

Innovations in Research

New treatment for hemophilia A changes the game

Though hemophilia may be well-known as a bleeding disorder, the condition itself is rare, occurring in approximately 1 in 5,617 live male births. This lifelong disease affects the blood's ability to clot and can lead to spontaneous bleeding, joint damage and chronic pain. Hemophilia's level of severity is based on the amount of clotting factor in the blood, and many patients receive multiple-times-weekly factor replacement therapy to replace the factor protein their blood lacks.

In February, the U.S. Food and Drug Administration approved a new, once-weekly therapy for treatment of hemophilia A. The new drug, Altuviiro, is a factor VIII replacement therapy that is intended to be used as an on-demand treatment to control bleeding episodes and as a way to manage bleeding during surgery.

"This approval marks an important clinical advancement for the hemophilia community, because we have an option that can achieve higher levels of factor activity with a single, simplified weekly dose," said Lynn Malec, MD, MSc, Versiti Comprehensive Center for Bleeding Disorders medical director and Versiti Blood Research Institute associate investigator in a BioPharma reporter article.



Lynn Malec, MD, MSc
Medical Director,
Versiti Comprehensive
Center for Bleeding
Disorders

Noelle Ott

When Noelle was 24 weeks pregnant, she began experiencing pain throughout her body and her platelet count dropped rapidly. Doctors diagnosed her with HELLP syndrome, a rare pregnancy complication that involves the breakdown of red blood cells, low platelet counts and elevated liver enzymes. Noelle delivered her son early, and both received blood, platelet and plasma transfusions to help them survive.

But the ordeal didn't stop there. Doctors determined that Noelle wasn't suffering from HELLP syndrome after all, but rather thrombotic thrombocytopenic purpura (TTP), a rare blood disorder where clots form in small blood vessels throughout the body, causing stroke and organ failure. Over the course of three months, Noelle received countless units of blood, platelets and plasma to help her recover. She is grateful for the blood donors whose gifts helped keep her and her son alive, and for

researchers who are looking for better treatments and cures for diseases like HELLP and TTP. "It's absolute life or death," she says. "I'm so thankful to have my life back!"

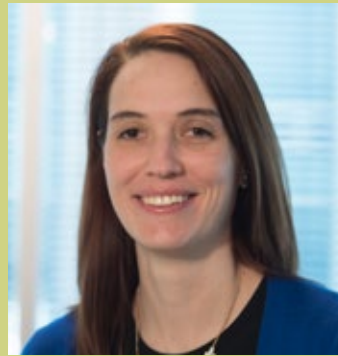


May is Women's Health Month

When Associate Medical Director of Hematology Juliana Perez Botero, MD, introduces herself to patients, she describes herself as a blood detective who collects clues about her patients to help solve a case. "A lot of what we do consists of gathering information from many sources and putting it together for each individual patient," she said. "Your patients aren't usually only yours; it's rare that we have a patient who has a hematology problem that doesn't affect anything else. Because of that, I see my job as helping others do their work, and making sure patients safely get whatever treatment they need."

One of the multidisciplinary treatment approaches Dr. Perez Botero is involved with is a combined hematology/obstetrics clinic with Jennifer McIntosh, DO, MS, at Froedtert Hospital. Pregnancies for women with pre-existing hematologic conditions can be complicated, with a number of pregnancy-exclusive disorders and hematologic manifestations. Inevitably, these women have questions about their pregnancies that also impact the care plans for their hematologic disorders. Left unaddressed, these women may experience severe complications during pregnancy and childbirth.

The clinic aims to provide comprehensive care for these patients in a collaborative environment. Drs. Perez



Juliana Perez Botero, MD

Botero and McIntosh meet with patients together to help formulate the best treatment plan possible. "Even when the final treatment decision is at the time of delivery, it helps women know what to expect," Dr. Perez Botero said.

Drs. Perez Botero and McIntosh have identified gaps in the clinical care of this specific population and are establishing research collaborations to address them. "Our goal is to partner with people who have knowledge and skills that complement our own and to bring practical knowledge to the clinical sphere," Dr. Perez Botero said. "While it's great to have all this knowledge, if we don't turn it into something useful, who's benefitting from it? It needs to mean something to people."

New and Noteworthy

Karin Hoffmeister, Hauske Family Endowed Chair in Glycobiology, named a Noteworthy Woman in STEM by BizTimes Media

Congratulations to Versiti Blood Research Institute Deputy Director Karin Hoffmeister, MD, Hauske Family chair in Glycobiology, who was recently named a Notable Woman in STEM by BizTimes Media. Dr. Hoffmeister's research focuses on glycans, or sugars, which are a basic building block of life. Better understanding of glycans and their role in human health may help researchers develop personalized therapies for patients with blood diseases. "The time is now to approach the complexity of sugars using

novel 'omics' tools and artificial intelligence to unravel the sugar-code," Dr. Hoffmeister said.



*Karin Hoffmeister, MD
Deputy Director VBRI*

Jieqing Zhu, PhD, published in prestigious scientific journal Cell

Senior Investigator Jieqing Zhu, PhD, and a team of researchers from Harvard University Medical School were recently published in the prestigious scientific journal *Cell* for their work studying cell receptors and how they can be better used to develop new therapies for bleeding and clotting disorders, cancer, autoimmune diseases, and many more.

The team set out to better understand why some of the drugs that target cell receptors—which have been long established as treatment targets—sometimes initiate unwanted, potentially harmful physiological responses in patients. Researchers determined that stabilizing a key water molecule could prevent these cell receptors from sending unwanted signals, marking a key turning point in the future of drug development for diseases like cancer, multiple sclerosis and osteoporosis.

Community Beacon of Hope: Roger Abbott Foundation

In 2011, Roger Abbott was diagnosed with myelodysplastic syndrome (MDS), a rare form of leukemia. He received weekly blood and platelet transfusions for more than five years, giving him more time with his family before passing away.

There is no cure for MDS, and its



exact causes are unknown. But with a better understanding of the immune system and how genetic mutations contribute to the disease, researchers may be able to develop new treatments—and even a cure—for MDS. The Roger Abbott Foundation was established to fund and support MDS research, giving hope to future generations.

Upcoming Events

Women Rocking Research

This event was created by an enthusiastic group of Versiti Blood Research Institute Foundation and Junior Advisory Board members to celebrate Versiti's remarkable women researchers. Speakers for the inaugural event include Senior Investigator Bonnie Dittel, PhD, and Investigator Renren Wen, PhD, who will talk about the immune system's essential role in human health.

The Immune System: Your Body's Defense System

Date: Tuesday, May 23, 2023

Time: 5-8 p.m.

Location: Milwaukee Athletic Club, 758 N. Broadway, Milwaukee, WI 53202

Tickets: \$20; includes cocktails and appetizers



Research & Roses

Date: Wednesday, June 28, 2023

Time: 5:30-8:00 p.m.

Location: Versiti Blood Research Institute

Speaker: Senior Investigator Peter Newman, PhD



Imagine Gala

Date: Friday, Sept. 8, 2023

Time: 5:30-10:30 p.m.

Location: The Wisconsin Club, 900 W. Wisconsin Ave., Milwaukee, WI 53233

Virginia Brooks Jefferson Award Recipient: Jackie Fredrick



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BRIghtLights

BRIghtLights is a quarterly newsletter published by the Versiti Blood Research Institute Foundation. Interested in learning more? Please contact the Foundation office: 414-937-6799.