PATIENTS & SPONSORS

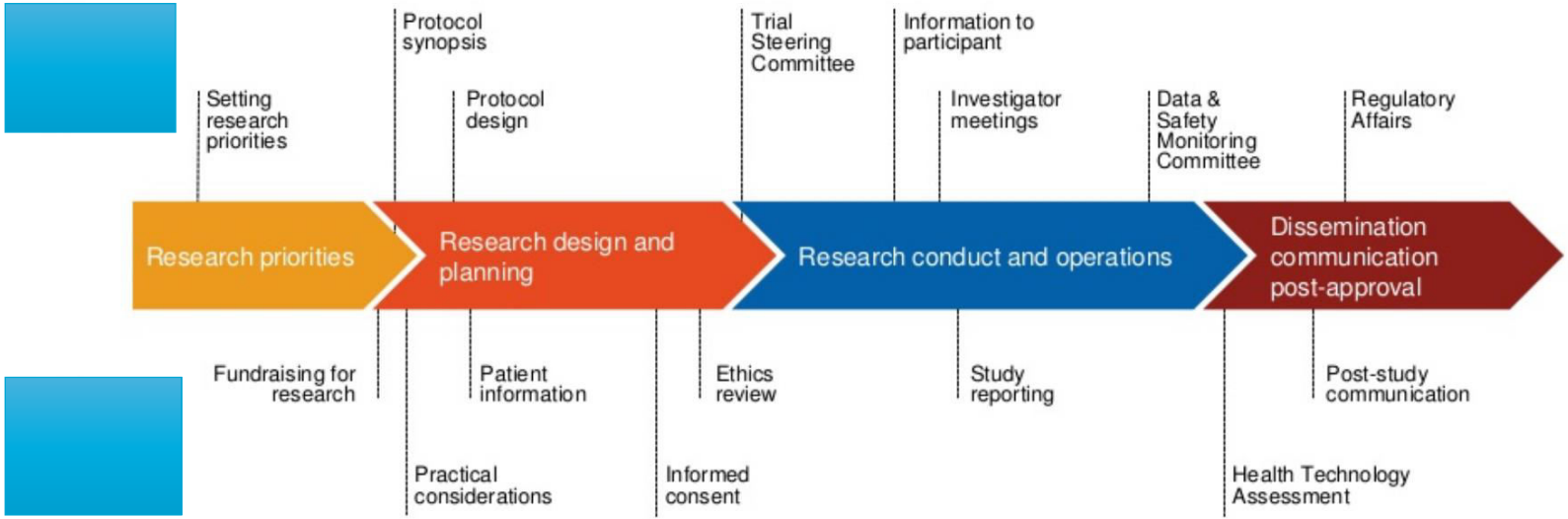
Regulatory Authorities & HTA Bodies

Rob Camp

12 May 2018, Vienna

EURORDIS.ORG

Patient involvement in practice within the R&D life cycle



Source: Geissler, Ryll, Uhlenhopp, Leto (2016)
Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405

A Community Advisory Board is

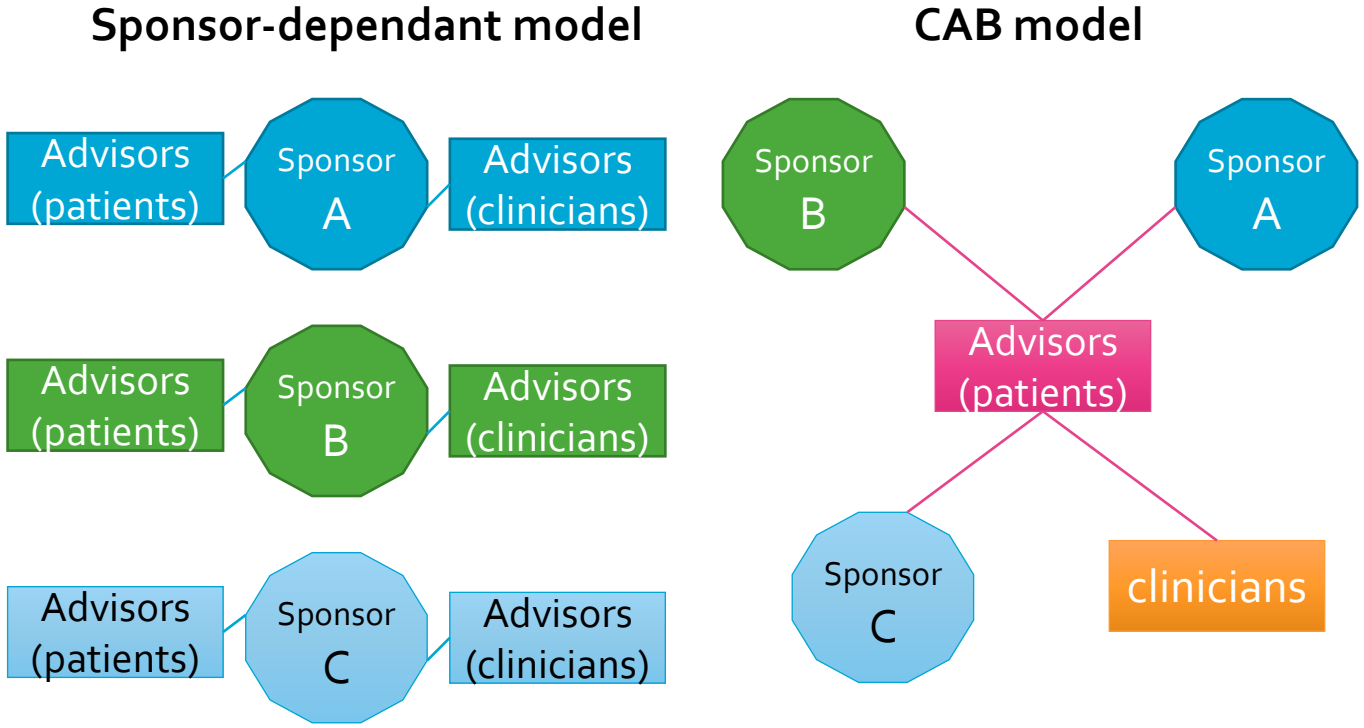
- A group of volunteer patients who offer their expertise to public or private sponsors of clinical research
  - Overall programme development
  - A single clinical trial
  - Other aspects beyond the research programme
- The same group of patients advises several sponsors in their field
  - Avoids selection of patients' representatives by the sponsor
  - Agenda and secretariat driven by the patients

Value

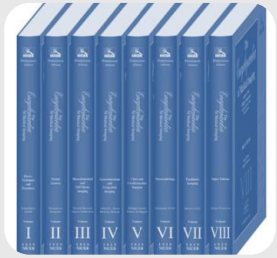
Positive

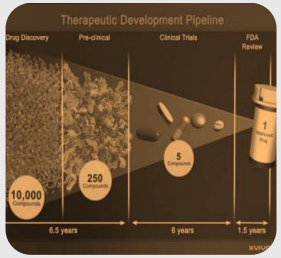
- Shorten the development time
- More patient-friendly PROMs
- Faster recruitment/less drop-outs
- Registries
- RD environment (Real-life experience + social responsibility)
- Higher quality results
  - Faster approval + market access

Same disease area, different sponsors



SCOPE





Trial protocol

All aspects, not just informed consent

Strategy trials

Collaboration with competitors

Compassionate use programme

Pipeline

drug development portfolio and plan

Reasonable pricing

World-wide Access

Patent availability to generics companies

Also Community Relations (DSMBs, Investigator Meetings), marketing practices and publicity…

EUROCAB in practice: the "patient investigator"

- Group of 10 - 16 trained patients (same disease or similar) committed to follow up the research over time
  - +16: 1 member, 1 alternate, same country
- Meet at regular intervals, same place
- EURORDIS mentor to help with the organisation, governance
- Costs borne by company/sponsor
- Charter / Memorandum of Understanding (Scope, commitment…)
- Agendas are public (transparency)
- Horizon scanning for priorities

Timeline - Example for a 2-day CAB (Arrive on Thursday evening)

Friday

Information sharing, briefing

Training, according to needs: pharmacologist, trialist…

Or Sponsor A

Saturday

Sponsor A or B

Lunch

Sponsor A or B or C

Departure

Why should CABs be part of the EUROCABs programme?

Experience: CABs benefit from experienced guidance on how to operate a CAB and do it well – we have experience!

Training: *CABs benefit from EURORDIS training (Summer School, Winter School)*

Credibility: CABs benefit from EURORDIS credibility and strong governance

Standard: CABs could qualify for EMA regulatory procedures as *patient investigators*

Up-to-date: CABs will be aware of initiatives along the products life-cycle

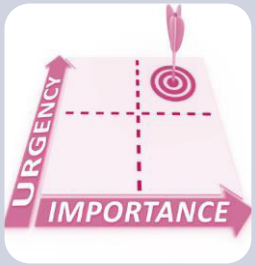
Visibility: CABs become more visible! *EUROCABs website being designed*

Which sponsors should be priority? *(on top of medical interest)*

Supports the OMP policy (orphan designation)

Requested or planning to request EMA scientific advice or protocol assistance

Requested or planning to request Early Dialogue (HTA) or parallel SA or MOCA



Applied to or part of the PRIME programme at EMA

History of submitting topics for joint HTA (EUnetHTA)

No record of bad practice

Forward ho!

- October ERTC meeting Barcelona, including financial value of listening to patients early
- CFE, Duchenne, TSC
- EUROCAB (P43 and P46)
- Monthly eMeetings
- rob.camp@eurordis.org and francois.houyez@eurordis.org