CLINICAL STUDIES DESIGNED WITH PATIENTS - THE EURORDIS COMMUNITTY ADVISORY BOARDS (CABs)

ABSTRACT: When developing a health technology requiring clinical studies, developers institute working relations with clinical investigators. Patient representatives can also create and manage advisory boards with product developers. Inspired by the model of HIV research in the 1990s, EURORDIS proposes a 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. CABs help set the agenda with the developer, work on topics such as study design, feasibility, informed consent, site selection, QoL and PROMs, and organize the meetings. Discussions cover compassionate use, pricing, relative efficacy, etc. There are confidentiality arrangements and minutes are taken. There are regular teleconferences for trainings and action plan updates.

As of 2019, 5 disease-specific CABs exist of approximately 12 members each and others are being formed. We have started to work on the metrics of markers of success. Evaluating the first surveys from 14 distinct CAB meetings with 19 companies shows that this form of shared decision-making is valuable as well as ethical for both parties.

Working relations continue, even when discussions become heated. All show interest in the co-creation possibilities of such collaboration and we look forward to measuring change via a Tracker. Patients see the utility and dedicate significant time and effort to this initiative during the year (with at least two grueling face-to-face meetings plus work and trainings in between). One CAB member commented, "CAB members can contribute quite more than I expected". One sponsor suggested that they have a "renewed enthusiasm for a patient centric research and development." This patient engagement programme, with collective thinking and exchange between patients and a collaborative mentality from both sides, ensures high quality and constructive dialogue with researchers and developers, and can eventually inform both HTA and regulatory decision-making.

Why monitoring and evaluation?

- To enhance the collaboration?
- To show that the collaboration made a difference?
- To learn about value from different perspectives?
- To understand the factors success depends on?

Analysis and use of data:

- Individual data and (success) stories?
- Average scores of all CABs?
- Compare across sponsors/CABs?
- Study the relationships between results?
- Track change over time?
- To report back to partners, internal use or also external?

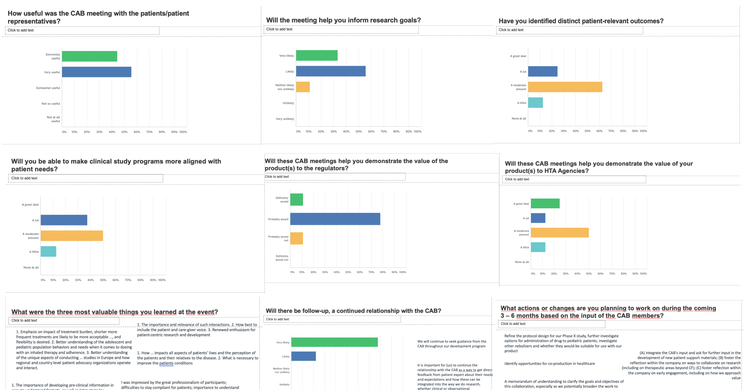
- **Expected outcomes**
  - Trial quality will improve
  - Patient interest in research will grow
  - Chances of a positive outcome of the trial/development due to
  - Better design, smarter comparator, patient-friendly practical aspects
  - Patients are retained, ie due to better info flow, better follow-up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

- **Next steps**
  - **How to measure achievements?**
  - **Are the measured outputs the correct ones?**
  - **How to verify?**

What EuroCAB patient participants feel about the collaborative activities through 2020

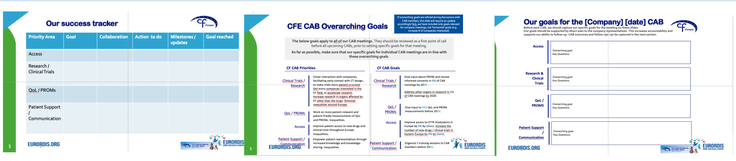
From the surveys we see a general satisfaction with the engagement. There is a broad desire to work together closer and more often on specific areas, areas that can be followed through a tool like the Tracker (see below). This requires a high level of commitment from the CAB to do this work. CABs in and of themselves are a serious committment from the patients' side to be more involved in the design, running, analysis and reporting of clinical research.

What EuroCAB sponsor participants feel about the collaborative activities through 2020



The satisfaction and desire to continue working can be clearly seen. From the sponsors' side, the compliance and legal issues of being able to follow up closely is a barrier, while in areas that can be followed over time a tool like the Tracker (see below) is used. This requires a higher level of commitment and buy-in not only from those in the room, but from decision makers who are often not in the room, along with the compliance and legal teams.

The Tracker - a tool to follow the agreed-upon changes over time



Because Clinical Research is an extended process, we need long-term documentation to record milestones and timings. The CFE CAB has developed this tool.

Some early learnings from EuroCABs thus far

EuroCAB has seen various **learnings and changes** through the meetings as well as the follow-ups. Above, in the graphs, we outline some of them: ie, the three most valuable learnings CAB members and sponsors take away from the meetings, sponsors' perceptions on if the meeting helped to inform research goals, their perceptions on ability to identify previously unknown patient-relevant outcomes and actions or changes sponsors are planning to work on in the near future. The **impacts** are more long-term but a vital part of this endeavor, where we hope to measure, amongst others, sponsors' perceptions on if the CAB meeting will enable them to make clinical study programs more aligned with patient needs, sponsors' perceptions on if the meeting helped to demonstrate the value of the product to the regulators/HTA agencies, the willingness to continue collaboration by both sides, and the change in personal life or company.

Examples of success: The **CF CAB** uses the Tracker above after each meeting, so that items can be followed up, measure how the CAB's input is used, etc. It ensures that the relationship is monitored and evaluated. They have worked extensively on reworking various Informed Consent forms as well as patient communication leaflets to various sponsors.

The **Duchenne MD CAB** has succeeded in: reducing the number of biopsies in three separate cases; reducing the number of tests and questionnaires in several studies; reducing the length of placebo to 1 year in one study, 6 months in another; improving the randomization ratio to placebo from 1:1 to 2:1, and in one case 3:1. They have convinced the majority of companies to share placebo data after the study, and to consider making individual patient data available to families.

The **Cystinosis CAB** discussed with a company the urgent necessity for their children to not stop their current successful therapy two months before administration of the investigative therapy. After some reflection the company revised the protocol where the previous therapy did not need to be stopped before day 0.

In order to do this more empirically, we need the buy-in not only of those present in the room but a long-term commitment to see this through from a corporate side. While a sponsor's goal is to get a product approved, the CAB's goal is to get the best product and information quickly and responsibly, and EURORDIS' goal is that this type of collaboration not only works but is measurable, presentable, and repeatable. In the coming months, with the help of PARADIGM, we hope to finalise the metrics and evaluation, and continue.

"(G)enerating knowledge through engagement with patients", according to Sean Wasserman, a young ally of TAC in the '90s who became an epidemiologist-turned-clinician in South Africa. "I loved the experience of generating knowledge through engagement with patients, and was immediately drawn into a new world of medical research", he says.

The Lancet, Volume 395, ISSUE 10228, P941, March 21, 2020, Sean Wasserman: rising star in TB-HIV research and medicine, Richard Lane, March 21, 2020, DOI:https://doi.org/10.1016/S0140-6736(20)30598-5

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In the past three years, EURORDIS has co-hosted 14 Community Advisory Boards with 19 sponsors on >35 products in 4 disease areas. Besides Tuberous Sclerosis Complex and Systemic Sclerosis, CABs for Cystic Fibrosis, Duchenne Muscular Dystrophy, Lymphomas, Cystinosis and HHT have all started. We expect to start new CABs in 2020.

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