IQVIA INSTITUTE FOR HUMAN DATA SCIENCE

Hematologic Cancer Opportunities for Patient Equality (HOPE)

ACHIEVING EQUITABLE AND TIMELY ACCESS TO HEMATOLOGICAL CANCER THERAPIES

Summary highlights from a multi-stakeholder panel discussion at the World Cancer Congress on September 17, 2024

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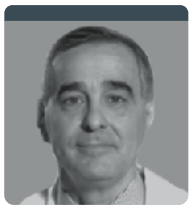

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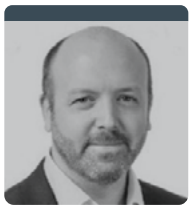

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The views of speakers do not necessarily represent those of their organizations.

Introduction

The IQVIA Institute for Human Data Science convened an in-person and virtual multi-stakeholder symposium at the World Cancer Congress in Geneva on September 17, 2024. The panel discussion, Hematologic Cancer Opportunities for Patient Equality (HOPE): Achieving Equitable and Timely Access to Hematological Cancer Therapies, covered the challenges and opportunities in accessing hematological cancer therapy across various regions. Participants included those with expertise across Europe and national experts from Canada and Poland. Building on the IQVIA Institute's published report on *Hematological Cancer Opportunities for Patient Equality (HOPE): Geographic disparities in access, reimbursement and hematology therapy utilization*, the aim of this session was to further the ongoing discussion about timely access to hematological cancer therapies.

The panelists comprised a broad range of stakeholders including hematology-oncology clinicians, policy leaders, patient advocates, and ex-payers.

This summary provides highlights from the discussion.

The symposium was organized by the IQVIA Institute for Human Data Science as a public service with funding from AbbVie and was intended to continue the timely conversation and propose a call to action for equitable patient access to hematology-oncology therapies.

Background

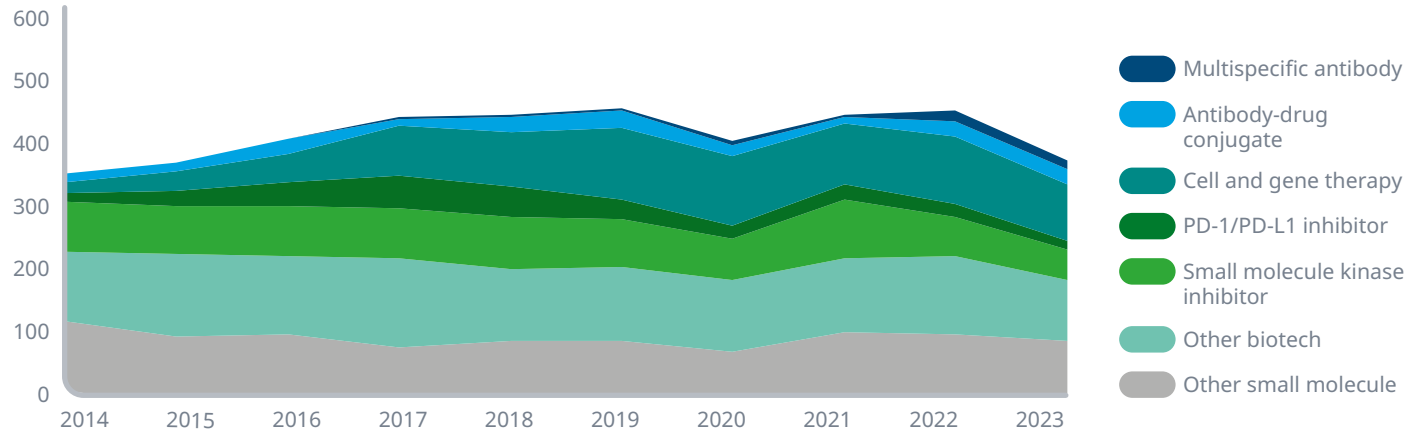
Advancements in oncology treatment have been steadily growing and potentially bring new treatment options to patients suffering from hematological malignancies. Research from the IQVIA Institute and many others have found that the availability and utilization of oncology therapies differ drastically across various geographies, leading to disparities in patient outcomes.[1–3] The symposium opened with an overview of the research findings from the report published by IQVIA Institute in June 2024 as an introduction to the themes of the panel discussion.

The findings highlighted that hematological cancer care is advancing rapidly due to the development, approval, and growing utilization of innovative modalities such as second and third generation tyrosine kinase inhibitors (TKIs), antibody-drug conjugates (ADCs), immunomodulatory drugs (IMiDs), and chimeric antigen receptor T cells (CAR T). However, despite meaningful improvements in clinical efficacy and quality of life metrics, high unmet need remains.

The current global research and development pipeline for hematological cancer also remains robust and therapies with novel mechanisms of action such as multi-specific antibodies, and cell and gene therapies are expected to emerge (Figure 1). Given this context, two key priorities are identified: health systems need to ensure existing therapies are readily available to patients while also preparing for additional waves of innovative therapies to enter the market.

The presence of significant differences in how patients across countries access hematological cancer therapies is largely driven by systemic factors such as variations in health technology assessment (HTA) processes, country level prioritization of clinical endpoints, and requirements for maturity of clinical data or manufacturer strategy. While these factors are shown to lead to limited or delayed access, some hematology cancer drugs can achieve faster access due to a strong efficacy profile, through targeting of restricted populations or through use of value-based contracts in payer negotiations.

Figure 1: Hematological cancer clinical trial start Phase I–III, by primary tested drug type, 2014–2023



Source: Global Trends in R&D 2024: Activity, Productivity, and Enablers. Report by the IQVIA Institute for Human Data Science. Hematologic cancer Opportunities for patient Equality (HOPE): Geographic Disparities in Access, Reimbursement and Hematology Therapy Utilisation. Report by the IQVIA Institute for Human Data Science.

Highlights from the panel discussion

The panel discussion centered on the following themes:

+ **Dynamics of hematological cancer drug clinical trials and payer evaluation**
+ **Therapy funding, sub-national access, and capacity**
+ **Ongoing HTA policy and other legislation and initiatives to increase access to therapy**

DYNAMICS OF HEMATOLOGICAL CANCER DRUG CLINICAL TRIALS AND PAYER EVALUATION

- There is stakeholder consensus that overall survival (OS) is the "gold standard" endpoint that measures clinical benefit by capturing the length of time patients are still alive after trial randomization.[4] Payer/HTA bodies prefer the availability of OS data. However, this endpoint may not always be reached for hematologic cancer conditions at the time of approval and payer assessment given the length of time it takes to capture that metric.

"OS is not a valid parameter to measure [patient] outcomes and give value to a drug. In some pathologies such as MM, NHL, and FL, we see the advantage of survival in around a decade. For this reason, I think we must move beyond OS and utilize other surrogate biomarkers that are important such as MRD and PFS. Perhaps these parameters should be enough to introduce the drug and later we can wait for real world evidence studies to decide if we want to keep reimbursing the therapy or not."

Francisco Javier Lòpez Jimènez, MD, PH.D,
Head of Hematology,
Ramón y Cajal University Hospital

- Advances in treatment of hematological cancer disease, especially in early stages, have prolonged patient survival and endpoints that capture quality of life metrics have increasingly become important. For hematological cancer trials (and other tumor types), use of surrogate endpoints (e.g., progression free survival (PFS), minimum residual disease (MRD), and patient reported outcomes (PRO)) to establish therapy value in the HTA process are imperative as they are proposed as more suitable endpoints (in addition to OS) given their capacity to capture more nuanced understanding of a therapy's effectiveness.[5]

- Regulators are more open to accepting and approving hematology-oncology therapies based on data submission from early phase trials given high unmet need. However, there are challenges in providing access to these therapies approved based on Phase I-II data, including prolonged wait times for reimbursement compared to therapies approved based on Phase III clinical submissions.[3]

- Panelists also mentioned that payer/HTA bodies have preferences for clinical evidence assessed using randomized clinical trials (RCTs) posing challenges for therapies treating rare and pediatric conditions where patient numbers are extremely limited.

- Additional complexities are also present while assessing the value of combination therapies for hematological cancer treatment. Traditionally, payer/HTA bodies have used criteria designed for monotherapies or combinations with a single novel branded component. These cost-effectiveness or budget impact criteria often fail to fully capture the value of therapies that combine two novel agents or in some cases the combination of branded and generic therapies.[3]

- More adaptive and flexible HTAs are needed, which can be achieved through alignment from stakeholders including physicians, patients, patient advocacy groups, regulators, payers, and policy makers. This is key to first help drive the understanding and then the standardization of disease relevant endpoints. Following this, methodologies that ensure consistent and robust data collection to generate evidence that demonstrate clinical value of novel therapies should be developed.

"Currently, the HTA procedures consider evidence only from randomized clinical trials and we know that it is solid evidence. However, it is not the only evidence that they should be considering because there are so many treatments for which it is not possible to conduct randomized clinical trials [due to the low size of certain patient populations or ethical issues with pediatric cancer treatments]. We submitted a statement endorsed by 39 stakeholders to the European Commission after which they recently expressed "openness" to consider alternative evidence. While not the ideal response, it gives us hope for changes in methodological guidelines to accelerate access and improve patient survival."

Antonella Cardone, CEO, Cancer Patients Europe

THERAPY FUNDING, SUB-NATIONAL ACCESS, AND CAPACITY

Highlighted discussion topics include:

- Over the past decade, the number of approved therapies for hematological cancer has increased, offering clinicians and patients a broader range of treatment options. However, approval and reimbursement of these therapies vary significantly across geographies, leading to suboptimal patient outcomes.[1–3]

Public finance challenges

- For countries such as Poland, high prices have historically limited access to new hematologic cancer products. For example, a decade ago health expenditures were ~€700 per citizen which was approximately one quarter the investment of other countries in Europe. This number has now doubled given a tremendous growth in the economy but is still significantly lower than the average spending in the European Union.

"While access to treatment in Poland has improved substantially since 2019, we still wait long times for diagnosis, lab tests, gene tests, molecular tests, etc., and this also needs to be improved to help the system."

Maciej Milkowski,
Former Finance Director/VP of the NFZ, Former Deputy MoH, Poland

- Poland has improved access to novel hematological cancer therapies since 2019 through additional healthcare funding, and the next challenge is that of infrastructure. There is a shortage of a skilled healthcare workforce such as physicians and diagnostic laboratory technicians contributing to a strained health care system with increased patient demand. Therapy access delays caused by infrastructure constraints such as long wait times for diagnostics (e.g., biomarker identification, use of companion diagnostics) then lead to lower utilization of novel hematological cancer therapies and has the potential to impact treatment outcomes.

Sub-national access challenges

- In cost-effective markets like Canada, the HTA assessment process usually defines patient eligibility restrictions based on clinical trial evidence and provides guidance to funders on place in therapy and therapy sequencing options, which may be narrower than what was submitted by the manufacturer. This is followed by national drug price negotiations, which vary in timing due to the provincial healthcare system. Patients in some provinces will subsequently have delayed access to therapies compared to other provinces.
- While HTA process and pricing negotiations occur at the national level, the final decision to provide access is made at the provincial level. This can lead to access delays due to differing spending priorities.
- The variability in access to hematological cancer therapies is also driven by the need for clinician and health system expertise. In Canada, where hospital care is very strained, the need for infrastructure and capacity to allow for patient access across regions to implement new therapies, irrespective of successfully going through the HTA process and national pricing exercise, can often be a barrier to access.

Capacity

- Europe and other regions face a rising cancer treatment demand due to increasing disease incidence and an aging population. Supply-side challenges include public finance constraints and an underappreciated growing crisis of health workforce shortage in key areas such as pathology and nursing, straining the healthcare system's ability to provide access to therapies.

"Even with national pricing, healthcare in Canada is at the provincial level. If we don't have a national pharmaceutical care program or national decision making on priorities of spending, then provincial jurisdiction decides on access for the population that they serve, which can lead to further disparities in availability."

Kathleen Gesy,
Former Provincial Director of Oncology Pharmacy Services, Saskatchewan Cancer Agency

- Alongside these constraints, there is a need to create a baseline understanding at the European and global level to compare country performance in access and utilization of hematological cancer therapies and other treatments.

ON-GOING HTA POLICY, OTHER LEGISLATION AND INITIATIVES TO INCREASE ACCESS TO THERAPIES

Discussion covered the following efforts:

- Joint Clinical Assessments (JCA) and Joint Scientific Consultations (JSC) are among the initiatives in place to promote collaboration and harmonization of HTA practices across Europe.
- The current HTA reform is tasked with the goal of improving the availability of novel therapies to patients through the creation of a sustainable HTA network across Europe, developing reliable, timely, and transparent information. It supports collaboration among European HTA organizations by facilitating efficient resource use, creating a sustainable knowledge-sharing system, and promoting good practices in HTA methods and processes.[6]
- Panelists emphasized that although the new EU HTA regulations aim to reduce the time to availability, ongoing measurement and stakeholder engagement are essential to ensure these regulations are effective and accurate from the outset.
- Additionally, involving patients and patient organizations in the HTA process will be crucial, as access to medicines affects not only patient health outcomes but also daily living, including employment. Policymakers should consider these indirect opportunity costs during drug negotiations.
- Reformed HTA systems must adapt to varying evidence availability (e.g., survival data challenges) and other needs (e.g., combination treatments) for hematological cancer drugs. This ensures a balance between timely patient access and the safety and efficacy of therapies.
- Panelists highlighted three key indicators of success for the new EU HTA regulations: reduced time to therapy access for patients, acceptance of new joint clinical assessments by countries, and the HTAs' adaptability to novel treatments.

"On paper, the objectives of the new HTA regulations in Europe are excellent in terms of accelerating access and increasing transparency, but we need to make sure that they are implemented properly."

Antonella Cardone,
CEO, Cancer Patients Europe

- In other geographies such as Canada, on-going initiatives to increase patient access include participation in Project Orbis, which is an initiative among international partners such as the FDA, Health Canada (HC), Australian Therapeutics Goods Administration (TGA), Brazil's National Health Surveillance Agency (ANVISA), and many others to accelerate access through joint regulatory submissions and review of oncology therapies among countries.[7]
- Canada has also introduced a time-limited recommendation approach, allowing drugs with early promising data to be recommended for reimbursement for a specific period. This acknowledges both the potential and the uncertainty of the therapy and provides patients with timely access.
- At the drug pricing level, Canada's pricing authority has identified a temporary access pricing program called pan-Canadian Pharmaceutical Alliance Temporary Access Process (pTAP) which is a mechanism for faster pricing of products that have been accepted under the time-limited reimbursement (TLR) pathway aimed at allowing patients to get early drug access.[8]

"In my opinion, the key indicators of success for the JCA are: 1. the reduction in time for patients to access therapies; 2. the extent to which new joint clinical assessments are accepted by countries; and 3. the adaptability of these assessments in quickly emerging areas of science and how receptive HTAs are to novel treatments."

Richard Price,
Head of Policy, ECO

A call-to-action

The call to action coming out of the panel discussion is centered around five key themes:

1. **Define and standardize clinical trial endpoints relevant for hematology-oncology therapies**
    - Stakeholder consensus (e.g., clinicians, regulatory agencies, payer/HTA bodies) on the type of surrogate clinical endpoints and their appropriate use need to be reached.
    - Continued validation of surrogate endpoints through methodological data collection process needs to be established to provide clear evidence of continued clinical efficacy and safety.
2. **Evolve current value assessment and pricing approaches for hematologic cancer therapies**
    - Once tumor-relevant endpoints have been standardized, the HTA review process should incorporate and adapt these endpoints during the value assessment exercise.
    - A robust value assessment system that balances data from hematological malignancy therapies that are regulatory approved based on early phase data and the need for faster patient access need to be developed.
    - Hematological cancer treatments are administered as doublet, triplet and even as quadruplet regimens. Methods for the evaluation of clinical benefit and pricing of combination therapies to ensure timely access for patients need to be established.
    - Availability of data regarding country level access and utilization of hematological cancer therapies (and other treatments) will enhance collective understanding of country level performance in terms of healthcare delivery and patient outcomes.
3. **Track and monitor the success and challenges of HTA reform initiative to determine efficiency and to course correct at the outset**
    - Monitoring the implementation of the JCA within the next year will be important to assess whether there is any improvement in time to access of therapies.
    - Close monitoring and evaluation will enable stakeholders to promptly identify and rectify any challenges.
4. **Coordinate health system readiness alongside regulatory and reimbursement evaluation of hematological cancer therapies**
    - Hematologic cancer care has become more complex requiring expertise from physicians and health systems.
    - As novel therapies are entering the market, it is imperative that health systems have infrastructure and capacity in place to administer these therapies to patients.
    - Stakeholders need to coordinate and prepare for the entry of novel hematologic therapies in parallel with regulatory and reimbursement evaluations of these therapies, rather than sequentially.
5. **Ensure patient representation in key decision-making process**
    - **HTA Process:** Involving patients and patient organizations in the HTA process is crucial, as access to hematological cancer therapies impacts not only patient outcomes but also their overall wellbeing and ability to contribute to society.
    - **Clinical Trial Design:** Including patients' perspectives in clinical trials offers key insights into symptom burden, treatment side effects, tolerability, and the impact of interventions on health and daily living – when coupled together with surrogate endpoints (e.g., PFS, MRD) which go beyond overall survival, are essential for evaluating the value of therapies.

About the Institute

The IQVIA Institute for Human Data Science contributes to the advancement of human health globally through timely research, insightful analysis and scientific expertise applied to granular non-identified patient-level data.

Fulfilling an essential need within healthcare, the Institute delivers objective, relevant insights and research that accelerate understanding and innovation critical to sound decision making and improved human outcomes. With access to IQVIA's institutional knowledge, advanced analytics, technology and unparalleled data the Institute works in tandem with a broad set of healthcare stakeholders to drive a research agenda focused on Human Data Science including government agencies, academic institutions, the life sciences industry, and payers.

Research Agenda

The research agenda for the Institute centers on 5 areas considered vital to contributing to the advancement of human health globally:

- Improving decision-making across health systems through the effective use of advanced analytics and methodologies applied to timely, relevant data.
- Addressing opportunities to improve clinical development productivity focused on innovative treatments that advance healthcare globally.
- Optimizing the performance of health systems by focusing on patient centricity, precision medicine and better understanding disease causes, treatment consequences and measures to improve quality and cost of healthcare delivered to patients.
- Understanding the future role for biopharmaceuticals in human health, market dynamics, and implications for manufacturers, public and private payers, providers, patients, pharmacists and distributors.
- Researching the role of technology in health system products, processes and delivery systems and the business and policy systems that drive innovation.

Guiding Principles

The Institute operates from a set of guiding principles:

- Healthcare solutions of the future require fact based scientific evidence, expert analysis of information, technology, ingenuity and a focus on individuals.
- Rigorous analysis must be applied to vast amounts of timely, high quality and relevant data to provide value and move healthcare forward.
- Collaboration across all stakeholders in the public and private sectors is critical to advancing healthcare solutions.
- Insights gained from information and analysis should be made widely available to healthcare stakeholders.
- Protecting individual privacy is essential, so research will be based on the use of non-identified patient information and provider information will be aggregated.
- Information will be used responsibly to advance research, inform discourse, achieve better healthcare and improve the health of all people.

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The IQVIA Institute for Human Data Science is committed to using human data science to provide timely, fact-based perspectives on the dynamics of health systems and human health around the world. The cover artwork is a visual representation of this mission. Using algorithms and data from the report itself, the final image presents a new perspective on the complexity, beauty and mathematics of human data science and the insights within the pages.

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