
- Quality of life
- Risk of stroke in cardiovascular procedures
- Severe spasticity therapeutics
- Spinal muscular atrophy therapeutics
- Tethered cord therapeutics
- Tuberous sclerosis complex therapeutics
- Whole exome sequencing in neurological diseases

Occupational Health

- Powered air purifying respirators in health care work settings

Oncology

- Adolescent and young adult oncology
- Brain tumor diagnosis and treatment
- Cancer in infancy
- Epidemiologic studies
- Genomic sequencing/molecular analysis
- Hematopoietic stem cell transplantation
- Hemophagocytic lymphohistiocytosis
- Hodgkin disease and non-Hodgkin lymphoma therapeutics
- Leukemia: New-onset, relapsed and refractory diagnosis and treatment
- Lymphoma: New-onset, relapsed and refractory diagnosis and treatment
- Melanoma therapeutics
- Molecular analysis for therapy choice
- Neuroblastoma: New-onset, relapsed and refractory diagnosis, treatment and genomics
- Pleuropulmonary blastoma
- Solid tumors: New-onset, relapsed and refractory diagnosis and treatment
- Survivorship, neurocognition, medication adherence and life after cancer
- Therapeutic leukapheresis
- Treatment-related toxicity

Ophthalmology

- Blepharitis therapeutics

Orthopedics

- Idiopathic slipped capital femoral epiphysis
- MRI utilization and surgery decision-making in pediatric musculoskeletal infections
- Scoliosis repair and mechanical devices
- Shoulder balance after thoracic fusion
- Spine disorders

Palliative care

- Palliative care in pediatric patients

Pediatric Intensive Care (PICU)

- Pediatric delirium

Pharmacy

- Cholestasis
- Uncomplicated community-acquired pneumonia

Primary care

- Juvenile neuronal ceroid lipofuscinosis (JUMP) therapeutics

Pulmonology

Bacterial pneumonia therapeutics
Cystic Fibrosis diagnosis, genetics and therapeutics
Enzyme replacement therapeutics
Nutritional diagnostics

Sleep study

Impact of parental smoking on sleep disorder diagnosis and outcomes

Surgery

Evaluation of hematuria in pediatric surgical patients
Management of post-tonsillectomy pain in obese and overweight children

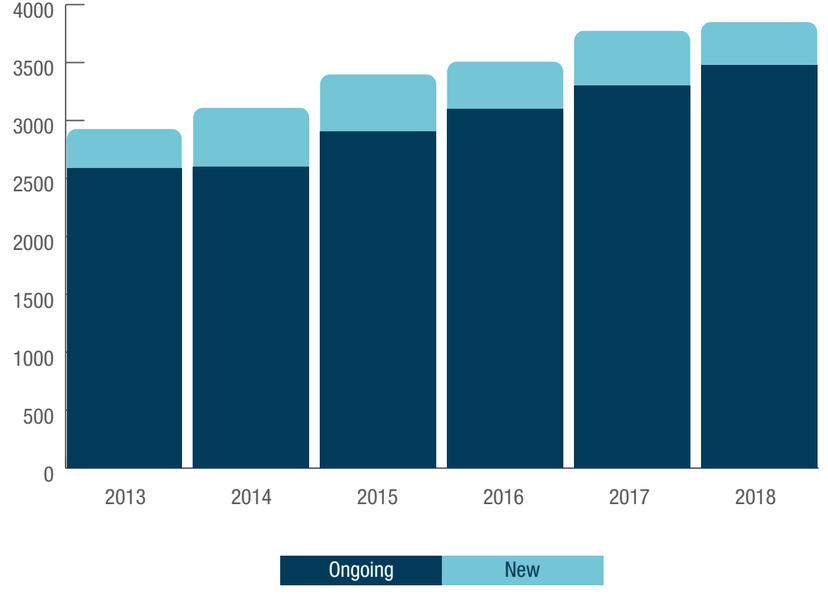
Trauma

CPR in pediatric trauma
Evaluation of need for trauma intervention
Evaluation of pediatric trauma team activation criteria
Pancreatic trauma
Pediatric submersion injury

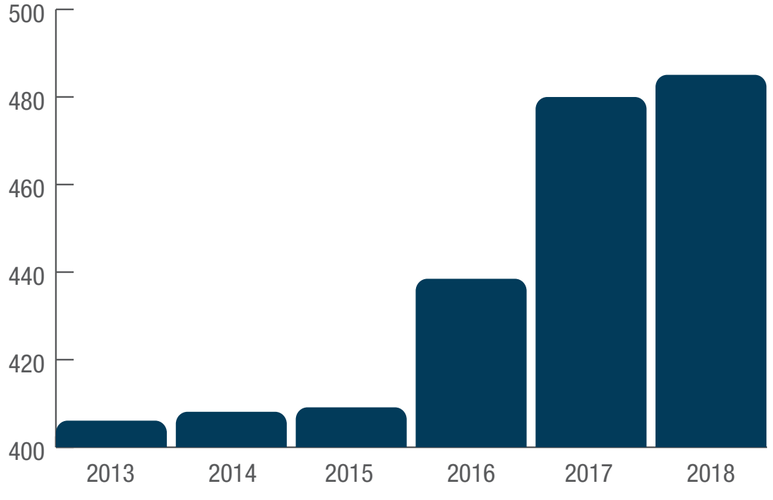
Urgent care

Mid-stream urine collection

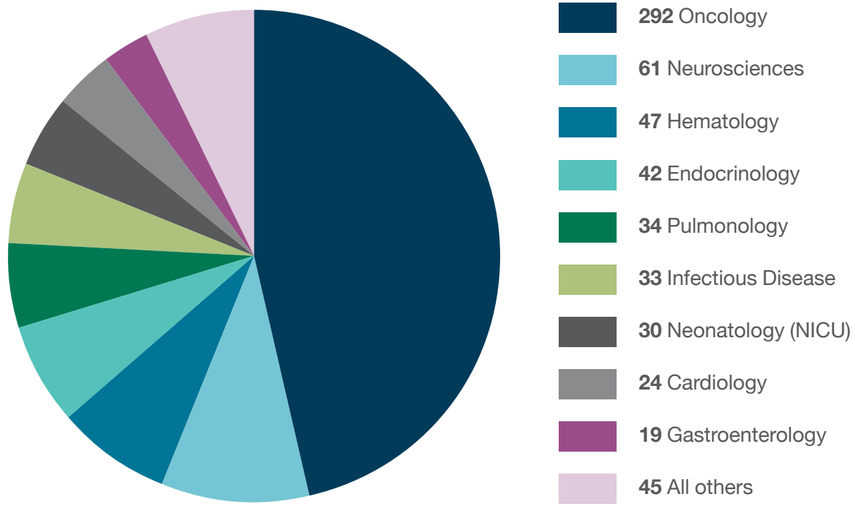
Active study participants



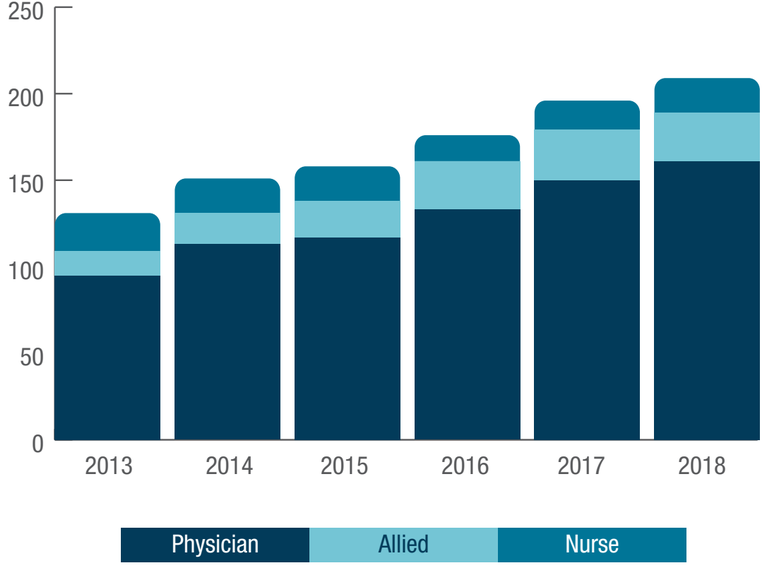
Active research studies



Total trials 2013-2018



Principal investigators



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