ACUTE LEUKEMIA GLOBAL SUMMIT- VIRTUAL, 15 OCTOBER 2021

www.acuteleuk.org
Acute Leukemia Global Summit
#BeLeukemiaAware
Agenda

Times in CET.

12.00 - 12.30
Welcome and Overview of ALAN activities

12.30 - 13.45
Regional sessions reporting

15 min break

14.00 - 15.30
Evidence-based advocacy

15.30 - 16.00
Q&A and closure
INTRODUCTIONS
About ALAN

- History – Founded in July 2017

- ALAN is:
  - an independent global network of patient organisations
  - dedicated to changing outcomes of patients with acute leukemia
  - by strengthening patient advocacy

- It aims to:
  - build capacity in the members of the network - deliver tailored services to acute leukemia patients and carers on the national level
  - join forces between organisations across countries – e.g. on the policy and research level
ALAN’s activities are:

➢ Patient-led
➢ Collaborative
➢ Evidence-based
ALAN 2021 ACTIVITIES – UPDATE

Initiatives of the Acute Leukemia Advocates Network
Ongoing Activities from 2020

• Advocate Development Program (ADP)
  – 8 webinars already done
  – Training will be running until Q4 2021
    https://acuteleuk.org/project/project-3/?53

• Global Quality of Life Survey
  – Publication – Manuscript submitted
  – Creation of Educational Materials
• What we did

  – Drug Development
    • Speaker: Samantha Nier
      ➤ https://acuteleuk.org/webinar-drug-development/
  – COVID-19 vaccination and immune challenges for leukemia
    • Hosts: ALAN and CLL AN
  – Patient involvement in clinical development
    • Speaker: Jan Geissler
      ➤ https://acuteleuk.org/webinar-patient-involvement-in-clinical-development/
  – The clinical trials we want!
    • Speaker: Bettina Ryll
      ➤ https://acuteleuk.org/join-our-webinar-on-24th-march-2021-1pm-cet-the-clinical-trials-we-want/
  – Patient reported outcomes, patient-relevant measures and endpoints
    • Speakers: Dagmara Kuliš, Madeline Pe, Sam Salek
      ➤ https://acuteleuk.org/upcoming-webinar-join-us-on-12th-may-2021-3pm-cet-patient-reported-outcomes-patient-relevant-measures-and-endpoints
Seminars

- **What we did**
  - **Focus on ALL – Highlights from EHA**
    - Speaker: Dr Tobias Menne
  - **Pediatric ALL (with CCI Europe)**
    - Speaker: Anita Kienesberger, CCI Europe
  - **Focus on AML – Highlights from EHA**
    - Speaker: Dr Manos Nikolousis
  - **Secondary AML and MDS (with MDS Alliance)**
    - Speaker: Prof. Moshe Mittelman
  - **Introduction to APL**
    - Speaker: Prof. Richard Dillon
  - **CAR-T: what’s new?**
    - Speaker: Dr Amit Patel

[https://acuteleuk.org/upcoming-seminars/](https://acuteleuk.org/upcoming-seminars/)
#BeLeukemiaAware #WorldLeukemiaDay

TODAY IS

World Leukemia Day

#WorldLeukemiaDay - 4th September 21
#BeLeukemiaAware #WLD21

https://acuteleuk.org/project/awareness-campaign/?53
On 15th March 2021, 13 networks of patient organisations wrote to ASH Executive Committee to highlight the current situation which is preventing patient advocates from attending the ASH Annual Meeting.

2 other supportive letters were sent:
- one signed by 50 national patient organisations
- one signed by our pharmaceutical supporters (+ topic addressed directly with ASH)

Few meetings with ASH

ASH took the decision to open free-registration for number of patient advocates (between 100 and 200 spots) to attend ASH 2021 either F2F or virtually.

Link for application:
Patient experience survey

This survey is being run by the ALAN in partnership with the CLL Advocates Network and CML Advocates Network. The questionnaire is about diagnosis, care and treatment for leukemia. Its purpose is to provide information to help us understand the key issues, experiences, and unmet needs for leukemia patients and their carers.

Taking part in this survey is voluntary and should take around 20 min to complete. Languages: English, Brazilian Portuguese, French, German, Hebrew, Italian, Korean, Simplified Chinese, Spanish and Russian

Patient survey
The questions should be answered by the person who has been diagnosed with leukemia. If help is given to complete the questionnaire, the answers should be given from the point of view of the person diagnosed with leukemia – not the point of view of the person who is helping. 

Carer survey
Opening soon

https://acutelleuk.org/project/patient-experience-survey/?53
Patient preference study

- **Objectives**
  - Better understand the outcomes of importance to patients and to illustrate their relative importance
  - Better understand and characterise the heterogeneity in patients' preferences
  - Support future technology appraisals and promote access to future treatment options

- **Project overview**

**COMPLETED**
- Literature review
- Interview stakeholders
- Steering group organisation
- Interactive workshop

https://acuteleuk.org/project/patient-preference-study/?53
Involvement in various initiatives

- HARMONY
- WECAN
- MRD Testing Collaborative
- European Cancer Organisation
- Share4Rare
- T2 Evolve
- IMI SISAQOL
- Etc.

https://acuteleuk.org/projects/
REGIONAL SESSIONS REPORTING
Our speakers

- **LATAM**: [Fernando Piotrowski](#), Executive Director ALMA (Argentina)
- **Middle East Africa**: [Bahija Gouimi](#), President and founder of AMAL Association (Morocco)
- **Eastern Europe**: [Mirjana Babamova](#), HEMA - Association for support of patients and caregivers of Hematology diseases (Macedonia)
- **Western Europe**: [Anne-Pierre Pickaert](#), Care4Access, representing Association Laurette Fugain (France)
Regional meeting - October 4

AGALEMO  Costa Rica
FUNDACIÓN COLOMBIANA DE LEUCEMIA Y LINFOMA  Colombia
ASOPALEU  Guatemala
MAXIVDA  Chile
ALMA  Argentina
SITUATION OF ORGANIZATIONS AND ACUTE LEUKEMIAS IN LATAM
organizations
Access to therapies

- Chemotherapies
- innovative treatments
  - Venetoclax
  - Blinatumomab
Access to therapies

Coverage

Private

Public
Patient contact

- Treatment Access
- Emotional Support
- Accompaniment
Activities

- Meetings
- Webinars
Fernando Piotrowski
direccion@asociacionalma.org.ar
Acute Leukemia Global Summit

15 October 2021

Bahija GOUMI
Founder and President of AMAL
CML Advocates Network SC member
Africa and Middle East representative
Middle East & Africa Region

- 73 countries
- Youthful population (50% in ME and 60% in Africa under the age of 24)
- Many languages
- Arabic is spoken by over 170 million people in Africa
- English is prevalent throughout the entire region
- Huge difference between countries (UAE and Niger)
- High illiteracy rate (120 million people)
- Internal conflicts / Political instability
- LMIC / Emerging markets
Challenges within the region

- Access is still a big issue in the region
- Lack of monitoring and diagnostic capacity
- Centralized treatment (late diagnostic, discontinuation, delays on treatment, disease complication, relapse, ...)
- Lack of specialized centers
- Financial incapacity to seek medical treatment (lack of health insurance)
- Social obstacles which include Stigma, poverty, cultural believes and traditional practices
- Absence of cancer data
- No clinical trials, no researches, no BMT
- Lack of psychological support in hospitals
- Information access
- Women are most affected by cancer, either she is the patient or the accompanying person
- Lack of patient empowerment
- Specific culture
- Patients approach the association only when they need help
As a region...

- We need to collaborate and work together to
  - Improve patients outcomes
  - Improve disease management in order to improve QoL of patients
  - Engage HCPs of the region to consolidate relations
  - Engage more the patient community locally
  - Collecting data (No cancer registry)
  - Building capacities of Patient organizations
  - Sharing best practices & organizing common events
  - Learn from strong networks of other regions
  - Developping active partnerships
**Actions**

**Education Awareness**
- **EDUCATION** of patients and parents
- Producing cancer educational materials
- Cancer prevention campaigns at schools and hospitals
- Media/Public awareness

**Support**
- Assistance of patients and parents (information, orientation, administrative procedures, donations, transportation fees ...)
- Psychological support (patient meetings, storytelling sessions, parties, gifts)

**Advocacy**
- Meetings with MOH
- Recommendations sent to medical and legal authorities
- Support of the HCPs in taking actions
- Advocacy awareness campaigns

**Medical training**
- General practitioners trainings
- Hematology lectures
- Nurses training
توصيات علاجية للأشخاص المصابين ببيضاض الدم النخاعي المزمن (اللوكيميا)
Patient meetings
Virtual meetings with physicians
General practitioner trainings

- April 2016
  Tinghir

- December 2016
  Marrakech

- December 2017
  Settat

- April 2018
  Boumalen Dades

- May 2019
  Marrakech

- December 2020
  Virtual

- June 2021
  Marrakech
Patient Home
Ongoing construction
Association AMAL (Association des Malades Atteints de Leucémies)

Association_Amal

Association_amal

Assoc.AMAL

bahijagouimi@gmail.com

0613281784
ACUTE LEUKEMIA GLOBAL SUMMIT
REGIONAL SESSION REPORT-EASTERN EUROPE

MIRJANA BABAMOVA, HEMA - ASSOCIATION FOR SUPPORT OF PATIENTS AND CAREGIVERS OF HEMATOLOGY DISEASES

www.acuteleuk.org

www.hema.org.mk
Differences between Eastern European countries those that are members of EU and the non EU members countries

**Non EU members countries in EE**

- Challenges with access to essential and novel therapies
- Challenges with access to clinical trials
- Lack of funding for PO’s
- Lack of advocates for Acute Leukemias
- Corruption
- People are afraid to lobby and stand up for their rights due to the political situation(s)
- Reimbursement lists have not been renewed for many years
- COVID-19 controversies in delay of treatment

**Countries members of EU in EE**

- Better access to treatment however at times there may be a delay in access (2 years or more)
- Limited funding
- Few blood cancer centers – investigators are struggling to enroll patients
- Lack of referrals to a specialized centers
- COVID-19 issues in the delay of treatment
- Lack of advocates for Acute Leukemias
Access to Innovative Therapies in the Balkan Countries

The reimbursement of therapies in MACEDONIA has been blocked in the last 15 years.

In SERBIA between 2007 and 2016 just 12 of the 228 new medicines to receive market authorization were approved for reimbursement.

In comparison, 148 new therapies were reimbursed in Slovenia, 83 in Bulgaria and 62 in Croatia.
Patient Organizations in Eastern Europe should work together on issues and Challenges

- Policy and Public Affairs work
- Health Technology Assessment (HTA) as a method of evidence synthesis that considers evidence regarding clinical effectiveness, safety, cost-effectiveness and also can include social, ethical and legal aspects of health technologies
- Integration of patients preferences into evidence-based practice
- Work with industry on market access
- EE and Balkan Community Advisory Boards (CABs)
- Working with healthcare professionals to design joint initiatives
- Establishing an Eastern Europe Blood Cancer Patient Organization Network

Most Patient Organizations in Eastern Europe ARE NOT involved in the access discussions
Thank You

TOGETHER WE CAN MAKE A DIFFERENCE!
REGIONAL SESSION – WESTERN EUROPE

www.acuteleuk.org
Hello, I am Anne-Pierre

Acute leukemia & bone marrow transplant patient advocate
Patient advocates recruitment challenges

Survival urgency & treatment brutality

- Few acute leukemia patients become advocates (vs. other blood cancer patient advocates)
Patient organisations’ continuity at risk

• Individual- & volunteer-dependent organisations

• Less money, but more things to do
  – Decrease in general public’s & industry’s financial support
  – Increase in COVID-related information requests
  – Increase in patient requests for financial support

Credits: https://www.smallbusinessact.com/blog/facteurs-de-perennite-d-entreprise/
Patient travel and financial burdens remain

- Geographical disparities in access to care
- More and more patients ask for grants to cover travel and/or treatments.

Need for more ambulatory treatments to reduce travel requirements

Patient access to treatments, but uneven patient organisations’ influence on HTA decisions

• Despite HTA delays, limited access issues on life-saving drugs.

• Patient organisations do not always get a chance to influence decisions.
Increasing use of haploidentical bone marrow transplants based on retrospective Italian & US data
Italian & US retrospective data potentially suggest similar efficacy in AML/MDS

<table>
<thead>
<tr>
<th>Study</th>
<th>Cond</th>
<th>N</th>
<th>aGvHD II-IV</th>
<th>cGvHD</th>
<th>Relapse-free mortality</th>
<th>Relapse</th>
<th>Overall survival</th>
<th>Event-free survival</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ciurea Blood 2015 (1)</td>
<td>MAC</td>
<td>Haplo: 104 10/10: 1245</td>
<td>Haplo: 16% 10/10: 33%</td>
<td>Haplo: 30% 10/10: 53%</td>
<td>Haplo: 14% 10/10: 20%</td>
<td>Haplo: 44% 10/10: 39%</td>
<td>Haplo: 45% 10/10: 50%</td>
<td>Haplo: 42% 10/10: 41%</td>
</tr>
<tr>
<td>Rashidi BBMT 2016 (2)</td>
<td>Mix</td>
<td>Haplo: 52 10/10: 88</td>
<td>Haplo: 40% 10/10: 36%</td>
<td>Haplo: 10% 10/10: 9%</td>
<td>Haplo: 27% 10/10: 27%</td>
<td>Haplo: 29% 10/10: 43</td>
<td>Haplo: 42% 10/10: 37%</td>
<td>Haplo: 44% 10/10: 30%</td>
</tr>
<tr>
<td>Rashidi BMT 2016 (4)</td>
<td>Mix</td>
<td>Haplo: 62 10/10: 21</td>
<td>Haplo: 40% 10/10: 19%</td>
<td>Haplo: 6% 10/10: 5%</td>
<td>Haplo: 22% 10/10: 16%</td>
<td>Haplo: 31% 10/10: 26%</td>
<td>Haplo: 53% 10/10: 58%</td>
<td>-</td>
</tr>
<tr>
<td>Gooptu, Blood 2021 (6)</td>
<td>MAC</td>
<td>HR: 0.66 p=0.05</td>
<td>-</td>
<td>-</td>
<td>No difference</td>
<td>No difference</td>
<td>No difference</td>
<td>No difference</td>
</tr>
<tr>
<td>Distasi BBMT 2014 (5)</td>
<td>RIC</td>
<td>Haplo: 2036 10/10: 284</td>
<td>HR: 0.70 p=0.022</td>
<td>-</td>
<td>No difference</td>
<td>No difference</td>
<td>No difference</td>
<td>No difference</td>
</tr>
</tbody>
</table>

Statistically significant results in favor of haplo BMT

Statistically significant results NOT in favor of haplo BMT

Gooptu, Blood 2021 (6)

https://ashpublications.org/blood/article/126/8/1033/34733/Haploidentical-transplant-with-
posttransplant-/-1 https://www.astrojournal.org/article/2018/8/2703196/108212/fulltext-
Italian & US retrospective data potentially suggest less GvHD, regardless of conditioning treatment

<table>
<thead>
<tr>
<th>Study</th>
<th>Cond</th>
<th>N</th>
<th>aGvHD II-IV</th>
<th>cGvHD</th>
<th>Relapse-free mortality</th>
<th>Relapse</th>
<th>Overall survival</th>
<th>Event-free survival</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ciurea Blood 2015 (1)</td>
<td>MAC</td>
<td>Haplo: 104 10/10: 1245</td>
<td>Haplo: 16% 10/10: 33%</td>
<td>Haplo: 30% 10/10: 53%</td>
<td>Haplo: 14% 10/10: 20%</td>
<td>Haplo: 44% 10/10: 39%</td>
<td>Haplo: 45% 10/10: 50%</td>
<td>Haplo: 42% 10/10: 41%</td>
</tr>
<tr>
<td></td>
<td>RIC</td>
<td>Haplo: 88 10/10: 737</td>
<td>Haplo: 19% 10/10: 28%</td>
<td>Haplo: 34% 10/10: 52%</td>
<td>Haplo: 9% 10/10: 23%</td>
<td>Haplo: 58% 10/10: 42%</td>
<td>Haplo: 46% 10/10: 44%</td>
<td>Haplo: 33% 10/10: 35%</td>
</tr>
<tr>
<td>Rashidi BBMT 2016 (2)</td>
<td>Mix</td>
<td>Haplo: 52 10/10: 88</td>
<td>Haplo: 40% 10/10: 36%</td>
<td>Haplo: 10% 10/10: 9%</td>
<td>Haplo: 27% 10/10: 27%</td>
<td>Haplo: 29% 10/10: 43</td>
<td>Haplo: 42% 10/10: 37%</td>
<td>Haplo: 44% 10/10: 30%</td>
</tr>
<tr>
<td>Rashidi BMT 2016 (4)</td>
<td>Mix</td>
<td>Haplo: 62 10/10: 21</td>
<td>Haplo: 40% 10/10: 19%</td>
<td>Haplo: 6% 10/10: 5%</td>
<td>Haplo: 22% 10/10: 16%</td>
<td>Haplo: 31% 10/10: 26%</td>
<td>Haplo: 53% 10/10: 58%</td>
<td>-</td>
</tr>
<tr>
<td>Gooptu, blood 2021 (6)</td>
<td>MAC</td>
<td>Haplo: 2036 10/10: 284</td>
<td>HR: 0.70 p=0.022</td>
<td>HR: 0.43 p=.0008</td>
<td>HR: 0.66 p=0.05</td>
<td>No difference</td>
<td>No difference</td>
<td>No difference</td>
</tr>
</tbody>
</table>


Statistically significant results in favor of haplo BMT
Statistically significant results NOT in favor of haplo BMT

AML  AML/MDS
Randomised controlled trials

- **MacHaploMud (AML/ALL):** Haplo vs. 10/10 < 55 ans
- **HAPLO-RESCUE (hematological malignancies):**
  1st BMT failure
- **Altergref (hematological malignancies):**
  Haplo vs. 9/10 < 55 ans
- **HAPLOMUDELDERLY (hematological malignancies):**
  Haplo vs. 10/10 > 55 ans

Different results expected

- Disease (AML, MDS, CML, Lymphoma etc.)
- Conditioning (MAC vs. RIC)
- Bone marrow vs. stem-cell
- Age
- Other/to be determined
Bone marrow registries further align on reducing the maximum age limit

<table>
<thead>
<tr>
<th>Bone marrow registry</th>
<th>Age limits</th>
<th># registrations</th>
<th>Year of creation</th>
</tr>
</thead>
<tbody>
<tr>
<td>DKMS (Allemagne)</td>
<td>17-55 years old</td>
<td>9,322,899</td>
<td>1991</td>
</tr>
<tr>
<td>DKMS Polska (Pologne)</td>
<td>17-55 years old</td>
<td>1,660,808</td>
<td>2009</td>
</tr>
<tr>
<td>DKMS UK (Royaume Uni)</td>
<td>17-55 years old</td>
<td>783,348</td>
<td>2013</td>
</tr>
<tr>
<td>Anthony Nolan (UK)</td>
<td>16-30 ans</td>
<td>836,291</td>
<td>1974</td>
</tr>
<tr>
<td>British Bone Marrow Registry [BBMR] (UK)</td>
<td>17-40 ans</td>
<td>375,651</td>
<td>1987</td>
</tr>
<tr>
<td>Welsh Bone Marrow Donor Registry</td>
<td>17-30 ans</td>
<td>72,133</td>
<td>1990</td>
</tr>
<tr>
<td>Registro Nazionale Italiano Donatori di Midollo Osseo (Italy)</td>
<td>18-35 years old</td>
<td>460,902</td>
<td>1989</td>
</tr>
<tr>
<td>REDMO (Spain)</td>
<td>18-40 years old (vs. 55 years old prior to Jan 1, 2018)</td>
<td>431,703</td>
<td>1991</td>
</tr>
<tr>
<td>French bone marrow donor registry</td>
<td>18-35 years old (vs. 51 years old prior to Jan 1, 2021)</td>
<td>321,121</td>
<td>1986</td>
</tr>
</tbody>
</table>

https://www.dondemoelleosseuse.fr/35-ans-le-nouvel-age-limite-pour-sinscrire-comme-donneurse-de-moelle-osseuse
QUESTIONS?
EVIDENCE BASED ADVOCACY
Our speakers

- **Zack Pemberton-Whiteley**, Chair of ALAN and CEO of Leukaemia Care (UK)
- **Jana Pelouchova**, Chair of Diagnoza Leukemie (Czech Republic), founder CML Advocates Network
- **Lauren Pretorius**, CEO Campaigning for Cancer (South Africa)
- **Tracey Iraca**, Executive Director MDS Alliance (USA)
Using evidence for advocacy impact

ZACK PEMBERTON-WHITELEY
LEUKAEMIA CARE – CHIEF EXECUTIVE OFFICER
ZACKPW@LEUKAEMIACARE.ORG.UK
Evidence-based advocacy – What is it?

Systematic and targeted collection, interpretation, generation and deployment of sound data and information, presented and used in patient advocacy with an objective in mind.

Advocating in a targeted, evidence-based, well-educated and professional manner, and measure impact and outcomes of what we do.
Why use evidence?

Credibility
Why use evidence?

Understanding
Responsibility on the patient community to use EBA to drive change...

UK based patient experience survey of 2,329 leukaemia patients from Leukaemia Care.

Used to identify unmet needs and to inform:

- Organisational strategy
- Campaigns – e.g. patient issues
- Evidenced access arguments – to inform NICE submissions
- Publish at scientific conferences
How to use evidence..

1. Listen to the evidence
2. Act on the evidence

**DO NOT** use evidence just to back up your existing point of view

I see here that I’m right about everything.
Methodology is key...
Does your evidence show what you think it does?

- Did you ask the right question?
- Did you ask the right people?
- Did you ask enough people?
- Is your conclusion what the data shows?
- Is it statistically valid?
Key elements of evidence generation for EBA

Steps:
- Strategy & Research question
- Survey design
- Implementation
- Survey/Study Conduct
- Data Analysis
- Publications & reporting

Tasks:
- Define objectives and research questions
- Questionnaire design
- Build survey, IT & database
- Recruitment & Progress Report
- Analysis
- Generate Presentations
- Selection of methodology
- Ethics approval
- Response monitoring
- Summary of main findings
- Selection of media and writing publication
- Question & survey library
- Data protection measures
- Identify professional writer
- Write publication

Slide from WECAN training course on Evidence-Based Advocacy
Be clear from the start how you want to use the data

YOUR PURPOSE DETERMINES WHAT TYPE OF PROJECT TO RUN...
Setting your strategy and research question?

At the **outset** of any project, and at any point you make significant **changes** to your plans, ask yourself:

- What am I trying to achieve?
  - What is your desired advocacy impact?
  - What do you want to show, and to whom?
  - What evidence do you need to back up your work?
  - What is already known and what do you need to collect?
  - What types of evidence will be most effective with your stakeholder group?
  - What is your analysis strategy?

- Everything flows back from this – e.g. type of evidence generation, publication decision – consider undertaking strategic review
As a general rule of thumb...

Use to help identify:

1. What **type** of evidence **generation**
2. **What** to collect evidence on?
3. Or whether you even need to collect any?

- Thinking here about what is known to **other researchers**
- And what is only **known** to patients and/or advocates

---

**What is already **known**?**
Considering type of evidence generation (e.g. survey)

Depending upon your aims there are different (and complementary) approaches:

➢ Pure Questionnaire – e.g. multiple choice, rank order or ‘tick box’ Qs
➢ Pure Interview – written/oral structured open Qs
➢ Mixture – e.g. some multiple choice and some open Qs

In terms of designing questions:

➢ Create your own – e.g. in a novel area
➢ Use existing questions
   ▪ Entire PROMs - within a questionnaire
   ▪ Individual Qs – from question and survey library
Don’t just generate evidence for the sake of it!

BE CLEAR AT THE START ABOUT WHAT DATA YOU NEED AND HOW IT WILL BE USED
Patients should also be involved in external evidence generation...

Case Study: Living with leukaemia
Project Overview

- **Aims** – Exploring UK leukaemia patient experience
- **Topics** – Following the journey from diagnosis onwards
- **Testing** – To refine the content
- **Comparability** – between annual versions

@ZPWLC
Methodology: Cohorts

CPES
• Patients identified using the NHS CPES survey

Leukaemia Care
• LC Database
• Had a valid postal address (for paper survey)

Anonymous
• Online link
• Wider blood cancer community
• LC email consent
Different output types

Reports and Presentations

Leukaemia Care
Living with Leukaemia
2018 Report

Scientific Publications

As a starter for follow-up projects...

+ Journal articles
How can patient groups use RWE in their activities?

EXAMPLE FROM LEUKAEMIA CARE (UK)
Defining Unmet Needs

**Identify:** What are the issues to focus on

**Measure**
- Quantify issues (e.g. the % of patients experiencing)
- Differences between particular groups, such as demographics, cancer type, regions, time

**Solutions:** Create a set of recommendations to address

**Further Questions** - Is there anything you need more evidence on?
Organisational Strategy

How are you going to address the unmet needs?

Questions to think about:
What are you already addressing?
What should you do differently?
What do you need to do more of?
What are you not doing?
What can you NOT do? What should be worked on by others?

Collect Further Evidence
Use Key Performance Indicators (KPIs) – to measure outcomes and impact
Collect data and compare – e.g. performance across years
Developing New Services

One you have identified an unmet need to focus on…

- Designing the service – what exactly do you need to address?
- Use evidence for your funding application
- Use feedback to refine existing services
  - Use Key Performance Indicators (KPIs) – to measure outcomes and impact
  - Individual feedback from users (e.g. survey)
- Consider running a small PILOT to show it works?
Campaigns – Awareness and Early Diagnosis

Understand your audience
- Public v Patient – are you targeting a specific demographic?
- What does the audience already know?

Use Evidence
- Your own (e.g. survey)
- External (e.g. NCIN Routes to Diagnosis)

Consider your message
- What does the audience need to know?
- Where to focus? – e.g. MPN, blood cancer or cancer
Campaigns – Patient Issues

- Using evidence to develop and run campaigns to address specific issues

- **Audience** – Patients? Clinical community?

- **Evidence** – Tailor to look at differences? Can you do a specific survey? Or breakdown of an existing survey?

- **Message** – What are you trying to change?

**Example: ‘Watch Wait Worry’ campaign**

- We developed:
  - Evidence Report – for clinicians
  - Supportive guidance for CLL patients on watch and wait
  - Social media campaign

- Poster at EHA on the emotional impact of watch and wait for CLL
Different HTA processes have different opportunities for patient organisations to get involved.

In most cases the consideration of patient perspective is **qualitative**, so it is difficult to understand the impact on decision making.

You can create **quantitative** evidence (e.g. patient surveys), but at present there is no mechanism for the inclusion of such evidence.

Focus on:
- Influencing the areas that influence decision making (e.g. cost-effectiveness)
- Impact not involvement
- Explaining existing evidence (e.g. trial) and the benefit from a patient perspective
EVIDENCE BASED ADVOCACY WITHIN GLOBAL NETWORKS AND NATIONAL ORGANISATIONS

➢ JANA PELOUCHOVA
➢ CHAIRPERSON, DIAGNOZA LEUKEMIE PATIENT SOCIETY, CZECH REPUBLIC
➢ CO-FOUNDER, CML ADVOCATES NETWORK

www.acuteleuk.org
FACTORS INFLUENCING ADHERENCE IN CML AND WAYS TO IMPROVE IT:

- survey of 2546 patients in 63 countries:
  - 8% of patients missed their doses in the last month (accidentally and on purpose)
- most impactful research - presented at EHA, ESH, poster ASH (implemented into clinical practice)
CML Advocates Network: Patients creating scientific evidence

Global study runned on 2012-2013 by the CML Advocates Network in 12 languages.

Identical questionnaire provided to a cohort of patients recruited in clinics in France, Germany and Italy, returned by patients in a pre-stamped envelope to an independent data center.

2151 patients responded online, 395 questionnaires were returned on paper.

CML ATLAS OF CENTRAL AND EASTERN EUROPE AND WEST ASIA:

- relevant data on access to therapies and monitoring tools to support CML organisation’s advocacy in the region.
CHRONIC MYELOID LEUKEMIA ACCESS TO TYROSINE KINASE INHIBITORS AND POLYMERASE CHAIN REACTION TESTING IN CENTRAL AND EASTERN EUROPE AND WEST ASIA REGION: A PATIENT ADVOCACY PERSPECTIVE.

Jelena Ćugurović1, Šarūnas Narbutas1, Jana Pelouchová1, Denis Costello1, Colla Marin1
1CML Advocates Network, Bern 7, Switzerland

INTRODUCTION

Chronic myeloid leukemia (CML) patient organisations representing countries of the CML Advocates Network Eastern Europe and West Asia region have come to conclusion that they share similar issues in access to treatment and monitoring tools.

The reason is the lack of a common database with the relevant information about CML.

OBJECTIVES

The goal was to create the CML Atlas of Central and Eastern Europe and West Asia region to showcase the most relevant data on CML about access to tyrosine kinase Inhibitors (TKIs) and monitoring tools to support CML patient organisations’ advocacy in those countries.

METHOD

The European online survey recruited CML patient advocates and doctors from 19 countries, representing over 26,000 CML patients from Armenia, Croatia, Czech Republic, Georgia, Hungary, Kazakhstan, Kosovo, Kyrgyzstan, Lithuania, Moldova, North Macedonia, Poland, Russia, Serbia, Slovenia, Tajikistan, Turkey, Ukraine and Uzbekistan.

The survey started on 1st December 2019 and lasted until 31st March 2020 covering the following topics:

• CML general information
• Availability of TKIs and PCR testing
• Clinical trials and treatment free remission data.

*Presented data as of 1st July 2020.

RESULTS

Number of countries where medicine is available, by type of availability

Number of countries where medicine is available, by cost coverage

Patients received with specific medicine in %

CONCLUSIONS

There are important differences in access to TKIs to treat CML patients between countries of the Central and Eastern Europe and West Asia region. While five TKIs are available on these countries, the majority of them are just available on insurance and/or donations. Access to free PCR testing for CML patients is not ensured in all countries. There are opportunities to improve access to high-quality and safe therapies and effective monitoring tools for CML patients in the region.

ACKNOWLEDGEMENTS

We would like to express our special thanks of gratitude to the CML Advocates Network members of the Central and Eastern Europe and West Asia region, CML patient advocates and to all who has collaborated supporting this research.

CONTACT INFORMATION

CML Advocates Network
info@cmladvocates.net
cmladvocates.net

https://john-goldman-cml-2021.esh.live/
FIRST STEP TO INVOLVING PATIENTS IN THE ASSESSMENT PROCESSES – CHANGE OF HEALTHCARE LEGISLATION IN THE CZECH REPUBLIC

- Patients’ Advisory Board of the Health Minister (2017 - 2021)
- Definition of Patient Organisation
- Implementation within the new legislation
THANK YOU FOR YOUR ATTENTION

➢ www.diagnoza-leukemie.cz
➢ facebook.com/diagnoza.leukemie
➢ jana.pelouchova@diagnozaleukemie.cz
REMOVING BARRIERS TO ACCESS FOR PATIENTS:
The right treatment; at the right time; with no barriers.
CAMPAIGNING FOR CANCER – A SNAPSHOTT OF WHAT WE DO

OUR VISION: To create a South Africa where people affected by cancer receive fair, appropriate, timeous, respectful and quality treatment and care.

The right information, support, treatment and care, at the right time, with no barriers

Incorporate the patient’s voice into a stakeholder’s strategy to provide a better standard of care

The truth around cancer stigmas and myths for earlier detection

Open-source health promotion, resources for patient groups across the region

www.campaign4cancer.co.za
TWITTER | FACEBOOK | INSTAGRAM @campaign4cancer
### WHAT DO PATIENTS ADVOCATES WANT?

<table>
<thead>
<tr>
<th>Access to effective treatments</th>
</tr>
</thead>
<tbody>
<tr>
<td>▪ Availability</td>
</tr>
<tr>
<td>▪ Affordability</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Timely decisions</th>
</tr>
</thead>
<tbody>
<tr>
<td>▪ Avoid Launch delays</td>
</tr>
<tr>
<td>▪ Manage Launch sequencing</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Contribute to the evidence generation process</th>
</tr>
</thead>
<tbody>
<tr>
<td>▪ Why is a new drug “essential”? To whom?</td>
</tr>
<tr>
<td>▪ Evidence of what it means to live with the disease</td>
</tr>
<tr>
<td>▪ Costs &amp; Quality of Life</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Contribute to the decision-making process</th>
</tr>
</thead>
<tbody>
<tr>
<td>▪ Participation in reimbursement committees</td>
</tr>
<tr>
<td>▪ Evidence submission to HTA bodies</td>
</tr>
</tbody>
</table>
WE NEED TO REFRAME THE DISCUSSION ABOUT ACCESS.

Start with the person.
“universal health coverage” as defined by the WHO provides for 3 goals:

1. Financial Risk Protection
2. Improved Access to Services
3. Improved Health Outcomes.

The WHO further describes “coverage” as a situation where citizens can access health services without incurring financial hardship: a system of protection which provides the equality of opportunity for people to enjoy an attainable level of health.
**Equality**

The assumption is that everyone benefits from the same supports. This is equal treatment.

**Equity**

Everyone gets the supports they need (this is the concept of “affirmative action”), thus producing equity.

**Justice**

All 3 can see the game without supports or accommodations because the cause(s) of the inequity was addressed. The systemic barrier has been removed.
ACCESSING TREATMENT - CASES STUDIES

**Education Awareness**
- Messaging not addressing KAP
- Once Behaviour change achieved not supporting services

**Screening Diagnosis**
- Access to screening facilities
- Repeat visits before referral
- No understanding/explanation diagnoses and need for treatment
- Equipment not working
- Medical Schemes not covering screening and prevention costs

**Treatment**
- Equipment not working
- Drugs not available
- Waiting periods for treatment
- Accessing follow up treatment not understood
- Medical not being purchased
- Schemes not reimbursing
- Protracted escalation process
- Caught in the middle of a technical he said she said

**Support**
- Little access to social services
- Little home care
- Explaining to family/carer of patient journey
- Accessing credible material
- Knowing where to find it

**Palliative Care**
- Access to adequate pain relief
- Few end of life resources
- Access to adequate pain relief
- Schemes not covering end of life care
THE ADVANCEMENT OF MEDICINE: WHAT IT MEANS FOR PATIENTS?
IT MEANS NOTHING IF THEY CAN’T ACCESS IT.
WHAT QUESTIONS TO ASK WHEN COLLECTING DATA TO SUPPORT PATIENT ACCESS?

Leadership

Can we really say patient’s are getting the right treatment, at the right time, with the least challenges?

Innovation

If we continue with the same mind set, we had before, are we dead in the water?

Commitment

Dedication to a cause or obligation that restricts freedom of action?
HOW WE HELP PATIENTS

- Sustained National Advocacy Effort
- Community
- One on One with Patients
This message is so simple, yet it gets forgotten. The people living with the condition are the experts.

Michael J Fox
Exploring an unmet need...

Do patients know their subtype and IPSS-R score? Knowing their subtype and IPSS-R score can empower patients to become shared decision-makers in their care.
Knowledge is Power initiative – Research

- Know Your Subtype; Know Your Score
  - Survey released October 2019 - 577 respondents
    - 421 patients; 122 caregivers; 34 other
  - Of the patients
    - 50% knew their subtype (50% did not)
    - 31% knew their IPSS-R score (69% did not)
  - Of the caregivers/others
    - 43% knew their loved one’s subtype (57% did not)
    - 25% knew their loved one’s IPSS-R score (75% did not)
- Conclusion: there is significant room for improvement in educating and empowering patients and caregivers to take a more active role in treatment decisions with their healthcare team, based on the knowledge of these individualized criteria
Knowledge is Power initiative – Infographics

- KIP infographics released in 2020 via social media, newsletters, website and email blasts (added Know Your Mutation to Know Your Score and Know Your Subtype)

KIP infographic released in 2020 via social media, newsletters, website and email blasts (added Know Your Mutation to Know Your Score and Know Your Subtype)

Knowledge is Power

- **ASK YOUR HEALTHCARE TEAM...**

- **KNOW YOUR SCORE**

The IPSS is an classification system used by doctors to help predict a person’s risk of developing AML, and overall survival without treatment.

**CATEGORIES & SCORES**

- Very Low: 0.0 – 1.0
- Low: 1.1 – 3.5
- Intermediate: 3.6 – 5.5
- High: 5.6 – 7.5
- Very High: 7.6 – 11.5

- **KNOW YOUR SUBTYPE**

MDS is classified into several different subtypes based on the following features:

- Blood cell counts
- Percentage of blasts in the bone marrow
- Cytogenetics

The Revised International Prognostic Scoring System (IPSS-R) is used to estimate life expectancy for a patient newly diagnosed with MDS without treatment and to estimate the risk of developing acute myelogenous leukemia (AML).

- A bone marrow biopsy and aspirate, cytogenetics and peripheral blood counts are used to determine your risk category (Very Low, Low, Intermediate, High, Very High).

**IPSS-R PROGNOSIS VALUES**

- Cytogenetics
- Hemoglobin
- MDS marrow blasts
- Platelets
- Absolute neutrophil count
Knowledge is Power initiative – Microsite

- KIP microsite released in 2021 [https://www.mdsknowledgeispower.com/](https://www.mdsknowledgeispower.com/)
Knowledge is Power initiative – Brochure

• KIP brochure distributed in 2021

This brochure is intended to help you better understand the diagnosis of MDS. Created by the MDS Foundation staff, Board of Directors, and medical and scientific leaders, it will explain the various MDS subtypes; how a prognostic scoring system is designed and where you can place yourself with the help of your physician and other health professionals.

You will learn about normal and abnormal blood cells; leukemic blasts; blood counts; chromosomes and molecular mutations that may assist your provider in further modifying your subtype and, possibly, selecting the type of therapy for you.

John M. Bennett, MD
Knowledge is Power initiative – Follow up

- Repeat Survey distributed by MDSF in October 2021 to determine if the initiative has made an impact to date. Includes the original questions with the addition of gene mutation questions.

- Complimentary global campaign through the MDS Alliance for MDS WAD 2021 - #LearnYourType
QUESTIONS?