ASH 2022
CML Advocates Network conference report

Denis Costello, ED CMLAN

Jan Geissler, Chair

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64th ASH Annual Meeting & Exposition

The 64th ASH Annual Meeting and Exposition took place in December 10-13, 2022 at the Ernest N. Morial Convention Center in New Orleans, Louisiana.

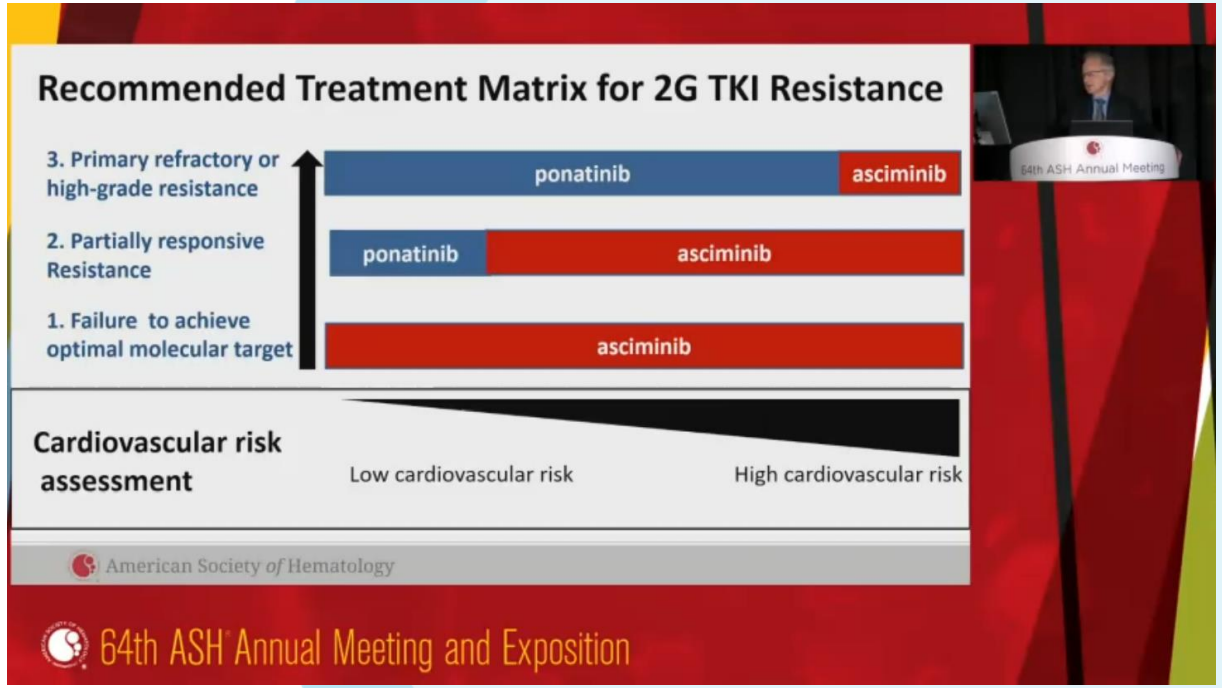
Representatives from the CML AN were there participating in several activities and sessions. Our chair Jan Geissler was invited to give a presentation on **the importance of patient involvement in data-driven blood cancer research** within the HARMONY Alliance special session. Zack Pemberton-Whiteley, a member of our Steering Committee presented a poster with results of a multi-country survey on **experiences and views of leukemia patients**. Other patient advocates like Gerald Clements, Lisa Machado, Pat García-González, Denis Costello, Samantha Nier, and Eglys González, were also there.

Here we present this conference report that summarizes the key CML highlights of interest to the patient advocacy community.

Education Session:
Beyond Routine Frontline Therapy of CML

Treatment of TKI resistant chronic phase CML. Timothy Hughes, MD

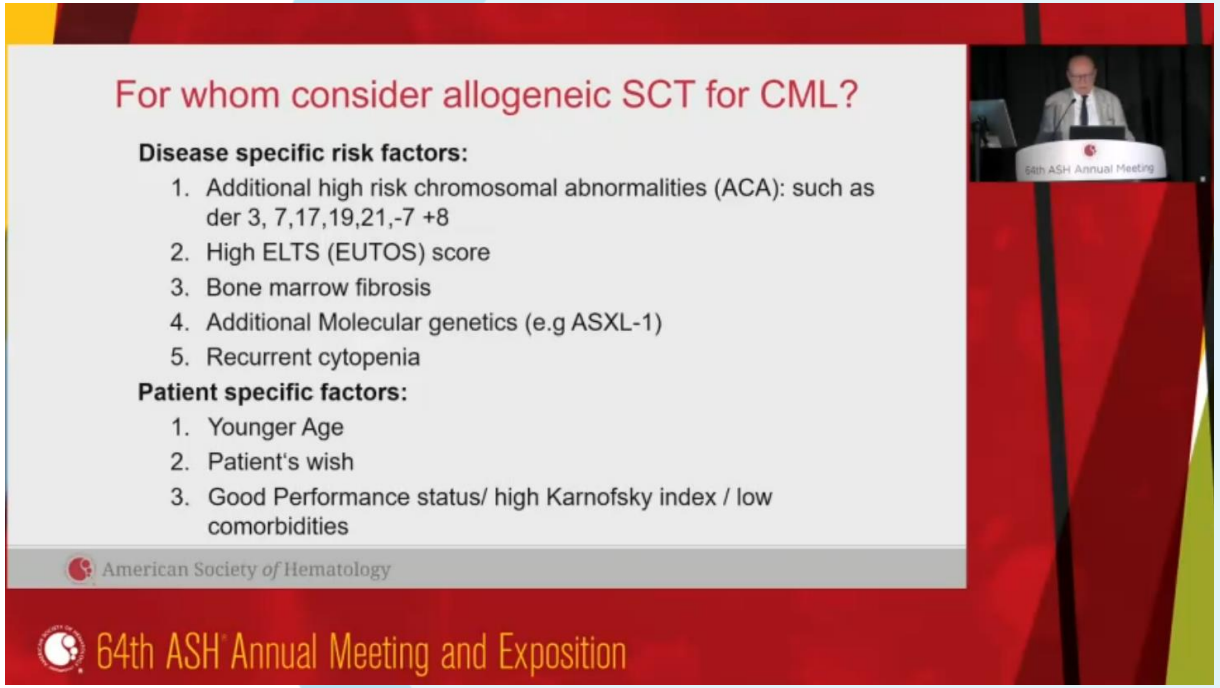
- Discussion of assessments and selecting the optimal TKI and TKI doses in CP-CML patients with TKI resistance. Tolerance, mutation profile, co-morbidities, and cardiovascular risk factors should be considered in the context of their prior response.
- Description and benefits of Asciminib were mentioned, concluding it has good efficacy and safety as monotherapy particularly when the disease is still responsive.
- Case reports and ongoing clinical studies were addressed. Indicating the complexity of an optimal third-line selection. Concluding that assessment to information regarding resistance status, mutations and CVS risk are needed.



Education Session:
Beyond Routine Frontline Therapy of CML

Transplantation in CML in the TKI Era: Who, When, How? Nicolaus Kroeger, MD

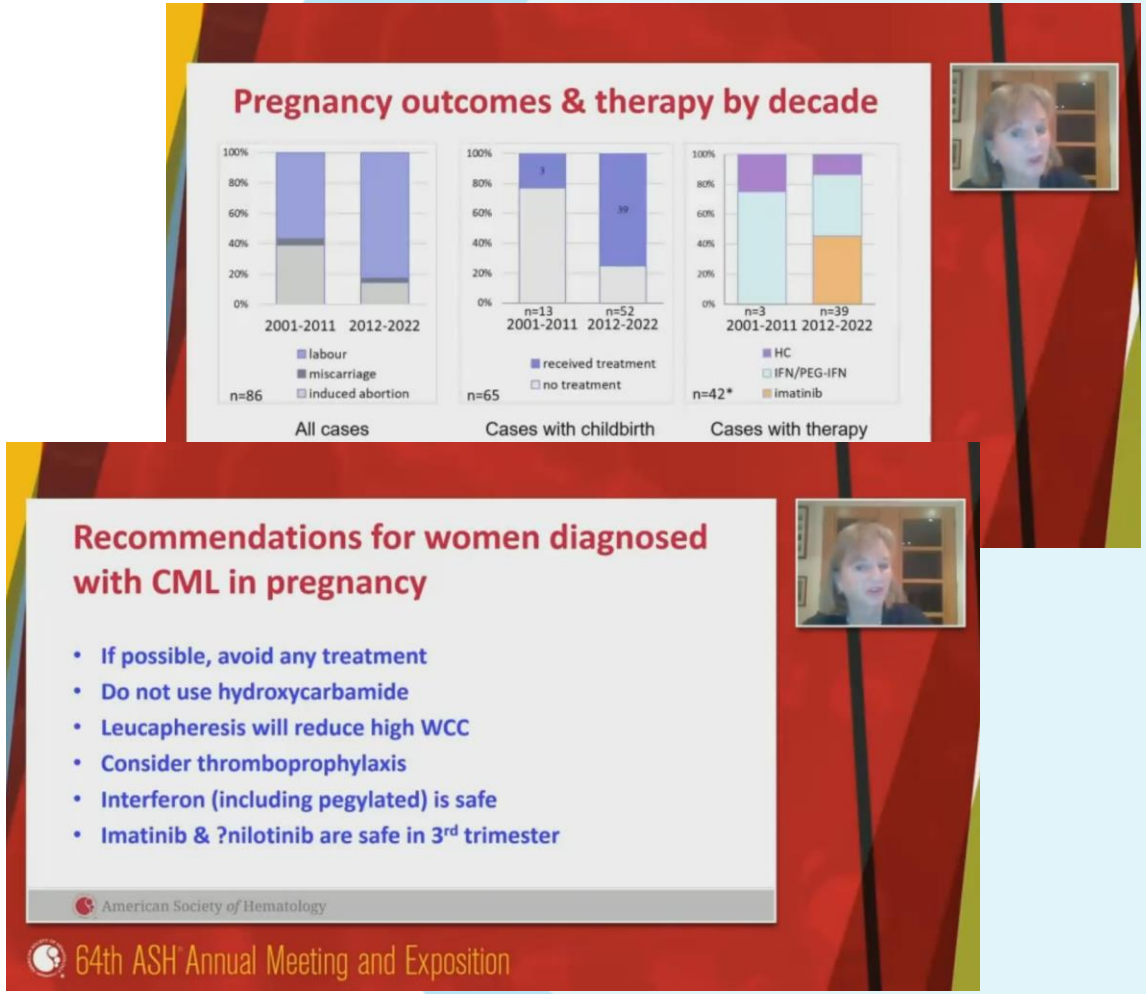
- Discussion on integrating allogeneic SCT as a curative treatment approach for chronic and advanced phases of CML. Use of allogeneic SCT in first and second chronic phase (CP1 & CP2), and in accelerated phase (AP). Conclusion is that it is still a curative treatment option for patients resistant or intolerant to TKIs, in both AP and BC. Better outcome in first chronic phase.
- Risk factors like cytogenetic, molecular genetics, BCR/ABL mutations, and BM fibrosis should be considered.
- Ongoing improvements in 3rd generation TKIs or STAMP inhibitors may modify further indications of allogeneic SCT.



Education Session:
Beyond Routine Frontline Therapy of CML

Treatment of CML in Pregnancy. Jane Apperley, MB.

- Discussion on safety and management of planned or unplanned pregnancies during CML.
- Identification of the impact of TKI in fertility in men, and on pregnancy of partners of male CML patients.
- Revision of animal model studies on outcomes of pregnancy on TKI.
- Considerations when diagnosing CML during pregnancy, and pregnancy in stablished CML.
- With advances in clinical research and data revision, lately are physicians more likely to let patient got to term and less like to recommend termination.



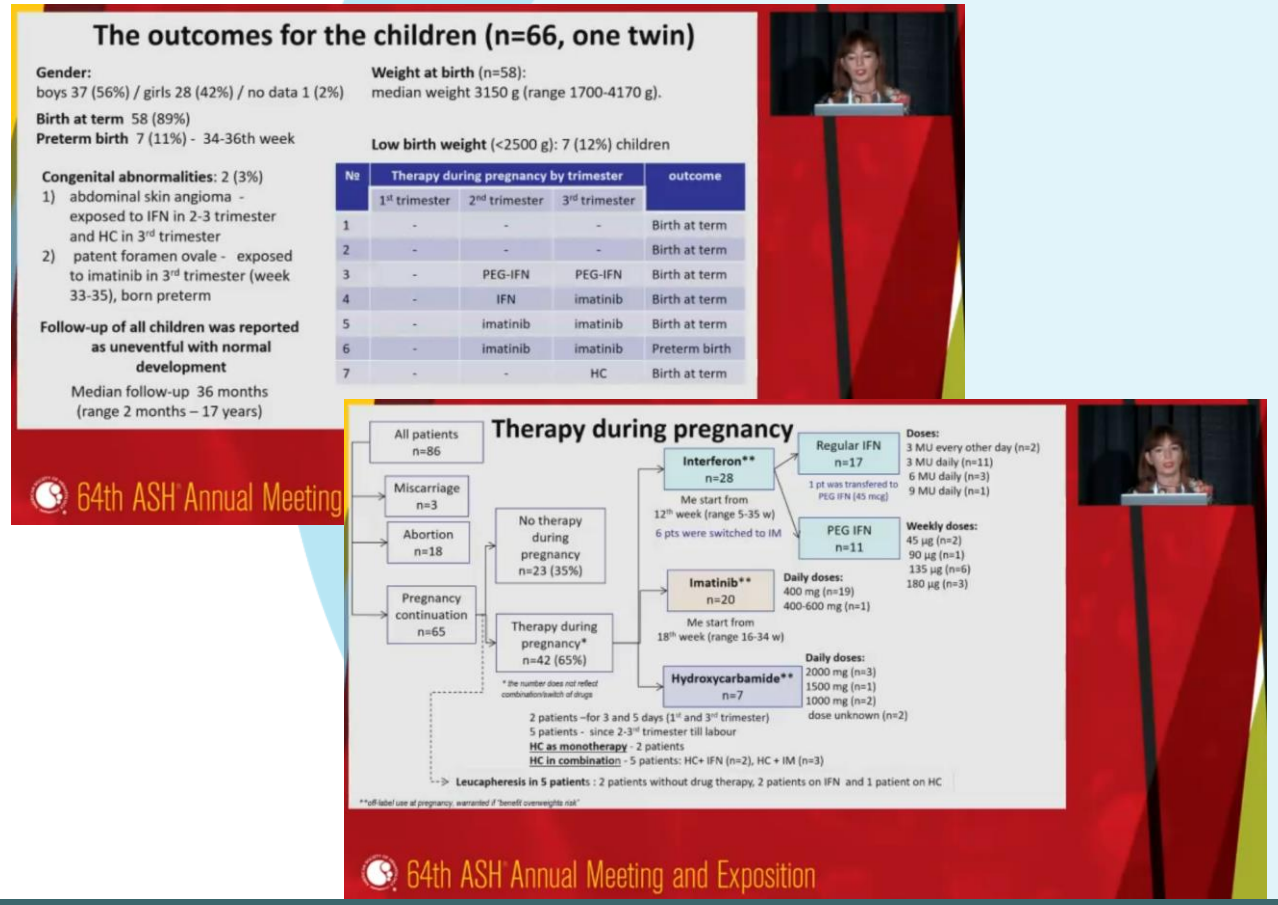
Oral Abstract Sessions

621. Chronic Myeloid Leukemia Diagnosed during Pregnancy: How to Manage? Description of 86 Cases from ELN International Registry

Elisabetta Abruzzese, MD

Retrospective and prospective data obtained from 11 countries through the ELN CML pregnancy registry since 2001. Analysis of clinical and demographic data, therapy, pregnancy outcomes and follow-up were performed.

- There is a real possibility of normal childbirth in women diagnosed with chronic phase CML.
- Adjusting the treatment by pregnancy trimester, the risk for both the safety of the mother and infant can be balanced.
- IFN / PEG-IFN can be used from the first trimester.
- The use of HC has no advantages over Imatinib.



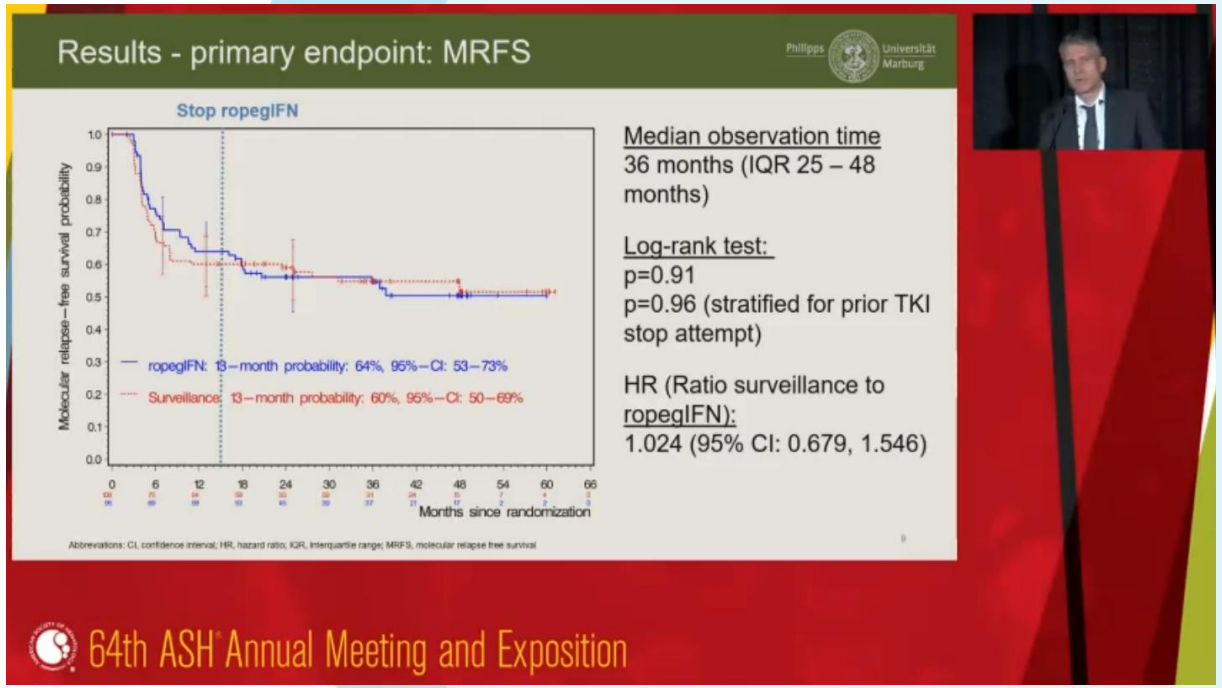
Oral Abstract Sessions

622. Efficacy of Ropeginterferon Alpha 2b in Inducing Treatment Free Remission in Chronic Myeloid Leukemia – an International, Randomized Phase III Trial (ENDURE, CML-IX) of the German CML-Study Group

Andreas Burchert, MD

Results from ENDURE, CML-IX (NCT03117816), a multicenter, international phase III trial evaluating the role of a novel form of pegylated proline interferon-alpha 2b (ropeg-interferon-alpha, ropeg-IFN) in inducing TFR. Period between May 2017 and June 2021.

- RopegIFN doeos not improve the chance of achieving TFR in unselected TKI pre-treated patients.
- TKI exposure of 6 years or more and no prior stop attempt favors TFR induction by ropegIFN.
- RopegIFN is very well tolerated.



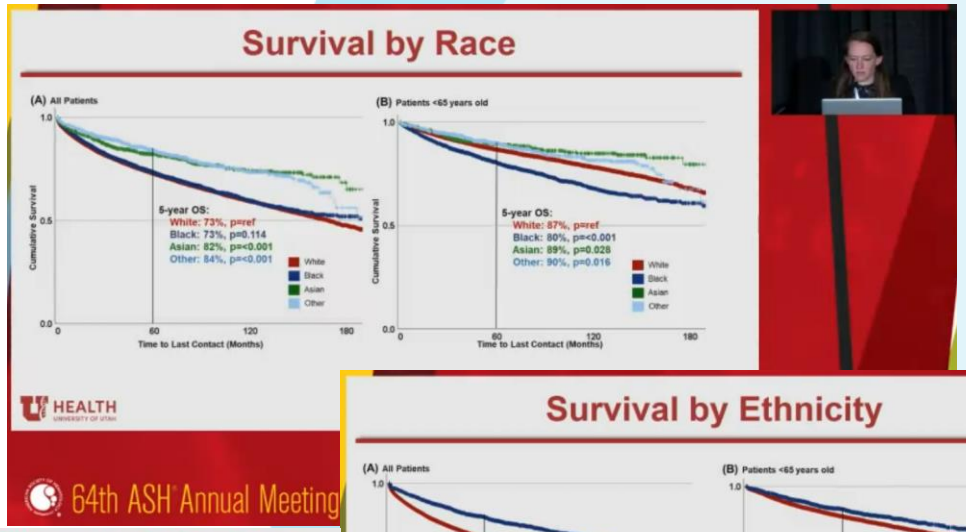
Oral Abstract Sessions

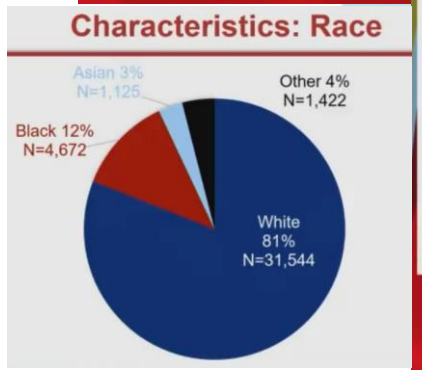
624. Racial and Ethnic Disparities in Survival Outcomes in Chronic Myeloid Leukemia

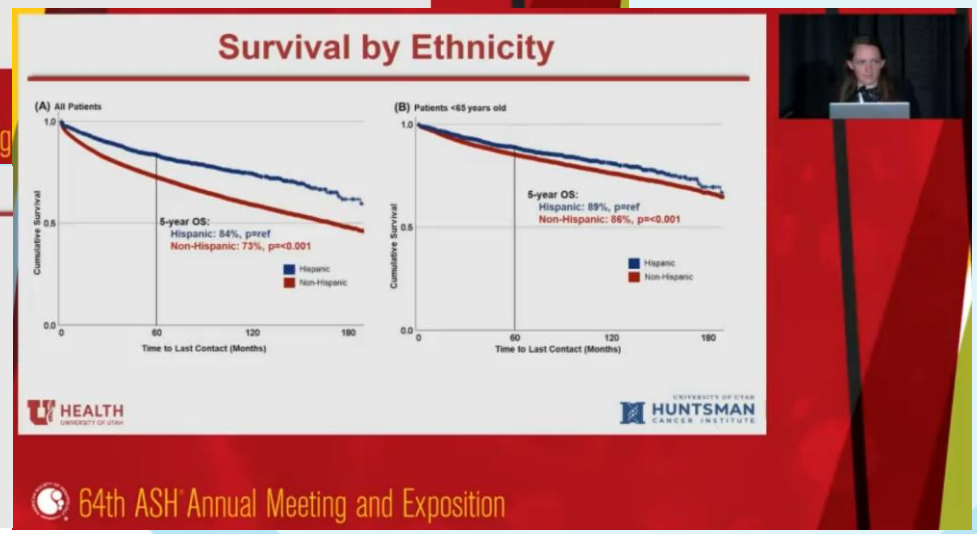
Catherine Sobieski, MD

The National Cancer Database was used to identify CML patients (pts) diagnosed from 2004-2019. Demographic and treatment characteristics were compared for White, Black, Asian, Hispanic and other minority populations. The OS was compared by race and ethnicity.

- Factors associated with improved survival include Asian and other races, Hispanic Ethnicity and Female gender.
- Limitations of the study due to underrepresentation of rural critical access hospitals, and lack of data on specific TKI therapy, adherence and side effects.
- Young black patients have worse OS than white patients
- Access to treatment remains a significant barrier.







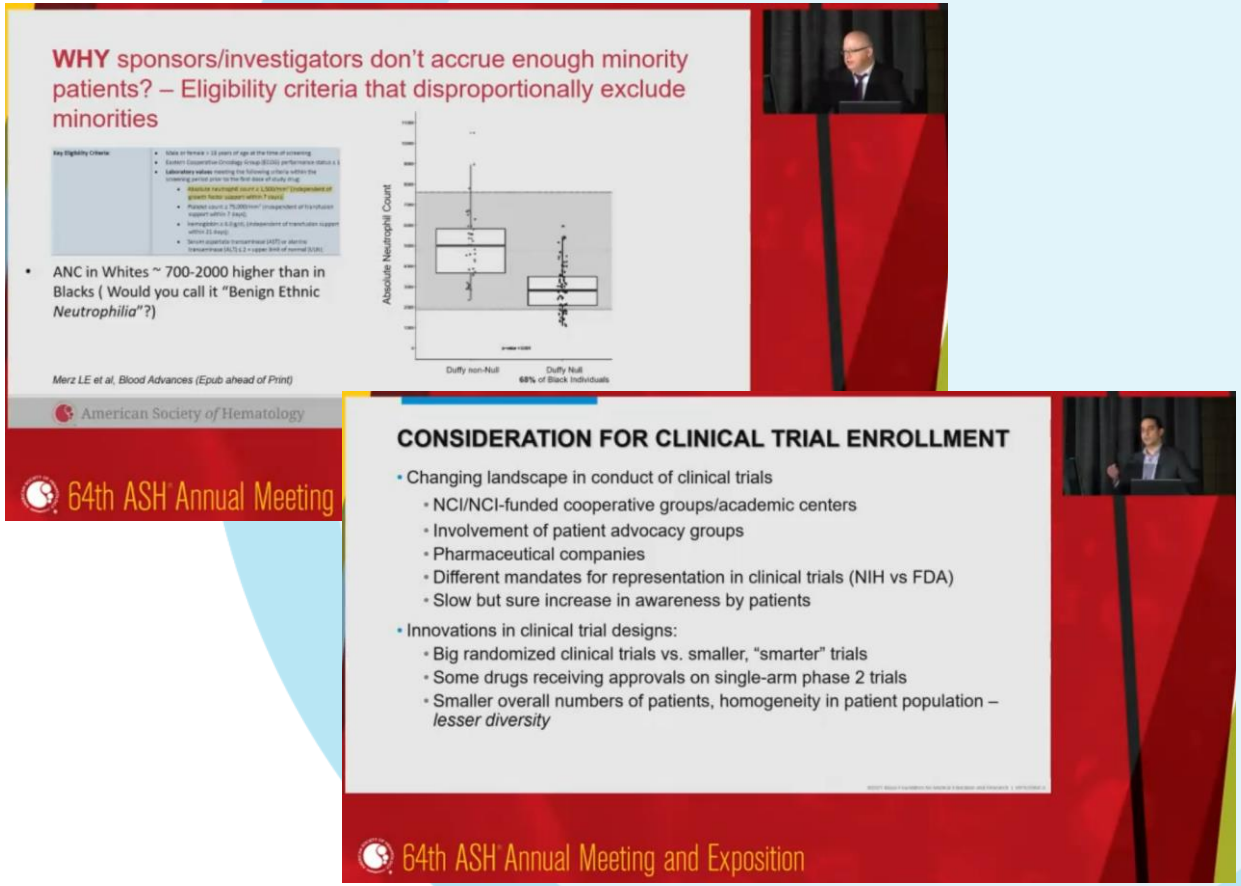
Spotlight Sessions

Underrepresented minorities in clinical trials for hematologic malignancies: what's the data on the data?

Luciano Megala Costa, MD, PhD. University of Alabama at Birmingham Hospital / Sikander Ailawadhi. Mayo Clinic / Rayne Rouce, MD. Baylor College of Medicine, Texas Children's Hospital

Multiple myeloma represents an excellent scenario to study disparities in clinical trial participation and outcomes. It is a condition 2-3 times more incident in Black than in White individuals. Although individuals of racial-ethnic minorities are under-represented in clinical trials, when enrolled their outcome is the same or even better than in other patient groups.

- Proper clinical trial representation is required.
- No matter how well resourced a CT might be, there are costs to receiving care associated with participation, and they become a barrier.
- The eligibility criteria disproportionally can exclude the minority patients to participate in CTs.
- Lack of diversity in the workforce. Approaches to increase the proportion of underrepresented investigators in clinical research are required.

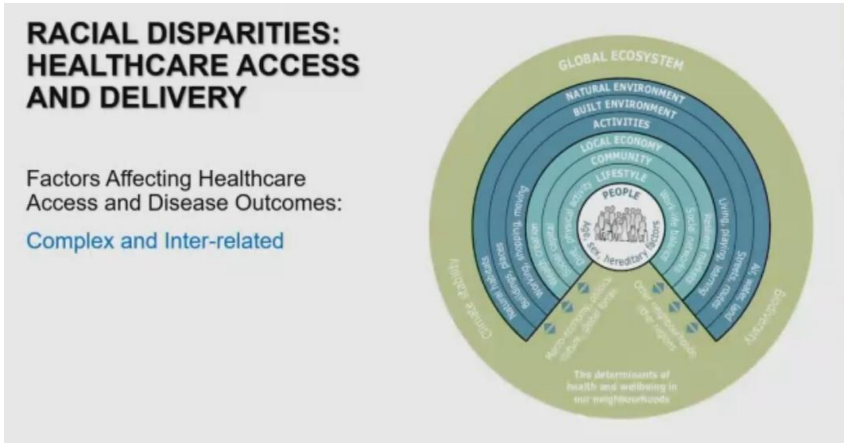


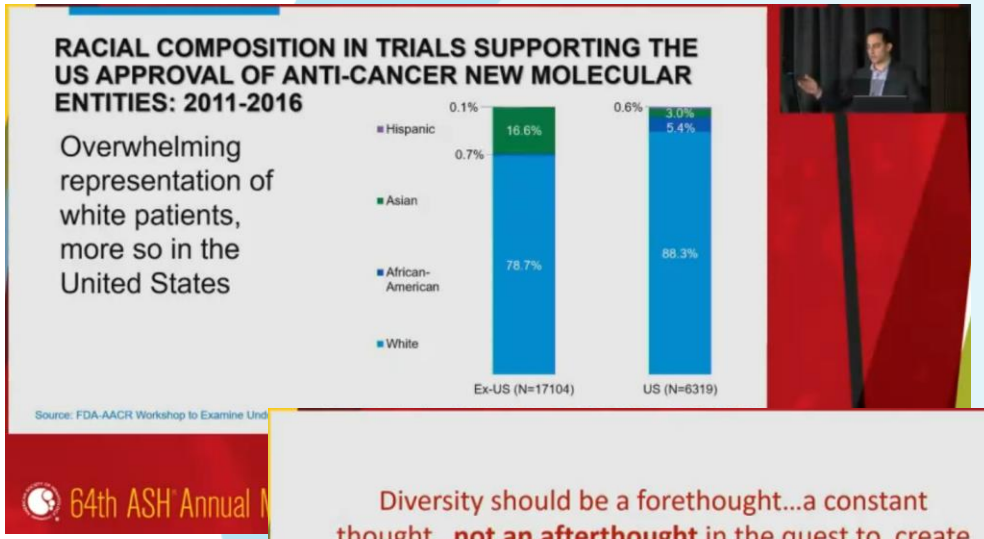
Spotlight Sessions

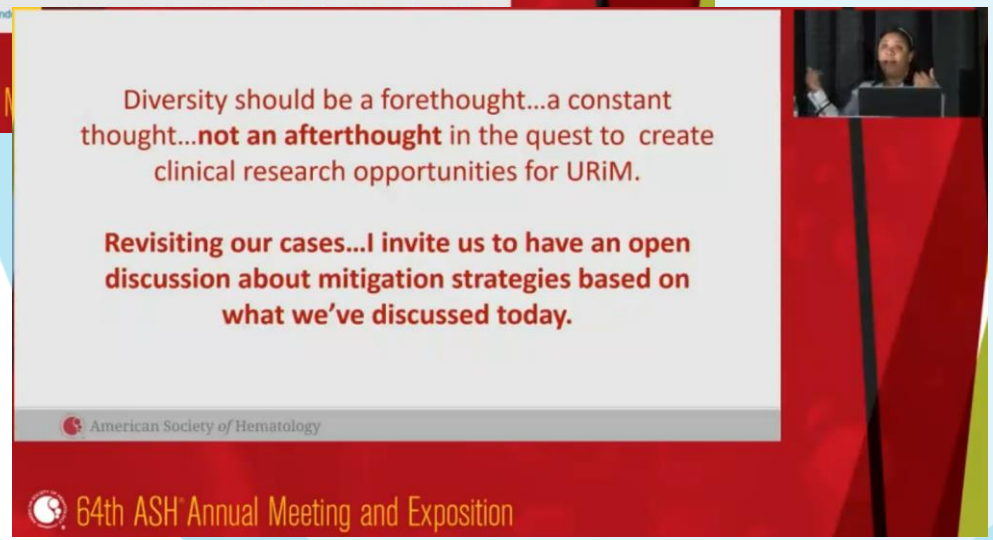
Underrepresented minorities in clinical trials for hematologic malignancies: what's the data on the data?

Potential strategies:

- Set concrete targets for trial enrolment
- Registration trials should specify how minority accrual will be optimized
- Having a trial diversity officer
- Provide culturally sensitive training to sites and staff
- Post-marketing specific data in minorities.







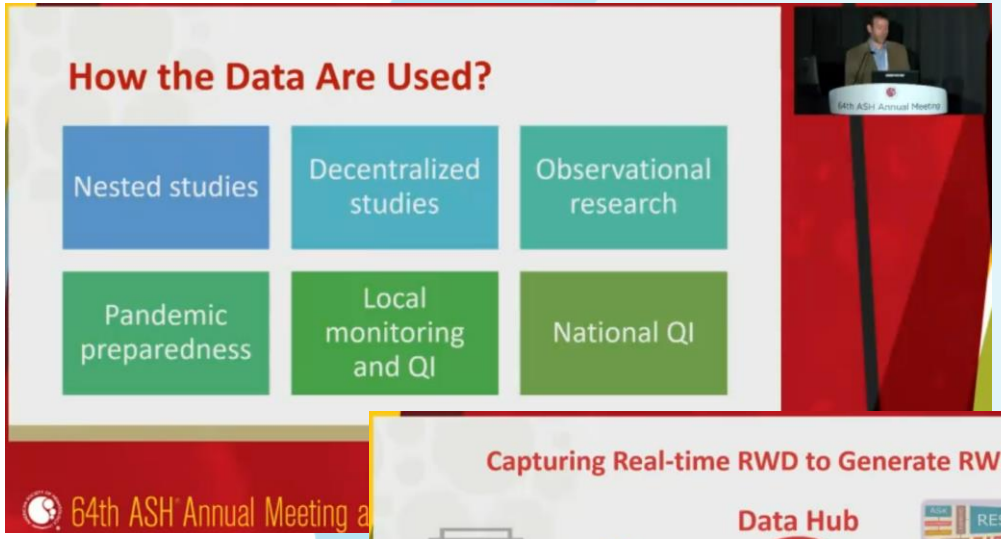
Special-Interest Sessions

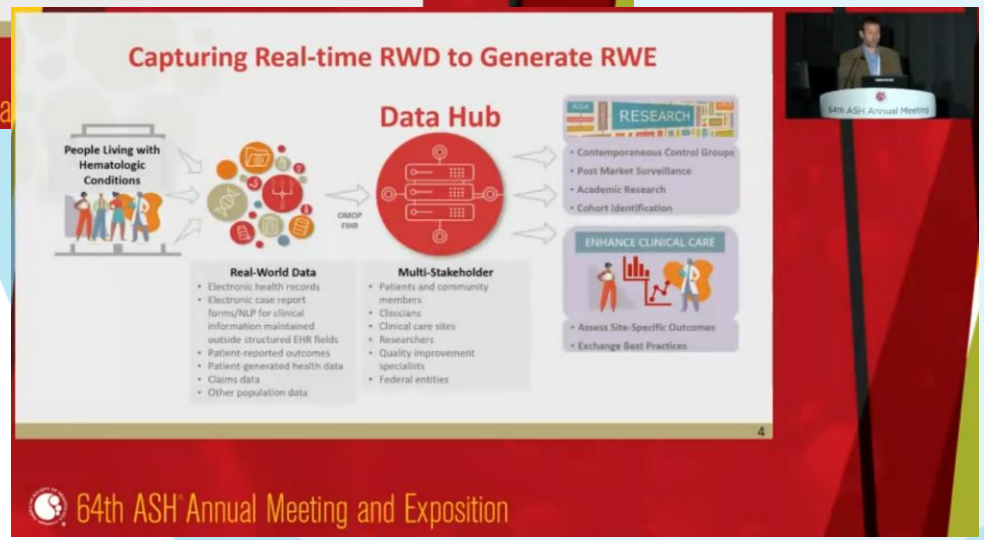
- **Driving Real-World Evidence for Hematologic Malignancy Research – A Joint ASH RC and EU HARMONY Session**

Impact of Real-World Evidence on Research and Clinical Care: Priorities from ASH RC Data Hub

Adam Sperling, MD, PhD

- The ASH Research Collaborative aims to accelerate progress in hematology, foster collaborative partnerships and improve lives of people affected by blood diseases.
- Focused on capturing RWD in order to enhance care and accelerate research in hematology.





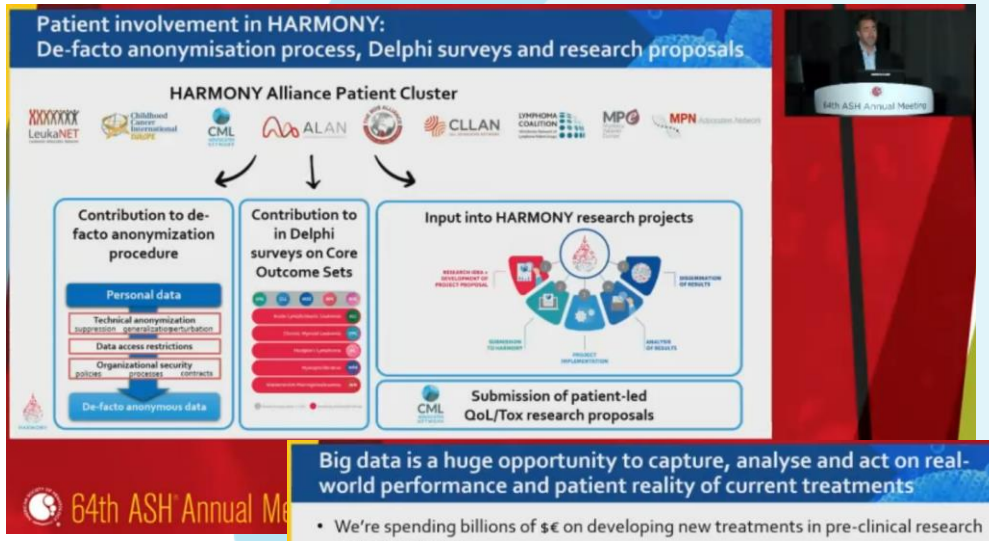
Special-Interest Sessions

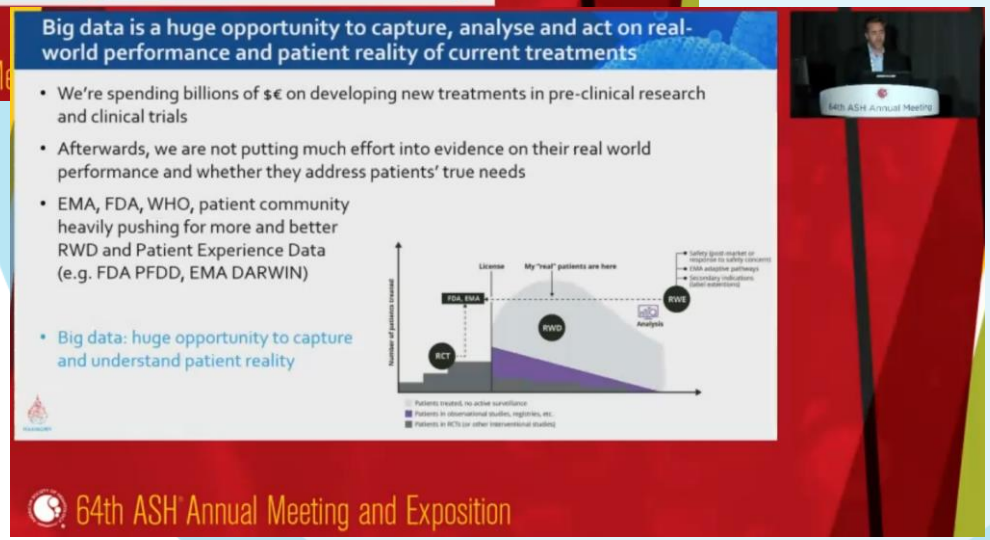
- **Driving Real-World Evidence for Hematologic Malignancy Research – A Joint ASH RC and EU HARMONY Session**

HARMONY Alliance: Moving Toward Multicenter Real-World Data Capture in the Hematologic Malignancies in Europe - A Patient's Perspective.

Jan Geissler.

- After pre-clinical and clinical studies more effort should be made in collecting evidence of drug performance in the real world.
- Unwillingness to share data is the biggest risk to big data, as it is not in the patients' best interest, leads to delays in collective learning and duplication of research.
- The HARMONY Patient Cluster of 8 global patient umbrella organisations in hematology has contributed to central parts of the HARMONY project: data anonymisation, research proposals and core outcome sets.





U.S. FOOD & DRUG ADMINISTRATION

FDA Patient Advocacy Session

During the meeting the FDA representatives gave an update on the relevant ongoing projects:

- Fostering Regulatory Science and Collaboration to Translate Real World Data into Real World Evidence.
- TEAM FoRWD - (**T**ranslational **E**valuation and **A**ssessment of **M**ethods to **F**acilitate use of **O**ncology **RWD**)
- Optimus - Get the dose right the first time
- FrontRunner - Advancing Development of New Oncology Therapies to the Early Clinical Setting
- Project Facilitate - Assisting Healthcare Providers with Expanded Access Requests for Investigational Oncology Products

For most of the meeting the importance of inclusion of PROs along the whole process of drug evaluations and development was addressed and discussed. As well as ways on how to get patients better involved in the drug development processes.

External sessions – ELN Breakfast

During the ELN Breakfast meeting held at ASH 2022 in New Orleans, reports from each WPs were presented, and an update session of the HARMONY Alliance was conducted. Within it, our Chair Jan Geissler presented a description of the research proposals submitted by the patient community to HARMONY.

- Prevalence of clinically relevant problems and symptoms in patients with CML
- A comparison study of "toxicity over time" and QoL among all available TKI options
- Correlation of demographic, disease and therapy factors with toxicity and tolerability of the different TKIs.

BeiGene Patient Advocacy Event

“Cancer & Mental Health: The Science & Art of Whole Patient Care.”

Discussions on mental health during cancer diagnosis and therapy as part of quality cancer care. Presented from the view of phycologists and social workers. Aiming for a more personalized approach and support to the patients and their carers.

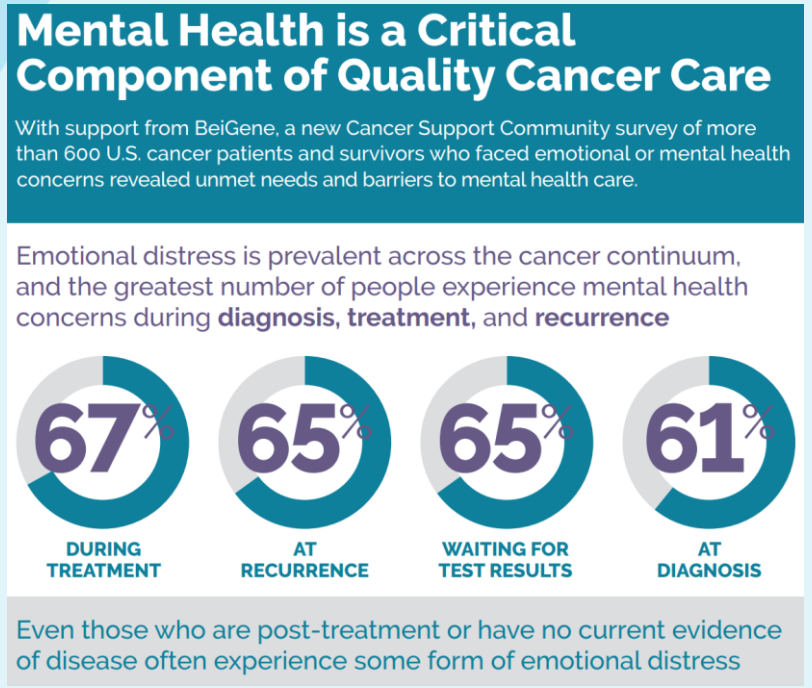
Allison Applebaum, PhD (Memorial Sloan Kettering Cancer Center)

Heather Honoré Goltz, PhD (University of Houston-Downtown)

Joseph A. Greer, PhD (Massachusetts General Hospital Cancer Center)

BeiGene has a program called **Talk About it: Cancer and Mental Health**.

- Mental health support can improve quality of life and health outcomes for individuals impacted by cancer.
- Aimed at patients and caregivers, healthcare professionals and policymakers.
- Designed to elevate the important intersection of mental health and cancer care.
- Talk About It will feature innovative empowerment strategies, advance public policy conversations, and inspire dynamic health equity initiatives to support people throughout their entire cancer journey.



iCMLf Forum: The next big challenges in CML?

- Free Treatment or Treatment Free? Where to invest limited resources in a highly manageable cancer

TKIs have not significantly affected the impact of a CML diagnosis on life expectancy, with outcomes remaining inferior in economically disadvantaged parts of the world. We need to ask how limited resources should be deployed for the greatest benefit.

- Disease Persistence in CML: Cell Intrinsic and Extrinsic Mechanisms

A lifetime of TKI therapy is associated with significant clinical and financial burden. While some individuals can discontinue therapy and remain disease free for years, low levels of disease persist in the majority of patients. Recent methodologic developments that enable to study of rare cell populations are providing an opportunity to understand this mechanism of this persistence, and identifying new targets.

- TKI Resistance: More Than Just Abl Mutations

Much attention has been paid to the development of kinase domain mutations as THE mechanism of resistance. However not all patients who develop resistance have detectable mutations. New data is emerging and old data re-emerging to understand resistance more broadly, and learn how to address it.

Panellist and patient advocate **Lisa Machado** expressed the patient community expectations on the ongoing research performed in CML and its therapies.

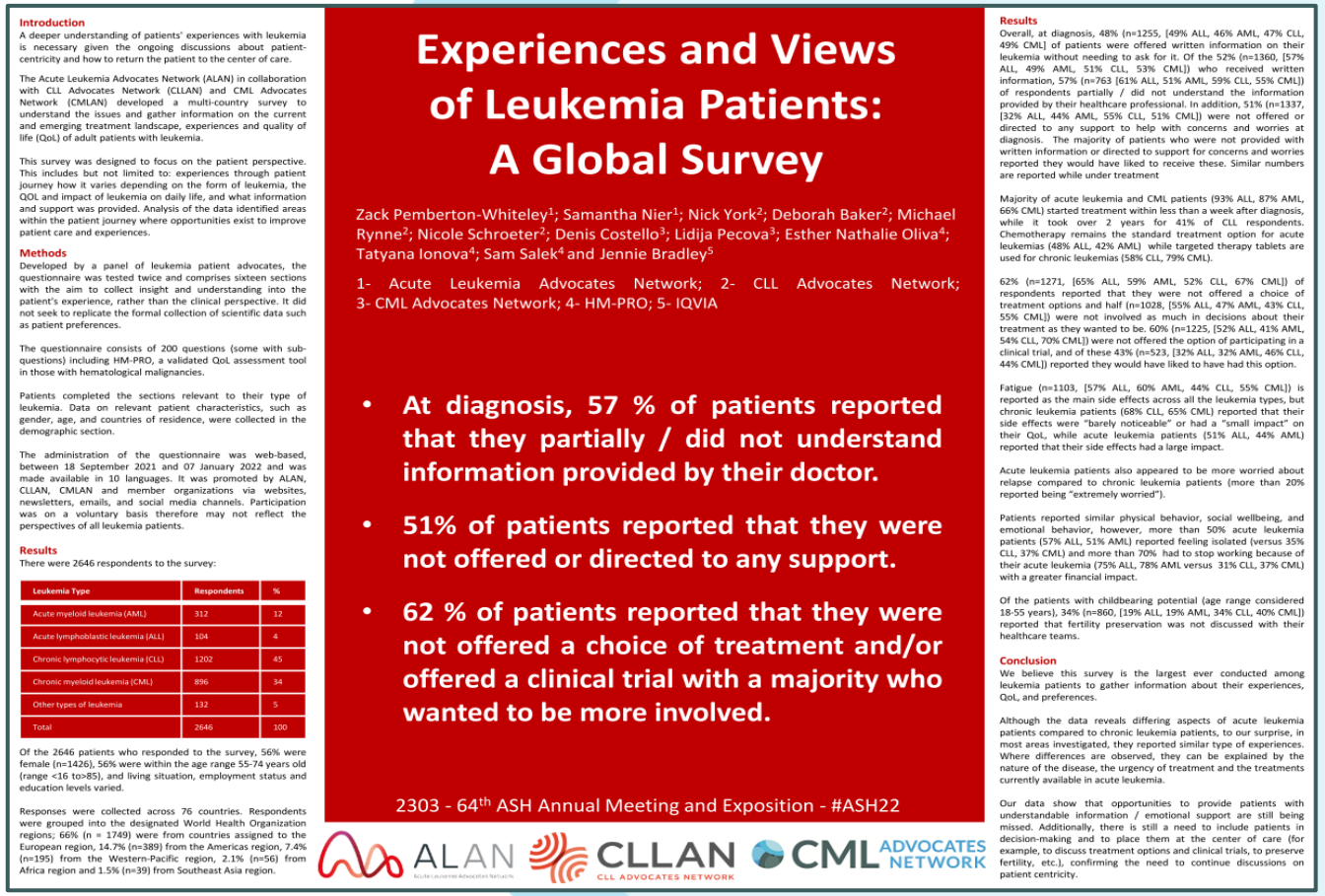
As well, the need of bringing clinical trials to underprivileged sites was brought up.

Poster Presentations

2303. Experiences and Views of Leukemia Patients: A Global Survey

Zack Pemberton-Whiteley, ALAN

- Acute leukemia patients appeared to be more worried about relapse than those with chronic leukemia
- Patients reported similar social wellbeing, social and physical behavior among acute and chronic leukemia, showing only few differences that can be explained by the nature of the disease, the urgency of treatment and available treatments in acute leukemia.
- 34% of patents with childbearing potential reported that fertility preservation was not discussed with their healthcare team.
- The data obtained supports that patient inclusion in decision-making and placing them at the center of care are still important needs. Efforts to achieve patient centricity are to be continued.



Introduction
A deeper understanding of patients' experiences with leukemia is necessary given the ongoing discussions about patient-centricity and how to return the patient to the center of care.

The Acute Leukemia Advocates Network (ALAN) in collaboration with CLL Advocates Network (CLLAN) and CML Advocates Network (CMLAN) developed a multi-country survey to understand the issues and gather information on the current and emerging treatment landscape, experiences and quality of life (QoL) of adult patients with leukemia.

This survey was designed to focus on the patient perspective. This includes but not limited to: experiences through patient journey how it varies depending on the form of leukemia, the QOL and impact of leukemia on daily life, and what information and support was provided. Analysis of the data identified areas within the patient journey where opportunities exist to improve patient care and experiences.

Methods
Developed by a panel of leukemia patient advocates, the questionnaire was tested twice and comprises sixteen sections with the aim to collect insight and understanding into the patient's experience, rather than the clinical perspective. It did not seek to replicate the formal collection of scientific data such as patient preferences.

The questionnaire consists of 200 questions (some with sub-questions) including HM-PRO, a validated QoL assessment tool in those with hematological malignancies.

Patients completed the sections relevant to their type of leukemia. Data on relevant patient characteristics, such as gender, age, and countries of residence, were collected in the demographic section.

The administration of the questionnaire was web-based, between 18 September 2021 and 07 January 2022 and was made available in 10 languages. It was promoted by ALAN, CLLAN, CMLAN and member organizations via websites, newsletters, emails, and social media channels. Participation was on a voluntary basis therefore may not reflect the perspectives of all leukemia patients.

Results
There were 2646 respondents to the survey:

Leukemia Type	Respondents	%
Acute myeloid leukemia (AML)	312	12
Acute lymphoblastic leukemia (ALL)	104	4
Chronic lymphocytic leukemia (CLL)	1202	45
Chronic myeloid leukemia (CML)	896	34
Other types of leukemia	132	5
Total	2646	100

Of the 2646 patients who responded to the survey, 56% were female (n=1426), 56% were within the age range 55-74 years old (range <16 to>85), and living situation, employment status and education levels varied.

Responses were collected across 76 countries. Respondents were grouped into the designated World Health Organization regions; 66% (n = 1749) were from countries assigned to the European region, 14.7% (n=389) from the Americas region, 7.4% (n=195) from the Western-Pacific region, 2.1% (n=56) from Africa region and 1.5% (n=39) from Southeast Asia region.

Experiences and Views of Leukemia Patients: A Global Survey

Zack Pemberton-Whiteley[1]; Samantha Nier[1]; Nick York[2]; Deborah Baker[2]; Michael Rynne[2]; Nicole Schroeter[2]; Denis Costello[3]; Lidija Pecova[3]; Esther Nathalie Oliva[4]; Tatyana Ionova[4]; Sam Salek[4] and Jennie Bradley[5]

1- Acute Leukemia Advocates Network; 2- CLL Advocates Network; 3- CML Advocates Network; 4- HM-PRO; 5- IQVIA

- **At diagnosis, 57 % of patients reported that they partially / did not understand information provided by their doctor.**
- **51% of patients reported that they were not offered or directed to any support.**
- **62 % of patients reported that they were not offered a choice of treatment and/or offered a clinical trial with a majority who wanted to be more involved.**

2303 - 64th ASH Annual Meeting and Exposition - #ASH22

ALAN CLLAN CML ADVOCATES NETWORK

Results
Overall, at diagnosis, 48% (n=1255, [49% ALL, 46% AML, 47% CLL, 49% CML] of patients were offered written information on their leukemia without needing to ask for it. Of the 52% (n=1360, [57% ALL, 49% AML, 51% CLL, 53% CML]) who received written information, 57% (n=763 [61% ALL, 51% AML, 59% CLL, 55% CML]) of respondents partially / did not understand the information provided by their healthcare professional. In addition, 51% (n=1337, [32% ALL, 44% AML, 55% CLL, 51% CML]) were not offered or directed to any support to help with concerns and worries at diagnosis. The majority of patients who were not provided with written information or directed to support for concerns and worries reported they would have liked to receive these. Similar numbers are reported while under treatment

Majority of acute leukemia and CML patients (93% ALL, 87% AML, 66% CML) started treatment within less than a week after diagnosis, while it took over 2 years for 41% of CLL respondents. Chemotherapy remains the standard treatment option for acute leukemias (48% ALL, 42% AML) while targeted therapy tablets are used for chronic leukemias (58% CLL, 79% CML).

62% (n=1271, [65% ALL, 59% AML, 52% CLL, 67% CML]) of respondents reported that they were not offered a choice of treatment options and half (n=1028, [55% ALL, 47% AML, 43% CLL, 55% CML]) were not involved as much in decisions about their treatment as they wanted to be. 60% (n=1225, [52% ALL, 41% AML, 54% CLL, 70% CML]) were not offered the option of participating in a clinical trial, and of these 43% (n=523, [32% ALL, 32% AML, 46% CLL, 44% CML]) reported they would have liked to have had this option.

Fatigue (n=1103, [57% ALL, 60% AML, 44% CLL, 55% CML]) is reported as the main side effects across all the leukemia types, but chronic leukemia patients (68% CLL, 65% CML) reported that their side effects were "barely noticeable" or had a "small impact" on their QoL, while acute leukemia patients (51% ALL, 44% AML) reported that their side effects had a large impact.

Acute leukemia patients also appeared to be more worried about relapse compared to chronic leukemia patients (more than 20% reported being "extremely worried").

Patients reported similar physical behavior, social wellbeing, and emotional behavior, however, more than 50% acute leukemia patients (57% ALL, 51% AML) reported feeling isolated (versus 35% CLL, 37% CML) and more than 70% had to stop working because of their acute leukemia (75% ALL, 78% AML versus 31% CLL, 37% CML) with a greater financial impact.

Of the patients with childbearing potential (age range considered 18-55 years), 34% (n=860, [19% ALL, 19% AML, 34% CLL, 40% CML]) reported that fertility preservation was not discussed with their healthcare teams.

Conclusion
We believe this survey is the largest ever conducted among leukemia patients to gather information about their experiences, QoL, and preferences.

Although the data reveals differing aspects of acute leukemia patients compared to chronic leukemia patients, to our surprise, in most areas investigated, they reported similar type of experiences. Where differences are observed, they can be explained by the nature of the disease, the urgency of treatment and the treatments currently available in acute leukemia.

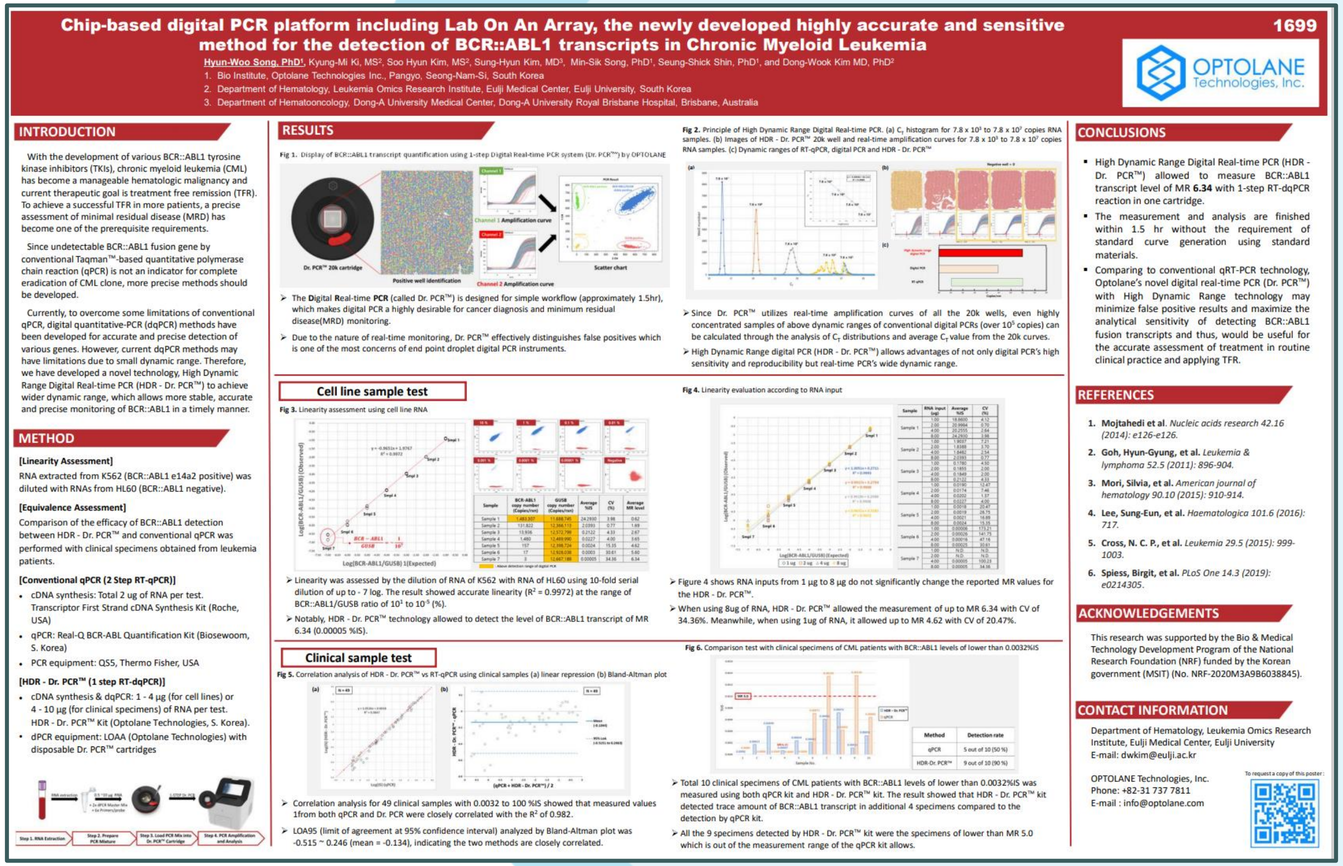
Our data show that opportunities to provide patients with understandable information / emotional support are still being missed. Additionally, there is still a need to include patients in decision-making and to place them at the center of care (for example, to discuss treatment options and clinical trials, to preserve fertility, etc.), confirming the need to continue discussions on patient centricity.

Poster Presentations

1699. Chip-Based Digital PCR Platform, Lab on an Array, the Newly Developed Highly Accurate and Sensitive Method for the Detection of BCR-ABL1 Transcripts in Chronic Myeloid Leukemia

Hyun-Woo Song, PhD. Optolane Technologies

- To overcome some limitations of conventional qPCR and that of the dqPCR methods, the establishment of clinical guideline is crucial for wide application on TFR in CML. This group developed a novel technology, **a chip-based real-time dqPCR platform (LOAA; Lab On An Array; so called Dr. PCR™)** to achieve more stable, accurate and precise monitoring of BCR-ABL1 in a timely manner.
- Results showed that Dr PCR efficiently detects clinical specimens with low copy number of BCR-ABL1 compared to conventional qPCR.

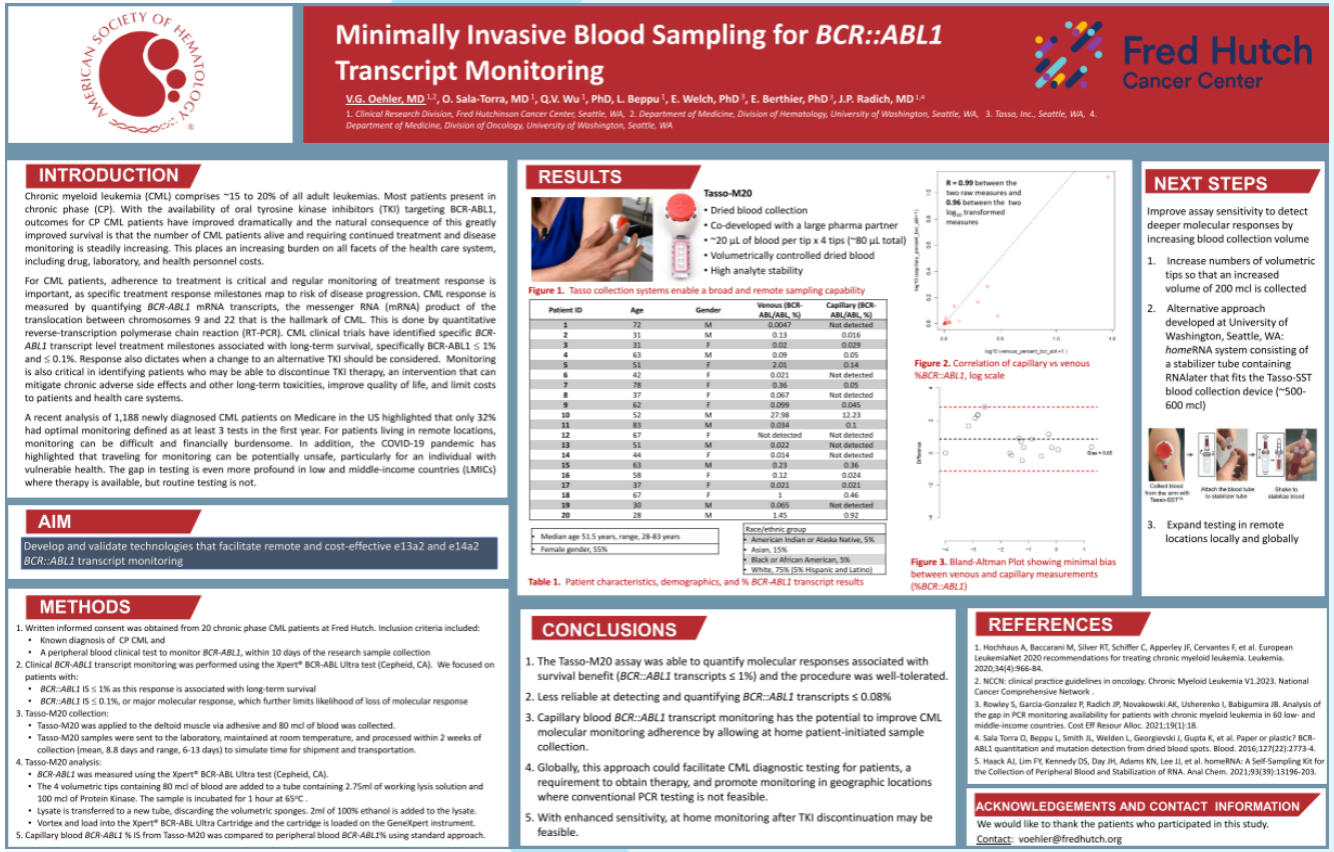


Poster Presentations

1690. Minimally Invasive Blood Sampling for BCR::ABL1 transcript Monitoring

Vivian Oehler, MD. Fred Hutchinson Cancer Center

- For patients living in remote locations, monitoring can be financially burdensome. To address this need, this group developed a system to measured e13a2 and e14a2 BCR::ABL1 transcripts in capillary blood collected using the Tasso-M20 device.
- The assay was able to quantify accurately molecular responses associated with survival benefit (BCR::ABL1 transcripts ≤ 1%) and major molecular response (BCR::ABL1 transcripts ≤ 0.1%), but was less reliable at quantifying BCR::ABL1 transcripts < 0.06%.
- This monitoring may improve CML molecular monitoring adherence by allowing at home patient-initiated sample collection.

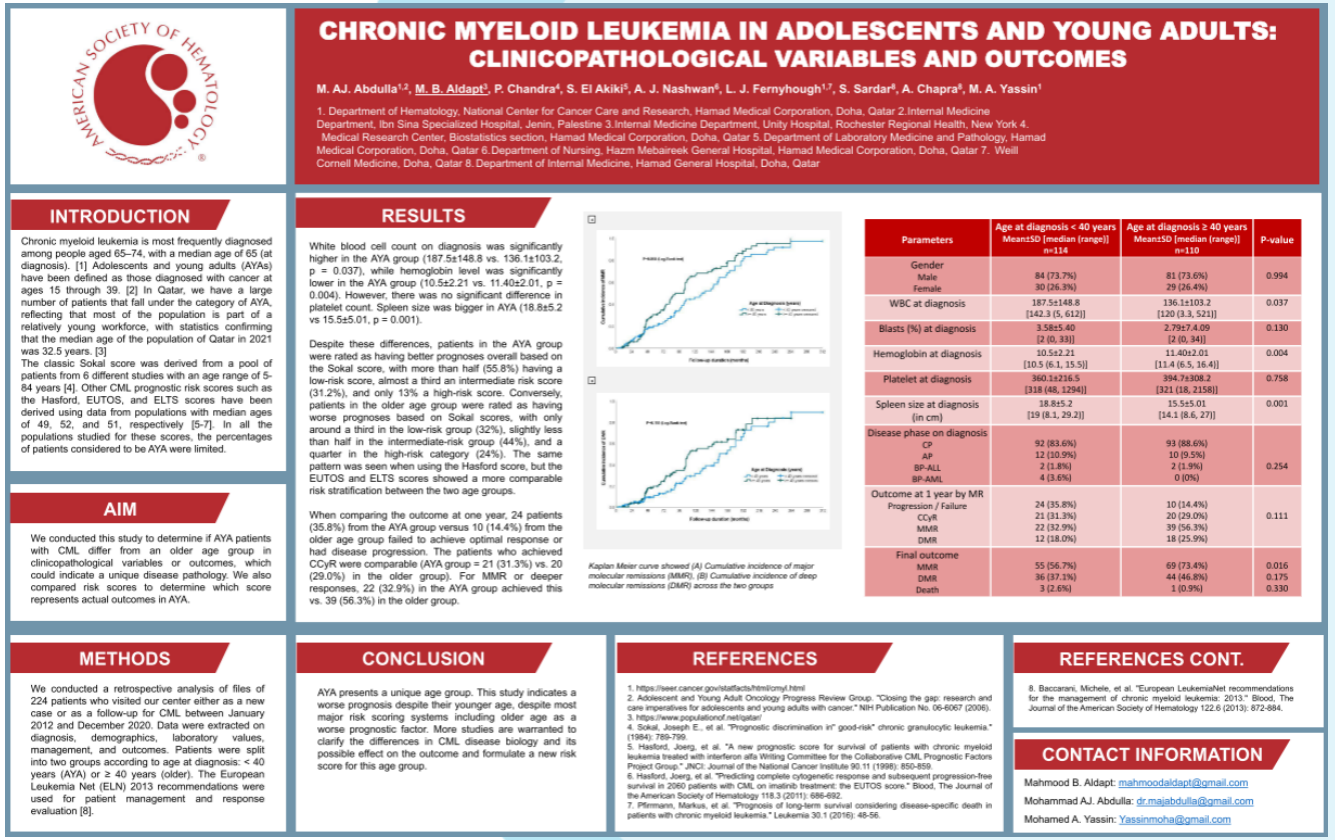


Poster Presentations

1702. Chronic Myeloid Leukemia in Adolescents and Young Adults: Clinicopathological Variables and Outcomes

Mohammad Abdulla, MD. Hamad Medical Corporation

- Adolescents and young adults (AYAs) are defined as those between the ages of 15 through 39.
- This study aims to find out if AYA age group differ from older age group in clinicopathological variables or outcome.
- Results indicated that this group have a worse prognosis.
- Unique challenges for this population include fertility issues, access to health care, and survivorship concerns.
- Treatment free remissions are more important and more relevant for this age group .



CHRONIC MYELOID LEUKEMIA IN ADOLESCENTS AND YOUNG ADULTS: CLINICOPATHOLOGICAL VARIABLES AND OUTCOMES

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INTRODUCTION

Chronic myeloid leukemia is most frequently diagnosed among people aged 65–74, with a median age of 65 (at diagnosis). [1] Adolescents and young adults (AYAs) have been defined as those diagnosed with cancer at ages 15 through 39. [2] In Qatar, we have a large number of patients that fall under the category of AYA, reflecting that most of the population is part of a relatively young workforce, with statistics confirming that the median age of the population of Qatar in 2021 was 32.5 years. [3]
The classic Sokal score was derived from a pool of patients from 6 different studies with an age range of 5-84 years [4]. Other CML prognostic risk scores such as the Hasford, EUTOS, and ELTS scores have been derived using data from populations with median ages of 49, 52, and 51, respectively [5-7]. In all the populations studied for these scores, the percentages of patients considered to be AYA were limited.

AIM

We conducted this study to determine if AYA patients with CML differ from an older age group in clinicopathological variables or outcomes, which could indicate a unique disease pathology. We also compared risk scores to determine which score represents actual outcomes in AYA.

METHODS

We conducted a retrospective analysis of files of 224 patients who visited our center either as a new case or as a follow-up for CML between January 2012 and December 2020. Data were extracted on diagnosis, demographics, laboratory values, management, and outcomes. Patients were split into two groups according to age at diagnosis: < 40 years (AYA) or ≥ 40 years (older). The European Leukemia Net (ELN) 2013 recommendations were used for patient management and response evaluation [8].

RESULTS

White blood cell count on diagnosis was significantly higher in the AYA group (187.5±148.8 vs. 136.1±103.2, p = 0.037), while hemoglobin level was significantly lower in the AYA group (10.5±2.21 vs. 11.40±2.01, p = 0.004). However, there was no significant difference in platelet count. Spleen size was bigger in AYA (18.8±5.2 vs 15.5±5.01, p = 0.001).

Despite these differences, patients in the AYA group were rated as having better prognoses overall based on the Sokal score, with more than half (55.8%) having a low-risk score, almost a third an intermediate risk score (31.2%), and only 13% a high-risk score. Conversely, patients in the older age group were rated as having worse prognoses based on Sokal scores, with only around a third in the low-risk group (32%), slightly less than half in the intermediate-risk group (44%), and a quarter in the high-risk category (24%). The same pattern was seen when using the Hasford score, but the EUTOS and ELTS scores showed a more comparable risk stratification between the two age groups.

When comparing the outcome at one year, 24 patients (35.8%) from the AYA group versus 10 (14.4%) from the older age group failed to achieve optimal response or had disease progression. The patients who achieved CCyR were comparable (AYA group = 21 (31.3%) vs. 20 (29.0%) in the older group). For MMR or deeper responses, 22 (32.9%) in the AYA group achieved this vs. 39 (56.3%) in the older group.

Kaplan Meier curve showed (A) Cumulative incidence of major molecular remissions (MMR). (B) Cumulative incidence of deep molecular remissions (DMR) across the two groups

Parameters	Age at diagnosis < 40 years Mean±SD [median (range)] n=114	Age at diagnosis ≥ 40 years Mean±SD [median (range)] n=110	P-value
Gender Male Female	 84 (73.7%) 30 (26.3%)	 81 (73.6%) 29 (26.4%)	0.994
WBC at diagnosis	187.5±148.8 [142.3 (5, 612)]	136.1±103.2 [120 (3.3, 521)]	0.037
Blasts (%) at diagnosis	3.5&±5.40 [2 (0, 33)]	2.79±7.4.09 [2 (0, 34)]	0.130
Hemoglobin at diagnosis	10.5±2.21 [10.5 (6.1, 15.5)]	11.40±2.01 [11.4 (6.5, 16.4)]	0.004
Platelet at diagnosis	360.1±216.5 [318 (48, 1294)]	394.7±308.2 [321 (18, 2158)]	0.758
Spleen size at diagnosis (in cm)	18.8±5.2 [19 (8.1, 29.2)]	15.5±5.01 [14.1 (8.6, 27)]	0.001
Disease phase on diagnosis CP AP BP-ALL BP-AML	 92 (83.6%) 12 (10.9%) 2 (1.8%) 4 (3.6%)	 93 (88.6%) 10 (9.5%) 2 (1.9%) 0 (0%)	0.254
Outcome at 1 year by MR Progression / Failure CCyR MMR DMR	 24 (35.8%) 21 (31.3%) 22 (32.9%) 12 (18.0%)	 10 (14.4%) 20 (29.0%) 39 (56.3%) 18 (25.9%)	0.111
Final outcome MMR DMR Death	 55 (56.7%) 36 (37.1%) 3 (2.6%)	 69 (73.4%) 44 (46.8%) 1 (0.9%)	0.016 0.175 0.330

CONCLUSION

AYA presents a unique age group. This study indicates a worse prognosis despite their younger age, despite most major risk scoring systems including older age as a worse prognostic factor. More studies are warranted to clarify the differences in CML disease biology and its possible effect on the outcome and formulate a new risk score for this age group.

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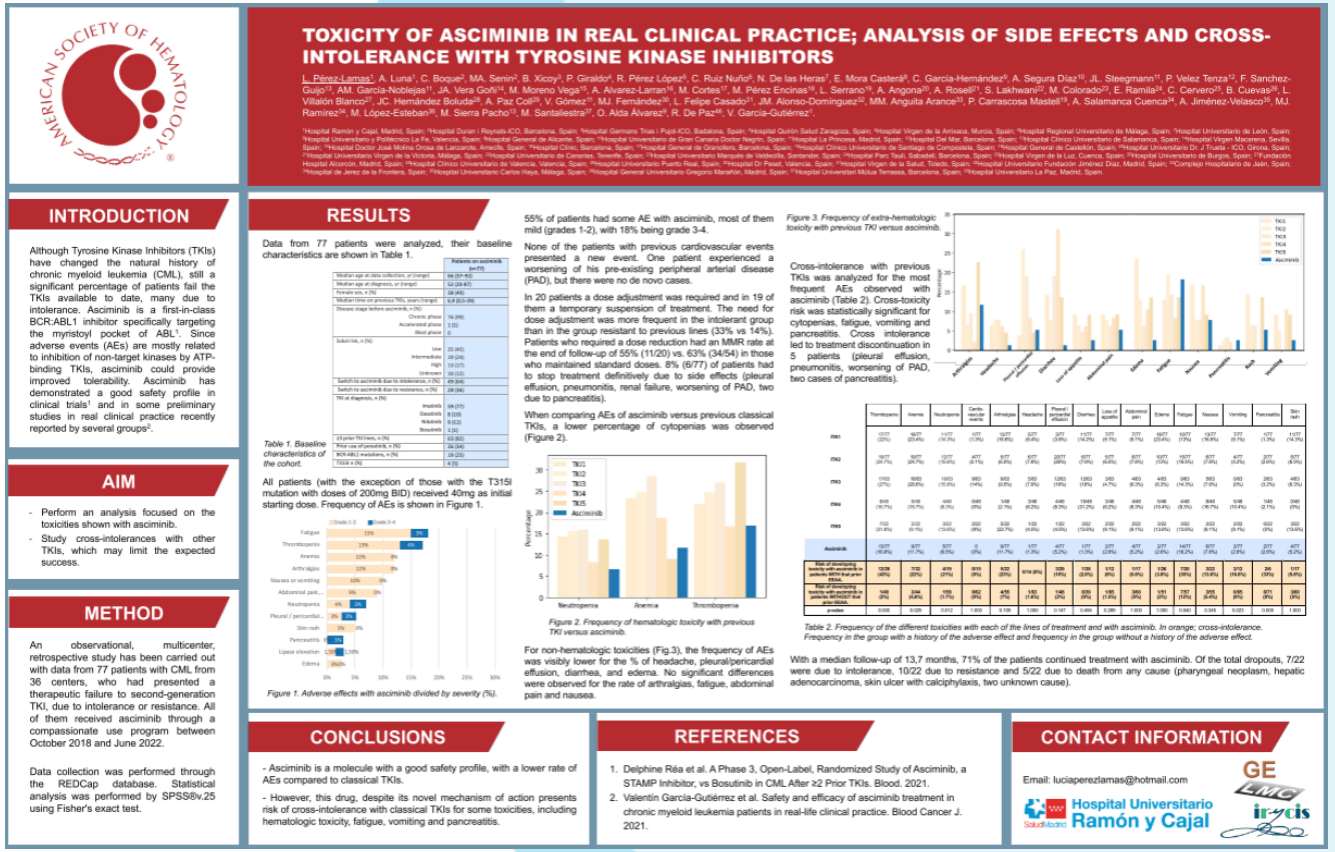
Poster Presentations

1708. Toxicity of Asciminib in Real Clinical Practice; Analysis of Side Effects and Cross-Intolerance with Tyrosine Kinase Inhibitors

Lucía Pérez-Lamas. Hospital Ramón y Cajal

- This is a retrospective study of 77 CML patients from 36 centers, who have failed the second-generation TKI. All of them received Asciminib through a compassionate program.
- A dose adjustment was required, being more frequent in the intolerant group than in the group resistant to previous lines.
- 73% of patients maintained or achieved a complete cytogenetic response (CCyR) and 60% a major molecular response (MMR).
- Responses were much better in patients who started asciminib for intolerance than in those who started it for resistance



TOXICITY OF ASCIMINIB IN REAL CLINICAL PRACTICE; ANALYSIS OF SIDE EFECTS AND CROSS-INTOLERANCE WITH TYROSINE KINASE INHIBITORS

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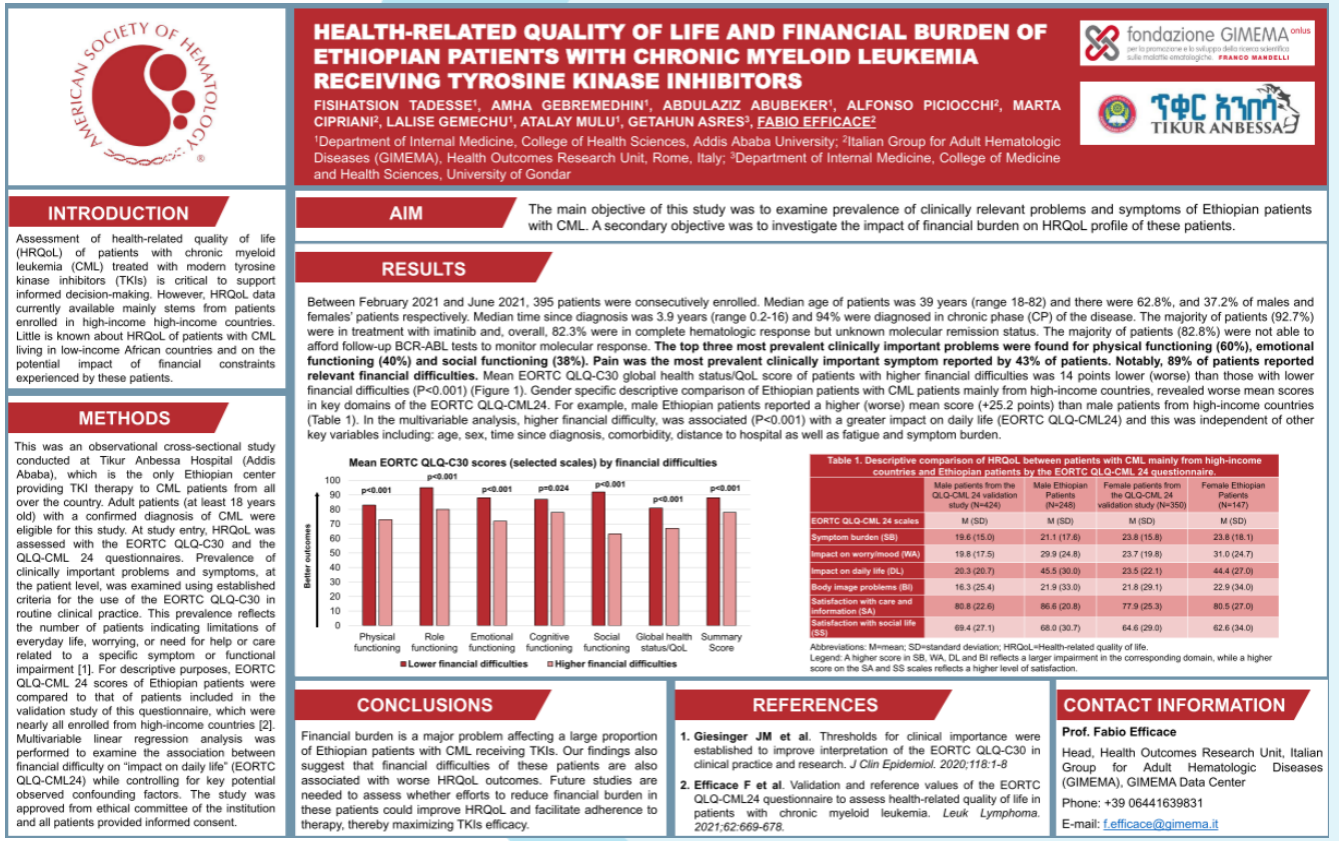
Poster Presentations

2306. Health-Related Quality of Life and Financial Burden of Ethiopian Patients with Chronic Myeloid Leukemia Receiving Tyrosine Kinase Inhibitors

Fabio Efficace, PhD. GIMEMA

- This study examined QoL and the impact of financial burden in QoL of patients from the only Ethiopian center providing TKI therapy to CML patients from all over the country. Tikur Anbessa Hospital
- The top three most prevalent clinically relevant problems were physical functioning (60%), emotional functioning (40%) and social functioning (38%), being pain the most prevalent symptom. Contrary to what is seen in high-income countries, financial difficulties was the most reported (89%) non-clinically relevant problem.
- It was observed a statistical significant association of higher financial difficulty with a greater impact on daily life.



HEALTH-RELATED QUALITY OF LIFE AND FINANCIAL BURDEN OF ETHIOPIAN PATIENTS WITH CHRONIC MYELOID LEUKEMIA RECEIVING TYROSINE KINASE INHIBITORS

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INTRODUCTION

Assessment of health-related quality of life (HRQoL) of patients with chronic myeloid leukemia (CML) treated with modern tyrosine kinase inhibitors (TKIs) is critical to support informed decision-making. However, HRQoL data currently available mainly stems from patients enrolled in high-income high-income countries. Little is known about HRQoL of patients with CML living in low-income African countries and on the potential impact of financial constraints experienced by these patients.

METHODS

This was an observational cross-sectional study conducted at Tikur Anbessa Hospital (Addis Ababa), which is the only Ethiopian center providing TKI therapy to CML patients from all over the country. Adult patients (at least 18 years old) with a confirmed diagnosis of CML were eligible for this study. At study entry, HRQoL was assessed with the EORTC QLQ-C30 and the QLQ-CML 24 questionnaires. Prevalence of clinically important problems and symptoms, at the patient level, was examined using established criteria for the use of the EORTC QLQ-C30 in routine clinical practice. This prevalence reflects the number of patients indicating limitations of everyday life, worrying, or need for help or care related to a specific symptom or functional impairment [1]. For descriptive purposes, EORTC QLQ-CML 24 scores of Ethiopian patients were compared to that of patients included in the validation study of this questionnaire, which were nearly all enrolled from high-income countries [2]. Multivariable linear regression analysis was performed to examine the association between financial difficulty on "impact on daily life" (EORTC QLQ-CML24) while controlling for key potential observed confounding factors. The study was approved from ethical committee of the institution and all patients provided informed consent.

AIM

The main objective of this study was to examine prevalence of clinically relevant problems and symptoms of Ethiopian patients with CML. A secondary objective was to investigate the impact of financial burden on HRQoL profile of these patients.

RESULTS

Between February 2021 and June 2021, 395 patients were consecutively enrolled. Median age of patients was 39 years (range 18-82) and there were 62.8%, and 37.2% of males and females' patients respectively. Median time since diagnosis was 3.9 years (range 0.2-16) and 94% were diagnosed in chronic phase (CP) of the disease. The majority of patients (92.7%) were in treatment with imatinib and, overall, 82.3% were in complete hematologic response but unknown molecular remission status. The majority of patients (82.8%) were not able to afford follow-up BCR-ABL tests to monitor molecular response. **The top three most prevalent clinically important problems were found for physical functioning (60%), emotional functioning (40%) and social functioning (38%). Pain was the most prevalent clinically important symptom reported by 43% of patients. Notably, 89% of patients reported relevant financial difficulties.** Mean EORTC QLQ-C30 global health status/QoL score of patients with higher financial difficulties was 14 points lower (worse) than those with lower financial difficulties (P<0.001) (Figure 1). Gender specific descriptive comparison of Ethiopian patients with CML patients mainly from high-income countries, revealed worse mean scores in key domains of the EORTC QLQ-CML24. For example, male Ethiopian patients reported a higher (worse) mean score (+25.2 points) than male patients from high-income countries (Table 1). In the multivariable analysis, higher financial difficulty, was associated (P<0.001) with a greater impact on daily life (EORTC QLQ-CML24) and this was independent of other key variables including: age, sex, time since diagnosis, comorbidity, distance to hospital as well as fatigue and symptom burden.

Mean EORTC QLQ-C30 scores (selected scales) by financial difficulties

Table 1. Descriptive comparison of HRQoL between patients with CML mainly from high-income countries and Ethiopian patients by the EORTC QLQ-CML 24 questionnaire.

	Male patients from the QLQ-CML 24 validation study (N=424)	Male Ethiopian Patients (N=248)	Female patients from the QLQ-CML 24 validation study (N=350)	Female Ethiopian Patients (N=147)
EORTC QLQ-CML 24 scales	M (SD)	M (SD)	M (SD)	M (SD)
Symptom burden (SB)	19.6 (15.0)	21.1 (17.6)	23.8 (15.8)	23.8 (18.1)
Impact on worry/mood (WA)	19.8 (17.5)	29.9 (24.8)	23.7 (19.8)	31.0 (24.7)
Impact on daily life (DL)	20.3 (20.7)	45.5 (30.0)	23.5 (22.1)	44.4 (27.0)
Body image problems (BI)	16.3 (25.4)	21.9 (33.0)	21.8 (29.1)	22.9 (34.0)
Satisfaction with care and information (SA)	80.8 (22.6)	86.6 (20.8)	77.9 (25.3)	80.5 (27.0)
Satisfaction with social life (SS)	69.4 (27.1)	68.0 (30.7)	64.6 (29.0)	62.6 (34.0)

Abbreviations: M=mean; SD=standard deviation; HRQoL=Health-related quality of life.
Legend: A higher score in SB, WA, DL and BI reflects a larger impairment in the corresponding domain, while a higher score on the SA and SS scales reflects a higher level of satisfaction.

CONCLUSIONS

Financial burden is a major problem affecting a large proportion of Ethiopian patients with CML receiving TKIs. Our findings also suggest that financial difficulties of these patients are also associated with worse HRQoL outcomes. Future studies are needed to assess whether efforts to reduce financial burden in these patients could improve HRQoL and facilitate adherence to therapy, thereby maximizing TKIs efficacy.

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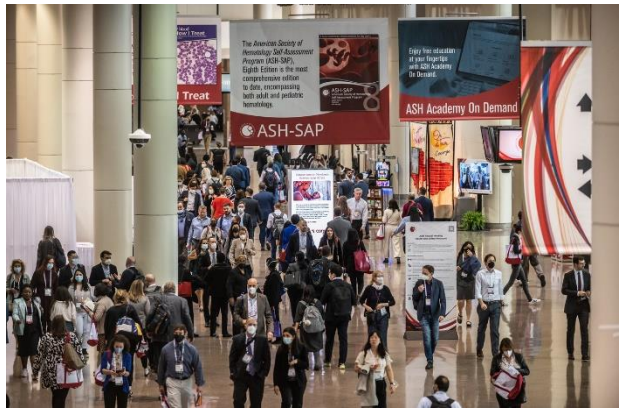
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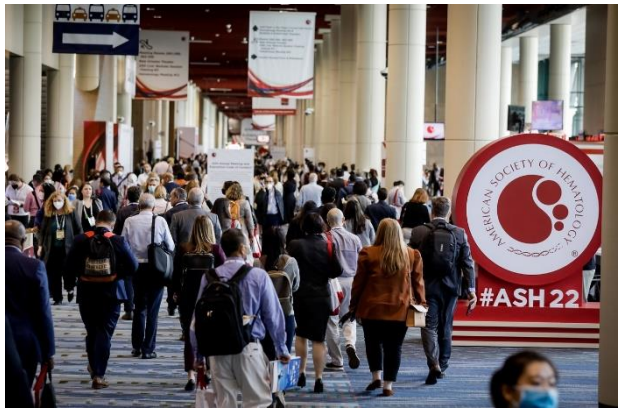
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