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Dear Friends,

I am pleased to present the Children’s Cancer & Blood Foundation’s (CCBF) Annual Report for the fiscal year ending on December 31, 2014. It has been my honor to serve as Chairman for the past eight years. CCBF is an organization dedicated to providing the best care possible to the children and families treated at the Division of Pediatric Hematology/Oncology of NewYork-Presbyterian Hospital/Weill Cornell Medical Center.

For 62 years, the Children’s Cancer & Blood Foundation has worked closely with the prestigious physicians and researchers at the Pediatric Division to provide comprehensive clinical care, foster cutting-edge research, and train the next generation of leaders in the field of pediatric hematology and oncology. This past year, with CCBF’s support, the Pediatric Division’s investigators and clinicians were able to conduct ground-breaking research and provide comprehensive clinical care that we are proud to share with you.

This important work would not be possible without the extraordinary generosity and foresight of those who donate to CCBF. All of the individuals, foundations and corporations that contributed to the Children’s Cancer & Blood Foundation in 2014 are listed in our Honor Roll of Donors; I would like to personally thank each and every one of you for helping us to ensure that all of our patients receive the best treatment available, provided by a team of compassionate clinicians and researchers. Your dedication allows us to maintain and expand our support of the Pediatric Division’s programs. I hope you will continue to join us in our crucial mission to research, care for, and cure pediatric cancers and blood disorders.

On behalf of all of the children with cancer and blood disorders that we help treat each year, as well as our dedicated physicians and researchers, thank you for your support.

Sincerely,

Les Lieberman
Chairman
OUR MISSION

To support the comprehensive clinical care of children living with cancer and blood disorders

To foster research to help understand the causes of childhood cancer and blood disorders

To sponsor the fellowship training of pediatricians of the subspecialty of pediatric hematology and oncology
For 62 years, the Children’s Cancer & Blood Foundation (CCBF) has helped the Division of Pediatric Hematology and Oncology at NewYork-Presbyterian Hospital/Weill Cornell Medical Center grow into one of the most comprehensive programs of its kind, boasting the nation’s largest thalassemia clinic, the most extensive hemophilia center in metropolitan New York, and the country’s largest center for research and care for Immune Thrombocytopenic Purpura (ITP). The Pediatric Division’s world-renowned experts have shaped the medical landscape with their research and continue to remain on the cusp of breakthroughs predicted to change the way the medical community treats children with cancer and blood disorders.

With the help of CCBF, children with cancer and blood disorders are capable of receiving the most advanced, comprehensive care provided by a team of pediatric specialists in an atmosphere of compassion, hope, and wellness. Each year, more than 500 patients receive consultations and treatment during 8,000 visits to the Pediatric Division. Many of CCBF’s patients are underinsured and unable to pay for costly medications and treatments that are not covered by insurance. Due to CCBF’s support, patients’ families receive the additional support necessary to cover the remaining costs of their treatment and care. In addition to the impact CCBF’s support has on the clinical treatment of its patients, countless children around the world are benefiting from the advances being made in CCBF laboratories by its world-renowned researchers.
HISTORY OF CCBF

The Children’s Blood Foundation was established in 1952 to support the research breakthroughs being made by Dr. Carl Smith. Dr. Smith was a physician and researcher at New York Hospital in the 1940s who began a community outreach program in Queens, New York to treat patients with Mediterranean anemia, which we now call thalassemia. From working with thalassemia patients and conducting research on the disease, Dr. Smith discovered a therapy that allowed affected children to receive frequent, regular transfusions on an outpatient basis instead of during lengthy hospitalizations. This revolutionary discovery changed the way chronically ill children with thalassemia and other types of blood disorders received treatment. The Children’s Blood Foundation was directly founded to encourage Dr. Smith’s work with thalassemia and support other important thalassemia and blood disorder research and care.

New York Hospital, Children’s Blood Foundation’s official home, merged with Presbyterian Hospital in 1997 to become NewYork-Presbyterian Hospital/Weill Cornell Medical Center. The world-renowned research at the Pediatric Division helped the hospital swiftly become one of the most comprehensive university hospitals in the world, and to this day is consistently ranked among the top ten pediatric hospitals in the nation.

The Children’s Blood Foundation changed its name to the Children’s Cancer & Blood Foundation in 2006, emphasizing the agency’s commitment to treat its entire patient population and turn no child with cancer or blood disorders away. Due to funding from CCBF, the Pediatric Division is able to remain at the forefront of groundbreaking research and provide comprehensive clinical care to all of its patients.
RENOVATIONS OF THE PEDIATRIC DIVISION

Because of scientific breakthroughs and new and improved treatment options, children diagnosed with cancer and blood disorders are living longer, healthier lives. Today, the cancer survival rate is over 80%. This tremendous progress has altered the landscape of treatment for these diseases as there previously was not an adult population requiring treatment. Prior to the recent renovations at the Division of Pediatric Hematology/Oncology, the only available treatment space for these patients as they transitioned into adulthood was the pediatric clinic space, which became over-crowded and not strictly pediatric anymore.

The Pediatric Division has a strong commitment to providing incomparable treatment for its pediatric patients with cancer and blood disorders. After evaluating the difficulties of providing optimal treatment to the increasing and aging patient population in the pediatric clinic space, the Hospital decided that it was time to renovate the Pediatric Division and to create a new center exclusively for the adult population to receive treatment. In June 2013, the adult population moved to the Hospital’s newly developed Adult Infusion Center.

With the adult patients’ transition, the pediatric clinic space became free to meet the needs of solely pediatric patients. The primary focus of the renovation was to create a suitable environment for the physicians to provide care in a more comfortable, age-appropriate environment for the patients receiving treatment. With this goal in mind, the Hospital began the renovation project in November 2013. The newly renovated Pediatric Division opened in June 2014.
Dr. Lisa Roth, Assistant Professor in the Department of Pediatrics and Director of the Adolescent and Young Adult Lymphoma Program, specializes in treating children, adolescents, and young adults with lymphoma. She is a member of the Children’s Oncology Group and an active participant in Hodgkin lymphoma and non-Hodgkin lymphoma clinical trials. She is also a member of the Weill Cornell Center for Lymphoma, a group that is internationally recognized for its leadership in the treatment of patients with lymphoma.

Lymphomas in children are typically fast growing, aggressive tumors that require high dose chemotherapy. Although many children are now cured of lymphoma, these children can suffer life-long health issues as a consequence of toxic chemotherapy treatment including heart failure, infertility, and second cancers. Dr. Roth is working on identifying novel targeted therapies that may be a less toxic treatment option than chemotherapy for children with lymphomas. In addition, these therapies may be able to rescue those patients with chemotherapy-resistant diseases. Specifically, Dr. Roth is testing two different targeted therapy drugs that may act as a “magic bullet,” targeting and destroying cancer cells without damaging healthy cells. These drugs may be particularly effective in Burkitt lymphoma, one of the most common lymphomas seen in children. Both of these drugs are already being studied in adults and Dr. Roth suspects that they might benefit children with Burkitt lymphoma all over the world.

Dr. Roth has been recognized for her work with awards from the National Institutes of Health (NIH), the Rally Foundation for Childhood Cancer, the Sass Foundation for Medical Research, and the Lymphoma Research Foundation.
Dr. David Lyden, along with a team of researchers, has demonstrated that DNA fragments packaged in tumor exosomes can be used to identify important genetic mutations in cancer. Since exosomes circulate in the blood, the discovery suggests that physicians could eventually use a blood test to detect tumors and to monitor patients’ response to treatments.

The findings show that exosomes contain double-stranded DNA, and this double-stranded DNA represents the entire genome of the cancerous tumor from which it originated. The study demonstrated that DNA associated with exosomes reflects the mutational status of their donor cells. This finding suggests the highly promising translational potential for exosomes, which can be obtained from a simple blood test.

The researchers also plan to study how exosomes introduce their cancerous DNA into healthy cells to better understand how cancer spreads throughout the body. The finding builds on earlier work by Dr. Lyden and his team who were the first to show that exosomes released by tumors can transport cancer-promoting proteins through the bloodstream and negatively affect healthy cells at distant sites. Importantly, the researchers demonstrated that the protein cargo of cancer exosomes could predict cancer spread in patients with aggressive skin cancers.

This work has been done over the past five years in Dr. Lyden’s laboratory. Studies showed that DNA can be associated with exosomes in two forms: it can be contained within the exosome particles or it can be attached to the surface of exosomes, which may have distinct roles for cancer diagnosis.
HIGHLIGHTS FROM THE CLINIC

The Comprehensive Hemostasis and Thrombosis Center, led by Dr. Catherine McGuinn, Dr. W. Beau Mitchell and Dr. Nicole Kucine, is devoted to providing comprehensive, family-centered care to children and adolescents with hemophilia, other inherited bleeding disorders, blood clots, or those children at increased risk for clots (thrombophilia).

For patients with bleeding disorders, Dr. McGuinn and Dr. Mitchell are working to identify early diagnostic tools to ensure patients with an increased risk of bleeding can be identified accurately. Additionally, the hemophilia team is also actively participating in clinical trials testing new, longer-lasting factor concentrate replacements to improve the therapeutic options available for patients with hemophilia.

For the patients at increased risk of clotting, Dr. Kucine and the thrombosis team are participating in multi-center clinical trials to determine the most effective treatment for blood clots in children, as well as identifying strategies for the prevention of blood clots in children who are hospitalized.

IN 2014, THE PEDIATRIC DIVISION HAD OVER 8,000 PATIENT VISITS. THE BREAKDOWN OF DISEASES TREATED IS AS FOLLOWS:

- Platelet Disorder: 34%
- Cancer: 27%
- Thalassemia: 19%
- Sickle Cell Anemia: 12%
- Hemophilia: 4%
- Other: 4%
Dr. James Bussel is the Director of the Platelet Disorders Center, specializing in the pathophysiology of and improved treatments for thrombocytopenia, especially immune thrombocytopenic purpura (ITP). Dr. Bussel has been a leading member of the CCBF team for nearly 30 years.

The pioneering work of the Center has revolutionized the treatment of platelet disorders, providing patients around the world with the most advanced treatment options available. Through his work at the Center, Dr. Bussel developed the first successful non-invasive treatment for fetal alloimmune thrombocytopenia (AIT), a disorder that affects fetuses, in which maternal platelet antibodies cross the placenta causing marked reduction in thrombocytes and functions in the fetus, resulting in brain hemorrhages and high prenatal and perinatal morbidity and mortality.

Today, because of this advanced trans-placental medical treatment consisting of weekly intravenous administrations of immunoglobulins, at-risk fetuses are identified and treated in the womb to increase platelet counts in the fetus, thereby lowering the risk for hemorrhages and allowing for a healthy baby to be born whose thrombocytopenia will then disappear within weeks.

Dr. Bussel lectures throughout the world and has chaired the American Society of Hematology (ASH) ITP symposium for ten years. He is also the Primary Investigator of the Cornell-Columbia TMH Clinical Trial Network. Among his more than 200 publications are six first or second authored original articles in the New England Journal of Medicine.
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Director, Adolescent and Young Adult Lymphoma Program

SUJIT SHETH, MD
Chief, Division of Pediatric Hematology and Oncology
Co-Director, Thalassemia Program
Professor of Clinical Pediatrics
PATIENT POPULATION SNAPSHOT

INSURANCE
Each year, more than 500 patients receive consultations and treatment during more than 8,000 visits to the Pediatric Division. Of these patients, approximately one-third do not have adequate insurance to cover the costs of treatment. CCBF support ensures that all patients, regardless of the family’s insurance coverage or ability to pay, can still receive the life-saving treatment they desperately require.

ETHNIC BACKGROUND
Children of all racial, ethnic and income backgrounds are affected by childhood cancers and blood disorders. Thus, the Pediatric Division serves a diverse patient population of various socioeconomic means. CCBF is dedicated to addressing the persistent socioeconomic and racial disparities in health care delivery and treatments by providing comprehensive and equal care for all patients.

DIAGNOSIS
Due to breakthroughs in research, children with cancer and blood disorders are increasingly living longer lives than ever before. By focusing on providing individualized care, the Pediatric Division ensures that each patient receives the most advanced, comprehensive care possible for his or her diagnosis.
Payton was a shy, quiet child when she was first diagnosed with sickle cell disease. She was always reserved, always withdrawn, always fearful. She also always seemed uncomfortable, no matter the situation. This is because Payton’s sickle cell disease, a serious genetic illness that leads to countless complications throughout life, led her to feel extreme pain in her bones, a common side effect of sickle cell disease. By her third birthday, she already had eight admissions to the hospital for treatment of her pain, and this frequent pain was preventing her from meeting her potential. At that point, Payton’s mother and her doctors agreed it was time to start Payton on a medication called Hydroxyurea.

This treatment changed her life. Her mother sees it as a miracle. The world finally got to meet the real Payton. Payton was not actually a quiet, fearful child at all when we met her. Instead, she is a little girl who loves to laugh and smile, is happy and playful, and is taking dance classes. The real Payton could only shine as the pain lessened and her disease symptoms became minimal. Hydroxyurea helped save Payton from her chronic pain and allowed her true self to shine.

For young children to take Hydroxyurea, it needs to be specially formulated into a liquid medication. This can easily cost $100 per month out of pocket, and for many families this is simply unaffordable. There are numerous children, just like Payton, who would benefit from Hydroxyurea, but the cost prohibits them from getting it. The Children’s Cancer & Blood Foundation helps ensure that no patient goes without life-saving treatments or medications.
Darren has been a repeat patient of CCBF's for several years now, despite being only nine years old. He has a chronic blood disease, which necessitates him receiving periodic transfusions. Over his last few visits at the Pediatric Division, he has been having trouble getting his IVs placed, which has been a painful process for him as he struggles to cope with his lengthy and painful treatments and his illness.

In order to help patients, like Darren, and their families cope and navigate the often stressful and emotional challenges of a child's illness, CCBF sponsors a full-time Child Life Specialist for the Pediatric Division. The Child Life Specialist provides children with age-appropriate preparation for medical procedures, pain management and coping strategies, and play and self-expression activities. She also provides information, support, and guidance to parents, siblings, and other family members. The CCBF Child Life Specialist worked with Darren to figure out how to help him make his treatment more comfortable.

While they were playing with a building block set and discussing the obstacle of placing the IVs, Darren noticed that his veins popped out a bit while he was rotating screws into the blocks. The Child Life Specialist helped Darren to understand that if he used this technique to make his veins show themselves more during IV transfusions, the process might be easier and less painful.

Thanks to the hands-on support provided by the CCBF Child Life Specialist, Darren was able to find a solution for his painful treatments. Now, he is much happier and less apprehensive of treatments and he comes ready with his technique to help make his veins more prominent in preparation for IV placements.
On November 18th, 2014, CCBF hosted its annual “Breakthrough Ball” benefit gala in the luxurious Grand Ballroom of the Plaza Hotel. A stunning success, the extraordinary evening raised over $1 million to provide much needed medication, chemotherapy, and other life-saving treatments to children with cancer and blood disorders treated at the Pediatric Division.

CCBF bestowed the prestigious “Breakthrough Spirit” award on Vincent Tese, Chairman of FCB Financial Holdings, for his unwavering commitment to making a genuine difference in the lives of others through his philanthropic endeavors. As a well-respected Board member of NewYork-Presbyterian Hospital, Madison Square Garden, and Cablevision, and through his long-time support of CCBF, Vincent has worked tirelessly to improve the lives of children in need.

The live and silent auctions were a huge success, featuring Super Bowl XLIX tickets, an unforgettable piece of art by Damien Hirst, and a Hamptons Golf Package with tee-offs at some of the most exclusive and highly-rated courses, among many other luxury items.

Topping off the magical evening was a special performance by Alumni from Broadway’s Jersey Boys, who performed an array of hits, including Pharrell Williams’s “Happy,” Michael Jackson’s “The Way You Make Me Feel,” and Amy Winehouse’s “Valerie.” The success of the event would not have been possible without the support of all of CCBF’s donors and sponsors.
EVENT HIGHLIGHTS

ART AUCTION
CCBF hosted its annual Contemporary Art Auction at Phillips auction house on June 18th, 2014. The cocktail reception and silent auction featured 33 works by top contemporary artists including Candida Höfer, Richard Pettibone, Richard Serra, Massimo Vitali, and Richard Wathen, among others. Attendees enjoyed after-hour access to Phillips, browsed the beautiful collection of paintings, photographs and sculptures, and bid on their favorite works. The event was a great success, raising over $100,000 to support CCBF’s work. CCBF would like to thank all the artists and attendees for their support and look forward to many more successful art auctions in years to come.

NEW YORK CITY MARATHON
CCBF was proud to be a charity partner of the iconic TCS New York City Marathon on November 2nd, 2014. Runners who joined Team CCBF added meaning to their miles by raising funds for children with cancer and blood disorders. CCBF would like to congratulate all of its runners on completing the 26.2 mile journey and making a difference in the lives of so many children. This year, Team CCBF surpassed its goal and raised over $30,000! CCBF would also like to thank Brooklyn Running Co. for donating jerseys for Team CCBF. The wonderful jerseys were a huge hit with CCBF’s runners, and helped spread the word to all those who were watching.
FINANCIAL RESULTS

STATEMENT OF REVENUES AND EXPENSES
Year ending December 31, 2013

Revenue
Public Support .................. $590,176
Special Events .................. $786,440
Investment Income .......... $308,186
Total Revenue ................. $1,684,802

EXPENSES
Program Services .............. $985,003
Management .................. $375,747
Fundraising .................... $78,271
Total Expenses ............... $1,439,021

BALANCE SHEET
Year ending December 31, 2013

Assets
Cash .............................. $393,379
Investments, at market . $3,763,105
Pledges receivable ........ $306,750
Interest/dividend receivable . $13,282
Donated art .................... $98,624
Total Assets ................ $4,575,140

LIABILITIES & NET ASSETS
Accounts payable ............. $227,617
Total Liabilities & Net Assets $4,347,523

EXPENSE DISTRIBUTION

- Program Services: 68%
- Management: 6%
- Fundraising: 26%
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