

### Mitochondrial Medicine Program

Our program provides multidisciplinary clinical care; advanced diagnostics and therapies; and individualized basic, translational, and clinical research programs to improve the health of patients of all ages who are living with mitochondrial disease. Our team focuses on finding the underlying cause of each patient's condition and collaborating with referring physicians and other specialists to develop the best course of treatment.

### Newborn and Infant Chronic Lung Disease Program

Our experienced team includes doctors, nurses and support staff who are experts in caring for infants with chronic lung disease (CLD). We offer the newest diagnostic approaches and the most innovative treatments, and we create a customized care plan for each child. We are also leading research into new treatment options that will improve outcomes for all children with CLD. Our multidisciplinary team works with specialists across the hospital to provide exceptional medical care for each patient — and psychosocial support for the entire family.

### Pediatric Thyroid Center

There is an increased incidence of thyroid cancer, thyroid nodules and hyperthyroidism in the pediatric population, and each diagnosis presents and is treated differently in children as compared to adults. Currently, the Pediatric Thyroid Center at CHOP is recognized as the leading center for the treatment of pediatric thyroid disorders in the country, and is the only center in the country with a supporting translational research program. Our thyroidectomy surgery outcomes are unparalleled, with astonishingly low complication rates. We will build on this reputation by defining the molecular landscape of pediatric thyroid cancer, confirming targets of diagnosis, determining markers of invasive behavior, and building a global thyroid community focused on advocacy and education.

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### Sickle Cell and Red Cell Disorders Curative Therapy Center (CuRED)

Sickle cell disease and beta thalassemia are devastating blood disorders that result in anemia, severe chronic pain and premature death. Currently, treatment is largely limited to supportive care. Our program aims to change the care pathways for red blood cell disorders. Building on CHOP's position as a global leader in cell and gene therapies, we will develop novel and curative gene therapies and stem cell transplants and deliver them to patients.

### Wyss/Campbell Center for Thoracic Insufficiency Syndrome

Thoracic insufficiency syndrome (TIS) is a complex condition that involves chest wall deformities that affect normal breathing and lung growth. The Wyss/Campbell Center for Thoracic Insufficiency Syndrome provides the most advanced medical and surgical care for children with TIS, including a multistage surgical procedure for rib cage deformities using the vertical expandable prosthetic titanium rib (VEPTR). Our team performs more than 200 VEPTR and other complex procedures each year, utilizing input from a multidisciplinary team devoted to the care of these complex patients. We offer an array of different surgical options tailored specifically to each child's particular deformity.



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# FRONTIER PROGRAMS

## BREAKTHROUGHS IN CARE

The future of pediatric medicine is here, now. At Children's Hospital of Philadelphia (CHOP), a trailblazing group of initiatives — known as Frontier Programs — are pioneering new advances in children's health at an astonishing pace.

Frontier Programs conduct visionary research that translates to cutting-edge clinical care. They offer answers often not available anywhere else in the world: a breakthrough therapy that teaches a child's own cells to fight cancer, a first-of-its-kind surgery for chest deformities, a lifesaving treatment based on a new understanding of the body's lymphatic system, and more. CHOP champions these programs and ensures that they receive critical support to accelerate their progress.

Frontier Programs were launched in 2016 with three selections. Additional programs have been added each year, and currently there are 18.

Discovery and innovation have been at the very core of CHOP's mission for more than 150 years. Today, by investing in Frontier Programs that bring the best minds to the most challenging conditions, CHOP is once again redefining what's possible.

## OUR FRONTIER PROGRAMS

### Biomedical Optical Devices to Monitor Cerebral Health

Maintaining oxygen levels in the brain during critical illness and events such as cardiac arrest is essential for subsequent quality of life upon recovery. However, currently there are no methods available to monitor brain oxygen to guide treatment and care in these circumstances. Our program seeks to develop a first-of-its-kind noninvasive optical device that monitors brain oxygen levels. Incorporating this device into lifesaving medical equipment will ensure that patients receive treatment to maximize their brain oxygen levels, thus maximizing their quality of life upon recovery.

### Cancer Immunotherapy Program

CHOP doctors and researchers helped develop the first national and international clinical trials for an immunotherapy known as chimeric antigen receptor ("CAR") T-cell therapy for children with advanced acute lymphoblastic leukemia (ALL). CAR T-cell therapy for B-cell ALL was the first-ever cell therapy to receive approval from the U.S. Food and Drug Administration. Our doctors have treated more than 375 children with relapsed or refractory ALL with the therapy and reported a complete remission rate of 90% on the pilot clinical trial that opened in 2012.



Center for Pediatric Airway Disorders

Our center is one of the largest multidisciplinary programs of its kind, treating neonates and children with congenital and acquired airway malformations or disorders. With a coordinated and comprehensive evaluation, our world-renowned experts provide innovative treatments as well as complex surgeries such as slide tracheoplasty, laryngotracheal reconstruction, laryngeal cleft repair and recurrent laryngeal nerve reinnervation. Leading the field in translational research regarding pediatric laryngology, the center established Bio2, a biomaterial and bioengineering laboratory to continue the pursuit of advancing the care of children with airway issues.

Center for Pediatric Heart Valve Disorders

Congenital heart defects are the most common birth defect globally; however, there is still no therapy to mitigate valve regurgitation in newborns and infants, and no valve replacement option that would eliminate the need for a redundant surgery as the patient ages. Our center intends to answer these unmet needs by providing coordinated care for pediatric valve patients, delivering novel imaging platforms to refine patient-specific modeling, offering personalized valve therapies to treat the individual patient’s needs, and defining therapeutic drug targets to mitigate the progression of the disease.

Center for Pediatric Inflammatory Bowel Disease

One of the largest centers of its kind in the United States, the Center for Pediatric Inflammatory Bowel Disease (IBD) treats about 2,000 children each year. Children come to our center from around the world to receive the latest treatments and to participate in clinical research studies. Our team of physicians, psychologists, social workers, nutritionists, nurses, educators, pediatric surgeons, laboratory technicians and clinical researchers supports them along the way.

Comprehensive Vascular Anomalies Program

Vascular anomalies — which occur when arteries, capillaries, veins or lymphatic vessels fail to develop correctly — can range from mild to very complex. Persons with complex vascular anomalies can suffer from debilitating complications due to disrupted blood and lymphatic flow, physical pressure on surrounding structures, enlargement of tissues and bones, and changes to normal metabolic processes. Our program expands CHOP’s expertise and our international reach in the care of those with complex vascular anomalies using an integrated clinical team with true multidisciplinary care. This team incorporates state-of-the-art genomics and personalized research strategies to characterize the causes of these conditions and identify targeted and potentially curative therapies.

Congenital Diaphragmatic Hernia Program

Congenital diaphragmatic hernia (CDH) is a structural birth defect in which the diaphragm fails to close. This allows contents from the abdomen to move into the chest, resulting in pulmonary hypoplasia or underdeveloped lungs, which can lead to life-threatening complications. Leveraging CHOP’s standing as the largest CDH treatment program in the country, our program aims to become the international epicenter for the care of CDH and to eliminate mortality and morbidity through new research and the development of novel devices.



Congenital Hyperinsulinism Center

Hyperinsulinism (HI) is a genetic condition in which insulin regulation fails, causing dangerously low blood glucose levels that can result in brain damage. Building on CHOP’s status and experience as the world’s leading HI center, our program seeks to further innovate by using personalized medicine to treat children with the condition. We will work to build a multidimensional patient data collection platform, develop more accurate diagnostic tools for insulinomas, and accelerate late-stage clinical trials for a promising CHOP-patented compound that ameliorates HI.

Epilepsy NeuroGenetics Initiative (ENGIN)

Epilepsy is the most common chronic neurological condition of childhood, affecting 500,000 children in the United States. More than 50% of children with epilepsy have an underlying genetic etiology. Our program seeks to diagnose, treat and ultimately cure epilepsy by expanding genetic testing to all children with epilepsy, thereby optimizing medical and surgical care and fueling development of precision therapies.

Jill and Mark Fishman Center for Lymphatic Disorders

The Jill and Mark Fishman Center for Lymphatic Disorders provides highly specialized care for children and adults with lymphatic leaks and lymphatic flow disorders. Experts in our center are developing advanced imaging and interventional technologies to map the anatomy and flow of the lymphatic system, making it possible for our team to identify and manage these conditions using minimally invasive treatment options. Our team has treated hundreds of children and adults with lymphatic flow disorders, making ours the largest and most active treatment program of its kind in the world.

Food Allergy Center

Food allergies affect nearly 6 million children in the United States and nearly 7% of patients cared for at Children’s Hospital of Philadelphia. Our Food Allergy Center is the only pediatric program nationally recognized for its expertise in diagnosing and treating all four types of food allergies: IgE-mediated, eosinophilic esophagitis, food protein-induced enterocolitis syndrome (FPIES), and food intolerance. We are developing new diagnostic tools and novel therapies with the goal of successfully treating — and in some cases curing — all food allergies.



Immune Dysregulation Program

For most children, the immune system helps fight illness. But for nearly 50,000 children in the United States, the immune system malfunctions, causing dangerous inflammation and organ damage and leaving children vulnerable to infection. Our program harnesses expertise from a team of immunologists, oncologists, hematologists, rheumatologists and others to provide cutting-edge diagnostic and genetic testing, coordinate care, and customize treatments. We are also engaged in groundbreaking research to better understand immune disorders and develop more effective therapies.

Minds Matter Concussion Care Program

Despite relatively high incidence, there has been very little progress in the diagnosis and treatment of pediatric concussions. Our program aims to change that, and we have built the leading concussion care center in the region; our focus on translational research allows us to incorporate cutting-edge diagnostics and therapeutics into the clinical care we provide. We have developed a comprehensive clinical assessment of concussions, while introducing aerobic treatment therapies and vestibular and visual rehabilitation. Our team will build on our progress to date by introducing preinjury testing to assess concussion susceptibility and implement clinical diagnostic tools to objectively phenotype concussions in order to prescribe personalized novel treatments.