

Focus on Pediatric Hematology/Oncology

FOCUS ON PEDIATRIC HEMATOLOGY/ONCOLOGY

Affiliated with Columbia University College of Physicians and Surgeons and Weill Cornell Medical College

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## Pediatric Hematology/Oncology at NewYork-Presbyterian Hospital

When a child is diagnosed with cancer or a serious blood disorder, it affects the whole family. Pediatric hematology/oncology specialists at NewYork-Presbyterian Hospital combine expertise with compassion when caring for every patient — and his or her family — who walks through our doors. Our goal is not only to provide effective therapies, but to return children to lives that are as normal and productive as possible, so they can grow up to be healthy adults.

We provide care for children and adolescents with cancer and blood disorders at two renowned academic medical centers: NewYork-Presbyterian/Columbia University Medical Center, which includes the Morgan Stanley Children's Hospital, and NewYork-Presbyterian/Weill Cornell Medical Center, which encompasses the Phyllis and David Komansky Center for Children's Health. Children receive



ANDREW KUNG, MD (LEFT) AND SUJIT SHETH, MD

exceptional care for a wide range of congenital and acquired blood disorders, including sickle cell disease, hemophilia, platelet disorders, and hemoglobinopathies — especially the thalassemias, for which we are internationally recognized.

We also provide high-quality care for children of all ages and types of cancer — leveraging a strong portfolio of clinical trials which provide patients with extraordinary access to the latest advances. In addition, research scientists at both campuses are conducting basic and translational research that is helping to improve the future for children with cancer and blood disorders.

Our focus on a multidisciplinary approach to care and access to a full array of pediatric specialists enables us to treat even the sickest patients. We invite you to learn about the strengths of our programs and encourage you to contact us for more information or to refer a patient.

**Andrew Kung, MD, PhD**  
Chief, Division of Pediatric Hematology/  
Oncology/Stem Cell Transplantation  
NewYork-Presbyterian  
Morgan Stanley Children's Hospital

**Sujit Sheth, MD**  
Director, Pediatric Hematology/Oncology  
NewYork-Presbyterian  
Komansky Center for Children's Health

One of the nation's leading hospitals for the treatment of cancer and blood disorders in children and adolescents.

## Komansky Center Leads the Way in Benign Pediatric Hematology

The Division of Hematology/Oncology at NewYork-Presbyterian Phyllis and David Komansky Center for Children's Health is the preeminent center in New York, New Jersey, and Connecticut for managing so-called "benign" hematologic disorders in children and adolescents. Our scientists also conduct pioneering basic research in the Children's Cancer & Blood Foundation laboratories in iron metabolism, erythropoiesis, beta globin gene transfer, and the exciting area of exosome and metastasis biology in cancer.

In two particularly strong initiatives, our clinicians and investigators are refining the care of young patients with platelet disorders and defining risk factors for thrombosis in hospitalized children.

**Pioneering the treatment of platelet disorders:** James Bussel, MD (right), Professor of Pediatrics, leads the Platelet Disorders Center at the Komansky Center, which sees more patients with these disorders than any other hospital in the Tri-State area. Dr. Bussel has been an international leader in the field, authoring seminal papers which form the very basis of how these disorders have been managed over the years. Patients with acute problems may be treated using well-established therapies with good success, but those with chronic conditions need more complex, ongoing therapy. These children remain at high risk of having bleeding episodes, spontaneously or with trivial trauma.



When bleeding occurs, particularly in the brain, it can cause significant damage. Because of this, the risk of bleeding requires significant restrictions in a child's day-to-day life, including activities in school, sports, and travel. Preventing these bleeding episodes is key.

Our Platelet Disorders Center leads the world in conducting clinical trials exploring novel agents for patients with refractory idiopathic thrombocytopenic purpura (ITP). Dr. Bussel has pioneered the use in children of the thrombopoietic agents romiplostin (Nplate®) and eltrombopag (Promacta®), which promote platelet production by stimulating precursor cells in the bone marrow. The scope and experience of this program make it unique worldwide. Children with any variety of platelet disorders may be referred

to our program by calling (212) 746-3400.

An innovative laboratory-based program is headed by another member of our Division — Beau Mitchell, MD, Clinical Assistant Professor of Pediatrics. His work focuses on understanding the biology of how platelets are actually made in the bone marrow from stem cells. Advances in this area will guide the clinical management of patients in a direct translational "bench-to bedside" manner.

**Pediatric thrombosis — a rising trend:** As children with complex medical disorders live longer — thanks to medical advances, invasive procedures, and complex surgeries — more of them are surviving with shunts, stents, and surgical hardware inside their bodies that place them at increased risk of forming blood clots. Children who've been treated for short bowel syndrome and those who have had any kind of organ transplant are also at risk. We have also become more aware of inherited conditions which predispose children to forming clots, mainly through better investigation of clots when they occur in parents or siblings.

The incidence of thrombosis among hospitalized children has risen ten- to 20-fold in recent years. This alarming rise has led to increased morbidity and occasionally mortality, and a significant problem of trying to balance the improved survival achieved through medical advances with the secondary risks these treatments bring.

At the Komansky Center for Children's Health, we have established a pediatric-focused thrombosis program. We have created a new registry for any child with a blood clot, coupling clinical information with data on potential risk factors from procedures, immobilization, and factors related to their environment (including lifestyle, medications, and activity) and inherited predisposition. The goal is to use this information to create an algorithm to assess thrombosis risk.

"With more data on risk factors, we will be able to pinpoint which patients need to receive prophylactic treatment and with what agent," said Nicole Kucine, MD, Assistant Professor of Pediatrics and lead physician for the pediatric thrombosis program. "This is a novel approach for children." In addition, we have established a comprehensive "anticoagulation clinic" for the short- and long-term follow-up of children who have had a thrombus — not only providing expert continuous care by a hematologist, but also gathering data which will guide factors such as treatment type and duration.

## Comprehensive Patient-Oriented Pediatric Cancer Care

NewYork-Presbyterian Morgan Stanley Children's Hospital has one of the largest pediatric oncology programs in the United States and is recognized by the National Cancer Institute (NCI) as a center of excellence.

### Patient-Centered Care and Clinical Research

To provide the fullest range of options for children with cancer, we maintain a broad portfolio of clinical, biological, behavioral, and complementary therapy protocols that allow us to provide comprehensive patient-oriented cancer care. Over 90% of families who present to our center choose to have their child participate in at least one clinical trial. For newly diagnosed patients, the Division participates in studies sponsored by the Children's Oncology Group (COG) and the Dana Farber Cancer Institute (DFCI) Leukemia Consortium.

For those for whom standard therapies fail, our center is one of only 20 programs in North America accredited as a member of the COG Phase 1 and Pilot Consortium, and the only member in the Tri-State area. We also provide patients with access to early-phase clinical trials sponsored by other national collaborative groups, such as DFCI and the Therapeutic Advances in Childhood Leukemia & Lymphoma (TACL) consortium. These studies allow us to offer patients access to emerging therapies when conventional options have been exhausted.

Our faculty hold national leadership roles in developing and leading NCI-sponsored trials of novel approaches to treat cancer, including for lymphoma, leukemia, neuroblastoma, liver tumors, kidney cancer, brain tumors, and bone cancers. We are committed to not only treating the disease, but also caring for the whole child and

## Bone Marrow Transplantation: the Only Cure for Sickle Cell Disease

Despite advancements in supportive care for sickle cell disease, the current median life expectancy for patients is only 45, and patients suffer from a range of symptoms such as pain, infections, anemia, stroke, and blindness. The Stem Cell Transplantation Program at Morgan Stanley Children's Hospital is one of the largest in the Tri-State area offering bone marrow transplantation to patients with sickle cell disease. With over a decade of experience, we have the expertise to provide state-of-the-art care for children with sickle cell disease who wish to pursue this curative option.

### Changing the Paradigm of Care

Although bone marrow transplantation is the only curative therapy for those affected with sickle cell disease, the toxicity associated with conventional transplantation approaches has posed a barrier for many patients. Our Stem Cell Transplantation Program has pioneered new approaches that have dramatically improved the outcome for patients undergoing transplantation from a matched sibling donor. Using a method that reduces the intensity of the transplant regimen, we have achieved 100% event-free survival rate over the last five years, utilizing sibling donors to cure patients with sickle cell disease.

Transplantation is also available for certain patients with sickle cell disease who do not have a matched sibling. We are exploring ways to reduce the toxicity of transplants using unrelated donors, including the use of stem cell selection and less intensive conditioning regimens. We also offer access to multiple national clinical trials, including one aimed at improving the success of bone marrow transplantation from an unrelated donor by reducing the intensity of the



THANKS TO BONE MARROW DONATED BY HIS BROTHER MICHAEL (LEFT), MALIK, 8, WAS CURED OF SICKLE CELL DISEASE.

conditioning regimen (the SCURT Study: Sickle Cell Unrelated Transplant Study).

"Given our outstanding outcomes, we have reached a point where we believe bone marrow transplantation should be an option for every child with sickle cell disease who has a matched sibling," explained Monica Bhatia, MD, Director of the Stem Cell Transplantation Program, who directs the program. "About one in five patients falls into this category and may benefit from this procedure. We've shown that bone marrow transplantation improves the overall quality of life for patients with sickle cell disease and their families."

### Personalized Care for Patients with Sickle Cell Disease

Patients referred to our program are evaluated by a team of physicians, nurse practitioners, coordinators, psychologists, and other pediatric specialists as needed. If patients elect to undergo transplantation, this team is expanded to include nurses, social workers, and child life specialists who together care for the child during and after the bone marrow transplantation. We work closely with the primary hematologist and pediatrician to develop a shared care model to ensure optimum care of patients during the post-transplant period.

**For more information or to schedule an appointment, call Elana Smilow, CPNP at (212) 305-8443 or Ria Hawks, CPNP at (212) 305-5593, or visit [nyp.org/kids/morganstanley](http://nyp.org/kids/morganstanley).**

entire family. The Center for Comprehensive Wellness helps provide coordinated support for families, including integrative therapists, social workers, child life, psychological support, and survivor wellness. Through philanthropic support, these services are available without charge to every patient.

### Translational Research

Our laboratory and translational research programs are focused on identifying new causes of childhood cancer and translating these discoveries into clinically relevant methods to diagnose and treat cancer. Recent advances from our program include the discovery of a



KARA KELLY, MD, WITH A YOUNG PATIENT

cause for treatment failure in patients with leukemia, as well as new causes of brain tumors.

We are now exploring the potential of next-generation sequencing to impact the care of patients in the clinic. We have implemented the Precision in Pediatric Sequencing (PIPseq) program and are now offering whole-genome sequencing to patients with high-risk or relapsed tumors. As the program expands, we believe these technologies will help direct individual patients to more beneficial therapies with fewer side effects.