

## FACT SHEET

*Not long ago, the life expectancy of a child diagnosed with thalassemia, hemophilia or other blood disorders was no more than a few years. Rarely did they live to become teenagers. Pediatric cancers were nearly one hundred percent fatal. Today, advances in determining the root causes of these diseases have led to improved treatment methodology and a brighter future for the child. Children suffering from blood disorders are living longer, more normal lives. Nearly eighty percent of those with cancer survive the disease.*

### CCBF Combines Research and Medical Care to Provide Hope for Children and Their Families

#### A History of Innovative Approaches:

The Children's Cancer & Blood Foundation (CCBF), through its support of the Division of Pediatric Hematology and Oncology, has created a standard of excellence in the treatment of children with chronic, life-threatening blood disorders since 1952. Children and their families receive the most advanced, comprehensive care provided by a team of pediatric specialists in an atmosphere of compassion, hope and wellness.

- ❖ The CCBF is the oldest and largest organization in the United States dedicated to the care of children suffering from blood diseases and cancer.
- ❖ The Pediatric Hematology and Oncology Division at New York Presbyterian Hospital/Weill Cornell Medical Center is one of the largest and most comprehensive programs of its kind in the United States. It is home to the largest thalassemia center in the United States, the largest hemophilia center in New York and the country's largest research and treatment center for ITP platelet disorder.
- ❖ Each year, our physicians treat more than 1,000 children through more than 10,000 inpatient and outpatient visits. No child is ever turned away for services, regardless of his or her ability to pay.

#### Research That Leads To Results:

The Division has a long history of combining scientific and clinical research to develop and test new drugs and therapies. This research has led to life-enhancing treatments such as Factors 8 and 9 to treat hemophilia and iron chelation therapy to alleviate iron toxicity in thalassemia patients. Multiple clinical trials are ongoing for the next generation of treatments.

- ❖ CCBF doctors are leading a clinical study involving hundreds of pediatric and adult cancer patients to identify how bone marrow stem cells spread in order to better understand and target tumor growth and metastases. The researchers are also looking at lymphomas, breast, colon, lung cancers and childhood leukemia.
- ❖ Our scientists have identified one of the first critical genes called "Id" that is expressed only in the development of new blood vessels within tumors.
- ❖ The NIH has approved human clinical trials on promising research has the potential to find a cure for thalassemia and other genetic blood disorders including sickle cell anemia. It involves replacing defective genes with normal ones which are the root cause of genetic blood diseases.
- ❖ CCBF scientists may have found a way to avoid removing the spleen in patients suffering from beta-thalassemia. Known as Cooley's anemia, it is the most commonly inherited disease in the world affecting Asians, African-Americans, as well as many people of Mediterranean descent.
- ❖ *The New England Journal of Medicine* published the results of an international multicenter study led by the Division, demonstrating that an oral platelet growth factor called eltrombopag successfully increased platelets and decreased bleeding in patients with ITP. Patients in our clinic have had great results with this new drug treatment
- ❖ We are successfully testing the use of certain proteins to prevent iron absorption in patients who undergo blood transfusions. This is among the most serious side effects of this treatment and can lead to early death.

- ❖ The work of our Comprehensive Hemophilia Diagnostic and Treatment Center was featured in an important Discovery Channel documentary entitled *Blood Detectives*.

### **Family Centered Care:**

The disease of the child is the disease of the family. Physicians, nurses and social workers treat the whole family, not just the child.

- ❖ Social workers work closely with the family to help the young patient's recovery and relieve the family's emotional anxieties. This assistance includes instruction on drug regimens and treatments, individual and family grief counseling and group support sessions for children and families that help them adjust to the stresses and complications arising from chronic illness.
- ❖ We have established the Fund for Critical After-Care which provides financial support to patients and their families once they have left the hospital for such important items as home care, transportation and medication.
- ❖ Our patients are ethnically, culturally and socioeconomically diverse. The predominantly bi-lingual staff reflects this diversity. Efforts are made to overcome language barriers between staff and patients and attention and care is always taken to recognize and appreciate different cultures' reactions and responses to potentially terminal diseases.

### **A World-Renowned Staff**

The Division staff has been recognized by winning multiple professional awards including the Creative Spirit Award of the Creative Center for Women with Cancer, Eagle and Rose Medal-Cooley's Anemia Foundation, First Prize of New Investigator Award-International Society of Experimental Hematology (ISEH) and the 2004 National Hemophilia Physician of the Year from the National Hemophilia Society.

Collectively, the doctors and scientists have published over 500 peer-reviewed articles and 200 textbook chapters. Findings have been published in prestigious scientific journals including, Nature, Blood, Journal of Pediatrics, Thrombosis and Haemostasis, Hemophilia and Science-Now.

#### **Alexander Aledo, M.D.**

Associate Attending Physician  
Associate Professor of Clinical Pediatrics  
Director of the Pediatric Oncology program

#### **James Bussel, M.D.**

Attending Pediatrician  
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#### **Susanna Cunningham-Rundles, Ph.D.**

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#### **Patricia Giardina, M.D.**

Chief of the Division of Pediatric Hematology and Oncology  
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#### **Robert Grady, Ph.D.**

Professor of Pediatric Medicine  
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#### **David Lyden, M.D., Ph.D.**

Vice-chairman for Academic Affairs  
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For more information about the Children's Cancer & Blood Foundation, please visit [www.ChildrensCBF.org](http://www.ChildrensCBF.org), or call 212.297.4336 or email: [info@ChildrensCBF.org](mailto:info@ChildrensCBF.org)