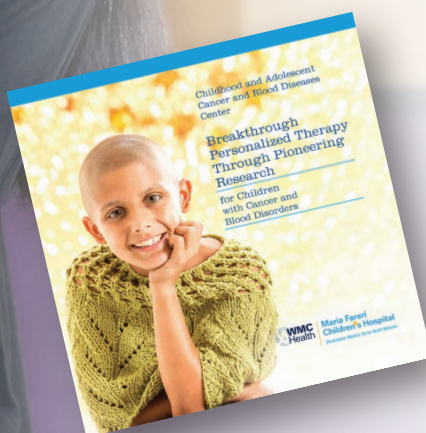




Childhood and Adolescent
Cancer and Blood Diseases
Center

Breakthrough Personalized Therapy Through Pioneering Research

for Children
with Cancer and
Blood Disorders



**Maria Fareri
Children's Hospital**

Westchester Medical Center Health Network



The Childhood and Adolescent Blood Diseases Center team, dedicated to positive outcomes for all patients.

On the cover: Juliana, a diffuse large b-cell lymphoma survivor, pictured in summer 2019 and during treatment in 2016.

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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 (WMCHHealth), is an internationally recognized, multidisciplinary diagnostic treatment and research center extending comprehensive, quality care while leading game-changing research in cancer and blood diseases.

Twenty-first-century treatment for the youngest cancer patients means a personalized, 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.

Our investigations are 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 deserves the science-based medicine and holistic support to sustain him or her – 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*

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Hematotherapy: 914.493.2373
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MariaFareriChildrens.org/hematology-oncology

Bench to Bedside Excellence

As 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 own 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 for 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.

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.



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. 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-of-the-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

Nurse Practitioners

As essential members of each specialty team, our Pediatric Nurse Practitioners are a dedicated group of professionals involved in every aspect of treatment. In partnership with your oncologist and with you, PNP's coordinate care from diagnosis through treatment and into survivorship while bringing a unique blend of energy, creativity, compassion, knowledge, and empathy into every patient experience.

Top row: Maggie Londono, Meghan Sturhahn, Connie Leonick, Megan Campbell.

Front row: Anne Panarella, Karen Wolownik, Sandi Fabricatore, Tara Giblin, Rebecca Belsky.

Compassionate Support from Social Worker Team



Social workers Barbara McLain and Rose Bartone are members of the Association of Pediatric Oncology Social Workers (APOSW).

A diagnosis of cancer or a serious blood disorder is a life-changing event. Pediatric oncology social work, as a specialty discipline, is committed to enhancing the emotional and physical well-being of children with cancer and their families. This support is based upon a unique body of knowledge and expertise in the areas of bio-psychosocial care and the impact of life-threatening illness on normal development and family life.

A pediatric oncology social worker can have many roles: providing support, counseling, education and referrals to community resources; serving as a school liaison; and handling case-management tasks for

children and families coping with cancer, blood disorders and other serious illnesses.

At diagnosis, each child and his/her family is assigned a social worker who will follow them from diagnosis and beyond. The social worker helps families manage and adjust to the diagnosis and the day-to-day challenges associated with a serious diagnosis and its treatment. Assistance includes:

Helping families navigate the medical system and bridge connections between the family and medical team.

Identifying resources for the child and/or the family, to enhance psychosocial well-being.

Assessing risk for financial hardship and making appropriate referrals for assistance.

Helping school-age patients remain connected with school to lessen the impact of treatment on their social, emotional and academic development, including helping with school re-entry.

Helping families and children advocate for their needs within the hospital and community.

Sibling support.

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.

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

Non-Malignant Diseases

Bone Marrow Failure Syndromes

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.

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 for children, adolescents and young adults.

Featured Programs



Jeremiah is a bone marrow transplant recipient and is doing well.



Warrior Princess Emma Levine has become a local celebrity following an excellent response to her personalized medicine that treats her rare form of kidney cancer.

Blood and Marrow Transplant

Our Blood and Marrow Transplantation Program consists of a multidisciplinary team dedicated to the therapeutic use of bone marrow, peripheral blood, cord blood and cellular therapy in the treatment of children, adolescents and young adults with malignant, non-malignant and serious blood-related disorders.

Unique Features

Accredited by the Foundation for the Accreditation of Cellular Therapy (FACT)

Accredited by the Children's Oncology Group (COG), experts in the field of childhood cancer

Accredited by the National Marrow Donor Program (NMDP)

Pediatric Blood and Marrow Transplantation Consortium (PBMTTC) member

Cellular and Tissue Engineering Laboratory certified as Good Manufacturing Practice (GMP) quality

Cord Blood Diseases and Regenerative Therapy Program

Cord Blood Transplantation Program

Unrelated Adult Donor Stem Cell Program

Haploidentical Stem Cell Program

T-Cell Depletion and Stem Cell Selection Program

High-Risk Leukemia Consortium (lead institution)

Lymphoma Cell Therapy Consortium (lead institution)

Sickle Cell Transplantation Consortium (lead institution)

Ten HEPA-filtered and positive-pressure inpatient rooms

Personalized and customized blood, marrow and cellular therapies

Multidisciplinary programs

Developmental and Experimental Therapeutics Program

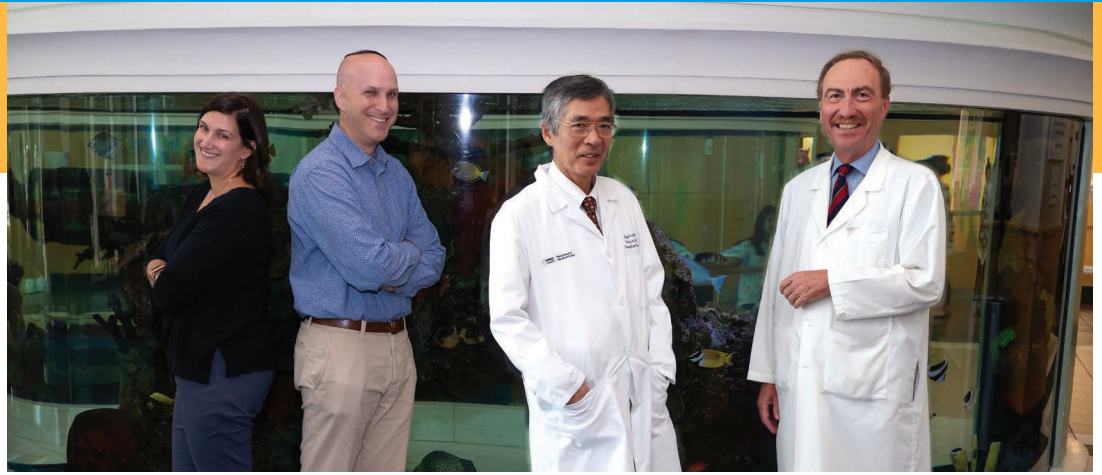
The Developmental and Experimental Therapeutics Program focuses on the translational and clinical application of new therapeutic strategies in early phase experimental clinical investigation. These therapeutic strategies are designed to determine the safety, feasibility, dose, schedule and early efficacy of new treatments during experimental clinical investigation in children, adolescents and young adults with both malignant and non-malignant (blood disease-related) disorders.

This unique comprehensive program has several state-of-the-art areas of expertise including phase I therapy, phase II therapy, bench-to bedside therapy (translational),

humoral immunotherapy, radioimmunotherapy, cellular immunotherapy, stem cell transplantation, targeted cellular therapy, immunotoxin therapy and others. The program also provides a special opportunity for students, residents and fellows to pursue scholarly research in developmental and experimental therapeutics in children, adolescents and young adults with malignant and non-malignant conditions. Most importantly, this program provides for personal and customized therapy and care to children, adolescents and young adults in our Childhood and Adolescent Cancer and Blood Diseases Center.



Jackie Basso, PNP, and Mia Bento, leukemia survivor, celebrating completion of her Children's Oncology Group chemotherapy protocol.



Rebecca Belsky, PNP, and Jeremy Rosenblum, MD, work together with Siego Nishida, MD, and Mehmet Ozkaynak, MD, to tackle all types of solid tumors.

Hematological Malignancy Program

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 main focus of the Hematological Malignancy Program is to investigate novel therapies that improve or maintain excellent outcomes for all patients while minimizing short- and long-term complications. Numerous investigator-initiated clinical trials, as well as larger consortium collaborations, help achieve this goal.

Solid Tumor Program

The Solid Tumor Program focuses on solid tumors. These are 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,

radiologists and radiation oncologists, gynecologists, otolaryngologists, a psychologist, nurse coordinators and social workers.

Patients benefit from unique less-invasive surgical techniques. Interventional radiologists participate in diagnosis and treatment strategies. An on-site True Beam Linear Accelerator is available for radiation treatment.

Liver Transplant Program

Hepatoblastoma is the most common pediatric liver tumor. A combination of chemotherapy and surgery is one of the treatment choices. This works well for some patients. If tumors are too big to be removed, liver transplant can be curative and one of the treatment choices. Liver Transplant Program teams can perform both liver resection and liver transplantation in pediatric patients. The program is approved for both pediatric and adult liver transplant by the United Network for Organ Sharing, a nonprofit organization that manages the United States' organ-transplant system under contract with the federal government.



Hunter Corcoran, brain tumor survivor.



Marc Arkovitz, MD, and Physician Assistant Jennifer Bricker make daily rounds to see all pediatric oncology patients requiring surgical procedures.

Neuro-Oncology Program

For patients with central nervous system tumors of the brain and spine, our Neuro-Oncology Program provides established treatment plans as well as access to clinical trials and the latest in individualized therapies driven by novel cellular, genetic and immune modalities. Pediatric brain tumors can be either benign or malignant, and our team of experts will support you in understanding your child's diagnosis and options for treatment from Day 1.

This cross-disciplinary team consists of experts in pediatric oncology, neurosurgery, radiation medicine and neuroradiology. We additionally have a dedicated neuropsychologist who will evaluate and monitor your

child's cognitive development before, during and after therapy. Depending on your child's diagnosis, we may additionally bring in specialists from fields including pediatric neurology, endocrinology and ophthalmology to ensure that your child receives comprehensive care. As with all of our programs, patients are supported from the time of diagnosis through treatment and survivorship by a nurse practitioner coordinator, social workers and a psychologist.

Surgical Oncology

The Childhood and Adolescent Cancer and Blood Diseases Center's surgical oncology services include access to our world-class pediatric surgical oncology team, state-of-the-art facilities, and dedicated pediatric anesthesiologists within Maria Fareri Children's Hospital.

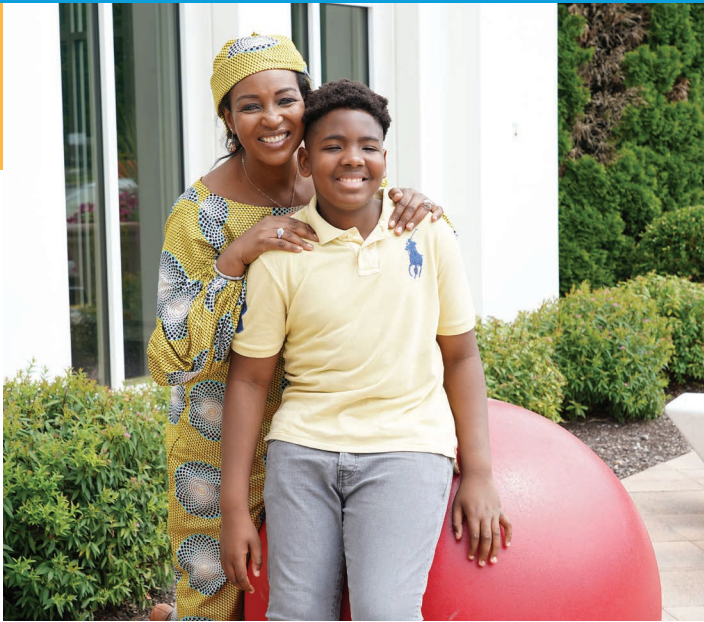
Surgical oncologists work together with the treatment teams to provide necessary surgical interventions as a primary or complementary component of a patient's treatment plan.

The surgical oncology team's main roles include performing:

Diagnostic Surgery: A biopsy to obtain a sample of tumor tissue to examine the tumor type, and to determine if it has spread to other parts of the body.

Supportive Surgical Interventions: The placement of central venous access devices to provide a route to safely administer intravenous chemotherapy or other treatments.

Surgical Treatment: The removal of tumors through both minimally invasive surgeries and complex open surgical resections.



Hanif Mouehla is cured of sickle cell disease following a bone marrow transplant with cells from his mother, Khuraira.

Sickle Cell Disease Program

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.

The program also has pioneered a clinical trial investigating defibrotide (a drug that stabilizes endothelial cells) to treat sickle cell disease-associated acute chest syndrome.

Histiocytic Disorders

The histiocytosis program consists of a multidisciplinary team focused on the diagnosis and treatment of histiocytic disorders in children and adolescents. Histiocytic disorders are a group of diseases that occur when there is an overproduction of histiocytes (cells that help prevent infection) or mononuclear phagocytic cells of bone marrow origin. Overproduction of these cells can lead to organ damage and cause disease, based on the type of the cells involved. Correct and complete pathologic diagnosis is essential because the different diseases require different treatment approaches. We focus on dendritic cell disorders,

including Langerhans cell histiocytosis (LCH), juvenile xanthogranuloma (JXG) and Erdheim-Chester Disease (ECD); macrophage cell disorders, including Hemophagocytic Lymphohistiocytosis (HLH) and Rosai-Dorfman Disease (RD); and malignant histiocytosis, including certain kinds of leukemia and malignant tumors.

Thrombosis and Hemostasis

The Thrombosis and Hemostasis Program is dedicated to treating children with bleeding and clotting problems. The team includes physicians, nurses and social workers with special interest in hemophilia, coagulation factor deficiencies and clotting disorders. Beyond the care of children afflicted with these conditions, we also provide family education, social interactions among families of affected children, home care and support as the goal of our team to help parents and families with their inevitable emotional challenges. We engage in ongoing research for developing innovative therapies and for addressing safety issues, such as complications or risks that may be associated with medications.

The program has implemented less-invasive oral medications to prevent recurring thrombosis in children.



In addition to her vast experience and active involvement in the treatment of pediatric oncology patients, Oya Tugal, MD, has focused in recent years on pediatric bleeding disorders and thrombosis. She is conducting clinical research in the treatment of venous thrombosis in children with a novel oral anticoagulant. Histiocytic disorders in children is one of her strong interests.



Primary Immunodeficiencies

The Childhood Primary Immunodeficiency Program is a multidisciplinary and comprehensive program dedicated to the diagnosis, prevention, treatment and long-term follow-up of children diagnosed with primary immunodeficiency syndromes. The program consists of several state-of-the-art programs in newborn screening, prevention, genetic diagnosis, stem cell transplantation, reduced intensity conditioning and adoptive cellular immunotherapy. The program also provides a unique opportunity for clinical, translational and basic research for students, residents and

fellows focused on the biology and treatment of primary immunodeficiency in children. Most importantly, the program is designed to provide customized and personal care and long-term follow-up to each child with a primary immunodeficiency.

The program is one of 42 centers in North America participating in the National Institutes of Health-funded Primary Immunodeficiency Treatment Consortium.

SCID/ TREC Screening Follow up Center

Since December 2018, all newborns in the United States are screened for Severe Combined Immune Deficiency (SCID), a potentially fatal disorder caused by improper development and function of immune system cells. New York State's Newborn Screening Program includes DNA analysis for T-Cell Receptor Excision Circles (TRECs) that can indicate whether a child may have SCID. Early diagnosis allows interventions to take place before serious infections occur. The newborn screening program refers infants with low or absent TRECs to immunologists at Maria Fareri Children's Hospital's Specialty Treatment Center for further evaluation. Infants diagnosed with SCID can be cured with an effective treatment plan that includes stem cell transplantation.



Justin, at left, a healthy survivor whose brain tumor was treated with chemotherapy and, later, radiation therapy using our True Beam Accelerator, pictured at right.

Radiation Therapies

We offer expertise and knowledge in radiation oncology to ensure effective treatment for both child and adult cancer patients.

Radiation is used to destroy cancer cells or to eliminate pain by directing high-energy radiation directly into cancer tumors, called “hot spots.” This process destroys existing cancer cells and keeps remaining cells from reproducing. As cells die, they are removed from the body by the patient’s natural waste-elimination processes.

Radiation Oncology

The Department of Radiation Medicine at Westchester Medical Center, flagship of the Westchester Medical Center Health Network (WMCHHealth), is a full-service radiation oncology cancer treatment center dedicated to offering the highest level of care to our cancer patients.

Radiation Medicine

Radiation is commonly used to safely treat cancer patients. The Department of Radiation Medicine provides several types of radiation therapy to help treat cancer patients (pediatric and adult) and some secondary symptoms associated with cancerous conditions.

Radiation Services

External Beam Radiation: We deliver radiation via our on-site True Beam Linear Accelerator, which uses cutting-edge technology such as image-guided radiation therapy, intensity-modulated radiation therapy, stereotactic radiosurgery and electron-beam radiation. These treatments significantly increase accuracy of radiation delivery, avoid exposure to healthy tissues and organs, reduce treatment time, and provide high-quality Cone Beam CT imaging of tumors and surrounding tissues.

In addition to conventional methods of radiation, the program offers several highly specialized, high-precision, treatments that target the growth of tissues while minimizing radiation to adjacent uninvolved healthy organs or tissues from high radiation doses (conformal radiation techniques).

Intensity Modulated Radiation Therapy: Using computerized three-dimensional models, radiation beams are modulated for size and angle and then focused directly into cancer tumors, delivering high doses of effective radiation to the cancer with very little damage to the surrounding area.

Stereotactic Radiosurgery: This “knifeless” surgery is used for tumors of brain and other areas and involves the use of ultra-thin beams of high-dose radiation focused directly onto the cancer growths. This technique reduces the amount of damage to surrounding tissue.

Brachytherapy/Internal Radiation: Brachytherapy works by putting radiation as close to cancer tumors as possible, sometimes even directly into tumors via wires, rods or seeds comprised of iridium or iodine radio-isotopes.

Chemoradiation: Combining chemotherapy and radiation can be an effective approach to treating and possibly destroying a wide variety of cancers. Our radiation and medical oncologists work closely with our surgeons to determine a customized plan of action for each patient prescribed chemoradiation.

Hyperthermia: Just as the name implies, hyperthermia is a heat treatment that exposes cancer tumors to high temperatures. The high heat can destroy the reproductive ability of cancer cells, and typically is delivered via ultrasound, microwaves or radiofrequency methods.

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, kid-friendly 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.

Patricia Pambianchi, Maria Tirado, Kim Christiano, Lauren Toribio and Rosemarie Shaw.



Unique Approaches with Minimal Toxicities, and a Family's Story

Carmen, at right, was the recipient of stem cells from her sister Edith, when her baby sister was merely 4 months old. This unique sibling-to-sibling transplant provided Carmen with life-saving stem cells that cured her of aplastic anemia.

As Carmen recovered from many of the side effects of the chemotherapy used during her treatment, Edith's cells started to attack healthy cells in Carmen's body. To combat this Graft-Versus-Host Disease (GVHD), another unique treatment was needed: extracorporeal photopheresis (ECP), a hematotherapy treatment provided only at Maria Fareri Children's Hospital in the Hudson Valley. During extracorporeal photopheresis, Carmen's blood circulated through a special machine. In the machine, a drug is added that makes the white blood cells from Edith sensitive to light. When the machine shines an ultraviolet light on the white blood cells, it activates the drug. All the cells are then given to Carmen, where the treated cells stimulate an immune response that diminishes the attack on her healthy cells.

Carmen's recovery also was challenged by a viral infection that worsened despite treatment

with all of the approved antiviral medications. Her body organs suffered due to exposure to multiple medications. Thanks to a clinical trial developed by Mitchell Cairo, MD, and his research team, and the extraordinary capabilities of our Cellular Tissue and Engineering Lab (CTEL), Carmen received a treatment using cytotoxic T lymphocytes (CTL). The hematotherapy team used a pheresis machine to remove cells from Carmen's mother that were found to have the ability to fight the virus that infected Carmen. After being processed in the laboratory, these CTLs were given intravenously to Carmen. They attacked the virus while sparing her body organs from the exposure and damage of standard antiviral drugs.

Fighting Graft-Versus-Host Disease using ECP and viral infections with CTLs are just some of the ways the Childhood and Adolescent Cancer and Blood Diseases Center is combating life-threatening complications using unique therapies that have minimal toxicities.

Thanks to these treatments, and the love of her family, Carmen now has a bright outlook.

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)



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, the defining feature of medicine's new frontier.

The state-of-the-art equipment in the CTCL 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 CTCL.

Most recently, the program created a novel and unique multi-viral Cytotoxic T Lymphocyte Consortium to treat refractory viral infections in children with immune deficiency.

With a focus on oncology, hematology, neurology, genetics and cardiovascular therapies, the CTCL 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 natural-killer 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.



Cancer survivors Daniel, at far left, and Alex, at right, join their doctor, Mitchell Cairo, MD, and celebrate their health at a New York Yankees game. Both Alex, diagnosed in his teens, and Daniel, diagnosed in his early 20s, benefited from the special services offered through the Adolescent and Young Adult Program.

Adolescent And Young Adult (AYA) Program

Patients between the ages of 15 and 39 receive specialized care through our Adolescent and Young Adult (AYA) Program. Did you know that more than 70,000 new cancer diagnoses occur in this population every year? The Childhood and Adolescent Cancer and Blood Diseases Center provides specialized care tailored for this age group.

The needs of AYA patients with cancer are multiple, complex and different from young pediatric patients or older adult patients. AYAs are typically navigating mature issues at the time of diagnosis. These include self-identity, independence, relationships, increasing school and professional responsibilities, and issues of sexuality and fertility. The AYA Program acknowledges and meets these needs head-on via a multidisciplinary team that includes a psychologist, social worker, nurse practitioners

and physicians specializing in care for AYA patients. Team members consult with newly diagnosed patients age 15 and older to provide age-appropriate resources, counseling and referrals on a wide range of topics personalized to each patient's needs. The many resources provided include counseling on the potential risks of treatment to future fertility, and access to local reproductive endocrinologists who specialize in fertility preservation for patients with cancer.

The mission of the AYA Program is to provide high-quality, evidence-based, coordinated care that promotes the physical, psychological and emotional health of adolescents and young adults with cancer during and after therapy by empowering them with the resources and knowledge they need to live an independent and fulfilling life.



Suzanne Braniecki and Lila Pereira.

Psychological Services

Patients facing treatment for chronic medical conditions face concerns that can stretch beyond the physical. It is also not uncommon for physical issues to impact emotional well-being and vice versa. Our psychological team addresses these needs by providing support for patients at each stage of treatment.

Psychotherapy Services

A pediatric psychologist offers psychotherapy for patients coping with illness, adjustment to life after treatment, compliance with medical treatment, and/or behavioral management of physical symptoms such as pain, nausea and sleep. Treatment may also focus on trauma, depression, anxiety, behavioral issues and a host of other concerns. Modalities

used include individual, group or family therapy utilizing cognitive behavioral therapy; motivational interviewing; parent-child interaction therapy; dialectical behavioral therapy skills; and other interventions. Additional services are available for siblings.

Neuropsychological Evaluation

Cognitive evaluation is available during different stages of treatment to aid in diagnosis and help prepare for school or occupational planning. Patients also may seek evaluation to better understand the impact of treatment on their cognition.

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 long-term survivorship



Luz and her baby.



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 Valley 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."

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.

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



Top: A commemorative plaque adorns the wall outside of Westchester Warriors Way, an entry for children and families to the Center for Pediatric Oncology and Cellular Therapy at Maria Fareri Children's Hospital.

Bottom: 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."

Patient and Family Advisory Council

The Patient and Family Advisory Council (PFAC) partners family members with staff to identify ways to enhance the patient experience and ensure family-centered care. Members of families actively in treatment, as well as those who have completed their therapies, use their experiences to develop innovative processes and contribute to the Childhood and Adolescent Cancer and Blood Diseases Center's goal of providing state-of-the-art compassionate care. PFAC publishes a quarterly newsletter.



“Life’s roughest storms proved the strength of our anchors.”

Andrew was treated for rhabdomyosarcoma, a type of solid tumor discovered in his leg. Today, he is an advocate for childhood cancer awareness and is dedicated to finding a cure. The inset photo shows Andrew during treatment in 2016.

Appointments, Referrals, Information
The Childhood and Adolescent Cancer and Blood Diseases Center

914.493.7997

Jessica.Hochberg@WMCHealth.org



**Maria Fareri
Children's Hospital**

Westchester Medical Center Health Network

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  @MFCHatWMC #MFCH

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 Hospital: Broadway Campus
Health Alliance Hospital: Mary's Avenue Campus | Margaretville Hospital