



Children's National®

December 2017



# The Impact of Ben's Run

AT CHILDREN'S NATIONAL HOSPITAL



For nearly 150 years, Children's National has been committed to providing exceptional, compassionate care to every child, no matter what. Ben's Run has been a committed partner with us to deliver on that promise. Through your continued support, you help propel groundbreaking research, pioneer unparalleled innovation, and bring hope to families when they need it most. We are proud to share with you how your generosity makes an immediate and lasting impact advancing the research of Kirsten Williams, MD, and improving care and outcomes for our patients with acute leukemia. Only through the power of philanthropy—and partnerships with donors like you—can we realize our boundless potential as the leading pediatric hospital for kids throughout the Washington metropolitan area, across the country, and around the world.



In as little as five days after a transplant, non-invasive FLT scans can reveal new cell growth as well as growth patterns throughout the entire body.

## A Picture, Not A Puncture

A bone marrow transplant is nothing short of a traumatic experience, involving intense chemotherapy, weeks of hospitalization, frequent testing, and sometimes long periods of isolation. In addition, post-transplant patients, their families, and the care team must wait—sometimes for as many as 30 days—to see if the procedure was successful through a painful bone marrow biopsy.

Thanks to support from Ben’s Run, Dr. Williams developed a painless methodology to detect transplanted cell growth anywhere in the body. The approach uses <sup>18</sup>Fluorothymidine, or FLT, previously used to study lymphoma and leukemia progression in adults; Dr. Williams is the first to translate this protocol for pediatrics. Results are as fast and painless as a CT scan and reveal where in the body newly transplanted cells grow, offering incredible insights into cellular activity at the most critical post-transplant stage.

This past year, Dr. Williams made impressive strides in FLT research. She obtained an Investigational New Drug (IND) Application from the Food and Drug Administration (FDA), a comprehensive submission that enables her to begin formally using FLT to treat patients at Children’s and marks the first step in bringing a new protocol to market. With this IND, Dr. Williams will study the methodology of using FLT to see if acute leukemia responds to RESOLVE, Dr. Williams’ promising new project that uses a patient’s own immune cells to fight back against resilient or relapsed leukemia. Importantly, the IND can also springboard additional applications to study future protocols which will help to rapidly advance research on FLT in pediatric applications.

Dr. Williams’ results will be published in *The Lancet Haematology*, acknowledging Ben’s Run at the start and conclusion of the paper. She is also planning a second paper on the resulting analysis on how well her FLT imaging protocol detects leukemia.

## Strengthen With RESOLVE

RESOLVE, a promising new treatment Dr. Williams and Dr. Catherine Bollard developed with Ben's Run's support, involves growing targeted cancer-fighting immune cells, or T-cells, that are trained to recognize and attack leukemia cells. Before RESOLVE, if relapse was detected or predicted, there was little care teams could do clinically, especially after bone marrow transplant fails. Thanks to Ben's Run, Dr. Williams and her team can now infuse these power-hitters at regular intervals to eradicate low levels of leukemia and offer many young patients and families something previously lost: hope.

While the investigation is still in its infancy, the results of RESOLVE are nonetheless impressive. Of the 16 patients enrolled, 75% responded to the treatment, meaning that RESOLVE halted or slowed leukemia growth. One of these patients has been cancer-free for more than a year, the longest period since his relapse after BMT. With such impressive results over the past years, this first phase of the investigation is nearing its conclusion, and Dr. Williams is actively sharing her progress with the broader scientific community. In 2017, she presented at four national and international meetings—acknowledging Ben's Run's support.

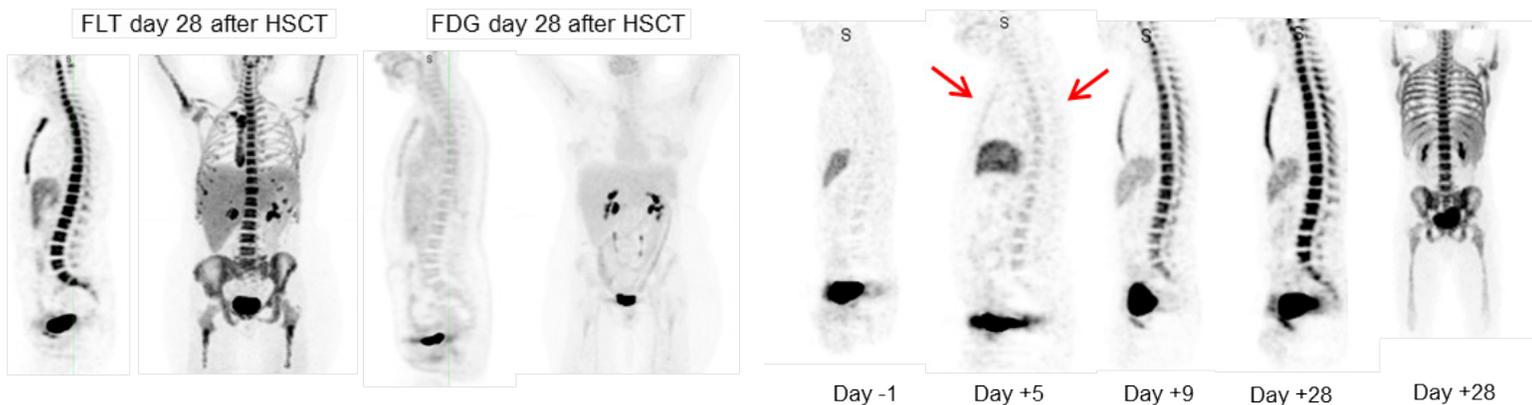


Image 1: Comparing FLT to non-FLT scans

Image 2: FLT reveals cell growth progression

## FLT In Action

In Image 1 (left), the two left-most body scans are with FLT showing a patient 28 days after bone marrow transplant. The new cells appear within the bones in black as well as in organs like the liver, kidney, and bladder. The right scans in Image 1 show the same patient without using FLT. The bones appear in a shadowy gray, a stark contrast to the deep black resolution in FLT scans. Because of the support of Ben's Run, Dr. Williams and her team can show patients where in their bodies cells are growing, without a painful or invasive test.

Image 2 (right) demonstrates how Dr. Williams' research advances scientific knowledge. Using FLT, she is able to capture the progression of cell growth (shown in black) within bone marrow. This information has the potential to inform further research into bone marrow transplantation and other fields.



Thanks to partners like Ben's Run, Dr. Williams is not just helping more kids recover; she is helping them recover in a nicer way.

## Blood Work, Not Guess Work

In prior years, bolstered by Ben's Run's support, Dr. Williams developed a groundbreaking blood test that detects low levels of leukemia without an invasive bone marrow biopsy. By testing the blood, clinicians can learn of relapse sooner and, ideally, before the disease progresses or advances to other parts of the body.

This past year, Dr. Williams continued to push the boundaries of blood testing using a new polymerase chain reaction (PCR) method. PCR is a technique commonly used in molecular genetics; it amplifies DNA or RNA sections to permit analysis even on very small samples. This is important in cancer research because there is much to learn about leukemia and how our bodies fight cancer cells, but researchers had been previously inhibited because existing methods require unfeasible amounts of blood to analyze.

Using this new PCR, Dr. Williams measures the levels of three distinct pieces of tumor RNA, "tumor bits," she calls them, which are present in blood even after leukemia is no longer present in bone marrow. These tumor bits spike when relapse is imminent or in its early phases, so this new blood analysis could help clinicians predict relapse and potentially intervene. In comparison, if Dr. Williams were to attempt to do this type of analysis using traditional PCR, she would require the entire research blood supply of Children's National to test just two of RNA segments. With new PCR, she can study three segments with a fraction of material.

“ With this new testing, clinicians are not just getting information in a less painful way; they can now get more—and better—information that can actually change health outcomes. ”

—Kirsten Williams, MD



Funding research is critically important. It can mean more than just novel investigations; it can mean more birthdays to celebrate.

## More Birthdays to Celebrate

A child is not just a small adult. That's why specialized pediatric care—and pediatric research—is so important. However, the funding landscape is highly competitive and dominated by adult medicine. Philanthropy from partners like you provide critical resources to spur groundbreaking research and support proof-of-concept explorations.

Importantly, your generosity provides more than just research funds. Through your contribution, you are helping to provide better care and better outcomes for patients impacted by leukemia. By enabling Dr. Williams' research, you are helping more kids not just grow up, but grow up stronger. More patients now have the opportunity to recover, survive, and have more birthdays, thanks to you. That's something to celebrate.





## Our Sincere Gratitude

Your generosity helps us pursue bold approaches, explore new ideas, and provide the best care for kids with leukemia. Thanks to your sustained support, Dr. Kirsten Williams and her team can continue to make meaningful progress and transform treatment options and outcomes for even the most complex cases.

We are proud to partner with you on this important work and are truly grateful for your support and commitment to Children's National.

On behalf of all who benefit, thank you.



“ We couldn't do this research without Ben's Run. These new therapies would not have been possible as it is hard to seek and secure grants for brand new approaches. Thanks to Ben's Run, we are pursuing cutting-edge therapies and ways to make transplant better for kids like Ben. ”

– Kirsten Williams, MD



**Children's National**

Children's Hospital Foundation  
ChildrensNational.org/giving 301-565-8500

 [Facebook.com/childrensnational](https://www.facebook.com/childrensnational)

 [Twitter.com/childrenshealth](https://twitter.com/childrenshealth)

 [Instagram.com/childrensnational](https://www.instagram.com/childrensnational)

 [YouTube.com/childrensnationalmed](https://www.youtube.com/childrensnationalmed)