

CML Advocates Network
Community Advisory Board

Update on CML Community Advisory Boards

**CML Horizons 2021, Jan Geissler
on behalf of the CAB co-chairs Pat & Jan, and CML-CAB members**

Why CML Community Advisory Boards?

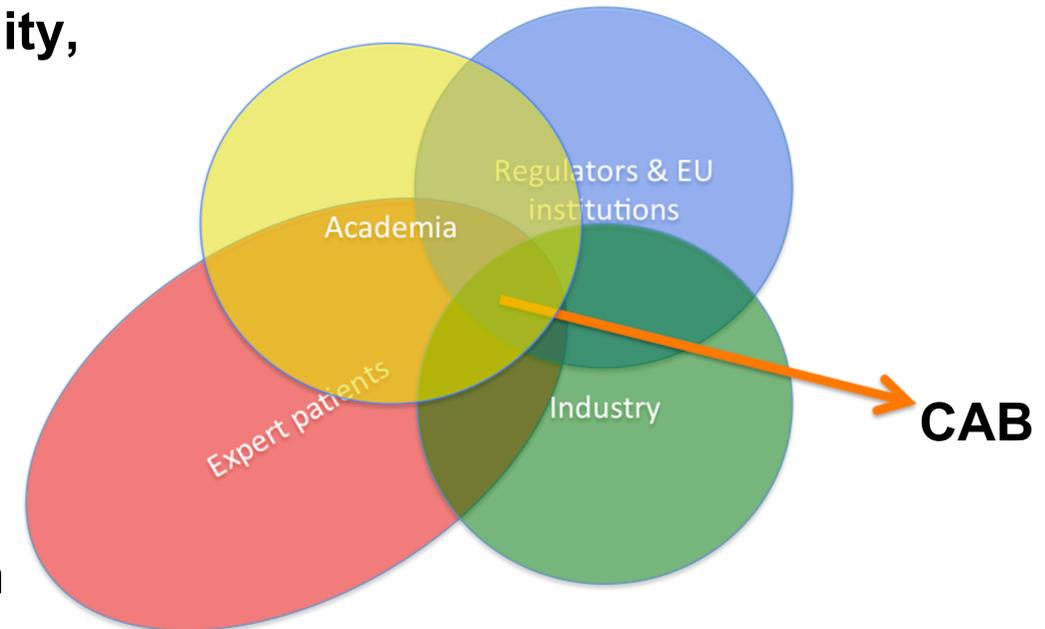
- **Research is key towards better outcomes**, but often the trials are done without patient input, do not deliver on unmet needs, do not deliver patient-relevant data (e.g. PRO, QoL), are run in the wrong countries, do not recruit
- **Care pathways** are often not reflecting true patient needs and real-life situations
- **Access** to treatment and monitoring is often suboptimal or non-existent – focus on the “most attractive markets”, not on highest unmet need (e.g. low/middle income countries)
- **Pharma advisory boards** are often not meaningful, have little impact on decisions and direction, barely provide reports or follow-up
- **Patient centricity** is often mainly a mission statement, not where the difficult decisions are made

How do Community Advisory Boards work?

Building on the successful model of the HIV community, a strategic committee/meeting to discuss research, access to treatment and care and collaboration

This is how it works:

- **We invite participants** from the companies
- **We set the agenda for a two-way dialogue** and pick the topics that are most meaningful to us
- **We respect confidentiality** to have a trusted and open dialogue
- **We report to our members** through our regional CAB members & written report
- **We ensure follow-up** through the CAB office, not one-off meetings
- **We train our CAB members**





CML-CAB Members

CAB chairs:

- Pat Garcia-Gonzalez (USA)
- Jan Geissler (Germany)

Eastern Europe:

- Šarūnas Narbutas (Lithuania)
- Jelena Cugurovic (Serbia)
- Jana Pelouchova (Czech Republic)

Africa:

- Bahija Gouimi (Morocco)
- Eunice Oreka (Nigeria)

Western Europe & Israel:

- Cornelia Borowczak (Germany)
- Felice Bombaci (Italy)
- Zack Pemberton-Whiteley (UK)
- Giora Sharf (Israel)
- Yair Bar David (Israel)

Asia:

- Rod Padua (Philippines)
- Yoke Choon Yong (Malaysia)
- Param Puthen (India)

Latin America:

- Mercedes Arteaga (Argentina)
- Silvia Castillo De Armas (Guatemala)

North America:

- Lisa Machado (Canada)
- Jerry Clements (USA)

CAB office: Denis Costello (CMLAN Exec Director) & Nicole Schröter (CAB officer)

12 CML-CAB meetings in 22 sessions from 2016-2021

Date	Company CAB sessions	CAB Training sessions for patient advocates
5/2016	Novartis, Pfizer	Drug development process and CML research
2/2017	Novartis, Takeda, Ariad	Partnerships; CABs as an advocacy tool
5/2017	Novartis, BMS, Pfizer, Incyte	Sessions of CML Horizons 2017
11/2017	Novartis, Incyte, Takeda	Collaboration with industry
5/2018	Novartis, Pfizer, Incyte	CML PRO & QoL instruments
11/2018	Novartis (+ CEO)	
3/2019	Pfizer, Incyte, Takeda	Strategic priority setting on CABs
5/2019	Novartis	CML Horizons 2019
11/2019	Novartis (+ Global President Oncol.)	
07/2020	Incyte, Takeda	
10/2020	1 st Academic CML-CAB	CML Cure Academy: preparatory workshops and crash courses
2/2021	Novartis	Access to Cancer Treatment in LMIC; Value proposition - where clinical meets access

What did we discuss with the companies in the CABs 2016-2021?

Topic area		Novartis	Pfizer	BMS	Ariad, Takeda, Incyte
Clinical / Research	Development pipeline, clinical trials, path to cure	Blue	Blue	Light Blue	Blue
	Therapy-free remission	Blue	Blue	Blue	Light Blue
	Patient-reported outcomes / quality of life	Blue	Blue	Light Blue	Blue
	Pediatric use of the drug	Blue	Blue	Blue	Light Blue
	Drug safety & side effect management	Blue	Blue	Blue	Blue
	Food effects (fasting)	Blue	Light Blue	Light Blue	Light Blue
	Trials: Selection of countries and sites	Blue	Light Blue	Light Blue	Light Blue
Access	Value proposition of the drug	Red	Red	Light Blue	Light Blue
	Access strategies in different regions	Red	Red	Light Blue	Red
Collabor. & other	Educational material	Orange	Orange	Light Blue	Orange
	Falsified drugs	Light Blue	Light Blue	Light Blue	Orange
	Collaboration barriers, compliance	Orange	Light Blue	Light Blue	Orange

CML-CAB // CAB Workgroups // Company Ad Boards: Where is the difference?

CML-CAB

■ Scope

- **Strategic** collaboration and discussion platform between CML patient community **leadership** and company **decision makers**

■ Framework

- CML-CAB and chairs define the agenda, CAB and company discuss community priorities
- Membership drawn from CAB membership pool

■ Examples

- 21 CML-CABs with companies 2016-2021

CML-CAB Workgroups

■ Scope

- Delegated from CAB in order to **operationalise** CAB tasks which need more constant interaction

■ Framework

- CML-CAB and company define the agenda jointly
- Participants drawn from CAB pool

■ Examples

- **Asciminib Workgroup, Access Workgroup, Market Research Project**
- Give patient input on HCP campaign
- Provide patient insight into the product development program
- Consolidate patient community's perspective on patient access to treatment in 5 countries

Company CML Advisory Boards

■ Scope

- Company to raise questions

■ Framework

- Participants picked by company
- Specific contract with individuals

■ Examples

- Novartis Patient-Centered Outcomes Digital Platform Advisory Board

Strategic priorities for the CML-CAB since 2019

Priority “Research”	Priority “Access”
Participation in clinical trials in low & middle income countries	Improve access to treatment
Increase of QoL of CML patients (which may include side effect management, therapy-free remission)	Improve access to monitoring
Achieve cure	

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TFR does not fully meet the CML patient community's expectation on "CML cure"

- **Recognition that cure is an unmet need** in CML, not a done deal with TFR
- **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~~

→ **Raise commitment and collaboration between all stakeholders, first and foremost patient community and CML researchers** to finding a real cure for CML

What kind of cure do we want for whom? What's the burden to get there?

Goal	Available to whom?
Chronification of CML (on life-long therapy)	All CML patients
Therapy-Free Remission	About 25-35% of CML patients if they have access to TKI+PCR
Cure for everyone	All newly diagnosed patients
Cure the multi-intolerant	Patients with significant side effects on TKI
Cure the multi-resistant	Patients with resistance against current TKIs or with advanced disease

- Additional therapy burden to eradicate CML?
- Long-term burden (of therapy, of monitoring)?
- Financial burden (on family, on healthcare system)?

1st Academic CML-CAB, Oct 2020

■ First every academic, CML-CAB 12 Oct 2020

- **8 researchers** working on novel approaches to CML biology & cure: vaccination, CAR-T, CRISPR, genetics, stem cell research, hedgehog, math modelling
- **16 CML-CAB members** from all world regions with 223 patient years of lived CML experience

■ Objectives

- Build a **dialogue** with academic researchers
- Present our definition of “cure” and unmet need in CML
- Look at different **approaches to cure**, and learn which of these are most promising to cure CML
- Identify how we as a **community can contribute** to achieve the vision of a cure for CML, e.g. collaboration, network building, fundraising

- **Short presentations** by 8 researchers on individual approach, enablers, barriers towards cure, followed by intensive discussions



Academic CAB 2020 – key takeaways

Enablers	Barriers	What can the community do?
<ul style="list-style-type: none"> • Willingness of patients to participate in research • Good collaboration with CML clinical teams & other research groups • Availability of samples for validation of research findings • Alliances (International CML Genomics Alliance / HARMONY Plus)? 	<ul style="list-style-type: none"> • Funding for research (research costs with modern new techniques are high, staf) • Cost of testing • Restrictions in data and sample sharing (ethics, compliance, patient consent, privacy) • Translation of findings in clinical care • Collaboration with clinical teams & other research groups (pooling efforts to enhance progress, sharing technologies and expertise) – see above under “enablers” 	<ul style="list-style-type: none"> • Encourage participation in preclinical studies by contributing samples • Encourage participation in clinical trials • Encourage pharma support for trials <p>Establish a “Research Network for CML cure” that helps coordinate, drive and fund research on CML cure</p>



Conclusions

- The CML-CAB is our key strategic tool to have a dialogue of experienced advocates and decision makers about **our community's** priorities and concerns
- Workgroups established to follow-up on CAB actions and projects
- Outcomes of the Academic CAB will be followed up