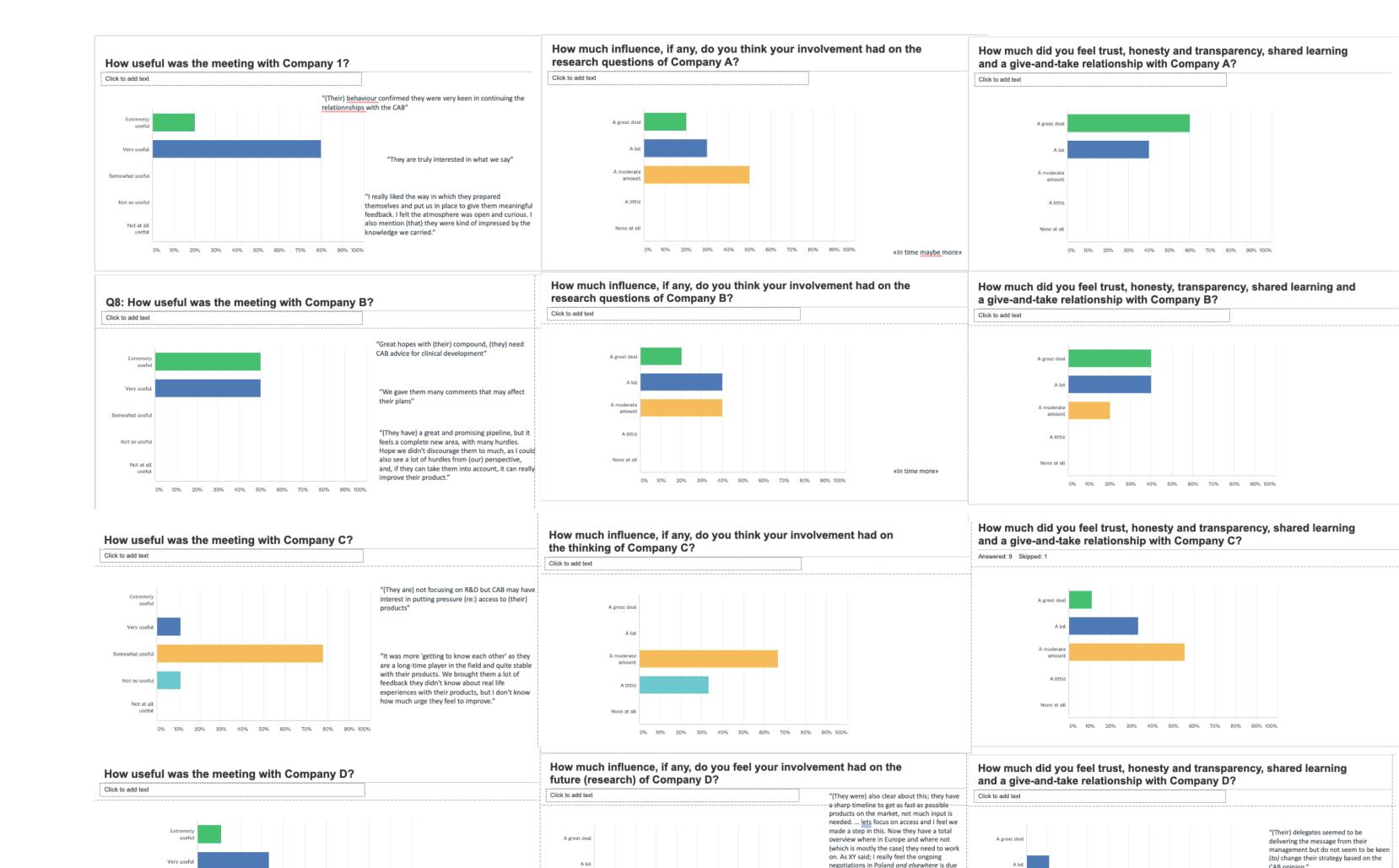
THE VALUE OF PATIENT **ENGAGEMENT IN** RESEARCH DESIGN

The EURORDIS patientled Community Advisory Boards (CABs)

Updated Abstract Purpose: When developing a health technology that requires clinical studies, developers institute working relations with clinical investigators. Patient representatives can also create and manage advisory boards with product developers. This was of high utility in the 1990s, in the development of products to treat HIV infection. Inspired by this model, the European Organisation for Rare Diseases (EURORDIS) proposes the EuroCAB programme to facilitate a two-way dialogue between patient representatives and medicine developers. As of 2019, 6 disease-specific CABs exist of approximately 12 members each and others are being formed.

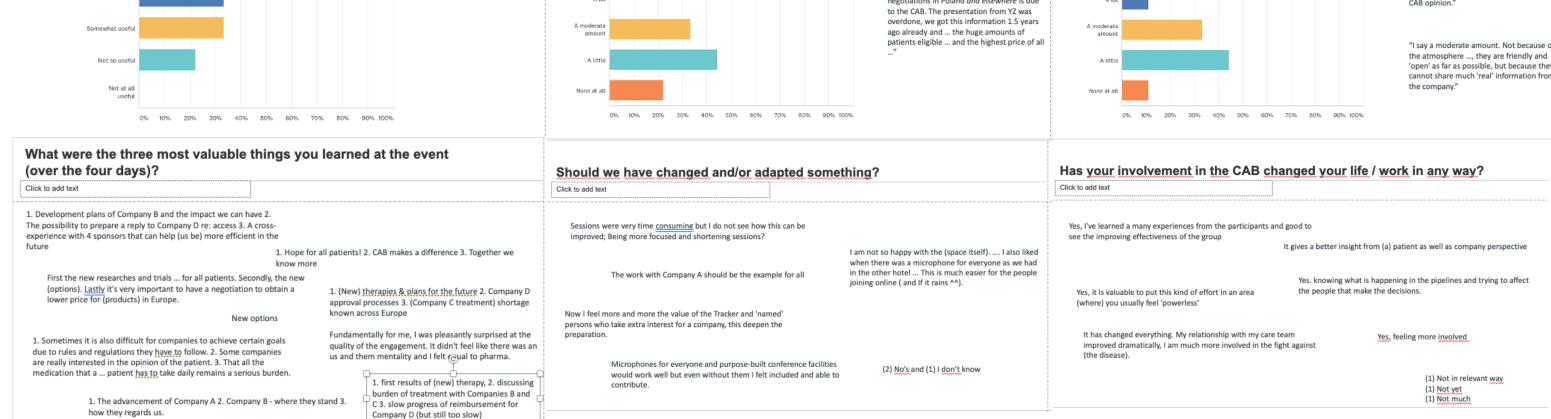
What EuroCAB patient participants feel about the collaborative activities through 2020



Methods: 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 help set the agenda with the developer, work on topics as diverse as study design, feasibility, informed consent and site selection, QoL and PROMs, and organize the meetings. Discussions also cover compassionate use, pricing, relative efficacy, etc. 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 some CABs have instituted working groups on access, psychological support, etc. The collaboration is evaluated via a post-meeting survey send to both CAB members and medicine developers. In addition, CABs have recently started to monitor outcomes of the meeting and progress towards their goals with a tracker tool.

Results: The results of the first surveys from 14 distinct CAB meetings with 19 companies show that this form of shared decision-making is valuable as well as ethical for both parties. We have seen that working relations always continue, even when discussions become heated. All involved show interest in the co-creation possibilities of such collaboration and we look forward to seeing progress and change via the tracker.

Conclusions: Monitoring and evaluation are crucial to understand whether and how the CABs are making an impact on medicine development. Demonstrating impact is challenging because of the contextualized nature and complexity inherent to patient engagement collaborations in research design. EURORDIS is working within PARADIGM on our monitoring and evaluation strategy, focusing on improving its comprehensiveness and including multi-stakeholder perspectives. Our current experiences with monitoring and evaluation show that the EuroCAB programme, with collective thinking and exchange between patients and a collaborative mentality from both sides, ensures highquality and constructive dialogue with researchers and developers and can eventually inform both HTA and regulatory decision-making.



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 higher level of commitment from the CAB side to do this work. CABs in and of themselves are a serious committment from the patients' side to be more involved in the design, carrying out, analysis and reporting of clinical research.

What EuroCAB sponsor participants feel about the collaborative activities through 2020



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

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 also external?

The satisfaction and desire to continue working is clearly seen here. From the sponsors' side, the compliance and legal issues of being able to follow up closely is 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 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



• 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, patientfriendly practical aspects
- Patients are retained, ie due to better info flow, • better follow-up of SÉs
- 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?

Because Clinical Research tends to be a long exercise, 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.

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 as quick a time as responsible, 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, 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, CABs for Cystic Fibrosis, Duchenne Muscular Dystrophy, Lymphomas and Cystinosis have all started. 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. #EUROCAB #ECRD2020

Special thanks for thoughtful input to our PARADIGM partners at VU-Athena, Amsterdam, NL