THE VALUE OF PATIENT ENGAGEMENT IN RESEARCH DESIGN

The EURORDIS patient-led Advisory Boards (CABs)

Updated Abstract: Purpose: When developing a health technology that requires clinical studies, developers should involve patients in the design, execution, and analysis of clinical trials. EURORDIS has led Community Advisory Boards (CABs) that bring together patient representatives and medico developers. As of 2019, 6 disease-specific CABs exist of approximately 12 members each and others are being formed.

Methods: EURORDIS invites developers to sign a Charter for collaboration with patients in clinical research, and provides guidance on selecting the right advisory board to help shape the development strategy. The CABs provide input to the design of clinical studies, feedback, informed consent site selection, QoS, and regulatory meetings. Conclusions: So far, EURORDIS has hosted 14 Community Advisory Boards with 19 sponsors on infection. Meetings last for 2 to 4 days with sessions with different developers, all under confidentiality. There are regular between-meeting teleconferences for trainings and action plan updates, and CABs have initiated working relations with Regulators and HTA agencies. In addition, CABs have recently started to monitor outcomes of the meetings and progress towards their goals with a tracker.

Conclusion: Patient engagement should be systematically built into the development process. Here, we present a framework for patient engagement in CABs and a tracker that monitors the impact on medicine development.

Why monitoring and evaluation? • To enhance the collaboration? • To show that the collaboration made a difference? • To learn from different perspectives on value? • 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 external use?

Expected outcomes • Trial quality will improve • Patient interest in research will grow • Chanay of a positive outcome of the treatment for others • Better design, smarter comparator, patient-friendly practical aspects • Patients are retained, ie due to better info flow, better follow-up of SEs • Regulatory and HTA can make better and faster decisions [better-defined QoS, DfT, 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

Some early learnings from EuroCABs thus far:

EuroCABs have seen various learnings and changes through the meetings as well as the follow-ups. Above, in the graphs, we outline some of them: 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 implications 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. In order to do this, 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 in a quick time as a responsible, and EURODios’ goal 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 finalize the metrics and evaluation, and continue.

In the past three years, we have co-hosted 14 Community Advisory Boards with 19 sponsors on 35 projects in 4 disease areas. Besides Tuberculosis Complex and Systemic Sclerosis, CABs for Cystic Fibrosis, Duchenne Muscular Dystrophy, lymphomas and Cystinosis have all started. We expect to start (1) new CABs in 2020.

R Camp, Patient Engagement Senior Manager, EURORDIS; F Houÿez, & Health Policy Advisor, EURORDIS. #EUROCAB #ECRD2020

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

The validation and decision to consider working closely was set here. From the sponsor’s side, the compliance and legal team of being able to follow up clearly a big step forward that can be followed through the Tracker (see below). From the patient’s side, the CAB members and medicine developers, it is clear that collaboration is evaluated via a post-CAB meetings with 19 companies show that this form of shared decision was a serious commitment from the patients’ side to be more involved in and of themselves are a serious commitment from the patients’ side to be more involved in research. Inspired by this model, the European Organisation for Rare Diseases (EURORDIS) is leading the way for Cystic Fibrosis, Duchenne Muscular Dystrophy, lymphomas and Cystinosis.

From the survey we see a general satisfaction with the engagement. There is broad desire to push together and move on specific areas that can be followed through a tool like the Tracker (see below). This requires a higher level of commitment from the CAB to do this work. CABs in the future transformation are a vehicle committed to transforming the way dialogue between researchers and patients are conducted. The concept of shared decision-making is closely a reality, but in areas that can be followed over time through a tool like the Tracker (see below). This requires a higher level of commitment from the CAB to do this work. CABs in the future transformation are a vehicle committed to transforming the way dialogue between researchers and patients are conducted.

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