



Children's National™

IMPACT REPORT SUBMITTED TO BEN'S GIFT, INC.

Sparking Lifesaving Scientific Breakthroughs for Children with Cancer

Despite advancements in the field of pediatric oncology, there are still far too many children like Ben whose lives are cut tragically short. Even stem cell transplant—often the option of last resort for children whose cancer repeatedly returns—can only save about 50 percent of these high risk kids. New scientific breakthroughs are desperately needed to save lives and offer new hope to critically ill children and their families. With its \$73,000 grant awarded to Children's National in 2013, Ben's Run is making a difference, sparking scientific breakthroughs to "ben-a-fit" vulnerable children in Washington, DC and around the world.

ACCELERATING GROUND-BREAKING RESEARCH

The timely contribution provided by Ben's Run in 2013 helped advance the leading-edge work of promising young physician-scientist Dr. Kirsten Williams, who is focused on finding innovative solutions for the most difficult chemotherapy-resistant leukemia cases. The funding generated by your dedicated donors and volunteers enabled Dr. Williams to develop a protocol to evaluate the efficacy of two promising drugs for the treatment of relapse of leukemia after hematopoietic stem cell transplantation. Like Ben, children undertaking this potentially life-saving procedure face many serious risks. Relapse is often identified late, due to infrequent marrow evaluations after transplant. Immune system therapies can lead to life-threatening Graft-versus-Host-Disease (GvHD). Intensive chemotherapy is toxic to children whose health is already severely compromised.

Partnering with Leukemia Specialist Dr. Reuven Schore, Dr. Williams designed and developed a protocol to treat leukemia and mitigate these adverse, sometimes life-threatening, effects. Her goal is to identify an innovative approach that permits graft-versus-leukemia effects, while constraining GvHD. The proposed trial will involve monitoring the peripheral blood for early detection of recurrent leukemia and confirming this with a standard bone marrow procedure. Following confirmation of disease, we will initiate therapy with two medications—Pravastatin and Bortezomib—both previously used in the post-transplant population and well-tolerated. Both have been shown to aid in leukemia eradication in mice and also alter the immune system to diminish the risk that the immune response will be diverted to attack the healthy organs.

Thanks to support from Ben's Run, the trial and consents and assent are now complete and have been approved by both Millennium (the company that will provide the drugs) and the Institutional Review Board at Children's National. Dr. Williams and her team currently await FDA approval and hope to begin enrolling children into the trial in early 2014. This will be the only trial of its kind specifically focused on relapsed acute myeloid leukemia after transplant. This trial will include translational studies to explore the lymphocyte profiles at the time of relapse and after therapy, in attempt to understand the cells that contribute to remission versus those that enable GvHD.



The investment provided by Ben's Run accelerated this cutting-edge work and is a testament to the indomitable spirit of the race's beloved namesake. Like Ben, the Children's National team "didn't come here to lose," and with your help, we will WIN the fight against childhood cancer.

THE RACE TO A CURE CONTINUES SWIFTLY

Sustained support generated by Ben's Run in 2014 is vital to our continuing work to advance lifesaving treatments for children. In pursuit of this worthy goal, Children's National has recruited the best and brightest minds. Our world-class clinicians, investigators, and basic scientists are making great strides toward improving outcomes in patients with cancer, but much remains to be done.

Ben's Run launched this critical research, and continues to invest in the project for completion. Following regulatory approval in early 2014, children will begin to be enrolled in this study. Proceeds from the 2014 Ben's Run will fund this innovative research to improve outcomes from bone marrow transplants for children with high risk leukemia. If the study is successful in treating leukemia, this will open the door for investigation into care for other solid tumors in the future. Investments in drug development and personalized cell therapy are offering hope to families whose children have relapsed and must seek last resort options to overcome childhood cancer. All findings will be reported to the Children's Oncology Group and within the scientific community to advance the basic knowledge of the field.

A key strategy to achieve our shared goal is to facilitate a collaborative environment that promotes the rapid development of translational approaches. By bringing together leading researchers like Drs. Williams and Schore at our main campus, where we also have the patient population necessary to conduct on site clinical trials, we can accelerate projects "from bench to bedside" in ways few other institutions can. We rely on generous financial support, like that provided through Ben's Run, to spark new innovations and bolster the groundbreaking experimental research underway.

The Children's Oncology Group is the world's largest organization devoted exclusively to childhood and adolescent cancer research. The Group unites more than 8,000 experts in childhood cancer at more than 200 leading children's hospitals, universities, and cancer centers across North America, Australia, New Zealand, and Europe in the fight against childhood cancer.

Philanthropic investments from visionary donors are what catalyze promising new research projects—accelerating them to the point where the National Institutes of Health and other investors will get involved.

With the support of Ben's Run we are now able to detect leukemia cells earlier. We are now able to scan high-risk patients and intervene with personalized therapies sooner. This is truly life-saving research and we could not have done it without your support.



ABOUT KIRSTEN WILLIAMS, MD

An invaluable member of the Blood and Marrow Transplantation team, Dr. Williams attended medical school at Georgetown University and undertook her pediatric residency at Rainbow Babies and Children's Hospital in Cleveland. She then pursued a pediatric hematology/oncology fellowship in the combined Johns Hopkins Hospital/NIH program. Dr. Williams continued at the NIH as a clinical research fellow until 2009 and as an Assistant Clinical Investigator until 2012. Her work involves clinical trials enhancing immune reconstitution post allo-transplant, as well as clinical trials for treatment of lung GvHD. She is the recipient of the 2012 Tucker Fellowship Award as well as the 2009 Bench to Bedside Award from NIH.