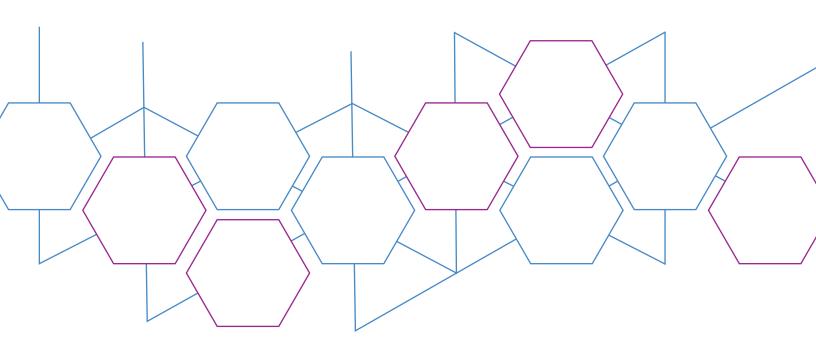


ABIGAIL WEXNER RESEARCH INSTITUTE

# DISCOVER

AN ECOSYSTEM OF INNOVATION, COLLABORATION AND EXCELLENCE IN PEDIATRIC RESEARCH





When your child needs a hospital, everything matters.



# NATIONWIDE CHILDREN'S FAST FACTS



More than 1.9 MILLION patient visits from all 50 STATES and **49 COUNTRIES** 



#### **AMERICA'S LARGEST**

neonatal network and provider of inpatient pediatric surgeries\*

\*Most recent data from CHA-member pediatric hospitals, based on highest number of NICU beds and inpatient surgeries



The Research Institute at Nationwide Children's Hospital

#### IS ONE OF THE TOP 10

NIH-funded freestanding pediatric research facilities in the U.S.









# RESEARCH FACTS

268 **PRINCIPAL INVESTIGATORS** 

1,956 **EMPLOYEES** 

93 POSTDOCTORAL RESEARCHERS FROM **12 COUNTRIES** 

**289K NET ASSIGNABLE SQUARE FOOTAGE AS REPORTED TO THE ASSOCIATION OF AMERICAN** MEDICAL COLLEGES

2024 **EXTERNAL AWARDS** 

FUNDING IN MILLIONS BY SOURCE

Program \$3.8 Industry \$12.2 Other \$24.3 Federal Other \$40.1 NIH Prime \$71.4



2024 \$152.0



### ABIGAIL WEXNER RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL

t Nationwide Children's Hospital, our commitment to research runs deep. We proudly acknowledge the significant role research has played in improving patient care and overall child health.

The Abigail Wexner Research Institute is a dynamic, collaborative, state-of-the-art environment for world-class research. Here, more than 200 scientists, 150 trainees and 1,900 employees work together toward one mission — to advance the field of pediatric medicine to improve outcomes for children everywhere.

We have been a top-10 National Institutes of Health-funded freestanding pediatric research institute for more than a decade and have doubled our NIH funding over the past five years. Additionally, our teams excel at translating discovery into commercially available solutions — from developing new therapeutics and devices to innovative tools for researchers and clinicians.

We are often asked what makes the AWRI and Nationwide Children's special. The short answer is simple: it's the people who have come together to conduct research for the betterment of children. The passion and dedication of our people, with institutional support that gives us the freedom to pursue excellence in a collaborative and innovative team science culture, make this place exceptional.

In the pages that follow, I invite you to learn more about research at the Abigail Wexner Research Institute and Nationwide Children's.

Dennis R. Durbin, MD, MSCE

President of the Abigail Wexner Research Institute at Nationwide Children's Hospital Professor and Vice-Chair for Research in the Department of Pediatrics at The Ohio State University College of Medicine

Abigail S. Wexner Endowed Chair in Pediatric Research

# ABIGAIL WEXNER RESEARCH INSTITUTE LEADERSHIP



**Dennis R. Durbin, MD, MSCE** *President* 



Joanne Turner, PhD Chief Scientific Officer



**Peter White, PhD**Chief Data Sciences Officer



**Cynthia A. Gerhardt, PhD** *Chief Clinical Research Officer* 



Amy J. Roscoe Vice President of Strategic Planning and Finance



Margaret Barkett, PhD Interim Director of Commercialization and Industry Relations

# **OUR CENTERS AND INSTITUTES**

he Abigail Wexner Research Institute is organized into centers of emphasis and institutes, all of which facilitate interdisciplinary team science by transcending traditional academic boundaries. The structure supports discovery and translation, enabled by outstanding shared resources. Each center or institute is home to talented faculty members, staff, graduate students, residents, postdoctoral fellows and other trainees.







### INTEGRATED CLINICAL CARE AND RESEARCH



Cynthia Gerhardt, PhD, Chief Clinical Research Officer

t Nationwide Children's the primary strategy for achieving best outcomes for children everywhere is investing in leading clinical and research programs. This is at the heart of Nationwide Children's strategic plan, with steps in place to achieve excellence in and integration of clinical care and research.

As an international leader in pediatric research, our scientists are transforming health in major ways. Clinical researchers at Nationwide Children's are committed to identifying new approaches for the prevention, diagnosis and treatment of childhood diseases.

"Our goal is to continue to build infrastructure that expedites clinical trials and catalyzes discoveries, so we can offer cutting-edge innovations and treatments to our patients faster," says Cynthia Gerhardt, PhD, chief clinical research officer at Nationwide Children's. "It's very important that the institution supports investigators by providing resources, training and funding for their trials under development."

More than 3,000 clinical research projects are underway at Nationwide Children's. These projects include single- and multi-site investigator initiated trials as well as national and international clinical studies. We are proud to be part of many consortia and collaborations designed to maximize increased research participation, especially for rare diseases, where the patient populations are small.

# **CLINICAL RESEARCH STRATEGIC INITIATIVES**



Increase Faculty and Staff Engagement



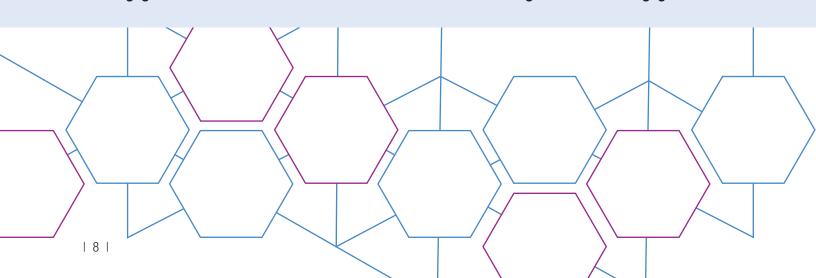
Expand Clinical Research Infrastructure



Align Education and Training



Enhance Community Engagement



# **OFFICE OF DATA SCIENCES**



Peter White, PhD, Chief Data Sciences Officer

t the Abigail Wexner Research Institute at Nationwide Children's Hospital, we are at the forefront of leveraging data to drive advancements in pediatric research, diagnostics, treatments and even disease prevention. As researchers, we generate a massive amount of data, holding immense potential to revolutionize health care. To fully harness this potential, we have taken the bold step of establishing the Office of Data Sciences (ODS).

The creation of the ODS marks a new chapter of excellence within our institute. This initiative is a testament to our strategic vision of unlocking the full capabilities of biomedical data sciences across our research endeavors. By integrating cloud computing, artificial intelligence (AI), machine learning (ML) and big data analytics, we aim to drive new discoveries and propel our research forward.

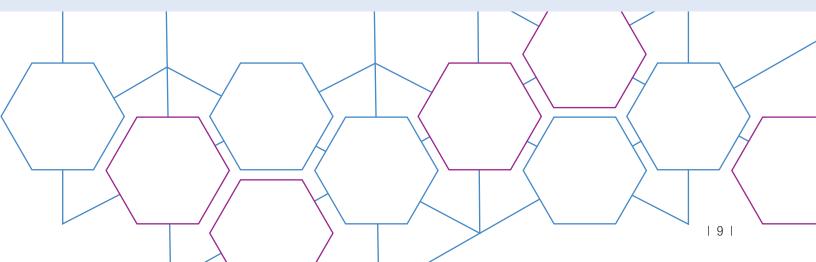
"As AWRI's inaugural chief data sciences officer, I am honored to lead this vital initiative," says Peter White, PhD. "Throughout my career at the Abigail Wexner Research Institute, I have had the privilege of empowering our research community with cutting-edge capabilities in genomics, data analysis and

interpretation. With the establishment of the ODS, we are poised to elevate these efforts, ensuring our research community has the tools and resources needed to excel."

The ODS will enable our research community to derive more insights from collected data while removing barriers to access. This fosters a culture of transparency and collaboration, ultimately enhancing our ability to conduct groundbreaking research. "In this endeavor, I am privileged to work closely with key stakeholders across our organization and our research centers of excellence, harnessing the full potential of biomedical data sciences and transformative AI to drive groundbreaking health research."

#### FOUR CRITICAL AREAS OF FOCUS

- DATA LAKE: Access to advanced data analytics and ML platforms via cloud computing technologies enables new research possibilities.
- DATA INTELLIGENCE: A strategic approach to Al application in research fosters a robust community of expertise.
- DATA TRANSLATION: Strategic translational initiatives accelerate the transformation of scientific data into tangible insights and applications.
- 4. DATA MASTERY: Education to cultivate data science expertise among groups presently underrepresented in the field grows the data science talent pool and makes knowledge more broadly accessible.



# INSTITUTE FOR MENTAL AND BEHAVIORAL HEALTH RESEARCH



Director: Eric Youngstrom, PhD

he Institute for Mental and Behavioral Health Research conducts innovative translational, clinical and epidemiological research focused on etiology, prevention and treatment of mental, emotional and behavioral disorders. In addition to collaborative efforts across the Nationwide Children's Hospital campus, institute members work in collaboration with The Ohio State University Department of Psychiatry and Behavioral Health and the OSU Institute for Behavioral Medicine Research. Embedded within the institute is the Center for Suicide Prevention and Research.

The Institute for Mental and Behavioral Health Research is located in the Big Lots Behavioral Health Pavilion, America's largest behavioral health facility on a pediatric medical campus.

# IN 2024, THE BIG LOTS BEHAVIORAL HEALTH SERVICE LINE HAD

274,763+ total outpatient behavioral health visits, providing care for

44,332+ unique patients



**56** Licensed Inpatient

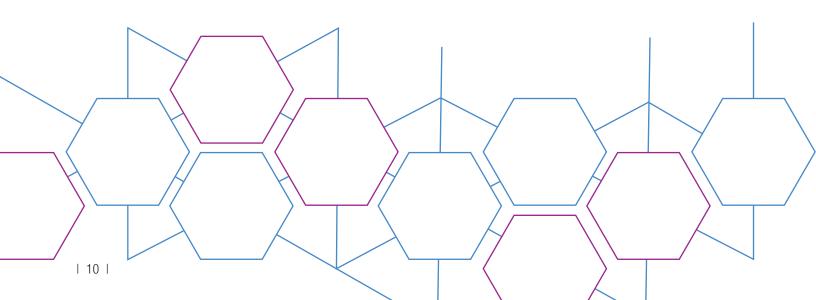
16 Staffed Youth Crisis Stabilization **Psych Beds Unit Beds** 



14,859

Inpatient Psychiatry and Youth Crisis Stabilization Unit **Patient Days** 





# STEVE AND CINDY RASMUSSEN INSTITUTE FOR GENOMIC MEDICINE

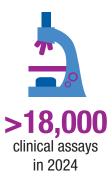


Executive Director: Richard Wilson, PhD



Co-executive Director: Elaine Mardis, PhD

he Steve and Cindy Rasmussen Institute for Genomic Medicine is an interdisciplinary institute that integrates the transforming power of genomics across research and clinical care at Nationwide Children's. The institute provides a unique environment that combines state-of-the-art testing within its clinical laboratory, advanced computational data analytics and basic science labs exploring a wide range of genetic mutations. These efforts partner clinical and genomics experts to fuel patient-centered research that explores the genetic and genomic underpinnings of diseases such as cancer, epilepsy, vascular malformations/ overgrowth syndromes, rare diseases and others. This process transforms patient care and makes genomic testing results accessible and meaningful for providers and the patients and families they serve.





2,036
Molecular Characterization
Initiative patients
in 2024

From 47 U.S. states, Washington D.C., Puerto Rico, Canada, Australia, New Zealand

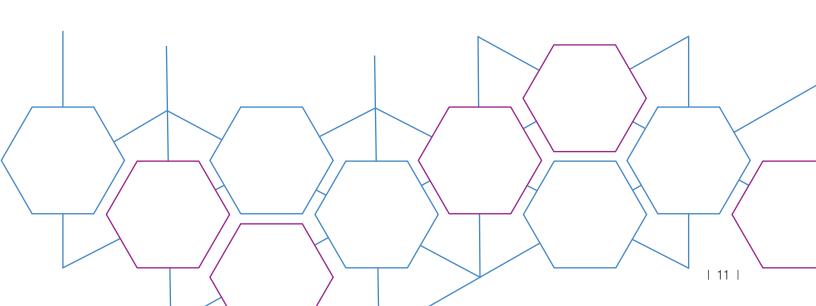


patients receiving
Rapid Genome Sequencing
in 2024 with an average
time of 3.5 days to provide
a preliminary clinical report



The Institute for Genomic Medicine currently has >300

faculty, staff and trainees



# BIOPATHOLOGY CENTER Director: Nilsa Ramirez, MD



The Biopathology Center (BPC) provides a full range of services related to biospecimen procurement, processing, banking and distribution to support clinical trials and translational research. The team manages complex biobanking efforts including the National Cancer Institute (NCI)-funded biorepositories of the Children's Oncology Group (COG) and SWOG; the NCI-funded Pediatric Division of the Cooperative Tissue Network; the Biospecimen Processing Center of the NCI's Office of Cancer Genomics; and numerous other sponsors.

The BPC also supports the NCI-funded Molecular Characterization Initiative for childhood cancer, overseeing biospecimen processing at the COG Biorepository and working in collaboration with the Institute for Genomic Medicine (IGM). Through this initiative, samples from more than 5,000 pediatric and young adult patients have been processed for genomic characterization by the IGM team, leading to improved diagnostic precision and targeted treatments for newly diagnosed patients.

#### **CENTER FOR BIOBEHAVIORAL HEALTH**

Director: Ahna Pai, PhD



The Center for Biobehavioral Health is a multidisciplinary group of scientists conducting cutting-edge research to advance our understanding of the interaction of biology and behavior and the impact on physical and mental health outcomes. Environmental, psychological, familial, societal and cultural factors are also examined for their influence on synergies between biology and behavior, health outcomes and potential targets of intervention. The breadth of biobehavioral research in the center spans from understanding human development across the lifespan to the development of lifestyle interventions for adolescents with congenital heart disease to digital health interventions for children with cancer.

#### CENTER FOR CARDIOVASCULAR RESEARCH

Director: Vidu Garg, MD

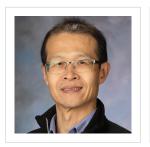


The Center for Cardiovascular Research is dedicated to discovering novel mechanisms underlying the development of pediatric cardiovascular diseases leading to innovative new therapies, improved clinical outcomes and preventive strategies promoting cardiovascular health in adults. The team's studies encompass basic science research, including the investigation of genetic, molecular and physiologic underpinnings of congenital and acquired heart diseases, as well as disease-focused translational and clinical research performed in collaboration with clinicians in The Heart Center at Nationwide Children's.

#### CENTER FOR CHILDHOOD CANCER RESEARCH

Interim Director: Ruoning Wang, PhD, through May 31, 2025

Director: Alexander Bishop, DPhil, June 2025





The center is dedicated to transforming the care of children, adolescents and young adults with cancer through cutting-edge research and clinical translation. It has experienced exceptional growth and success, driven by strong integration and collaboration among its laboratories, clinical divisions and other partners, including The Ohio State University Comprehensive Cancer Center – Arthur G. James Cancer Hospital and Richard J. Solove Research Institute.

Research in the center focuses on understanding biological mechanisms and developing therapeutic strategies for pediatric sarcomas, brain tumors and other malignancies. Investigators and trainees have consistently secured substantial extramural funding (including R01, U54, T32, ACS and DOD grants) and have published in top-tier journals. Their work has also advanced innovative therapies, successfully bringing oncolytic viral therapy and natural killer (NK) cell therapy into multiple first-in-human clinical trials.

#### CENTER FOR CHILD HEALTH EQUITY AND OUTCOMES RESEARCH

Director: Deena J. Chisolm, PhD



The center advances child health and well-being through research in and across clinical care, health services, health policy and community initiatives. Investigators in the center aspire to lead the nation in groundbreaking research that produces reduced disparities, vibrant communities, flourishing families and equitable and best outcomes for all children.

The center's annual Communities Aiming to Reach Equity Summit (CARES) brings together health care practitioners, researchers and community partners to highlight innovative research and practices focused on advancing health equity.

#### CENTER FOR CLINICAL AND TRANSLATIONAL RESEARCH

Director: William Smoyer, MD



The center creates an environment in which patients, families, researchers and clinicians work together to integrate evidence-based medicine with evidence-generating medicine. Such an approach ensures children benefit from the latest advances in both medical knowledge and innovative treatments to improve their health.

The center features scientists investigating several diverse areas of research, with faculty from three departments representing eight different subspecialties.

#### **JERRY R. MENDELL CENTER FOR GENE THERAPY**

Director: Kevin Flanigan, MD



The center is dedicated to developing novel genetically based therapies for inherited diseases. The mission is pursued by a passionate team of laboratory scientists working toward clinical applications and clinical scientists bringing these new therapies into clinical trials. The center works closely with regulatory experts to drive promising therapies from bench to bedside. Two of the first eight gene therapies approved by the FDA were developed in this center. We also now host an NIH/NICHD Wellstone P50-funded Muscular Dystrophy Specialized Research Center, which will further catalyze translational development.

#### **CENTER FOR INJURY RESEARCH AND POLICY**

Director: Gary Smith, MD, DrPH



The center works globally to reduce injury- and violence-related pediatric death and disability. The center translates innovative injury research into education, advocacy and advances in clinical care. From design changes in consumer products to new, evidence-based public policy, the center's work is advancing child safety at local to global levels. The center was a Centers for Disease Control and Prevention (CDC)-designated Injury Control Research Center (ICRC) from 2008-2024 and the only ICRC focused on pediatric injury and violence research and prevention during that time.

The center conducts national programs to mentor the next generation of injury researchers and advocates. It also supports regional and national coalitions of organizations and individuals working collaboratively to prevent injury, which is the leading cause of child death in the United States. Since its establishment in 1999 at Nationwide Children's Hospital, the Center for Injury Research and Policy has been leading the way to a safer world.

#### THE KIDNEY AND URINARY TRACT CENTER

Co-directors: Linda Baker, MD, and John David Spencer, MD Research Director: Michael Brian Becknell, MD, PhD







Investigators on the center study acute kidney injury, chronic kidney disease, urinary tract infections (UTI), congenital anomalies of the kidney and urinary tract (CAKUT), nephrotic syndrome and more. Clinician scientists and basic scientists aim to discover new therapies and diagnostic approaches,

validate them in preclinical models and translate these discoveries to the bedside to transform the management and prevention of these disorders.

The center fosters collaborative integration of Nationwide Children's nephrology and urology clinical care and research, as well as collaborations with experts at Nationwide Children's, OSU, nationally and abroad.

The center is also dedicated to training the next generation of scientists. The Student Urinary Tract Program in Education and Research (SUPER) is an NIH-funded opportunity for undergraduate, graduate and medical students to pursue mentored research and consider careers in pediatric nephrology and urology.

#### **CENTER FOR PERINATAL RESEARCH**

Director: Patrick Gallagher, MD



The center has a unique, integrative approach to research that extends from before birth to long after. In close collaboration with the Section of Neonatology at Nationwide Children's, other Abigail Wexner Research Institute centers and the Division of Maternal-Fetal Medicine of Ohio State, the Center for Perinatal Research conducts innovative basic, translational, epidemiologic and clinical research focused on the fetus and newborn. This center also houses the Ohio Perinatal Research Network, which accelerates clinical research throughout the perinatal division, creating and sustaining a platform for patient-centered research. The overall goal is to improve the outcome of the fetus, newborn and infant,

particularly those born prematurely and other newborns with health problems, by developing novel strategies to diagnose, treat and prevent disease.

#### **CENTER FOR REGENERATIVE MEDICINE**

Director: Christopher Breuer, MD

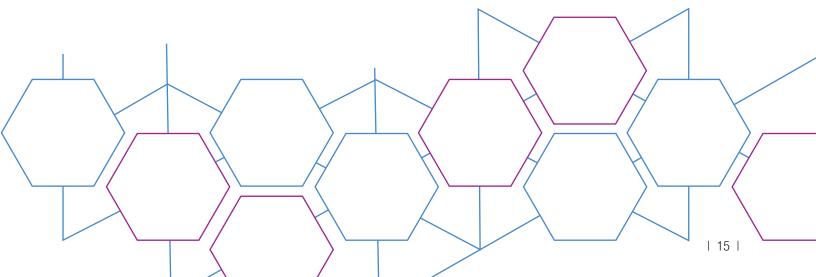


The center applies the principles of engineering, computational modeling and life sciences to develop biological substitutes to restore or improve tissue function, with a focus on cardiac and tracheal grafts. This center's ongoing development of a tissue-engineered vascular graft has received a Breakthrough Device designation from the FDA.

This team is continuing the second phase of the human tissue-engineered vascular graft clinical trial for patients undergoing a Fontan repair. The center has also fostered new partnerships with sponsors and labs to help develop the next generation of biomaterials that have the ability to regenerate body tissues.



Scan the QR code to learn more about Research at Nationwide Children's



#### **CENTER FOR SUICIDE PREVENTION AND RESEARCH**

Director: Jeff Bridge, PhD



Suicide has emerged as a leading cause of death for children and adolescents in the United States, but suicide is preventable. The center, which is housed within the Institute for Mental and Behavioral Health Research, conducts research aimed at understanding the epidemiology of child and youth suicide and suicidal behavior, examining risk and protective factors that contribute to youth suicide and suicide attempts and developing and implementing evidence-based intervention strategies for health care settings, schools, community centers and faith-based organizations to prevent and reduce suicide and suicidal behavior among youth.

Within the center is the Center for Accelerating Suicide Prevention in Real-World Settings (ASPIRES), supported by P50 Center grant funding from the NIH. ASPIRES aims to accelerate the development and implementation of effective interventions to reduce suicide in children and adolescents.

The signature project funded by ASPIRES is "Stepped Approach to Reducing Risk of Suicide in Primary Care" (STARRS-PC). Additional exploratory projects consider additional factors and approaches to understanding and preventing youth suicide.

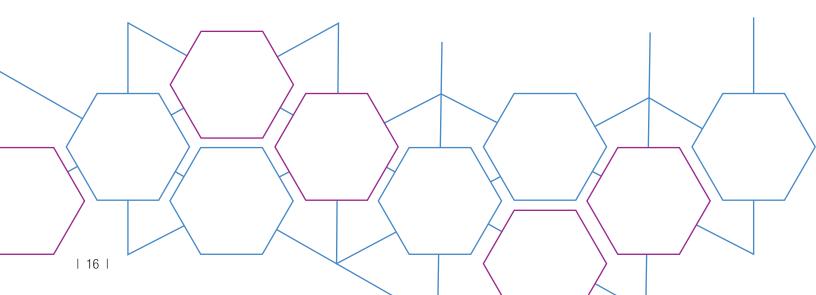
#### CENTER FOR MICROBE AND IMMUNITY RESEARCH

Interim Director: Joanne Turner, PhD

Interim Associate Directors: Amit Kapoor, PhD, and Jennifer Edwards, PhD



The Center for Microbe and Immunity Research (CMIR) is focused on fundamental and translational research on common and emerging microbial infections, and diseases of immune dysfunction. Investigators are highly collaborative and work with clinicians to pioneer multidisciplinary research on infectious diseases, and mechanisms to harness the immune system to prevent or treat infectious diseases that are of significant health concern. The center supports cutting-edge research aimed at characterizing microbial pathogenesis and defining protective or detrimental immune responses to infectious diseases and translating this knowledge to innovate and improve therapeutics and vaccines.



#### **EDUCATION AND TRAINING**

esearch and education are vital to the mission of Nationwide Children's Hospital. Housed in the Abigail Wexner Research Institute, the Office of Trainee Affairs supports and empowers trainees to embark on successful and satisfying careers in research.

The **Office of Trainee Affairs (OTA)** provides support to all trainees from the moment that they enter the doors of AWRI through onboarding assistance as well as providing targeted educational opportunities, career development and resources to fit all learners' needs throughout the varying stages of their academic and career development. The office's programs and services extend to K-12 learners, undergraduate and graduate students, postdoctoral and early career trainees, staff and faculty.

The Office of Trainee Affairs engages and supports trainees through:

- Community outreach activities and events
- Educational programming
- Individual career development planning
- Responsible Conduct of Research training
- · Scientific writing training and editing services
- Training grant support and guidance
- Accessible funding, fellowships and awards
- Wellness and collegiate events

The **Research Institute Trainee Association (RITA)** is similar to other postdoctoral associations at universities. It is modeled after guidelines established by the National Postdoctoral Association (of which AWRI is a member). RITA is a committee comprised of peer-elected students and trainees, organized to support and enhance the trainee experience while at AWRI. RITA offers an abundance of opportunities to help trainees succeed in their professional development, including the following:

- Trainee-driven career and professional development opportunities
- National Postdoctoral Appreciation Week events
- Family-friendly organization-wide events
- Community outreach programs
- Poster of the Month Program



Scan the QR code to learn more about the Office of Trainee Affairs



### RESEARCH AFFINITY GROUPS

ed by a tenured, NIH-funded investigator, Research Affinity Groups at Nationwide Children's stimulate collaboration and scientific interchange among basic science and clinical investigators relating to a particular disease. These groups work to increase the scope of research, enhance our core facilities and nurture a milieu in support of training the next generation of practitioners in a particular disease.

# Critical Illness and Injury Research Affinity Group Faculty Lead: Christopher P. Montgomery, MD Center for Microbe and Immunity Research

This group is focused on increasing the breadth and depth of research at Nationwide Children's centered on critical illness and injury. The group aims to facilitate collaboration among investigators at Nationwide Children's and OSU by cross-pollination of research spanning diverse methodologies, with the goal of bridging investigation from the cell to the community. Traditional seminars are complemented by a dedicated Advanced Research Competency series that supports the development of physician-scientists focused on critical illness and injury discovery.

# Fetal Research Affinity Group Faculty Lead: Adolfo Etchegaray, MD Fetal Medicine

This group brings together experts in fetal therapy, neonatology, genetics, molecular biology and other perinatal fields to develop and advance cutting-edge therapies that can be used to treat fetuses and neonates with a wide range of genetic and medical disorders.

# Nephrology and Urology Research Affinity Group Faculty Lead: Ashley R. Jackson, PhD Kidney and Urinary Tract Center

The primary mission of this affinity group is to enhance the health of children with diseases of the kidney and urinary tract by engaging in high-quality, cutting-edge translational research. This affinity group provides a strong foundation for multidisciplinary collaboration and supports trainee and junior faculty development.

# Single Cell Research Group for 'Omics Technology Faculty Co-leaders: Katherine Miller, PhD Institute for Genomic Medicine and Ryan Roberts, MD, PhD Hematology, Oncology & BMT

The Single Cell Research Group for 'Omics Technologies (SCRGOT) provides a collaborative platform that brings researchers together in the development and implementation of state-of-the-art single-cell technologies. Through educational meetings, team consultations, visiting lectures and an annual intensive training experience (the Coder Upgrade "boot camp"), the SCRGOT increases awareness, expands accessibility and grows capacity for single-cell analysis at Nationwide Children's Hospital, The Ohio State University and within the wider scientific community.

# Blood Disease Research Affinity Group Faculty Lead: Bryce Kerlin, MD Center for Clinical and Translational Research

This affinity group facilitates cohesion and synergies amongst investigators working on blood, vascular science and related disciplines, increasing awareness of blood disease research at Nationwide Children's and OSU.

"In order to have meaningful impact on child health challenges in the future, we need to bring teams of complementary experts together to collaborate effectively, and research affinity groups are one of the means by which we facilitate this."

— Dennis Durbin, MD, MSCE, president, Abigail Wexner Research Institute

#### RESEARCH AFFINITY GROUPS

# Primary Care Research Network Faculty Lead: Laura Hart, MD

The Primary Care Research Network (PCRN) is a consortium of pediatric primary care practices focused on transdisciplinary and collaborative research projects on common conditions and services in primary care. The PCRN consists of the 14 pediatric primary care practices that make up the Division of Primary Care Pediatrics, the 18 school-based health centers and two mobile care centers that Nationwide Children's operates, and collaborations with practices across the state of Ohio via Partners For Kids® (Nationwide Children's pediatric accountable care organization). The Network supports the conduct of both observational and interventional research within the clinics that make up the network with an aim to spread innovations to transform pediatric primary care.

# Pediatric Trauma and Burn Research Affinity Group Faculty Lead: Henry Xiang, MD, MPH, PhD, MBA Center for Injury Research and Policy

The mission of the Pediatric Trauma and Burn Research Affinity Group is to facilitate team science and innovative pilot studies by establishing multidisciplinary collaborations. This group is made up of researchers, clinicians and statisticians at Nationwide Children's and OSU. The major goals are to develop research projects, publish peer-reviewed papers and support pilot projects to collect preliminary data for extramural grant applications.

# Oral-GI Microbiology Research Affinity Group Faculty Lead: Michael Bailey, PhD Center for Microbe and Immunity Research

This group brings together like-minded clinicians and basic scientists actively involved in research in oral and gastrointestinal microbiology as it affects human disease, thus creating a university-wide network to leverage expertise and treat disease.

### Neurodevelopmental Research Affinity Group Faculty Lead: H. Gerry Taylor, PhD, ABOO/CN Center for Biobehavioral Health

The mission of the Neurodevelopmental Research Affinity Group is to facilitate research collaborations on neurodevelopment and neurodevelopmental disorders in children by increasing awareness of common research interests, creating new synergies in research methods and broadening the scope and outreach of research activities in the community and beyond.

# Lung Research Affinity Group

Faculty Lead: Mitchell Grayson, MD Allergy & Immunology, Center for Clinical and Translational Research

The Lung Research Affinity Group focuses on research that drives understanding of the mechanisms underlying lung disease broadly, with a special focus on those that involve inflammatory diseases of (or in) the lung.

# Molecular and Cellular Medicine Research Affinity Group

Faculty Lead: Mingtao Zhao, PhD, DVM Center for Cardiovascular Research

This group creates synergy among researchers and trainees with a common interest in elucidating disease mechanisms at the cellular and molecular levels. This group provides regular interdisciplinary scientific exchange opportunities among research teams who dissect molecular and cellular mechanisms of disease, particularly sharing knowledge on cutting-edge tools and approaches.

he Abigail Wexner Research Institute at Nationwide Children's has a wealth of resources ensuring researchers have the tools needed to advance the hospital's mission. From core facilities to computational resources and regulatory offices, researchers are well supported to enhance child health through high-quality, cutting-edge research according to the highest scientific and ethical standards.

The **Animal Resources Core** provides the highest quality animal husbandry, veterinary care and technical support in an ethical and compliant manner. Accredited by AAA-LAC International, the core is registered as a research facility under the Animal Welfare Act (AWA) with the U.S. Department of Agriculture (USDA) and has an assurance on file with the NIH Office of Laboratory Animal Welfare (OLAW). The ARC also provides study and technical support for in vivo imaging and behavior study needs through the Preclinical Behavior and Imaging Core (PBIC) which includes rodent models and now expanding to other large animal models. An ARC Wellness Committee and Safety Committee ensure the health of the ARC faculty, staff and animals.

The **Biobehavioral Outcomes Core** employs a team of psychometricians who conduct developmental, neuropsychological and behavioral assessments for studying infants, children, adolescents and adults with wide-ranging medical, developmental and psychiatric diagnoses. For investigators assessing behavioral,

cognitive, emotional or social outcomes and processes in their research, the core develops detailed procedural plans for each study to assist with research design, grant applications, scheduling participants, conducting assessments, qualitative data assessments and post-processing data and preparing manuscripts describing these data.

The **Biobehavioral Outcomes Core (BBOC)** assists research teams on the use of quantitative and qualitive methods to assess developmental, neuropsychological/cognitive, behavioral, affective and social processes relevant to a wide range of age groups (infancy to adulthood) and populations with varying medical, developmental and psychiatric diagnoses. Our directors consult with investigators to select assessment tools that are optimal for identified study aims for observational research as well as clinical trial development and implementation. Assessment plans can include standardized neuropsychological tests and performance-based measures, questionnaires, qualitative interviews and focus

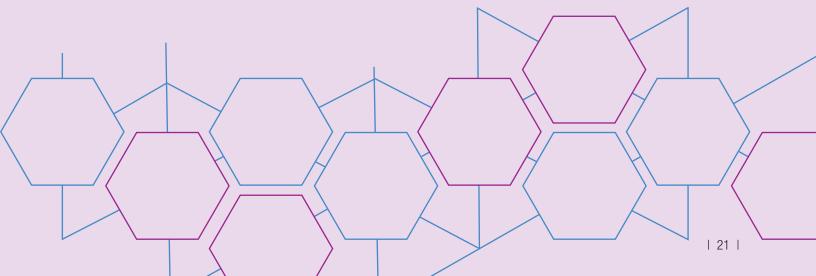


groups. The BBOC assists research teams with detailed protocols and procedure plans in collaboration with research coordinators or other study staff. Our team of psychometricians conduct standardized and semi-structured assessments at appointments scheduled through a shared online calendar. Plans include verified scoring of quantitative measures and collaborative coding of qualitative data with study teams. Finally, the BBOC can consult with study teams on broader questions regarding research design, grant applications and preparing manuscripts to disseminate results.

The function of Biologics Manufacturing Resource (BMR) is to provide safe and controlled manufacturing of cell-based products to Principal Investigators initiating early-stage clinical trials at Nationwide Children's Hospital and the Abigail Wexner Research Institute. The BMR serves as a mechanism for technology and manufacturing transfer from an investigators academic laboratory environment to a cleanroom environment following appropriate Current Good Manufacturing Practices (CGMP). A critical step in the manufacturing transfer is process development in a dedicated laboratory environment where the investigators initial cell manufacturing process is scaled up and optimized for the CGMP environment with the assistance of the BMR team. Following process development, cell-based therapies are manufactured for clinical trials in the BMR cleanroom suites. The Resource comprises more than 2,300 square feet of laboratory suites, including ISO 7 cleanrooms, a quality control laboratory, a process development laboratory and more. The BMR currently manufactures a wide range of cellular

products, including natural killer (NK) cells, chimeric antigen receptor (CAR) T and NK cells and tissue engineered vascular grafts for clinical trials. The BMR also manages the isolation of pancreatic islets of Langerhans for patients with chronic pancreatitis undergoing Total Pancreatectomy Islet AutoTransplantation (TPIAT) at Nationwide Children's. The facility manufactures irradiated CSTX002 feeder cells at clinical grade or research grade for the ex vivo expansion of NK cells.

The Biopathology Center's Histopathology, Processing and Banking Core supports basic science and clinical trial projects for investigators at Nationwide Children's and The Ohio State University by providing biospecimen processing, banking and logistical services. The core is accredited by the College of American Pathologists and technologists are IATA-certified for shipping biospecimens and dangerous goods. Histology services include human and animal tissue processing, embedding (paraffin), sectioning (formalin-fixed, paraffin-embedded (FFPE) tissue blocks and frozen optimal cutting temperature (OCT) compound-embedded tissues) and routine hematoxylin and eosin (H&E) staining. Selected special stains are also available. Additional handling, slide scraping and macrodissection (for nucleic acid extraction) may be requested. Other services include biospecimen processing (blood fractionation and aliquoting, solid tissue portioning), cohort management/ tissue banking (biospecimen collection kits, receiving, accessioning, storing and shipping of biospecimens), nucleic acid extractions and quality control, document redaction and digital pathology services.



The **Biostatistics Resource** at Nationwide Children's Hospital (BRANCH) is an extension of The Ohio State University's Center for Biostatistics that provides comprehensive collaboration and assistance for grant development, data analysis and interpretation of statistical results, producing publication-ready outcomes.

**Clinical Research Services** is the portal through which clinical investigators access streamlined coordination of services necessary to initiate clinical research projects, regardless of funding source. Clinical Research Services supports all types of clinical research studies from initiation to completion according to Good Clinical Practice and federal, state and institutional regulations and policies.

The **Computational Genomics Group** oversees multiple pipelines that analyze large and complex genomics data sets generated by investigators at the Rasmussen Institute for Genomic Medicine in collaboration with others across the organization and at OSU. The group continues to develop highly optimized solutions to

address the substantial processing, networking and big data challenges arising from genomic science.

The **CRISPR/Gene Editing Core** at Nationwide Children's Hospital provides investigators with design and implementation of CRISPR/gene editing services. Projects with the CRISPR/Gene Editing Core include brief studies that gather preliminary data for grant applications and large end-to-end projects such as genome screening or preclinical studies to bring novel uses of CRISPR/gene editing closer to the clinic. In addition, the core develops novel genome editing approaches for both preclinical and clinical applications.

The **Flow Cytometry Core** offers several platforms for phenotypic, functional and quantitative analyses of cells, cell-derived analytes and small particles. The core also offers data acquisition and analysis training and access to FlowJo analysis software.



The **Genomics Services Laboratory (GSL)** offers advanced sequencing technologies to provide high quality genomic analysis as a critical research resource. The GSL offers expertise in all aspects of next generation sequencing from project design, library preparation to sequencing and data analysis. The GSL provides sequencing services utilizing Illumina and PacBio technologies, and single cell and spatial genomics assays by 10X Genomics. The laboratory is housed within the Steve and Cindy Rasmussen Institute for Genomic Medicine, a nationally recognized provider of expertise in multiple aspects of genomics data generation and analysis.

The **Immune Monitoring Core** offers sample processing, assay development and multiple qualified platforms of immunological testing in support of pre-clinical studies and phase 1 clinical research trials in cell and gene therapy. These include monitoring for anti-vector and transgene responses to adeno-associated viral (AAV) vector gene therapy in human, small animal and large animal models.

The human **Induced Pluripotent Stem Cells (iPSC) Core** offers a variety of services to investigators at Nationwide Children's, The Ohio State University, throughout central Ohio and across the country. Some of these services include human iPSC line generation and cryopreservation, cardiomyocyte and endothelial cell differentiation, quality control and enrichment training. The core also provides access to iPSC lines from an established congenital heart disease biorepository at Nationwide Children's.

**IT Research & Innovation** collaborates with investigators to develop impactful and innovative health information technology (IT) solutions using data, analytics, technology and user experience designs. Services are offered in various areas such as research data management, data management

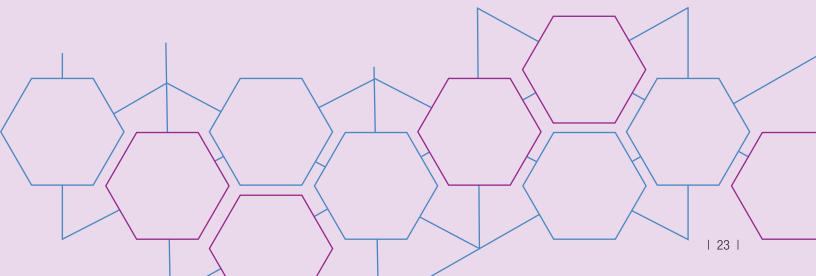
and sharing consultation machine learning, predictive modeling, high-performance computing, geographic information science, custom application development (web/mobile/virtual reality) and user experience (UX) study. IT Research & Innovation also operates Graphics Support Services, which assists with medical and non-medical illustration, video and animation, and more.

The **Microscopy Core** offers expertise, technical support and education in principles and best practices of high-quality image acquisition and rigorous and reproducible image analysis. Core staff provide consultations, instrument training and user assistance for a variety of high-end widefield and confocal microscopes and image analysis workstations. Support is available for all steps from experimental design to preparation of figures and manuscripts. Instrument training and user support for scanning electron microscopy (SEM) and atomic force microscopy (AFM) are also available.

#### OTHER RESOURCES

Nationwide Children's researchers have access to the **Ohio Supercomputing Center** located on The Ohio State University campus, less than 10 miles from Nationwide Children's, and its Ruby Cluster, Oakley Cluster and Owens Cluster systems and other resources.

A **commercial clinical manufacturing facility**, operated by Andelyn Biosciences, a for-profit spin-off from Nationwide Children's, produces materials for Phase 1, 2 and 3 gene therapy clinical trials and ensures the safety of manufactured biologic products according to U.S. Food and Drug Administration (FDA) cGMP guidelines.



# OPERATIONAL RESOURCES AND FACILITIES

The Institutional Animal Care and Use Committee (IACUC) reviews all research activities involving the use of animals and operates in full accordance with the Animal Welfare Act and the Health Research Extension Act. The animal research program at Nationwide Children's has been accredited by the Association for Assessment and Accreditation of Laboratory Animal Care International (AAALAC) since 1976, a distinction which indicates the highest standard of ethical and compliant animal research practices to ensure the humane treatment of animal subjects.

The **Institutional Biosafety Committee (IBC)** is responsible for evaluating and approving all research involving infectious or potentially infectious agents, recombinant DNA or RNA, and biological toxins. The committee ensures compliance with current safety regulations and guidelines as issued by the U.S. Departments of Health and Human Services and Agriculture, the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC).

The **Institutional Review Board (IRB)** provides oversight of all research involving human subjects. For more than a decade, Nationwide Children's has been accredited by the Association for the Accreditation of Human Research Protection Programs, Inc. (AAHRPP). This accreditation highlights clinical study participant safeguards that surpass state and federal requirements.

The **Office of Finance and Sponsored Projects** supports grant and contract funding throughout the entire lifecycle of an award, from application to closeout. The department offers expertise in review, submission and negotiation of extramural grants, contracts, and other sponsored programs.

The **Office of Research Compliance and Integrity** ensures all research is conducted according to the highest scientific and ethical standards and in compliance with regulations from the NIH, U.S. Food and Drug Administration (FDA) and all other government agencies with authority over research activities.

The **Office of Research Regulatory Affairs** helps move discoveries at the lab bench into clinical testing to develop novel approaches to prevent and treat pediatric diseases. The FDA closely regulates human participation in clinical trials involving new drugs and devices and the Office of Research Regulatory Affairs guides investigators through this complex submission, review and approval process.

#### And Many More...

AWRI offers institutional support covering the entire spectrum of research. From teams dedicated to helping manage export controls, clinical monitoring, purchasing, glassware, intramural funding, high-performance computing and more, our researchers are enabled to achieve their goals.



#### HIGHLIGHTED PARTNERSHIPS

#### THE OHIO STATE UNIVERSITY

#### **Clinical and Translational Science Institute (CTSI)**

The Ohio State University, Wexner Medical Center and Nationwide Children's have come together to form the OSU Clinical and Translational Science Institute. Nationwide Children's receives funding annually in support of CTSI activities, and faculty and trainees are eligible to compete for pilot and training grants through the CTSI.

#### **Ohio Perinatal Research Network (OPRN)**

Nationwide Children's and OSU have developed the OPRN, a research collaborative dedicated to better understanding preterm birth and its complications. A major effort of OPRN is a research repository for clinical data and specimens from infants delivered preterm and their mothers. Additional collaborating institutions include OhioHealth, Mt. Carmel and community partners in the Ohio Better Birth Outcomes (OBBO) project aimed at mothers and physicians to prevent preterm births.

# OSU Comprehensive Cancer Center – Arthur G. James Cancer Hospital and Richard J. Solove Research Institute (OSUCCC – James)

A collaborative agreement between Nationwide Children's and OSUCCC – James broadens the study of childhood cancers and accelerates the transfer of key research findings to the clinical setting. The unique relationship among the institutions is stimulating novel research and developing collaborations in pediatric cancer-related studies that will also have applications to adults. Among the innovative outcomes of this collaboration are the Cellular Therapy and Cancer Immunology Program and a proton therapy center.

#### **OTHER**

#### **Additional Ventures**

AWRI is the recipient of a \$1 million Innovation Fund, gifted by nonprofit foundation Additional Ventures. AWRI joins four other research institutions in a large-scale coordinated research effort to identify new avenues to functionally cure patients with single ventricle heart defects (SVDs). Investigators from the Center for Regenerative Medicine are members of the Additional Ventures Cures Consortium, a \$10 million multi-institutional project focused on developing a cure for single ventricle disease using a regenerative medicine approach.

#### **Nationwide Pediatric Innovation Fund**

Established in 2014 by the Nationwide Foundation, the Nationwide Pediatric Innovation Fund supports pediatric research and innovative projects to advance the science and practice of pediatric health care. Innovation Fund investments are directed long-term to both clinical and research program development and recruitment in areas of greatest priority.

#### **PEDSNet**

PEDSNet is a large, national community of hospitals and health care organizations, researchers and clinicians, and patients and families. Nationwide Children's is a founding member of this multi-specialty network that conducts observational research and clinical trials across multiple children's hospital health systems.

#### **REV1 Ventures**

Nationwide Children's is a sustaining member of Rev1 Ventures, an organization created to accelerate innovation, business growth, job creation and prosperity in the 15-county region of central Ohio. Rev1Ventures works to create new companies, strengthen existing businesses, open doors to technology resources and support the attraction and retention of technology-based businesses.

#### And Many More...

Nationwide Children's also partners with Battelle Memorial Institute and many other organizations. Researchers at AWRI collaborate with a variety of institutions, present at national meetings, publishing in highly respected journals and earn national research grants to further their fields of study.

# TECHNOLOGY COMMERCIALIZATION: GUIDING IDEAS TO SOLUTIONS

he Nationwide Children's Office of Technology Commercialization offers expertise in technology transfer, facilitating partnerships between inventors and industry to bring discoveries to patients, clinicians and scientists around the world. The resourceful team guides inventors and partners through complex processes with creative solutions. The Office of Technology Commercialization facilitates the partnering of innovative technology and translational infrastructure with industry to benefit patient care, the community and the general public.



Follow the Office of Technology Commercialization at Nationwide Children's Hospital on LinkedIn.



Explore our startups and more!

"We are committed to making the necessary investments in advancing commercialization. Through Nationwide Children's efforts, we are thrilled to see the number of startups based on our technologies grow each year, expanding on our diverse portfolio of innovations in gene and cell therapy, therapeutics, medical devices and digital health. Our innovations are leading the way to benefit the lives and care of children everywhere."

- Margaret Barkett, PhD, Interim Director, Director of Licensing, Office of Technology Commercialization

#### TYPES OF INNOVATIONS



**Biomarkers:** Detect and measure normal biological processes, pathogenic processes or responses to interventions.



**End User Innovations:** Improve clinical or business practices and patient outcomes with improved/ novel medical devices and software.



**Gene Therapies:** Deliver new genetic material to replace impaired or harmful genetic material to treat various conditions.



**Therapeutics:** Prevent or treat diseases.

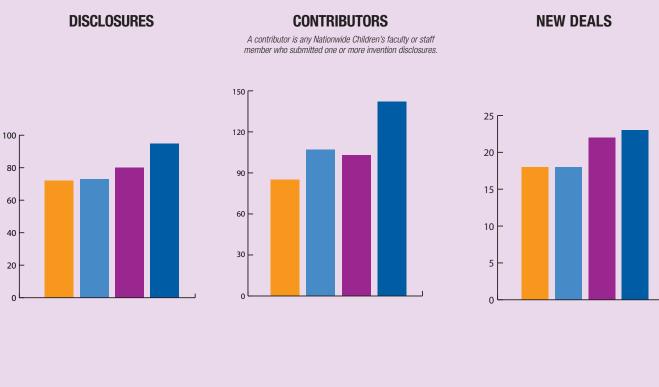


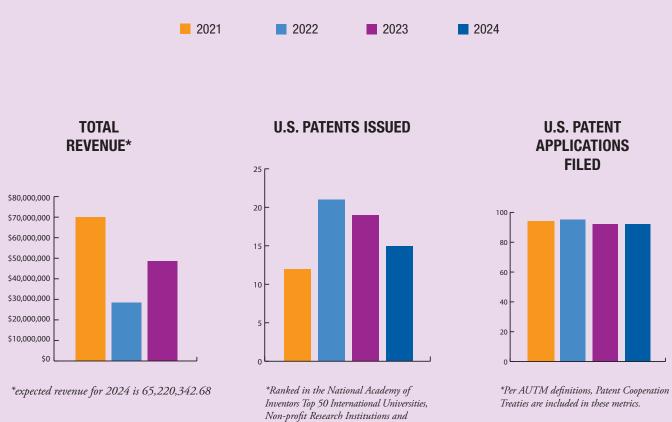
Tissue Engineering Innovations: Combine scaffolding, seeded cells and biologically active molecules into functional tissues, allowing a patient's own cells to be used to grow new tissue or organs for repair.



**Research and Clinical Tools:** Enhance therapeutic or research activities.

# **TECHNOLOGY COMMERCIALIZATION BY THE NUMBERS**





Government Agencies list in 2023.

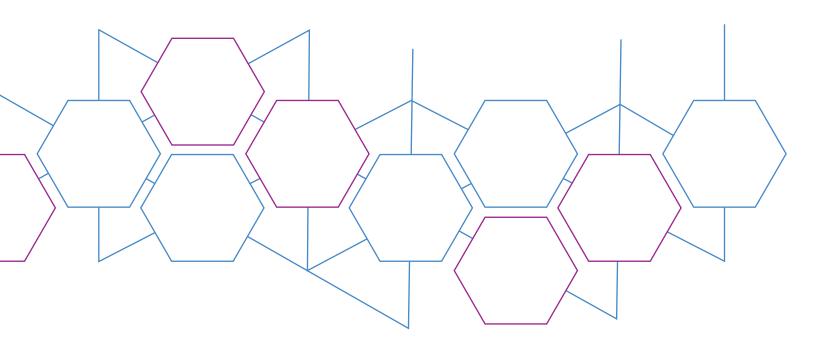
# ABIGAIL WEXNER RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL

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When your child needs a hospital, everything matters.