WITHIN REACH

HEMATOLOGY-ONCOLOGY IN FOCUS: Targeted therapies reaching the community

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IF YOU READ ONE PAGE, LET IT BE THIS ONE.

55%

of Americans receive cancer treatment in community settings.[1]

60%

of patients referred for CAR-T treatment actually receive it.[2]

73%

of healthcare professionals report a gap between the availability of novel treatments and successful use of them in clinical practice.[1]

3/4

oncologists are overwhelmed by the pace of treatment innovation.[1]

An oncologist sits in another consult. This morning, she's faced – yet again – with a list of impossible choices. Because for the patient across from her, like so many she's seen before, she'll be forced to balance an impossible equation to move forward with a treatment plan.

Clinical data. The patient's prognosis. Their insurance coverage. Where they live. Family dynamics. Whether they'll even get a seat in the apheresis chair before their disease progresses... on, and on.

Her patient is desperate for answers. This is what doomscrolling truly looks like, in an increasingly confusing digital age in which AI is just beginning to further muddy the waters. Treatment costs and coverage. Mental health support. Relationship tips for navigating the five-ton strain that cancer has put on their marriage. And is it even worth it? Being away from their family with the crucial time they have left?

And don't forget the caregiver, ever-nearer to their own breaking point. They've taken up the burden where they can, but the tolls are serious. Financial pressure – do we have the money now? Will there be any money left... after? Anxiety. Fear. Paperwork. Hours on the phone navigating an incomprehensible healthcare system. A to-do list that only changes and never gets shorter, with no one to turn to. The burden of a caregiver is a unique, singular, quiet suffering.

In hemato-oncology, these are the issues. Each of them sits in their own space in a much broader world that can only be described as an ever-shifting, complex dance of forces. With scientific, systems, and societal factors colliding, to stall innovation.

But we are here to make sense of it all. To break it down, identify each part of the equation and solve for what can be solved.

Welcome to 'Hematology-oncology in focus', Avalere Health's flagship showcase report asking why targeted treatments aren't reaching every patient possible and what we can do differently in the biopharmaceutical industry to ensure innovations turn into therapies that change care.

This showcase report is part of 'Within Reach' – Avalere Health's ongoing anthology built on a simple truth:

THAT NO SINGLE STORY CAN CAPTURE THE REALITY OF CANCER.

At its heart, 'Within Reach' is driven by the voices that matter most. By placing patient and stakeholder experiences at the center, we challenge assumptions and reframe what meaningful impact looks like. The result is a richer, more human understanding of oncology: one that equips decision-makers to act with precision and purpose.

> “Biopharmaceutical companies launching hemato-oncology products have missed an opportunity. They’ve tackled each of the domains of science, society, and systems independently rather than as a single interconnected ecosystem.”
>
> **– Amar Urhekar,**
> *CEO, Avalere Health*

EVERY PATIENT POSSIBLE IS NOW WITHIN REACH

We are living through one of medicine’s most extraordinary moments. Targeted hemato-oncology treatments that were once the realms of science fiction are transforming lives. The pipeline has never been more promising. And yet, too many patients remain out of reach.

Historically, biopharmaceutical companies launching hemato-oncology products have missed an opportunity to look at the complete picture. Tackling each of the domains of science, society, and systems independently rather than as one interconnected ecosystem.

Clinical trial strategies developed in isolation. Patient engagement plans disconnected from access realities. Support programs that sit in one corner of the organization, while market access strategies sit firmly in another. Each team doing important work. None of them working as one.

In straightforward therapeutic categories, that fragmentation is inefficient. In complex hem-onc environments, it can be fatal.

The real barriers to treatment and care don’t live neatly within any single domain. They emerge in the tensions between them. That’s where patients fall through the cracks. And that is precisely where biopharmaceutical companies have the greatest opportunity to make a difference.

This ‘Within Reach’ showcase helps lay the groundwork in answering that challenge. Because in a world where everyone is used to soundbites and snippets, we believe this topic deserves deep interrogation.

Drawing on the collective expertise of hemato-oncology specialists across our Advisory, Medical, and Marketing practices, we’ve gone beyond the data to examine what’s really holding patients back – and what it will take to change it.

We spotlight the tensions with real patient stories and leverage our *SenseMaking* strategic framework to explore where science, systems, and society collide. We uncover not just what the evidence says, but what it enables, who it reaches, and who it leaves behind.

BECAUSE SCIENTIFIC BREAKTHROUGHS ONLY MATTER IF THEY REACH EVERY PATIENT POSSIBLE. RIGHT NOW, TOO OFTEN, THEY DON’T.

– Amar Urhekar,
CEO, Avalere Health

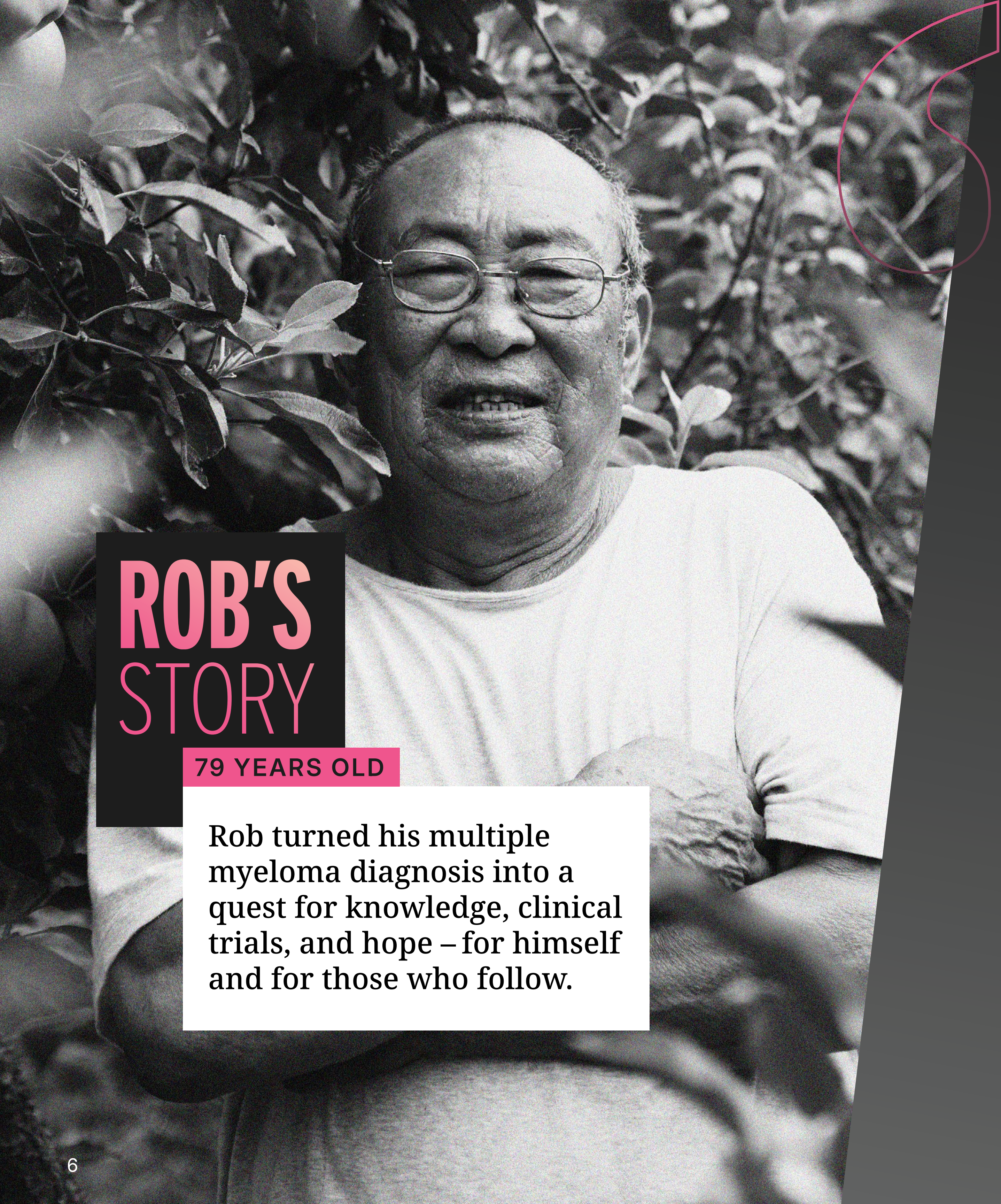
GETTING TO THE HEART OF THE TENSIONS

PART I

SCIENTIFIC BARRIERS

WHERE TENSIONS COLLIDE

The blood cancer space is intensely competitive. While the science is moving at lightning speed, on the ground, healthcare professionals are struggling to keep up. It's brands that provide clarity amid complexity – simplifying the treatment journey – that will capture the minds and hearts of physicians and patients.



ROB'S STORY

79 YEARS OLD

Rob turned his multiple myeloma diagnosis into a quest for knowledge, clinical trials, and hope – for himself and for those who follow.

> *The doctor came in and told us, "You've got multiple myeloma. You'd better get your house in order. You don't have long."*

We were coming back from a cruise, and I developed a high fever on the plane back. My wife was packing me with ice packs to try to get my temperature down. As soon as we landed, we immediately headed to the emergency room.

At first, the doctors thought it was an infectious disease. Then, they thought it was my heart. It wasn't until they discovered I had anemia and checked my bone marrow that they diagnosed me with multiple myeloma.

The doctor who diagnosed me wasn't a specialist. I left the hospital with a lot of questions. I wondered 'What in the world is multiple myeloma?'

Fortunately, my instinct was to start researching. There were just a couple of hospitals with multiple myeloma specialists in my local area. My first doctor hooked me up with a stem cell transplant to buy me some time. It was very rapid. Within a year, I went from diagnosis to stem cell treatment, including the bridging treatment in between.

I was feeling good, so I decided to move closer to my daughter. However, I knew my multiple myeloma wasn't cured. I needed to find a new specialist. I found a very bright oncologist on the leading edge of clinical trials. I told him: 'I'm willing to do whatever helps other people.'

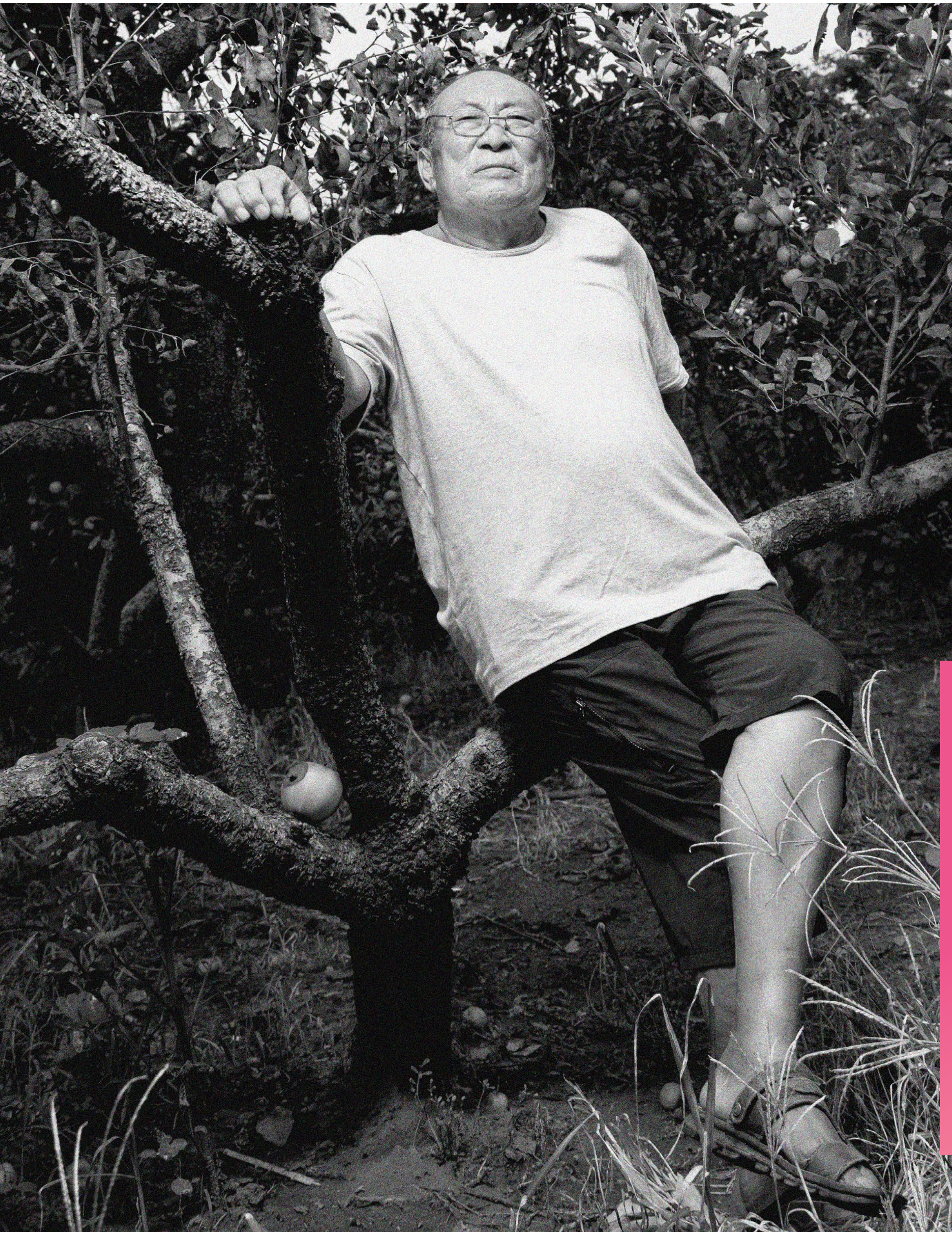
I've been on three clinical trials. Science has come a long way in the last few years. I'm lucky because there are four multiple myeloma specialists at my hospital now – they take a team approach to treatment.

I feel for those in rural areas or who have no access to a specialist hematologist or an oncologist who has no idea what they're doing, so they can't get the best treatment.

I've joined a bunch of patient groups. When I go to meetings, I listen out for advice on what to do when this treatment ends and if I go out of remission. The question is: do I go for another bispecific or do I go for a CAR-T? I'm always searching for the next possibility.

I've looked at CAR-T very seriously, and I've talked to my current provider. I'm 79 years old; I've responded well to a bispecific, so perhaps another one would work too. I think we'll cross that bridge if we come to it.

I'M A FIRM BELIEVER IN RESEARCH. I'M A FIRM BELIEVER IN FINDING A CURE. If you're willing to take the chance of trying a clinical trial, go for it. Of the ones I've been involved with, some have been successful, some have not, but trials advance the whole disease prevention issue and the chance of finding a cure. Without those trials, we would never get there.



No patient should leave a hospital unclear about their disease...

IN OUR EXPERIENCE...

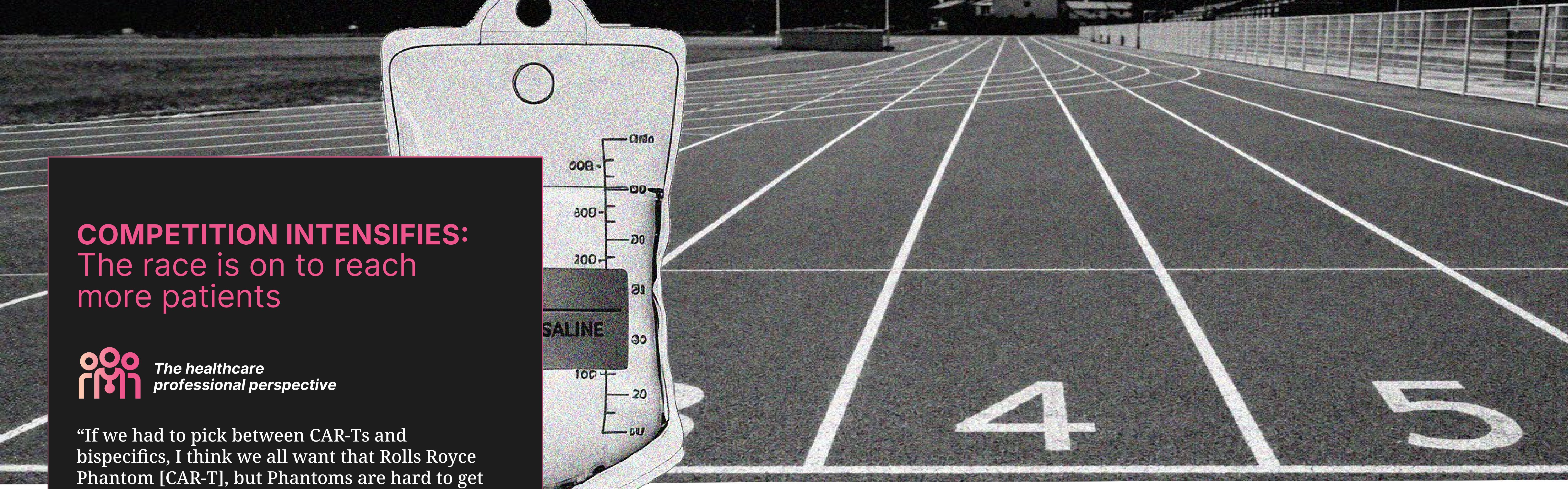
"What strikes me about Rob's story is his questions about his next phase of treatment. If pharma companies want treatments like CAR-T therapies to be considered for a broad spectrum of patients, they need to carefully consider how they communicate data. When a healthcare professional sees an idealized patient profile based on narrow clinical trial eligibility, they may hesitate to make a referral. Producing real-world evidence focused on the patient populations most likely to benefit from therapy, and sharing insights from both key opinion leaders and community oncologists on the realities of the treatment process, can help shift perspectives and drive clarity."

–Jeff Bergen,

PhD, Scientific Director, Medical, Avalere Health

MEET **JEFF**

Jeff has 11 years' experience in medical communications, supported by more than a decade of scientific research in immunology, T-cell biology, and vaccines. His experience spans immuno-oncology, hematologic malignancies, and solid tumors. A client-focused scientific lead with a strong editorial background, Jeff is currently focused on CAR T-cell publications. He specializes in high-quality scientific publications, congress support, and medical affairs deliverables across the product lifecycle.



COMPETITION INTENSIFIES: The race is on to reach more patients

The healthcare professional perspective

> “If we had to pick between CAR-Ts and bispecifics, I think we all want that Rolls Royce Phantom [CAR-T], but Phantoms are hard to get and until [we can get them] it’s the [bispecifics] that make the world go round.”
>
> **– Ajai Chari,**
> *MD, Director of the Multiple Myeloma Program and a Professor of Clinical Medicine at the University of California, San Francisco*[3]

The hemato-oncology landscape is rapidly evolving into a high-stakes competition where bispecific antibodies, trispecific constructs, and CAR-T therapies are redefining standards of care and commercial potential.

FOR ALMOST A DECADE, CAR-T TREATMENTS HAVE DOMINATED THE HEMATOLOGIC CANCER SPACE AS THE MOST PROMISING TREATMENTS FOR DRIVING A DURABLE RESPONSE in patients with B-cell malignancies, such as relapsed/ refractory multiple myeloma and Diffuse Large B-Cell Lymphoma. However, bispecific antibodies are fast gaining popularity with oncologists and patients due to their ability to drive deep responses and off-the-shelf accessibility.

Although bispecific antibodies show a less durable response than CAR-T therapy, with patients requiring more rounds of treatment, they can be delivered in outpatient environments and require less extensive monitoring, overcoming several access hurdles for patients and clinicians. As a result, many are wondering whether these novel immunotherapies could not only eventually rival the efficacy of CAR-T treatments but also meet healthcare professionals’ and patients’ demands for a simpler, more cost-effective experience.

Trispecific platforms, still in clinical development, are now pushing the envelope further, simultaneously engaging multiple tumor markers to overcome resistance mechanisms and amplify potency.

IN OUR EXPERIENCE...

> Behind every treatment decision is a real person navigating fear, hope, and difficult choices. The drugs that will truly make an impact aren't just winning a commercial battle – they're asking how we can improve the journey for a patient like Rob and for every oncologist looking to make a decision that leads to the best possible outcome for their patients.”

– Jessica Holzhauer,

Director, Scientific Strategy, Medical, Avalere Health

MEET JESSICA

Jessica has spent more than a decade working in oncology. As a strategic medical affairs director, she is skilled in communicating complex science about cutting-edge cancer treatments in a relatable way, understanding the importance of the caregiver in shared decision-making, and putting the patients first.

MEET CLARE

Clare has more than 25 years of experience in research, development, and commercialization of biotechnology and medical devices. She has worked across the product lifecycle, leading strategy and research, specializing in market access. She was the recipient of the Precision Cancer Consortium Wayfinder Award in 2025 for her work to drive access to comprehensive genomic profiling for all patients, regardless of location or background.

> With more manufacturers launching multiple treatments within the same tumor types, a crystal-clear portfolio positioning approach is vital. It’s about creating a clear patient pathway – who gets what, and when – and generating evidence that optimizes transitions between treatments, minimizes cross-resistance, and maximizes lifetime patient benefit. Companies that align clinical development, biomarker strategy, and market access around this sequencing logic will be best positioned to capture value across the treatment continuum and avoid cannibalizing their own innovations.”

– Clare Jones,

Executive Director, Global Market Access, Advisory, Avalere Health

BRIDGING THE GAP:
From breakthrough science to real-world care

The healthcare professional perspective

> “What happens after early CAR T-cell therapy relapse? Our salvage options and sequencing aren’t clearly defined. Do we go to bispecific antibodies? Do we return to traditional regimens? These decisions need clearer guidance.”

– Prerna Mewawalla, MD[4]

Patients with blood cancers face a challenging journey, receiving several lines of treatment from chemotherapy and radiation to stem cell transplants and targeted medicines. This complexity is compounded by the rise in combination treatments, reducing the likelihood that cancer cells will evade single-target or single-action therapies. Bridging treatment turns a multi-step journey into a moving puzzle, where timing, sequencing, and patient fitness all have to line up carefully.

The result is a complicated environment for oncologists who are navigating sequencing across multiple lines of treatment. Knowing when to treat a patient and with which therapy is becoming an incredibly long equation filled with costly compromise at every step of the way.

Gaps are fueled by a lack of guidance, awareness of patient resources, and real-world experience with novel therapies – including feedback on outcomes and exposure to patients with diseases like multiple myeloma and large B-cell lymphoma.

Critical expertise on sequencing, managing immune-related toxicities, complex monitoring, and anticipating resistance, is concentrated in academic centers, leaving community clinicians to make high-stakes decisions with limited real-world guidance and without the immediate support of a multidisciplinary team. This lack of clarity can lead clinicians to default to familiar treatments rather than the targeted, layered strategies patients need.

> “Community oncologists aren’t referring patients for CAR-T treatments all the time. They have concerns: unfamiliar territory, losing continuity with their patients, and uncertainty about what happens after treatment. When oncologists are shown a fuller picture – how treatment works, what their role looks like afterwards, and that their patient will come back to them – they are more likely to refer with confidence.”

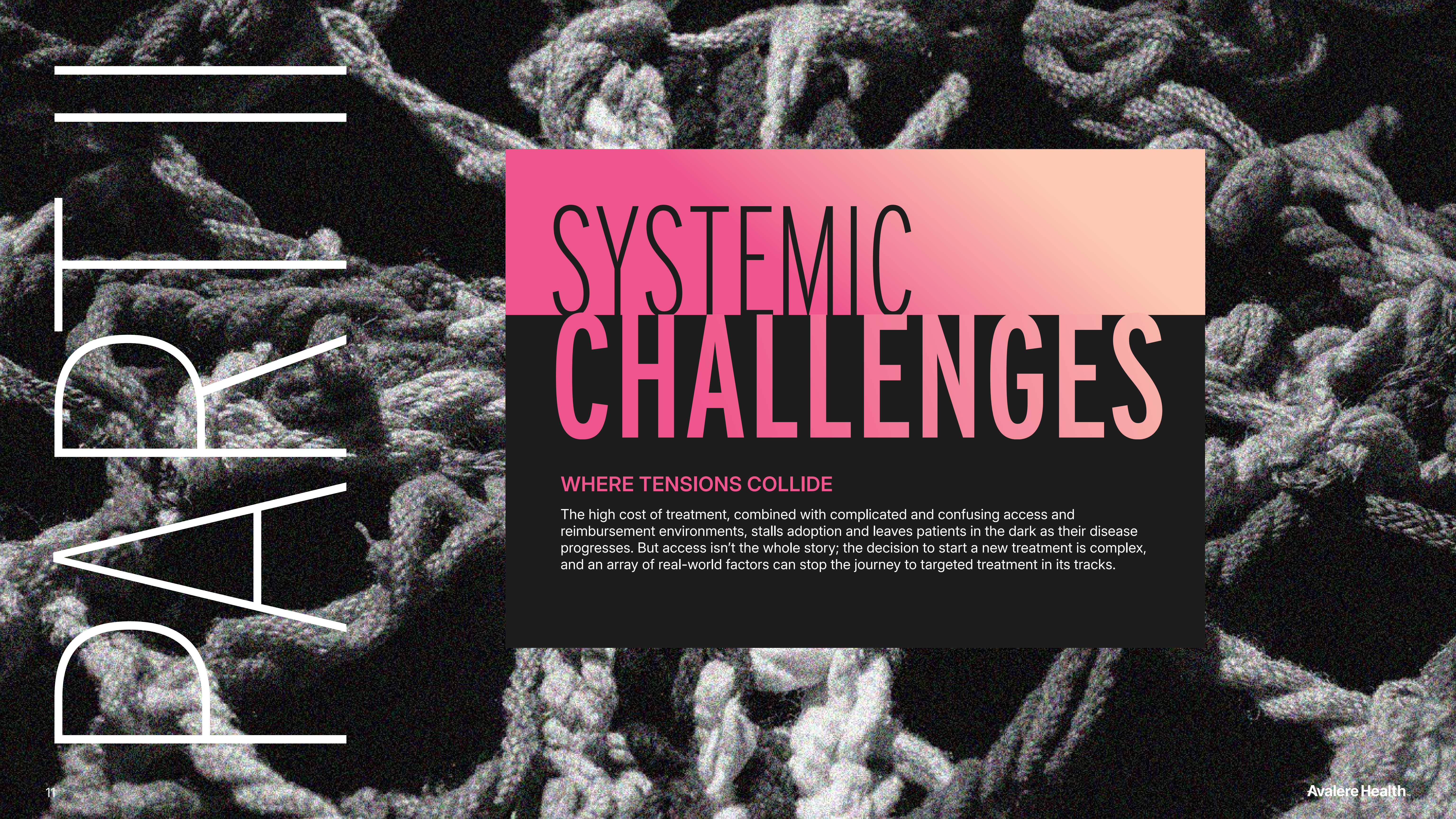
– Jeff Bergen,

PhD, Scientific Director, Medical, Avalere Health

> "As new treatments become available and conversations start to turn from prolonging life to the whisper of a cure, new challenges will emerge. Clinical trials need to be designed with the definition of a “cure” in mind. When will healthcare professionals know a patient is “cured?” When can they stop treatment, if ever? These are the answers healthcare professionals will need to understand treatment duration, feel comfortable managing patient expectations, and eventually de-escalate or discontinue treatment."

– Jessica Holzhauer,

Director, Scientific Strategy, Medical,
Avalere Health



PART II

SYSTEMIC CHALLENGES

WHERE TENSIONS COLLIDE

The high cost of treatment, combined with complicated and confusing access and reimbursement environments, stalls adoption and leaves patients in the dark as their disease progresses. But access isn't the whole story; the decision to start a new treatment is complex, and an array of real-world factors can stop the journey to targeted treatment in its tracks.

KAREN'S STORY

Karen, a patient with multiple myeloma, went from unexplained fatigue and a back injury to navigating stem cell therapy and ultimately receiving CAR-T treatment. Now in remission, she shares her journey of hope, persistence, and survival.

> *I had absolutely no multiple myeloma symptoms other than fatigue. I just assumed that I was getting older.*

My husband and I were moving a treadmill into our house, and I injured my back lifting it up. I immediately lost about three inches of height.

At first, we thought it was a minor back injury. I saw a nurse practitioner who sent me for physical therapy. When I finally had an appointment with a doctor, she told me that I had shattered several vertebrae in my back and that she suspected I could have multiple myeloma. I had never heard of multiple myeloma in my entire life.

The doctor told us not to Google it, but of course we did. The information on Google in 2021 was not up to date. It sounded like an absolute death sentence.

In December 2021, I started stem cell treatment. I was worried because I felt like I was jumping to the last-case scenario, but I was assured that wasn't the case. I was a healthy person on no medication at all before, and then I was swamped with a lot of medications – it was very overwhelming.

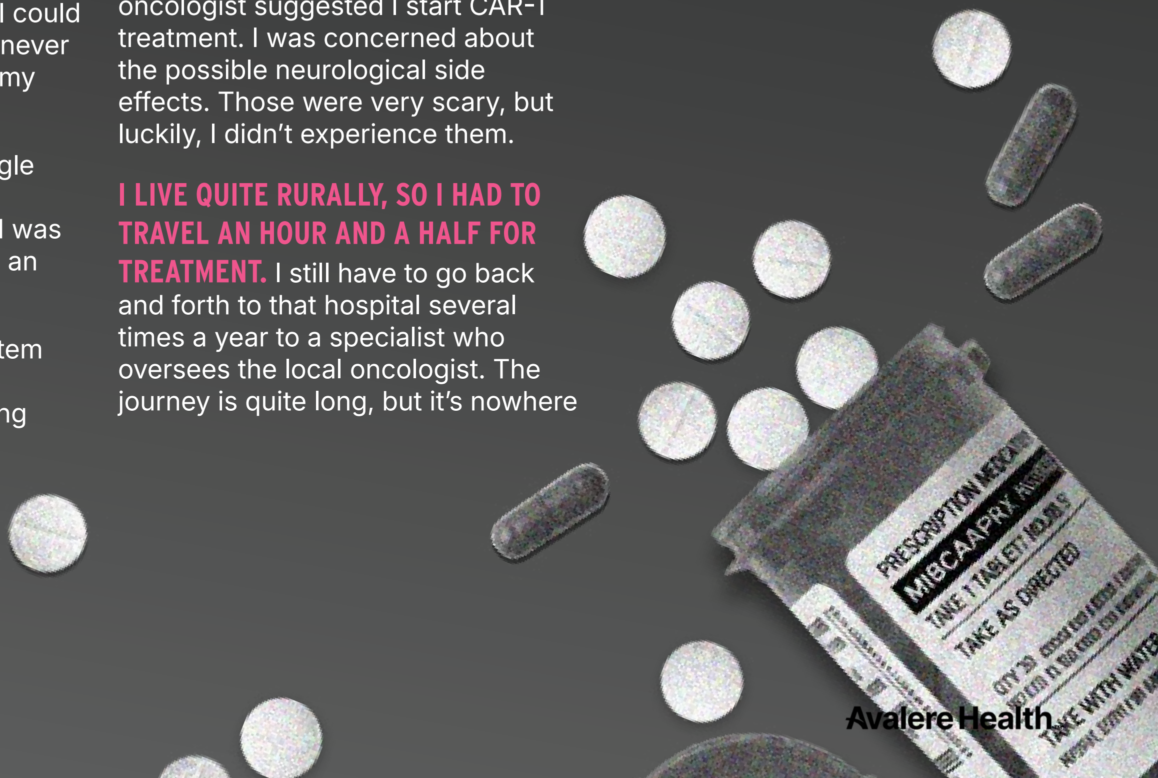
My insurance carrier discovered the hospital I was dealing with wasn't certified as a center of excellence, which derailed my treatment. I had to travel to a different clinic, which didn't store cells for a repeated stem cell transplant. That meant, when I relapsed a year and a half later, I lost my chance for a second round of stem cell treatment.

The next step on my journey was treatment with a proteasome inhibitor. Then, in 2024, my oncologist suggested I start CAR-T treatment. I was concerned about the possible neurological side effects. Those were very scary, but luckily, I didn't experience them.

I LIVE QUITE RURALLY, SO I HAD TO TRAVEL AN HOUR AND A HALF FOR TREATMENT. I still have to go back and forth to that hospital several times a year to a specialist who oversees the local oncologist. The journey is quite long, but it's nowhere near as far as some people travel for treatment, so I'm quite fortunate.

I feel really lucky to have had CAR-T treatment. When I was first diagnosed, it wasn't readily available. It was almost talked about like a cure. Everybody wanted it, and people were passing away and never getting the opportunity to have it. Some people were getting it through clinical trials, but they were struggling and fighting each other for access.

By the time I got treatment, the conversation had changed from mentions of a cure to one around the data showing that people have managed to go three years in remission.



> *Progress only counts if patients survive long enough to benefit from it...*

IN OUR EXPERIENCE...

"Karen's story is a powerful illustration of just how many challenges patients face between diagnosis and the right treatment – and how much rides on decisions that patients have no control over. Many of the barriers she spoke of were systemic. The science existed. But the treatment journey was harder than it needed to be. Progress only counts if patients survive long enough to benefit from it, and if the system is equipped to deliver it equitably. Our job is to make sure the system that failed her in those early years is not the system the next patient encounters. The expansion of CAR-T treatment to the community setting gets patients one step closer to accessing care close to home. "

–Maddi Davidson,
Principal, Advisory, Avalere Health

MEET **MADDI**

Maddi has 10+ years of experience in oncology payment and healthcare policy. She leads oncology work focused on alternative payment models, the evolving landscape, and policy areas, including the Inflation Reduction Act (IRA) and Medicare Part B reimbursement. Maddi leads market access analytics to drive strategic insights and improve patient access. Her commitment to advancing cancer care and health equity earned her a spot among other leaders, winning the 2025 Association of Value-Based Cancer Care's 40 Under 40 in Cancer award for policy and patient advocacy.

GEOGRAPHY:

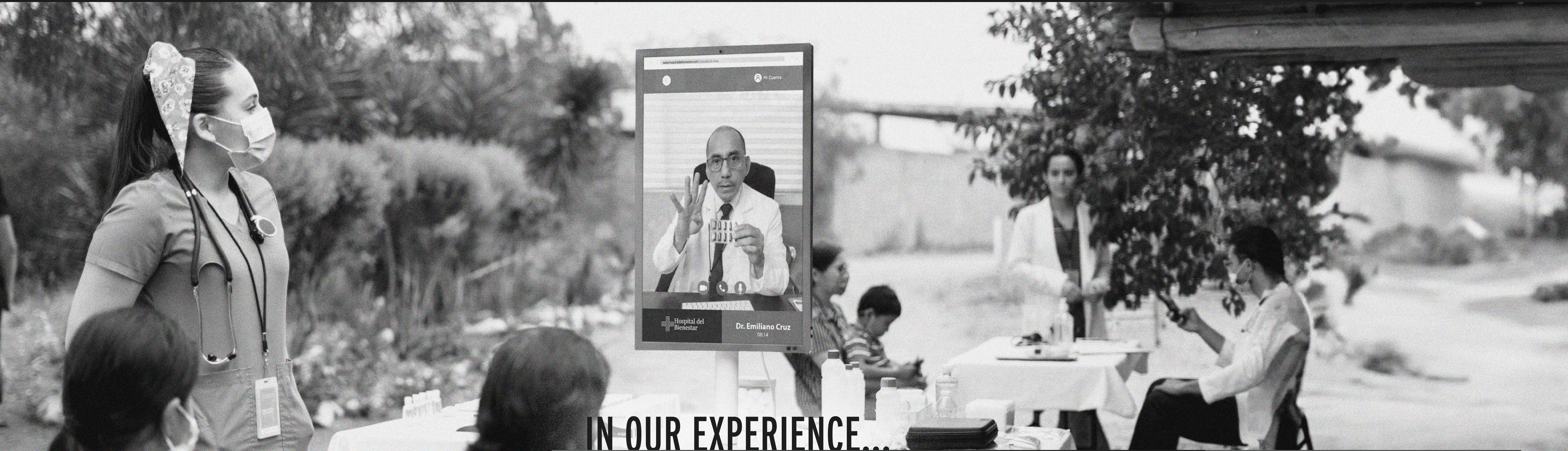
Where a patient lives still determines what treatment they receive

ACCESS REMAINS ONE OF THE BIGGEST HURDLES FOR PATIENTS IN NEED OF NOVEL TREATMENTS. Across the US, just 3% of hospitals offer CAR-T therapy, mostly at academic centers tied to specific brands, leaving the vast majority of patients far from care.[5]

34% of states have no open CAR-T or bispecific antibody trials, making participation out of reach for a variety of patients.[2]

For many, accessing these potentially life-saving treatments means uprooting their lives, adding financial strain and emotional stress at a time when stability matters most.

In addition, no government insurance programs cover the cost of travel and housing for patients traveling to receive CAR-T treatment, a high indirect cost of treatment. Patients must depend on manufacturers and charities for assistance.[6]



IN OUR EXPERIENCE...

"Where a patient lives shouldn't dictate how long they live. Patients living in underserved areas face long drives, delayed treatment, and limited access to specialists. Healthcare providers in these areas learn to accept the limitations of patient circumstances and work with what they have. Physician motivations don't vary by geography, but the variables that factor into their decision-making do. To truly reach every patient, we need to build solutions that bridge these gaps – investing in regional treatment hubs, telemedicine support, and robust patient navigation programs specifically designed to reach underserved communities."

– Jahda Hill,

Senior Vice President, Medical and Scientific Affairs, Marketing, Avalere Health

MEET **JAHDA**

With 12 years of experience and a specialized focus in oncology, Jahda has worked on several oncology brands across the product life cycle. Her experience covers acute lymphoblastic leukemia, acute myeloid leukemia, multiple myeloma, and HER2+ metastatic breast cancer. At Avalere Health, she drives promotional medical strategy and oversees the creation of rigorous, compliant communication platforms in the biotech space.

TIME:
The long and uncertain road to targeted treatment

The healthcare professional perspective

> “Can you imagine this being one of your family members, and you’ve got a product that can work, but you can’t get it for two months, and your brother or sister, your mom, your dad, are going to get too sick and succumb to the disease? We’re giving a lot of bispecifics to our myeloma patients, because we can’t wait two months [for CAR-T treatment].”

– Joseph P. McGuirk
DO, director of the Division of Hematologic Malignancies and Cellular Therapeutics at the University of Kansas Cancer Center[7]

EVEN WHEN PATIENTS ARE ELIGIBLE FOR ADVANCED THERAPIES, THE TIME IT TAKES TO RECEIVE TREATMENT BECOMES A CRITICAL CONSTRAINT

CAR-T manufacturing is highly personalized, time-sensitive, and capacity-constrained, requiring precise coordination from cell collection to reinfusion, with little margin for delay or failure.

Bispecific antibodies, though off-the-shelf, bring their own challenges: scaling biologics manufacturing, managing cold-chain logistics, and ensuring healthcare systems are equipped to handle step-up dosing and adverse event monitoring.

Emerging trispecific antibody therapies push these boundaries further, targeting multiple tumor antigens simultaneously to overcome resistance and deepen responses. However, these treatments also face challenges in manufacturing, scalability, and immunogenicity, adding further complexity to complicated treatment regimens.[8,9]

Together, these therapies are pushing the limits of existing infrastructure, demanding a rethinking of how we manufacture, deliver, and support advanced treatments at scale.

IN OUR EXPERIENCE...

> "In cell therapies for hem-onc, the best therapy from an efficacy perspective and the right treatment aren't always the same thing. Delays, clinic capacity, and patient endurance turn innovation into an access problem, and for some patients, time is the variable they can least afford."

–Victoria Shea,
Vice President, Brand Strategy, Marketing, Avalere Health

MEET **VICTORIA**

Victoria has over a decade of experience in brand strategy – dedicated to challenging conventional thinking, simplifying the complex, and inspiring fresh perspectives. With a background in public health, a career history of launching oncology products across solid tumors and blood cancers, as well as deep experience in commercializing CAR-T treatments, Victoria is committed to translating complex science into powerful campaigns for the stakeholders who need it most.

COVERAGE AND REIMBURSEMENT:
The critical gatekeepers

The healthcare professional perspective

> “Multiple patients have died waiting for their CAR T-cell therapy to be approved by insurance.”

– Brittney Baer,
BSN, RN, and patient care nurse coordinator for the Immune Effector Cell Program, Vanderbilt-Ingram Cancer Center[10]

While insurers spend time debating decisions, patients face a gut-wrenching wait, hoping their condition does not deteriorate before they can access treatment.

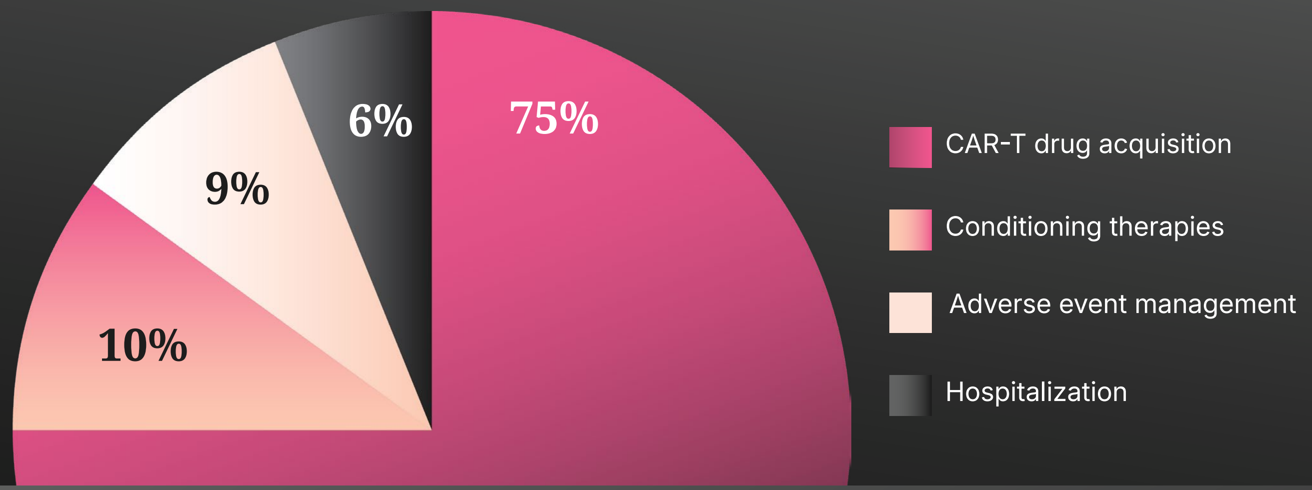
$475,000

The cost of CAR-T treatment per infusion.[11]

$1,000,000

The approximate maximum cost of total care, with the median cost of total care amounting to more than $600,000.[11]

A breakdown of the average cost of CAR-T treatment*



The commercial insurance paradox

While commercial insurers show higher reimbursement rates for CAR-T treatment and a greater likelihood of access, utilization management processes can take weeks to negotiate, requiring extensive documentation and repeated payer-provider interactions.

During this time, treatment cannot proceed. Patients may become sicker, making them ineligible for treatment. Variability across insurers and the need for dedicated administrative teams further slow the process, meaning even well-insured patients can face critical delays to care.

Patients undergoing treatment with bispecific antibodies face similar hurdles, including complex prior authorization processes. Additionally, bispecific therapies are often used in combination with other immunotherapies, which can trigger off-label status and lead to insurance denials and delays.[13] The result is a confusing landscape where healthcare professionals must meticulously plan their prior authorization strategies or risk patients missing out on treatment.

The challenges faced by patients on Medicare

Medicare payments often fall short of covering the full cost of CAR-T treatment, forcing hospitals to weigh financial risk before deciding whether to proceed or limit patient access. The challenges are furthered by complex payment structures that increase provider exposure to losses. For both bispecific antibodies and CAR-T therapies, additional delays are introduced by state-by-state variability in coverage and reimbursement, requiring additional administrative navigation before treatment can begin.[12]

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*Figures are estimated from published health economics research and are approximate. Sources: PMC systematic review (2024), Prime Therapeutics real-world study (2021), AJMC (2024).

IN OUR EXPERIENCE...

> "We need to come together as an industry to re-evaluate the way these treatments are paid for. It is unacceptable that oncologists have to spend valuable time working out how to avoid insurance denials. Value-based models are proving effective in countries such as Italy and Spain, where outcomes-based staged payments have been introduced, tying payment to factors such as patient response or survival over time. These models make it vital to supplement clinical trial data with real-world evidence demonstrating long-term effectiveness and quality-of-life metrics, which show a product’s sustained value."

–Brian Gillespie,
Managing Director,
Advisory, Avalere Health

MEET BRIAN

Brian has more than 25 years of leadership experience in global market access, reimbursement strategy, and innovation. He drives forward-thinking access strategies that balance commercial success with improved patient outcomes, ensuring sustainable growth in today’s dynamic global healthcare ecosystem.

INFRASTRUCTURE:
Shifting care beyond the hospital

In US inpatient settings:

CAR-T therapies were traditionally delivered inpatient and restricted to specialized centers under strict monitoring requirements. Inpatient CAR-T is reimbursed under the Medical Severity Diagnosis Related Group (MS-DRG). Under this model, payment is based on historic patterns of treatment, making it challenging for hospitals to cover the significant overheads associated with storing and administering novel CAR-T therapies.

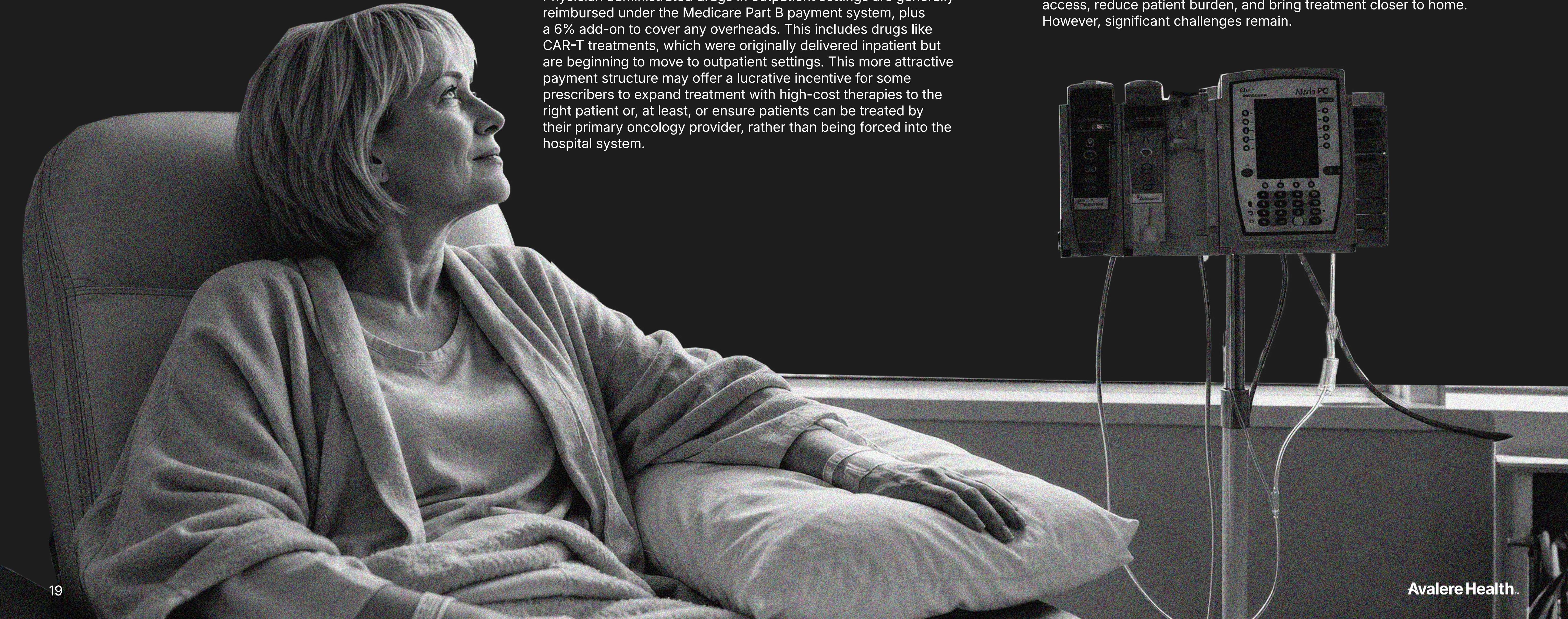
In US outpatient settings:

Physician administrated drugs in outpatient settings are generally reimbursed under the Medicare Part B payment system, plus a 6% add-on to cover any overheads. This includes drugs like CAR-T treatments, which were originally delivered inpatient but are beginning to move to outpatient settings. This more attractive payment structure may offer a lucrative incentive for some prescribers to expand treatment with high-cost therapies to the right patient or, at least, or ensure patients can be treated by their primary oncology provider, rather than being forced into the hospital system.

A new incentive for moving CAR-T treatment outpatient

To help improve access to CAR-T treatment and support adoption in outpatient settings, in June 2025, the FDA removed Risk Evaluation and Mitigation Strategy (REMS) requirements for approved BCMA- and CD19-targeting CAR-T therapies. Under the new guidance, hospitals and clinics no longer need to be certified to deliver CAR-T treatment and conduct patient monitoring, encouraging more community providers to consider the treatment.

Patients are now asked to remain close to the healthcare facility for two rather than four weeks. This shift has the potential to expand access, reduce patient burden, and bring treatment closer to home. However, significant challenges remain.



IN OUR EXPERIENCE...

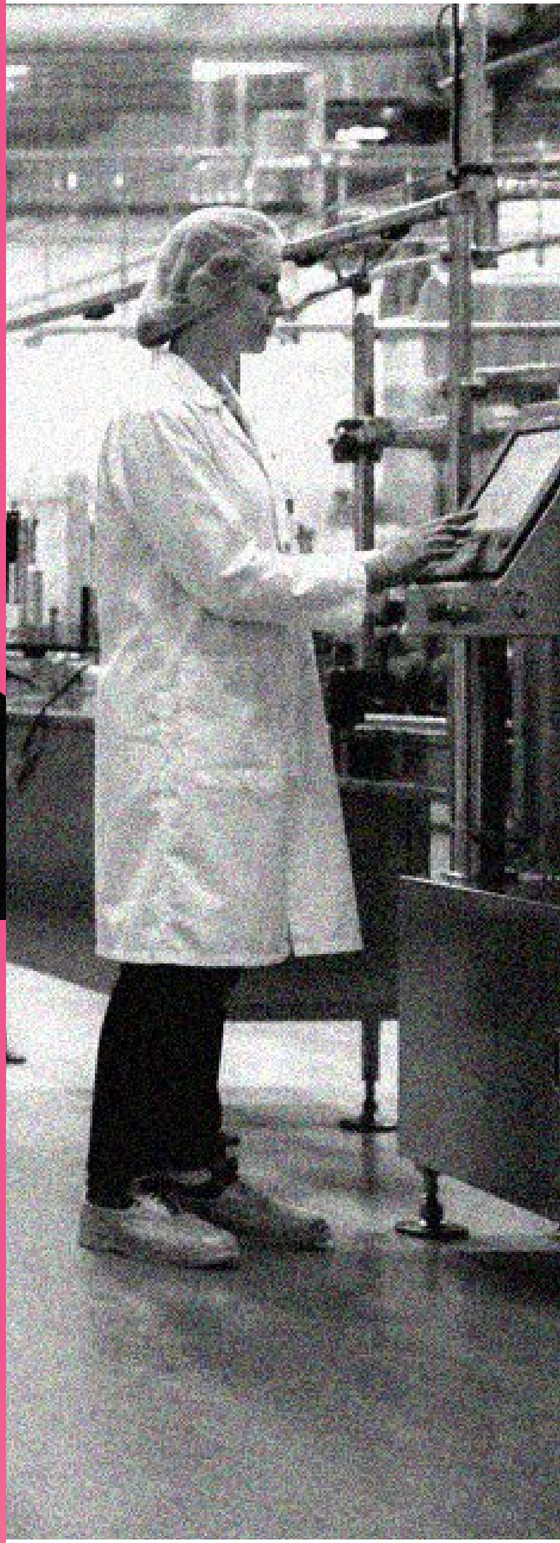
> "The removal of REMS requirements for certain CAR-T therapies is an important step toward expanding outpatient care. However, as more sites look to offer CAR-T treatment, gaps in infrastructure, training, and accreditation standards may limit where patients can receive treatment. At the same time, shifting payer expectations and coverage policies could influence care pathways, bringing therapy closer to some patients while creating new barriers for others. The real measure of progress will be whether these changes translate into faster, more equitable, and less burdensome access to life-saving care."

–Kolton Gustafson,

MPH, Principal, Advisory,
Avalere Health

MEET **KOLTON**

Kolton is a specialist in federal and health policy developments in cell and gene therapy, with deep experience in government payment systems, reimbursement, provider perspectives, drug pricing, physician payment, and healthcare reform. Prior to joining Avalere Health, he was a senior analyst at Mass General Brigham and worked as a research assistant at the Institute in the Department of Cell Therapies, where he worked with bone marrow patients.



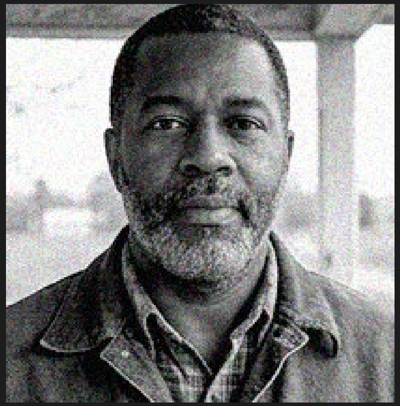
Patient perspectives on the removal of risk mitigation strategies (REMS) for CAR-T treatment

A recent study of 18 patients with relapsed/refractory multiple myeloma or diffuse large B-cell lymphoma revealed a striking contrast: while the healthcare community welcomes the removal of REMS for CAR-T therapy, patients remain apprehensive, fearing the potentially serious and fatal side effects like cytokine release syndrome (CRS) and neurological toxicities.[14]

Although studies have shown that 98% of CRS events and 96% of Immune Effector Cell-Associated Neurotoxicity Syndrome cases occur in the first 15 days after treatment, patients remain worried that the burden of monitoring for these severe side effects could fall on their caregivers.[15]

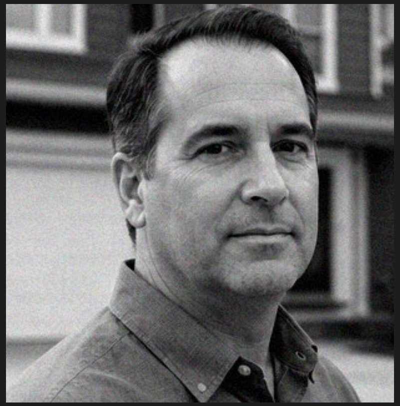
As brands explore opportunities to move CAR-T therapy infusions into the outpatient settings to avoid clinic capacity pressures, mitigating patient fears will become an imperative.

The patient perspective on changing REMS requirements



"I think the most important thing would be being able to access immediate medical help if it were called for…Being able to phone somebody, a real person, and being able to talk to that person is very important."

"It's certainly much more comfortable in your own home, in your own bed, and having just the comforts of that versus being in a hospital bed and… being woken up at all hours of the day and night."



"You better have a nurse there for at least a couple of days in the house until you stabilize…You better have at least a caregiver in your home, someone there who knows how to dial 911 and won't freak out or someone who knows how to help you take your temperature or whatever has to be done."

"I would have probably a lot of questions… Is it safe to go home? Is it safe to stay here? What behooves me the best? What do the professionals think? What does my doctor think?"

Source: Bixby TJ, et al. Oncol Ther. 2023 Sep;11(3):303-312.

IN OUR EXPERIENCE…

"CAR-T adverse effects have made headline news, making it likely that patients and caregivers will become aware of frightening side effects while researching the treatment. Addressing these fears head on will take kind, transparent, and proactive support, crafted in language patients understand. Healthcare professionals will also need to be equipped with the language and tools to address fear and hesitancy in the clinic, before it becomes a barrier to treatment. Co-creating materials with patient and caregiver groups early on and ensuring caregivers feel equipped to monitor for side effects, will help ensure patients feel supported at this crucial stage of the CAR-T treatment journey."

– Victoria Shea,
Vice President, Brand Strategy, Marketing, Avalere Health

PART III

SOCIETAL IMPACT

WHERE TENSIONS COLLIDE

Breakthrough science means little if patients and caregivers are left to navigate fear, burnout, and barriers alone – true innovation means caring for the needs of the whole person, understanding that the relationship with their care team, financial circumstances, and dynamics at home can all change their treatment experience.

KELLY'S STORY

Kelly is a 49-year-old mom to three kids. Her journey started when she was diagnosed with smoldering myeloma – a precursor condition to multiple myeloma that can remain stable for many years. In 2025, her disease progressed to multiple myeloma. She shares her story of resilience as she navigates her new diagnosis.

> *I was diagnosed with multiple myeloma in 2025. It was difficult and frustrating.*

Some patients with smoldering myeloma can stay in that state for many years or even a lifetime. I never thought I would progress to the full-blown disease. I have no words to express exactly how I felt.

I asked my doctor a lot of questions and, of course, turned to Google search. The first treatment regime didn't work well – after six scans, my disease was spreading. So we decided to do targeted immunotherapy treatment instead.

My treatment takes place every four weeks, and it takes multiple days to administer. I share a room with two other patients, so the experience is tough – especially with the side effects.

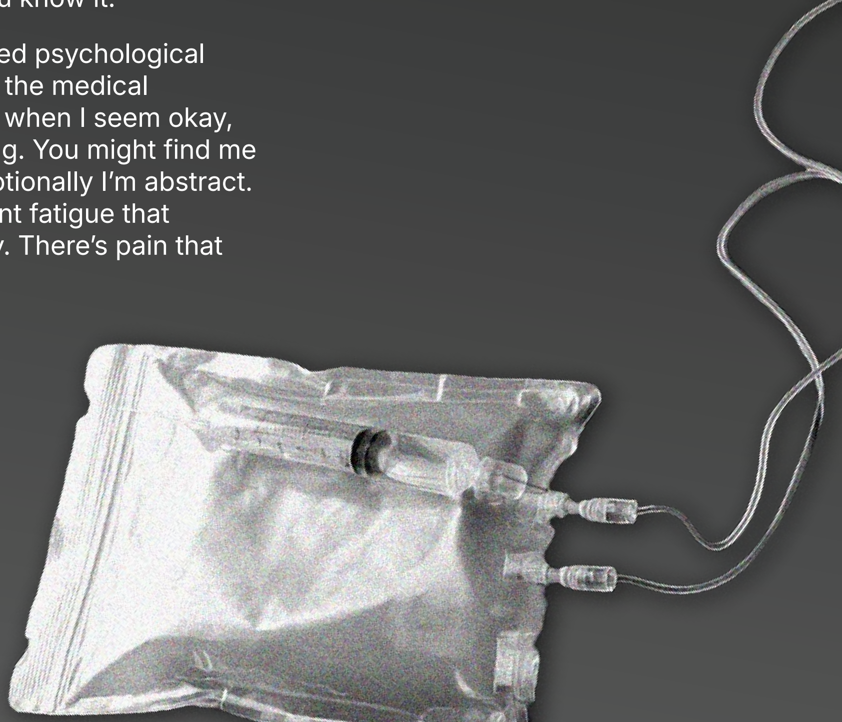
The hospital setting is crazy – it feels as if you could get an infection at any time. I'm scared of contracting other diseases. To minimize the risk of infection, you can only have one visitor, which for me is my spouse. It means I have to leave my kids behind. It's hard, but I have no choice. I have to get this treatment because I need to be around for them. I'd like to see more ways of keeping families together.

More could be done to make the treatment environment friendly for every patient – those in early stages and even those in late stages – and treat them well so that they can feel comfortable, at home, and appreciated. Give them as much information as possible so they can understand the treatment journey, how the medication works, and how to go about possible side effects. Those things matter – they make you more comfortable, feel more confident. It helps you come back the next time and know that you'll be home before you know it.

And patients need psychological support beyond the medical treatment. Even when I seem okay, I'm still struggling. You might find me smiling, but emotionally I'm abstract. There's persistent fatigue that doesn't go away. There's pain that is always there. Inside, I'm spiraling because of the fear of progression, the fear of death, the fear of complications.

> **CAREGIVERS ARE ALMOST COMPLETELY FORGOTTEN, AND THEY CARRY THE SAME BURDEN AS PATIENTS.**

When you're going through side effects, they shoulder the burden. I'd like to see physical and mental training, along with more support groups for caregivers. That includes helping to understand how things like intimacy in a relationship might be affected.



Patients don't stop being parents, partners, or loved ones...

IN OUR EXPERIENCE...

"No one is prepared for the moment a ‘watch and wait’ diagnosis becomes something far more serious. Perhaps the hardest part of Kelly’s story is not just the illness, but the separation it forces. Patients don’t stop being parents, partners, or loved ones when they enter the hospital. Biopharma and healthcare systems must do more to protect those connections, because healing is harder when family is out of reach."

– Jadha Hill,

Senior Vice President, Medical and Scientific Affairs, Marketing, Avalere Health

THE PSYCHOLOGICAL BURDEN: The invisible weight of living with an incurable disease

According to one study of hematologic cancer patients in England:

85%
of healthcare professionals reported receiving no training for assessing and managing hemato-oncology patients' psychological needs.[16]

76%
of patients reported their mental and emotional health had been impacted by their hematologic cancer diagnosis.[16]

IN THE US:

69%
of cancer patients said they required mental health support throughout their treatment journey.[17]

34%
of cancer patients said mental health support was not available for them.[17]

CAR-T and bispecific therapies offer transformative hope, but the path to treatment is grueling for both patients and their caregivers. Patients with blood cancers often cycle through multiple intense regimes, making it likely that they could be dealing with lingering adverse events or even post-traumatic stress disorder because of their disease and healthcare experiences.

Often, patients report anxiety, depression, fear, sadness, and even post-traumatic stress disorder as they navigate the uncertainty of sequencing and comprehend that their disease may not be curable.

Delays to treatment caused by logistical and insurance challenges, and disease progression can leave patients feeling all the more overwhelmed, particularly when their care diverges from their treatment plan at short notice.

Despite these common issues, healthcare professionals remain ill-equipped to address mental health concerns, reporting a lack of training in managing patients' psychological needs.[16]

IN OUR EXPERIENCE...

> "Patient support in oncology must go beyond access and insurance. It's about creating a personalized, living system that evolves with the patient at every stage of their journey. In CAR-T especially, where the journey involves coordination, anticipation, and multiple logistical steps, patients and their families need personalized information at each phase. When support is designed around the full arc of the experience, scientific innovation becomes something patients can actually live through and benefit from equitably – ensuring no patient is left behind in this rapidly evolving landscape."

– Victoria Shea,

Vice President, Brand Strategy, Marketing, Avalere Health

THE SECOND PATIENT:
Caregivers under strain

Caregivers take on an immense, multidimensional burden: coordinating travel and lodging when treatment centers are far from home, navigating complex medical instructions, managing daily needs during recovery, and providing round-the-clock emotional support, all while balancing jobs, childcare, and household responsibilities. Beyond this, and what support materials often miss, is the impact treatment can have on relationships, a couple's intimacy, and family dynamics.

The emotional toll is profound. Studies show that caregiver distress during advanced cancer treatment is high, with impacts on mental health, sleep, and overall quality of life that can persist long after the therapy concludes.[18] Family members also frequently report anxiety, depression, and burnout as they witness loved ones endure intense therapy, make critical medical decisions, and cope with uncertain outcomes.

> *To support the patient, they need help supporting themselves.*

IN OUR EXPERIENCE...

"Kelly's comment about intimacy really struck me. It shows that support has to go beyond helping caregivers navigate the side effects and look for adverse events. We need to look at the whole experience, from supporting them in their responsibilities at home to helping them speak to their children about the disease and guiding them through how a marriage could change because of cancer. Ultimately, it's about honoring and recognizing that caregivers are an essential part of the patient's treatment journey, and that to support the patient, they need help supporting themselves."

– Jadha Hill,

Senior Vice President, Medical and Scientific Affairs, Marketing, Avalere Health

EQUITY IN QUESTION:
The people left behind

Despite the high prevalence of blood cancer in Black and Hispanic populations, among patients with myeloma who received CAR T cell therapy on a clinical trial:

1%

of multiple myeloma CAR-T recipients were African American.[19]

5.4%

of multiple myeloma CAR-T recipients were Hispanic.[19]

In the U.S., Black and Hispanic patients remain severely underrepresented in both treatment and clinical trials. The problem is compounded by geographic and socioeconomic barriers, with patients from lower-income communities far less likely to receive treatment:

- Just 7.3% of CAR-T admissions come from areas where the median household income is below $40,000.[19]
- 33% of Black patients live in a county where there is a CAR-T therapy or bispecific antibody trial, leaving many communities entirely cut off from the frontiers of care.[20]

These numbers are more than statistics; they represent real people whose access to potentially curative therapies is shaped by race, income, and ZIP code rather than medical need.

IN OUR EXPERIENCE...

"If we're serious about equity, we have to design for the realities of people's lives: bringing care closer to underserved communities, embedding navigation and support early, and, critically, partnering with the people patients already trust. Working alongside community organizations and faith leaders helps ensure education is not only accessible but credible and culturally relevant. And when patients see themselves reflected in care teams, materials, and support programs, it sends a powerful signal that these therapies are for them, not for someone else."

– Jadha Hill,

Senior Vice President, Medical and Scientific Affairs, Marketing, Avalere Health

PART IV

Five strategic imperatives for reaching EVERY PATIENT POSSIBLE WITH BLOOD CANCER

1. Start with a *Sense*Making analysis

Healthcare professionals, patients, and payers don't experience therapies in isolation. Understanding their real-world pressures, barriers, and behaviors across the full care journey is essential for uncovering complexity and revealing untapped potential.

A *Sense*Making approach allows you to examine the interconnected domains of science, society, and systems, exposing the tensions between them to reveal the barriers preventing a novel treatment from reaching every patient with blood cancer.

It means bringing together access, policy, medical, and marketing teams to:

ANALYZE

the science, engaging with clinical experts to discover what's clinically meaningful, relevant, differentiating, and defensible, looking beyond what the data says to see what it enables, resolves, and changes.

DECODE

the systems, revealing how healthcare works on the ground – the pathways, incentives, time to access, policy blockers, service design issues, and reimbursement barriers.

UNCOVER

the true impact on society, including the complex relationships between individuals and communities, to uncover what people think and feel, their lived experience, fears, cultural cues, stigmas, motivations, and the emotional context in which decisions are made.

> "This *Sense*Making approach only works if each domain is analyzed together. The magic happens when you explore the tensions between them. That's where you discover the insights that demand action and the untapped opportunity to reach every patient possible."
>
> **– Clare Jones,**
>
> *Executive Director, Global Market Access, Advisory, Avalere Healthh*

2. Focus evidence generation on real-world clinical relevance

Clinical relevance sits where science meets a community oncologist's decision, a patient's geography and nuanced needs, and an insurer’s coverage criteria. Relevance no longer just hinges on the most efficacious product, but on its real-world application – and that means evidence needs to work harder.

I

ENSURE EVIDENCE REFLECTS HOW THERAPIES ARE ACTUALLY USED: tolerability, treatment burden, and outpatient feasibility

II

FOCUS ON REAL-WORLD OUTCOMES THAT MATTER TO CLINICIANS AND PAYERS ALIKE: sequencing, durability, and quality of life

III

DESIGN STUDIES THAT REFLECT DIVERSE PATIENT NEEDS FROM THE START: so no population is left out of the value story

> “When evidence reflects real lives – the systemic and societal challenges patients face – it stops being theoretical and starts transforming a clinically active drug into a therapy that actually reaches the people who need it most. That's the standard the industry should be building toward, and it’s what demonstrates sustained value to payers, providers, patients, and policymakers alike."

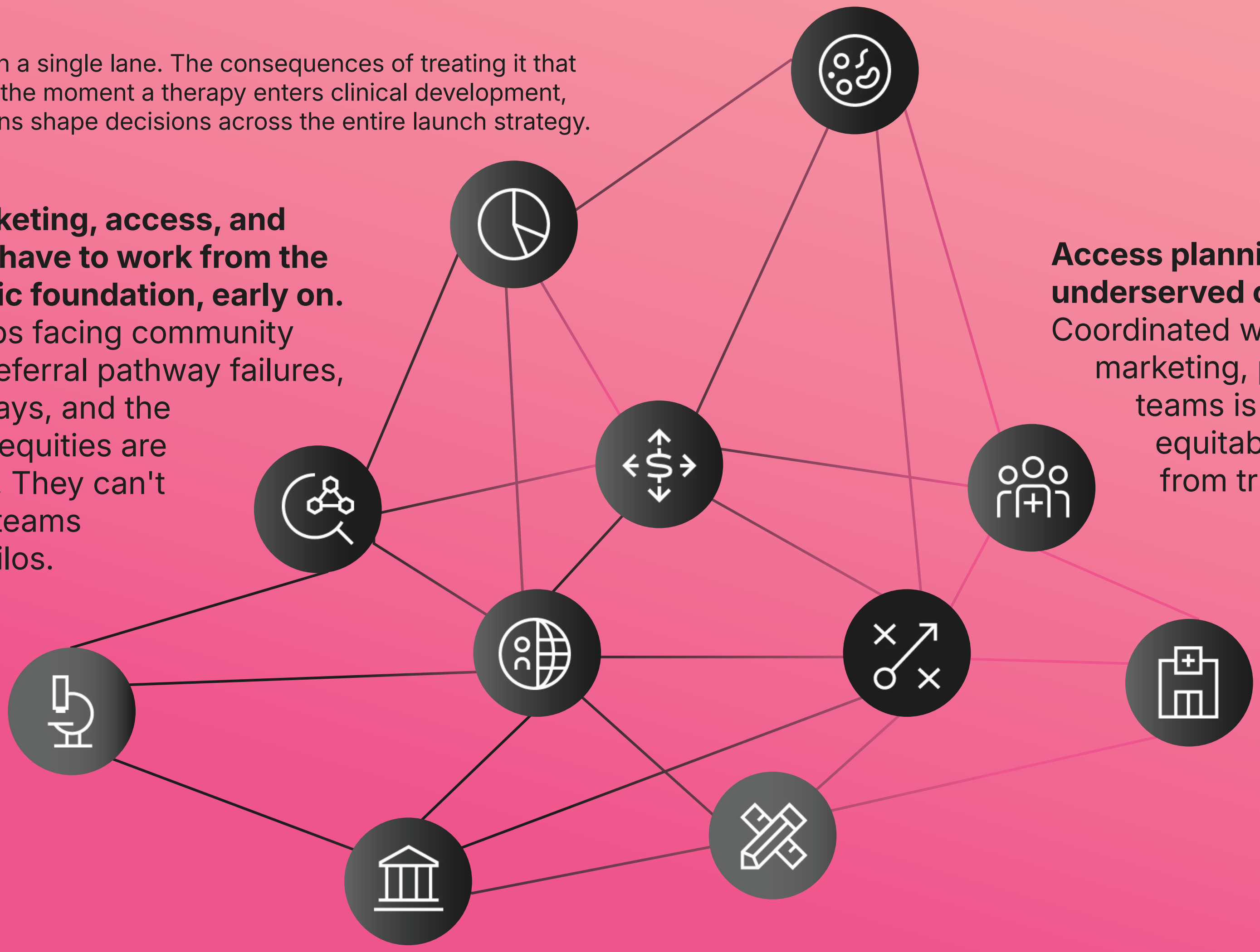
– Jessica Holzhauer,
Director, Scientific Strategy, Medical, Avalere Health

3.

Build access into every strategy from day one

Access cannot live in a single lane. The consequences of treating it that way are stark. From the moment a therapy enters clinical development, access considerations shape decisions across the entire launch strategy.

Medical, marketing, access, and policy teams have to work from the same strategic foundation, early on. Education gaps facing community oncologists, referral pathway failures, insurance delays, and the geographic inequities are all connected. They can't be solved by teams operating in silos.



Access planning must prioritize underserved communities. Coordinated work across medical, marketing, policy, and access teams is essential for ensuring equitable treatment pathways from trial to launch.

> “
>
> When medical is generating evidence that directly supports the access story, policy teams are shaping the environment in which that evidence lands, and marketing is translating it into tools that change behavior at the point of care – that's when access stops being a downstream problem and starts being a source of genuine competitive advantage."

–Maddi Davidson,

Principal, Advisory, Avalere Health

4. Meet community oncologists where they are with smarter, more targeted education

The real challenge is the community setting, where most patients first present and where oncologists are time-pressured, under-resourced, and often default to familiar regimens because they lack the confidence or clarity to do otherwise.

Continuously evaluate your positioning: The blood cancer landscape is crowded, complex, and ever-changing, with new competitors entering the market and health policies evolving. Updating product positioning in response to these changing dynamics not only provides a strategic advantage but also positions brands as a trusted partner, on the pulse of evolving healthcare professionals' needs.

Make sequencing the centerpiece of the strategy: Build education that maps out a clear patient pathway and gives clinicians something they can actually use. Deliver it to them in accessible formats they can engage within their own offices.

Facilitate connections between community and oncologists and academic centers: Create forums for knowledge sharing, publish shared perspectives, and support the development of referral pathways. These initiatives not only help build the confidence needed to identify and refer eligible patients early, before their disease progresses, but they also help address fears about safety, cost, and the complexity of the treatment process.

Ensure educational programs reflect the realities of diverse patient populations: Equip community oncologists to identify, refer, and treat patients from all backgrounds confidently. Peer-to-peer forums and local insights help clinicians overcome biases and systemic inequities.

Consider a virtual campus tour: Virtual experiences, like an immersive digital campus tour of an academic center, can help community oncologists understand what happens to the patient at each step of the treatment journey. The experience, viewed from the oncologist's own office, can be paired with peer-to-peer support that enables oncologists to connect with other clinicians experienced in the treatment dynamic.

> “In a landscape this complex, clinicians trust experience. The most effective education isn't delivered to oncologists – it's built with them. Involving community physicians in the design of educational programs, incorporating real-world feedback on what's working and what isn't.”

– Jeff Bergen,
PhD, Scientific Director, Medical, Avalere Health

5. Move from treatment support to total journey support

Patients aren't waiting for a brochure; they're scrolling for answers, often in the middle of the night when fear is loudest. Support programs need to show up in those moments, on the right channels, with information that's honest, accessible, and empathetic, and includes stories from patient peers.

PATIENT SUPPORT

Patient support is not a portal or a program. It's the humanity around a treatment. The best support understands where change matters most

BEFORE	DURING	AFTER
Prepare	Build clarity	Continue with support
Understand options	Grow confidence	Adapt over time
Reduce fear	Take next steps	Stay connected

The best support understands where change matters most, then shows up in relevant ways

ACCESSIBLE

CULTURALLY RELEVANT

BEHAVIOR-BASED

RESPONSIVE

CAREGIVER SUPPORT

Take caregiver support seriously as a clinical priority. Caregivers need resources that go beyond clinical information.

- ✓ **Practical navigation**
- ✓ **Financial guidance**
- ✓ **Mental Health support**
- ✓ **Relationships & intimacy**
- ✓ **Family dynamics**

> “
>
> Design support around transitions, not just treatment. Some of the most acute moments of patient distress happen at transition points – when a treatment stops working, when a care plan changes unexpectedly, or when a patient has to travel to a different center. These are the moments where anxiety peaks and where the right support can make the biggest difference."

– Victoria Shea,

Vice President, Brand Strategy, Marketing, Avalere Health

> “
>
> The companies that show up as genuine partners in solving real problems will earn the trust of clinicians, patients, and payers in ways that purely commercial approaches never will. In a landscape where the barriers to treatment are as significant as the barriers to efficacy, that trust itself is a source of competitive advantage."

– Jadha Hill,

Senior Vice President, Medical and
Scientific Affairs, Marketing,
Avalere Health

HAVE WE PIQUED YOUR INTEREST?

Reaching every eligible patient demands more than clinical data. It demands a deep understanding of how science, systems, and society interact – and where they collide. That's not something generalist thinking can deliver.

THAT'S WHERE WE COME IN.

Book a *Sense*Making landscape consultation call with our Advisory, Medical, and Marketing oncology experts. Reveal the tensions holding your therapies back and the opportunities your competitors haven't discovered.

Your patients can’t wait. Neither can you.

Book a *Sense*Making landscape consultation with our oncology experts.

BOOK NOW

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