



VII Simposio anual GELMC

Perspectiva de los pacientes y calidad de vida (Patients perspective & Quality of Life)

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LeukaNET / CML Advocates Network – Scientific Project Manager

24/02/2023



Leukämie-Online e.V. / LeukaNET is an independent and vital online community for leukaemia patients in German-speaking countries.

- Translating and reporting news
- Provides background information on different forms of the disease, treatment options and other relevant information
- Discussion forums and webinars
- A Web Links Database that contains references to relevant information on CML in English language on the internet.

LeukaNET collaborates very closely with patient organisations all across the world, being also a co-founding member of the **CML Advocates Network**

Leukämie online.de
Die Online-Gemeinschaft zum Thema Leukämie

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Willkommen bei Leukämie-Online!

Leukämie-Online ist eine unabhängige, deutschsprachige Wissens- und Kommunikationsplattform zum Thema Leukämie. Diese wird von Leukämiepatienten betrieben und ist gemeinnützig. Das Angebot fördert aktive, informierte und selbstbestimmte Patienten durch umfangreiche Informationen über Neuigkeiten und Hintergründe zur Forschung und Behandlung von Leukämien. Interaktive Foren ermöglichen zudem den direkten Erfahrungsaustausch.

CML-Online-Patientenseminar: "Neues vom ASH aus New Orleans: CML-Therapie 2023 - The Big Easy" – Ihr könnt Euch jetzt anmelden!

Vom 02.02.2023, 15:30 | Von Stefan Huber | Drucken

Unter dem Titel „Wissenshorizonte – aktuelle Perspektiven auf ein Leben mit CML“ führen die Deutsche CML-Allianz und LeukaNET e.V. eine monatliche, deutschsprachige Online-Seminarreihe für CML-Patient*innen und Angehörige durch. Die Teilnehmer haben darin die Gelegenheit, direkt von CML-Expert*innen und CML-Patientenvertreter*innen zu lernen und Fragen zu stellen.

Im Online-Seminar für CML-Patient*innen und Angehörige am **28.02.2023** von 17:15-18:30 wird **Prof. Dr. Andreas Hochhaus** zum Thema **"Neues vom ASH aus New Orleans: CML-Therapie 2023 – The Big Easy?"** sprechen und dabei entsprechend Aktuelles, Relevantes und Neues zur CML-Therapie von der letztjährigen 64sten ASH Annual Meeting & Exposition vorstellen und einordnen. Das Seminar wird moderiert von Jan Geißler, 1. Vorsitzender LeukaNET e.V.

Neues vom ASH aus New Orleans: CML-Therapie 2023 - The Big Easy?

Prof. Andreas Hochhaus
28. Februar 2023

Online-Patientenseminar über "Perspektivwechsel: vom Paralympics-Traum zur CML-Therapie in Pandemiezeiten": Aufzeichnung nun abrufbar

Vom 20.01.2023, 07:03 | Von StefanH | Drucken

Im Online-Patientenseminar von LeukaNET und der Deutschen CML-Allianz am 19.01.2022 sprach **Benjamin Lenatz**, Paratriathlet und CML-Patient, zum Thema **"Perspektivwechsel: vom Paralympics-Traum zur CML-Therapie in Pandemiezeiten"** sprechen und ging dabei auf eine Reihe verschiedener Aspekte ein, unter anderem die Doppelbelastung durch Querschnittslähmung und CML, seinen Weg vom Leistungssport zum Gesundheitssport, Bewältigungsstrategien und -mechanismen, Routinen und Verhaltensmuster, die er für sich gefunden hat, und welche Strategien sich hier möglicherweise auch für Nicht-Athleten eignen, aber auch Themen wie etwa Erwerbsminderungsrente oder die Verträglichkeit von Asciminib im Vergleich zu anderen TKIs. Das Seminar wurde moderiert von Jan Geißler, 1. Vorsitzender von LeukaNET e.V.

Die Aufzeichnung des Seminars ist nun hier abrufbar – einfach links unten auf die Wiedergabe-Taste klicken und den Ton aufdrehen!

Online-Seminarreihe für CML-Patienten und Angehörige:
"Wissenshorizonte: aktuelle Perspektiven auf ein Leben mit CML"

**Perspektivwechsel:
vom Paralympics-Traum zur CML-Therapie in
Pandemiezeiten**

Benjamin Lenatz
19. Januar 2023

1:00:27 **Leukämie**

*Working together
to find a life
without CML*

GET INVOLVED



Welcome to the CML Advocates Network

A network of 128 chronic myeloid leukemia (CML) patient organizations from 93 countries on all continents, the CML Advocates Network serves as a resource for leaders of CML patient groups. As a patient and caregiver-led organization, it was established and is run by patients and caregivers. The organization aims to educate members about advocacy and health equity, empower patient groups to advocate, and implement advocacy initiatives in both low-middle-income (LMI) and western countries. It also aims to facilitate best practices sharing among patient advocates globally.

During the 4th International "New Horizons in Cancer" conference in Dublin in 2005 for patient advocacy groups fighting CML and GIST, the idea for the CML Advocates Network was initially discussed. "New Horizons" conferences originated in 2002 and have become one of the world's most influential networking and knowledge-sharing events for patient groups dealing with CML and GIST.



CML Advocates Network (CMLAN) is an international organization that connects 128 CML patient organizations from 93 countries on all continents, serving as a resource for leaders of CML patient groups. The organization aims to:

- Educate members about advocacy and health equity
- Empower patient groups to advocate
- Implement advocacy initiatives in both low-middle-income (LMI) and western countries
- Facilitate best practices sharing among patient advocates globally.

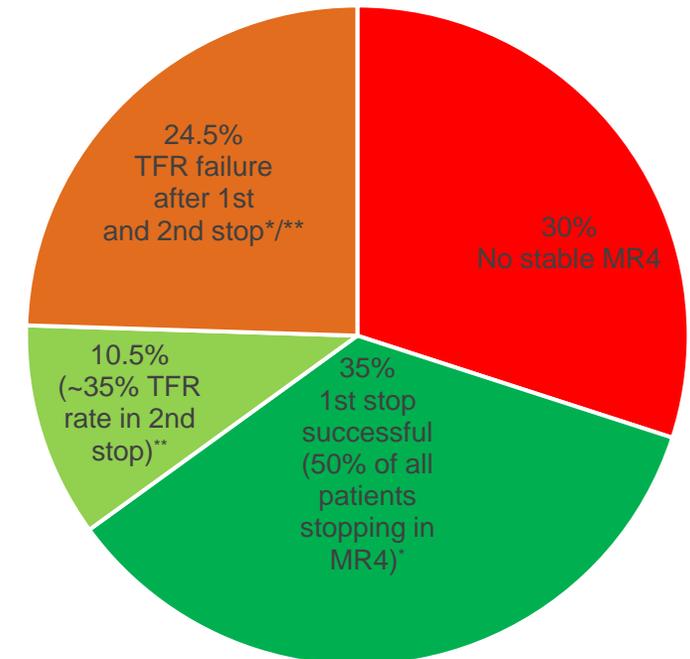
Our mission to improve the lives and survival of all people affected by CML, by empowering advocates and driving world-wide change in access, knowledge, diagnostics, treatment, and care.

The CML patient community: perspective and needs

- Patients need and want good research, based on best possible evidence
- Patient involvement from the first steps of Research and Development and all phases of clinical trials
- Recognition that cure is still an unmet need in CML, not a done deal with TFR
- Commitment and collaboration between all stakeholders, including the patient community and CML researchers working together to find a real cure for CML
- Need of a joint definition of the problem that we are trying to solve:
 - All CML patients TFR
 - can live a normal, long life TFR
 - with equivalent quality of life of a normal person TFR
 - in absence of any CML disease TFR
 - without need of any CML therapy TFR
 - without need of continuous monitoring TFR

TFR for CML patients

- With TKI/STAMP and best available care, patients have achieved good and long life, however not a completely normal life
 - Still is a chronic cancer with constant monitoring
 - Many therapies with significant side effects which bother in the long run
 - Patients are still dying on progression or bad care
- Approximately 40-60% of patients who discontinue TKI therapy after achieving DMR experience a recurrence within 12 months of stopping treatment, in some cases as early as one month into discontinuation.
- We need more curative therapies and good **quality of life** for those that cannot achieve TFR



TFR failure and success

* TFR failure rates vary from 45% to 65% in various STOP studies, e.g. STIM, EUROSKI, DASFREE, ENESTop, ENESTFreedom.

** RE-STIM, NAUT

Importance of Quality of Life Data

Quality of life (QoL) comprises more than just the economic output of a country and living standards of people. It includes a wide range of factors that influence what people value in life, beyond material characteristics.

When treating patients, clinicians should keep in mind the importance of social functioning and connect patients to resourceful and applicable support groups.

Do we really know how well patients feel under current long-term therapies?

We need to have:

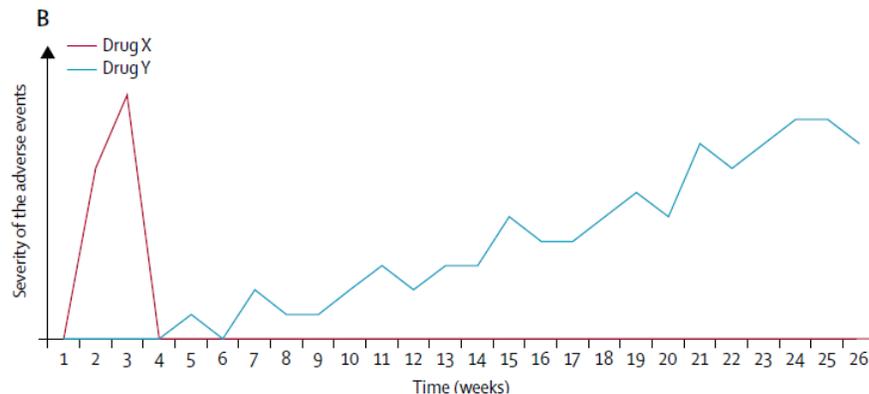
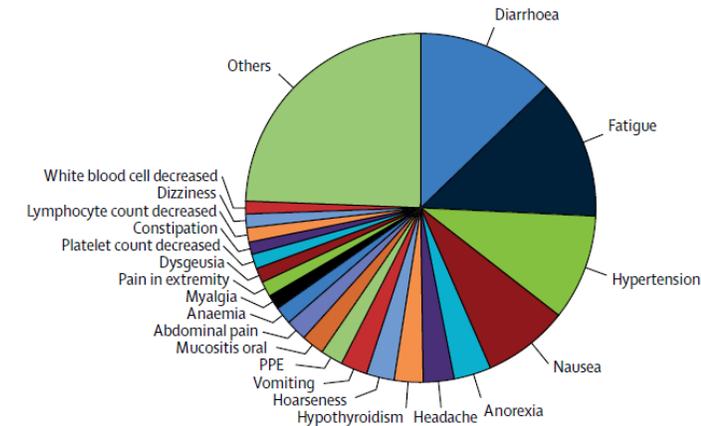
- Solid data on incidence, severity and trajectory of side effects and other QoL impact factors
- Shared information of patients with very bothersome side effects and multi-intolerance
- Patients understanding of underlying mechanisms (therapy choice, side effect management)
- Incorporation of PROs into current clinical safety and treatment information



Limitations of current AE reporting in Clinical Trials

Going beyond maximum grade

- Many times interesting HRQoL data is collected but published in a minimal way
- Usually end point/one point toxicity are presented as a summary table of the high-grade toxicity. But that does not tell anything about chronicity, transience and cumulative effects
- Nothing on toxicity over time, toxicity progression or long term follow-up affecting the description of tolerability in retrospective cohorts.
- Barely any published data on duration and trajectory of side effects and quality of life over time
- Establishment of longer-term post-marketing surveillance or registries is vital



Longitudinal graphs of the prevalence of specific AE (toxicity over time) would provide more information on the development of AEs. How they arise, whether they become cumulative, or resolve with: supportive care, dose modification, or therapy cycle or course.

PROs used to measure QoL: Issues encountered in clinical studies

- Little data collected or poor quality of data
- Little use of PROMs or issues in choosing one

PRO measurement in most hematology trials is insufficient

- PRO assessments provide important complementary information from the patient's perspective on functional outcomes and the trajectory of symptoms over time
- PRO version of the Common Terminology Criteria for Adverse Event (PRO-CTCAE) allows for self-reporting of symptomatic adverse events, mapping to the well established CTCAE system for clinician reports. No QoL
- Use of PROs in hematology trials is poor

	Phase 1	Phase 1/2	Phase 2	Phase 3	Other	Pilot	Total
Leukaemia trials	1/49 (2%)	0/7	2/68 (29%)	5/11 (50%)	0/1	0/1	8/137 (6%)
Lymphoma trials	0/23	1/11 (9%)	6/63 (10%)	1/3 (33%)	..	0/1	8/101 (8%)
Myeloma trials	0/5	1/4 (25%)	2/13 (15%)	7/11 (64%)	..	0/2	10/35 (29%)

Data are number of trials with PROs/total number of trials (%). CTEP treatment trials (all phases) were activated between June 30, 2004, and Dec 31, 2016.

In total, only 26 (10%) of 273 adult haematology clinical trials sponsored by the National Cancer institute had PROs. CTEP=Cancer Therapy Evaluation Program.

Table 3: Patient-reported outcomes (PROs) in 273 haematology adult trials sponsored by the National Cancer Institute

PROs used to measure QoL:

Why some PROMs in CML are more helpful than others?

There are generic and condition-specific PROMs. Both have advantages and disadvantages, and therefore need to be combined for a better outcome

PROMs allow healthcare professionals to provide more patient-centred care, and to be informed about the patient's experience with their disease. It creates:

- an environment where patients can be treated based on their personal experiences,
- it facilitates shared decision making,
- and will eventually improve patient outcomes.

	Advantages	Disadvantages
Generic PROMs	<ul style="list-style-type: none"> • Easy to compare across conditions and health systems 	<ul style="list-style-type: none"> • Generic, thus less clinical details
Condition-specific PROMs	<ul style="list-style-type: none"> • More clinical detail 	<ul style="list-style-type: none"> • Higher assessment burden • Difficult to compare across conditions

Moving towards patient-centered decision-making in chronic myeloid leukemia: assessment of quality of life and symptom burden.
Baccarani et. al., Haematologica. 2014

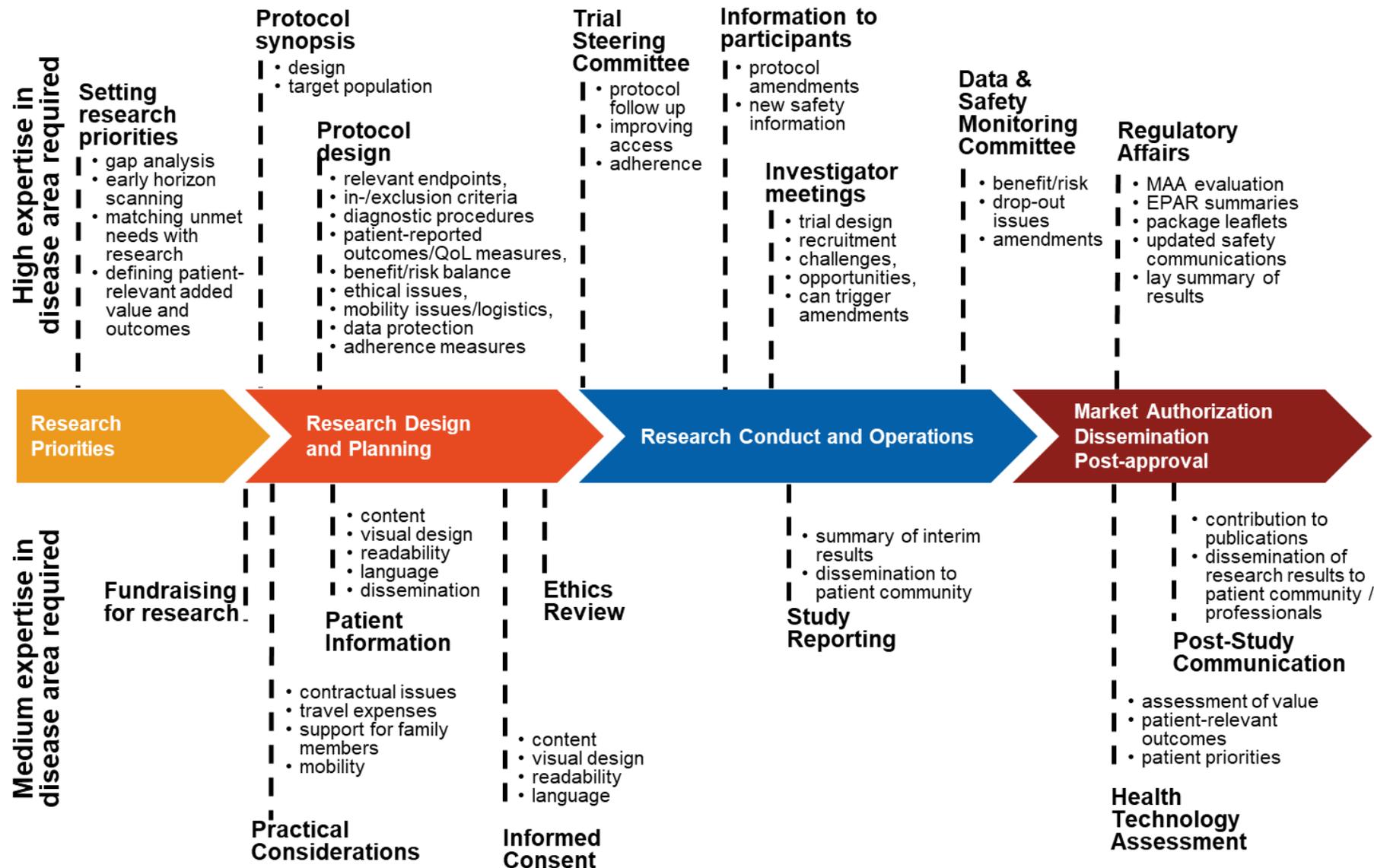
Questionnaire	Main purpose	N. items	Time recall	Domains/ scales measured	Scoring and Interpretation
EORTC QLQ CML-24* Website for requesting permission to use: http://groups.eortc.be/qol/modules-development-and-available-use	Assess Quality of Life in CML Patients	24	Patients are asked to evaluate their Quality of Life during the last week	-Impact on daily life; -Impact on worry/mood; -Body image problems; -Symptom burden; -Satisfaction with care and information; -Satisfaction with social life	Score ranges between 0 and 100 for all scales (except for two [†]) with higher scores indicating worse outcomes.
MDASI CML Website for requesting permission to use: http://www3.mdanderson.org/depts/symptomresearch/	Assess the severity of symptoms and the impact of these on daily functioning in CML patients	26	Patients are asked to evaluate their symptoms during the last 24 hours	-Symptom severity -Impact of symptoms on daily functioning	Score ranges between 0 and 10; higher scores indicate increased symptom burden

*This questionnaire should be used in conjunction with the EORTC QLQ-C30 to comprehensively assess HRQOL in CML patients. [†]For the following two scales: satisfaction with care and information and satisfaction with social life. Higher score indicates better outcomes.

Importance of patient involvement in R&D

- We are spending billions on developing new treatments in pre-clinical research and clinical trials, and not much effort into generating evidence on how they perform in real world and whether they address patients' true needs
- Decisions are taken on clinical evidence, not on real-world data or patient experience data
- Patients are valuable partners in research as they have unique insights into unmet needs of patients, patients' preferences, their expectation on treatment effect, quality of life and living with cancer.
- Patient organisations can help to frame meaningful research questions, identify relevant variables and data sources, analyse and interpret the data, and disseminate relevant findings in the clinical and patient communities.

Patient involvement in medicines R&D



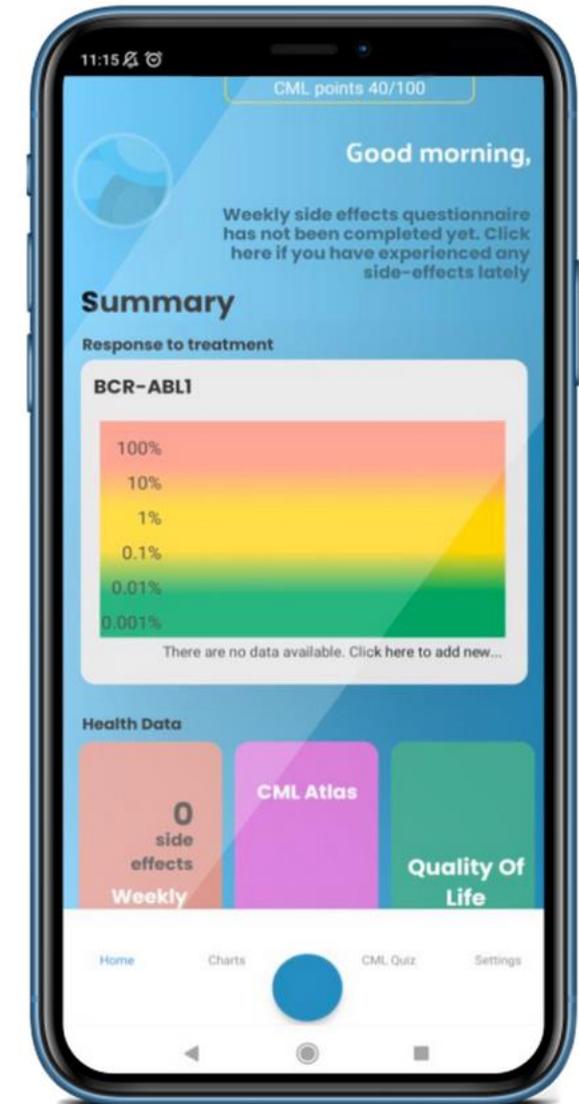
Improving Patient Involvement in Medicines Research and Development
Geissler et al., Ther Innov Regul Sci. 2017.

Current efforts on CML QoL

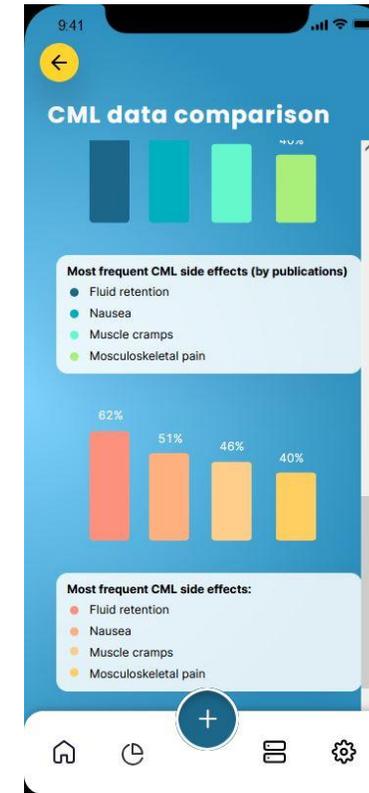
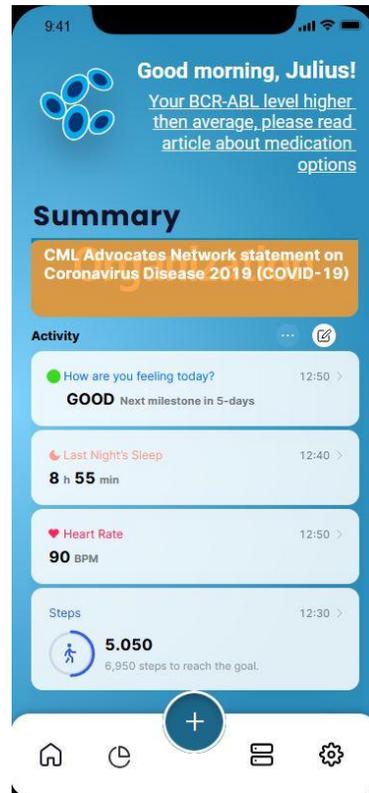
“Know your CML” App & Web

The Know Your CML app offers an abundance of tools that allow you to record and monitor the status of your CML, learn more about the condition, find peer support and share your health data with your doctor and others

- Self-recording of data on lab results and quality of life, based on EORTC-QLQ30+CML24
- Analysis of anonymized experience of all users of the app
- Keep track of your disease course by adding your BCR-ABL1 count
- Generate a health report containing a comprehensive and detailed overview of your health data over any period of time
- The recommendations section provides a wealth of valuable and lay-friendly information on CML, diagnosis and treatment



“Know your CML” App & Web

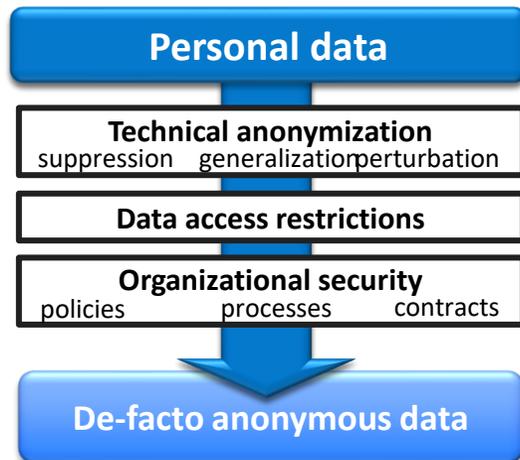


Patient involvement in HARMONY

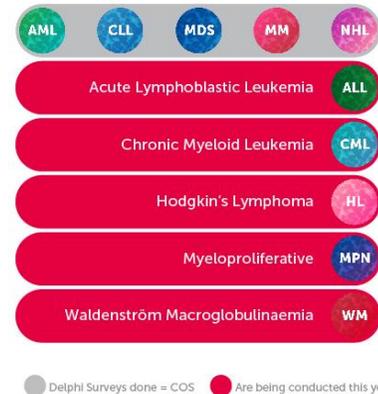


HARMONY Alliance Patient Cluster

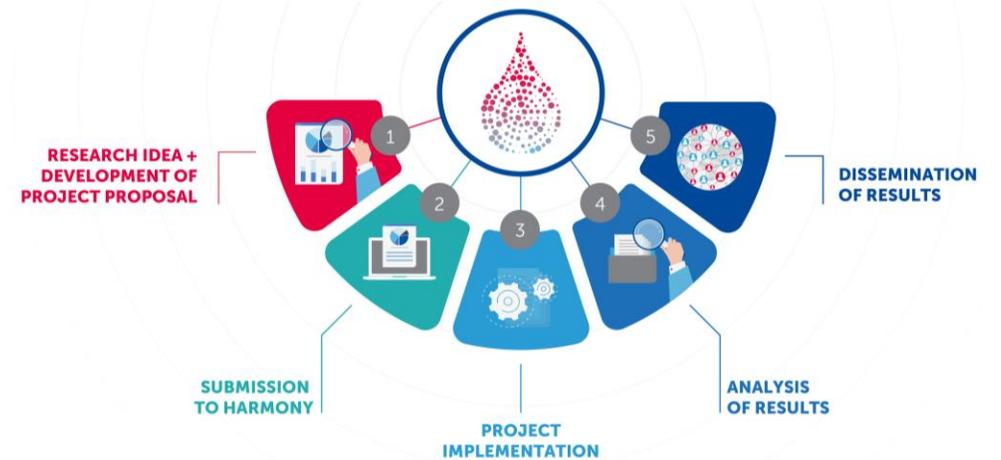
Contribution to de-facto anonymization procedure



Contribution to in Delphi surveys on Core Outcome Sets



Input into HARMONY research projects



Submission of patient-led QoL/Tox research proposals



Patient-led research proposals in CML QoL

- **Prevalence of clinically relevant problems and symptoms in patients with CML**

Describe prevalence of clinically relevant problems and symptoms in patients with CML, analysed by key patients' characteristics and type of treatment.

- **A comparison study of toxicity over time and QoL among all available TKI options**

Despite TKIs have been widely used for more than two decades, there is still little knowledge on time points, transience, and cumulative effects of all available TKIs. This proposes to obtain toxicity and QoL data from patients treated with TKIs considering all stages of the disease and variable therapy doses

- **Correlation of demographic, disease and therapy factors with toxicity and tolerability of the different TKIs**

There is still no accurate system to predict intolerant or non-responsive patients, with high relevance in improving prognostic factors on therapy-related morbidity, tolerability, and quality of life. The approach is to identify significant correlations between demographic, disease and/or therapy-related conditions with toxicity (CTCAE) and tolerability among treated patients

Thanks to our team at LeukaNET, CMLAN and other organizations that support us.

Thanks to CML patients, carers and advocates for their hard work and support.



Backup slides

PROs used to measure QoL: Comparing MDASI vs EORTC PRO in CML

MDASI-CML	EORTC QLQ-C30 + QLQ-CML24	
Symptom burden questionnaire	Quality of Life questionnaire	
Development - 1 country (USA)	QLQ-CML24 Development - 10 countries	
	EORTC QLQ-C30	EORTC QLQ-CML24
<p>Contains 20 symptom items and 6 interference items:</p> <ul style="list-style-type: none"> • General Activity • Work • Walking • Enjoyment of life • Mood • Relationships with other people 	<p>A 30-item questionnaire</p> <p>Contains 9 multi-item scales:</p> <ul style="list-style-type: none"> • Five functional scales (physical, role, cognitive, emotional, and social) • Three symptom scales (fatigue, pain, and nausea and vomiting) • One global health and quality-of-life scale 	<p>A 24-item questionnaire</p> <p>Contains 13 symptom items and 11 interference items:</p> <ul style="list-style-type: none"> • incl also hair loss, sweating, eyes • Impact on worry/mood (4 items) • Impact on daily life (3 items) • Body image problems • Treatment burden • Satisfaction with care and information (2 items) • Satisfaction with social life
Total of 26 items	Used in combination EORTC QLQ-C30 + QLQ-CML24. Total of 54 items	

Patient involvement at EMA happens in a consistent structure with a pool of approved patient experts.

Patient Involvement at the EMA

