27th Workshop of the EURORDIS Round Table of Companies (ERTC)

Patient engagement in the product life-cycle and community advisory boards (CABs)

Tuesday, 16 October 2018 (09:00 to 17:00)
Recinte Modernista – Barcelona - Spain

CONCEPT PAPER

Introduction

Patient engagement is a relatively new term that can be associated with similar terms such as patient involvement, patient-centricity and patient collaboration. All of these terms often lack a clear definition and are used to label things beyond their scope.

Patient engagement is now present in the form of mobile apps, on social media, through electronic monitoring and even big data. It can also be related to improved health outcomes, on either an individual or public health level and connected to behaviour change (exercising more, smoking less).

In research, patient engagement is defined as: the meaningful involvement of patients, caregivers, clinicians, and other healthcare stakeholders throughout the research process - from topic selection through design and conduct of research to dissemination of results (according to PCORI - the Patient-Centered Outcomes Research Institute).

This definition may change over time, but it's a simple jumping-off point for our discussion on how to get patients involved in research with the sponsors of the research (companies, as well as academic researchers).

EURORDIS' role

EURORDIS has written a *Charter for Clinical Trials* that outlines a collaboration based on trust and transparency. This Charter demands nothing more of the sponsor than a commitment to work together with patient networks throughout the research and development process.

We call these particular patient networks "CABs" (Community Advisory Boards) due to their specific composition and commitment to collaboration in research. This collaboration may take specific forms and requires regular meetings (face-to-face, eMeetings, etc.) and sharing of information between the different stakeholders. What is the basis of this

collaboration? A *Memorandum of Understanding* provides guidelines about the form and objectives of the collaboration.

CABs may give input on topics agreed upon by both parties, including: real-life constraints of the participants that may be underappreciated in a first draft of a protocol; the main objectives; the quality of life questions and measurements; patient-reported vs patient-relevant outcomes and how to measure them; patient involvement in designing the true questions that are of importance to the end user; inclusion/exclusion criteria; selection of the sites; interim analyses and the writing up of results.

Both the Charter and Memorandum are available online. They are legally non-binding and are "good faith" documents that show a desire to work together.

Workshop objectives

This workshop will provide valuable information to its participants with the following objectives:

- **Understand** which best practices EURORDIS promotes for working with patients to develop orphan medicinal products
- **Understand** the objectives and outcomes of PARADIGM and how patient engagement fits therein and in the product life-cycle as a whole
- **Learn** about the concept of CABs including the method, contractual implications, limits and financial model
- **Learn** about the value that companies can get from CABs (including their monetary value) – with live testimonies of their efficacy from industry and patients
- **Learn** from the experience of existing CABs
- **Take-away** key information to share with colleagues and advocate internally to invest in CABs
- **Understand** how EURORDIS can advise and provide guidance to your company and facilitate the creation of a CAB (and what is outside of EURORDIS' scope of involvement)

This ERTC workshop will give you the opportunity to learn how CABs are contributing to meaningful medicines development and to create synergies with fellow participants.

EURORDIS CABs in practice

The CAB members themselves fit a profile – they are from the same disease area, work with national or local patient groups, have an interest in science and commit to at least two years of involvement. They will follow a rigorous online and eMeeting training guided by EURORDIS (regulatory concerns, clinical trial development, ethics, etc). The training is supplemented by professional input during the CAB meeting itself (mouse models, gene therapy, personalised medicine, etc), based on the experience of EURORDIS staff who have been involved in discussions with clinical trial sponsors for more than 100 protocols since the 1990s.

EURORDIS appreciates the complexity of this project and doesn't want to underestimate the work needed to do this and do this well. CAB chairs and co-chairs and EURORDIS mentors and sponsors can be on the phone and communicating quite regularly. Professional note-takers are vital. Logistical and practical planning are major.

Some outstanding questions EURORDIS aims to address are a behavioural best practice agreement (i.e., press releases, Early Dialogue, PRIME); availability of data (GDPR); simultaneously working with regulatory authorities and HTA mechanisms; the size and capacity of sponsors to participate ;insider trading agreements; confidentiality; access issues, patient information before and during a study and after approval; communication to patients both during a study and after a study; strategy trials (multi-company trials); the EUROCAB seal of excellence; agreement or contract between EURORDIS and the patient network, and between the patient network and the sponsor.

After experiencing the utility of CABs in other disease areas like HIV and AIDS, opportunistic diseases, hepatitis and tuberculosis, combined with a desire of sponsors in 2018 to work closer with patients in a collaborative way, we highly recommend CABs in the rare disease area. Some of the values achieved in these other disease areas can easily migrate to rare diseases, whether it be to shorten the overall development time (due to faster recruitment, fewer drop-outs), or a better measurement of value to patients and providers and payers.

We have set up two strong CABs, for Cystic Fibrosis and Duchenne Muscular Dystrophy, are preparing a number of new ones for 2019, and have experienced a keen interest for such a collaboration. We would be most interested in your concerns and desires to make this as useful a project as possible.

Beyond CABs, EURORDIS has tirelessly worked since its foundation towards consistent and meaningful engagement of patients along the whole medicines life-cycle and has played a central role in representing the patients' voice in European scientific and regulatory procedures.

Contributing to meaningful medicines development: PARADIGM

More recently, EURORDIS joined PARADIGM, a 34-partner consortium of patient and non-profit organisations, healthcare companies, regulators and academia. PARADIGM stands for Patients Active in Research and Dialogues for an Improved Generation of Medicines and is funded by the Innovative Medicines Initiative. The project aims to develop tools and resources to allow the effective and systematic inclusion of patients and to design an innovative roadmap to ensure long-term sustainability of patient engagement in three key decision-making points in medicines R&D, including research priorities setting, clinical trial design and early dialogues with regulators and HTA bodies.

PARADIGM is 'gathering' best practices in the field and the CABs are amongst them.

At the same time, PARADIGM will allow broader diffusion or adoption of these practices broadly in the ecosystem.