

The Childhood and Adolescent Cancer and **Blood Diseases Center** 

### Leading Research Guides Personalized Care

The Childhood and Adolescent Cancer and Blood Diseases Center at Maria Fareri Children's Hospital, a member of the Westchester Medical Center Health Network (WMCHealth), is an internationally-recognized, multidisciplinary diagnostic treatment and research center extending comprehensive, quality care while leading game changing research in cancer and blood diseases.

for the youngest cancer targeted approach that's both focused and gentle – one that reduces toxicity and recruits groundbreaking modalities to treat cancerous tissues. Under the direction of Mitchell Cairo, MD, Division Chief, Pediatric Hematology, Oncology and Stem Cell Transplantation at Maria Fareri Children's Hospital, our team draws upon expertise in pediatric hematology, oncology, stem cell transplantation, surgery, pathology, genetics, radiation oncology and immunology to personalize this approach for each child.

Twenty-first-century treatment patients means a personalized,

Our investigations are basic, translational, and clinical meaning that breakthroughs in the laboratory translate directly into patient care. By taking the lead in several trials investigating targeted antibodies, cellular therapy and stem cell transplants, the Center has immediate access to pioneering research that informs therapeutic strategies and enhances quality of care.

Each day we gain expertise, build resources and achieve breakthroughs in the treatment of cancer and blood disease. From bone marrow transplantation to stem cell and gene therapy, we are unlocking treatments and blazing trails to pinpoint the cause, course of personalized treatment, and cure.

We also know, as you do, that each child is unique, and each one deserves the science-based medicine and holistic support to sustain them - in body, mind and spirit – through the rigors and challenges of sickness. With our team of specialists collaborating in the care of each child, we implement breakthrough personalized therapy through pioneering research. From the bedside to behind the scenes, our approaches center on our patients.



"Since children have 70 to 75 years ahead of them, our strategies allow enhanced survival with an approach that's more gentle and far less toxic."

Mitchell Cairo, MD Division Chief, Pediatric Hematology, Oncology and Stem Cell **Transplantation** Maria Fareri Children's Hospital

Featured on our Cover: Julianna, cancer fighter

# Bench to bedside excellence

Division Chief, Pediatric Hematology, Oncology and Stem Cell Transplantation at Maria Fareri Children's Hospital, Dr. Cairo leads a team of researchers to develop and investigate novel strategies of targeted therapies and cellular strategies, including gene therapy and stem cell regenerative therapy, to activate the body's defense system and replace damaged cells with thriving ones. Cellular therapy for childhood cancer and blood disorders has become one of the most promising areas of medical research. The promise of unlocking treatments and cures to rare disorders by harnessing the power of the patient's own body renews the efforts of Dr. Cairo's team to make advances that save lives while minimizing unwanted side effects.

Maria Fareri Children's Hospital provides an unmatched level of care in the region, drawing upon both standard protocols and novel therapies to tailor care to each patient and advance the state of medicine itself. Driven by research and delivered to patients, our hematology and oncology programs represent the gold standard of preventive, diagnostic, innovative and therapeutic strategies. With specialists available for consultation around the clock, our Childhood and Adolescent Cancer and Blood Disease

Center provides state-ofthe-art, compassionate and customized care for each young child and adolescent in the following pediatric clinical and therapeutic programs:

#### **Clinical**

Hematological Malignancies

Histiocytic Disorders

Neuro-Oncology

Solid Tumors

Bone Marrow Failure

Primary Immunodeficiencies

Sickle Cell Disease

Thrombosis and Hemostasis

#### **Therapeutic**

Blood and Marrow Transplantation

Cellular Therapy

Hematotherapy

Developmental and Experimental Therapies

Wellness and Survivorship

#### Healing together

At Maria Fareri Children's Hospital, families play an active role in the healing process.



Our Child Life and Creative Arts
Therapy team works closely with
pediatric oncology patients and
families as they adjust to the physical
and emotional rigors of cancer
care. Using music, literature, art and
therapeutic play, team members
guide children through difficult
moments by encouraging them to
be creative and discover their own
strength.



The Ronald McDonald House of the Greater Hudson Valley, just steps away from Maria Fareri Children's Hospital, provides a temporary home away from home for families of children facing illness.



Our "allogeneic donor" treatment program for certain cancers and blood disorders takes parents' involvement in the healing process to new, unprecedented levels.

Maria Fareri Children's Hospital has pioneered a bench-to-bedside therapeutic approach to collect stem cells from parents to support children with sickle cell disease, aplastic anemia, acute leukemias and lymphomas.

### Regeneration and Repair

#### **Malignant Diseases**

Acute Lymphoblastic Leukemia

Acute Myeloid Leukemia

**Chronic Myeloid Leukemia** 

Juvenile Myelomonocytic Leukemia

Non-Hodgkin Lymphoma

(Burkitt, Large Cell Lymphoblastic, Anaplastic)

**Hodgkin Lymphoma** 

Neuroblastoma

**Brain Tumors Wilms'** 

**Tumor Sarcomas** 

**Germ Cell Tumors** 

**Liver Tumors** 

Hemoglobinopathies

**Immunodeficiencies** 

**Metabolic Disease** 

**Autoimmune Disease** 

Found in adult bone marrow and a baby's umbilical cord, stem cells carry the astonishing potential to develop into different cell types, creating an internal repair network that replenishes cells without limit. For a baby or child with cancer or blood disease, an influx of healthy stem cells can restore the ability to produce healthy blood and immune cells, setting the patient on a course toward wellness. Our Blood and Marrow Transplantation (BMT) Program consists of a multidisciplinary team of specialists dedicated to the therapeutic use of bone marrow, peripheral blood (stem cells released from the bone marrow to bloodstream), cord blood and cellular therapy for children, adolescents and young adults with malignant, non-malignant and serious blood-related disorders.

The Developmental and Experimental Therapeutics Program focuses on the translational and clinical application of new therapeutic strategies in early-phase clinical investigation. This program is designed to gauge the safety,

feasibility, dose, schedule and early efficacy of new therapeutic strategies during experimental clinical investigation in children, adolescents and young adults with both malignant and non-malignant (blood disease-related) disorders.

#### Our areas of expertise

Phase I/II Clinical Trials

Bench to Bedside Therapy (Translational)

Humoral Immunotherapy

Radioimmunotherapy

Cellular Immunotherapy

Stem Cell Transplantation

Targeted Cellular Therapy

Immunotoxin Therapy

These specialties yield the opportunity for students, residents and fellows to pursue scholarly research in this field. Most importantly, this program provides personal and customized medical therapy and care to children, adolescents and young adults.

## Featured Programs



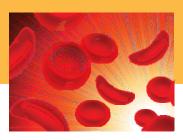
The Hematological Malignancy Program (Leukemia/Lymphoma) consists of a multidisciplinary team focused on the prevention, diagnosis and treatment of cancers arising in the blood, bone marrow and lymphatic system. The multi-disciplinary team consists of pediatric oncologists, surgeons, pathologists, radiation oncologists, bone marrow transplantation specialists, cellular therapy specialists, a psychologist, neuropsychologist, nurse practitioners and social workers.



The Solid Tumor Program focuses on solid tumors, abnormal masses of tissue, in children, adolescents and young adults, including neuroblastoma, kidney tumors such as Wilms' tumor, liver tumors such as hepatoblastoma and hepatocellular carcinoma, bone tumors such as Ewing's sarcoma and osteosarcoma, soft tissue tumors such as rhabdomyosarcoma, and germ cell tumors in the ovaries and testicles. The multi-disciplinary team includes pediatric oncologists, surgeons, urologists, pathologists, radiation oncologists, gynecologists, otolaryngologists, a psychologist, nurse coordinators and social workers.



Our Neuro-Oncology Program provides both standard treatment plans and research protocols, as well as individualized therapy driven by novel cellular and immune modalities. The crossdisciplinary team includes a pediatric neurosurgeon, pediatric neuro-oncology staff, a pediatric radiation oncologist, pediatric neuropsychologist, pediatric neurologist, neuroradiologist, nurse coordinators and social workers.



The Sickle Cell Disease Program unites an experienced team of pediatric hematologists, bone marrow transplant specialists, a neuropsychologist, nurses and social workers advancing both standard and novel treatment strategies. Identified through the Hudson Valley Newborn Screening Program, patients and their families receive care in a family-centered atmosphere, with testing and appointments with sub-specialists accomplished on the same day. This program is the founding center in the Haploidentical Sickle Cell Disease Consortium stem cell transplant clinical trial, a study that recruits parents as bone marrow donors to replace sickle red blood cells with healthy red blood cells in their child.

### Hematotherapy

Clinical services don't have to feel so...clinical. Our full-service apheresis and transfusion center provides care to patients of all ages at both Maria Fareri Children's Hospital as well as our newly renovated, kidfriendly outpatient center that has individual patient bays for comfort and privacy, flat-screen televisions and complimentary Wi-Fi.

We offer consultation and treatment plans for many types of hematotherapy services, including:

Therapeutic plasma exchange

Peripheral stem cell collection

Red cell exchange

Extracorporeal photopheresis

Leukapheresis and plateletpheresis

Blood transfusions

Blood product transfusions

Phlebotomy

Extracorporeal photopheresis

The Hematotherapy Center has equipment to transfuse blood components and infuse blood product derivatives and medications, such as:

Red blood cell or platelet infusions

Factors, iron and other infusion treatments

Therapeutic phlebotomy for conditions such as hemochromatosis or polycythemia vera

As with all clinical services, physicians are available 24 hours a day.



Althea, sickle cell anemia survivor

Child Life specialist Lauren Nittoli with Ashley, cancer fighter





ABOL AGUE



Kaeli, cancer fighter, at right, with Aidan, Kaeli's twin and biggest supporter





# Pediatric Cancer Research Foundation Laboratory\*

By aligning vast clinical experience with unmatched scientific expertise, the Pediatric Cancer Research Foundation Laboratory centers on six areas of groundbreaking research:

Cancer genetics and treatment of childhood and adolescent hematological malignancies

Genetic re-engineering of immune cells to enhance cellular cancer immunotherapy

Tumor immunology and target identification for humoral immunotherapy

Stem cell biology, cancer stem cells and regenerative therapy

Targeted therapy for childhood, adolescent and young adult tumors

Translational (bench to bedside) therapeutic consortiums for childhood and adolescent cancer therapy In addition, our laboratory has developed several national translational research consortia focusing on stem cell transplantation and cellular therapy for high-risk patients with acute leukemia, lymphoma cell therapy, pediatric sarcoma cell therapy, familial stem cell transplantation for sickle cell disease, and non-Hodgkin lymphoma translational research and treatment. Due to our "bench to bedside and back" research, children travel from across the country, and the world, to receive treatment at Maria Fareri Children's Hospital. Findings in the Pediatric Cancer Research Laboratory translate directly into patient care through the support of the Cellular and Tissue Engineering Laboratory.

\* Supported by the Pediatric Cancer Research Foundation (pcrf-kids.org)





Cancer fighter, Julianna, with Dr. Mitchell Cairo and her parents

# Cellular and Tissue Engineering Laboratory

The Cellular and Tissue **Engineering Laboratory** (CTEL) within the Children and Adolescent Cancer and Blood Disease Center at Maria Fareri Children's Hospital supports a range of translational programs in oncology, hematology, neurology, genetics and cardiology that harness stem cell, cellular, tissue and gene therapies. The laboratory consists of an 8,000-square-foot "current good manufacturing practice" (CGMP) facility. But this manufacturing "plant" doesn't turn out cars, pens, candies, clips or anything that can be seen with the naked eye. Instead, it genetically re-engineers human cells, which is the defining feature of medicine's new frontier.

The state-of the-art equipment in the CTEL is designed to facilitate cellular therapy (CT) under investigational new drug applications (IND), in support of cellular therapy that will treat a variety of disorders and diseases. All pediatric and adult blood and marrow transplantation programs, and other regenerative

cellular therapy programs at Westchester Medical Center and Maria Fareri Children's Hospital, rely on cell manufacture and processing in the CTEL.

With a focus on oncology, hematology, neurology, genetics and cardiovascular therapies, the CTEL supports a range of translational programs that harness stem cell, cellular, tissue and gene therapies. The research team has Food and Drug Administration approval for several clinical protocols to treat such disorders as sickle cell disease, in which red blood cells deform into a "sickle" shape. Therapeutic action in this case uses the patient's mother or father as a donor, with peripheral blood as a stem cell source, to replace damaged sickle cells with healthy red blood cells. Our research team is also investigating a clinical protocol to alleviate the effects of severe hypoxic ischemic encephalopathy, the lack of oxygen delivered to a baby's brain - resulting in neuron injury, cell death, and compromised brain, heart and motor function. In this protocol,

stem cells from the newborn's cord blood work with stem cells derived from the newborn's placenta to support cell recovery.

Bringing these therapies to fruition allows Maria Fareri Children's Hospital, and the Westchester Medical Center Health Network, to create and provide patient-specific therapies without requiring patients to travel far from home. Our aim to bring tomorrow's therapies to patients, today, invigorates our mission to advance medicine, provide state-of-the-art care, and keep all children happy and healthy.



#### Case Study: Natural Killers

It sounds like a movie, but these cells are an actual population of white blood cells that hunt down and kill cancer cells - in other words. they're "cytotoxic." These naturalkiller cells are small in number, but in the Cellular and Tissue Engineering Laboratory (CTEL), once derived from peripheral blood or cord blood, they can increase, expand and "beef up" to grow even more powerful and effective. In diseases such as lymphoma, leukemias, sarcomas, neuroblastoma and brain tumors the activated NK cells can be triggered to hone in on specific antigens or receptors on tumor cells, killing them to eradicate or shrink the tumor in the patient.

## Wellness and Survivorship

The Institute of Medicine tells us that survivorship begins at the time of diagnosis, and yet treatment of childhood cancers and blood disorders may have effects long after therapy ends. Therefore, continued long-term care that supports pediatric and adult health is key.

In the Wellness and Survivorship Program at Maria Fareri Children's Hospital, our goal is to prevent, detect and treat problems that could arise from treatment for cancer and blood disorders, including from stem cell transplantation. As with each program, a multidisciplinary team of specialists is dedicated to the holistic care of each patient, providing comprehensive evaluation of the physical, psychological and social needs of our survivors. This approach ensures optimal healthcare delivery to this unique population. The program

additionally participates in the development of clinical research trials to reduce the long-term effects of chemotherapy and radiation on our young and growing patients.

The survivorship "care team" consists of a pediatric hematologist/oncologist, nurse practitioner, neuropsychologist and social worker who are specially trained in caring for survivors. Along with a multidisciplinary team of specialists with expertise in the field, patients in the program also receive a personalized Survivorship Care Plan, which provides a summary of their treatment and a customized plan for follow-up in the years to come. Recommendations are based on our vast experience caring for survivors, as well as guidelines published by the Children's Oncology Group, experts in the field of childhood cancer and beyond.

The Wellness and Survivorship Program:

Monitors and manages physical and psychological late effects

Provides health education to survivors regarding their diagnoses, treatment exposures and potential late effects

Provides referrals to specialists and community resources

Encourages activities that promote health and wellness

Supports social, educational and vocational needs

Assists with financial and insurance issues

Guides transition from pediatric to adult-focused healthcare

Empowers survivors to advocate for their own healthcare needs

Facilitates research into longterm survivorship







Jack, a cancer fighter, with Darshan Patel, MD

## Our Family-Centered Care Philosophy: Hope and Healing

Maria Fareri Children's Hospital is the combined vision of hundreds of parents, medical professionals and community leaders who shared a fervent desire to create a family-centered place of care where parents are not visitors, but partners. The vibrant and playful surroundings become an integral part of the healing process.

As the leading treatment center for the Hudson Vallev and Fairfield County, Maria Fareri Children's Hospital provides unsurpassed medical expertise, family-centered compassionate care, and cutting-edge treatments delivered by internationally renowned teams of pediatric specialists and allied health practitioners. Multidisciplinary excellence builds a synergy among the diagnosis, treatment and follow-up of each patient - creating a personalized approach that's unique to each child, within an atmosphere that fosters hope and healing by removing the "hospital" from "hospitalization."

Our unmatched level of care is delivered in an equally state-of-the-art building,

designed specifically from the point of view of children and their families. Active family involvement creates the environment that allows us to provide advanced care, especially through the physical and emotional rigors of prolonged illness. From the minute you arrive at the hospital, we're in this together.

Children and adolescents in treatment for cancer or blood disorders at Maria Fareri Children's Hospital may stay here for a while, but they will never feel "hospitalized." Instead, they will engage with families, peers, care providers and others in age-specific neighborhoods on their journey toward healing.



Maria Fareri, after whom the Children's Hospital is named, wrote an essay about her wish "for the health and well-being of all the children in the world."



"Life's roughest storms proved the strength of our anchors."

Andrew, cancer fighter





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Westchester Medical Center Health Network includes:

Westchester Medical Center | Maria Fareri Children's Hospital Behavioral Health Center | MidHudson Regional Hospital Good Samaritan Hospital | Bon Secours Community Hospital St. Anthony Community Hospital | Health Alliance: Broadway Campus Health Alliance: Mary's Avenue Campus | Margaretville Hospital