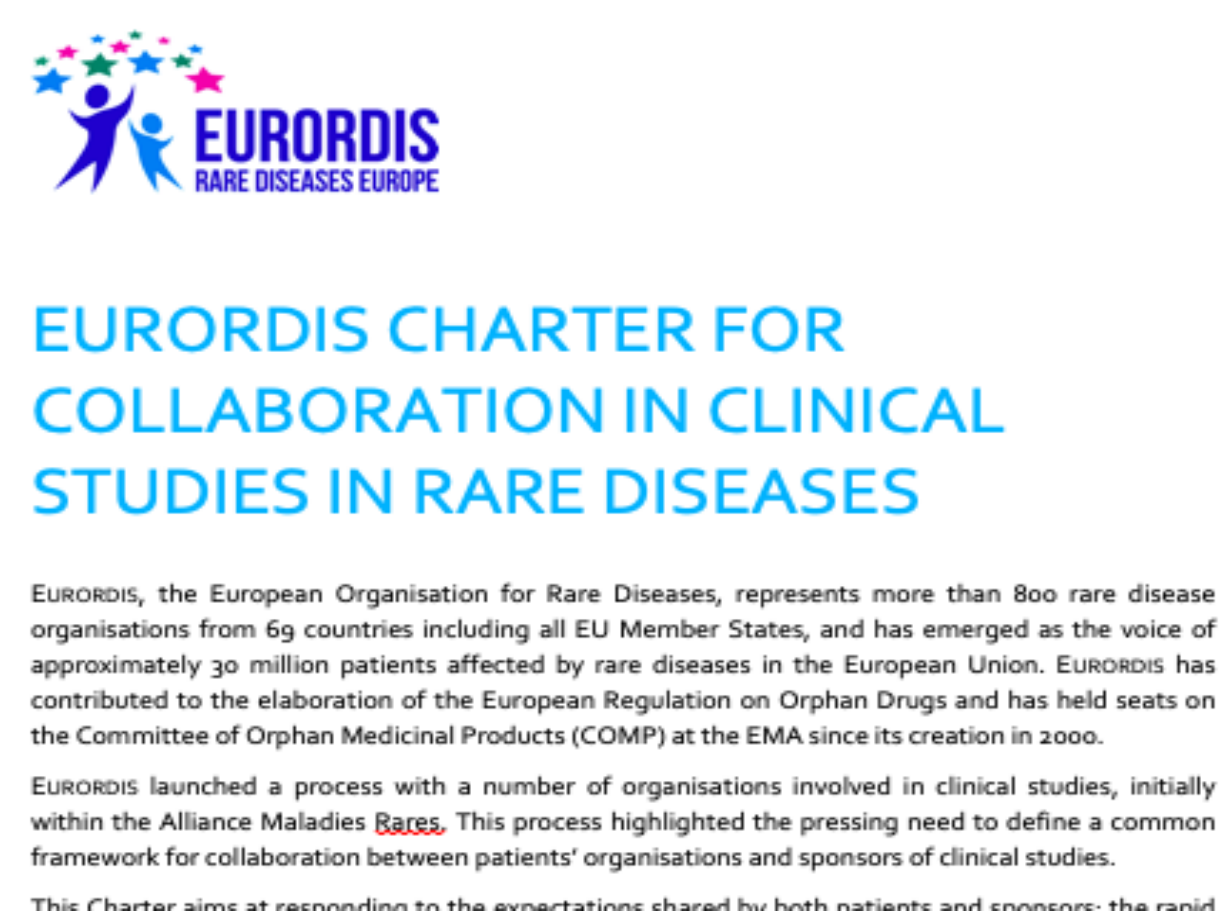


Patient Engagement in Research

The EURORDIS patient-led Community Advisory Boards (CABs)

When developing a health technology that requires clinical research, developers institute working relations with clinical investigators. Patient representatives as well can create advisory boards (CABs). This was of high utility in the 1990s, in particular for the development of products to treat HIV infection. Inspired by this model, the European Organisation for Rare Diseases (EURORDIS) proposes a new programme of CABs.

EURORDIS invites developers to sign a **Charter for Collaboration with patients in clinical research**, and provides guidelines together with a mentoring and training programme for patient networks.



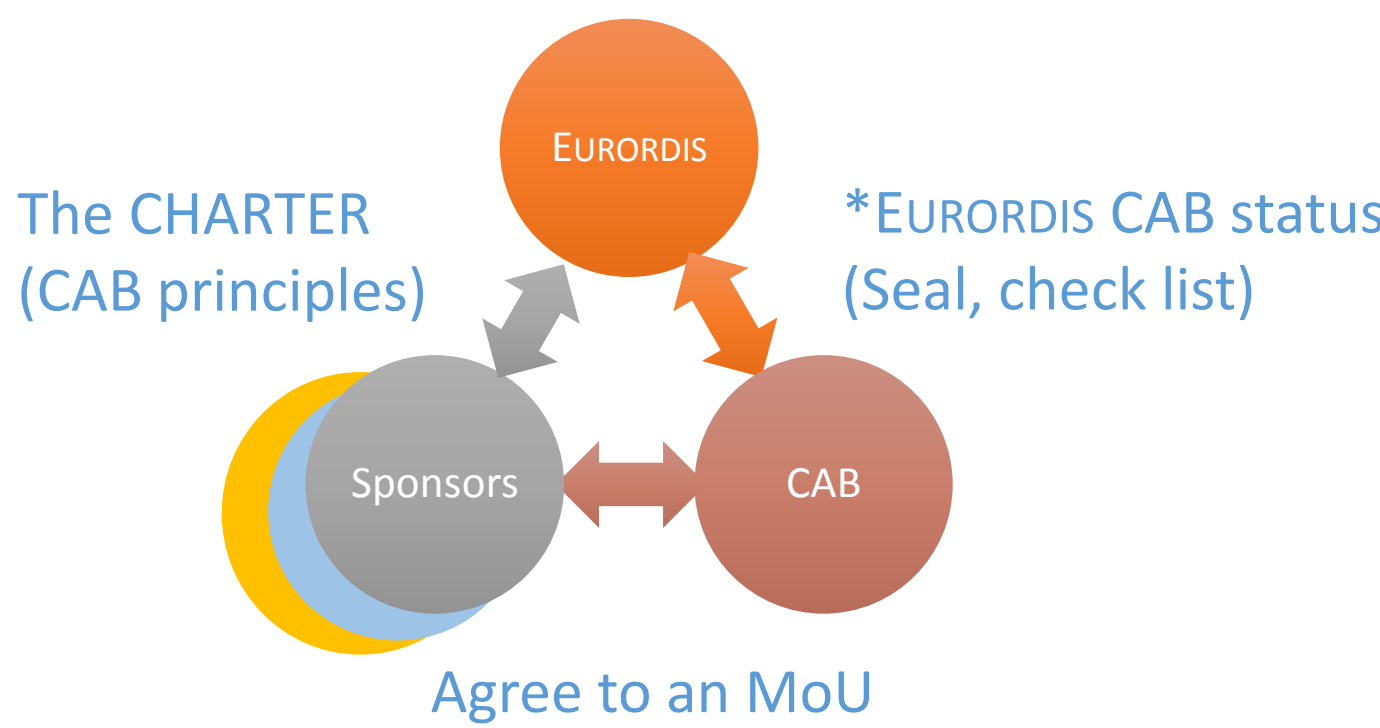
CABs and the developer co-create the agenda, work on topics as diverse as study design, feasibility, informed consent and site selection, QoL and PROMs, and organise the meetings. Discussions also cover compassionate use, pricing, relative efficacy, etc. Meetings last for 2 to 4 days during which sessions with different developers can take place, along with trainings.

We have started to work on the metrics of markers of success.

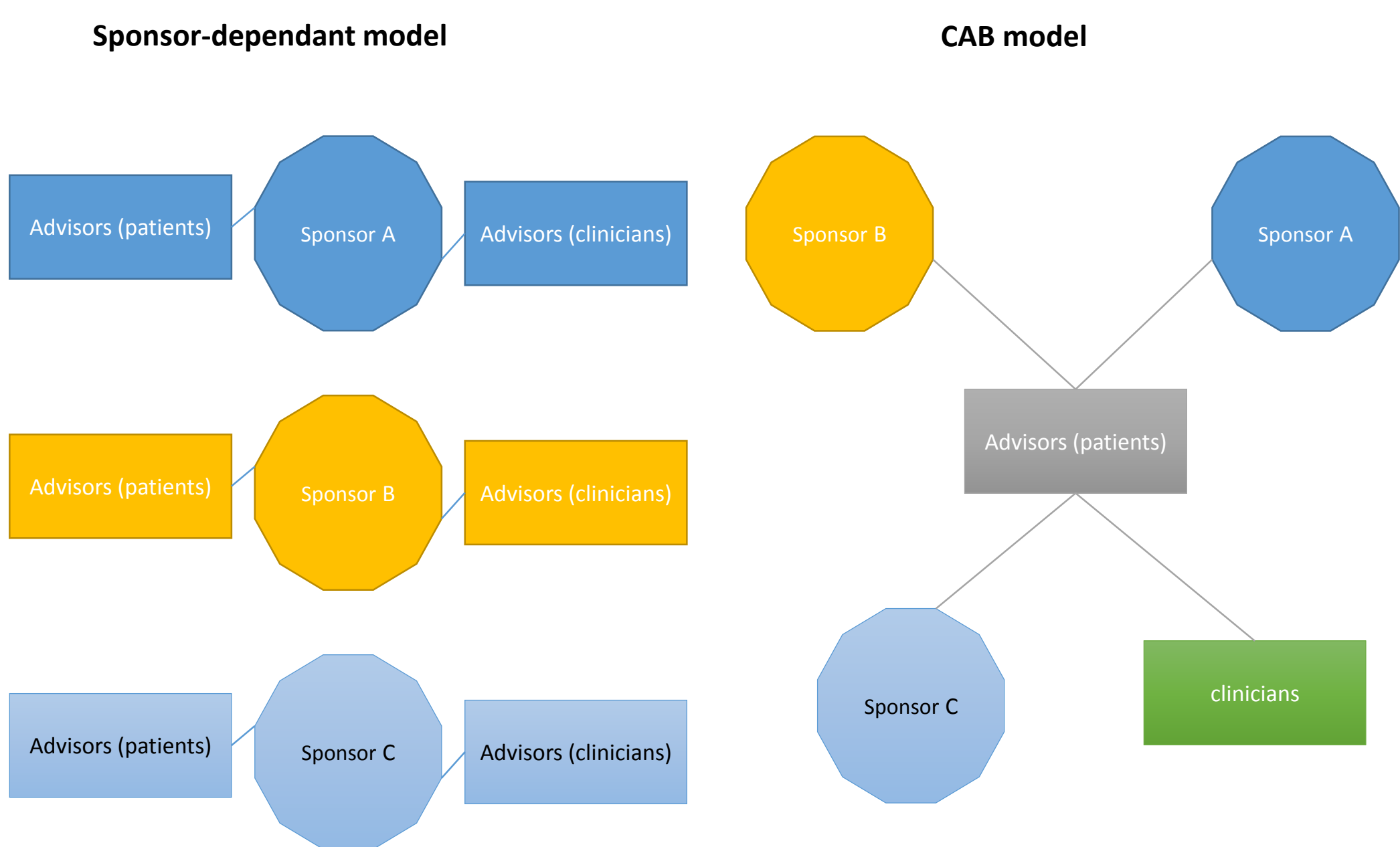
This patient engagement programme, with collective thinking and exchange between patients and developers, ensures high quality dialogue and can inform both HTA and regulatory decision-making.

- Expected outcomes
 - Trial quality will improve
 - Patient interest in research will grow
 - Chances of a positive outcome of the trial/development due to
 - Better design, smarter comparator, patient-friendly practical aspects
 - Patients are retained, ie due to better info flow, better follow-up of SEs
 - Regulators and HTA can make better and faster decisions (better-defined QoL, PROMs, etc)

2019 EUROCAP Programme



- The Charter is an expression of mutual intentions and aspirations
- The Charter is not legally binding
- The collaboration is based on respect & trust and is not tokenistic
- The CAB is recognised as an independent body and is not structurally dependent on the sponsor
- The work and the structure is transparent
- Agendas are cooperatively designed
- The dialogue is meaningful and of high quality
- Collaboration between the sponsors and the CAB is timely, where input can make a difference
- Confidentiality is respected by both sides
- All interactions are considered non-promotional



To receive the EUROCAP Seal of good practice

- 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
- Important to have
 - Scientific secretariat
 - Certification that CAB members followed the eLearning (Open Academy...)
 - Horizon scanning activities

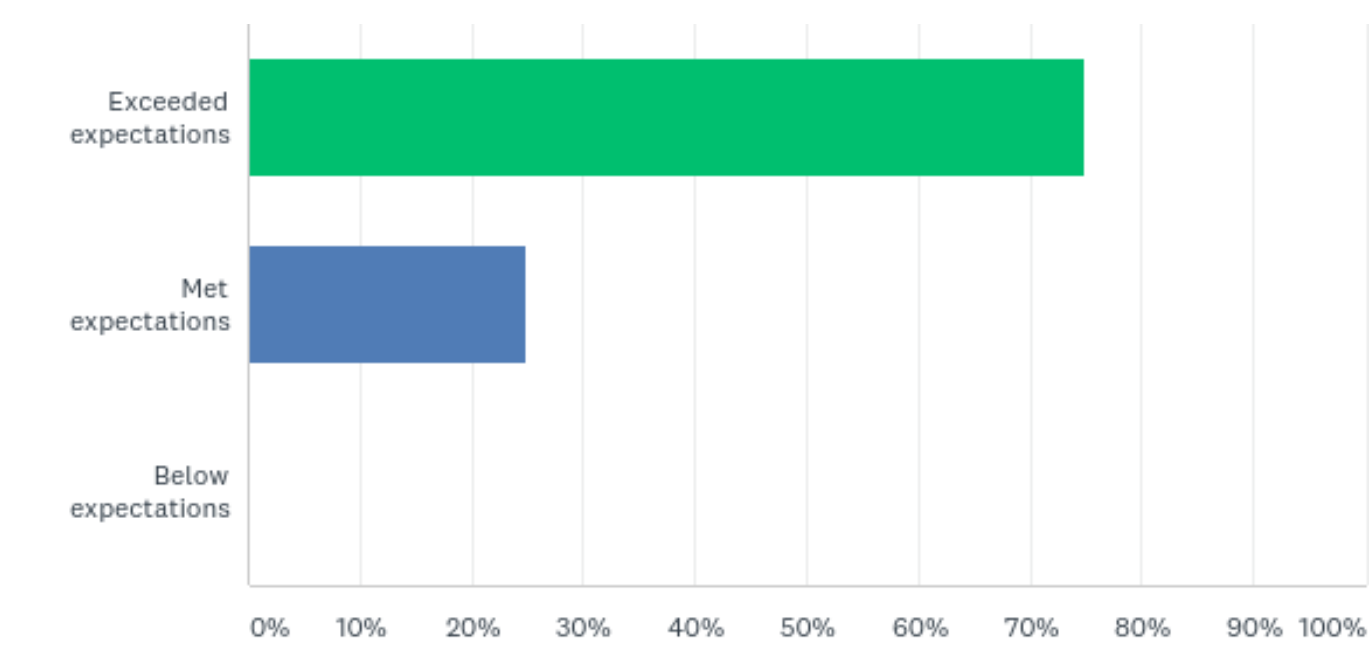
Utility – Tracker: Table of possible topics

	discussed yes/no	if yes, main contribution	outcome ?
Target population			
Study feasibility, practicalities			
Informed Consent			
Endpoints, including PROs and PROMs, <u>QoL</u>			
Comparators / Standard of Care			
Study Burden			
Recruitment			
Substantial amendment to protocol			
Unexpected event			
Compassionate use			
Pricing, marketing strategy			

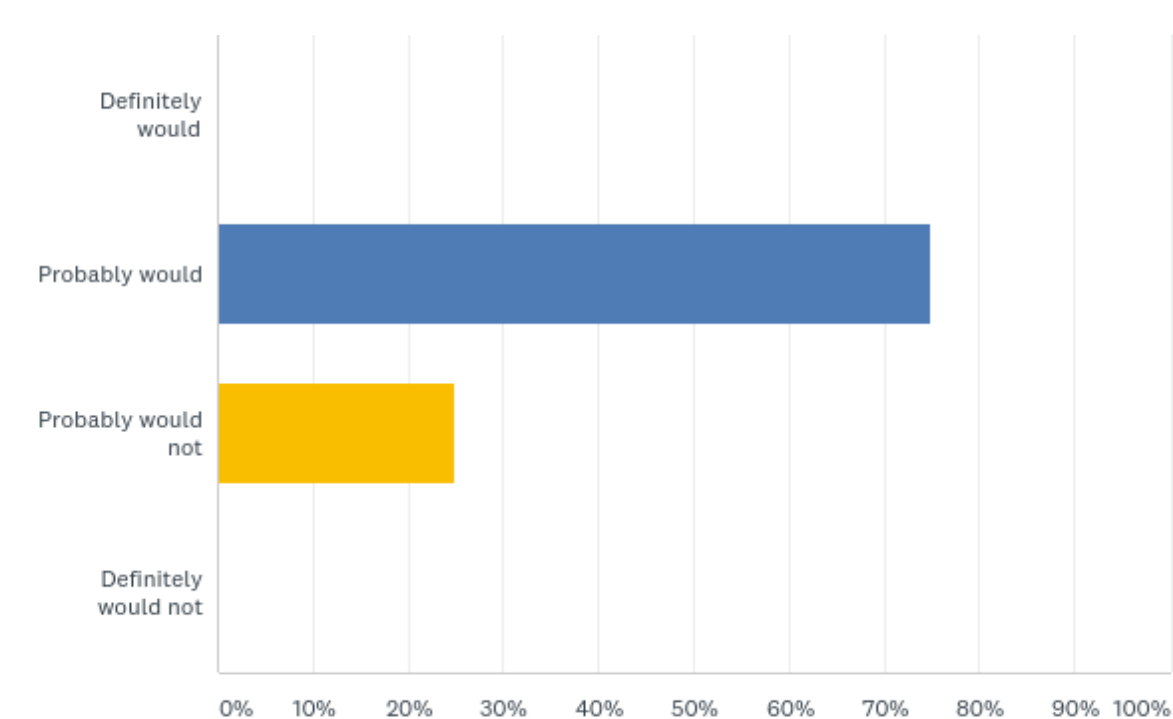
DISCUSSION: In the past three years, we have co-hosted 14 Community Advisory Boards with 19 sponsors on 35 products in 4 disease areas. Besides Tuberous Sclerosis Complex and Systemic Sclerosis, we have instituted CABs for Cystic Fibrosis, Duchenne Muscular Dystrophy, Lymphomas and Cystinosis. We expect to start (3) new CABs in 2020.

R Camp, Patient Engagement Senior Manager, EURORDIS; F Houÿez, Information & Access to Therapies Director & Health Policy Advisor, EURORDIS. #EUROCAP #ISPOREurope

How useful was the meeting with the CAB?



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



Will there be follow-up, a continued relationship with the CAB?

