

Leukemia



Cancer survivors and twins Bailey and Lily Dove are now leading normal and inspire the search for targeted cancer treatments.

The Dove Twins' Story

Twins Lily and Bailey Dove were diagnosed with leukemia just 18 months apart. Their strong bond allowed them to help each other through their treatment, which was challenging and involved multiple blood and platelet transfusions. Thanks to treatment at Children's Wisconsin through Versiti's Medical Sciences Institute, they are now both cancer-free and able to live normal lives. It's patients like Lily and Bailey who inspire the research that we do at VBRI, to bring treatments and cures to cancer patients so they can live long and healthy lives.

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It felt amazing to finally be able to move on. To be physically healthy again, it felt amazing.

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Bailey Dove
Leukemia Patient

The Search for a Targeted Cure

Leukemia is a complex and often aggressive cancer of the blood and bone marrow that affects over 500,000 people in the United States, with over 60,000 new cases diagnosed each year.

Although treatment for leukemia has advanced in recent years, it still causes 20-25,000 deaths per year in the US alone, with only two-thirds of patients surviving longer than 5 years from their diagnosis, making this devastating disease a priority for VBRI researchers. The survival rate is even lower for acute myeloid leukemia (AML), with less than one-third of patients surviving more than 5 years after diagnosis. Current treatments also lead to toxic side effects because they can also damage healthy cells as well as cancer cells.

A hope for leukemia patients? **Targeted therapies that work differently from standard treatments, selectively attacking cancer cells.** These therapies are based on the genetics or molecular drivers of an individual's leukemia. VBRI researchers are working to uncover these drivers, providing growing hope for breakthroughs that can transform care and restore quality of life for patients and their families. Targeted and molecular therapeutics retrain the body's existing defense systems to target cancer, which could lead to far fewer side effects and better outcomes for patients.

Global Collaborations that Lead to Cures

At VBRI, our researchers are harnessing cutting-edge techniques to study the foundational basis of cancers, including leukemia. But they can't do this alone—our researchers collaborate with leading scientists and physicians worldwide. **It takes a global community to research cures.**



The Pulikkan Lab investigates both targeted leukemia treatments and techniques that could reduce treatment resistance.

Targeted Therapies that Provide Real Hope

John Pulikkan, PhD is developing targeted therapies for, and investigating the genetic and molecular mechanisms of, AML. Using advanced humanized mouse models, **his team is testing and validating potential new treatments for two high-risk AML subtypes.**

The first subtype is caused by a mutation to the CEBPA gene. Dr. Pulikkan and his team have identified a promising treatment target for this AML subtype based on inhibiting a protein that regulates gene expression. The team is also pursuing treatments for the second subtype, caused by a mutation called inversion 16, which is one of the most frequent causes of AML. They have developed a targeted inhibitor that has been patented, licensed and is moving toward clinical trials.

Importantly, the lab isn't only developing new treatments; they are also trying to get ahead of treatment resistance, when a patient who initially responds well to drug treatment becomes less responsive to the treatment over time, is a frequent challenge in the treatment of leukemia.

This work is the result of global collaborative efforts. The lab's research is based on samples from patients at hospitals worldwide, and collaborations with pharmaceutical companies and clinicians make it possible to test these potential treatments quickly. Through this work, Dr. Pulikkan is advancing innovative therapies that could significantly improve survival and outcomes, not only for patients with leukemia, but all cancer patients.

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One drawback of targeted therapy is that patients will develop resistance over time. We are running projects to know what the potential mechanisms of resistance are so we can prepare in advance.
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John Pulikkan, PhD
Associate Investigator

Advancing Cures for Leukemia and Beyond

At VBRI we are uniquely focused on hematologic diseases, developing targeted therapies that will not only improve the lives of patients with leukemia, but all cancer patients. **We research cures, bringing the science beyond the lab and into the lives of patients.**