Precision Hematology

VBRI's development of targeted therapeutic approaches uses a patient's genetic makeup to improve standards of care.

Targeting Treatment and Personalizing Diagnosis

Alan Mast, MD, PhD knows first-hand the importance of research into blood disorders. When his daughter developed a blood clot in her brain, he found himself reflecting on how far research has brought us. **"50 years ago, she would have only had a 70% chance of survival. 30 years ago, she would have been in the hospital for two weeks, receiving intravenous solutions. Now she's able to be treated as an outpatient with a pill."** She was able to resume her normal life and go back to work just days later.

Dr. Mast attributes much of the progress that has been made in bleeding and clotting disorders to studying the basic mechanisms of disease, "That's what attracted me to doing research. I wanted to understand how your body works at a level that you can't see." Dr. Mast's lab investigates Tissue Factor Pathway Inhibitor (TFPI), a protein that prevents the formation of blood clots. By understanding how TFPI interacts with surrounding tissues such as blood vessels and platelets, their work has not only deepened understanding of bleeding disorders like hemophilia, but also yielded some unexpected results. Their research is now leveraging a precision medicine approach to diagnosis–allowing for faster, more accurate diagnosis of vascular risks and abnormalities.

> *"Precision hematology can go beyond blood—it may unlock answers to neurodevelopmental disorders we've never understood before."*

Alan Mast, MD, PhD

Precision. It's in Our Blood.

Diseases of the blood affect millions, ranging from blood clots to hemophilia and from cancer to rare diseases. Blood diseases are inherently elusive and complex, and patients are too often treated with ineffective or even dangerous "one-size-fits-all" approaches. At VBRI, our search for cures includes **precision hematology, the application of personalized, targeted approaches to the diagnosis, treatment, and management of diseases of the blood.** VBRI researchers seek to improve diagnosis and care by tailoring approaches to an individual's unique genetic, molecular, or clinical information to deliver the most effective therapies with the fewest side effects.

One area where precision hematology holds immense promise is **hemophilia, a genetic disorder caused by a missing protein that is responsible for controlling bleeding.** Although rare, the disease is serious and can cause internal bleeding and organ damage. Precision hematology offers new hope for patients who rely on multiple infusions and drug treatments to manage their disease.

Uncovering What the Blood Can Tell Us

VBRI investigators use the power of foundational research to fully understand blood disorders, and apply this understanding to an even broader range of disorders of the brain and body. **Our specialization is the key to unlocking new discoveries,** diagnostic approaches, and treatments.

Putting Patient Care First

Lynn Malec, MD and her team work at the intersection of precision hematology and patient care at Versiti's Comprehensive Center for Bleeding Disorders. Over the past several years, they have been building a gene therapy program for patients living with hemophilia along with processes necessary to administer treatments in a sterile environment. Her team also offers knowledge-building sessions on gene therapy with potential patients, helping patients stay informed on cutting-edge medicine and new potential treatments.

In 2025, Versiti became one of the first institutions in the US to administer a life-changing therapy for hemophilia B. The FDA-approved drug, HEMGENIX, delivers a functioning factor IX gene copy to the liver to start producing the blood clotting factor IX, which is lacking in patients with hemophilia B. The patient, Cody Gunst, showed a response to gene therapy and has been able to discontinue his prior preventative hemophilia medications.

"At Versiti, we are constantly looking for new ways to improve the way we treat our patients, and we want our patients to have access to different options."

Lynn Malec, MD
Senior Medical Director,
Associate Investigator

Cody's Story

Cody Gunst suffered from hemophilia B his whole life. Side effects from his disease presented throughout his body, ultimately resulting in advanced arthritis and multiple ankle surgeries. Dr. Malec consulted with Cody about the benefits of changing his treatment plan and leveraging a personalized medicine approach, and he became one of the first patients in the US to receive the newest gene therapy to treat hemophilia. Although the treatment didn't erase his hemophilia, Cody's risk of bleeding is dramatically reduced, and he no longer needs regular intravenous infusions. VBRI's unique combination of patient care and cutting-edge science has given Cody, and patients like him, new hope.

"Now any time I accidentally bang my knee or hit my arm, I don't have to worry about it causing a bleed. It used to be very stressful. Now my joints are protected, and I don't have to always carry my pharmacology product with me or cut my plans short to run home to do an infusion."

- Cody Gunst, Hemophilia B Patient

Cody Gunst, pictured alongside his Versiti medical team, was one of the first patients in the United States to receive HEMGENIX, a groundbreaking gene therapy for severe hemophilia B.

Working Directly With Patients to Bring Research to Life

At VBRI, our research teams work directly with patients to bring discoveries from the lab into patient's lives, safely and rapidly. **As a community, we research cures.**