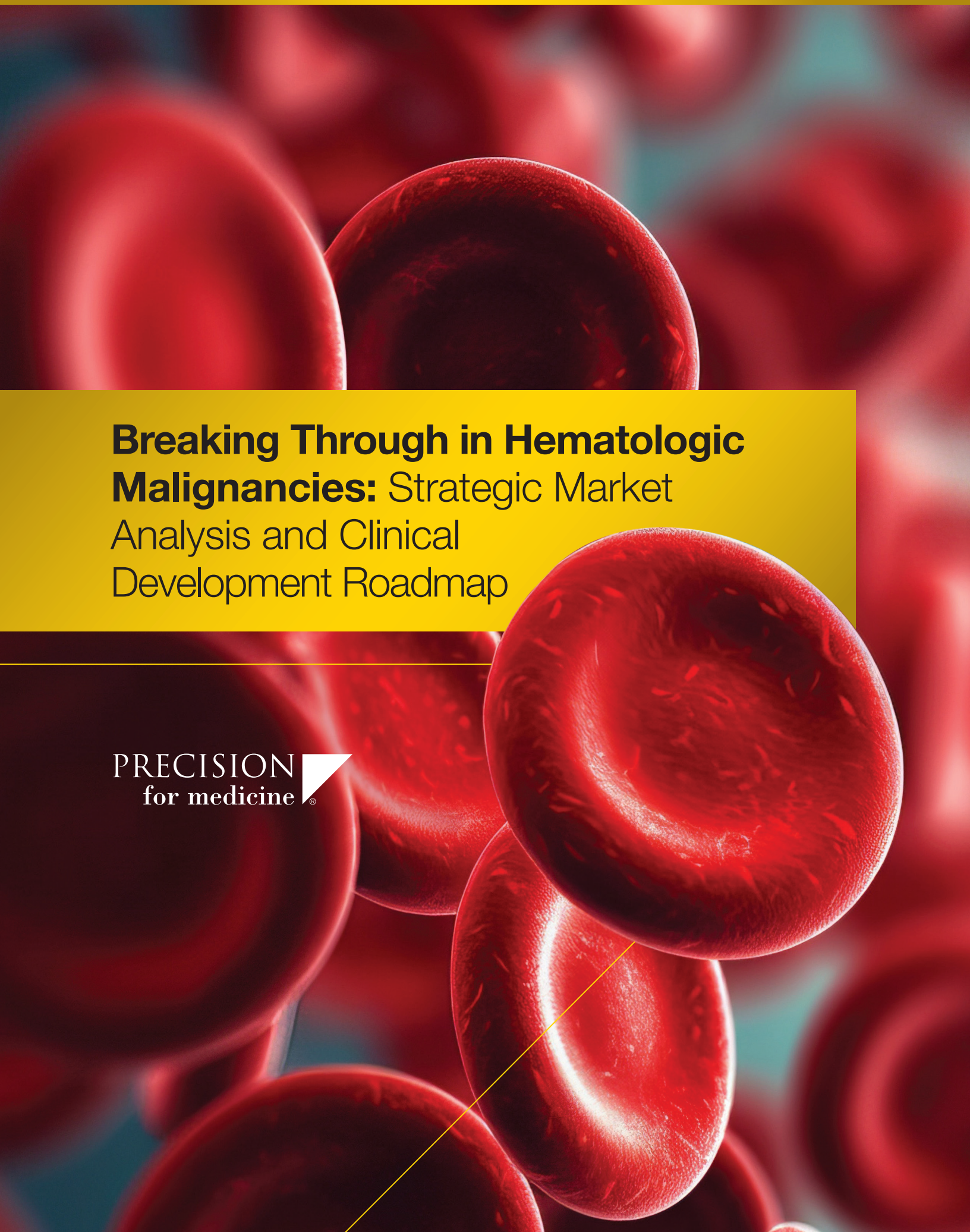

Breaking Through in Hematologic Malignancies: Strategic Market Analysis and Clinical Development Roadmap

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Approximately every 3 minutes, one person is diagnosed with leukemia, lymphoma, or myeloma.[1]

The hematology oncology landscape has experienced dramatic evolution over the past decade, driven by both advancements in therapeutic innovations and a rising global incidence of hematologic malignancies. As the world faces a growing cancer burden, hematologic indications such as leukemia, lymphoma, and multiple myeloma (MM) present unique challenges that demand specialized approaches in research, drug development, and clinical trial design.

Key drivers of this shift include demographic changes—particularly the rapid aging of the global population—and the emergence of cutting-edge therapies that are reshaping treatment paradigms. The rise of personalized medicine and targeted therapies, including immunotherapies, is enabling more effective, less toxic treatments. These developments are not only improving patient outcomes but also raising the bar for research, trial execution, and commercialization.

However, these advancements come with significant challenges. The complexity of treating hematologic malignancies requires deep scientific expertise, sophisticated data management, and robust clinical infrastructure. Pharmaceutical companies, CROs, and healthcare providers must work in closer collaboration to navigate these evolving demands. At the same time, healthcare systems worldwide are grappling with the resource-intensive nature of hematology-oncology trials, which typically involve higher costs, longer patient monitoring, and more complex biomarker assessments compared with solid tumor studies.

This market analysis explores the growing incidence of hematologic malignancies, the demographic forces driving these trends, and the innovations shaping the future of hematology-oncology. By examining both the rising clinical demand and the emerging therapeutic landscape, this report aims to provide a comprehensive understanding of the opportunities and obstacles in this dynamic field.

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Chapter 1

Hematology-Oncology Market Analysis

Understanding the Rising Incidence of Hematologic Malignancies

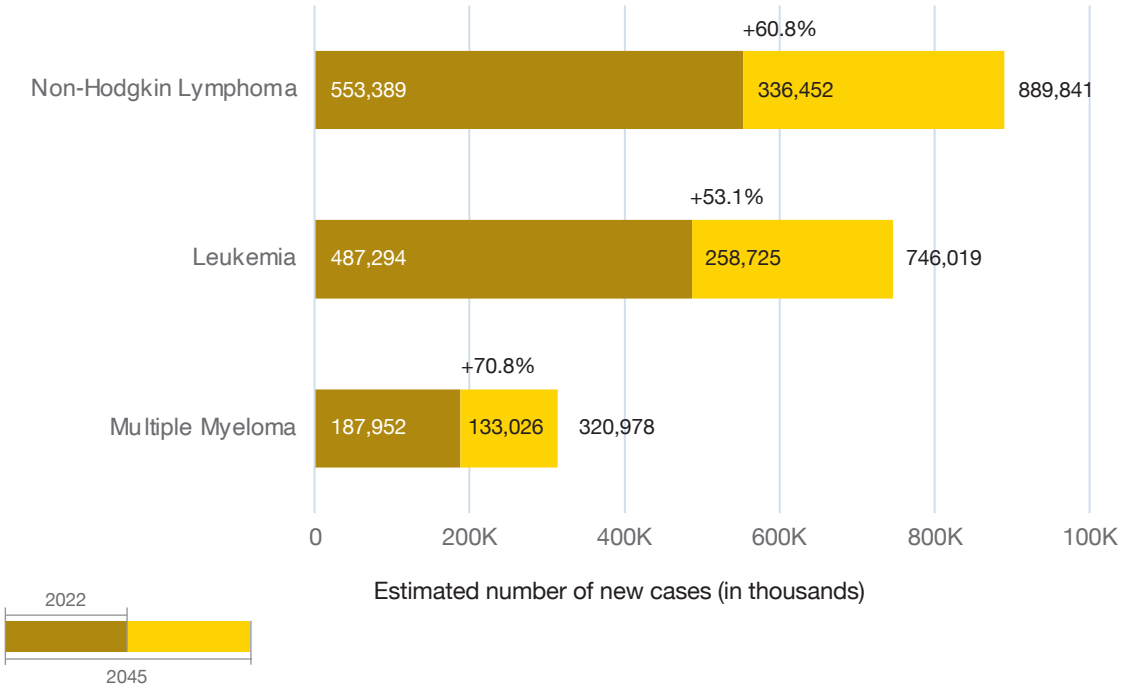
The global burden of cancer continues to rise, with a particularly notable increase among older populations that is significantly contributing to the growing demand for specialized treatments in hematology-oncology. As the incidence of cancer escalates globally, there is an increasing focus on hematologic malignancies such as lymphoma, leukemia, and myeloma, which are particularly prevalent among aging individuals. Projections for these cancers over the next 2 decades present a concerning trajectory.

The hematology-oncology market is projected to nearly double from $8.13 billion in 2021 to $14.83 billion by 2029, driven by an aging global population and breakthrough therapeutic innovations.

According to data from GLOBOCAN[2], MM is expected to experience the most dramatic growth within the realm of hematologic cancers. The number of new cases of MM is projected to surge from 187,952 in 2022 to approximately 320,978 by 2045,

marking an alarming 70.8% increase. Similarly, the incidence of non-Hodgkin's lymphoma (NHL), which encompasses a wide range of lymphoid cancers, is forecasted to increase from 553,387 annually to 889,841, a substantial rise of 60.8%. Leukemia, a group of cancers affecting the blood and bone marrow, is also projected to see significant growth over the coming decades. The number of new leukemia cases is expected to rise from 487,294 in 2022 to 746,019 by 2045, reflecting a 53.1% increase. These significant rises underscore the growing challenges of managing these diseases and the need for continuous improvements in therapeutic approaches globally.

Figure 1. **Estimated Number of New Cases From 2022 to 2045**



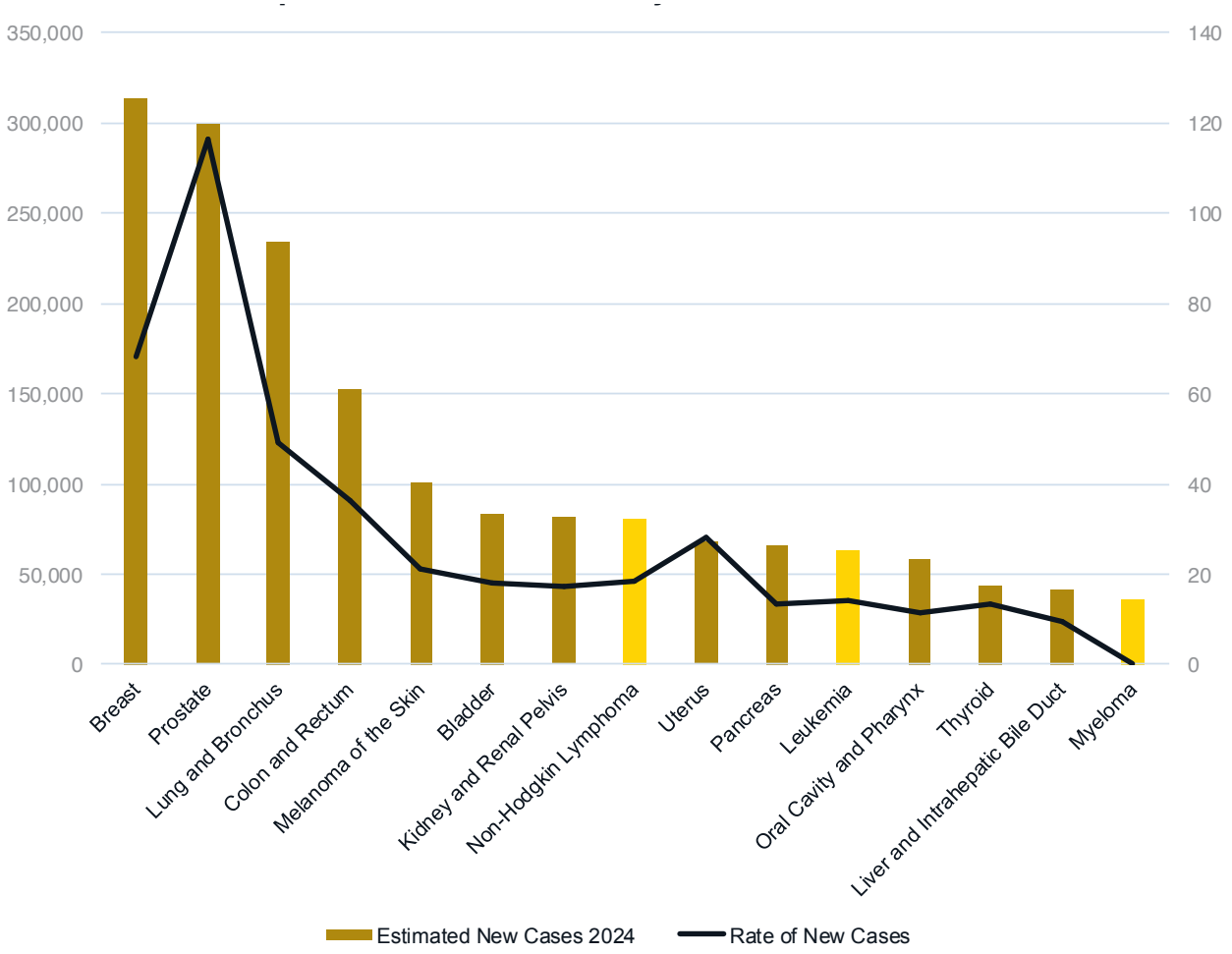
The Impact of an Aging Population

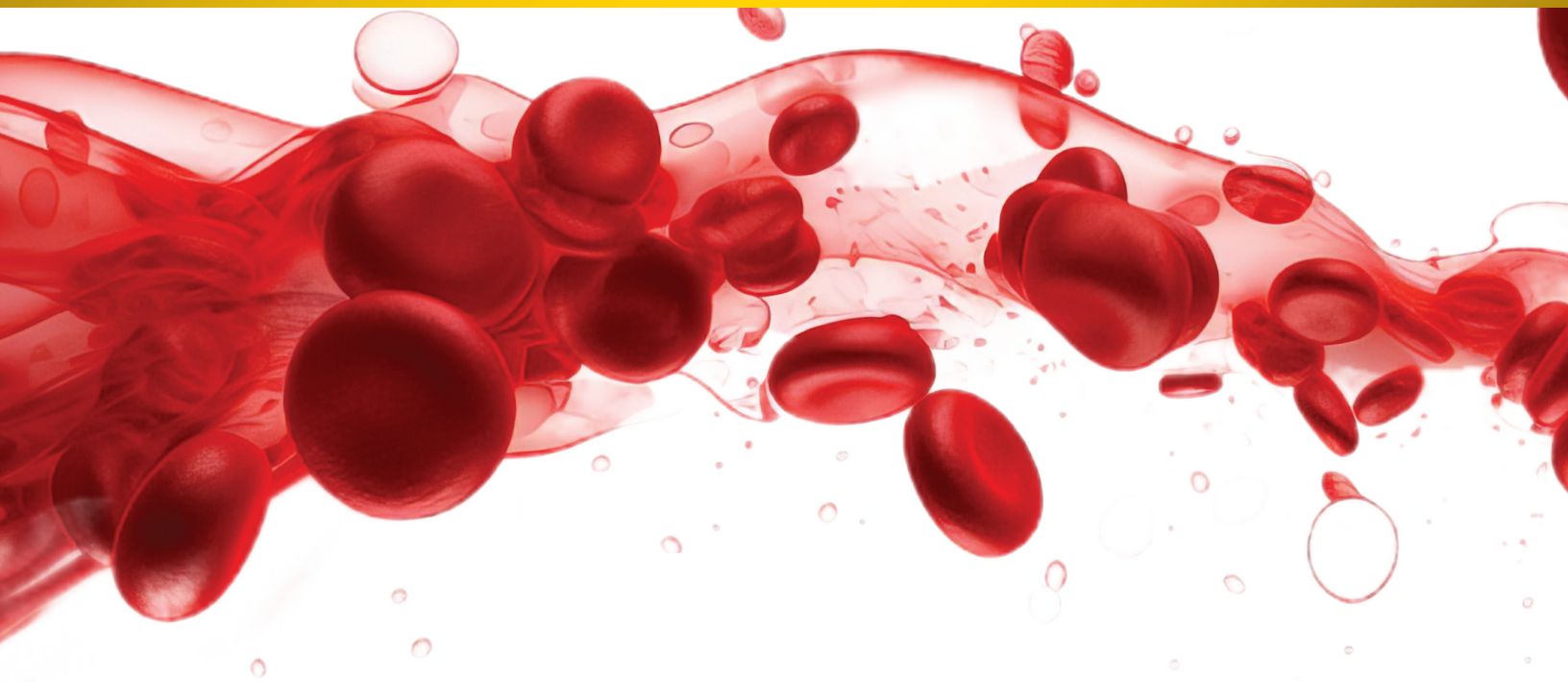
The global phenomenon of population aging, driven by the rise in the geriatric population, represents a profound demographic shift with far-reaching implications for healthcare systems worldwide. According to the United Nations 2023 World Social Report,[3] the number of adults aged 65 years and older is projected to more than double, increasing from 761 million in 2021 to approximately 1.6 billion by 2050. This dramatic increase is largely a result of significant advancements in healthcare, nutrition, and living conditions, which have collectively contributed to a longer lifespan.

The aging demographic has profound implications for various sectors of healthcare, particularly in

the realm of hematology-oncology. Hematologic malignancies, such as MM, NHL, and leukemia, are extremely common. In the United States alone, NHL is the eighth most common cancer among new diagnoses, accounting for 4% of all new cancer cases,[4] while leukemia (all types) is the eleventh most common, making up 3% of all new cases,[5] and myeloma is the fifteenth most common.[6] These cancers are typically diagnosed in elderly populations, with the average age at diagnosis around 67 years.[7] As such, the growing geriatric population is expected to drive an increase in the incidence and diagnosis of these cancers, placing additional pressure on the healthcare systems globally. The aging population is not only contributing to the overall burden of hematologic cancers but also shifting the landscape of hematology-oncology treatment and care, with an increased need for therapies in these indications.

Figure 2. **Top 15 Cancers in World by 2024 New Incidence**

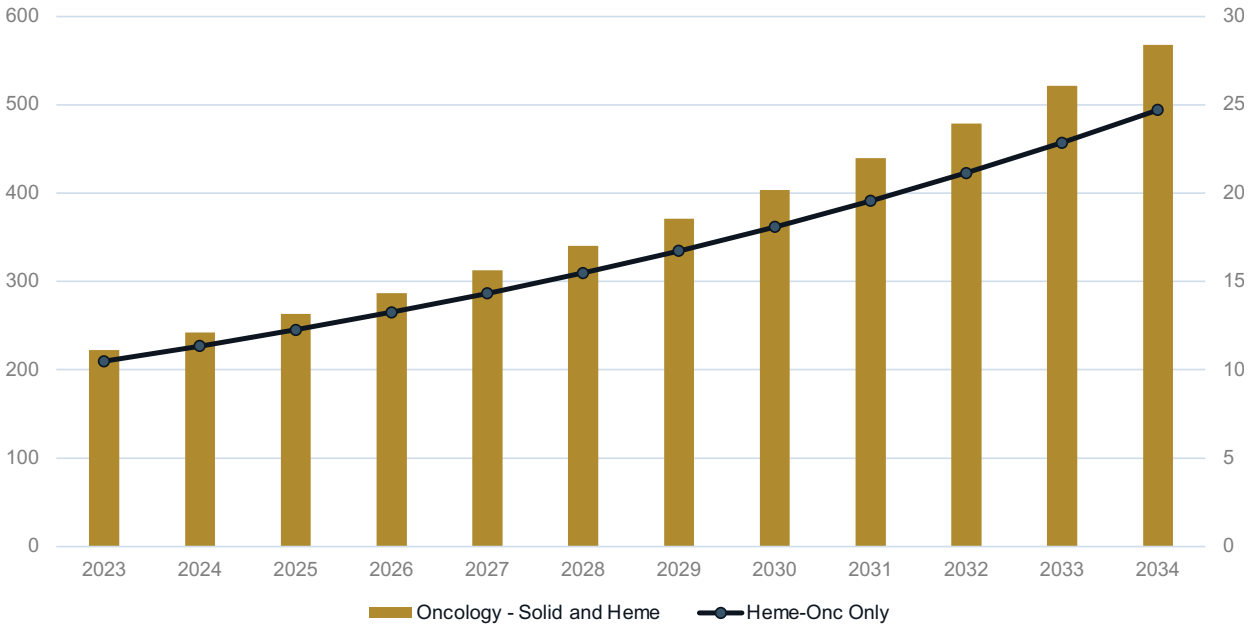




Quantifying the Hematology-Oncology Market

The oncology market's valuation has surged to an estimated $222.36 billion US dollars[8] at the end of 2023, with a compound annual growth rate of 8.9% projected through the next 10 years. Overall, it is expected to hit $521.6 billion by the end of 2033. The expected growth rate in hematology-oncology is only slightly less. At the end of 2023, the hematologic malignancies market was valued at $10.49 billion. It was forecasted to grow at 8.1% from 2024 to 2034, reaching $24.7 billion.[9] This robust growth reflects not only the rising incidence of hematologic malignancies but also the rise in geriatric population and favorable healthcare infrastructure.

Figure 3. **Expected Growth of Global Oncology Market Size, 2024-2034 Projections**



Chapter 2

Global Landscape of Hematology-Oncology Clinical Trials

Globally, the hematology-oncology clinical trial landscape is one of the most dynamic and resource-intensive sectors in pharmaceutical development. These newly approved therapies are driving an influx of trials, increasing demand for specialized resources, and reshaping how treatments are tested and delivered.

Geographic Distribution and Regional Dynamics

With the global scale of hematology-oncology trials set, the geographic distribution of these efforts reveals distinct patterns in which this research is concentrated and evolving across different regions.

- North America, particularly the United States, continues to dominate the trial landscape. US sites participate in 32.6% of all hematology-oncology trials globally, with 2027 ongoing/planned trials.[13]

As of 2024, more than ~6,200 active hematology-oncology trials are competing for patients globally. These trials command almost 22% of total oncology research and development, despite hematologic malignancies accounting for only 9.4% of total cancer incidence.[12]

- European trial activity demonstrates distinct regional patterns:
  - Western European sites participate in 847 ongoing/planned trials, with France, Spain, Italy, and Germany leading in trial volume. Here, the emphasis is on early phase treatment with more than 37% of trials in this region being Phase 1 or Phase 1/2[14]
  - Eastern European sites, particularly in Poland and Czechia, have emerged as crucial recruitment centers for Phase 3 studies, offering combination of efficient startup timelines and access to treatment-naïve patients. Of the 259 ongoing/planned hematology-oncology trials with site locations in Eastern Europe, 104 contain a Phase 3 component[15]
- Looking at Asia Pacific (APAC), inclusive of Australia/Oceania, there are 3370 planned/ ongoing hematology-oncology trials, some 1516 of which are early phase (1/2), but the APAC region also hosts more than 320 Phase 3 trials in this therapeutic area. NHL trials are the most common focus in this area[16]
  - Of the 3370 ongoing/planned APAC trials, 2440 of these are being conducted in China only. Interestingly, 1644 of these trials are academic rather than industry-sponsored studies. Details such as these should always be factored in when evaluating countries and/or regions for trial planning purposes as they may artificially skew the competitive landscapes

Although, historically, North America and Western Europe have dominated the hematologic malignancy clinical trial landscape, we are beginning to see new key geographic regions emerge, including Eastern Europe and APAC (excluding China).

Eastern Europe

A combination of favorable factors in Eastern Europe support trial efficiency, quality, and patient recruitment. First, the region has a large, diverse patient population with a high incidence of hematologic cancers, creating an ample pool of eligible participants. Diseases like lymphoma, leukemia, and MM have rising prevalence in the region, making it easier to recruit patients who meet trial eligibility criteria.

Secondly, Eastern European countries have well-developed healthcare infrastructures and a high concentration of medical centers and oncology specialists who are experienced in clinical research. Many Eastern European countries have invested in upgrading their healthcare facilities and research capabilities, making them attractive to sponsors looking for skilled research teams and high-quality data. The region's medical professionals are also often multilingual and well-trained, easing collaboration with international trial sponsors and ensuring high standards of data collection and patient care.

Cost efficiency is another factor driving trials to this region. Conducting clinical trials in Eastern Europe is generally more cost-effective than in Western Europe or North America. This cost efficiency extends to aspects like operational costs, recruitment, and monitoring, enabling sponsors to allocate resources to more trial sites or expand sample sizes, thereby increasing the statistical power of studies. Additionally, patient populations in Eastern Europe often have limited access to innovative therapies through their healthcare systems, leading to high patient interest and willingness to participate in trials that provide access to novel treatments. This motivation to participate can lead to higher recruitment and retention rates, which are critical to completing trials on time and maintaining robust datasets.

The regulatory environment in Eastern Europe is also becoming more aligned with international standards, facilitating smoother trial approvals, and reducing bureaucratic delays. Many Eastern European countries are members of or adhere to the European Medicines Agency (EMA) guidelines, which makes regulatory processes more transparent and reliable for international sponsors.

These combined factors make Eastern Europe an increasingly attractive region for hematologic malignancy clinical trials, offering sponsors a large patient pool, skilled researchers, cost advantages, and high data quality, all within a supportive regulatory framework. As a result, the region is likely to play an even greater role over the coming years in global clinical trials for hematologic malignancies, contributing to advances in treatment and research.

Asia Pacific

The APAC region, excluding China, is also becoming a key area for hematologic malignancy clinical trials, driven by several unique factors that support trial feasibility, patient recruitment, and diverse data collection. A primary advantage is the region's large, genetically diverse population, which includes patients with varying disease presentations, treatment responses, and genetic profiles. Countries like India, Japan, South Korea, Australia, and Singapore have high and rising incidences of hematologic cancers such as leukemia, lymphoma, and myeloma, which creates an ample pool of eligible patients and allows for more robust, ethnically diverse data in multiregional clinical trials.

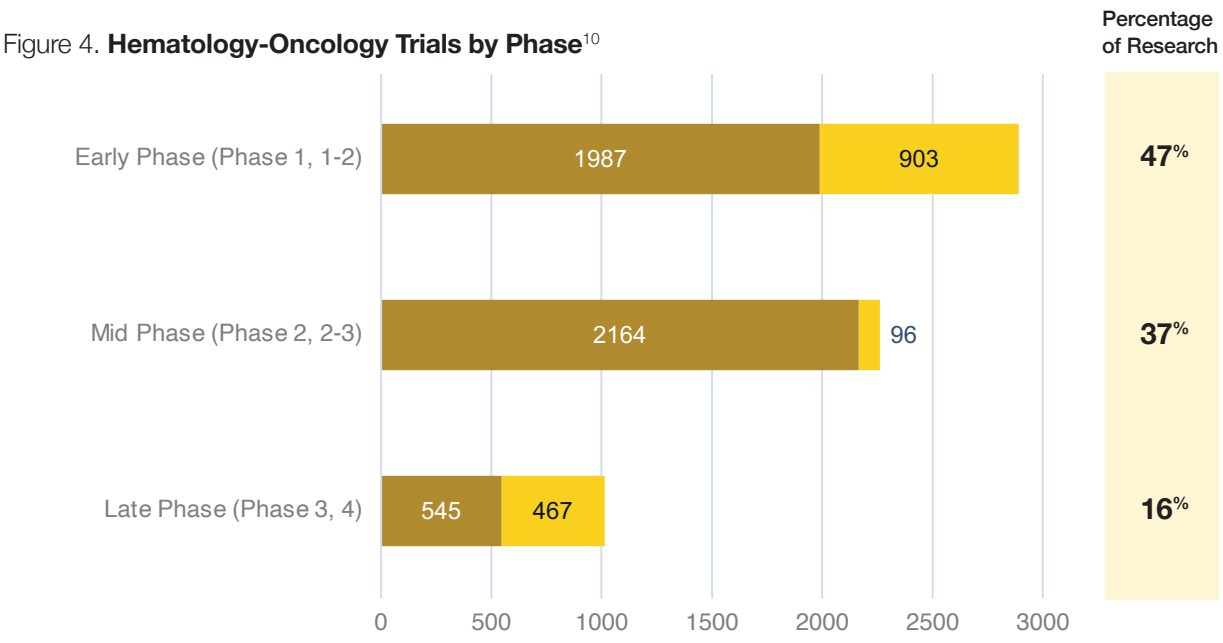
APAC countries have been investing heavily in healthcare infrastructure and clinical research capabilities, which has contributed to an increase in the number of qualified medical professionals and specialized research facilities. Many of these countries have established centers of excellence for oncology and hematology research, particularly in Japan, South Korea, and Singapore. These centers often have partnerships with international academic institutions and biopharmaceutical companies, fostering high-quality, collaborative research and ensuring compliance with international standards for trial conduct, data management, and ethical practices.

Similar to Eastern Europe, cost efficiency is another compelling factor in the APAC region as clinical trials in many of these countries can be conducted at significantly lower costs than in North America or Europe. Lower operational and logistical costs in these countries make it feasible for sponsors to establish multiple trial sites, increase sample sizes, and maintain long-term studies with reduced financial strain. Additionally, APAC countries often have high recruitment and retention rates due to patients' interest in accessing innovative therapies that may not yet be widely available through their public healthcare systems.

Regulatory environments across APAC are also becoming more streamlined and harmonized with international standards. Many countries, including Japan, South Korea, and Australia, have regulatory bodies that closely follow or collaborate with international agencies like the US Food and Drug Administration (FDA) and EMA. This alignment reduces regulatory delays, making the region more attractive for international sponsors seeking a more efficient trial start-up process. Additionally, Japan and Australia are particularly notable for having expedited approval pathways for innovative therapies, allowing for faster access to novel treatments and enabling trials to progress more swiftly.

Lastly, APAC's strategic geographic location makes it a critical region for global clinical trials. The region's diverse patient populations, combined with an increasingly supportive infrastructure for clinical research, provide valuable data that improve the global applicability of trial findings. As a result, the APAC region is emerging as a vital area for hematologic malignancy clinical trials, offering access to a genetically diverse patient population, cost-effective operations, high-quality clinical sites, and a favorable regulatory landscape – all of which contribute to advancing the development of novel therapies in hematologic malignancies.

Figure 4. **Hematology-Oncology Trials by Phase**[10]



Source: Citeline, TrialTrove, Oct 2024.

The landscape of hematologic malignancy clinical research is evolving rapidly. Across the various trial phases, distinct distribution patterns and study designs have emerged, reflecting a more strategic shift towards improving patient outcomes and accelerating drug development.

Early-phase studies (Phase 1 and 1-2) comprise 47% of active hematology/oncology studies globally. Hematologic malignancy research has seen a marked increase in early-phase development, with a higher relative proportion of first-in-human studies vs solid tumor research. Early-phase studies show a significant concentration of trials conducted at academic medical centers, which tend to be at the forefront of developing and testing novel therapeutic modalities such as bispecific antibodies, cell therapies, and antibody-drug conjugates (ADCs).

In Phase 2, we observe a notable shift toward using expansion cohorts to assess efficacy across multiple indications and patient subsets, and the implementation of innovative trial designs, such as basket and umbrella trials, is on the rise. These studies increasingly rely on biomarker-driven patient selection, enabling more precise targeting of therapies. There is also a significant rise in investigator-initiated trials, reflecting academic interest and investment in this phase for exploring emerging therapies and mechanisms of action. We have observed a notable increase in new trials launched during 2024 in the mid-phase space.

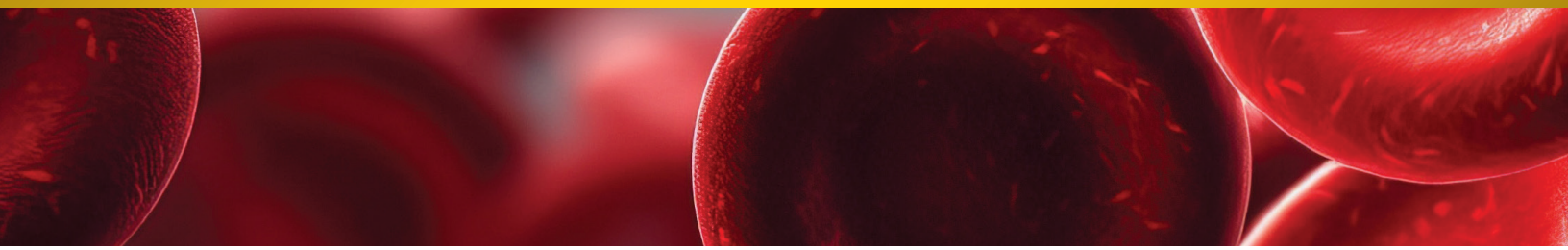
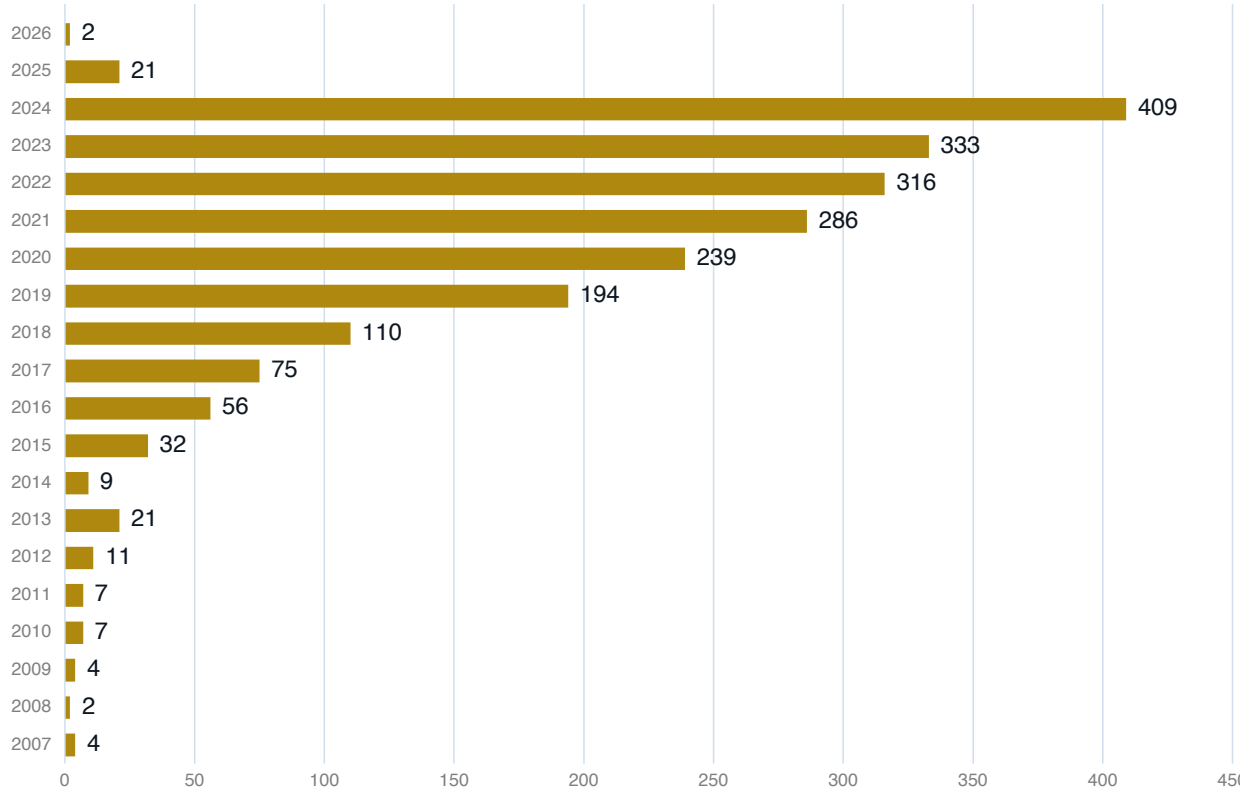


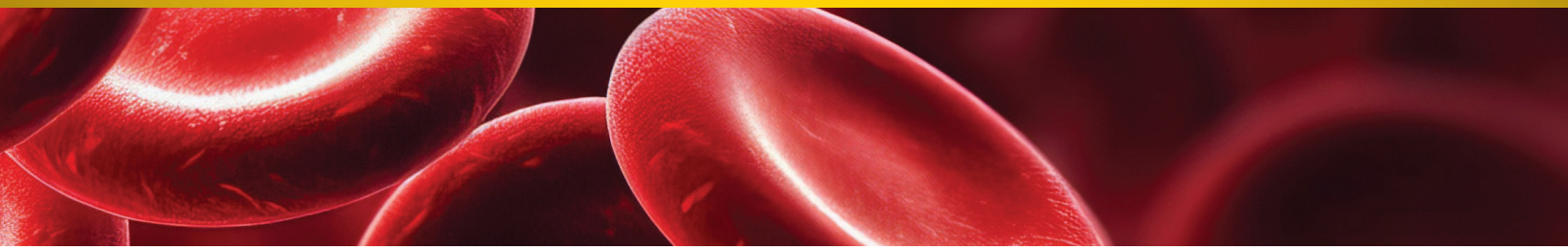
Figure 5. **Mid Phase Trials by Start Date**[11]



Source: Citeline, TrialTrove, Oct 2024.

Later-phase research in hematologic malignancies shows a relative decline in traditional Phase 3 trials. With enhanced regulatory flexibility for breakthrough therapies and more broad acceptance of robust Phase 2 data for conditional approvals, we are seeing an increased emphasis on Phase 3b and Phase 4 studies that focus on real-world evidence (RWE) and comparative effectiveness. Additionally, there is a growing number of post-marketing commitment studies to better understand how novel treatments perform relative to standard-of-care therapies in diverse, broad patient populations.

These distributions reflect the increasing complexity and innovation in hematologic malignancy research, with early-phase studies exploring cutting-edge therapies; mid-phase studies optimizing patient selection through innovative strategies; and late-phase trials focusing on real-world applicability and comparative outcomes.



Therapeutic Focus and Indication-Based Trends

The treatment landscape for hematologic cancers like leukemia, lymphoma, and myeloma has changed dramatically in recent years. This section delves into the primary cancer types in hematology-oncology, highlighting how new therapies are reshaping the way we approach these diseases.

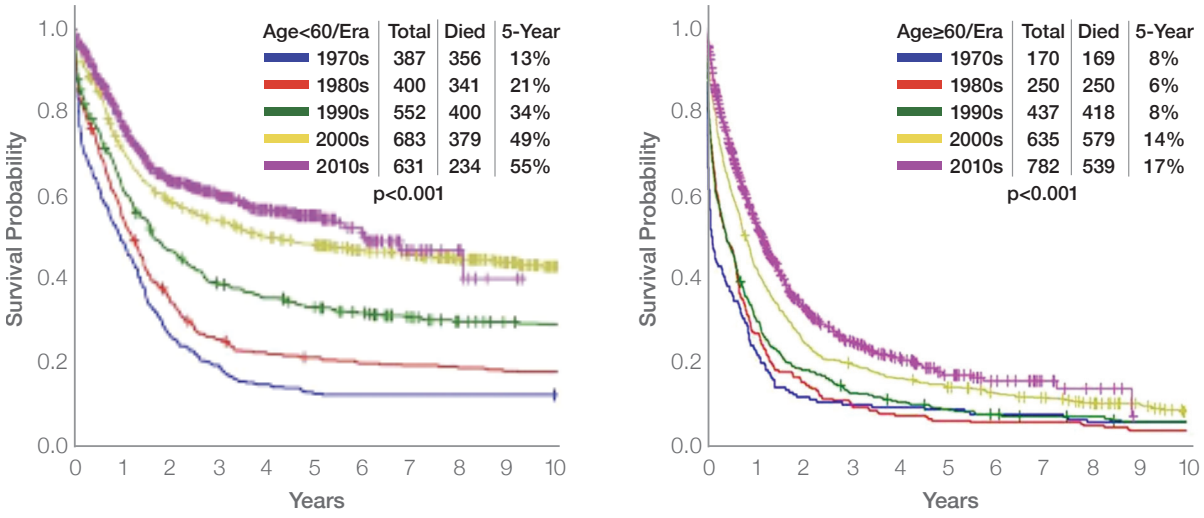
Leukemia

Recent therapeutic innovations in leukemia demonstrate marked advancement in immuno-oncology (I/O) and precision-targeted approaches, with minimal/measurable residual disease (MRD) emerging as a pivotal endpoint across modalities. Chimeric antigen receptor (CAR) T-cell therapies targeting CD19 have achieved unprecedented MRD-negative complete remission rates in acute lymphoblastic leukemia (ALL), particularly in relapsed/refractory (r/r) populations where Kymriah (tisagenlecleucel) has demonstrated complete remission rates exceeding 80%.17 The emergence of bispecific antibodies, exemplified by Blincyto's (blinatumomab) CD19/CD3 targeting, provides a therapeutic option for MRD-positive B-ALL patients.

MRD enables early identification of treatment response, which correlates closely with overall survival (OS). Of the approximately 2750 active trials in leukemia, 35% include MRD as a primary or secondary endpoint to facilitate faster decision-making in drug development. MRD-driven trials are transforming the drug development landscape by offering a faster, more precise way to evaluate treatment response. This has allowed researchers to better assess remission depth and long-term prognosis, particularly in high-risk subtypes.

In both AML and ALL, the use of MRD-driven trials has shown promise in streamlining drug approval processes, with regulators increasingly accepting MRD data as a path to accelerated approval.

Figure 6. **Survival of De Novo Acute Myeloid Leukemia at MD Anderson (1970-2017) by Age and Treatment Era**



Left panel: age <60 years; right panel: age 60+ years

Non-Hodgkin's Lymphoma

Over the past 5 years, several new treatments for NHL have received FDA approval, including Monjuvi (tafasitamab-cxix) in combination with lenalidomide on July 31, 2020,[18] for specific types of diffuse large B-cell lymphoma (DLBCL). Just over 6 months later on February 5, 2021,[19] a CAR-T cell therapy, Breyanzi (lisocabtagene maraleucel) received approval for large B-cell lymphoma. The approvals of Polivy (polatuzumab vedotin) and Ukoniq (umbralisib) for DLBCL and high-grade B-cell lymphomas on April 19, 2023,[20] have provided the most recent new therapeutic option for some NHL patients.

The integration of ctDNA monitoring and metabolic imaging, such as PET scans, into lymphoma trials offers more precise assessments of early treatment response, which can lead to faster trial conclusions.

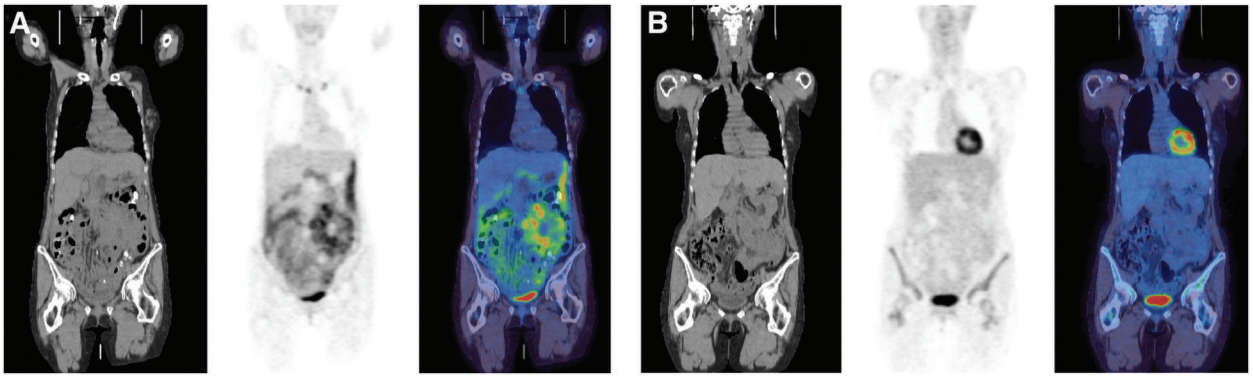
These treatments target specific molecular markers or engage the immune system in novel ways, marking a shift towards more personalized and effective therapeutic approaches in NHL. The increase in FDA-approved therapies over the past 5 years aligns with ongoing efforts in clinical research to address the high unmet needs for these patients.

Currently, there are more than 1120 trials globally focused solely on NHL. Trial designs for these

indications must balance short-term treatment response with long-term outcomes like progression-free survival (PFS), which can be tricky to measure.

For indolent subtypes, PFS remains a critical, yet difficult-to-measure endpoint. New strategies combining molecular data with imaging techniques are being developed to shorten trial durations without sacrificing accuracy in outcome assessments. By integrating ctDNA with metabolic imaging, researchers can gain a real-time, more nuanced view of a patient's response, minimizing the delays often seen in indolent lymphomas. This approach is particularly valuable in reducing the length of trials, making them more cost-effective and efficient.

Figure 7. **PET/CT Response Assessment in Lymphoma**



(A) At staging, patient with diffuse large B-cell lymphoma had extensive peritoneal and omental disease, which is often difficult to assess with CT. (B) Interim scanning after 2 cycles of chemotherapy showed complete metabolic response.

Graphic source: https://jnm.snmjournals.org/content/50/Suppl_1/21S

Myeloma

MM has seen the largest surge in clinical research over the past 10 years. In recent years, we have seen approval of 2 new CAR-T therapies and 3 bispecific T-cell engagers. Currently, the FDA-approved treatment options for myeloma patients who have received at least 4 lines of prior treatment include:

- Bispecific T-cell engagers
  - Teclistamab-cqyv (Tecvayli), a B-cell maturation antigen (BCMA) targeting CD3, was FDA-approved October 25, 2022.
  - Talquetamab (Talvey), a bispecific antibody-targeting CD3 and GPRC5D, was approved on August 9, 2023.
  - Elranatamab-bcmm (Elrexfio), a BCMA-targeting CD3, was FDA-approved on August 14, 2023.
- CAR-T therapy
  - Idecabtagene vicleucel (Abecma) received FDA approval in March 2021. On April 4, 2024, the FDA approved Abecma for the treatment of r/r MM after 2 or more prior lines of therapy including an immunomodulatory agent (IMiD), a proteasome inhibitor (PI), and an anti-CD38 monoclonal antibody.

– Ciltacabtagene autoleucel (Carvykti) was approved in February 2022. On April 5, 2024, the FDA granted approval for patients with r/r MM who have received at least 1 prior line of therapy, including an IMiD and a PI, and who are refractory to Revlimid.

These approvals were initially only available to patients requiring at least a fifth line of therapy. Although Carvykti is the first and only approved BCMA-targeted therapy approved for the treatment of patients with r/r MM who have received at least 1 prior line of therapy, Abecma is now approved as a third-line treatment.

Modern myeloma trials increasingly incorporate patient-reported outcomes (PROs) alongside MRD, reflecting the shift in focus towards improving quality of life in addition to clinical efficacy.

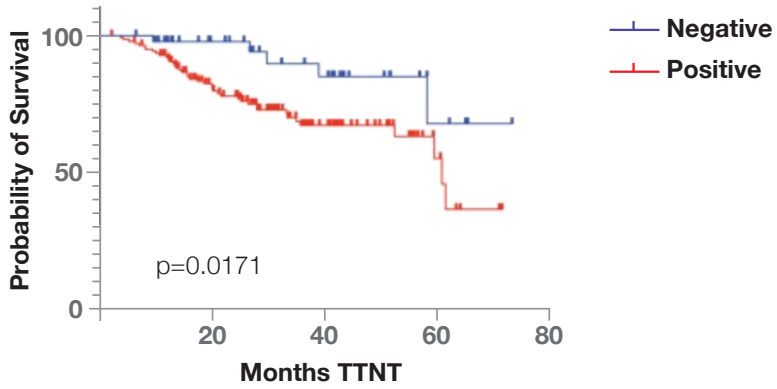
While there is great interest in developing new targets for MM, the overall saturation of the MM market means that novel therapeutics are frequently approved as later-line therapies. Subsequent clinical trials are conducted for label-expansion purposes to earlier lines of treatment. Of the 1093 active trials targeting myeloma, an additional 87 are using the 5 forementioned FDA-approved fifth-line CAR-T and bispecific therapies for myeloma. An additional 280 active trials are in a fourth-line or greater treatment setting, which will hopefully yield some therapies focused not only on achieving remission, but also on improving patients' quality of life by considering treatment-free intervals and sustained MRD negativity as part of the overall treatment plan.

The therapeutic landscape in hematologic malignancies demonstrates remarkable evolution across leukemia, NHL, and myeloma, characterized by increasingly sophisticated approaches to treatment and trial design. Recent approvals in these indications represent a paradigm shift in treatment approach, with unprecedented response rates in previously refractory populations. This is particularly evident in myeloma where approvals of cell therapies and bispecific antibodies have dramatically expanded the arsenal for a heavily pretreated patient population.

Looking forward, the field appears poised for continued innovation, with a clear trajectory toward more personalized therapeutic approaches, accelerated development pathways, and sophisticated trial designs. The challenge moving forward will be to balance the rising incidences in these indications with a rapid pace of therapeutic advancements.

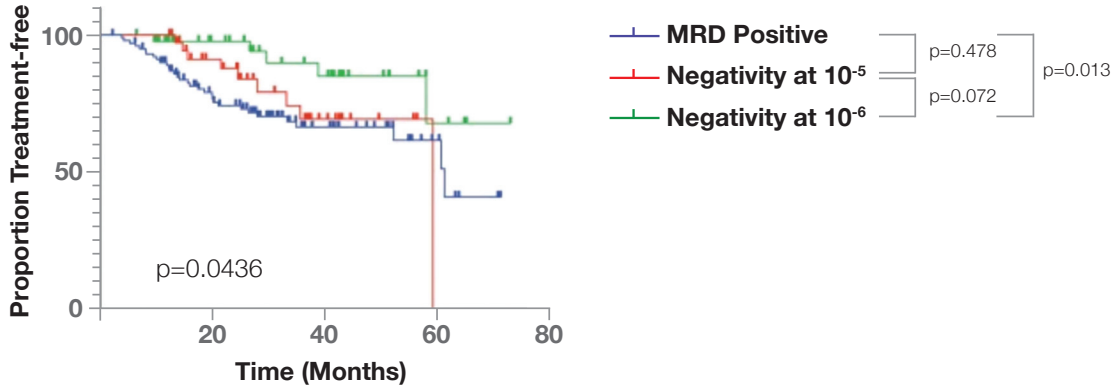
Figure 8. **TTNT by MRD Thresholds**

A) TTNT by MRD Negativity at 10-6 versus Positive



No. at Risk				
MRD Negative at 10-6	45	31	19	5
MRD Positive at 10-6	141	94	37	8

B) TTNT by MRD Negativity Threshold



No. at Risk				
MRD Positive	103	67	28	8
Negative at 10-5	38	28	10	1
Negaitve at 10-6	45	31	19	5

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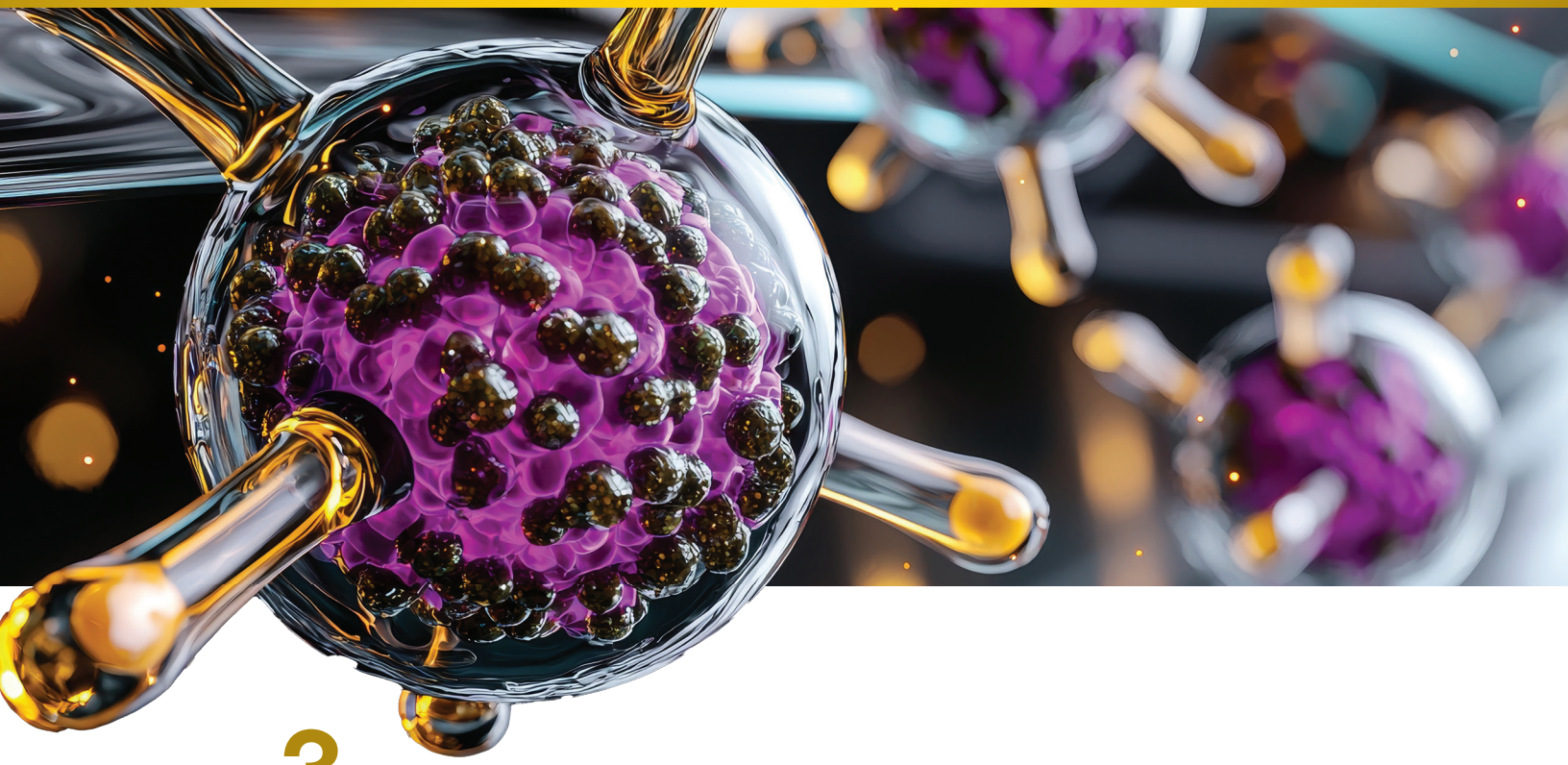


Chapter 3

Emerging Therapies and Innovations

In recent years, the development of innovative and emerging therapies has transformed treatment paradigms for many hematologic oncology indications. These therapies represent a clear shift toward more personalized, targeted, and immunotherapy-based approaches, moving away from traditional non-specific chemotherapy regimens. The promise of these emerging treatments lies in their potential to deliver superior efficacy while simultaneously minimizing toxicity, thus improving the overall quality of life for patients. As a result, they are poised to further revolutionize the management of hematologic malignancies, transitioning some indications from terminal to more chronic diseases. Below, we highlight some of the most promising and cutting-edge approaches currently under development.

Cell Therapies

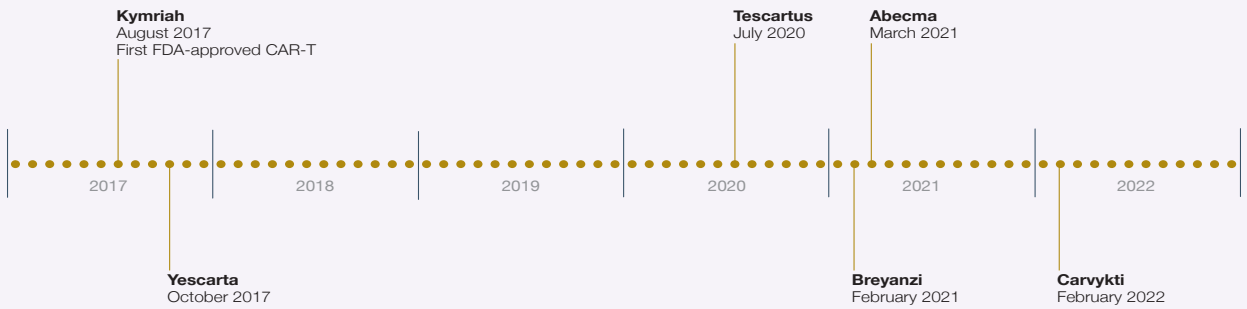
Cell-based therapies, particularly CAR-T therapy, have achieved significant breakthroughs in treating hematologic malignancies. These innovative therapies work by genetically altering a patient's immune cells, enabling them to more effectively recognize and destroy cancer cells.

Several of these therapies have received FDA approval for specific hematologic indications, including:

- Kymriah (tisagenlecleucel)
- Yescarta (axicabtagene ciloleucel)
- Abecma (idecabtagene vicleucel)
- Breyanzi (lisocabtagene maraleucel)
- Carvykti (ciltacabtagene autoleucel)
- Tecartus (brexucabtagene autoleucel)

As research progresses, the potential applications of cell therapy are anticipated to broaden, allowing for targeted treatments against a wider range of cancers and in earlier lines of therapy. This expansion holds promise for improving outcomes in blood cancers, as these ongoing advancements in the field may pave the way for more personalized and effective cancer treatments, with the hope of significantly enhancing patient survival and quality of life.

Figure 9. **Timeline of FDA-Approved CAR-T-Cell Therapies**

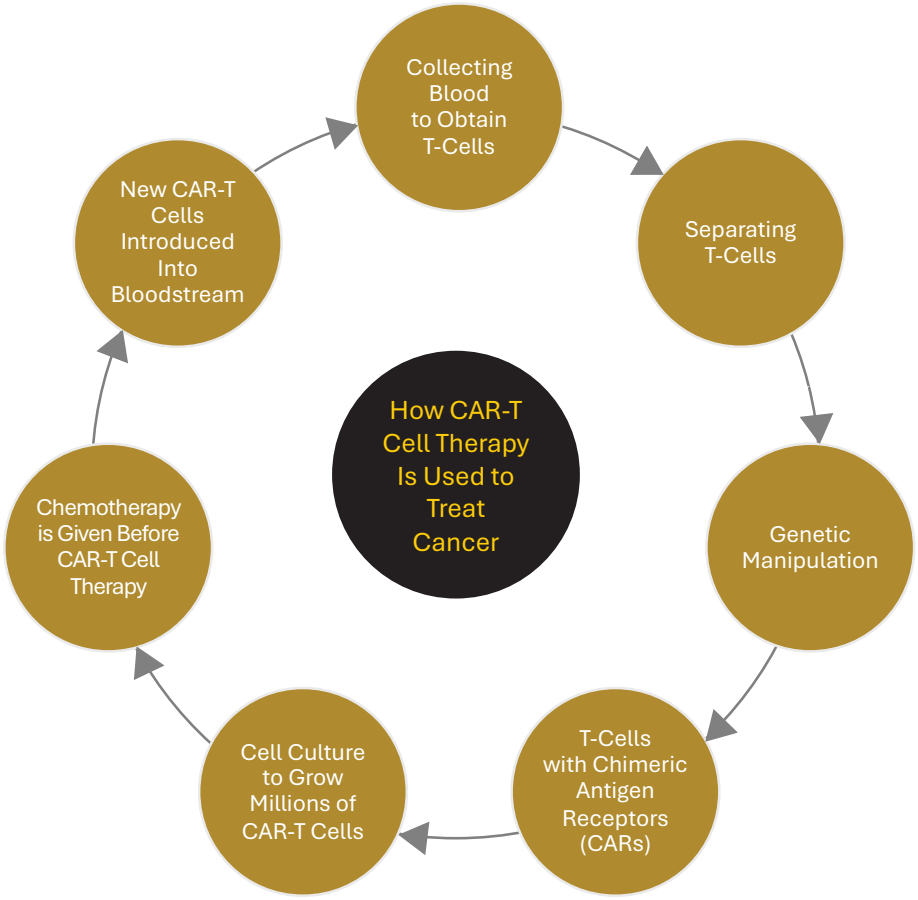


Producing CAR-T cell therapies used to take weeks, but thanks to new technologies in development, the process can now be completed in just a few days. This will make these treatments more scalable, opening the door for wider use.

These advances will not only cut costs but will address the scalability challenges that have hindered wider adoption. By streamlining production, companies will be able to manufacture at scale, paving the way for these therapies to be more widely accessible.

Innovations in manufacturing protocols, such as the use of IL-7 and IL-15 to boost T-cell fitness, have enabled shorter production times without compromising efficacy[21].

Figure 9. **Overview of Autologous CAR-T-Cell Manufacturing**



Targeted Therapies

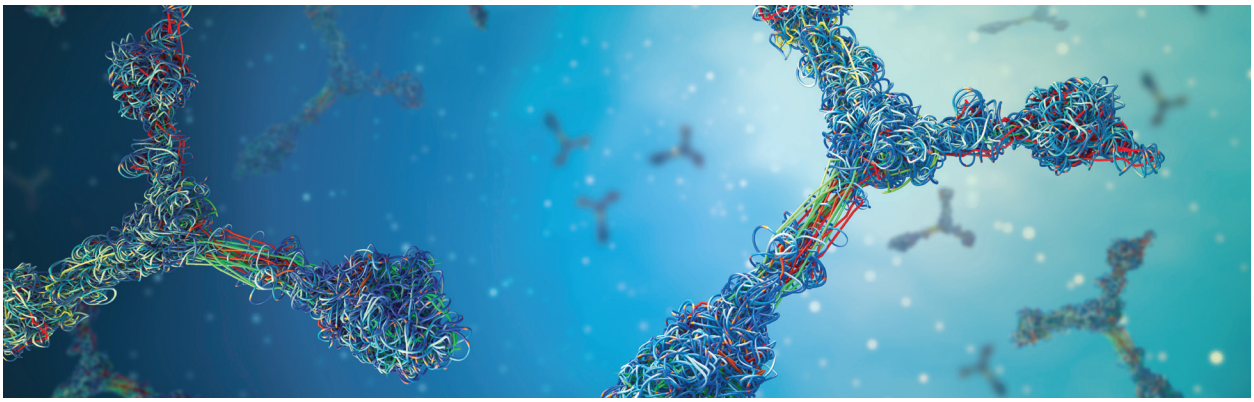
In hematologic cancers, a variety of targeted therapies have been developed to address specific genetic mutations or abnormal protein expressions driving disease progression. For example, tyrosine kinase inhibitors (TKIs) such as imatinib have transformed the treatment of chronic myelogenous leukemia (CML) by selectively targeting the BCR-ABL protein, a fusion protein that promotes cancer cell growth. Likewise, proteasome inhibitors like Velcade (bortezomib) and Kyprolis (carfilzomib) have proven highly effective in treating MM, contributing significantly to prolonged patient survival and disease management.

Targeted therapies represent an advanced cancer treatment approach that precisely focuses on specific molecular pathways or genetic mutations essential for cancer cell growth and survival. By inhibiting these critical pathways, targeted therapies can precisely inhibit key pathways that cancer cells rely on, thus blocking cancer progression with greater specificity than traditional chemotherapy, which often impacts both cancerous and healthy cells. This precision-driven approach aims to maximize effectiveness while minimizing harm to normal cells, thereby reducing side effects and improving the quality of life for patients.

Numerous targeted therapies have received FDA approval, emphasizing their clinical impact and utility in treating blood cancers. For instance, imatinib

(Gleevec) was the first TKI to receive FDA approval for CML, revolutionizing the standard of care by achieving high response rates and allowing many patients to achieve long-term remission. AML treatment has also substantially benefited from several new targeted therapies, including Tibsovo (ivosidenib) and Idhifa (enasidenib) for IDH1 and IDH2 mutations and Xospata (gilteritinib) and Vanflyta (quizartinib) for FLT-3 mutated AML. In CLL and SLL, FDA-approved agents such as Brukinsa (zanubrutinib) and Imbruvica (ibrutinib) have become integral in treatment protocols, offering effective options for patients that contribute to prolonged survival and improved outcomes.

These approvals underscore the importance of precision medicine in oncology and highlight the success of targeted approaches in treating hematologic malignancies. As research advances, additional targeted therapies are expected to emerge, further broadening the scope of personalized treatment options and offering patients increasingly effective and tailored options.



Bispecific Antibodies

Bispecific antibodies are engineered to bind to 2 distinct antigens simultaneously, typically one on a cancer cell and the other on an immune cell receptor. This dual-targeting mechanism allows them to actively recruit and stimulate the immune system to recognize and eliminate cancer cells with enhanced precision. Their unique approach has shown promise in hematologic malignancies, as they can directly engage immune cells, such as T-cells, to target and destroy malignant cells. Unlike traditional chemotherapy, which can affect both healthy and cancerous cells, bispecific antibodies are designed to bind the cancer cells and immune cell simultaneously to trigger an immune response, potentially reducing side effects and improving patient outcomes.

Several bispecific antibodies have already gained approval from the FDA, underscoring their clinical potential. These FDA approvals reflect the growing recognition of bispecific antibodies as transformative treatments within oncology, offering patients new options that leverage the immune system's inherent capabilities against cancer. As research continues, additional bispecific antibodies are expected to reach the market, broadening their applicability across various types of cancer and improving therapeutic strategies in oncology.

Table 1. **FDA-Approved Bi-specific Antibodies in Hematologic Malignancies**

Trade Name	Active Ingredient	Year Approved	Indication
Blincyto	blinatumomab	2014	To treat Philadelphia chromosome-negative relapsed or refractory B cell precursor ALL
Tecvayli	teclistamab-cqyv	2022	To treat relapsed or refractory MM
Lunsumio	mosunetuzumab-axgb	2022	To treat relapsed or refractory follicular lymphoma
Epkinly	epcoritamab-bysp	2023	To treat relapsed or refractory DLBCL
Columvi	glofitamab-gxbm	2023	To treat relapsed or refractory DLBCL or large B-cell lymphoma

Bispecific antibodies targeting CD19, CD20, BCMA, and GPRC5D are showing remarkable promise in preventing antigen escape, especially in relapsed or refractory patient populations. However, durability and toxicity remain areas of concern, particularly with cytokine release syndrome (CRS) and immune effector cell-associated neurotoxicity syndrome (ICANS). By reducing the need for multiple therapeutic regimens and simplifying administration and manufacturing (providing an "off the shelf" alternative to autologous cell therapy), these antibodies offer a less resource-intensive approach that is increasingly appealing to patients and healthcare systems.

Newer bispecific antibodies, with optimized dosing schedules and improved toxicity management protocols, are enabling outpatient administration for certain patients, reducing the burden on healthcare systems.

Table 2. **Single-Agent Response Rates in R/R MM**

Therapy	Modality	Target	Median prior lines	Manufacturing time, days	Infections/ grade ≥3, %	CRS/grade ≥3, %	ICANS/ grade ≥3, %	ORR/ CR, %	mPFS, mo	mOS, mo	EMD efficacy
Taiquetamab (800 µg Q2W)	BsAb	GPRC5D	5	N/A	34/7	80/0	5/0	73/38.7	11.9	12-mo OS 77.4%	ORR 45%
Teclistamab	BsAb	BCMA	5	N/A	76.4/44.8	72.1/0.6	14.5/0.6	63/39.4	11.3	18.3	ORR 36%
Elranatamab	BsAb	BCMA	5	N/A	73.6/26.4	65.1/1.2	5.8/2.3	61/35	NR; 15-mo PFS 50.9%	NR; 15-mo OS 56.7%	ORR 38%
Idecabtagene vicleucel	CAR T	BCMA	3	49	58/28	88/5	15/3	71/39	13.3	NR	ORR 55.7%; mPFS 7.2 mo
Ciltacabtagene autoleucel	CAR T	BCMA	2, 6	44	62/26.9	76.1/1.1	4.5/0.1	84.6, 97/73.1	34.9; 12-mo PFS 75.9%	NR; 12-mo OS 84.1%	NR

Abbreviations: BCMA, B-cell maturation antigen; BsAb, bispecific antibody; CAR T, chimeric antigen receptor T-cell therapy; CR, complete response; CRS, cytokine release syndrome; EMD, extramedullary disease; GPRC5D, G-protein-coupled receptor class C group 5 member D; ICANS, immune effector cell–associated neurotoxicity; mOS, median overall survival; mPFS, median progression free survival; N/A, not applicable; NR, not reached; ORR, overall response rate; OS, overall survival; PFS, progression-free survival; Q2W, every 2 weeks; R/R MM, relapsed/refractory multiple myeloma.

Antibody-Drug Conjugates

ADCs represent a cutting-edge cancer treatment that combines the targeted approach of monoclonal antibodies with the potent effects of cytotoxic drugs. By linking a chemotherapy agent to a monoclonal antibody, ADCs can deliver these powerful drugs directly to tumor cells, targeting specific antigens present on the cancer cell surface. This precise delivery mechanism helps to spare healthy tissues, thereby reducing systemic toxicity and minimizing side effects typically associated with conventional chemotherapy.

The FDA has approved several ADCs, highlighting their therapeutic potential and clinical value. For instance, Adcetris (brentuximab vedotin) is an FDA-approved ADC used in treating Hodgkin lymphoma and anaplastic large cell lymphoma. It targets the CD30 antigen, which is commonly expressed on malignant cells in these cancers, allowing precise drug delivery to tumor sites. Another FDA-approved ADC, Polivy (polatuzumab vedotin), has become an important treatment for certain types of DLBCL, targeting the CD79b protein, which is found on the surface of most B cells. These approvals underscore the FDA's recognition of ADCs as valuable tools in hematologic oncology, offering a balance of potency and precision that is challenging to achieve with traditional treatments.

ADCs have demonstrated significant promise in the treatment of hematologic malignancies, including various types of lymphoma and leukemia. By focusing on cell-specific antigens, ADCs can achieve targeted destruction of cancer cells, offering improved treatment outcomes for patients with blood cancers.

With ongoing research and development, ADCs continue to emerge as a key area in cancer therapeutics. As more ADCs gain FDA approval, they are expected to broaden the range of treatment options available for hematologic cancers and solid tumors alike, pushing the boundaries of precision medicine in oncology and providing new hope for patients facing challenging diagnoses.

Table 3. **FDA-Approved ADCs in Hematologic Malignancies**

Drug	Gemtuzumab ozogamicin	Brentuximab vedotin	Inotuzumab ozogamicin	Loncastuximab tesirine	Polatuzumab vedotin-piiq
Brand Name	Mylotarg	Adcetris	Besponsa	Zynlonta	Polivy
Target	CD33	CD30	CD22	CD19	CD79b
Indication	R/R AML	R/R HL , sALCL	R/R ALL	R/R DLBCL	R/R DLBCL
ORR	26%-33%	75%-86%	81%-88%	48%-70%	63%
CR	13%-16%	34%-38%	36%-39%	24%-46%	40%
Key Trial	ALFA-0701	SG035-0003	INO-VATE	LOTIS-2	POLARIX
FDA Approval	2000 (original), 2017 (re-approval)	2011	2017	2021	2019

Checkpoint Inhibitors

I/O has revolutionized cancer treatment, particularly through the development of immune checkpoint inhibitors (CPIs). These therapies target and block immune checkpoint-molecules used by tumors to evade immune detection, effectively “releasing the brakes” on the immune system and allowing it to recognize, target, and eliminate cancer cells. This approach has reshaped the treatment landscape, initially gaining momentum in the fight against solid tumors and now expanding into hematologic cancers, where it has shown promising results in certain types of lymphomas and acute leukemia.

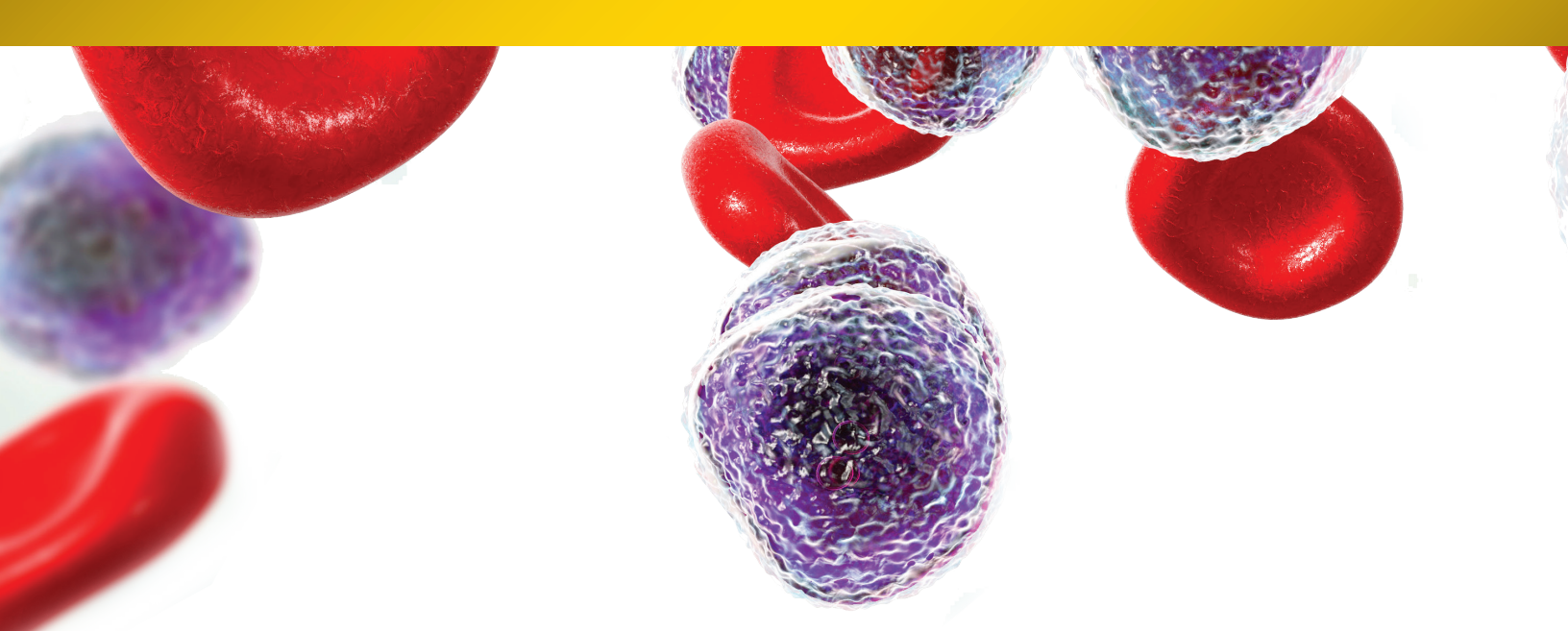
FDA approvals of CPIs underscore their transformative role in cancer care. For example, pembrolizumab (Keytruda) and nivolumab (Opdivo) were among the first CPIs approved for various solid tumors, including melanoma and non–small cell lung

cancer, demonstrating substantial improvements in patient outcomes and survival rates. These CPIs have since been approved for use in some hematologic malignancies. Nivolumab, for instance, has received FDA approval for use in relapsed or refractory classical Hodgkin lymphoma, marking an important step forward in immunotherapy for hematologic malignancies.

Additionally, CPIs remain predominately focused on solid tumors, and they are being studied in other hematologic cancers aside from Hodgkin lymphoma to determine if they may be effective in treating subtypes of lymphoma and leukemia. This expansion of I/O therapies provides renewed hope for patients who previously had limited treatment options, potentially offering longer-lasting responses and fewer side effects compared to traditional therapies.

The ongoing development and FDA approval of immune CPIs signal a new era of targeted cancer treatment, broadening options for patients across a range of cancer types. As I/O research continues to advance, CPIs and other immunotherapies are expected to become integral components of personalized cancer treatment, paving the way for innovative strategies in hematologic malignancies.

Combination strategies, particularly those involving immune CPIs, are reshaping the trial landscape, enabling faster recruitment and better outcomes in hematologic malignancies.



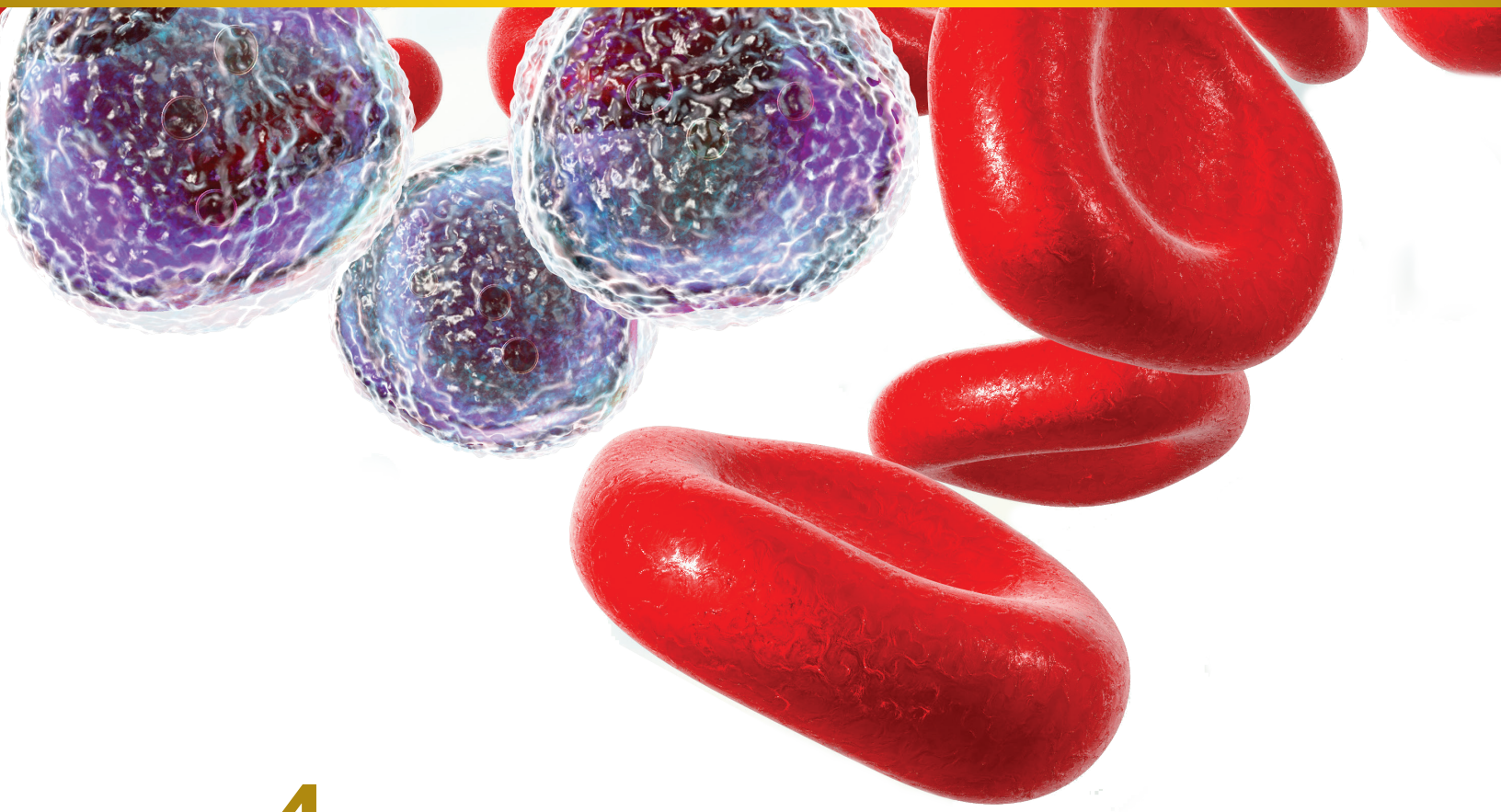
Conclusion

In conclusion, the expanding hematologic malignancy market is more than a response to rising incidence and an aging population; it represents a crucial shift in the approach to blood cancers, with profound implications for patient care and clinical research. As the prevalence of hematologic cancers like lymphoma, leukemia, and MM continues to grow, so does the demand for treatments that not only extend survival but also enhance quality of life. Innovative therapies, including targeted therapies, bispecific antibodies, ADCs, cell therapies, and I/O agents like CPIs, are meeting these needs with unprecedented precision and effectiveness.

These advancements push beyond the limits of traditional treatments, offering patients a new level of therapeutic specificity and durability. For healthcare providers and researchers, the development of these therapies marks a vital step toward personalized, less toxic treatments that adapt to the unique characteristics of each cancer. For patients, these innovations mean the possibility of longer remission, fewer side effects, and a greater quality of life.

This evolving landscape also underscores the importance of sustained investment in clinical research to address unanswered questions about optimal combinations, dosing, biomarkers, and long-term safety. By fueling a deeper understanding of these therapies, clinical research ensures that they are safely and effectively integrated into practice, ultimately providing patients with better outcomes and shaping the future of hematologic oncology care.

Hematologic malignancies, such as leukemia, lymphoma, and MM, have distinct clinical challenges that require innovative therapeutic approaches. As new treatments evolve, researchers are finding better ways to design trials, measure success, and improve patient outcomes.



Chapter 4

Clinical Trial Design Optimization

In the current environment of clinical trials, optimizing trial design is increasingly important. Researchers face the challenge of demonstrating both efficacy and safety, while also navigating complex regulatory requirements and the expectations of payers. Innovative trial designs are becoming essential in addressing these challenges. This discussion will examine how incorporating commercial considerations into trial design can improve market access and streamline the process of introducing new therapies to patients.

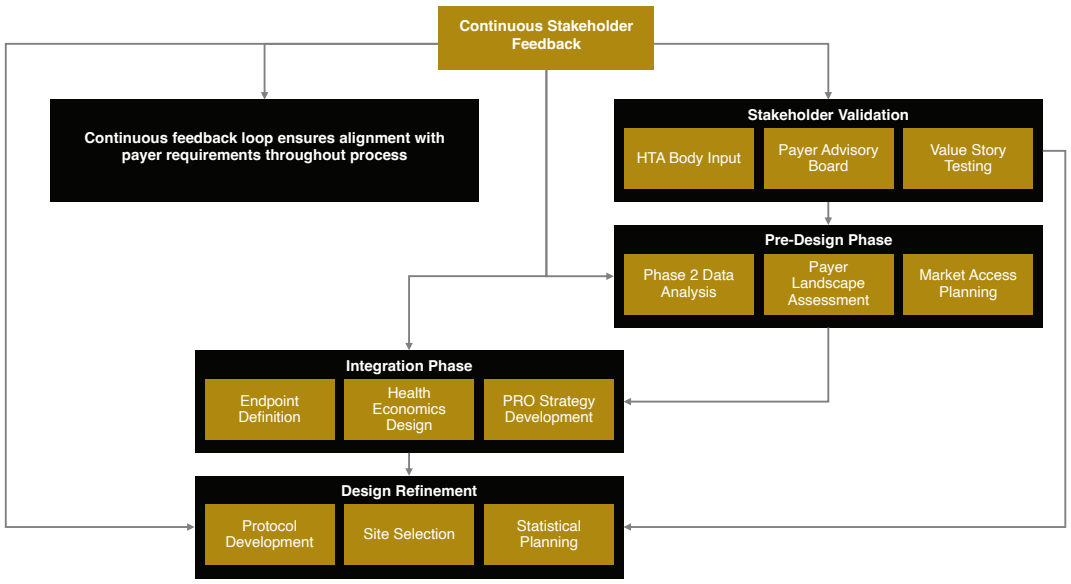
Key components of this approach include payer-rationalized trial design, which aligns clinical outcomes with payer needs, and the use of RWE, which provides insights from actual patient experiences. Additionally, decentralized clinical trials (DCTs) are gaining traction, offering flexibility and accessibility for participants. By focusing on these strategies, researchers can enhance the effectiveness of clinical trials, ensuring that new treatments reach patients efficiently while meeting the demands of regulatory bodies and payers. This balanced approach can contribute to a more effective clinical trial process without compromising on safety or efficacy.

Payer-Rationalized Clinical Trial Design

Integrating payer insights into Phase 3 clinical trial design—referred to as "payer rationalized clinical trial design"—is crucial not only for regulatory approval but also for overcoming access and reimbursement challenges in diverse global markets. Engaging payers early in the process ensures that trial endpoints, inclusion criteria, and study populations are aligned with payer expectations of clinical and economic value. This research can also help biopharma companies identify potential hurdles in securing timely market access, signaling when a launch may not be financially viable in certain countries. In these cases, it can inform clinical trial site selection by indicating where trial data may have the most impact and guiding companies toward regions where the commercial opportunity aligns with the clinical objectives. By considering these factors, biopharma companies can strategically allocate resources and optimize market entry planning.

> *"One of the significant challenges for manufacturers, whether large or small, is finding enough time between completing Phase 2 trials and moving into Phase 3 to incorporate payer insights effectively into regulatory submissions. Integrating these insights early can significantly impact market access success, but it requires careful planning and cross-functional collaboration to navigate these tight timelines."*
>
> *– Greg Gregory*

Figure 10. **Payer-Rationalized Design**



Trial Endpoints

In recent years, clinical trials for hematologic malignancies have expanded beyond traditional endpoints like OS and PFS to include more nuanced and meaningful measures of disease response, patient experience, and long-term outcomes. One such endpoint is MRD negativity, which refers to the detection of low levels of cancer cells remaining post-treatment, assessed through advanced methods like flow cytometry and next-generation sequencing. MRD negativity is now a strong predictor of long-term remission and survival, especially in diseases like myeloma and some forms of leukemias. In addition, event-free survival, which measures the time from treatment initiation until a predefined event such as relapse, progression, or death, offers a more immediate indication of therapeutic efficacy compared to OS and is particularly useful in conditions like acute leukemias, where early treatment response significantly correlates with long-term outcomes.

Patient-centered endpoints have also gained prominence. PROs, which capture patients' subjective experiences regarding symptoms, side effects, quality of life, and overall treatment satisfaction, are now critical for assessing the impact of treatment on patients' lives, especially as therapies are developed to minimize toxicity. Similarly, depth of response measures such as complete response, very good partial response, and stringent complete response are emerging as valuable endpoints, especially in MM, as deeper responses are often linked to MRD negativity and longer progression-free intervals.

Endpoints like TTNT and disease-free survival (DFS) are also gaining traction. TTNT assesses the duration from initial treatment to the need for subsequent therapy due to progression or relapse, providing a real-world measure of treatment durability. DFS, in which patients remain disease-free post-treatment, is especially valuable in trials with curative intent, such as in some lymphomas or leukemias, where long-term remission is a goal. Further, quality-adjusted life years (QALYs) and progression-free survival 2 (PFS2) have emerged as endpoints that offer a more nuanced view of therapeutic impact. QALYs combine survival with quality of life by adjusting survival time for quality, while PFS2 assesses the time from randomization to the second objective progression or death, capturing both initial and subsequent treatment outcomes to better evaluate the cumulative effect of sequential therapies on patients' quality of life.

Immunotherapy trials have also introduced immune-related endpoints, such as immune response markers and sustained immune activation. These are important for understanding the unique dynamics and durability of immune-based therapies, particularly with treatments like CAR-T-cell therapy or immune CPIs. In some chronic hematologic malignancies, treatment-free remission (TFR) has become an essential endpoint. TFR measures the duration a patient can maintain remission without ongoing therapy, which is increasingly relevant as patients seek periods of remission without continuous treatment, such as in CML.

Lastly, the incorporation of RWE endpoints is becoming more common, capturing treatment effectiveness, adherence, and outcomes within diverse populations outside of clinical trial settings. Let's dive further into the nuances of RWE.

Real-World Evidence

The integration of RWE in clinical trial design is evolving from a complementary approach to a core strategy, as regulatory bodies and payers increasingly prioritize evidence that reflects actual patient experiences. RWE can be used to guide the selection of clinically relevant endpoints that extend beyond traditional measures, such as PFS, to include outcomes that demonstrate real-world effectiveness, such as quality of life, healthcare utilization, or adherence patterns. Additionally, RWE can identify subpopulations with specific comorbidities or demographic characteristics that may experience differential treatment responses, thereby refining inclusion and exclusion criteria to ensure trials are appropriately powered for these nuances.

By leveraging RWE, biopharma companies can not only enhance the external validity of clinical trials but also proactively address anticipated questions from health technology assessment (HTA) bodies and payers regarding the generalizability of trial results to broader patient populations. This strategy allows for the generation of evidence that supports both regulatory approval and payer decision-making, potentially accelerating reimbursement timelines. Furthermore, the use of RWE in adaptive trial designs can facilitate ongoing adjustments based on interim data, thereby aligning trial execution more closely with real-world clinical practices and evolving standards of care.

> *"While the integration of real-world evidence into clinical trial design holds significant promise, we are still in the early stages of understanding how to fully operationalize this approach for long-term success. The challenge lies not only in incorporating RWE into study design but also in creating standardized methodologies and frameworks that can be consistently applied across trials. As the industry matures, we need to continue refining how we leverage RWE to ensure that it meaningfully enhances the clinical and economic value demonstrated to regulators and payers."*
>
> *– Greg Gregory*

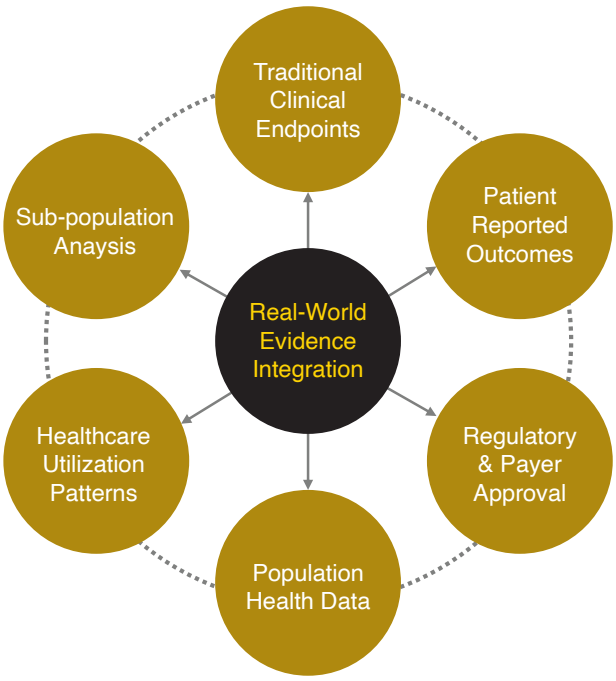
FDA Diversity Guidance

Incorporating the FDA's guidance for diversity into clinical trial design for hematologic malignancies is essential to ensure that clinical research is representative of the populations affected by these diseases. By following FDA recommendations, researchers can enhance the generalizability of trial findings, improve patient access, and ultimately lead to more effective and equitable treatment options. Several strategies are recommended to incorporate diversity into clinical trial design.

Design eligibility criteria that minimize unnecessary exclusions based on age, gender, race, ethnicity, and comorbidities. While maintaining safety, broader criteria can help ensure that the trial population better reflects the diversity of patients diagnosed with hematologic malignancies. Allow for flexible screening processes to accommodate patients with various healthcare access levels and those who may have different baseline health statuses.

Use qualitative research methods, such as focus groups or interviews, to gather insights from diverse patient populations about their preferences and barriers to participation.

This feedback can guide trial design to make it more appealing and accessible. Also, include PROs that are relevant to different demographics. Understanding the specific impact of treatment on quality of life, side effects, and daily functioning can help tailor therapies to diverse populations.



In summary, incorporating the FDA's guidance for diversity into clinical trial design for hematologic malignancies involves a multifaceted approach that begins with trial design. By broadening inclusion criteria, allowing for flexibility in screening, incorporating patient feedback, and using patient-centered endpoints, researchers can enhance the validity and applicability of trial findings, ultimately leading to improved treatments for all patients affected by hematologic cancers.

Decentralized Clinical Trials

Incorporating decentralization into the design of hematologic malignancy trials can offer benefits, such as increased patient accessibility, improved recruitment and retention rates, and enhanced data collection methods. Hybrid trials that include some decentralized elements, combined with traditional approaches, might be immediately feasible and provide opportunities for increasing participant reach, streamlining processes, and cost savings.

There remains some work ahead to understand when and how decentralized elements should be used in hematologic oncology trials. Implementing FDA guidance on DCTs into the design of hematologic malignancy trials involves:

- Adapting protocols to allow for remote patient assessments, where applicable
- Developing robust patient recruitment strategies, such as by using digital platforms
- Enhancing the informed consent process by implementing digital consent options
- Using remote monitoring and data collection by using items such as wearable devices to collect patient data in real time or by the use of mobile health applications for patients to report any adverse reactions, record drug compliance, and perform quality-of-life questionnaires
- Incorporating real-time data review and analysis for early identification of safety signals
- Providing training and education for site staff on DCT methodologies as well as ensuring that resources and materials are provided to support and educate patients about the DCT process

By thoughtfully implementing these strategies, researchers can effectively integrate FDA guidance on DCT into the design of hematologic malignancy trials. This approach not only enhances patient accessibility and engagement but also ensures compliance with regulatory standards, ultimately leading to more robust and relevant clinical research outcomes.

"With new treatments allowing me to live longer with myeloma, quality of life is a big concern. Not having to make my cancer the sole focus of living motivates me to continue to fight. Having options for treatments, follow-up procedures, and doctor visits gives me that freedom. I appreciate being able to do more from home or from places close to my home. Frequently navigating a large city and large hospital can be a big burden and time drain that impacts my ability to live and enjoy life as much as possible."

– quoted by actual myeloma patient, diagnosed 2015

Conclusion

The integration of payer perspectives and RWE into clinical trial designs represents a shift toward more comprehensive and practical approaches to drug development. By aligning trial designs with payer expectations and real-world scenarios, biopharma companies are better positioned to meet regulatory and market access requirements efficiently. The incorporation of diverse trial endpoints, including patient-centered and immune-related measures, enriches the data landscape, offering a nuanced understanding of therapeutic efficacy and patient quality of life.

Moreover, the emphasis on RWE underscores the industry's commitment to generating data that accurately reflect patient experiences and treatment outcomes beyond the controlled trial environment. This approach not only enhances the external validity of trials but also facilitates more informed decision-making by HTA bodies and payers, potentially expediting reimbursement processes.

In addition, adhering to FDA diversity guidance and integrating decentralized trial components further broadens the inclusivity and reach of clinical research. By designing trials that reflect the demographic diversity of the patient population and incorporating digital tools for remote data collection, researchers can ensure that clinical outcomes are representative and applicable to broader patient groups.

Ultimately, the convergence of these strategies within clinical trial design aims to deliver therapies that are not only effective and safe but also accessible and equitable to all patients. As the industry continues to evolve, the ongoing challenge will be to refine these methodologies, ensuring they are consistently applied and that they yield meaningful insights into the clinical and economic value of new treatments. Through thoughtful integration of these elements, the future of clinical trial design holds the promise of more robust, relevant, and patient-centric research outcomes that advance healthcare for all.

Full Conclusion

Advancing Hematologic Malignancy Research

The expanding hematology-oncology market is more than a response to rising incidence and an aging population; it represents a crucial shift in the approach to hematologic malignancies, with profound implications for patient care and clinical research. As the prevalence of hematologic indications such as lymphoma, leukemia, and MM continues to grow, so does the demand for treatments that not only extend survival but also enhance quality of life.

Innovative therapies, including targeted therapies, bispecific antibodies, cell therapies, ADCs, and CPIs, are meeting these needs with unprecedented precision and efficacy. These advancements push beyond the limits of traditional treatments, offering patients a new level of therapeutic specificity and durability. For healthcare providers and researchers, the development of these therapies marks a vital step toward personalized treatments with reduced toxicity. For patients, these innovations mean the possibility of longer remission, fewer side effects, and an increase in quality of life.

This evolving landscape also underscores the importance of sustained investment in clinical research to address unanswered questions about optimal combinations, dosing, biomarkers, and long-term safety. By fueling a deeper understanding of these therapies, clinical research ensures that they are safely and effectively integrated into clinical practice, ultimately providing patients with better outcomes and shaping the future of hematologic oncology care.

As the industry continues to innovate and push the boundaries of what is possible in hematologic malignancies, the challenge will be to balance the rising incidences in these indications with a rapid pace of therapeutic advancements. Through thoughtful integration of diverse strategies, from innovative trial designs to the incorporation of real-world evidence, the future of hematology-oncology holds the promise of more robust, relevant, and patient-centric research outcomes that advance healthcare for all.

Authors

Jen Vance

Jen Vance is a seasoned hematology/oncology expert at Precision, bringing over 20 years of experience in the CRO industry. She has a proven track record in portfolio management, clinical development planning, therapeutic strategy, and providing in-depth therapeutic expertise. Jen has extensive experience in hematology-oncology drug development, spanning Phase 1 to Phase 4 clinical trials. She specializes in hematologic malignancies and has worked across a broad range of therapeutic approaches, including traditional chemotherapies, targeted therapies, cell therapies, chemotherapies, checkpoint inhibitors, bispecific antibodies and anti-body drug conjugates.

Jen has extensive expertise in both myeloid and lymphoid hematologic malignancies, with a focus on a wide range of conditions. Her experience includes working with myelodysplastic syndromes, myelofibrosis, acute myeloid leukemia (AML), chronic myelogenous leukemia (CML), and chronic myelomonocytic leukemia (CMML). In the lymphoid malignancy space, Jen has significant experience with acute lymphoblastic leukemia (ALL), as well as a broad range of non-Hodgkin lymphoma (NHL) subtypes, including diffuse large B-cell lymphoma (DLBCL), follicular lymphoma (FL), marginal zone lymphoma, mantle cell lymphoma, chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL), peripheral T-cell lymphoma (PTCL), cutaneous T-cell lymphoma (CTCL), and Waldenström macroglobulinemia (WM).

Jen blends deep therapeutic insight with strategic operational expertise to help clients accelerate the path from molecule development to market. Her ability to navigate both the scientific and operational aspects of drug development ensures efficient, targeted progress toward regulatory approval and commercialization.

Greg Gregory

Greg is a recognized leader with more than 25 years of experience shaping global market access, pricing, and commercialization strategies in the life sciences sector. Specializing in rare diseases, orphan drugs, and vaccines, Greg is known for delivering innovative pricing models and access strategies that have driven growth for top biopharmaceutical firms and small emerging companies alike.

In his role as EVP & Managing Director of Precision AQ's Commercial Consulting team, Greg oversees global strategic and analytic consulting practices, leading initiatives that have expanded market access, optimized reimbursement models, and advanced value-based care. His deep expertise spans a range of therapeutic areas, including immunology, hematology, oncology, respiratory, rare diseases, and digital therapeutics.

With a strong foundation in biomedical research, Greg blends scientific insight with business strategy to help clients navigate payer challenges and improve patient access globally.

References

1. Leukemia & Lymphoma Society. Facts and Statistics | Leukemia and Lymphoma Society. Lls.org. Accessed October 30, 2024. https://www.lls.org/facts-and-statistics/facts-and-statistics-overview
2. Cancer Tomorrow. Global Cancer Observatory. Accessed October 30, 2024. https://gco.iarc.fr/tomorrow/en/dataviz/bars?cancers=34_35_36&group_cancers=0&multiple_cancers=1&bar_mode=stacked&multiple_populations=0&types=0&mode=cancer&populations=900&years=2045
3. World Social Report 2023: Leaving no one behind in an ageing World. United Nations. Accessed October 30, 2024. https://www.un.org/development/desa/dspd/wp-content/uploads/sites/22/2023/01/WSR_2023_Chapter_Key_Messages.pdf
4. Non-Hodgkin's Lymphoma - Cancer Stat Facts. SEER. Accessed October 30, 2024. https://seer.cancer.gov/statfacts/html/nhl.html
5. Leukemia - Cancer Stat Facts. SEER. Accessed October 30, 2024. https://seer.cancer.gov/statfacts/html/leuks.html
6. Common Cancer Types. National Cancer Institute. Accessed October 30, 2024. https://www.cancer.gov/types/common-cancers#:~:text=The%20most%20common%20type%20of,are%20combined%20for%20the%20list
7. Leukemia - Cancer Stat Facts. SEER. Accessed October 30, 2024. https://seer.cancer.gov/statfacts/html/leuks.html
8. https://www.biospace.com/laxman-dige-precedenceresearch-com
9. https://finance.yahoo.com/news/advancements-targeted-therapies-immunotherapies-propels-162800995.html
10. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
11. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
12. Leukemia & Lymphoma Society. Facts and Statistics | Leukemia and Lymphoma Society. Lls.org. Accessed October 30, 2024. https://www.lls.org/facts-and-statistics/facts-and-statistics-overview
13. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
14. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
15. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
16. Citeline. Trialtrove. Accessed October 30, 2024. https://clinicalintelligence.citeline.com/trials
17. Pasquini MC, Hu ZH, Curran K, et al. Real-world evidence of tisagenlecleucel for pediatric acute lymphoblastic leukemia and non-Hodgkin lymphoma. Blood Advances. 2020;4(21):5414-5424. doi: https://doi.org/10.1182/bloodadvances.2020003092
18. Research C for DE and. FDA grants accelerated approval to tafasitamab-cxix for diffuse large B-cell lymphoma. FDA. Published online June 11, 2021. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-grants-accelerated-approval-tafasitamab-cxix-diffuse-large-b-cell-lymphoma
19. Research C for DE and. FDA approves lisocabtagene maraleucel for relapsed or refractory large B-cell lymphoma. FDA. Published online June 11, 2021. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-approves-lisocabtagene-maraleucel-relapsed-or-refractory-large-b-cell-lymphoma
20. Research C for DE and. FDA approves polatuzumab vedotin-piiq for previously untreated diffuse large B-cell lymphoma, not otherwise specified, and high-grade B-cell lymphoma. FDA. Published online April 20, 2023. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-approves-polatuzumab-vedotin-piiq-previously-untreated-diffuse-large-b-cell-lymphoma-not
21. Sheykhhasan M, Ahmadieh-Yazdi A, Vicidomini R, et al. CAR T therapies in multiple myeloma: unleashing the future. Cancer Gene Therapy. Published online March 4, 2024:1-20. doi:https://doi.org/10.1038/s41417-024-00750-2

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