Community Advisory Boards

•EuroCAB Progress report

December 2018

Community Advisory Boards (CAB) Update

- What are CABs?
- Progress so far
- Feedback from the CABs for Duchenne & Cystic Fibrosis
- Metrics
- Readiness to launch new CABs

A Community Advisory Board is

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

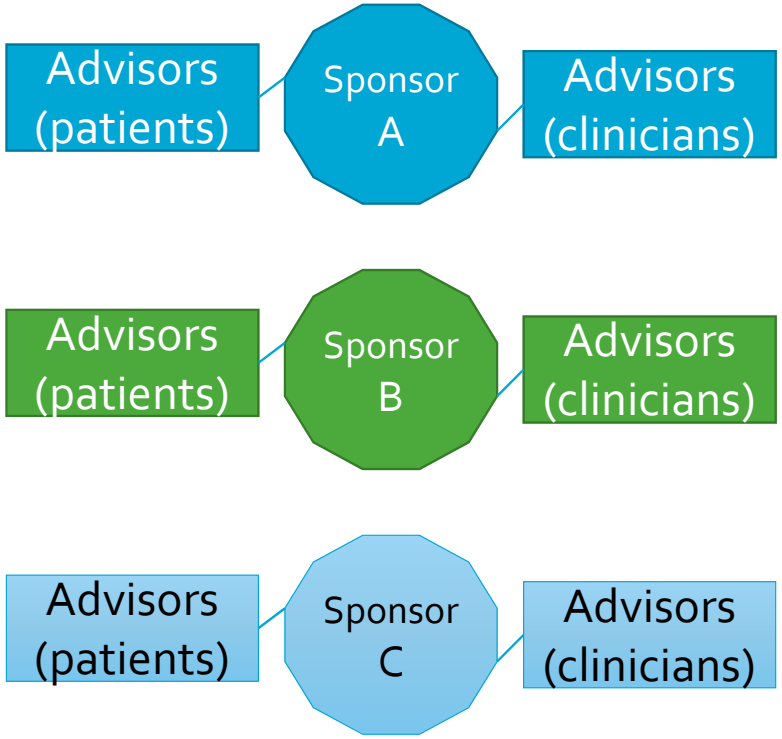
- Overall programme development
- A single clinical trial
- Other aspects beyond the research programme

The same group of patients advises several sponsors in their field

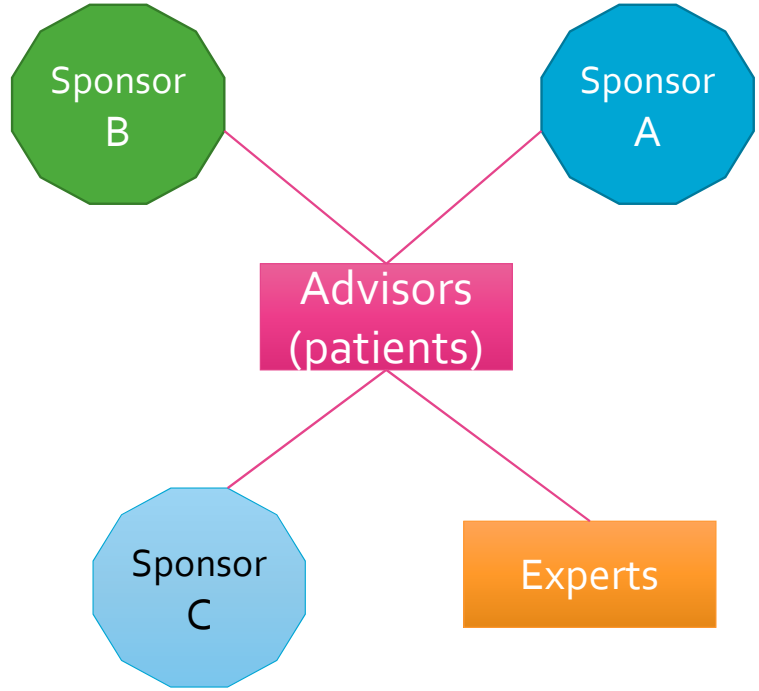
- Avoids selection of patients' representatives by the sponsor
- Agenda and secretariat driven by the patients

Same disease area, different sponsors

Sponsor-dependant model



CAB model



CAB Results

MEAT (multi-experimental agents trial)

DUET studies (Ph 2/3 study)

FDA asked Tibotec/J&J to submit an NDA before study completion

5 drugs approved in 9 years (HIV, HCV, TB)

*More on the way (microbicide, long-lasting injections)

Why translate this history into RD in 2018?

- To equal or surpass what has been done in areas like HIV
- We add Guidelines and a Seal of Good Patient Engagement Practice (GPEP)
- Metrics
- Training
- Cross-company meetings
- A structured programme, sustainable, that becomes a standard and part of the operating procedure in industry

F. Houÿez – Director of Treatment Information and Access
R. Camp – P.E manager, Community Advisory Boards

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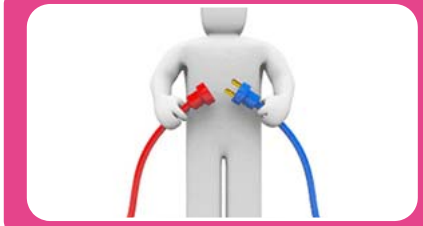
The EUROCAB programme: incubator, mentor, advisor

Identification of areas where CABs are needed

- Call to Members
- Feedback
- Webinars to patient networks, meetings, preparatory phase (6 months)



Matchmaking with industry

- Contact or help contacting developers /sponsors that correspond to Network needs
- Receive direct requests from developers

Mentoring

- Preparing members/help preparing and running CAB meetings
- Keeping guidelines up-to-date, developing policies, qualification with authorities
- Treatment activist advice

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R. Camp – P.E manager, Community Advisory Boards

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Guidelines

How to create and operate a CAB

Travel, subsistence and compensation of time spent

Code of Conduct for CAB members

Confidentiality undertaking

Insider Trading prevention

(industry working group)

How to evaluate your CAB?

Developed

In development

Trainings

- Webinar: How to negotiate price and pricing policies (CF and Duchenne)
- Training webinars (Open Academy) with Duchenne CAB
  - 4 June - Ethics
  - 7 July – Medical Research
  - 16 Sep – Regulatory + EMA
  - 21 Nov - HTA
- December 2018 CEF:
  - Metrics: How to evaluate CABs?
  - How to scan your horizon
    - To become part of Eurordis Summer School on Development and Regulatory Affairs
- Training webinars (Open Academy) with CFE CAB
  - 2 March - Ethics
  - 5 Apr – Medical Research
  - 2 Oct – Regulatory + EMA
- Other meetings (pt engagement):
  - 23 Mar - AEMPS
  - 27 July – ISPOR Lima
  - 7 Nov - IMI 10th anniversary Madrid

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R. Camp – P.E manager, Community Advisory Boards

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Campaign in 2018

Jean-Marc and Eva for e-meetings and e-news

E-meetings / face-to-face with Industry

- 5 March - Charter signers
- 19 March - non signers
- 26 March - Novartis
- 28 March - Chiesi
- 2 April - Johnson & J.
- 27 April - NDA Reg. (f2f)
- 11 May - Celgene (+ 3 others)
- 29 May - Roche
- 30 May - Santhera
- 19 June - Boehringer (f2f)
- 20 June – Pfizer
- 3 July (24 participants, 21 end 1st hour)
- 7 Aug – Actelion
- 23 Aug – Novartis
- 24 Aug – Wave
- 28 Aug – Roche
- 28 Sep – Roche
- 11 Oct – DLA Piper
- 4 Oct – Roche
- 1 Oct – Sarepta
- 16 Oct - Celgene
- 6 Nov – Vifor
- 6-8 Nov - WODC
- 19 Nov - IQVIA

E-meetings / face-to-face with EURORDIS members

- 11 April - webinar
- 14 April - Ectodermal dysplasia (f2f Murcia)
- 24 April - Lymphomas (LCE)
- 14 May - webinar
- 26 May - LHON France (f2f)
- 28 June - moderating EUPATI's webinar on CABs
- 3 July: Pituitary diseases
- 6/7 July - LCE GA Prague (f2f)
- 13 July - Cystinosis GA Berlin (f2f)
- 17 Aug – E-TSC
- 20 Sep – PHA
- 24 Sep - SMA
- 16 Nov – HHT meeting Oslo (f2f)
- 21 Nov - SMA

Electronic communication and conferences

- 4 April - Member News
- 18 April - Member News
- 2 May - Member News
- 10/12 May - Poster ECRD
- 12 May - Presentation ECRD
- 6 June - Member News
- 13 June - EURORDIS eNews
- 15 June - Summer School
- 20 June - Member News
- 22 August: U. Copenhagen (Birthe)
- 26/27 September - CIRS London
- 16 October - ERTC Barcelona
- 25 October: GBF Foresight Training
- 7 November: EU Patient Summit
- 12 November: ISOP Geneva

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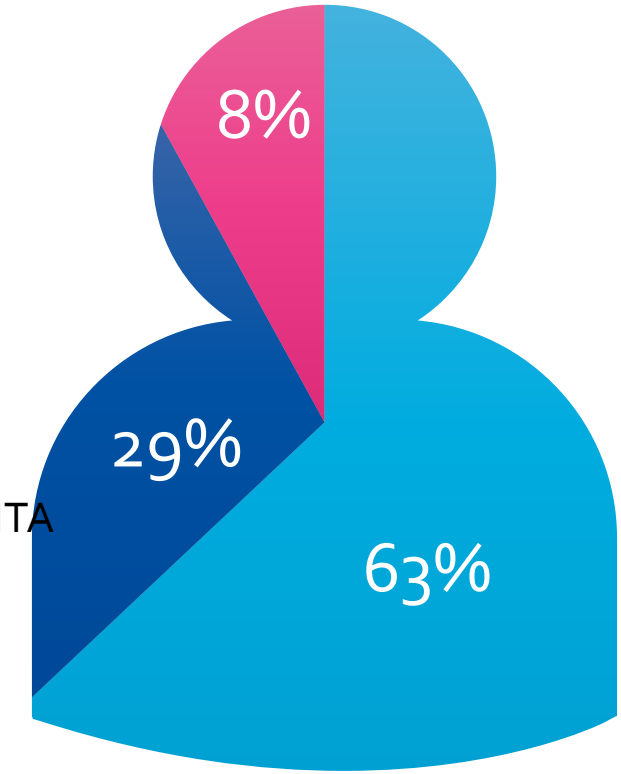
Who attended the ERTC workshop on CABs? 16 October 2018, Barcelona



- Industry
- Patients, staff included
- Academia, regulators, HTA

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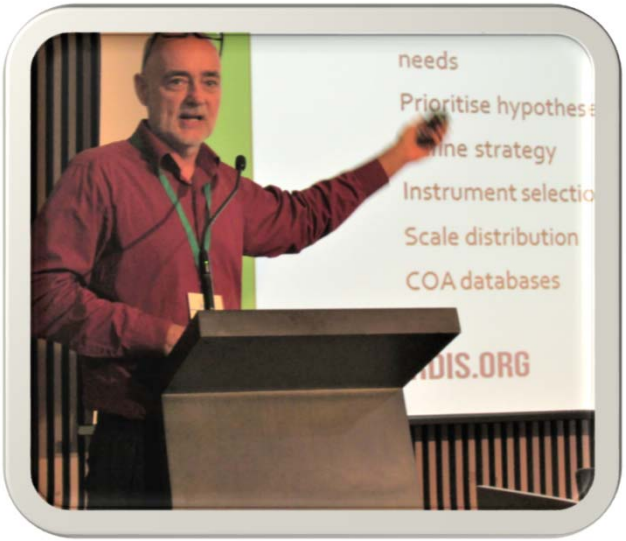
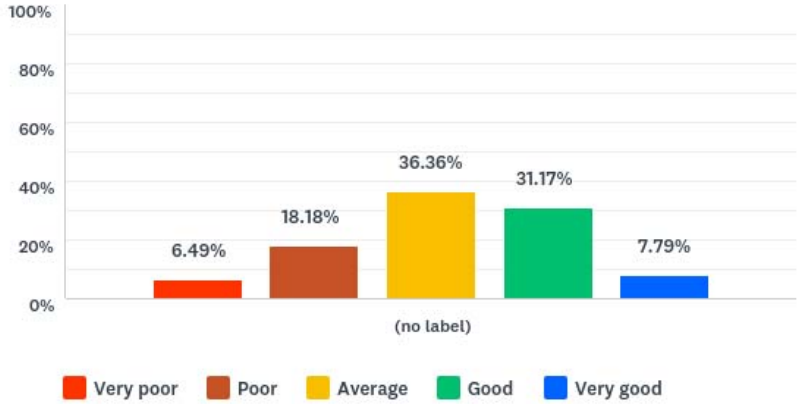
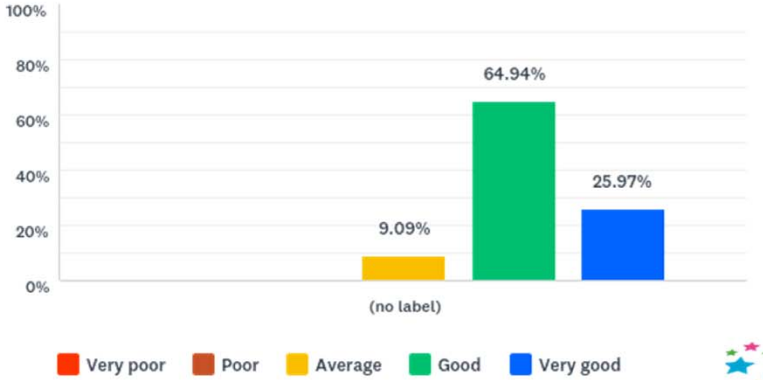
Workshop evaluation
77 respondents



Q5 How would you rate your degree of knowledge for CABs before the workshop in Barcelona?



Q6 How would you rate your degree of knowledge for CABs after the workshop in Barcelona?



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Some quotes

Head of Department Virology and Gene therapy

French National Agency for Medicines and Health Products Safety (ANSM)

Luciana Ballini, Italy (HTA Emilia-Romagna)

The whole spectrum of decision makers should be taken into account, as it encompasses all those deciding on the same intervention and if they are not fully and equally informed, strident disagreements and tensions can occur: the policy makers, the clinicians, the patients

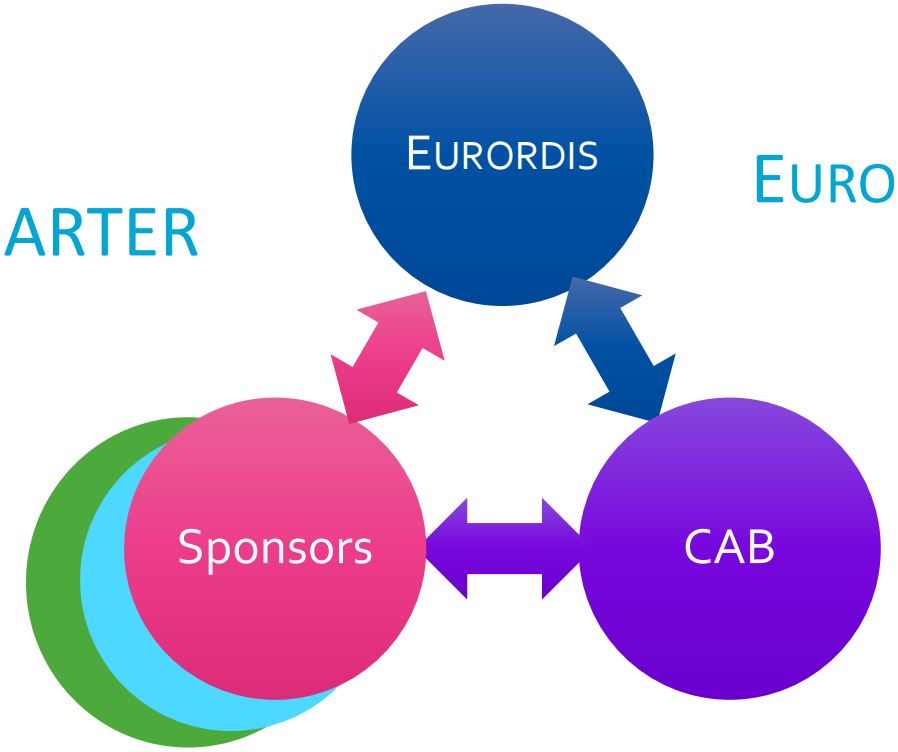
Italian HTA Agency
Emilia Romagna Region

Chair of EUnetHTA JA3 General Assembly

Former Co-Chair of the SEED project

EUROCAB Programme

The CHARTER



EUROCAB Seal

Memo of Understanding

F. Houÿez – Director of Treatment Information and Access
R. Camp – P.E manager, Community Advisory Boards

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CAB Seal criteria

- Mandatory
  - Open Call for volunteers
  - Agendas and members are made public
  - Work with different developers in the field (when applicable)
  - Memorandum of Understanding for each development / research project / study
  - Minutes and follow-up of each meeting
  - EuroCAB contract signed between CAB and Eurordis
  - Elected CAB chair
- Nice to have
  - Scientific secretariat
  - Certification that CAB members followed the e-learning (Open Academy)…
  - Horizon scanning activities

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EMA meeting, 10 Dec 2018

- *Presentation of the Programme*: Short history of CABs
  - Their scientific importance across the entire product life-cycle, Operational aspects for the creation and running of CABs in rare diseases, **The concept of "Patient Investigator"**
  - CAB evaluation
- *Points for discussion*
  - Sharing CAB minutes with regulators, organising dialogue between CABs and experts
  - Giving advice to industry, sharing opinion with regulators: transparency requirements, policy on conflicts of interest
  - Overall, could the EuroCAB programme qualify for EMA as a valid platform and/or source of patient feedback on the development of a product? Scientific Advice is the place.

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How do we measure the value of…?

Informing / guiding research goals

Reducing development time

Identifying patient-relevant endpoints

Better fitting patient needs

Demonstrating the value of a treatment

Identifying previously unmet patient needs

Evaluation

- Athena Institute (PARADIGM)
  - First discussion with Duchenne CAB 30 November 2018 and how to define metrics
  - Table of possible topics discussed yes/no if yes, main contribution:
    - Target population
    - Study feasibility, practical
    - Informed Consent
    - Endpoints, including PROs and PROMs
    - Comparators / Standard of Care
    - Quality of Life / Study Burden
    - Compassionate use
    - Pricing, marketing strategy

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Duchenne CAB Session 27 Nov – 1 Dec 2018, Amsterdam, 3 sponsors, 3 agendas to prepare

28/11

Arrival

Preparation for 3 companies

Non-affiliated investigator about PPMO*

29/11

Company A
Feasibility of non-stop rollover trial and PPMO* strategy and pipeline

Company B
Clinical trial burden
Endpoints and outcome measures for very young / ambulant / non-ambulant DMD population

30/11

Company C
Is delaying the start of assisted ventilation relevant?
Outcomes measures and trial duration
Home-based monitoring device?

Debrief 3 companies

Internal training on pricing models / mechanisms

01/12

HERCULES
PARADIGM projects

Organisational matters

Departure

* phosphorodiamidate morpholino oligomer

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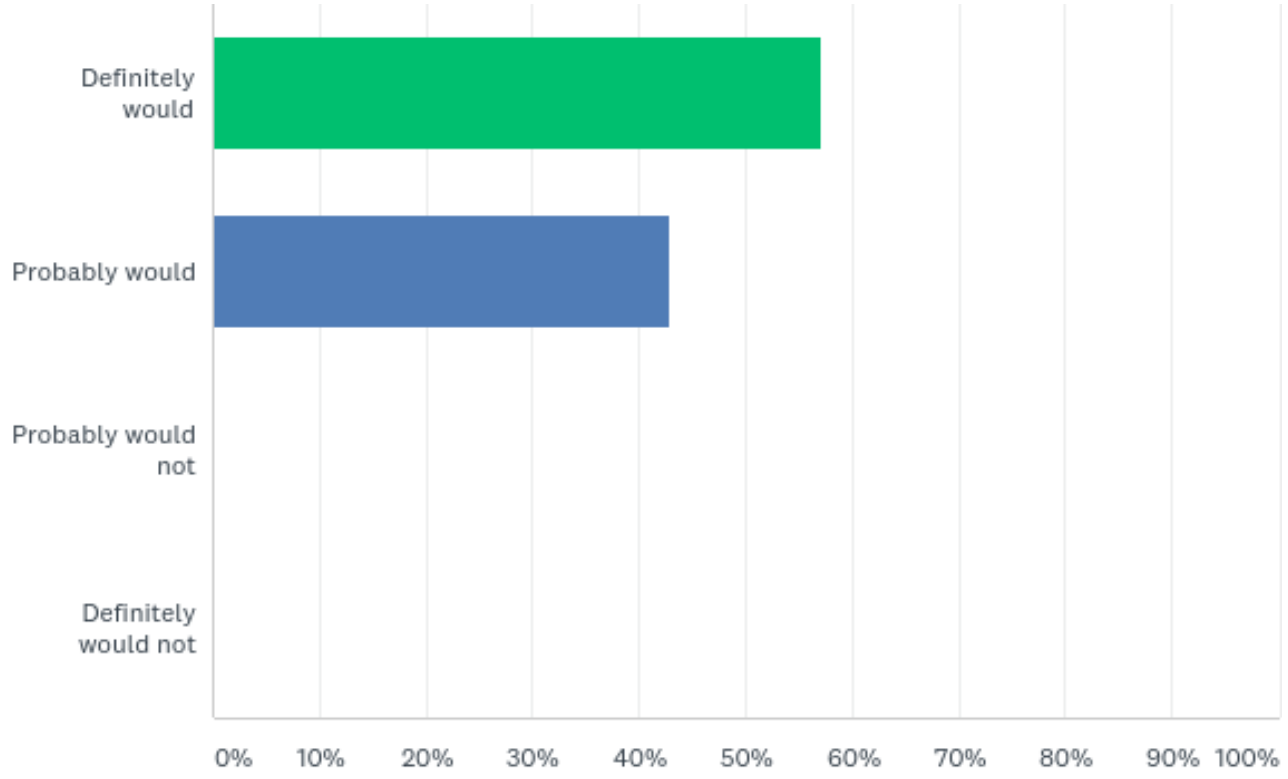
CFE Action!

nent Information and Access
mmunity Advisory Boards

Which of these three areas are you willing to work in?

ANSWER CHOICES	RESPONSES	
Access (geographic access incl. compassionate use and expanded access, and/or mutational)	63.64%	7
QoL (Patient-reported and relevant outcomes measures, lessening medications, adherence, burden of treatments...)	54.55%	6
Communication (Informed consent, videos, apps, etc)	36.36%	4
Total Respondents: 11		

Can these CAB meetings help you demonstrate the value of the product to the regulators?



Metrics

- Are changes made? Do we see them?
- Can we measure them? Tracking
- Have any changes been suggested by existing CABs that we can see?

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Metrics of success

- Better recruitment, more diversity of participants (when needed)
- Better retention, better adherence to protocol (similar to retention)
- less amendments (better patient experience in the trial)
- more inclusive, more sensitive, more ethical design
- more understandable language in documents
- faster or more comprehensive expanded access
- faster completion (faster approval)
- patients included in analysing
- patient involvement in post-trial follow-up

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Update

7 CABs
13 sessions

- Confirmed

- Duchenne (2)

- CF-Europe (2)
- Lymphomas (3)
- Pompe (1)
- Cystinosis (2)
- Ataxia / HSP (2)
- HHT (1)

4 CABs
6 sessions

- Answer soon

- Osteogenesis imperfecta
- Pituitary syndrome
- Fabry
- Myasthenia Gravis

7 CABs

- On hold

- E-TSC
- Systemic Sclerosis
- Sickle Cell
- Beta-Thalassemia
- Amyloidosis
- Leber Hereditary Optic Neuropathy
- Pulmonary hypertension

Planning 2019

Mentors and # CABs

Governance

- 1st meeting of CAB chairs and mentors: 25 March
- Meeting with industry working group: 26 March
- Advice from DLA Piper lawyers for the Charter

Outreach to members

- Now, we consolidate
- Train more mentors

Communication

- Abstract submitted for a presentation at HTAi 2019
- ISPOR 2019 (1/3/19)
- DIA workshops ?
- DIA USA 2019?
- EMM 2019 Bucharest: session

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Soon to be in place

CAB Portal online

- EUROCAB - The Gate

CAB Seal

- Finalise the checklist
- Audit the first CABs

EMA Qualification

- Scientific exchanges between CABs and EMA
- Letter of Support

EuroCAB Programme plan

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- EuroCAB programme: outreach, now to consolidate
  - Still developing the back-end: SOPs (guidelines), contracts, administrative processes, impact evaluation
  - Mentors will grow as programme grows
- Communication on the programme: repetition, repetition, repetition

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Thank you for your attention

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Difficulties, challenges

- **Different networks**
  - More or less organised
  - Overall slow decision-making
  - 1 GA per year but important decision
- **Communication**
  - Need for several e-meetings with each network
  - Participation in General Assemblies is a plus
- **Mentoring**
  - Time needed to learn the disease and its treatments overlooked
- **Funding**
  - Even if reasonable, the costs for a CAB session are an investment for sponsors
- **Leadership**
  - To start a CAB: a committed project leader is needed
  - They exist, but it sometimes take time to find them!