

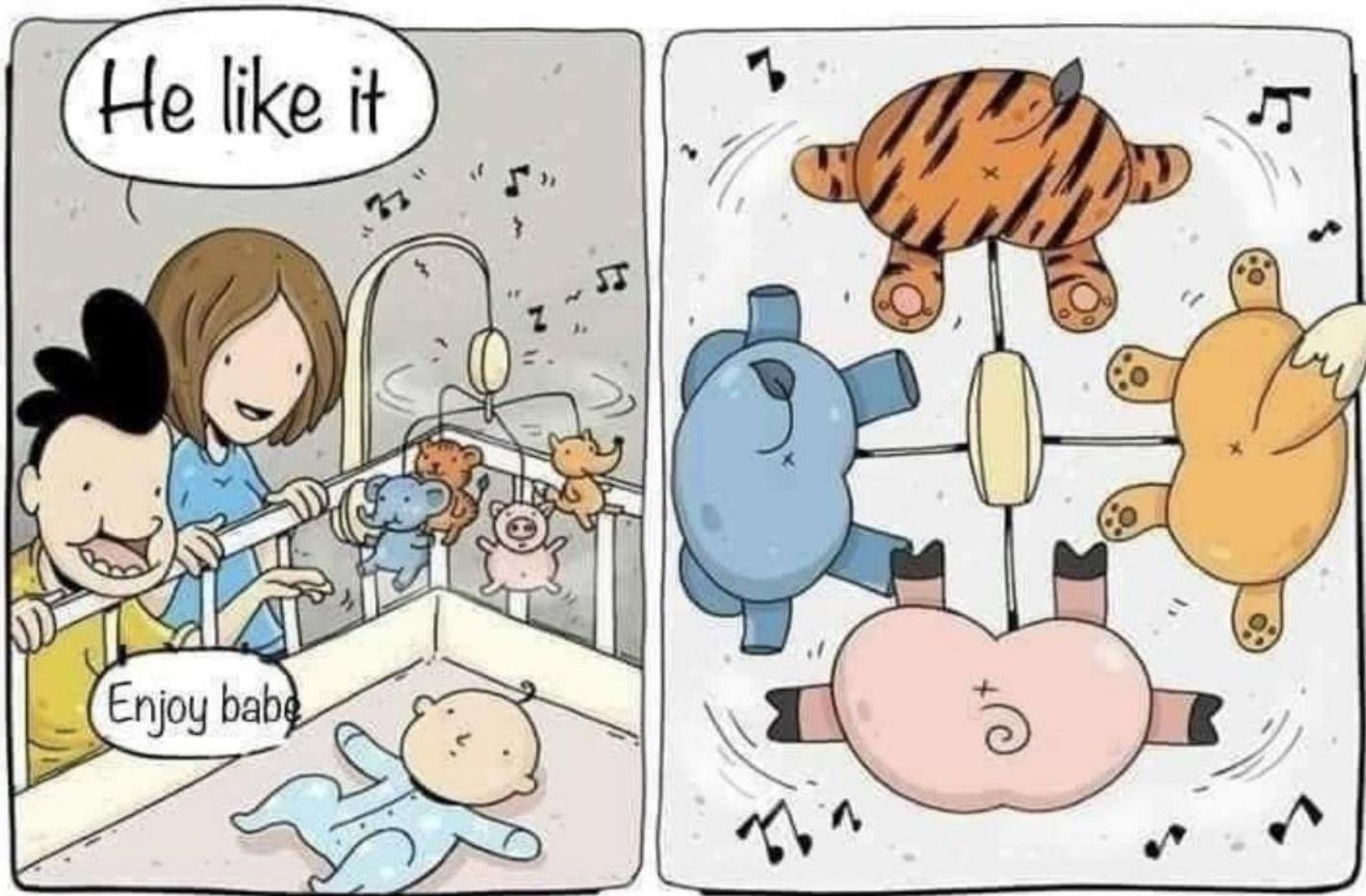
# Why do we need Evidence Based Advocacy?

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ZACK PEMBERTON-WHITELEY

CHIEF EXECUTIVE OFFICER – LEUKAEMIA CARE

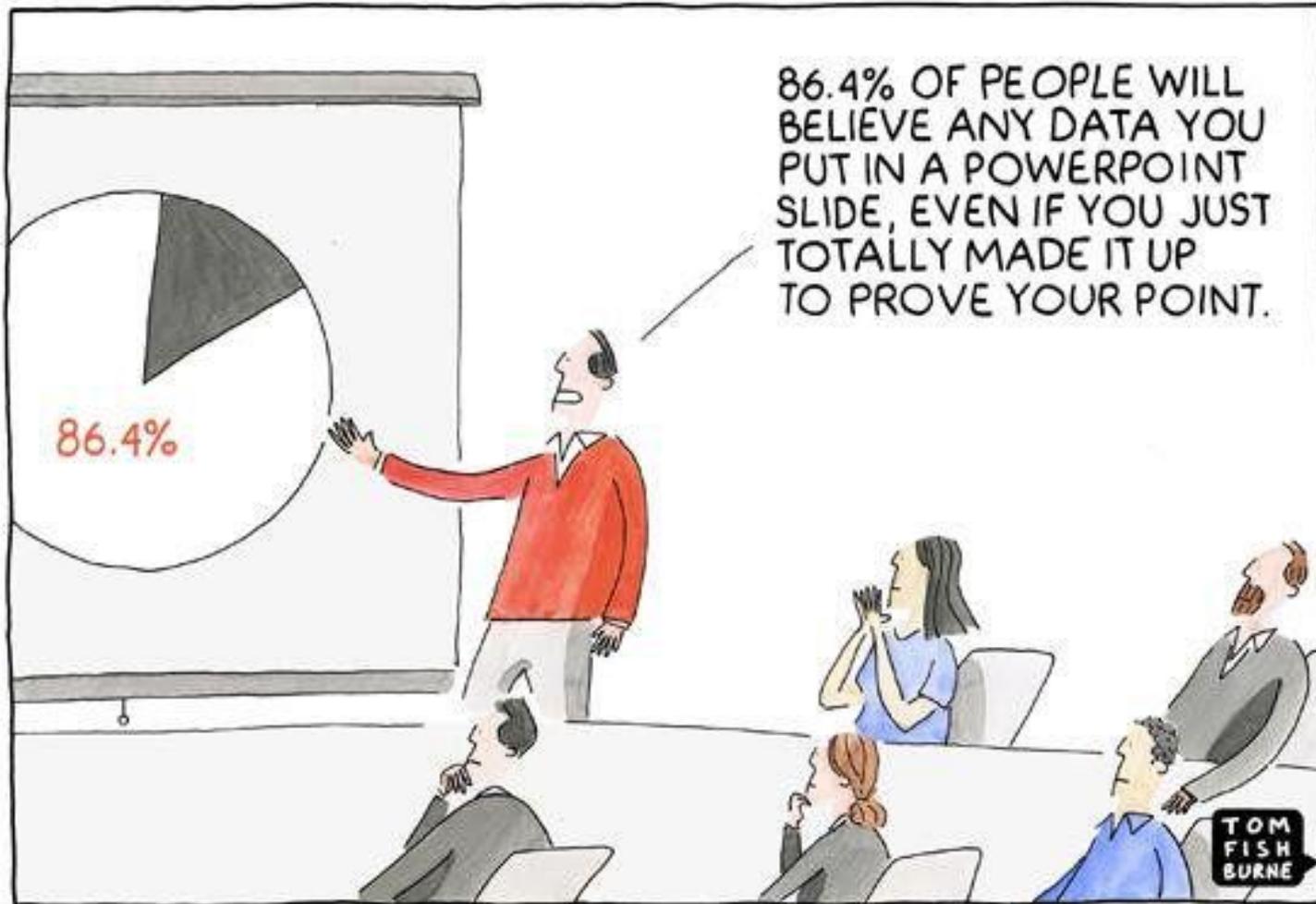
30.10.22 AT CMLHZ22



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Why use  
evidence?

**Understanding**



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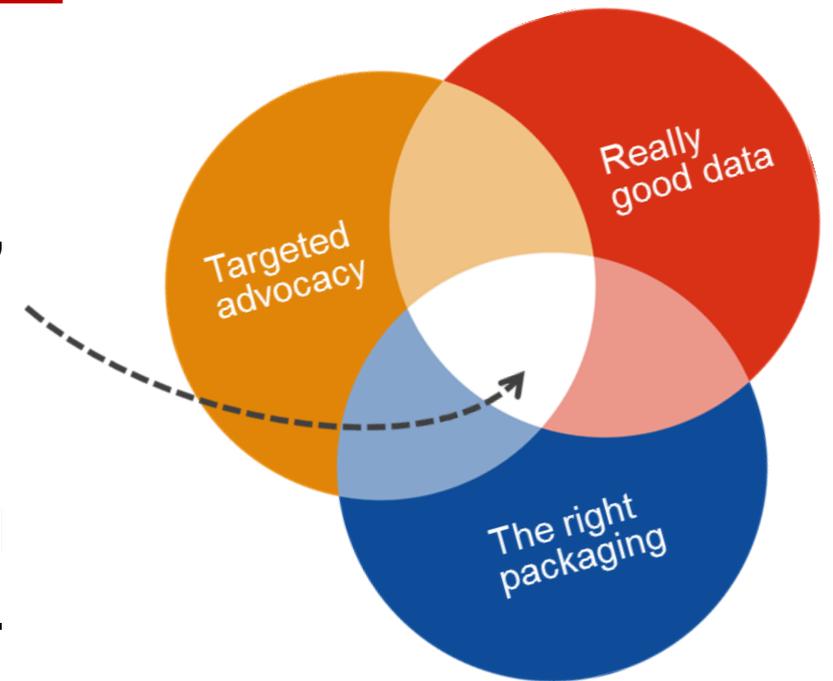
Why use  
evidence?

**Credibility**

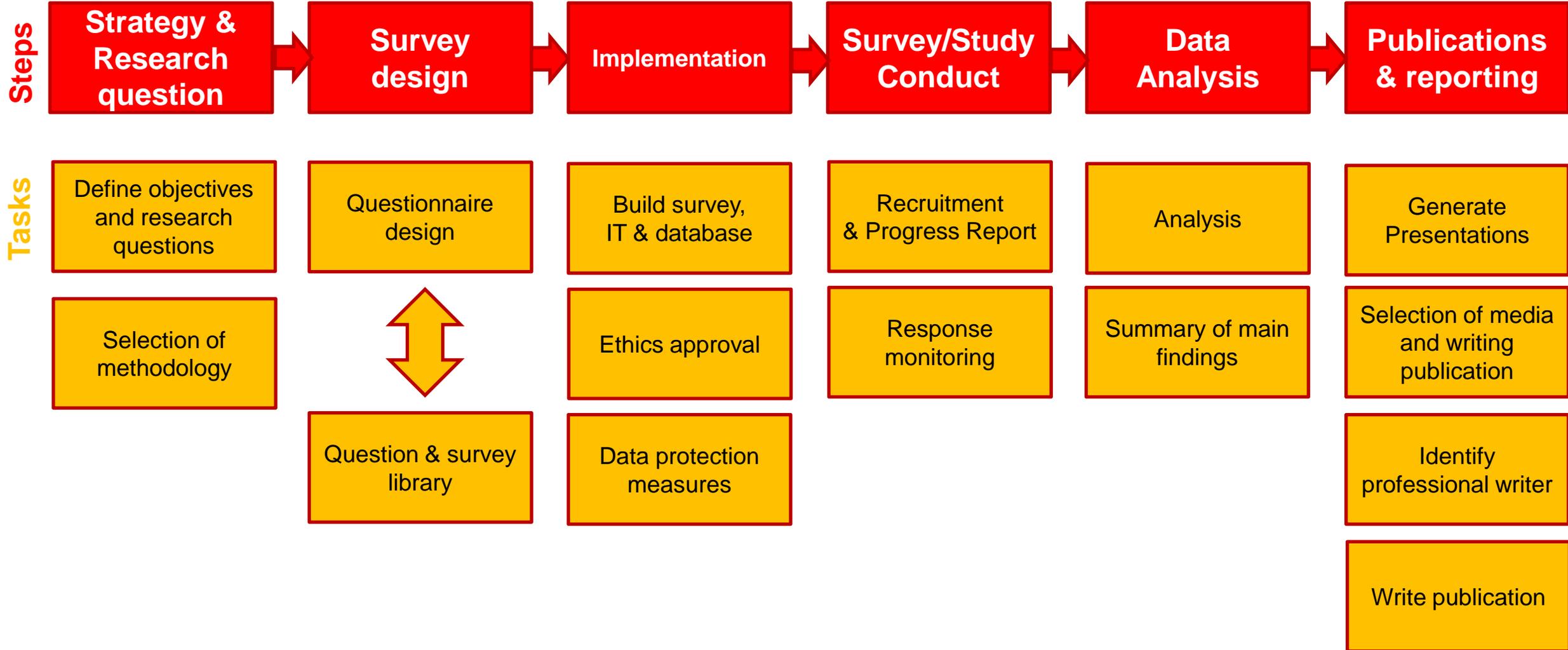
# Evidence-based advocacy – What is it?

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.



# WECAN Training on Evidence Generation



# Example – Acute Leukemia Advocates Network – QOL Survey

- Recent changes in how acute leukemia is treated – lots of new options
- A need for greater understanding and prioritisation of quality of life in acute leukemia
- Poor prognosis (e.g. 5 year OS in AML less than 20%) and an urgent need to improve survival

Table 1: Correlations between HM-PRO scores and Q13-Q18 (patient-reported experience)

part a score	Part A score	Part B score	Q13	Q14	Q15	Q16	Q17	Q18
part a score	1.000							
part b score	0.632	1.000						
q13	0.388	0.307	1.000					
q14	0.481	0.365	0.775	1.000				
q15	0.330	0.470	0.566	0.503	1.000			
q16	0.408	0.312	0.647	0.685	0.498	1.000		
q17	0.685	0.524	0.440	0.440	0.553	0.519	1.000	
q18	0.578	0.433	0.346	0.403	0.519	0.519	0.519	1.000

Figure 1: Boxplots showing the distribution of HM-PRO Part A scores according to the three disease states: in treatment, in remission following treatment, relapsed after treatment.



<sup>1</sup>A small amount of random noise was added to the points for display purposes

**Introduction**  
 One of the most changes in acute leukemia treatment, there is an urgent need for greater understanding of the factors affecting quality of life of acute leukemia patients in the journey of patients.

**Methods**  
 This survey comprises 98 items and was designed based on a literature review of quality of life of acute leukemia patients followed by input from clinical and patient advocacy experts. HM-PRO, an instrument to measure patient-reported outcomes in patients with hematological malignancies, was incorporated into the survey for measurement of quality of life and symptoms. This is a composite measure consisting of Part A (Impact/Quality of Life) and Part B (Signs and Symptoms). Your scores on each part represent your experience with each.

**Questionnaires**  
 Questionnaires are a measure for disease state (undergoing treatment, in remission following treatment or relapsed following treatment).

**Results**  
 There were 373 respondents in the survey and 200 completed the survey.

Response Category	Count
Age of respondents and age of illness	13
Gender	14
Acute leukemia type	2
Acute leukemia treatment	15
Quality of life from impact/quality of life	17
Quality of life from signs and symptoms	18

There was no evidence of any differences in the HM-PRO scores for Part A – quality of life (Q13-Q18) or Part B – signs and symptoms (Q19-Q24) between acute leukemia types.

## Identifying differences in the quality of life of patients with acute leukemia: a global survey

Zack Pemberton-Whiteley<sup>1</sup>, Jan Geisler<sup>2</sup>, Sophie Wintrich<sup>3</sup>, Esther Oliva<sup>3</sup>, Sam Salek<sup>3</sup>, Tatyana Ionova<sup>3</sup>, Rosemary Tate<sup>3</sup>, Jennie Bradley<sup>3</sup>

<sup>1</sup> - Acute Leukemia Advocates Network; <sup>2</sup> - HM-PRO; <sup>3</sup> - Quality Health Limited

Acute leukemia patients are more likely to have a reduced quality of life if they:

- are undergoing treatment
- are female
- report a worse patient experience.

4785 - 61<sup>st</sup> American Society of Hematology Conference - #ASH19

**Results**  
 Of these 373 were men and 233 female and 3 provided no gender. The median Part A score for males and females was 24.5 and 23.0, respectively (p=0.27). The median Part B score was 23.5 and 23.5 for males and females, respectively (p=0.92) with females reporting greater burden of signs and symptoms.

The analysis suggests that there is a difference in the HM-PRO scores between disease states, with those in remission following treatment having lower HM-PRO scores (better quality of life) than those currently undergoing treatment.

**Figure 1: Boxplots showing the distribution of HM-PRO Part A score according to the three disease states: in treatment, in remission following treatment, relapsed after treatment.**

**Figure 2: Boxplots showing the distribution of HM-PRO Part B score according to disease state.**

**Conclusion**  
 Based on this survey data, the study evidences that acute leukemia patients in remission report a better quality of life than those currently undergoing treatment. However, there is no evidence of any difference in the HM-PRO scores between acute leukemia types which needs further exploration in dedicated studies. In terms of gender differences, the results indicate that female patients experience greater impact on signs and symptoms. Furthermore, patients with worse reported experience in each category, Q13-Q18 have a lower overall quality of life, suggesting that reporting impact in these areas may enhance overall quality of life.





## New Insights from the ALAN Survey: Quality of Life (QoL) in acute leukemia

**Zack Pemberton-Whiteley**  
Chair  
Acute Leukemia Advocates Network  
Scientific Working Group on QoL and Symptoms



## GLOBAL QUALITY OF LIFE SURVEY

A survey of patients to understand their experiences, key issues, and unmet needs throughout their journey with acute leukemia.

Pemberton-Whiteley et al, Identifying Factors Associated with Variations in Quality of Life for Patients with Different Acute Leukemia Types: A Global Survey, Poster presented at European Hematology Association 2020

**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, but not limited to: experiences through patient journey how it varies among types of leukemia, the QoL and impact of leukemia on daily life, and patient information needs that were 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 gain insight into understanding into the patient's experience, rather than the clinical perspective. It does not seek to replicate the formal collection of scientific data such as phase 4 trials.

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 living situation 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 newsletters, emails, and social media channels. Participation was on a voluntary basis and therefore may not reflect the perspectives of all leukemia patients.

**Results**

There were 2646 responses to the survey:

Leukemia Type	Patients	%
Acute Myeloid Leukemia (AML)	312	12
Acute Lymphocytic Leukemia (ALL)	104	4
Chronic Lymphocytic Leukemia (CLL)	202	8
Chronic Myeloid Leukemia (CML)	896	34
Chronic Neutrophilic Leukemia (CNL)	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 (n=1486), 66% were living in a household with 1-2 people, and living situation, employment status and income varied.

Responses were collected from 10 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<sup>1</sup>; Samantha Nier<sup>1</sup>; Nick York<sup>2</sup>; Deborah Baker<sup>2</sup>; Michael Rynne<sup>2</sup>; Nicole Schroeter<sup>2</sup>; Denis Costello<sup>3</sup>; Lidija Pecova<sup>3</sup>; Esther Nathalie Oliva<sup>4</sup>; Tatyana Ionova<sup>4</sup>; Sam Salek<sup>4</sup> and Jennie Bradley<sup>5</sup>

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 - 64<sup>th</sup> ASH Annual Meeting and Exposition - #ASH22



**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 results were reported while under treatment.

Majority of acute leukemia and CML patients (93% ALL, 87% AML, 93% CLL, 93% CML) started treatment within less than a week after diagnosis, while 10% of CLL patients waited over 2 years to start treatment. 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 option at diagnosis (n=1271, [65% ALL, 47% AML, 43% CLL, 55% CML]) were not involved as much in decision-making about their treatment as they wanted to be. 60% (n=1225, [52% ALL, 41% AML, 54% CLL, 70% CML]) were offered the option of participating in a clinical trial, and of these 43% (n=523, [32% ALL, 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 (51% ALL, 51% AML, 51% CML) reported that their side effects were "barely noticeable" on their QoL, while acute leukemia patients (51% ALL, 44% AML, 44% CLL, 44% CML) 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 "extremely worried").

Patients reported similar physical, cognitive, social well-being and emotional behavior, however, more than 50% of 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, 75% AML, 75% CLL, 75% CML) with a greater financial impact.

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

**Conclusion**

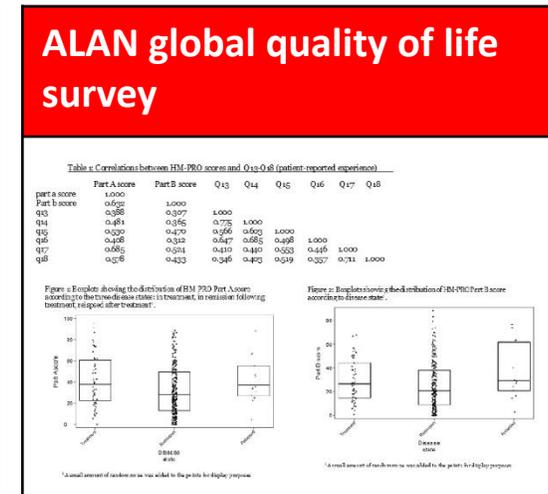
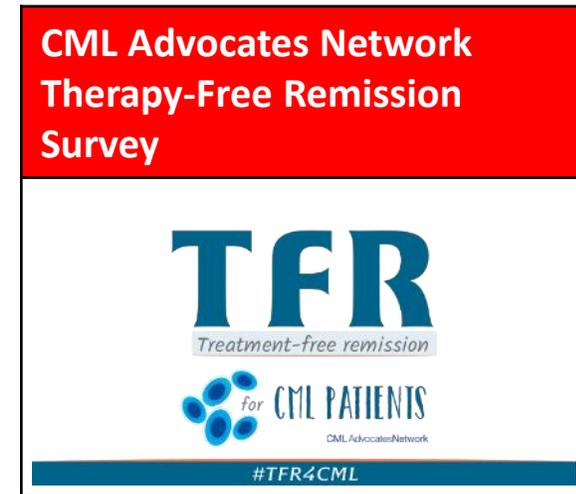
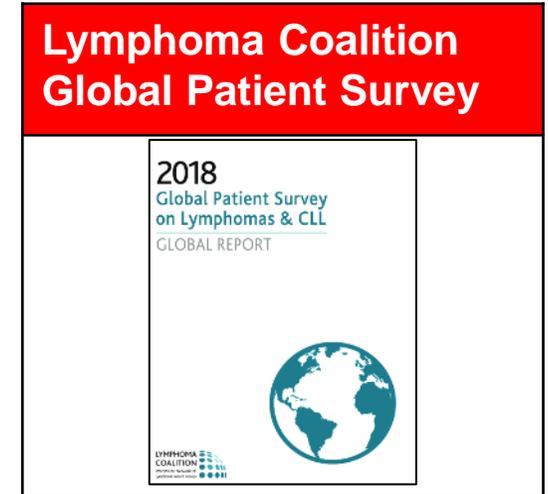
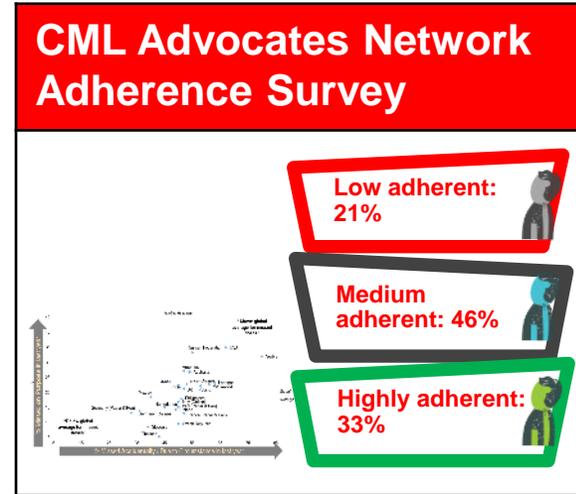
We believe this survey was a valuable tool for gathering information about the experiences and preferences of leukemia patients.

Although the survey 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. Differences are observed but they can be explained by the nature of the disease, the current 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.

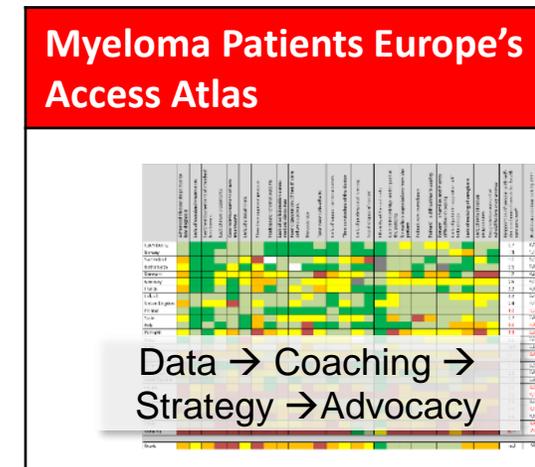
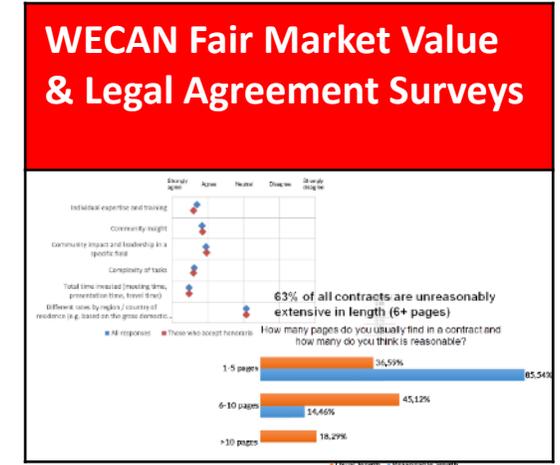
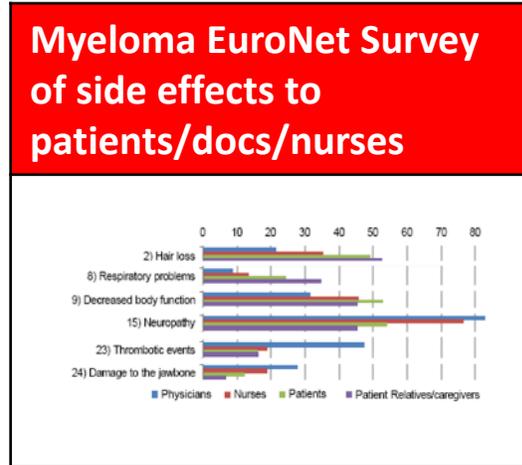
# Disease Mapping

- Most types of evidence generation done by patient advocacy groups involve disease mapping (in different forms)
- Often used to look at areas such as:
  - Patient experience
  - Quality of life
  - Burden of disease and Unmet Needs
  - A specific issue – e.g. adherence



# Generating data on inequalities

- Can be done in a variety of ways. This may involve desk based research or be collected from different stakeholders (e.g. surveys or interviews)
- Evolution of disease mapping into the more complicated ‘inequality mapping’ – looking for subgroups with differences (e.g. worst outcomes, lowest access)
- Often looked at by demographic (e.g. country, age, gender, or by a specific category)



# Advocacy Purpose

## 1. What is your desired advocacy impact?

- What are the priority questions for your organisation that you think need addressing?
- What are the 'hot topics' in your cancer? Is the patient perspective well understood by other stakeholders?
- For each topic - work out what the real problem is – and what you need to do to address this
- Don't run a survey without a specific advocacy purpose!

## 2. What do you want to show, and to whom?

- Focus on your audience – tailor evidence and message
- What do they already know? What do you want them to know?
- Do I need to publish? What are the timelines for this?

Steps

**Strategy &  
Research  
question**

Tasks

Define objectives  
and research  
questions

Selection of  
methodology

# What evidence do you need?

- We're 'advocates not academics' – only collect evidence because we want to **do something with it**
- Evidence generation is costly and takes a lot of time
- Ideally - collect the minimum amount of data needed

## Ask yourself:

- What am I trying to achieve?
- What evidence do you need to back this up?
- What is already known and what do I need to collect?
- What types of evidence (and outputs) will be most effective with your intended audience (e.g different stakeholder groups)?

Steps

Strategy &  
Research  
question

Tasks

Define objectives  
and research  
questions

Selection of  
methodology

# What is already **known**?

## 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

	Known (YOU)	Not Known (YOU)
Known (OTHERS)	EXISTING EVIDENCE	DESK RESEARCH
Not Known (Others)	QUANTITATIVE	QUALITATIVE

# Case Study: Living with leukaemia

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LEUKAEMIA CARE

# Project Overview



Aims – Exploring UK leukaemia patient experience



Topics – Following the journey from diagnosis onwards



Testing – To refine the content



Comparability – between annual versions



## Patient Survey

This questionnaire is about your care and treatment for a blood cancer. Its purpose is to provide information, which can help the NHS and Leukaemia CARE monitor and improve the quality of health services for future patients with blood cancer.

**Taking part in this survey is voluntary. Published reports will not contain any personal details.**

### Who should complete the questionnaire?

The questions should be answered by you, as the person who has been treated for a blood cancer. If you need help to complete the questionnaire, the answers should be given from your point of view – not the point of view of the person who is helping.

### Completing the questionnaire

For each question please tick clearly inside the box that is closest to your views using a black or blue pen. Don't worry if you make a mistake; simply cross out the mistake and put a tick in the correct box.

### IMPORTANT INFORMATION

To make sure the information we collate is useful, we need to collect some personal details from you and access information held about you in other NHS databases. The purpose of collecting this information is to generate aggregated statistics about the care and treatment people receive. These statistics will be used to compare the differences in care and treatment by different providers and to understand what may be causing these. The results will be used to measure and improve the quality of healthcare services.

By completing this questionnaire you are giving your consent for the information provided to be used for the above purposes. Specifically, you are agreeing that:

- Your personal details and relevant health information can be held and used by an organisation contracted to Leukaemia CARE to analyse the data.
- Your personal information will be handled securely and anonymised after analysis and before any publication.
- Your personal information will not be released by anyone working on behalf of Leukaemia CARE unless required by law or where there is a clear overriding public interest.
- You can withdraw the information you give Leukaemia CARE in this questionnaire upon request, up to the point at which data are analysed and personal details removed.

If you have any queries about the questionnaire, please call the FREEPHONE helpline number on 0800 783 1775

LE17CORE

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# Different output types

Reports and Presentations

Scientific Publications

As a starter for follow-up projects...

**Leukaemia Care**  
YOUR Blood Cancer Charity

## Leukaemia Care Living with Leukaemia

2018 Report



www.leukaemiacare.org.uk



### Leukaemia Care The Emotional Impact of Watch and Wait for CLL

YOUR Blood Cancer Charity

**Background:** A cancer diagnosis can have a significant impact on a patient's mental health, watch and wait being no exception. For CLL patients, who have been identified as a particular source of distress for CLL patients, there are at least 100,000 conducted interviews with CLL patients and their families to explore their experience of living with CLL. The survey was designed to explore a range of issues that patients and their families face, including a section on watch and wait. The survey was conducted in a paper questionnaire or via an online link. 102 CLL patients responded to the survey. To the best of our knowledge, this is the largest ever CLL patient survey to be conducted in the UK.

**Aims:** Leukaemia Care conducted a survey of CLL to identify and quantify the issues CLL patients face, particularly on watch and wait (WW).

**Conclusion:** This survey highlights areas of need to reduce the psychological impact of a CLL diagnosis. There is a need to improve communication at diagnosis, including a tailored explanation of CLL and the rationale for WW, providing high quality written information to take away. The findings suggest that patients on WW need support, such as CDS access, as much as those starting treatment.

**Key findings:** 65% of all respondents reported that they were emotionally affected by their diagnosis. However, when groups of CLL patients are more likely to be emotionally affected by their diagnosis than other groups (e.g. CLL patients who were not on WW).

**Results:** Understanding CLL and watch and wait: 65% of all respondents reported that they were emotionally affected by their diagnosis. However, when groups of CLL patients are more likely to be emotionally affected by their diagnosis than other groups (e.g. CLL patients who were not on WW).

**Method:** The survey was conducted in a paper questionnaire or via an online link. 102 CLL patients responded to the survey. To the best of our knowledge, this is the largest ever CLL patient survey to be conducted in the UK.

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### Leukaemia Care The Financial Impact of Acute Leukaemia

YOUR Blood Cancer Charity

**Background:** The financial impact of cancer is well documented in the "Survival" Commissioned by UK cancer charity. It has found that 65% of patients are affected with an average of a £1,000 per month. The financial impact of cancer is well documented in the "Survival" Commissioned by UK cancer charity. It has found that 65% of patients are affected with an average of a £1,000 per month. The financial impact of cancer is well documented in the "Survival" Commissioned by UK cancer charity. It has found that 65% of patients are affected with an average of a £1,000 per month.

**Aims:** Leukaemia Care surveyed acute leukaemia patients, to identify key concerns and issues faced throughout their cancer journey, including the financial impact.

**Summary/conclusion:** Most acute leukaemia patients report experiencing financial issues and often must give up work, either permanently or temporarily, which could also impact on their financial situation for years to come. Not all patients are receiving information to help them find financial assistance. The support and advice given by hospitals about the financial impact of leukaemia needs to improve, incorporating signposting to specialist financial support. In addition, further work could be done to determine whether the patients who do seek assistance get help that is adequate for their needs. Financial advice is an additional stress for cancer patients that needs to be addressed by charitable organisations and policy makers alike.

**Key findings:** 65% of all respondents reported that they were emotionally affected by their diagnosis. However, when groups of CLL patients are more likely to be emotionally affected by their diagnosis than other groups (e.g. CLL patients who were not on WW).

## Leukaemia Care Living with Leukaemia

YOUR Blood Cancer Charity

**Background:** The Cancer Patient Experience Survey (CPEX) is commissioned by NHS England annually. However, what remains different from other surveys, including the fact that scores are available for individual sites. The survey is conducted in a paper questionnaire or via an online link. 102 CLL patients responded to the survey. To the best of our knowledge, this is the largest ever CLL patient survey to be conducted in the UK.

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**Introduction:** The implications of UK patient opinions about treatment options for CLL.

**Patients want a choice of treatment and are supportive of new options offering a treatment-free period, but require the support for their individual needs and concerns.**

**Background:** Leukaemia Care conducted a survey of CLL patients to explore their experience of living with CLL. The survey was designed to explore a range of issues that patients and their families face, including a section on watch and wait. The survey was conducted in a paper questionnaire or via an online link. 102 CLL patients responded to the survey. To the best of our knowledge, this is the largest ever CLL patient survey to be conducted in the UK.

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+ Journal articles

**Leukaemia Care**  
YOUR Blood Cancer Charity

**Leukaemia Care  
Living with Leukaemia**

2018 Report



[www.leukaemiacare.org.uk](http://www.leukaemiacare.org.uk)

**NICE**  
National Institute for  
Health and Care Excellence



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How can patient groups use EBA in their activities?

EXAMPLE FROM LEUKAEMIA CARE (UK)

