Organise your own Community Advisory Board!

Engaging with industry

EMM 2025
24 May, Riga

EURORDIS
RARE DISEASES EUROPE

ACT EU workshop 19 February 2025

An initiative to facilitate clinical trials in the EU

2,000 attendees!

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- How to influence a clinical trial sponsor, private or academic?
- **CABs are simply the most powerful tool to influence R&D of new medicines**

- Unanimity of patient advocates in the room
  more efficient than 2 to 3 people invited to a company meeting
- The power to convince before trial enrolment begins
- Precedents, with success

- — High quality dialogue

François Houÿez | Director of **Treatment information and Access**

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2018 to 2025

17+ CABs created

Atypical Hemolytic Uremic S.
Cystic Fibrosis (16 products)
Limb Girdle Muscular Dystrophy
Psoriasis
Dravet syndrome
Hered. Haemorr. Telangiectasia
Pulmonology
Multiple Sclerosis
Myotonic Dystrophies

And also (autonomous)
Cystinosis, Duchenne Muscular Dystrophy (20 products), Angelman, Lymphomas, Thalassemia, European Tuberous Sclerosis Complex, Systemic sclerosis, Neurofibromatosis
…

43+ products discussed

130+ trained CAB members

53 clinical trial protocols reviewed

Training
120 hrs online
100 hours f2f

27+ companies involved
30+ CAB meetings over a **7** year period

21 Charters signed with industry (Good Engagement Practices)

F. Houÿez | EURORDIS

CABs 2025

EUPATI Spain and EURORDIS

Introduction

Rob Camp

PATIENT ENGAGEMENT IN THE DESIGN AND CONDUCT OF CLINICAL TRIALS

CABs EXIST SINCE THE 90s (Cancer, HIV, haemophilia…)

EuroCAB programme launched 2016 (partnership with EUPATI-Spain)

https://globalcab.org/

A group of patients who offer their expertise to public or private sponsors of clinical research

- Overall programme development
- A single clinical trial
- PROMs, Compassionate use, pricing and access, side effects…

The same patients advise several sponsors in their field

- Avoids selection of patients' representatives by the sponsor

Agenda and secretariat driven by the patients

Guidelines, SOPs, templates, decision tracker etc.

Companies engage with CABs to discuss unmet needs, impact of the disease, how to measure efficacy etc.: are mutual expectations met?

EUPATI Spain

- Patient education *(www.eupati-es.org)*
  - Train the trainers
    - Courses (PV, Lifecycle of R&D, etc)
    - Working with regulators and industry
  - Search engine for Clinical Studies
  - Presentations / webinars at national network meetings
  - Manuals - how to / why enter a study; HTA reports
  - Clinical Studies week (20 May) (videos, events)
  - H2O vice-presidency
- Toolbox
- Regulatory Authorities
  - AEMPS, EMA
- Community Advisory Boards *(globalcab.org)*
  - 14 disease areas
  - PROMs and PREMs; shorten development time (r/r); RWE

- Spanish Ministry of the Interior – registered non-profit Civil Society Organisation 1/1 n.616532

Manuals – how to understand a study and if it's for you; how are HTA decisions made?

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CAB work breakdown

Patient network* secretariat work includes

- Planning and preparation of meetings - Agenda building
- Building Community advisory board membership and maintaining engagement
    - Finding new members
- Training and Webinars
- Improving the inclusion of quality-of-life metrics and patient-reported outcome measures in research
- Keeping positive work atmosphere and communication and good relationships with individuals, patient organizations, pharmaceutical industry
- Researching information about trials and conferences (horizon scanning)
- Advocating for wider access to clinical trials and treatments across Europe
- Facilitate follow-up

- *apart from **administrative** secretariat and **mentoring / guidance** secretariat

CAB Budget breakdown

CAB Members offer

- their lived experience, including identifying areas of unmet patient and societal need
- and are paid a compensation for the time spent, as volunteers should not be losing revenues.
  - the only part of the budget that is broken down by hour because most companies' legal departments want to see it laid out this way

Question 1

- Different CABs
- Year each CAB started
- Numbers of members
- Numbers of products they work on
- Average number of meetings per year

CABs meet once or twice a year – 2 to 3 days (session)
During a session they can meet with 1 to 4 companies

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CAB	Foundation	Nb. Members	Nb. products	Members Countries
Tuberous Sclerosis Complex (TSC)	2017	8		ITA, POR, SPA
Limb Girdle Muscular Dystrophy (LGMD)	2018	7	1	FIN, FRA, GER, ITA, NLD, NOR, SPA, GBR, India, South Africa, USA
Cystic Fibrosis (CF)	2016	16	16	BEL, FIN, GER, HUN, ITA, LAT, NLD, POL, POR, SPA, GBR, Israel, Turkey, Ukraine
Duchenne Muscular Dystrophy (DMD)	2018	15	20	BEL, FRA, GER, ITA, NLD, ROM, SPA, SWE, GBR, Canada, Israel, Italy, Turkey, USA
Multiple Sclerosis (MS)	2020	10	2	BEL, CRO, FRA, IRE, ITA, NLD, POR, ROM, SPA
Psoriasis	2020	8		CRO, GRE, ITA, NLD, POR, SLN, SPA
Cystinosis	2020	9	2	BEL, FRA, GER, IRE, NLD, SPA, GBR, Mexico, USA
atypical Hemolytic Uremic Syndrome (aHUS)	2021	12	3	GER, POL, GBR, Australia, Brazil, Canada, Egypt, India, Singapore, Turkey, USA
Dravet syndrome	2021	5	5	CRO, GER, ITA, NLD, SPA, Serbia
Myotonic Dystrophy (MD)	2022	7	7	NLD, SWI, GBR, USA
Hereditary Hemorrhagic Telangiectasia (HHT)	2022	8		GER, IRE, ITA, NLD, NOR, SPA, SWI
Pulmonology	2023	15		GER, ITA, NLD, SPA, GBR, Israel, USA
Lymphomas	2019	11		3 CABs: Follicular Lymphoma, Mantle Cell Lymphoma, Chronic Lymphocytic Leukaemia
Total		131	56	
Average (CI)		10 (8-12)	7 (2-12)	
Median		9	4	

Question 2

- Involvement of patients in the design and the conduct of clinical trials:
- Can you describe how CAB intervenes at the design phase of a clinical trial?
- And during the clinical trial, in terms of advising the sponsor and/or informing trial participants on unexpected side effect, substantial amendments, trial interruption or trial results?

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Question 3

- Do you follow-up the changes you proposed to the clinical trial protocols ?
- How do you evaluate the impact your CAB has on the product R&D?
- Examples of “successes”, or changes you obtained in the design or practical aspects of a trial?

Our success tracker

Priority Area	Goal	Collaboration	Action to do	Milestones / updates	Goal reached
Access					
Research / Clinical Trials	Studies in patients from 2 to 5	Epidemiology: how many?	To revise entry criteria in CTs / PIP	Every 6 months: review of progress	Achievement date
QoL / PROMs					
Patient Support / Communication					

Question 4

- CABs and their independence vis-à-vis developers : how do they ensure independence?
- Do CABs exchange views with regulators and/or HTA? How?
- CAB members participate in meetings with companies, and this is considered as consulting under policy 44 Handling competing interests (EMA) .
  - How do they address this within their organisations?
  - Are CABs aware of this and does it play a role in their decision on how many members from the same organisation to include in a CAB?
- Benefits of CABs engaging with different pharmaceutical companies and not just one?

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Question 5

- How do you evaluate the importance of having trained CAB members?
- Is training a pre-requisite to join a CAB, or do your members learn by doing?

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Principles and CAB Seal Criteria

Must have

- Open Call for volunteers (patient groups, social networks…), composition decided by patients
- Agendas and composition made public (members' names)
- Work with different developers in the field (when applicable)
- Memorandum of Understanding for each project
- Minutes and follow-up of each meeting
- Elected CAB chair

- **Important to have**
  - Scientific secretariat

Guidelines

- How to start, how to operate, Travel and accommodation, Compensation for time spent, Declaration of interests, Insider trading prevention, Code of Conduct for CAB members

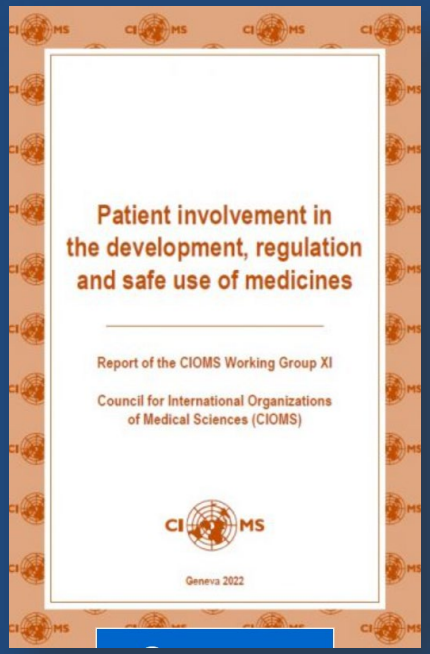
Advocacy & Implementation

01

CIOMS report on Patient Involvement in the Development, Evaluation and Safe Use of Medicines.[1]

ICH E8 on general considerations for clinical studies: Engaging stakeholders in study design.[2]

CTCG Project on Patient Involvement



02

IHI Consortium submitted (compassionate use, involvement of CABs)

Capacity building on HTA (EUCAPA), fair price calculators & price discussion

Scaling up: AI tool for more systematic information to patient networks

Regulators / sponsors

HTA / Payers

cf also IMI PARADIGM

1: https://cioms.ch/working-groups/working-group-xi-patient-involvement/
2: https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/ich-guideline-e8-r1-general-considerations-clinical-studies_en.pdf

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Any questions?

If interested in creating a CAB, or in helping mentoring CABs, contact us!

Rob.camp@eupati-es.org
francois.houyez@eurordis.org