TRANSLATING HEMATOLOGIC ONCOLOGY DISCOVERIES INTO PRACTICE

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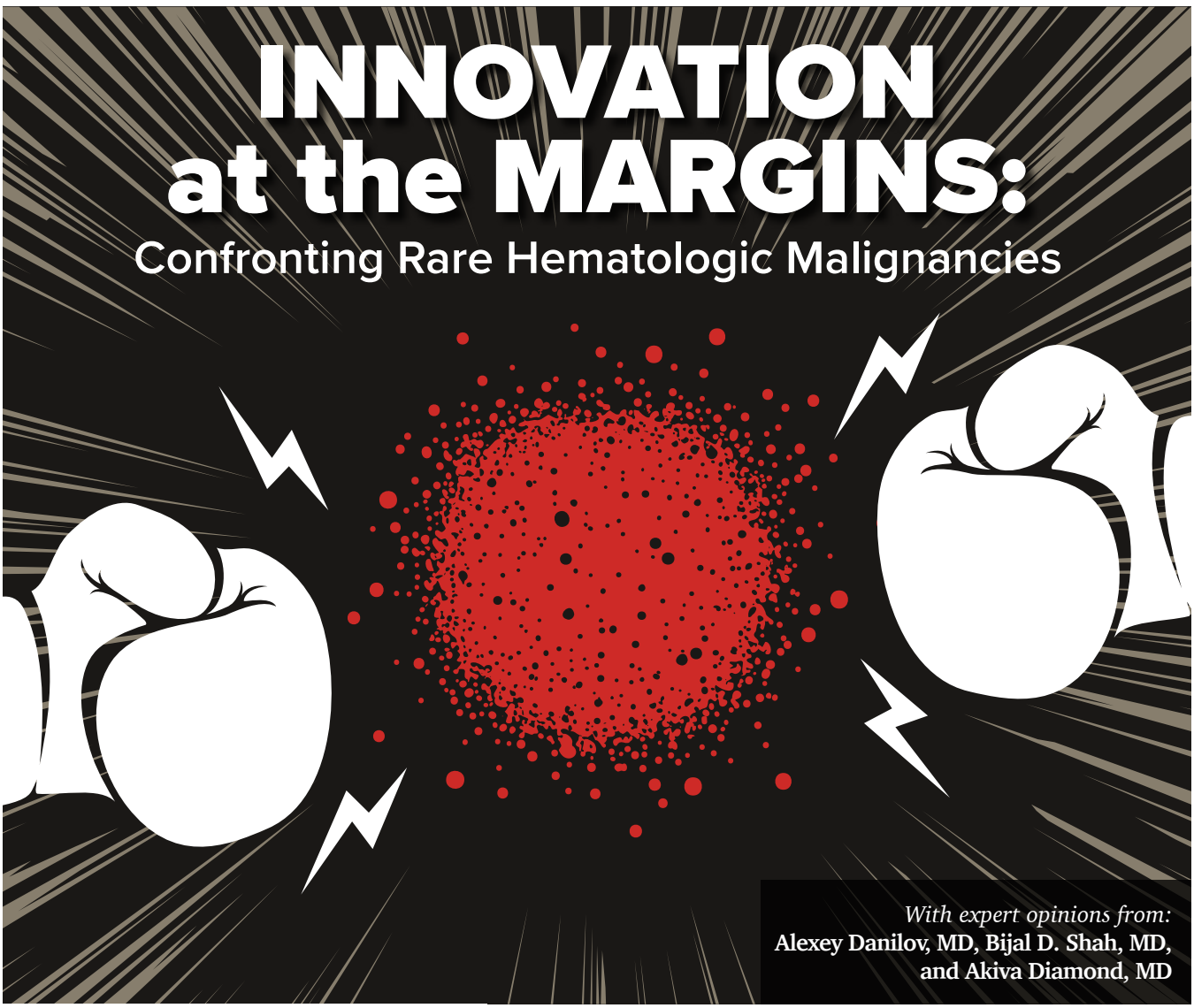
Confronting Rare Hematologic Malignancies



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MAIL TO:

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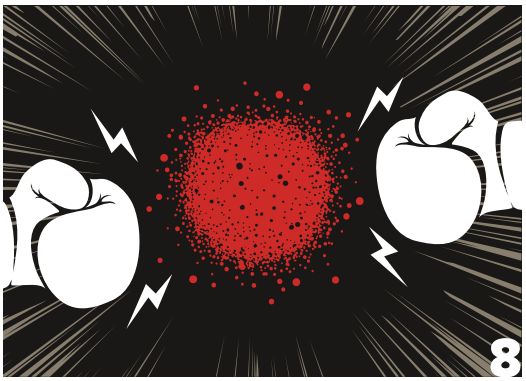

Innovation at the Margins:
Confronting Rare Hematologic Malignancies

Although rare diseases like hematologic malignancies can be difficult to diagnose and treat, therapeutic breakthroughs continue to push the field forward.

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GET TO KNOW
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Dr. Shernan Holtan talks strength training, bass playing, her passion for improving access to hematopoietic stem cell transplant, and how she integrates her personal hobbies into her professional career.

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Efficacy Is No Longer Enough: Why Safety, Feasibility, and Cost Now Matter

Tycel Phillips, MD
Associate Editor

We have reached an interesting inflection point in mantle cell lymphoma (MCL). For years, our field strove to develop treatments that could provide a "magic bullet" to target this difficult-to-treat disease. We have made great progress; today, we have four to five frontline regimens that have demonstrated fairly equivalent, but impressive, short-term response rates and duration of response. But this success has created a new challenge: when multiple regimens work similarly well, how do we choose between them?

The answer used to be simple: pick the most effective option. Today, that decision has become far more complex. Cost remains a major issue globally, and several countries have withdrawn or do not offer certain highly effective treatments to patients because regulatory agencies do not consider them cost effective. This issue remains a major conversation even here in the United States, as our healthcare systems grapple with the financial sustainability of new therapies.

"On the surface, BTKis seem like an unqualified win. But we are learning that short-term wins do not guarantee long-term success."

Consider what is happening with Bruton's tyrosine kinase inhibitor (BTKi)–based regimens such as TRIANGLE and ECHO. These treatments match or exceed the efficacy of our traditional chemotherapy plus autologous stem cell transplantation (ASCT), and they do so without the morbidity concerns that have made us want to move away from transplant for decades. Although the efficacy of BTKis in MCL has been noted for more than a decade, access to these drugs remains limited globally.

Another practical consideration with new effective therapies becoming available is sequencing. On the surface, BTKis seem like an unqualified win. But we are learning that short-term wins do not guarantee long-term success. When patients receive upfront BTKi-based therapy, whether it consists of the 2-year TRIANGLE regimen or the 2-year BOVen approach, we simply do not have long-term data on what happens at relapse, whereas we know what is coming with the indefinite BTKi exposure in the ECHO regimen. Because MCL is not curable, it is not a question of if, but when, relapse will occur. But if we use the BTKi upfront, even if we achieve 20 years of remission, what do we do when that ends? Can we rechallenge with another BTKi? Should we move on to chimeric antigen receptor (CAR) T cells or bispecific antibodies? What do we do in countries where these modalities are not available? Do we worry at all, or just assume something better will be there in the future? Despite all our recent therapeutic advances, very few agents remain effective after BTKi resistance develops.

This gets even more complicated when you look at current options. CAR T-cell therapy, particularly brexucabtagene autoleucel, is currently our most effective treatment for MCL. However, it is unavailable in some countries and has limited access in the US due to restrictions on where treatment can be received. Will we get broader access to this modality, even though the data do not support this treatment as curative in MCL? Does the million-dollar-per-patient price tag become impossible to justify under those criteria? Does the lack of randomized studies of this modality in MCL raise any flags? If we examine the basis for this question, specifically for a select group of younger patients in their 40s and 50s, it's important to note that, historically, when a patient experiences a relapse after ASCT, long-term data suggest we can achieve remissions lasting more than 20 years. In addition, during a relapse, we know that using a BTKi often

results in another five or more years of disease control. Do we assume that the first-line BTKi-based regimen will achieve remission at a higher rate than sequential regimens? Maybe, but this would be less of a question if we were sure that during a relapse, we could reuse the BTKi or have another appropriate replacement therapy to turn to at that time.

Today, if a patient experiences a relapse during treatment with a covalent BTKi, we turn to CAR T cells or pirtobrutinib. Of the two, CAR T cells are the most effective but also have the most baggage. In addition to cost, we have to consider logistical issues and treatment burden. Safety issues with CAR T-cell therapy require patients to stay near the hospital for long periods with a caregiver present, forcing them out of work and sometimes requiring relocation. They are also required to have a caregiver with them for 24 hours a day for at least 2 weeks. Concerns with CAR T cells include cytokine release syndrome, neurologic complications, liver toxicity, and infections. Taken together, the "cost" of this treatment goes beyond just financial. Although bispecific antibodies could fill the void, they are not approved for MCL. In addition, they require 3 to 4 weeks of step-up dosing with hospital-based monitoring, followed by treatment every 3 weeks indefinitely. Bispecific antibodies and CAR T-cell therapies both increase the risk for infections and may require prophylaxis that some patients cannot tolerate. These are factors that we need to weigh against therapeutic benefit.

As we look at value across a patient's disease course, older patients face questions about healthcare system investment when working years are behind them, and life expectancy may be limited regardless of cancer treatment. Younger patients contribute more working years to the economy, justifying higher upfront costs, but they also need treatment strategies that preserve options for decades.

What needs to change? We need to follow up on completed studies to better assess how they compare with historical treatments. Improved patient stratification is also needed, as we know that patients without certain high-risk features will do well with standard options. As we look to future drugs and studies, it would behoove the global community to incorporate better control arms in global phase 3 trials. It is difficult to conduct meaningful comparative trials when your control arm would be considered substandard in some countries, which will negatively impact accrual in those regimens. Regulatory agencies and pharmaceutical companies need to find ways to support more uniform, robust control arms without bankrupting either the healthcare system or the drug development process.

"For clinicians, the change in first-line treatment of MCL means that individualized decision-making has never been more important … Today in my practice, the discussions I have with patients are different from those I had 3 years ago, and they will likely be different 3 years from now."

For clinicians, the change in first-line treatment of MCL means that individualized decision-making has never been more important. The conversation you have with each patient will invariably be different. For younger patients, you will need to weigh the need to maximize lifespan while minimizing long-term toxicities without limiting future options. For older patients, you will likely want to prioritize quality of life over the few extra months that some treatments may provide. Mixed into this is your ability to access certain therapies, which could limit your options.

Today in my practice, the discussions I have with patients are different from those I had 3 years ago, and they will likely be different 3 years from now. In the end, I remain steadfast about what the data currently demonstrate and what they do not. Now, we are left with making treatment decisions with excellent short-term data but significant uncertainty about long-term consequences. Our patients deserve that transparency as we work together to choose the path that best fits their individual situations, resources, and goals.

The Art and Science of Community Hematology Practice in 2026

Integration of Value-Based Care in Support of Primary Provider Practice and Hematologic Collaboration

*By **Kimberly Ku, MD***

Hematologists and primary care providers (PCPs) are increasingly sharing cases in managing routine complete blood counts (CBCs), with 76.9% of PCPs reporting they obtain selective screening CBCs and 48.9% testing all new patients for anemia regardless of symptoms or risk factors.[1] Although no guidelines explicitly recommend routine anemia screening for asymptomatic outpatients, the de facto practice of reviewing routine CBCs seems to be widely accepted by healthcare stakeholders as a normal part of an annual examination.

Kimberly Ku, MD

We, as a medical field, have yet to generate high-quality evidence demonstrating that a CBC as a screening tool for asymptomatic patients improves survival or other patient-centered outcomes. A prospective study of 595 medical outpatients found that routine CBCs had limited usefulness as a case-finding tool, leading to new management for only 0.5% of patients, and these were cases of treatment for iron deficiency.[2] Understandably though, it seems the alternative of not offering CBCs would probably feel like a step backward in medical practices compared with acknowledging some of the limitations of using the CBC as a screening tool.

Similarly, standard population-based reference intervals provide limited clinical value because the one-size-fits-all approach undermines precision medicine goals meant to capture patient-specific variation. An article in *Nature* explores the substantial individual variation in hematologic setpoints wherein individual routine CBC indices fluctuate around stable, patient-specific values, suggesting that interpreting results relative to an individual's own baseline values provides more clinically meaningful information than comparing the results to population norms.[3] The use of such intervals becomes particularly relevant in the era of the 21st Century Cures Act in which patients can see their CBC results (most notably the aberrant findings with additional red call-out boxes and high and low signifying arrows) even before getting the chance to receive any interpretive communications from their care team.

Simultaneously, no guidelines from Choosing Wisely or other organizations explicitly recommend against screening for anemia in outpatients, although Choosing Wisely does recommend against obtaining routine or repeated CBCs for hospitalized patients.[4]

A recent didactic article expounds on this contrary finding in which we hold this premise of routine CBCs as a means of early leukemia detection, effective treatment, and allowing patients to live longer. Yet such evidence does not currently exist to uphold the CBC as a screening tool, let alone account for the impact on clinical decision-making in the context of current-day practice limitations, resource utilization, and introduction of uncertainty, which warrants reassuring patients appropriately and addressing their anxiety.[5]

Additionally, a survey of 1,353 attending and 689 house staff physicians revealed that only four of 11 routinely reported CBC parameters were selected as frequently or always useful by more than 90% of physicians: hemoglobin, hematocrit, platelet count, and white blood cell count. Such findings indicate that many physicians do not use much of the data provided in routine CBC reports.[6]

A recent editorial in *Haematologica* goes even further in exploring how the CBC has evolved to include as many as 30 variables in some laboratories. You can probably imagine the likelihood of a PCP encountering out-of-range values of trivial relevance yet still requiring reconciliation for both patient and physician in collaboration with a local community hematologist.[7]

Alternative approaches with evidence of clinical value do exist. Such an instance is the use of combined reference regions (CRRs) for multiple CBC indices, shown to improve mortality risk prediction compared with univariate reference intervals. In a study in which CRRs were developed for pairs of CBC indices, investigators found that the 95% CRR identified individuals with an increase of more than sevenfold in 5-year mortality risk, significantly improving risk estimation for all (21 of 21) patient subsets defined by current univariate reference intervals.[8]

In another instance, trends from checking longitudinal CBCs show promise for cancer detection through a dynamic prediction model that examines trends in hemoglobin, mean corpuscular volume (MCV), and platelet measurements. This model demonstrated good performance for 2-year risk for colorectal cancer,[9] mirroring the clinical pearl of seeking

colonoscopic workup for patients with iron deficiency, which many hematologists know well. Patient-level declines in hemoglobin and MCV and rises in platelets increased the likelihood of a cancer diagnosis within 2 years. Such a model could be especially helpful for those referrals to hematology because of elevated platelet counts in terms of assisting the PCP with balancing multiple interpretive dimensions of the CBC even before referral to a hematologist.

In terms of a systems-based solution, automated laboratory algorithms integrated into laboratory information systems—such as reflex testing protocols for anemia evaluation—have demonstrated potential to reduce unnecessary test ordering while maintaining diagnostic utility and accuracy.[10-12]

Another incidental finding requiring thoughtful, nuanced interpretation, which cannot always be reliably captured by a routine CBC, is the increasing awareness of clonal hematopoiesis (CH). This entity is defined by a hematopoietic stem cell, which can develop into different types of blood cells, making cells with the same genetic mutation, or clones. In emulating a pre-malignant state, CH can often be found with increasing age in patients with no symptoms and no aberrations detected by the CBC and can progress to blood cancers, especially myelodysplastic syndromes and acute myeloid leukemia.

"We, as a medical field, have yet to generate high-quality evidence demonstrating that a CBC [complete blood count] as a screening tool for asymptomatic patients improves survival or other patient-centered outcomes."

In a study from a large community practice, Tennessee Oncology, 29.7% of patients undergoing comprehensive genomic profiling had clonal hematopoiesis of indeterminate potential (CHIP)—most identified incidentally during solid tumor workup as a somatic genomic mutation in the absence of a causal relationship or the existence of cytopenias—with nearly two-thirds having high-risk mutational profiles requiring further hematologic subspecialist interpretation, particularly those with the confounded effects of treatment-related cytopenias.[13]

Even with the relatively high prevalence as noted in the Tennessee Oncology study,[13] the National Comprehensive Cancer Network (NCCN) does not currently advise any type of screening for CH outside of research. Specialized CH clinics at major centers have emerged to provide risk stratification, counseling, and clinical trial access for affected patients.[14-16]

In summary, future prospective randomized controlled trials that could support and guide the previously mentioned clinical observations for PCPs working in concert with community hematologists and oncologists include the following: 1) comparing automated algorithm-driven triage with standard care in hematology/oncology referrals, measuring time to diagnosis, treatment initiation, healthcare utilization, and patient-reported outcomes; 2) integration of CH risk stratification tools into primary care clinical decision support systems to identify high-risk patients requiring urgent hematology evaluation; 3) standardization of eConsult protocols to allow comprehensive assessment of underlying etiologies between referring and referred providers, while maintaining resource efficiency gains of providers determining necessity of referrals in coordinated fashion on behalf of patient-shared decision-making and autonomy; 4) cost-effectiveness analyses comparing different triage strategies, particularly in the context of increasing incidental detection of CH through comprehensive genomic profiling.

This article is the first installment in a two-part series. The continuation will appear in the March issue of Blood Cancers Today.

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Get to Know

Learn more about the leaders, innovators, and educators in hematologic oncology

Shernan Holtan, MD

Dr. Shernan Holtan talks strength training, bass playing, her passion for improving access to hematopoietic stem cell transplant, and how she integrates her personal hobbies into her professional career.

*By **Melissa Badamo***

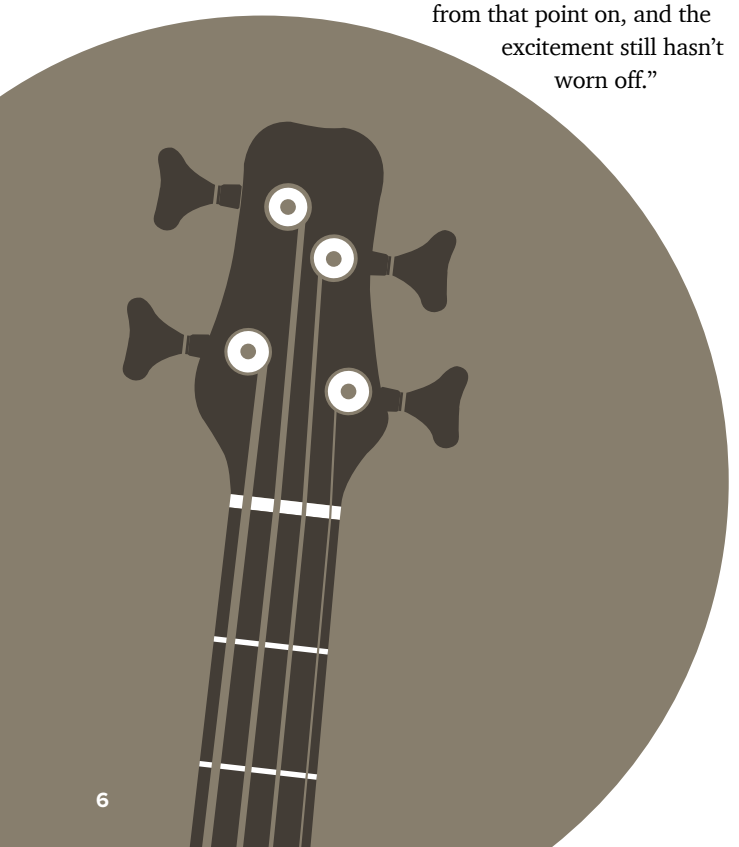
Small Town, Big Dreams

Prior to becoming a hematologist-oncologist, **Shernan Holtan, MD,** grew up in a small town in western Nebraska. There, she attended a country school of fewer than 100 students and later enrolled in Hastings College, where she considered pursuing a career in music.

"My first love was music," Dr. Holtan shared. "I spent a lot of time writing and learning music as a kid up into my teenage years. I couldn't play anything well, but I loved music theory and creating music."

After some soul searching, Dr. Holtan later pivoted to medicine and attended medical school at the University of Nebraska Medical Center, where she discovered an interest in hematology and transplant medicine.

"I didn't know about the field of bone marrow transplantation until my fourth year of medical school, where I completely fell in love with the idea of curing cancer with the immune system," she said. "I knew this is what I wanted to do from that point on, and the excitement still hasn't worn off."



Dr. Holtan currently serves as Chief of the Blood and Marrow Transplantation Section of the Department of Medicine at Roswell Park in Buffalo, New York, where she conducts clinical and translational research on hematopoietic stem cell transplant (HSCT) outcomes, graft-versus-host disease treatment and prevention, relapse prevention, and other cellular regenerative therapies.

Dr. Holtan describes the proudest moment of her career as reinvigorating Roswell Park's transplant program to vastly improve patient outcomes in Buffalo, an underserved community.

"We know from databases from the CIBMTR [Center for International Blood and Marrow Transplant Research] that our transplant center is located in one of the lowest income regions in the country," she explained. "Buffalo is a very diverse community, so we needed to lean on mismatched unrelated donors. Through a tremendous amount of work over the past couple of years, we've been able to transform our program to have some of the best survival outcomes that I know of in the world. It's remarkable considering some of the major challenges our patients have in getting to transplant."

According to Dr. Holtan, the 2024 one-year survival rate for Roswell Park's allogeneic transplant program is 92.6%, achieved while approximately doubling transplant volumes. By modernizing and streamlining transplant procedures, the center also helped reduce medication costs by about $7 million.

"It's been a ton of work, but to see the results of that effort and show what we can do when we all work together for a common goal has been very gratifying," she added. "We've come a long way in our field, and now we can have excellent outcomes with mismatched unrelated donors. It's a vastly different field compared to 20 years ago. The biggest barrier to transplant is no longer donor availability."

Improving Geographical Access to HSCT

Despite major progress in stem cell transplantation, unmet needs remain. According to Dr. Holtan, geographical access to HSCT remains a significant challenge for patients in rural areas.

"I think back to my childhood in western Nebraska, where there is limited access to hematology oncology. Someone

needing a transplant or cell therapy would likely have to travel to Denver, Colorado, which presents both geographic and insurance coverage barriers."

The other option is driving eight hours to the University of Nebraska in Omaha to stay within state lines, Dr. Holtan explained.

"What I'd like to work on is breaking down the geographic barriers to transplant, minimizing the amount of time that a patient spends at a transplant center, then decentralizing a lot of the care so that it can go back to their home communities," she said. "That's the biggest unmet need and where I'm going to be focusing my time: making sure that no matter where you are in the country, you have access to this care, can afford to receive it, and still have excellent outcomes."

According to Dr. Holtan, efforts to improve transplant access include advancing care networks by building relationships with community doctors and nurses, training more transplant physicians, and ensuring access to telemedicine.

"So many patients opt out of this potential cure because it's too far away and too expensive," she added. "If we can leverage the research that has led to safer outcomes, including the transplants at home and all the supportive care that we can now do in a community setting, now is the time to invest in that type of infrastructure so that more patients can access this care."

A Fitbit Pilot Study: Frailty and HSCT

Dr. Holtan and her team recently embarked on a Fitbit pilot study to gain insight into the activity and sleep patterns of patients who underwent myeloablative allogeneic HSCT. She also mentored a medical student while conducting the pilot study, and they published their results in *Frontiers in Medical Technology* in December 2025.[1]

In the study, nine patients received Fitbit smartwatches in an inpatient setting to monitor their steps per day and hours of sleep per night. Patients were considered relatively young and healthy, Dr. Holtan noted, with a median of 48 years. However, two patients met the criteria for frailty, characterized by muscular weakness, weight loss, and poor exercise tolerance.[1]

"This was something we dreamed up during [the] COVID [pandemic] to understand the lived experience of our patients," Dr. Holtan explained. "If we could put a Fitbit on someone, we could understand what their actual life was like in terms of the number of steps they take a day, their rest time, and the quality of their sleep."

Dr. Holtan and her team found that frailty after transplant was not only associated with decreased physical activity, but also very poor sleep. Many patients experienced sleep interruptions during the night, resulting in zero hours of sleep recorded.[1]

"This was eye-opening and a glimpse into how exhausting this procedure must be to go almost a month without good quality sleep," she explained. "It's led us to think about how we can optimize care so that people can get rest."

From Weight Room to Clinic: Building Resilience and Connection Through Powerlifting

As a strength training enthusiast, Dr. Holtan ensures her patients are informed about the importance of physical health in improving HSCT outcomes.

"I've always liked lifting weights, but I started to do this with a coach about 10 to 12 years ago and learned about the techniques of powerlifting," she said. "A lot like music, I found it to be immersive. When you have a couple hundred pounds on your back, it's hard to think about anything else!"

In 2019, Dr. Holtan set a national record in the squat with the United States Powerlifting Association. More than just a hobby, Dr. Holtan translates her interests into her practice by empowering patients to exercise and strength train.

"I talk about that routinely with my patients," she added. "In the interest of connecting with people, I share what I do and I hope I can inspire them to do something similar and focus on building strength. I'll even demonstrate exercises in the office, exercise with people on the hospital units, and challenge them. I think that contributes to the positive outcomes of our transplant program. We want people to be out of the hospital. We want them to be experiencing self-efficacy through movement. At the end of the day, we do transplants to get people their lives back so that they're not dependent on the medical system anymore."

From powerlifting to learning punk and metal songs on her bass guitar, sharing her interests and fostering a human connection with patients is at the heart of Dr. Holtan's clinical practice.

"Being able to genuinely connect with people is what leads to good outcomes," she said. "Therapy isn't just the pill that you give. It's the hope. It's the conversation. It's the encouragement."

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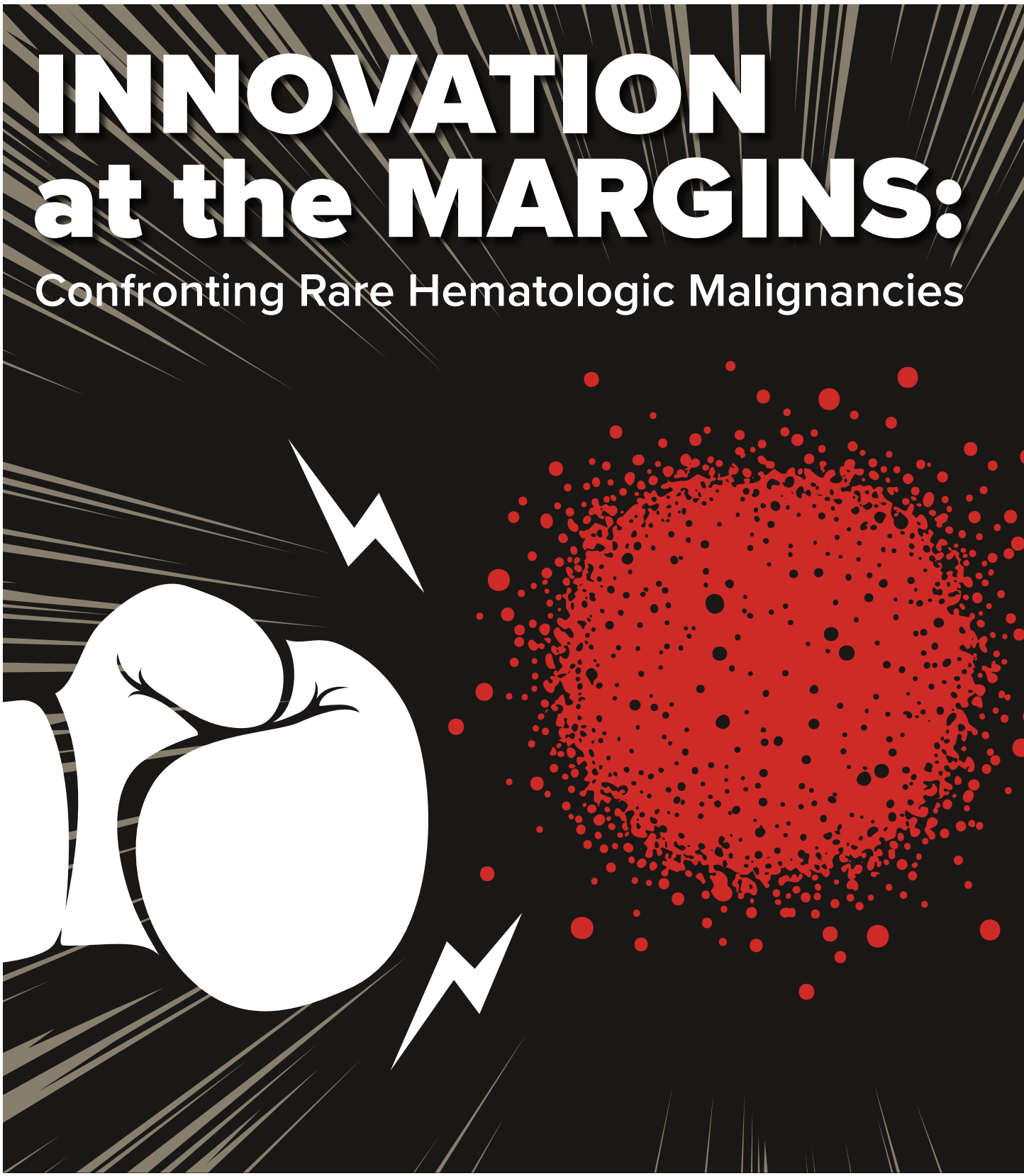
INNOVATION at the MARGINS:

Confronting Rare Hematologic Malignancies



*By **Leah Lawrence***

Using the definition provided by the National Cancer Institute (NCI), most, if not all, hematologic malignancies are considered rare diseases. Defined as any disease that affects fewer than 200,000 people in the US, or anywhere from 15 to 60 per 100,000 in various definitions, rare diseases can be difficult to diagnose and treat, with advances often coming few and far between.[1]

"Most hematologic malignancies fall into the rare disease category," said **Alexey Danilov, MD, PhD,** a Professor in the Department of Hematology & Hematopoietic Cell Transplantation at City of Hope. "For example, the incidence of chronic lymphocytic leukemia, one of the most common leukemias, is about 20,000 newly diagnosed cases per year in the US."[2]

Ultrarare cancers are defined as affecting fewer than 1,000 people each year in the US, or about two cases per 100,000 per year.[3,4] Within hematologic malignancies, there are quite a few ultrarare conditions. For example, the incidence of primary myelofibrosis, a myeloproliferative neoplasm, is estimated to be about 1.5 cases per 100,000 people.[5] Each year, about 700 people are diagnosed with hairy cell leukemia.[6] Mantle cell lymphoma (MCL) is estimated to affect 0.5 to 1 in 100,000 people.[7] The list goes on: T-cell lymphoma, Castleman's disease, acute promyelocytic leukemia, Waldenstrom Macroglobulinemia and more.

"The issue is that across all malignancies we have become more and more precise in finding subclasses and genetic alterations to target," said **Akiva Diamond, MD,** an Assistant Professor of Medicine - Hematology and Oncology at Baylor College of Medicine. "When we start breaking down everything into these subclasses, every subclass becomes ultrarare."

Benefit of Collaboration

When dealing with such small numbers of patients affected by these diseases, conducting research can be challenging.

"It is more challenging to push large pharmaceutical companies to invest in the treatment of rare malignancies unless there is a quick 'win' that can be generated from a smaller study," said **Bijal D. Shah, MD,** an Associate Member in the Department of Malignant Hematology at Moffitt Cancer Center. "Collaborative trials, which include cooperative group studies, are critical in meeting gaps in trials."

Dr. Diamond is part of a phase 1 trial group at Baylor College of Medicine that incorporates both solid tumors and hematologic malignancies. He emphasized the amount of time and effort it takes to start any clinical trial, even when working as part of a cooperative group.

"Let's say a company reaches out to me to ask if we can put a patient with myelofibrosis on a trial. We have limited resources,

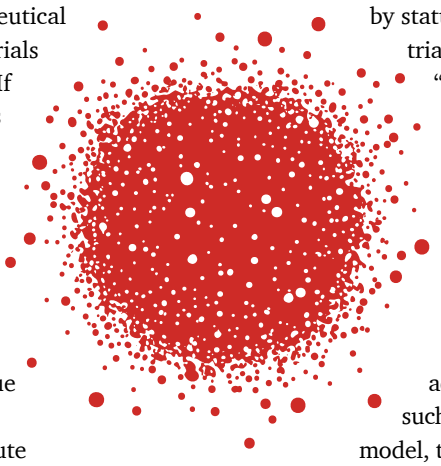
limited trial nurses—all of which must be trained on each protocol—and limited staff to guide patients through the process," Dr. Diamond said. "The work it takes to open a trial often makes it prohibitive if you are only going to put one patient on it."

Another important contribution to research on rare diseases is investigator-initiated trials, which are "much better about pushing trial enrollment into 'real world' patients that otherwise would not meet eligibility," Dr. Shah said.

"A third closely related mechanism [for rare disease research] is building out trials within groups that are recognized by NCI," Dr. Shah added. "Again, these smaller groups are not formally part of the larger cooperative group mechanism, but because of this recognition, it can make it much easier for NCI-designated Cancer Centers to run these trials at lower cost, which can also help to make trials more feasible and accessible for patients."

One major goal for those working in rare diseases should be to find ways to streamline the research process, Dr. Diamond said.

"Other countries open trials much more quickly than the US, and that is why—in talking to pharmaceutical companies—they like to run earlier phase trials internationally now," Dr. Diamond added. "If we can succeed at streamlining that process for rare hematologic malignancies, we could apply that lesson to research into all rare diseases."



Breakthroughs

Despite the many challenges associated with conducting research in these rare diseases, therapeutic breakthroughs continue to be made.

Dr. Shah pointed to recent progress in acute lymphocytic leukemia (ALL), which is diagnosed in about 1.9 per 100,000 per year.[8] In recent years, blinatumomab was approved by the FDA for CD19-positive Philadelphia chromosome-negative B-cell precursor ALL,[9] and the CAR T-cell therapy obecabtagene autoleucel was approved for adults with relapsed or refractory B-cell precursor ALL.[10]

There have been several advances in MCL in recent years with the discovery of Bruton tyrosine kinase (BTK) inhibitors. The TRIANGLE trial, which showed that adding ibrutinib to chemoimmunotherapy benefited younger patients with MCL, redefined how MCL was treated in the frontline setting, according to Dr. Danilov.[11] In 2023, the FDA granted accelerated approval to another BTK inhibitor, pirtobrutinib, for relapsed or refractory MCL.[12]

BTK inhibitors, such as ibrutinib and zanubrutinib, have also been approved for Waldenstrom macroglobulinemia.[13] Advancements in T-cell lymphomas are far behind those in B-cell lymphomas, where a number of new regimens have been approved. In contrast, it has been more than a decade since any new therapies were approved for Castleman's disease,[14] a reminder that there is always more work to be done.

"There are several other exciting smaller studies that have met the threshold for inclusion within the NCCN guidelines (which can influence insurance coverage), but not FDA approval yet," Dr. Shah noted.

Regulatory Pathways

The FDA has developed different guidance and regulatory pathways that allow for faster advances for patients with rare diseases.

In 2025, the FDA announced the Rare Disease Evidence Principles, which were designed to give clearer guidance to sponsors on the types of evidence that can be used to demonstrate substantial evidence of effectiveness.[15]

"It is well understood that developing drugs for rare diseases can make it difficult or even impossible to generate substantial evidence of safety and efficacy, as required by statute, using multiple traditional clinical trials," the FDA said in a press release. "Instead, rare disease drug developers and the FDA must work together to identify alternative methods for meeting the statutory standard that are both rigorous and viable for rare disease populations."

With strict eligibility criteria, this new pathway offers assurance to sponsors that drug review will encompass additional supportive data in the review such as evidence from a relevant nonclinical model, therapeutically relevant pharmacodynamic data and other clinical data like case reports.

The FDA also grants orphan drug designation for products to prevent, diagnose, or treat a rare disease; incentives include tax credits for qualified clinical trials, exemption from user fees, and potential seven years of market exclusivity after approval.[16] Fast Track designation helps to facilitate expedited development and review of new drugs or biologics that address unmet medical needs.[17]

"There is also the pipeline of accelerated approval that allows for earlier approval of drugs for serious conditions; most drugs for cancers that are relapsed can gain accelerated approvals," Dr. Diamond said. "The FDA may also look at surrogate markers like response rate or minimal residual disease, or at data from a single-arm study, or a study comparing a treatment against a historical control."

All of this acknowledges that it is very difficult, if not impossible, to conduct large phase 3 randomized controlled studies in rare and ultrarare diseases.

Confronting Rare Diseases

"For some hematologists, treating these rare diseases may be more commonplace, but for many oncologists, they may only see one or two cases of these rare diseases a year, or even over their whole career," Dr. Danilov said.

In cases where clinicians may be seeking emerging data or guidance, referral may be the best option.

"There is no question in my mind that this is where expert referral—to an NCI Cancer Center—at diagnosis plays an important role … ideally to a physician with known, established presence in addressing that specific malignancy," said Dr. Shah. "This can help not only in making sure diagnosis is accurate, but also in guiding towards the best trials."

Dr. Shah emphasized that this in no way disparages community oncologists or larger referral centers. When dealing with rare diseases, he said, experience plays a considerable role in diagnosis, standard of care management, access to newer therapies, and access to novel and collaborative clinical trials.

Dr. Diamond agreed, adding that even among NCI Cancer Centers, certain programs will have expertise in certain ultrarare diseases. For example, Baylor has specific expertise on Epstein-Barr virus-mediated lymphomas.

In cases where referral may not be an option, there are some resources available, Dr. Diamond said.

"There are references and resources out there like *Blood*'s 'How I Treat' articles, in addition to clinical guidelines," Dr. Diamond said. "Often I find the best approach is finding the person who wrote the paper or the guideline—their email is often included—and corresponding with them directly."

When recently faced with a case of Langerhans cell histiocytosis, Dr. Diamond used this strategy to speak directly with experts.

"There is a lot of nuance involved," he said. "For example, maybe in the trials they used 60 mg of a drug but in real life no one uses more than 40 mg because it isn't well tolerated. That information isn't always published; you only know if by treating patients."

If the decision is made to initiate treatment of rare diseases locally, without referral to expert centers, consultation with these centers may still be of benefit. There are cases where a patient may choose to be referred in later lines of therapies, and establishing that relationship early can be beneficial.

"I think the future is finding ways to partner in functional ways—ie, diagnosis, treatment, and trials—with our colleagues in the community to co-manage patients so they can remain closer to home," Dr. Shah said. "Again, this is hard! But that would be where I hope we ultimately transition when we talk about collaborative clinical trials."

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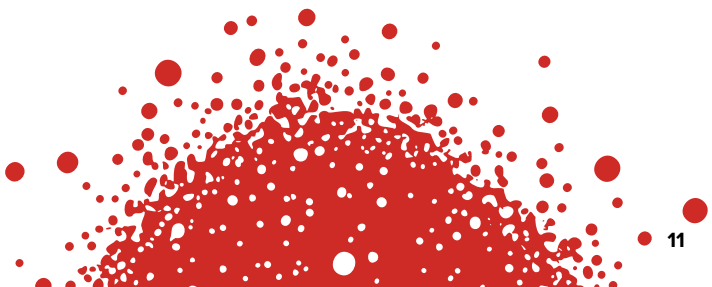


Regulatory Actions

Regulatory approvals, designations, and guidance in the field of hematologic oncology

Scan to learn more.

December 3, 2025

FDA Approves Expanded Indication for Pirtobrutinib for Relapsed or Refractory CLL/SLL

The expanded indication includes patients with covalent Bruton tyrosine kinase (BTK) inhibitor-pretreated chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL) earlier in their treatment course. Pirtobrutinib, a non-covalent BTK inhibitor, achieved a progression-free survival rate of 14 months compared with 8.7 months for investigator's choice therapy in the BRUIN CLL-321 trial.

December 21, 2025

FDA Approves Mosunetuzumab-Axgb for Patients With Relapsed or Refractory Follicular Lymphoma

The FDA has approved a new formulation of mosunetuzumab-axgb, a CD20 × CD3 bispecific antibody developed by Genentech, for relapsed or refractory follicular lymphoma as a one-minute subcutaneous injection. Results are based on the GO29781 study, in which mosunetuzumab-axgb achieved an overall response rate off 77% and a complete response rate of 62%, with a consistent safety profile.

January 7, 2026

KLN-1010 Receives FDA Clearance of Investigational New Drug Application

KLN-1010, an in vivo BCMA chimeric antigen receptor (CAR) T-cell therapy for Relapsed and refractory multiple myeloma, is en route to its first clinical trial following Investigational New Drug Application acceptance.

January 11, 2026

FDA Announces Regulatory Flexibilities for Cell and Gene Therapies

The FDA increased regulatory flexibilities on cell and gene therapy requirements, with the goal of expediting product development. As a result, the FDA will no longer require three Process Performance Qualification (PPQ) lots for process validation.

January 26, 2026

IBI3003 Trispecific antibody Receives FDA Fast Track Designation for Relapsed or Refractory Multiple Myeloma

IBI3003, an anti-GPRC5D/BCMA/CD3 trispecific antibody, has received Fast Track Designation from the FDA for patients with multiple myeloma who have received four or more lines of therapy. According to data presented at the 67th American Society of Hematology Annual Meeting, IBI3003 resulted in an overall response rate of 83.3% with low-grade cytokine release syndrome.

January 27, 2026

FDA Approves D-VRd for Newly Diagnosed Multiple Myeloma

The FDA has approved daratumumab and hyaluronidase-fihj with bortezomib, lenalidomide, and dexamethasone (D-VRd) for adult patients with newly diagnosed multiple myeloma ineligible for autologous stem cell transplant. In the phase 3 CEPHEUS trial, the quadruplet combination achieved a higher measurable residual disease negativity rate (60.9% vs 39.4%) and complete response rate (81.2% vs 61.6%) compared with VRd alone.

Highlights from the **2026 TANDEM MEETINGS | TRANSPLANTATION & CELLULAR THERAPY MEETINGS OF ASTCT AND CIBMTR, FEBRUARY 4-7, SALT LAKE CITY, UTAH.**

Real-World Evidence Signals Shift in CAR-T for Advanced B-Cell ALL

*By **Nichole Tucker***

Obecabtagene autoleucel (obe-cel) resulted in a higher rate of response and lower grade 3 toxicity compared with brexucabtagene autoleucel (brexu-cel) in real-world patients with relapsed or refractory (R/R) B-cell acute lymphoblastic leukemia (ALL).

These findings from an analysis of the ROCCA registry presented by **Yannis Valtis, MD,** at the 2026 Tandem Meetings, represent the first real-world data of obe-cel in this patient population.

Yannis Valtis, MD

Brexu-cel and obe-cel currently anchor the chimeric antigen receptor (CAR) T-cell therapy landscape for patients with R/R B-cell ALL. Although brexu-cel delivered high complete response (CR)/complete response with incomplete count recovery (CRi) rates and meaningful survival benefits in the ZUMA-3 trial, the frequency of grade 3 adverse events tempered enthusiasm, sharpening the focus on the need for a safer, next-generation option.

"One of the key messages is that the role of CAR T-cell therapy in ALL is continuing to evolve. We now have many effective options for B-cell ALL, but as some of these therapies move into the frontline setting and become standard of care, there will always be a subset of patients who need additional treatment. Despite the progress we've made, this remains a challenging disease," co-investigator **Lori Muffly, MD,** associate professor of medicine (blood and marrow transplantation and cellular therapy) at Stanford Medicine, told *Blood Cancers Today*.

Lori Muffly, MD

The research team compared real-world records of 84 evaluable patients treated with obe-cel with 54 patients who received brexu-cel. Based on unadjusted toxicity analyses, the rate of cytokine release syndrome (CRS) was 59% in the obe-cel cohort versus 93% in the brexu-cel cohort ($P<0.001$), and the rate of immune effector cell–associated neurotoxicity syndrome (ICANS) was 17% in the obe-cel cohort versus 50% in the brexu-cel cohort ($P<0.001$). Early immune effector cell–associated hematotoxicity occurred in 89% of the obe-cel cohort versus 74% of the brexu-cel cohort.

Results showed a higher incidence of low-grade CRS after the second infusion compared with the first. Several patients who did not experience CRS after the initial infusion developed low-grade CRS after the second infusion, highlighting a modest increase in inflammatory toxicity with subsequent dosing, Dr. Valtis noted. ICANS remained uncommon in this cohort. However, as in the case of CRS, some patients who did not experience ICANS after the first infusion developed low-grade ICANS after the second infusion, suggesting that neurologic events, although rare, may emerge with repeat exposure, Dr. Valtis explained. These toxicities were predominantly low grade, supporting a manageable safety profile overall.

Efficacy outcomes were assessed at day 28 from the first obe-cel infusion, and analyses were not adjusted for disease burden. Obe-cel achieved a 92% CR/CRi rate, compared with a 95% CR/CRi rate with brexu-cel, with no statistically significant difference between the two therapies. Measurable residual disease negativity rates were high in both groups. Efficacy findings also showed a 6-month progression-free survival of 74% and an overall survival rate of 90% with obe-cel, with a median follow-up of approximately 137 days from the first CAR T-cell infusion. The impact of maintenance strategies after CAR T-cell therapy, including allogeneic stem cell transplantation and tyrosine kinase inhibitors, remains an important area for future investigation, according to Dr. Valtis.

"There is a real opportunity to better understand where CAR T-cell therapy—particularly whether it can be curative on its own—fits into the treatment paradigm," Dr. Muffly said. "I'm very hopeful that CAR T-cell therapy will be incorporated more broadly for high-risk patients, and I would encourage providers who have not yet used obe-cel to consider it, given its favorable safety profile and relative ease of use. This is an encouraging time for those of us who treat ALL."

Reference

Valtis Y, et al. Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR. Abstract 32.

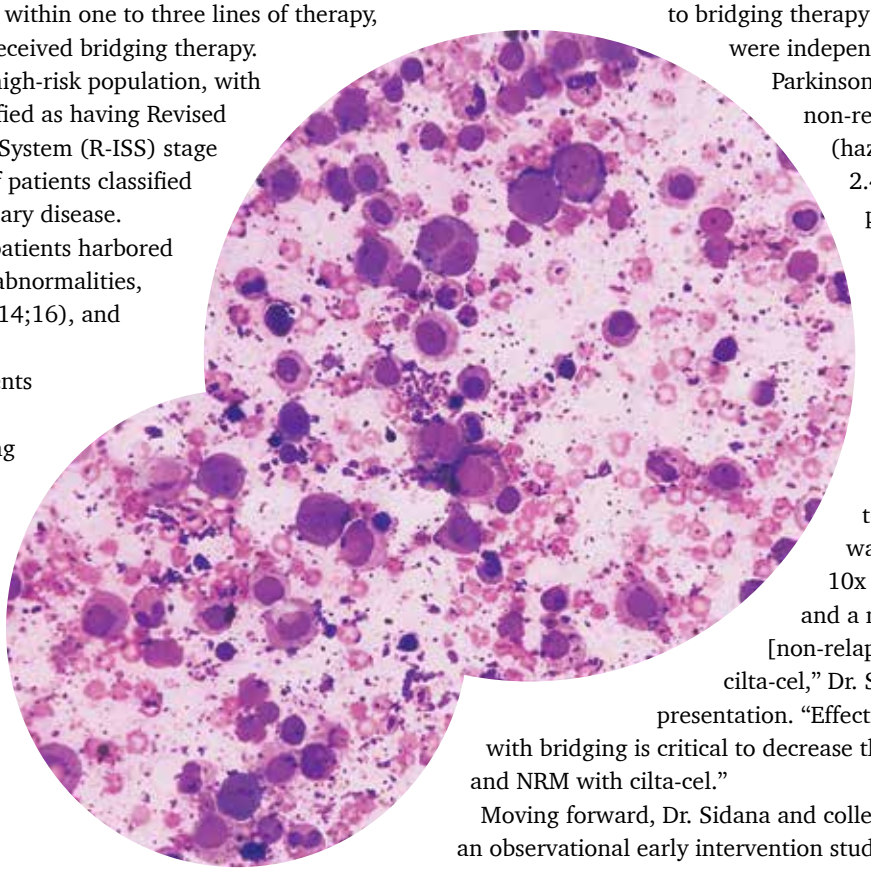
Risk Factors for Neurotoxicity and Non-Relapse Mortality Among Patients Treated With Cilta-Cel for Relapsed or Refractory Multiple Myeloma

*By **Melissa Badamo***

Despite its high efficacy in relapsed or refractory multiple myeloma (MM), ciltacabtagene autoleucel (cilta-cel) brings a risk of delayed neurotoxicity and non-relapse mortality. A retrospective study presented at the 2026 Tandem Meetings by **Surbhi Sidana, MD,** of Stanford University School of Medicine, identified high absolute lymphocyte count (ALC) and non-response to bridging therapy as potentially modifiable risk factors for these adverse events.

Surbhi Sidana, MD

The US MM Immunotherapy Consortium study gathered data from 761 patients (median age, 65 years) treated with cilta-cel chimeric antigen receptor (CAR) T-cell therapy at 15 centers. All patients had a median of five prior lines of therapy, 16% received cilta-cel within one to three lines of therapy, and 89% of patients received bridging therapy. This was a relatively high-risk population, with 18% of patients classified as having Revised International Staging System (R-ISS) stage III disease and 27% of patients classified as having extramedullary disease. Additionally, 39% of patients harbored high-risk cytogenetic abnormalities, including del(17p), t(14;16), and t(4;14).



Ten percent of patients experienced delayed neurotoxicity, including Parkinsonism (2.9%; n=22) and cranial nerve palsy (4.6%; n=35). Dr. Sidana and colleagues found that the risk of delayed neurotoxicity was higher in patients who did not respond to bridging therapy. Delayed neurotoxicity occurred in 12% of patients who did not respond to bridging therapy, compared with 6% of patients who responded. Similarly, Parkinsonism occurred in 5% of patients who did not respond, compared with 0.5% who responded ($P<0.05$). Despite an overall response rate of 91% and a complete response rate of 68%, most patients (21/22) who developed Parkinsonism did not respond to bridging therapy.

The researchers also found a link between higher ALC and delayed neurotoxicity. Patients with Parkinsonism had a median peak ALC of 5.88/μL, compared with 1.17/μL among patients without Parkinsonism ($P<0.001$). The risk of Parkinsonism was 12% in patients with ALC greater than 3000/μL, compared with 1% in patients with ALC less than or equal to 3000/μL.

"This can serve as a biomarker to identify patients for preemptive interventions and risk mitigation measures," said Dr. Sidana during her presentation.

According to multivariable analysis, peak ALC greater than 3000/μL (odds ratio [OR]: 12.7; $P<0.001$) and non-response to bridging therapy (OR: 9.9; $P=0.03$) were independent risk factors for Parkinsonism. Additionally, non-response to bridging (hazard ratio [HR] 2.41; $P=0.046$), poor performance status greater than or equal to 2, high-risk cytogenetics, and age 70 years or older were independent predictors of non-relapse mortality.

"Non-response to bridging therapy was associated with 10x risk of Parkinsonism and a much higher NRM [non-relapse mortality] with cilta-cel," Dr. Sidana said during her presentation. "Effective tumor debulking with bridging is critical to decrease the risk of Parkinsonism and NRM with cilta-cel."

Moving forward, Dr. Sidana and colleagues have launched an observational early intervention study across four institutions to evaluate whether dexamethasone reduces the risk of Parkinsonism in patients with high CAR-T expansion.

Reference

Sidana, S, et al. 2026 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR. Abstract 419.

Rapcabtagene Autoleucel Delivers Promising First-Line Outcomes in High-Risk LBCL

*By **Nichole Tucker***

Jason Westin, MD, MS

In first-line patients with high-risk large B-cell lymphoma (LBCL), rapcabtagene autoleucel demonstrated encouraging efficacy and safety profiles, according to results from a phase 2 study led by **Jason Westin, MD, MS,** of the University of Texas MD Anderson Cancer Center.

According to Dr. Westin, who presented the results at the 2026 Tandem Meetings, chimeric antigen receptor (CAR) T-cell therapies are practice changing in relapsed or refractory (R/R) LBCL. These therapies represent an alternative to frontline chemoimmunotherapy, which is associated with poor outcomes.

"Rapcabtagene autoleucel is a promising next-generation CAR T-cell therapy with several features that could represent meaningful advances for patients. The manufacturing timeline appears notably shorter than existing products, which is important in aggressive lymphomas where time matters. In addition, early data suggest a favorable safety profile, with a high proportion of patients avoiding significant cytokine release syndrome," Dr. Westin told *Blood Cancers Today*.

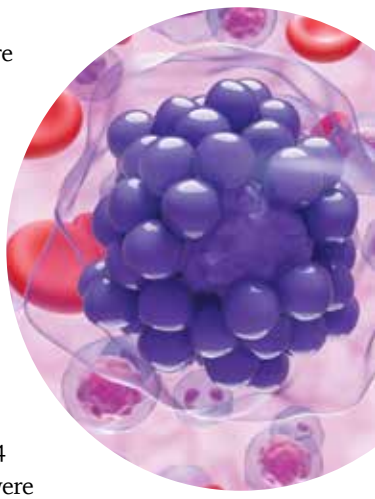
In the phase 2 study, rapcabtagene autoleucel achieved an objective response rate (ORR) of 89% (95% CI, 77%-96%) with a complete response (CR) rate of 74% (95% CI, 60%-85%) and partial response rate of 15%. Results come from 53 patients with R/R LBCL treated in the upfront setting. Among all 47 responders, the median duration of response (DOR) was not reached, but there was 79% probability of ongoing responses at the 6-month timepoint. Among patients who reached a CR, median DOR was not reached with an 86% probability of ongoing responses at 6 months.

In addition to the overall patient population, subgroups stratified by factors such as elevated lactate dehydrogenase before CAR T-cell therapy, overall response at screening, cell of origin, presence of rearrangements in *MYC/BCL2/BCL6,* bridging therapy, and risk factors were also observed. The efficacy was consistent across the subgroups. Notably, in the 27 patients with an International Prognostic Index (IPI) score of 3 and not double- or triple-hit lymphoma, the CR rate was 78% (95% CI, 58%-91%). In addition, the 26 patients with an IPI score of 4 or 5 or double- or triple-hit lymphoma achieved a CR rate of 69% (95% CI, 48%-96%).

Median progression-free survival (PFS) was not reached in the overall population or in the 29 patients who reached a CR at 3 months. However, the estimated 6-month PFS was 76% in the overall population, and 96% for patients with a CR at 3 months.

"The safety profile, I think, was favorable, and not surprisingly," Dr. Westin stated during his presentation. "Adverse events [AEs] were seen in the majority of patients. One hundred percent of patients had some degree of AEs."

The AEs of special interest in the study were any-grade cytokine release syndrome (CRS) and immune effector cell–associated neurotoxicity syndrome (ICANS), which occurred in 49% and 8% of patients, respectively. CRS events were predominantly low grade. Among patients with ICANS, one patient experienced a grade 3 event.



There was a high frequency of cytopenias during the study, including any-grade neutropenia, anemia, and thrombocytopenia in 94%, 68%, and 59% of patients, respectively. Grade 3 neutropenia, anemia, and thrombocytopenia were seen in 13%, 42%, and 15% of patients, respectively. Cases of grade 4 neutropenia and thrombocytopenia were seen in 79% and 19% of patients, respectively. Most cytopenias resolved by month 3, Dr. Westin explained.

These phase 2 findings warrant further study to confirm the comparative benefit of rapcabtagene autoleucel in patients with first-line, high-risk LBCL.

"These findings are encouraging, and I look forward to seeing results from larger clinical trials to better understand its role in treating patients," Dr. Westin told *Blood Cancers Today*.

Reference

Westin J, et al. 2026 Tandem Meetings | Transplantation & Cellular Therapy Meetings of ASTCT and CIBMTR. Abstract 56.

Cryopreserved Organ Donor Bone Marrow Offers Successful New Graft Source for HSCT

*By **Melissa Badamo***

Ossium Health's cryopreserved, organ donor bone marrow achieved successful engraftment as an off-the-shelf graft source for allogeneic hematopoietic cell transplantation (HSCT), according to early results published in *Cytotherapy*.[1,2]

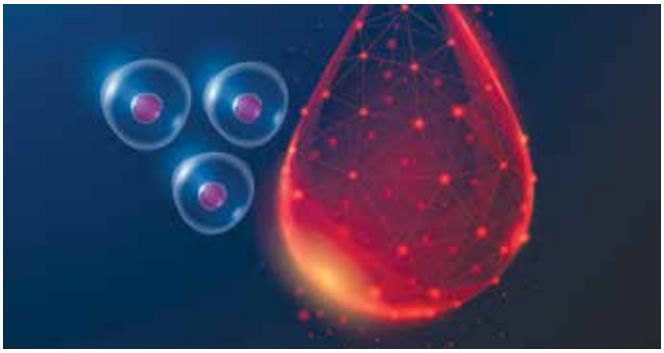
Led by **Erik J. Woods, PhD,** Chief Science Officer, Executive Vice President, and Co-Founder of Ossium Health, the research team reported results from the first three patients engrafted with "HPC, Marrow," Ossium Health's organ donor hematopoietic stem/progenitor cell (HPC) product. The study team enrolled these patients in Ossium Health's HOPE program, an expanded-access program for patients with acute leukemias who are unable to participate in the ongoing, first-in-human PRESERVE 1 trial.[3]

Erik J. Woods, PhD

Although bone marrow cryopreservation preserves hematopoietic stem/progenitor cells (HPC) shelf life and facilitates HSCT, there remains "significant" variation in product quality among different transplant collecting centers.[2] To address this challenge, HPC, Marrow was produced in a controlled, dedicated facility and developed and validated using Good Manufacturing Practice (GMP) principles.[2]

"With an off-the-shelf, cryopreserved bone marrow graft, patients can proceed to transplant precisely when clinically indicated, rather than waiting weeks to months for donor identification, scheduling, and collection," Dr. Woods told *Blood Cancers Today*. "This is particularly impactful for patients requiring urgent transplantation, as it may allow them to avoid additional cycles of chemotherapy or the risk of relapsing while awaiting a donor."

Ossium Health's cryopreserved bone marrow bank—which consists of vertebral segments from organ donors aged 7 to 55 years old—achieves shelf-life flexibility while controlling variability.[2] According to Dr. Woods, reducing cryopreservation variability ultimately improves graft quality, functional viability, and patient outcomes.



All three patients (aged 63-68 years old) included in the study had high-risk acute myeloid leukemia (AML) and were in first complete remission. All patients received graft-versus-host disease (GVHD) prophylaxis and a reduced-intensity conditioning regimen (fludarabine and melphalan or fludarabine and busulfan) before receiving an average of 4.75×10^6 CD34+ cells/kg and 1.81×10^7/kg CD3+ cells/kg.[2]

All patients achieved rapid neutrophil and platelet engraftment, full blood and bone marrow donor chimerism, and restored immune system function.[1,2] HPC, Marrow also demonstrated a robust safety profile with no infusion-related toxicity, cytokine release syndrome, or adverse events related to dimethyl sulfoxide, which was used for cryopreservation. All patients experienced acute GVHD, which resolved after treatment. One patient died due to a bacterial infection unrelated to HPC, Marrow.[2]

"Rates and severity of GVHD have been relatively low given the degree of mismatching, and all GVHD has been successfully managed," said Dr. Woods. "Importantly, no patient has experienced disease relapse."

Overall, these early findings show that HPC, Marrow provides a timely, high-quality graft source for patients without matched donors, shifting the HSCT landscape for patients with mismatched donors, who historically had poor outcomes.[2] Future studies will compare outcomes in patients receiving transplants with organ donor bone marrow versus traditional hematopoietic stem cell sources, Dr. Woods said.

"These early clinical results provide strong evidence supporting both the safety and efficacy of Ossium's organ donor bone marrow…we're extremely encouraged by the early clinical data, as they demonstrate that organ donor bone marrow performs at least as well as, and potentially better than, conventional hematopoietic stem cell sources," he concluded.

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Pioneering 3D Genomics Diagnostic Tests to Augment Lymphoma Clinical Care, Research

*By **Andrew Moreno***

At Fox Chase Cancer Center, Temple Health's top-tier comprehensive cancer center in Philadelphia, Pennsylvania, the diagnosis technology used for clinical evaluation of lymphomas is making a significant advancement. It is the culmination of a long collaboration with the Arima Genomics, Inc. laboratory in Orlando, Florida, as described in a joint press release from the two institutions.

At the center of this advancement are the laboratory's Aventa tests, which apply Hi-C genomics technology that enables clinicians to examine the whole genome in three dimensions for lymphomas and other malignancies. Clinicians thus gain actionable insights by observing the exact pathological changes in DNA structure associated with cancer.

Tom Willis, PhD

"Arima's Aventa tests provide the most sensitive detection for gene fusions and rearrangements, which are particularly important for the diagnosis and treatment of lymphomas and sarcomas," commented Arima chief executive officer, **Tom Willis, PhD.** "We are thrilled that Fox Chase is showing the leadership to bring these tests to their patients on such a broad scale."

The Cancer Epigenetics Institute at Fox Chase has long held a scientific research partnership with Arima's laboratory. Now, Fox Chase will be the first comprehensive cancer center in the world to extensively incorporate Arima's 3D-genomics technology into its standard protocol to evaluate lymphoma and several other tumors.

Johnathan Whetstine, PhD

"This partnership represents a true bench-to-bedside translation of discovery. Our longstanding relationship with Arima has evolved from basic research collaboration to clinical implementation. Together, we're redefining how genome organization can guide diagnosis and treatment," remarked Cancer Epigenetics Institute director **Johnathan Whetstine, PhD.**

For the Center's clinicians who manage lymphoma, the Aventa tests will help diagnose the disease with greater scrutiny to detect malignant changes than prior evaluation methods, as well as inform treatment selection. The tests' ability to present a malignancy's whole genome for investigators' analysis will also be a boon to treatment development research.

"It allows us to see not just what genetic changes occur, but how the DNA itself is organized, information that may reveal new therapeutic targets or confirm findings with greater precision," Dr. Whetstine elaborated.

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Which Factors Influence Clinical Trial Enrollment for Patients With Cancer?

*By **Melissa Badamo***

Clinical trials offer crucial evidence for the safety and efficacy of investigative cancer treatments—but according to a study published in *JNCCN,* certain patient characteristics may play a larger role in clinical trial participation than others, therefore highlighting the need for greater trial inclusivity.

The retrospective cohort study found that a patient's income and financial resources were stronger predictors of trial participation than geographic proximity or traditional demographic characteristics such as race and ethnicity.

"Our results suggest that clinical trial enrollment is driven less by who patients are and more by what participation demands of them," senior author **Richard Hoehn, MD,** Associate Professor of Surgery at Cleveland Medical Center in Ohio, told *Blood Cancers Today*. "Many patients are not opting out of trials; they are being structurally excluded by the practical burdens of participation."

Richard Hoehn, MD

To identify factors that influence patient enrollment, Dr. Hoehn and colleagues searched electronic medical records of adult patients with cancer treated at University Hospitals in Cleveland. Of those 12,630 patients, 649 (5.1%) participated in clinical trials related to cancer treatment.

The researchers utilized several methods to ensure balance of covariates such as patient age, biological sex, and cancer type between the two groups: patients who participated in clinical trials and those who didn't. These methods include propensity score matching, stepwise logistic regression, and the Boruta machine learning algorithm.

Using the Boruta machine learning algorithm, the researchers identified income, property ownership, and household factors as the strongest predictors of clinical trial enrollment. Meanwhile, race and ethnicity, education/college attendance, and distance to closest relatives had less influence on participation in clinical trials.

According to logistic regression, patients with higher income were 67% more likely to enroll in clinical trials than patients with lower income, and patients with Medicaid were 29% less likely to enroll than patients with private insurance (odds ratio [OR], 0.71; 95% CI, 0.53–0.93).

Non-Hispanic Black patients (OR, 0.70; 95% CI, 0.54–0.89) and patients in other minority groups (OR, 0.57; 95% CI, 0.36–0.88) were less likely to enroll in a clinical trial compared with non-Hispanic White patients. However, after adjusting for income, non-Hispanic Black race was no longer significant (OR, 0.85; 95% CI, 0.64–1.11).

Notably, the authors also found that geographic proximity to clinical trial sites did not strongly deter enrollment, as there were no clear patterns between distance and enrollment rates. Patients residing in areas within a 20-minute driving time of a clinical trial site had a median enrollment rate of 8.0%, compared with 7.6% among patients outside the buffer (P=0.932).

Although the study was limited to a single hospital system in one region, these results emphasize that financial stability plays a critical role in accessing advanced treatment options for cancer. Dr. Hoehn emphasized the importance of improving equity in trial enrollment to enhance generalizability of clinical trial findings.

"Inequitable enrollment limits both fairness and scientific validity," he said. "When trial populations systematically overrepresent patients with greater financial and logistical flexibility, the results may not generalize to the broader population we treat in practice. That can lead to therapies being approved based on evidence that does not fully reflect real-world patients, which ultimately risks widening disparities in outcomes rather than narrowing them."

According to Dr. Hoehn, improving equity in clinical trials requires looking beyond the individual level and focusing on changing systems.

"Interventions that reduce the financial and logistical burden of participation—such as transportation support, flexible scheduling, decentralized or virtual evaluations, and pragmatic trial designs—are likely to be more effective than education alone," he said. "If we want trials to be inclusive, we have to make participation feasible, not just theoretically available."

Reference

Dong W, et al. *JNCCN.* 2025;24(1). Published online December 17, 2025. doi:10.6004/jnccn.2025.7092

Editor's Picks

In each issue of Blood Cancers Today, *we will take a closer look at a particular topic in hematologic malignancies. This month,* ***Mehdi H. Hamadani, MD,*** *Co-Editor-in-Chief of* Blood Cancers Today *and Professor of Medicine and Chief of Hematologic Malignancies at the Medical College of Wisconsin, shares insight on recent research in Transplantation and Cellular Therapy.*

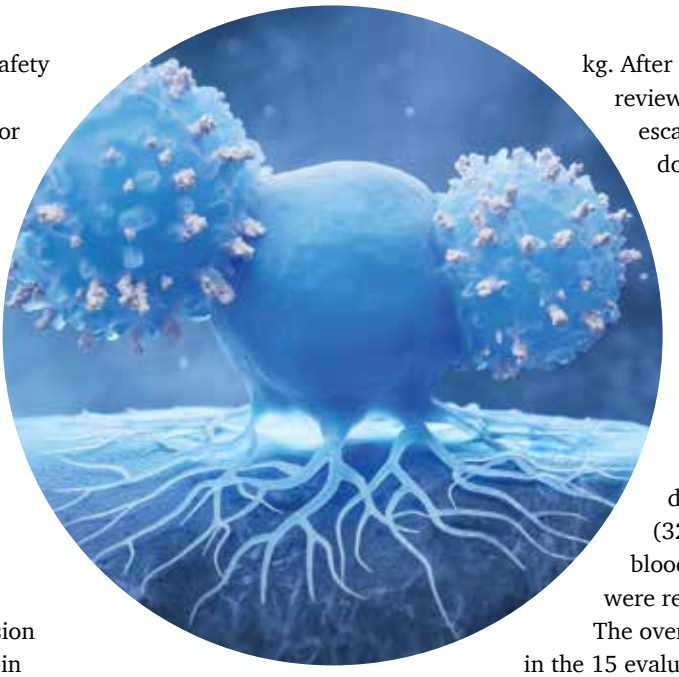
Visit bloodcancerstoday.com to stay up to date on the latest news in each area of hematologic oncology.

Mehdi H. Hamadani, MD

TRANSPLANTATION AND CELLULAR THERAPY

AZD0120 Demonstrates Favorable Safety Profile in Phase1b Trial for Patients With Multiple Myeloma

By ***Lauren Evoy Davis***



A clinical trial evaluated the safety and tolerability of BCMA-directed chimeric antigen receptor (CAR) T-cell therapy for newly diagnosed and relapsed or refractory (R/R) multiple myeloma (MM). Patients who have MM, which makes up approximately 20% of all blood cancers, tend to need several lines of therapy, and R/R MM is common. The investigators presented their findings at the 67th American Society of Hematology (ASH) Annual Meeting & Exposition.

Following on the success of the phase 1 trial of a single infusion of AZD0120 (formerly GC012F) in China, investigators concluded it was safe and demonstrated deep responses, and they moved forward with a phase 1b/2 trial.

Investigators across multiple research centers enrolled 25 patients in the clinical trial and gave them infusions of AZD0120, an autologous BCMA/CD19 dual-targeting CAR T-cell therapy that uses the FasTCAR rapid manufacturing platform.

Eligibility requirements included being at least 18 years old, having a diagnosis of progressive RRMM, receipt of three or more prior lines of therapy (including a proteasome inhibitor, immunomodulatory drug, and anti-CD38 antibody), and an Eastern Cooperative Oncology Group (ECOG) performance status score between 0 and 1. In addition, the trial allowed participants who had previously received BCMA-directed therapy for 6 months or longer to enroll.

Participants underwent lymphodepletion to prepare for a single infusion of AZD0120. One group received 1×10^5 cells/kg, and patients in a second group received 3×10^5 cells/kg. After the first infusion, a safety review committee determined dose escalation and the recommended dose for phase 2 of the trial.

Some patients experienced adverse effects (AEs). These included cytokine release syndrome (CRS) (64%), decreased neutrophil count (56%), and anemia (32%). The most common grade 3 or higher AEs were decreased neutrophil count (52%), decreased lymphocyte count (32%), and decreased white blood cell count (32%). No deaths were reported.

The overall response rate was 100% in the 15 evaluable patients, and the median time to response was 0.9 month for both groups. Three participants in the first group continued to have no measurable residual disease after 12 months.

The investigators concluded that AZD0120 was well tolerated and patients experienced deep responses, which is promising for the future of MM treatment.

Reference

67th American Society of Hematology Annual Meeting & Exposition. Abstract No. abs25-11319.

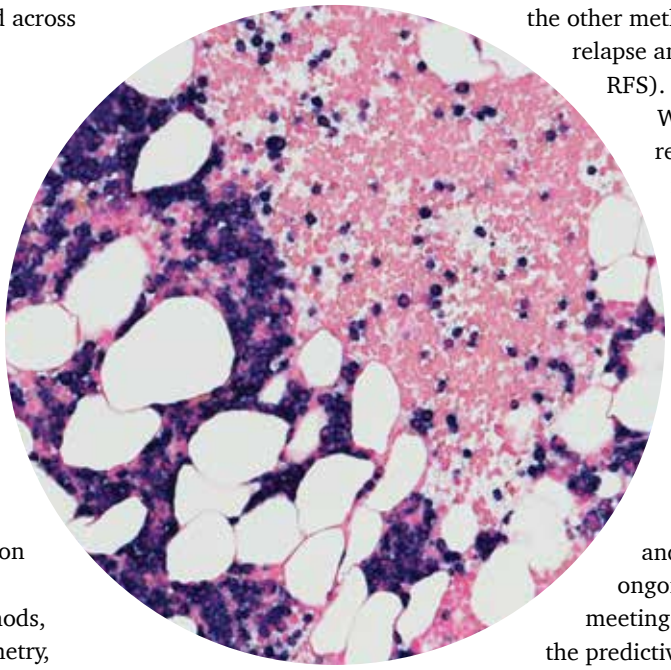
Editor's Insight:

"Preliminary phase 1b data suggest that AZD0120, a FasTCAR-manufactured dual BCMA/CD19 CAR T-cell therapy, is well tolerated with no severe CRS or neurotoxicity and shows encouraging deep responses (100% ORR and high MRD negativity) in heavily pretreated relapsed/refractory multiple myeloma. The early safety and efficacy signal is promising, but longer follow-up and larger cohorts are essential to assess durability and comparative benefit versus existing single BCMA-directed therapies."

NGS-MRD Better Predicts Relapse After Bone Marrow Transplant in B-ALL

*By **Robert Zadotti***

A correlative biology study in patients with B-cell acute lymphoblastic leukemia (B-ALL) has reported favorable results with next-generation sequencing measurable residual disease (NGS-MRD) detection. The main factors observed were relapse, event-free survival (EFS), and overall survival (OS) rates, as compared across intervention practices.



The analysis of the Pediatric Transplantation & Cellular Therapy Consortium (PTCTC) ONC1401 study by a team led by **Amanda K. Johnson, MD, MPH, CPH,** of the University of Utah/Intermountain Primary Children's Hospital, is based upon preexisting research for the "improved predictive power" of NGS and aims to determine its efficacy regarding MRD treatments.

NGS has emerged as a detection technology for MRD, with more accurate results than other methods, such as multichannel flow cytometry, detecting MRD 2-3 logs lower. Dr. Johnson and her team based their predictions on these findings, with their own study based upon the findings of a 2015 subset analysis of the Children's Oncology Group ASCT0431 trial, which showed better results for NGS regarding relapse, EFS, and OS compared with multicolor flow cytometry (MFC) in both pre- and post-hematocrit (HCT).

Utilizing samples from patients transplanted between 2015 and 2021 across nine different US centers, Dr. Johnson and her team hypothesized that BM NGS pre- and post-HCT would be highly predictive of relapse occurrence, relapse-free survival (RFS), and non-relapse mortality (NRM) in patients undergoing HCT for B-ALL and would be a superior detection method to MFC. A secondary hypothesis was that PB NGS would outperform BM MFC and could be a reasonable post-HCT relapse-monitoring approach.

The team's cohort consisted of 121 children and young adults with B-ALL, with a median age of 12 years (range, 1-27). The bone marrow (BM) results displayed a higher sensitivity of NGS, with 27.3% of patients identified as positive MRD by NGS, while only 9.1% of patients had positive results by MFC. NGS BM clearly predicted a higher risk for relapse than either NGS PB or BM MFC (SHR 3.58 (1.33, 9.60) versus 1.83 (0.65, 5.20) versus 1.58 (0.44, 5.71); and inferior rates of RFS (HR 2.00 (1.03, 3.87) versus 1.67 (0.79, 3.55) versus 0.68 (0.21, 2.24), respectively. NGS BM also demonstrated a higher C-index compared with the other methods (0.64 vs 0.58 vs 0.54 for relapse and 0.60 vs 0.56 vs 0.51 for RFS).

While 24 patients in the study relapsed post-HCT, BM NGS MRD managed to predict relapse 5.7x more frequently than BM MFC and more than twice as often as PB NGS. Dr. Johnson and her team believe that key attention to BM NGS MRD defining relapse risk could facilitate planned post-HCT interventions to prevent relapse.

Going forward, Dr. Johnson and her team plan to present ongoing landmark analyses at a meeting and will include assessments of the predictive accuracy of post-HCT BM and PB NGS against BM MFC, as well as the effect of graft-versus-host disease on outcomes. These findings aim to further communicate the advantages of NGS as an MRD detection method for CYA and lead to better intervention practices.

Reference

Johnson A, et al. *Blood*. 146 (Supplement 1): 511. doi:10.1182/blood-2025-511.

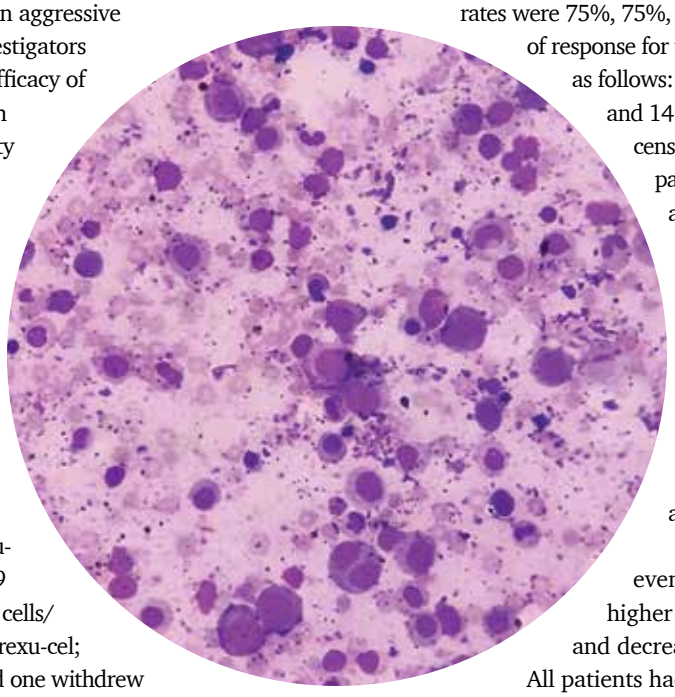
Editor's Insight:

"This prospective multicenter analysis demonstrates that NGS MRD in bone marrow pre-transplant is a stronger predictor of relapse risk and inferior relapse-free survival than both peripheral blood NGS and standard multicolor flow cytometry in children and young adults undergoing allogeneic HSCT for B-ALL. The findings support the potential of high-sensitivity NGS MRD to refine risk stratification and guide pre- and post-transplant interventions, though prospective validation and integration into clinical decision algorithms are needed."

ZUMA-25: Analysis of Brexucabtagene Autoleucel in Patients with Relapsed or Refractory Burkitt's Lymphoma

By ***Lauren Evoy Davis***

In a phase 2 study, investigators evaluated brexucabtagene autoleucel (brexu-cel) in patients with relapsed or refractory (R/R) Burkitt's lymphoma (BL), an aggressive hematologic malignancy. The investigators reported preliminary safety and efficacy of a trial of brexu-cel in patients with RRBL at the 67th American Society of Hematology (ASH) Annual Meeting & Exposition.



The research team initially enrolled 12 patients with RRBL who had already received one line of chemoimmunotherapy. Before the first infusion, two patients died as a result of progressive disease.

All 12 patients underwent leukapheresis, and 10 received an infusion of brexu-cel (2×10^6 anti-CD19 chimeric antigen receptor [CAR] T cells/kg). Two patients did not receive brexu-cel; one died of progressive disease and one withdrew from the study.

The median age of patients was 50.5 years, and 50% were male. At baseline, eight participants had an Eastern Cooperative Oncology Group (ECOG) performance status of 1. All had received prior treatment including chemotherapy, rituximab, or both. One patient had previously received an autologous stem cell transplant. Eight patients received bridging therapy, including chemotherapy, radiotherapy, or both.

The median time from infusion of brexu-cel to first (or best) response was 29 days. One patient went from stable disease to a complete response (CR) at the 9-month mark. None of the participants with a partial response experienced conversion to a CR.

Of the participants with a CR, at 6 months, duration of response (DOR), progression-free survival (PFS), and overall survival (OS) rates were 75%, 75%, and 100%, respectively. Durations of response for the five patients with a CR were as follows: 4.83+, 4.96, 10.18+, 11.33+, and 14.88+ months (+ indicates censoring). At data cutoff, four patients with a CR were alive with a continued response without additional therapy, and one had died of progressive disease after subsequent treatment and a stem cell transplant. For the five patients with a partial response, DOR ranged from 0.03 to 4.24 months; at data cutoff, two were alive with progressive disease, and three had died.

All patients experienced adverse events (AEs). Nine had grade 3 or higher AEs, including pyrexia (100%) and decreased neutrophil count (50%). All patients had any-grade cytokine release syndrome (CRS); one (10%) had grade 3 CRS. Neurologic events occurred in eight patients, and six had grade 3 events. The median peak CAR T-cell level was 41.9 cells/µL, and the median time to reach the peak level was 8 days.

In their conclusion, the investigators point out that although the preliminary data on efficacy and safety show promise for treatment of RRBL, the analysis is limited by the short follow-up period and small number of patients.

Reference

Van Dorp S, et al. 67th American Society of Hematology Annual Meeting & Exposition. Abstract No. 569.

Editor's Insight:

"This phase 2 substudy indicates that brexucabtagene autoleucel delivers a high objective response rate in relapsed/refractory Burkitt lymphoma, a historically chemotherapy- and immunotherapy-resistant disease. Although limited by small sample size and short follow-up, the results support further investigation of CAR-T in aggressive B-cell lymphomas beyond current indications. Longer follow-up is imperative to assess durability of these early responses."

Oscar Lahoud, MD, Expands Blood Cancer Care as Chief Medical Officer of NYU Langone Hospital–Brooklyn

*By **Melissa Badamo***

As the newly appointed Chief Medical Officer of NYU Langone Hospital–Brooklyn, **Oscar Lahoud, MD,** aims to bring novel, innovative therapies to patients with blood cancers and beyond in the most populated New York City borough.


Oscar Lahoud, MD

In this new role, Dr. Lahoud will collaborate with section chiefs at NYU Langone Hospital–Brooklyn to ensure high-quality, accessible care for all patients without the need for travel.

"My goal is to help bring the highest caliber of academic medicine to the borough," Dr. Lahoud told *Blood Cancers Today*. "Brooklyn is one of the top three most populated cities in the country, excluding the other boroughs, yet very few academic health centers have invested in the borough itself. Our goal is to be one of the global leaders so that people would never have to travel for any part of their care outside of the borough."

An expert in hematological malignancies, Dr. Lahoud specializes in bone marrow stem cell transplantation and cellular therapies for patients with lymphoma, leukemia, myelodysplastic syndrome, myeloproliferative neoplasms, and multiple myeloma. He joined NYU Langone Hospital–Brooklyn in 2024 as Section Chief of Hematology, later becoming Director for Strategy and Network Operation then Deputy Chief Clinical Officer at the Perlmutter Cancer Center.

He previously served as Senior Medical Director for Strategic Partnerships at Memorial Sloan Kettering Cancer Center, where he was instrumental in expanding bone marrow transplant access in the New York metropolitan area through the Opening Stem Cell Access to the Regionals (OSCAR) program.

"For people who undergo bone marrow transplant or CAR [chimeric antigen receptor] T-cell therapy, the frequency of visits after transplant can be very high. It can be weekly or twice a week for several months," Dr. Lahoud explained. "I helped build partnerships with local community providers across the New York metropolitan area."

Despite these efforts, patients still had to travel to Manhattan for bone marrow transplant or a major surgery, Dr. Lahoud noted.

"I saw an opportunity to bring an unmet need to the borough," he added. "The opportunity at NYU presented itself with a very shared vision."

Dr. Lahoud and his team are in the process of opening a bone marrow transplantation and CAR T-cell therapy center in Brooklyn, slated for 2027. He also continues to expand access to clinical trials, noting that 90% of patients enrolled at his center are from underrepresented minorities.

"Historically, that is a patient population described as 'underserved' or had little access to therapies," he added. "Now, they have access to that. None of this is offered at any other health center in the borough."

Dr. Lahoud has led several clinical trials on cell therapies in blood cancers, including the phase 3 iMMagine-3 trial of anitocabtagene autoleucel (anito-cel) for patients with relapsed or refractory multiple myeloma who received one to three prior lines of therapy. The trial is enrolling patients across NYU Langone's health system in Brooklyn, Long Island, and Manhattan.

"Anito-cel has a unique construct and a much higher transduction efficiency, which seems to be associated with more on-target effect, so potentially more enhanced efficacy with less toxicity," Dr. Lahoud explained. "We've been very successful at enrolling patients on the study, and the very first patient enrolled and randomized was actually one of my patients from Brooklyn. That was humbling and rewarding, because nobody else has given patients from the borough access to innovative trials."

Photo Courtesy of NYU Langone/Joe Carrotta

Dr. Lahoud also launched an investigator-initiated trial on a prophylactic intervention to eliminate the risk for bispecific antibody-associated cytokine release syndrome and immune effector cell-associated neurotoxicity. By reducing these toxicities, he hopes to increase access to bispecifics not only for patients in Brooklyn, but also in rural communities.

"The values of NYU have always been to provide outstanding care regardless of who you are, where you come from, and where you live," he concluded. "That vision is what attracted me to NYU, to be world-class leader right here in Brooklyn."

Leo Wang, MD, Appointed Division Chief of Cell, Gene, and Transplantation Therapy at City of Hope

*By **Melissa Badamo***

With more than a decade of professional experience as a pediatric hematologist-oncologist and immunologist, **Leo Wang, MD,** has stepped into a new role as division chief of Cell, Gene, and Transplantation Therapy at City of Hope in Duarte, California.

Leo Wang, MD

In this new role, Dr. Wang will help accelerate the development of novel, life-saving cellular therapies for pediatric patients at City of Hope and beyond.

"At City of Hope Children's Cancer Center, we have very long been committed to bringing better therapies faster and more efficiently to children with cancer and other life-threatening diseases," Dr. Wang told *Blood Cancers Today*. "We have a long legacy of bringing promising medicines to kids as quickly and safely as possible. I'm excited to build on that and expand the portfolio of cutting-edge therapies that are available to our patients."

Dr. Wang also leads City of Hope's Pediatric Immunotherapy Research Laboratory, where he conducts clinical trials of gene therapies and chimeric antigen receptor (CAR) T-cell therapy to treat graft-versus-host disease, blood cancers, and other malignancies. In the field of pediatric blood cancers, **Lindsey Murphy, MD,** an assistant professor in the Department of Pediatrics at City of Hope, is also leading innovative trials of CAR T-cell therapy for acute lymphocytic leukemia (ALL).

"For instance, we are very excited about potentially opening a clinical trial to target BAFF-R in relapsed ALL patients," Dr. Wang stated. "We are also testing new CD19-targeted immunotherapies, which have recently made a huge difference in the treatment of pediatric ALL management."

Outside of hematology-oncology, Dr. Wang launched a new clinical trial of CAR T-cell therapy for children with aggressive brain tumors.

"We take the T cells out of the patient, reprogram them to recognize and kill cancer cells, and then put them back into the brain," he explained.

Ultimately, Dr. Wang hopes to translate the success of cellular therapy in blood cancers to brain cancer following the FDA approvals of ciltacabtagene autoleucel, lisocabtagene maraleucel, and other immunotherapy agents.

"Our T-cell therapy has radically transformed how we think about and treat blood cancers," he said. "It's been one of the biggest success stories of the past 10 years. Many of us have shifted focus to try to bring that revolution to solid tumors. There are lots of differences between solid tumors and blood tumors. We're trying to address those in the lab."

Moving forward, Dr. Wang has one major goal: to bring better therapies to patients faster, more efficiently, and more equitably.

"Being a pediatric hematologist-oncologist has been a tremendous honor and a privilege," he added. "Walking with families as they grapple with the worst thing to happen to them as a family and to see their strength, their grace, and the joy they're able to summon during that process is humbling, awe inspiring, and incredibly motivating. That has made this truly a vocation for me. I get to bring the gift of that motivation back to the lab, drive discovery, and create better therapies, then bring those therapies back into the clinic to patients who need them."

Hematologist-Oncologists Recognized at 2026 Tandem Meetings

*By **Melissa Badamo***

It's officially awards season in the field of transplantation and cellular therapy as the American Society for Transplantation and Cellular Therapy (ASTCT) and Center for International Blood and Marrow Transplant Research (CIBMTR) rang in their annual Tandem Meetings, February 4-7, 2026, in Salt Lake City, Utah.

This year, two hematologist-oncologists were honored at the annual meeting, which focused on presenting the latest developments in transplantation and cellular therapy.[1]

ASTCT E. Donnall Thomas Award

James Ferrara, MD, received the E. Donnall Thomas Award for his contributions to the advancement of knowledge in blood and marrow transplantation. Dr. Ferrara currently serves as the Ward-Coleman Professor of Cancer Medicine and Director of the Center for Translational Research in Hematologic Malignancies at the Icahn School of Medicine at Mount Sinai in New York City, where he conducts clinical trials on graft-versus-host-disease (GVHD) treatment.[1]

James Ferrara, MD

Dr. Ferrara was a part of a research team studying the role of RIP1 inhibition in improving immune reconstitution and reducing GVHD mortality, highlighting its potential as a non-immunosuppressive treatment.[2] He also founded the Mount Sinai Acute GVHD International Consortium (MAGIC), a biorepository that identifies biomarkers to predict patient outcomes and guide GVHD therapy.[1]

(continued on next page)

ASTCT Lifetime Achievement Award

Robert Negrin, MD

Robert Negrin, MD, received the ASTCT Lifetime Achievement Award for his contributions to the field of bone marrow transplant and cellular therapy. He is a professor of medicine and former chief of the Division of Blood and Marrow Transplantation and Cellular Therapy at Stanford University from 2000 to 2020, and he currently serves as 2026 President of the American Society of Hematology (ASH).[1]

With more than 300 published manuscripts, his research focuses on understanding immune-mediated reactions and GVHD in patients with hematologic malignancies. Most recently, he was an investigator in the phase 3 PRECISION-T trial, in which Orca-T improved survival without moderate-to-severe chronic GVHD compared with allogeneic hematopoietic stem cell transplant.[3]

"Being selected to receive the Lifetime Achievement Award from the American Society of Transplantation and Cellular Therapy is a huge honor," Dr. Negrin told *Blood Cancers Today*. "However, an honor like this highlights the many contributions of a large number of scientists and physicians that I have had the privilege to work with over many years. This award is for them as much as it is for me. We are all deeply grateful."

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2. Prado-Acosta M, et al. *Sci Transl Med.* 2023;15(727):eadf8366. doi:10.1126/scitranslmed.adf8366
3. Meyer E, et al. *Blood.* 2025031313. Published online December 12, 2025. doi:10.1182/blood.2025031313

Married Couple Wins Gretener-Thürlemann Prize for Decades of Immunotherapy Research

By ***Sara Karlovitch***

A husband-and-wife team whose work has contributed significantly to the development of immune checkpoint inhibitors received the University of Zurich's first-ever Gretener-Thürlemann Prize.[1]

Gordon Freeman, MD

Arlene Sharpe, MD, PhD

Gordon Freeman, PhD, a professor of medicine at Dana-Farber Cancer Institute and Harvard Medical School, and **Arlene Sharpe, MD, PhD,** the Kolokotrones University Professor and chair of the Department of Immunology at Harvard Medical School, were honored with a prize worth half a million Swiss Francs, approximately $625,000 in US dollars. Married since 1978, the duo has worked together since 1980 and published their first joint paper in 1993 in *Science*, which pertained to their work on B7.[1]

"Winning this award together is incredibly meaningful, both professionally and personally. As a married couple, we've shared a long journey in science, and this recognition feels like a celebration of that shared commitment as much as of the work itself," said Dr. Freeman.

Notably, the couple's research showed that the PD-L1 and PD-L2 proteins produced by cancer cells can interact with PD-1. A 2000 article co-published by Dr. Freeman in the *Journal of Experimental Medicine* found that when PD-L1 binds to PD-1 T-cell receptors, it inhibits T-cell activity by activating an inhibitory pathway. Thus, immune responses that would otherwise attack the tumor do not.[2]

The research spurred the development of checkpoint inhibitors, which help restore the immune system's ability to recognize and attack cancer cells. More than 25 cancer types use this therapy class, including B-cell lymphoma and Hodgkin lymphoma.[3]

Drs. Sharpe and Freeman, whose laboratories are only a 10-minute walk from each other in the Longwood Medical Area in Boston, are currently working to further understand the signaling pathways of the immune system. They hope to discover additional genes and molecules involved in the process, potentially expanding the treatment population who could benefit from PD-L1/PD-1 inhibitors. Technological advancements in artificial intelligence and single-cell analysis could accelerate development in this area, eventually leading to novel therapies.

"I believe cancer immunotherapy will advance both incrementally and in transformative ways. At present, PD-1/PD-L1 inhibitors benefit roughly 20% of patients with solid tumors," said Dr. Freeman. "In the near term, I expect incremental improvements in response rates, potentially pushing them toward 30%."

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2. Freeman G, et al. *J Exp Med.* 2000;192(7):1027-1034. doi:10.1084/jem.192.7.1027
3. Boldt C. Accessed January 26, 2026. https://www.mdanderson.org/cancerwise/what-cancers-can-be-treated-with-immunotherapy.h00-159695178.html

Calendar

April 10–11
The International Ultmann Chicago Lymphoma Symposium
Chicago, Illinois

April 17–19
Clinical Multidisciplinary Hematology & Oncology: The 20th Annual Review – 2026
Scottsdale, AZ

April 17–22
American Association for Cancer Research (AACR) Annual Meeting 2026
San Diego, CA

April 19–21
24th CML Horizons Conference
Tbilisi, Georgia

April 29–May 2
2026 American Society of Pediatric Hematology/Oncology (ASPHO) Conference
Minneapolis, MN

May 29–June 2
2026 American Society of Clinical Oncology (ASCO) Annual Meeting
Chicago, IL

June 8–9
22nd Global Summit on Hematology and Blood Disorders
Dubai, UAE

June 11–14
European Hematology Association (EHA) 2026 Congress
Stockholm, Sweden

PHYSICIAN'S WEEKLY

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